Appendix A: Search Strategies

Last search date: November 9, 2010

Search Strategy: PubMed/MEDLINE®

All Child: 0-18 years=3709

#107	Search #104 AND #106	17:44:42	9536
#106	Search "Humans"[Mesh]	17:29:48	10812726
#104	Search #102 AND #103	17:29:21	11232
#103	Search #55 OR #88 OR #90 OR #101	17:28:45	472713
#102	Search #45 OR #47	17:27:46	227780
#101	Search “Fabry Disease” OR “Fabry’s disease” OR “Farber Lipogranulomatosis” OR “Fabry Disease” OR “Fabry’s disease” OR “Farber Lipogranulomatosis” OR Gangliosidos* OR “Sandhoff Disease” OR “sandhoff’s disease” OR “Gaucher Disease” OR “gaucher’s disease” OR “Niemann-Pick Disease*” OR “Tay-Sachs Disease” OR Aspartylglucosaminuria OR “beta-Mannosidosis” OR Mucolipidos* OR “Wolman Disease” OR “Ceroid Lipofuscinos*” OR “Ceroid-Lipofuscinos*” OR galactosialidosis OR Cystinosis OR “Sialic Acid Storage Disease” OR “salla disease” OR “peroxisomal storage disorder*” OR adrenomyeloneuropath* OR “immune cytopenia*”	17:27:12	12149
#90	Search "Ewing’s Sarcoma" OR "Wilms Tumor" OR Rhabdomyosarcoma* OR Retinoblastoma* OR Medulloblastoma* OR PNET OR "Primitive Neuroectodermal Tumor*" OR Astrocytoma* OR Mucopolysaccharidos* OR Sphingolipidos* OR "Lysosomal Storage Disease*" OR "Glycogen Storage Disease*" OR "Niemann-Pick Disease*" OR Adrenoleukodystrophy OR "Juvenile Rheumatoid Arthritis" OR "Systemic Lupus Erythematosus" OR SLE OR Scleroderma OR "Crohn Disease" OR “Crohn’s disease” OR "Autoimmune Disease*"	17:12:00	199885
#88	Search (((((((("Mucopolysaccharidoses"[Mesh] OR "Sphingolipidoses"[Mesh]) OR "Lysosomal Storage Diseases"[Mesh]) OR "Glycogen Storage Disease"[Mesh]) OR "Niemann-Pick Diseases"[Mesh]) OR "Adrenoleukodystrophy"[Mesh]) OR "Arthritis, Juvenile Rheumatoid"[Mesh]) OR "Lupus Erythematosus, Systemic"[Mesh]) OR "Scleroderma, Systemic"[Mesh]) OR "Crohn Disease"[Mesh]) OR "Autoimmune Diseases"[Mesh]	17:03:42	366524
#55	Search (((("Sarcoma, Ewing's"[Mesh] OR "Wilms Tumor"[Mesh]) OR "Rhabdomyosarcoma"[Mesh]) OR "Retinoblastoma"[Mesh]) OR "Medulloblastoma"[Mesh]) OR "Neuroectodermal Tumors, Primitive"[Mesh]) OR "Astrocytoma"[Mesh]	16:48:48	67468
#47	Search "stem cell*" OR "bone marrow"	16:46:12	227780
#45	Search "Bone Marrow Transplantation"[Mesh] OR ("Hematopoietic Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell	16:37:28	66363

Transplantation"[Mesh] OR "Stem Cell Transplantation"[Mesh])

Additional searches were performed using

"stem cell*" OR "bone marrow"
Search "Bone Marrow Transplantation"[Mesh] OR ("Hematopoietic Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Stem Cell Transplantation"[Mesh]) OR "stem cell*" OR "bone marrow"

AND

"Precursor Cell Lymphoblastic Leukemia-Lymphoma"[Mesh] OR "Leukemia, Myeloid, Acute"[Mesh] OR "acute lymphoblastic leukemia" OR acute myeloid leukemia"

"Lymphoma, Non-Hodgkin"[Mesh] OR "non-Hodgkin* lymphoma*"

"Hodgkin Disease"[Mesh] OR "hodgkin lymphoma"

"Leukemia, Myelomonocytic, Juvenile"[Mesh] OR "juvenile myelomonocytic leukemia"

"Leukemia, Myelogenous, Chronic, BCR-ABL Positive"[Mesh] OR "chronic myelogenous leukemia"

"Myelodysplastic-Myeloproliferative Diseases"[Mesh] OR "myelodysplastic disease*"

"Neuroblastoma"[Mesh] OR neuroblastoma*

"Leukodystrophy, Globoid Cell"[Mesh] OR "globoid leukodystrophy"

"Leukodystrophy, Metachromatic"[Mesh] OR "metachromatic leukodystrophy"

"Fucosidosis"[Mesh] OR fucosidosis

"alpha-Mannosidosis"[Mesh] OR "alpha-mannosidosis" OR "alpha-mannosidoses"

"Peroxisomal Disorders"[Mesh] OR "peroxisomal storage disorder*" OR adrenoleukodystroph*

"Osteopetrosis"[Mesh] OR osteopetrosis

“bone marrow failure” OR "Fanconi Anemia"[Mesh] OR "Fanconi* anemia" OR "Dyskeratosis Congenita"[Mesh] OR "dyskeratosis congenita" OR “Shwachman-Diamond” OR "Anemia, Diamond-Blackfan"[Mesh] OR "Diamond-Blackfan" OR "Diamond Blackfan"

"Ependymoma"[Mesh] OR ependymoma*

"Glioma"[Mesh] OR glioma*

"Choroid Plexus Neoplasms"[Mesh] OR ("choroid plexus" AND (tumor OR tumour OR tumors OR tumours OR neoplasm*))

medulloepithelioma* OR (supratentorial AND (PNET OR "primitive neuroectodermal")) OR pineoblastoma* OR "cerebral neuroblastoma*" OR ganglioneuroblastoma* OR ependymoblastoma* OR "atypical teratoid/rhabdoid tumor*" OR "Pinealoma"[Mesh] OR ("Rhabdoid Tumor"[Mesh] AND atypical AND teratoid*) OR "Astrocytoma"[Mesh] OR "Oligodendroglioma"[Mesh] OR astrocytoma* OR oligodendroglioma* OR "glioblastoma multiforme"

"Diabetes Mellitus, Type 1"[Mesh] OR ("type 1" AND (diabetes OR diabetic OR DM)) OR "juvenile diabetes"

"Neoplasms, Germ Cell and Embryonal"[Mesh] AND germ) OR "germ cell tumor*"

Searches were also performed in EMBASE and the Cochrane Central Register of Controlled Trials for the above disease entities.

Additional searches were performed for the disease entities above and NOT the stem cell transplantation set to obtain literature on the therapeutic measures to be used as comparisons.

Diabetes

#15	Search (#10 AND #13) NOT #5 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	10:23:50	82
#14	Search (#10 AND #13) NOT #5	10:21:22	3314
#13	Search "Immunosuppression"[Mesh] OR immunomodulation OR immunosuppressant OR immunosuppressive OR "immune modulation" OR "immune suppression"	10:20:45	449395
#10	Search "Diabetes Mellitus, Type 1"[Mesh] OR ("type 1" AND (diabetes OR diabetic OR DM)) OR "juvenile diabetes"	10:17:59	60849
#5	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	10:16:06	73273

Other Autoimmune Diseases

#23	Search (#20 AND #13) NOT #5 AND (severe OR refractory OR "poor prognosis") Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	10:31:11	184
#22	Search (#20 AND #13) NOT #5 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	10:30:15	650
#21	Search (#20 AND #13) NOT #5	10:29:02	8441

#20	Search (("Arthritis, Juvenile Rheumatoid"[Mesh] OR "Lupus Erythematosus, Systemic"[Mesh]) OR "Scleroderma, Systemic"[Mesh]) OR "Crohn Disease"[Mesh]	10:27:58	84547
#13	Search "Immunosuppression"[Mesh] OR immunomodulation OR immunosuppressant OR immunosuppressive OR "immune modulation" OR "immune suppression"	10:20:45	449395
#5	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	10:16:06	73273

Ewing's Sarcoma

#42	Search #40 NOT #5 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	10:45:50	191
#41	Search #40 NOT #5	10:44:21	1548
#40	Search (#27 AND #39) AND #32	10:44:03	1668
#39	Search (("Recurrence"[Mesh] OR "Neoplasm Recurrence, Local"[Mesh])) OR "secondary "[Subheading] OR recurrent OR recurrence OR (stage AND IV) OR secondary OR metastatic OR metastas*	10:43:30	997415
#32	Search "therapeutic use "[Subheading] OR "therapy "[Subheading] OR therapy OR treatment OR therapeutic*	10:39:44	7369913
#27	Search "Sarcoma, Ewing's"[Mesh] OR (Ewing* AND sarcoma)	10:34:48	6400
#5	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	10:16:06	73273

Wilms Tumor

#52	Search #50 NOT #5 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	10:54:02	198
#51	Search #50 NOT #5	10:52:09	1869
#50	Search (#48 AND #49) AND #32	10:51:45	1961
#49	Search (("Recurrence"[Mesh] OR "Neoplasm Recurrence, Local"[Mesh])) OR "secondary "[Subheading] OR recurrent OR recurrence OR (stage AND IV) OR secondary OR metastatic OR metastas* OR "unfavorable histology" OR relapse OR relapsed	10:51:09	1034703
#48	Search "Wilms Tumor"[Mesh] OR (wilm* AND (tumor OR tumors OR	10:49:05	11596

tumour*))

#32	Search "therapeutic use "[Subheading] OR "therapy "[Subheading] OR therapy OR treatment OR therapeutic*	10:39:44	7369913
#5	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	10:16:06	73273

Rhabdomyosarcoma

#60	Search #58 NOT #5 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	11:00:54	217
#59	Search #58 NOT #5	10:59:18	1362
#58	Search (#56 AND #57) AND #32	10:59:03	1437
#57	Search (("Recurrence"[Mesh] OR "Neoplasm Recurrence, Local"[Mesh])) OR "secondary "[Subheading] OR relapse OR relapsed OR refractory OR "high-risk" OR extraocular OR recurrent OR recurrence	10:58:29	666401
#56	Search "Rhabdomyosarcoma"[Mesh] OR rhabdomyosarcoma*	10:56:51	10678
#32	Search "therapeutic use "[Subheading] OR "therapy "[Subheading] OR therapy OR treatment OR therapeutic*	10:39:44	7369913
#5	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	10:16:06	73273

Retinoblastoma

#67	Search #65 NOT #5 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	11:10:56	86
#66	Search #65 NOT #5	11:09:35	654
#65	Search (#64 AND #57) AND #32	11:09:12	679
#64	Search "Retinoblastoma"[Mesh] OR retinoblastoma*	11:08:08	15967
#57	Search (("Recurrence"[Mesh] OR "Neoplasm Recurrence, Local"[Mesh])) OR "secondary "[Subheading] OR relapse OR relapsed OR refractory OR "high-risk" OR extraocular OR recurrent OR recurrence	10:58:29	666401
#32	Search "therapeutic use "[Subheading] OR "therapy "[Subheading] OR therapy OR treatment OR therapeutic*	10:39:44	7369913

#5	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	10:16:06	73273

Germ Cell Tumors

#74	Search #72 NOT #5 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	11:24:57	254
#73	Search #72 NOT #5	11:24:57	3127
#72	Search (#70 AND #49) AND #32	11:20:04	3295
#70	Search ("Neoplasms, Germ Cell and Embryonal"[Mesh] AND germ) OR "germ cell tumor*"	11:18:53	11580
#49	Search (("Recurrence"[Mesh] OR "Neoplasm Recurrence, Local"[Mesh])) OR "secondary "[Subheading] OR recurrent OR recurrence OR (stage AND IV) OR secondary OR metastatic OR metastas* OR "unfavorable histology" OR relapse OR relapsed	10:51:09	1034703
#32	Search "therapeutic use "[Subheading] OR "therapy "[Subheading] OR therapy OR treatment OR therapeutic*	10:39:44	7369913
#5	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	10:16:06	73273

CNS Embryonal Tumors

#121	Search #120 NOT #117 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	14:00:56	276
#120	Search (#110 AND #49) AND #32 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	13:59:47	301
#117	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	13:57:12	73273
#110	Search medulloblastoma* OR medulloepithelioma* OR (supratentorial	13:52:54	7707

	AND (PNET OR "primitive neuroectodermal")) OR pineoblastoma* OR "cerebral neuroblastoma*" OR ganglioneuroblastoma* OR ependymoblastoma* OR "atypical teratoid/rhabdoid tumor*"		
#49	Search (("Recurrence"[Mesh] OR "Neoplasm Recurrence, Local"[Mesh])) OR "secondary "[Subheading] OR recurrent OR recurrence OR (stage AND IV) OR secondary OR metastatic OR metastas* OR "unfavorable histology" OR relapse OR relapsed	10:51:09	1034703
#32	Search "therapeutic use "[Subheading] OR "therapy "[Subheading] OR therapy OR treatment OR therapeutic*	10:39:44	7369913

CNS Glial Tumors

#131	Search #129 NOT #117 Limits: Humans, Clinical Trial, Editorial, Practice Guideline, Randomized Controlled Trial, Case Reports, Comparative Study, Controlled Clinical Trial, Guideline, English, All Infant: birth-23 months, All Child: 0-18 years, Publication Date from 1995/10/01 to 2010/07/12	14:27:12	251
#129	Search (#126 AND #128) AND #32	14:25:22	2586
#128	Search ("Recurrence"[Mesh] OR "Neoplasm Recurrence, Local"[Mesh]) OR relapse OR relapsed OR recurrent OR recurrence OR "high-risk"	14:24:54	526384
#126	Search "Astrocytoma"[Mesh] OR "Oligodendroglioma"[Mesh] OR astrocytoma* OR oligodendroglioma* OR "glioblastoma multiforme"	14:22:55	26291
#32	Search "therapeutic use "[Subheading] OR "therapy "[Subheading] OR therapy OR treatment OR therapeutic*	14:21:03	7369913
#117	Search "Bone Marrow Transplantation"[Mesh] OR ("Stem Cell Transplantation"[Mesh] OR "Peripheral Blood Stem Cell Transplantation"[Mesh] OR "Cord Blood Stem Cell Transplantation"[Mesh] OR "Hematopoietic Stem Cell Transplantation"[Mesh]) OR "bone marrow transplant*" OR "stem cell support" OR "stem cell transplant*"	13:57:12	73273

Appendix B. Excluded Studies

Retrieval Code (field 12)

DNG	do not retrieve full copy
GET	retrieve full copy
UNC	uncertain; needs check by second reviewer

Two Arms of Study Code (field 12)

NAR	narrative review portion
SYS	systematic review portion

Selection Decision Code

(after reviewing retrieved article, enter into field 12)

INC	include
EXC	exclude (with codes for exclusion reasons)

Full Review Codes (field 42)

I. Key Question Codes

NRQ	not relevant question (note if ANM, NDE, NRD, NRI, NRO)
Q1	comparative benefits HSCT vs Ctx in solid tumors
Q2	comparative harms HSCT vs Ctx in solid tumors
Q3	comparative benefits HSCT vs other Tx in IMD
Q4	comparative harms HSCT vs other Tx in IMD
Q5	comparative benefits HSCT vs other Tx in autoimmune diseases
Q6	comparative harms HSCT vs other Tx in autoimmune diseases
Q#?	unclear if relevant to any key question

II. Study Design Codes

ADB	administrative database
ANM	animal study
CEA	cost/cost-effectiveness analysis
CCS	case-control study
COH	cohort study
COM	commentary
CR	case report ($n \leq 5$)
CS	case series
D?	design unclear/possibly relevant
DAC	diagnostic accuracy study
DUP	duplicated patient population
EDT	editorial
FLA	foreign language article
GUI	guideline
INV	in vitro
LTR	letter
MA	meta-analysis
NAB	no abstract
NPC	not relevant comparator
NPD	no primary data
NRD	not relevant disease
NR	not relevant disease b/c part of narrative review
NRE	not relevant design
NRI	not relevant intervention
NRO	not relevant outcome (or no follow-up)
NRP	not relevant population
NRS	not relevant study
PI	phase I trial
PII	phase II trial
PRG	prognostic study
PRO	prospective single-arm study
QEX	quasi-experimental study (nonrandomized comparative)
RAD	radiology study
RCT	randomized controlled trial
REG	registry
RET	retrospective study
REV	review article
SR	systematic review
STG	disease staging study
XSL	cross-sectional study

III. Sample Size Code (single-arm only)

FEW	$n < 10$
N10	$10 \leq n < 25$
N25	$25 \leq n < 50$
N50	$50 \leq n < 100$
N100	$n \geq 100$
N?	n unclear

IV. Basic Disease Codes

AID	Autoimmune disease
ALD	adrenoleukodystrophy
ALL	acute lymphoblastic leukemia
AMA	alpha-mannosidosis
AML	acute myelogenous leukemia
AMN	adrenomyeloneuropathy
ASP	aspartylglucosaminuria
AST	astrocytoma
BMA	beta-mannosidosis
BMF	bone marrow failure
CLF	ceroid lipofuscinosis
CLL	chronic lymphocytic leukemia
CML	chronic myelogenous leukemia
CNS	CNS tumors, NOS
CRN	Crohn's
CYS	cystinosis
DME	Diabetes mellitus type 1
DNS	disease not specified
ESF	Ewing/Ewing sarcoma family of tumors
ENV	Evan's syndrome
FAB	Fabry disease
FAR	Farber disease
FUC	Fucosidosis
GAL	galactosialidosis
GAUI	Gaucher I
GAUII	Gaucher II
GAUIII	Gaucher III
GCT	germ cell tumor
GLD	globoid leukodystrophy
GM1	GM_1 gangliosidosis
GSDII	glycogen storage disease II
HGB	hemoglobinopathy
HL	Hodgkin lymphoma
IBD	inflammatory bowel disease
ICP	immune cytopenia
IMD	inherited metabolic disease
JML	juvenile myelomonocytic leukemia
JRA	juvenile rheumatoid arthritis
MDS	myelodysplasia
MED	medulloblastoma
MLII	mucolipidosis II
MLIII	mucolipidosis III

MLIV	mucolipidosis IV
MLD	metachromatic leukodystrophy
MPSI	Hurler syndrome
MPSII	Hunter syndrome
MPSIII	Sanfilippo syndrome
MPSIV	Morquio syndrome
MPSVI	Maroteaux-Lamy syndrome
MPSVII	Sly syndrome
NBL	neuroblastoma
NHL	non-Hodgkin lymphoma
NPA	Niemann-Pick A
NPB	Niemann-Pick B
NPC	Niemann-Pick C
OSC	osteosarcoma
OST	osteopetrosis
PID	primary immune deficiency
PNET	primitive neuroectodermal tumor
RBA	retinoblastoma
RMS	rhabdomyosarcoma
SAL	Salla disease
SAN	Sandhoff disease
SAS	sialic acid storage disease
SCL	scleroderma/SS
SLE	systemic lupus erythematosus
STG	stage of disease
STS	soft tissue sarcoma
TAY	Tay-Sachs disease
WIL	Wilms tumor
WOL	Wolman disease

IV. Disease code modifiers

HR	high risk
LR	low risk
MET	metastatic
NEW	newly diagnosed
PRD	progressive disease
REC	recurrent disease
REF	refractory
REL	relapsed
REM	remission
SEV	severe
STG	stage of disease

V. Disease Code Characteristics

GRW	growth
HRD	hearing defects
HSM	hepatosplenomegaly
IQ	intelligence quotient
JNT	joint
MR	mental retardation
NCD	neurocognitive development
NMD	neuromuscular development
ORT	orthopedic/skeletal
SOH	state of health
SPE	speech
SZS	seizures

V. Intervention Codes

AUT	autologous HSCT
ALO	allogeneic HSCT
BSC	best supportive care
CHM	chemotherapy
CRT	chemoradiotherapy
ERT	enzyme replacement therapy
HSCT	hematopoietic stem cell transplantation
IMM	immune suppression
INS	insulin therapy
PAL	palliative
PRI	primary treatment (previously untreated)
SEQ	sequential high-dose chemotherapy with single autologous HSCT
SUR	surgery only
T?	treatment unclear
TAN	tandem autologous HSCT
TBI	total body irradiation
UMB	umbilical cord blood HSCT

VI. Outcome Codes

CNR	continuous remission
CRM	complete remission
CVS	cardiovascular AE
DFR	drug free remission
DFS	disease-free survival
DSS	(cancer) disease-specific survival
EFS	event free survival
ENG	engraftment
ESO	esophagus AEs
FU?	follow-up uncertain
GVH	graft versus host disease
INF	infection
HEM	hematologic toxicities
HEP	hepatic AEs
HRT	heart AEs
LC	local control
LNG	lung AEs
LRC	locoregional control
MFS	(distant) metastasis-free survival
MIR	minor response
MUC	mucositis
NV	nausea/vomiting
OAE	other AE
OS	overall survival
OTE	other time-to-event outcome
OTO	otologic/auditory AEs
O?	outcome unclear
PFS	progression-free survival
PR	partial remission
PRD	progressive disease
QOL	quality of life
RFS	recurrence free survival
REN	renal toxicities
RET	retinopathy
RSP	tumor response
SEL	serum enzyme levels
SKN	skin AEs
STD	stable disease
TAE	toxicty/adverse events (not specified)
TRM	treatment-related mortality
TTR	time-to-recurrence
VPR	very good partial response

[No Author]. Effect of intensive diabetes treatment on the development and progression of long-term complications in adolescents with insulin-dependent diabetes mellitus: Diabetes Control and Complications Trial. Diabetes Control and Complications Trial Research Grou. J Pediatr 1994 125(2):177-88.
Rec#: 75370
Reprint: exc nrp

[No Author]. Adverse events and their association with treatment regimens in the diabetes control and complications trial. Diabetes Care 1995 18(11):1415-27.
Rec#: 62830
Reprint: exc nrp

[No Author]. Early worsening of diabetic retinopathy in the Diabetes Control and Complications Trial. Arch Ophthalmol 1998 116(7):874-86.
Rec#: 62640
Reprint: exc nrp

[No Author]. Effect of intensive therapy on residual beta-cell function in patients with type 1 diabetes in the diabetes control and complications trial. A randomized, controlled trial. The Diabetes Control and Complications Trial Research Group. Ann Intern Med 1998 128(7):517-23.
Rec#: 62660
Reprint: exc nrp

[No Author]. Retinopathy and nephropathy in patients with type 1 diabetes four years after a trial of intensive therapy. The Diabetes Control and Complications Trial/Epidemiology of Diabetes Interventions and Complications Research Group. N Engl J Med 2000 342(6):381-9.
Rec#: 62520
Reprint: exc nrp

Abbott, I. R., S. J. Gaskill, T. C. Hayes, and B. J. Menick. 28-Month-Old girl with delayed ambulation and speech. Pediatr Neurosurg 2000 33(3):162-6.
Rec#: 76430
Reprint: exc nro

Abd El-Aal, H. Role of radio-iodinated meta-iodo benzyl guanidine in assessment of children with neuroblastoma at NEMROCK. J Egypt Natl Canc Inst 2006 18(4):375-81.
Rec#: 2370
Reprint: exc nrp

Abdel-Haq, N., S. Savasan, M. Davis, B. I. Asmar, T. Painter, and H. Salimnia. Asaia lannaensis bloodstream infection in a child with cancer and bone marrow transplantation. 2009.
Rec#: 110
Reprint:

Aberg, L., E. Kirveskari, and P. Santavuori. Lamotrigine therapy in juvenile neuronal ceroid lipofuscinosis. Epilepsia 1999 40(6):796-9.
Rec#: 58000
Reprint: exc nri

Aberg, L. E., M. Backman, E. Kirveskari, and P. Santavuori. Epilepsy and antiepileptic drug therapy in juvenile neuronal ceroid lipofuscinosis. Epilepsia 2000 41(10):1296-302.
Rec#: 57870
Reprint: exc nri

Adams, C., C. S. August, H. Maguire, and J. T. Sladky. Neuromuscular complications of bone marrow transplantation. Pediatr Neurol 1995 12(1):58-61.
Rec#: 22300
Reprint: EXC NRS

Adkins, E. S., R. Sawin, R. B. Gerbing, W. B. London, K. K. Matthay, and G. M. Haase. Efficacy of complete resection for high-risk neuroblastoma: a Children's Cancer Group study. J Pediatr Surg 2004 39(6):931-6.
Rec#: 8730
Reprint: exc nri

Al Salloum, A. A. Cyclophosphamide therapy for lupus nephritis: poor renal survival in Arab children. Pediatr Nephrol 2003 18(4):357-61.
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Appendix C: Systematic Review Data Abstraction

Appendix Table C1: Design, Participant Selection and Enrollment Ewing's Tumors

Study (Investigator, country, record.)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Bernstein, USA/Canada 2006	6290	malig nh	esft	mets at dx	110		phase 2 study	110	0	
Bhatia,USA, 2007	43210	malgi nh	esft	metastatic dz	60	1992-1994	Children's Oncology Group therapeutic protocol	60		protocol also included less intense CT regimens for other subgroups- not abstracted
Burdach, Germany and Austria, 2000	14310	malig nh	esft	relapse (early, late or multiple) or primary multifocal disease	28	1986-1994	cs	28	0	
Burdach, Germany, 2003	10030	malig nh	esft	primary multifocal or early relapse	32	1986-1994 (single HSCT) 1995-2000 (tandem HSCT)	Comparative study using historical controls	32		study included 54 patients who underwent single or tandem HSCT and survival reported as <=17 yrs of age or >17
Burke, USA 2007	4060	malig nh	esft	"high risk" defined as pelvic primary (n=5) and/or mets (n=4)	7	1992-2003	consecutive pts with es	7	0	One pt age 23 not abstracted
Costa, USA, 2008	1710	malig nh	esft	NR	1	2000-2007	CR	1	0	
Drabko, Poland 2005	6680	malig nh	esft	"high risk"- 1st line therapy with mets or relapse	21	1996-2002	cs from two centers	21	0	
Fazekas, Austria, 2008	2720	m nh	esft	CR1	1		CR	1	0	

Study (Investigator, country, record.)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Hara , japan 1998	17950	malig nh	esft	advanced dz or CT-resistant or relapse relapse n=1	3	1993-1997	cs	3	0	
Harimaya, Japan, 2003	9850	malig nh	esft	primary dx, high-risk (spinal column)	2		cs	2	0	
Hawkins, USA 2000	15360	malig nh	esft	grp 1: CR2 n=8 PR2 n=1 grp 2: CR1 n=2 CR2 n=2 CR3 n=1 PD n=2	grp 1 n=9 grp 2 n=7	1993-1997	cs	16	0	2 different conditioning regimens used grp 1 and grp 2
Kasper, Germany, 2006	2570	malig nh	esft	upfront therapy, 3 with mets transplanted in CR n=4 and PR n=1	5	1998-2004	cs	5	0	other patients >21 yrs not abstracted
Kogawa, Japan, 2004	8410	malig nh	esft	primary dx	1		cr	1	0	
Koscielniak Germany 2005	7860	m nh	esft	relapsed after tandem auto-auto HSCT	1	1998	CR	1	0	
Kushner, USA, 1995	21430	malig nh	esft	newly dx'd poor risk b/c of tumor volume >100 cm3 or mets to bone or BM	24		Prospective CS	24	0	
Kushner, USA, 2001	14240	malig nh	esft	newly diagnosed with mets to bone or BM - if achieve VGPR or CR, eligible for HSCT	10	1990-1998	cs	10	0	only abstracted pts <21 for HSCT and the 5 pts <21 who did not proceed to HSCT

Study (Investigator, country, record.)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Laws, Germany, 2003	9450	malig nh	esft	relapsed	2	1988-1998	cr	2	0	study included a total of 18 patients, but age was only reported for 2 pts
lucas, usa 2008	2450	malig nh	esft	relapsed with mets	1		CR	1	0	
Lucidarme, france, 1998	17610	malig nh	esft	refractory dz 1st PR n=1 2nd PR n=1 PD n=1	3	1987-1995	phase 2 study	3	0	
Meyers, USA, 2001	13670	malig NH	ESFT	newly diagnosed metastatic to bone and/or BM	32	Feb 1996-Nov 1998	CS	32	9 patients did not proceed to HSCT b/c 4 had progressive dz, 2 secondary to toxicity and 3 who died from toxicity during high-dose phase of the therapy.	
Milano, Italy, 2006	43290	malig nh	esft		36	1990-2005				
Navid, US and Canada, 2006	5930	malig nh	esft	mets or tumor >8cm	11	1996-2000	prospective phase II trial	9	2	
Numata, Japan, 2002	12130	malig nh	esft	primary dx	1	May 1996	CR	1	0	

Study (Investigator, country, record.)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Oberlin, France, 2008	46850	malig nh	esft	newly dxd with mets		1991-1999	cs			study transplanted 75 patients; survival data reported as <15 yrs of age and >=15; only abstracted <15 yr data
ozkaynak, usa 1998	18540	malig nh	esft	2nd CR n=4 2nd VGPR n=5 1st CR n=5 1st VGPR n=1 (5 pts transplanted in 1st CR or VGPR were high-risk- 4 with mets at dx bone and/or BM and one had large pelvic primary)	15	1992-1995	cs	15	0	
pession, italy, 1999	16120	malig nh	esft	CR 2 n=2 PR n=1	3	1992-1994	cs	3	0	
Prete, italy 1998	17210	malig nh	esft	"high risk" (large pelvic mass and/or metastatic dz)	17	1993-1997	cs	17	0	

Study (Investigator, country, record.)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Sari, Turkey, 2010	42790	malig nh	esft	mets at dx	36	1992-2005	CS	36	0	study included pts with nonmet dz- did not abstract b/c only survival report for high risk pts was by mets vs nonmets
Tanaka, Japan, 2002	11770	malig NH	ESFT	PD n=1 CR1 n=5	6	"HSCT since 1986"	CS	6		one patient 35 y/o not abstracted
van winkle, USA, 2005	43550	malig nh	esft	recurrent/refractory	22	1992-1996	CS	22	0	
Yaniv, Israel, 2004	9100	malign nh	esft	"high risk" mets at dx, poor response to CT defined as <90% necrosis at definitive surgery, primary tumor not resecAppendix Table 1, relapsed	11			11	0	

Appendix Table C2: Participant Characteristics Treatment Ewing's Tumors

Study (Investigator, country, record.)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Burdach, Germany and Austria, 2000	14310	28		at tx 15 yrs (8-21)		50,50		primary site for relapsed: long bone n=9 pelvis n=1 scapula n=1 chest wall/rib n=1 primary site for multifocal disease: various	entire study incl 36 pts; only abstracted <21 yrs old
Burdach, Germany, 2003	10030	32			<= 17 yrs				
Burke, USA 2007	4060	7		14 yrs (.5-17)		71, 29	mets in 4 (lung n=2, bone BM and liver n=1, bone and lung n=1)	primary tumor site pelvis n=5 scapula n=1 chest wall n=1	
Costa, USA, 2008	1710	1	15 yrs at first HSCT			NR	NR	es	
Drabko, Poland 2005	6680	21		at tx 15 yrs (6-21)		52,48	at HSCT: CR1 n=10 CR2 n=1 PD n=1 PR n=9	pelvis n=3 long bone n=9 vertebra n=1 sternum or clavicle n=3 scapula n=1 rib n=1 skull n=1 NR n=2	
Fazekas, Austria, 2008	2720	1	13 yrs at diagnosis			100,0	stage IV	pelvis	
Hara , japan 1998	17950	3		5 yrs (2-12)			relapsed n=1 stage 3 n=1 stage 4 n=1	PNET n=2 ES n=1	
Harimaya, Japan, 2003	9850	2	12yrs and 14yrs			50,50	localized to spinal column		

Study (Investigator, country, record.)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Hawkins, USA 2000	15360	16		at HSCT 14.6 yrs (6-21)				grp 1 2/9 with mets; primary tumor site long bone n=5, axial n=3, kidney n=1 grp 2: 4/7 mets; primary tumor site long bone n=2 axial n=5	
Kasper, Germany, 2006	2570	5		at HSCT 19 (17-21)				mets: lung n=2 bone n=1	
Kogawa, Japan, 2004	8410	1	7 yrs			0,100		primary cervical spine, epidural, extra-osseous	
Koscielniak Germany 2005	7860	1	15 YEARS AT TANDEM TX 15 yrs + 8 mos at relapse/allo			0,100	initial stage - disseminated dz with BM mets		
Kushner, USA, 2001	14240	5		16.5 y (8-21 yrs)		70,30	mets to bone or BM	primary site of tumor pelvis n=4 long bone n=3 chest wall n=1 paraspinal n=1 perineum n=1	
Laws, Germany, 2003	9450	2			at HSCT 14 yrs and 19 yrs	0,100	relapsed	primary tumor femur n=2	
lucas, usa 2008	2450	1	4 YRS			0,100	stage IV	iliac crest with mets to BM, multiple vertebrae, ribs, bilateral lung	
Lucidarme, france, 1998	17610	3		8.5 yrs (2-17) at 1st tx		68, 32	mets at HSCT n=3		age is median for all 22 patients in this mixed study

Study (Investigator, country, record.)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Meyers, USA, 2001	13670	32		13 yrs (1-22)		62,38	metastatic to bone or BM	primary site: pelvis n=12 chest wall n=5 femur n=3 multiple sites n=6 other n=6	
Navid, US and Canada, 2006	5930	9		14.9 yrs (11.7-17.4 yrs)		67, 33	nomets n=3 mets n=6)5 underwent HSCT	HSCT pubis n=1 kidney n=1 chest wall n=1 femur n=1 rib n=1 no HSCT ilium n=1 thorax n=1 leg n=1 rib n=1	
Numata, Japan, 2002	12130	1	20 years at HSCT			0,100		ES- inguinal area	
Oberlin, France, 2008	46850			12.3 yrs (2 m0s-25 yrs))		59,41			
ozkaynak, usa 1998	18540			15 (5-21)		53,47			
pession, italy, 1999	16120	3		6 yrs (3-12)		33,66			
Prete, italy 1998	17210			8 (5-14)		65,35	metastatic dz at dx n=14 localized dz at dx n=3 BM involvement n=3		
Tanaka, Japan, 2002	11770	6		at dx 17.5 yrs (8-19)		66,33	"hi risk" incl large tumor size, pelvic location,lung mets, pleural cavity involvement	sternum with pleural cavity disssem n=1, ilium n=2 (one with sacral invasion), spinal cord n=1, chest wall with lung involvement n=1 and humerus n=1	

Study (Investigator, country, record.)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Yaniv, Israel, 2004	9100	11		13 yrs (0.3-19)		64,36		scapula n=1 cranium n=1 ilium n=3 femur n=2 abdomen n=1 radius n=1 sacrum n=2	

Appendix Table C3: Participant Characteristics Comparator Ewing's Tumors

Study (Investigator, country, record.)	Record Number	Group (N)	Age (mean)	Age (median)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
bernstein, USA/Canada 2006	6290	110		14.6 yrs (3.0-27.3)	white 74% Afr Amer 6% Hispanic 15% other 5%	61,39		primary site extremity 36% pelvis 29% spine 5% chest wall 16% other 14% mets site isolated lung 35% lung + other 15% isolated bone 13% isolated BM 7% other 30%	12% of patients between 20-31 yrs old
Bhatia,USA, 2007	43210	60		total cohort (which included pts that rec'd lesser intensity regimens than the n=60) age at dx: 12 yrs (0-30)		56,44			
Kushner, USA, 1995	21430	non-metastatic dz n=17 metastatic dz n=7		nonmet 15 yrs (1.5-21) mets 17 yrs (9-21)		non met 76,24 met 86,14		nonmets (ESFT and PNET): primary tumor site chest wall n=4 long bone n=7 paraspinal n=1 pelvis n=3 thigh n=1 retroperitoneal n=1 mets: primary tumor site long bone n=3 pelvis n=3 perineum n=1 sites of mets lung only n=3 mult sites incl bone or BM n=4	

Study (Investigator, country, record.)	Record Number	Group (N)	Age (mean)	Age (median)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Milano, Italy, 2006	43290	36		115 mos (20-214)			"high risk"- tumor vol >200 ml or site with poor px (pelvis, chest wall or vertebra) or pulmonary or BM mets at dx First diagnosis pts with mets n=16 (44%)	pelvis n=9 (5 bone and 4 soft tissue) femur n=2 scapula n=2 hip n=2 clavicle n=1 vertebra n=4 humerus n=3 tibia n=3 abdomen n=2 rib n=1 fibula n=2 chest wall n=4 pretibial soft tissue 1 foot soft tissue 1 radius 1 mets lung n=9 BM mets n=7	

Study (Investigator, country, record.)	Record Number	Group (N)	Age (mean)	Age (median)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Sari, Turkey, 2010	42790	36	all pts <18 yrs old for pts with mets 89% <15 and 11% >=15			39,61		primary tumor chest wall n=4 vertebra n=3 pelvis n=10 extremity n= 19	
van winkle, USA, 2005	43550	22		14.1 yrs (2.8-22.5 yrs) age of all pts in the study which included other tumor types besides ESFT		57,43	recurrent/refractory 1 pt with extraosseus ESFT	sites of recurrence lung 28% extremity 28% pelvis 10% H/N 10% other 24%	

Appendix Table C4: Treatment Characteristics Ewing's Tumors

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
bernstein, USA/Canada 2006	6290	110							CT +/- complete surgical resection +/- full-dose RT or lower dose RT to microscopic residual dz.Up to 3 metastatic sites excl BM with RT	CT: I, E, vincr, doxorub, CPM)	
Bhatia,USA, 2007	43210	60							high-intensity CT	doxorubicin, CPM and ifos	
Burdach, Germany and Austria, 2000	14310	28	for auto [n=21] BM n=2 PB n=17 BM+ PB n=2 for allo [n=7] all BM	auto n=21 allo n=7		MEL, Eto, Carbo, TBI n=10 MEL, E, TBI n=15 MEL, E, carbo n=1 MEL, TBI n=1 E, TBI n=1					

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Burdach, Germany, 2003	10030	<+17 yrs single HSCT n=18 tandem n=14		single auto or tandem auto auto	all pts recd local RT to met sites	single TBI,MEL,E +/- carboplatin tandem MEL, E times 2					
Burke, USA 2007	4060	7	pb	single auto n=1 tandem auto n=6	complete surgical resection n=6 no surgery n=1 RT n=2 (one to primary tumor and one to an orbital met)	1st: Eto, Carboplatin, CPM 2nd: MEL, CPM n=4; Thio, CPM n=1; MEL and TBI n=1		rec'd for fever, nutrition and hematologic indications prn (n=7)			All pts achv'd CR after first HSCT; only 6 went on to 2nd HSCT b/c one pt progressed with local and metastatic dz 30 days after 1st HSCT
Costa, USA, 2008	1710	1	NR	auto	vincristine, CY, doxorubicin, ifos, VP-16						

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Drabko, Poland 2005	6680	21	pb	auto		BUS, MEL n=12 MEL, VP-16, TBI n=1 MEL, VP16, CBCA n=6 Treo, Mel n=2					
Fazekas, Austria, 2008	2720	1		auto	hemipelvectomy	BUS, MEL					
Hara , japan 1998	17950	3	bm or pb or both	auto	no preHSCT surgery or RT	double-conditioning regimen thio and MEL		TPN, Abx			
Harimaya, Japan, 2003	9850	2	pb	auto	surgery n=2 (one partially resected; one en bloc) RT n=1 (pt partially resected)	carboplat and E n=1 carboplat, E, ifos n=1			partial surgical resection, multiagent CT, RT	VAIA	

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Hawkins, USA 2000	15360	16	pb n=15 bm n=1	auto n=14 syngeneic n=1 allo n=1 (HLA-matched sibling)		grp 1: BUS, MEL, Thio followed by HSCT then total marrow myeloablative RT followed by a second HSCT grp 2: BUS, MEL, Thio		prophylactic Abx if low granulocyte count			
Kasper, Germany, 2006	2570	5	pb	auto		MEL and E n=2 BUS and MEL n=3					
Kogawa, Japan, 2004	8410	1	pb	auto	surgery and RT	NR					
Koscielniak Germany 2005	7860	1	mismatched related	allo	tandem auto-auto local RT	BUS, Thio, Flu, CPM					

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Kushner, USA, 1995	21430	2		auto	surgery GTR n=1 no surg n=1	MEL, TBI			non met dz CT, surg, RT	CT CPM, doxo, VIN, ifos, E nonmets: GTR n=14 inoperable n=2 amputation n=1 RT n=7 met dz: GTR n=3 no surg n=4 RT 71 % (n=5)	
Kushner, USA, 2001	14240	5	bm and pb n=3 bm n=2	auto	RT n=4	TBI, MEL or thio, carboplatin			induction CT and in one pt RT		
Laws, Germany, 2003	9450	2		auto	resection of primary tumor with wide margins n=2 RT to mets n=2	TBI, MEL, E n=1 NR n=1					

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
lucas, usa 2008	2450	1		allo, matched mother	chemotherapy leading to resolution of disease at primary tumor site, BM, and lungs and sAppendix Table 1disease in the vertebrae and ribs for 6 months	BUS, MEL thymoglobulin	cyclosporin and methotrexate				
Lucidarme, france, 1998	17610	3	bm or pb	auto x 1 (n=1) auto x 2 (n=2)	surgery for primary tumor n=1 (pt with PD) and RT after HSCT after	thio RT n=1		TPN Abx			
Meyers, USA, 2001	13670	23	pb	auto	local RT of primary tumor and mets sites	TBI, MEL, Eto		filgrastim	repeated cycles of CT		9 patients were not transplanted b/c did not achieve good response in primary tumor and all mets sites

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Milano, Italy, 2006	43290	36							CT n =16 conservative surgery after CT n=14 RT n=3	ICE/CAV n=18 ICE n=2 CECAT n=16	
Navid, US and Canada, 2006	5930	9		auto	surgery n=6 RT n=7	CPM and E n=3 CPM, Topotecan n=2			4 patients did not undergo HSCT b/c did not achieve PR or CR with induction CT.		
Numata, Japan, 2002	12130	1	pb	auto	conventional CT and regional RT	carboplatin, e, ifo					
Oberlin, France, 2008	46850										
ozkaynak, usa 1998	18540	15	bm n=7 bm and pb n=8	auto		MEL, Carbopl, E +/- CPM					
pession, italy, 1999	16120	3	bm	auto	one patient RT to primary tumor	BUS, E, thio					
Prete, italy 1998	17210	17	pb	auto		BUS, E, Thio (n=16) L-PAM (n=1)					

Study (Investigator, country, record.)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Sari, Turkey, 2010	42790	36							CT only 8% CT and RT 55% CT and surgery 6% CT,RT and surg 22%	CT EVAIA vincr, ifos, mesna, E, adriamy, actino-D	
Tanaka, Japan, 2002	11770	6	PB	auto	surgery n=2 RT n=2 both surg and RT n=2						
van winkle, USA, 2005	43550	22							CT	ICE	
Yaniv, Israel, 2004	9100	11	pb and bm	auto		MEL, E , carbopl or BUS and MEL					

Appendix Table C5: Outcome Assessment Treatment Ewing's Tumors

Study (Investigator, country, record.)	Record Number	Group (N)	Primary Outcomes
Burdach, Germany and Austria, 2000	14310	28	EFS
Burdach, Germany, 2003	10030	<=17 yrs	EFS
Burke, USA 2007	4060	7	DFS OS
Costa, USA, 2008	1710	1	OS
Drabko, Poland 2005	6680	21	DFS OS
Fazekas, Austria, 2008	2720	1	DFS
Hara , japan 1998	17950	3	DFS OS
Harimaya, Japan, 2003	9850	2	OS
Hawkins, USA 2000	15360	16	EFS
Kasper, Germany, 2006	2570	5	OS
Kogawa, Japan, 2004	8410	1	OS DFS
Koscielniak Germany 2005	7860	1	PFS
Kushner, USA, 1995	21430	2	PFS
Kushner, USA, 2001	14240		EFS
Laws, Germany, 2003	9450	2	DFS
lucas, usa 2008	2450	1	OS
Lucidarme, france, 1998	17610	3	OS
Meyers, USA, 2001	13670	32 and 23	EFS
Navid, US and Canada, 2006	5930	9	EFS OS
Numata, Japan, 2002	12130	1	DFS OS
Oberlin, France, 2008	46850	<15 yrs	EFS OS
ozkaynak, usa 1998	18540	15	EFS OS (NR)
pession, italy, 1999	16120	3	OS DFS
Prete, italy 1998	17210	17	EFS OS
Sari, Turkey, 2010	42790		
Tanaka, Japan, 2002	11770	6	DFS OS
Yaniv, Israel, 2004	9100		

Appendix Table C6: Outcome Assessment Comparator Ewing's Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes
bernstein, USA/Canada 2006	6290	110	EFS	OS
Bhatia,USA, 2007	43210			
Kushner, USA, 1995	21430	24	PFS	
Milano, Italy, 2006	43290	36	EFS OS	
Sari, Turkey, 2010	42790	36	EFS OS	
van winkle, USA, 2005	43550	22	OS	

Appendix Table C7: Time to Event Outcomes Treatment Ewing's Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	2 yr	5 yr
Costa, USA, 2008	1710	1	73 + months			
Drabko, Poland 2005	6680	21		24 mos (13-59 mos)	HSCT in CR .68 not in CR <.10	
Fazekas, Austria, 2008	2720	1				
Hara , japan 1998	17950	3	DOD 3 mos A with D 25+ mos A NED 59+			
Harimaya, Japan, 2003	9850	2	A without D 67+ months DOD from recurrent tumor at 28 mos			
Kasper, Germany, 2006	2570	5	A NED 93+ mos A NED 70+ A NED 59+ A NED 46+ DOD 30 months			
Kogawa, Japan, 2004	8410	1	60 months +			
Koscielniak Germany 2005	7860	1				
lucas, usa 2008	2450	1	9 months + with disease			
Lucidarme, france, 1998	17610	3	1 tx DOD 2 mos (n=1) of PD 2 txs DOD 7 mos after 2nd tx (?) 2 txs A NED 28+ mos after first tx			
Meyers, USA, 2001	13670					
Navid, US and Canada, 2006	5930	HSCT DOD n=2 at 27 and 28 mos A NED n=3 at median 67 mos (57-73)				
Numata, Japan, 2002	12130	1	50+ months			
Oberlin, France, 2008	46850	<15 yrs				49%
ozkaynak, usa 1998	18540	15				
pession, italy, 1999	16120	3	DOD 7 months ANED 58+ A w D 53+			
Prete, italy 1998	17210	17		15 (1-40 mos)	70%	
Tanaka, Japan, 2002	11770		n=1 DOD 19 mos n=5 A NED median 57 mos (42-90 mos)			

Appendix Table C7 Time to Event Outcomes Treatment Ewing's Tumors Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	2 yr_2	3 yr_2	5 yr_2	Outcome_3	Med (mos)_3
Burdach, Germany, 2003	10030		EFS in <=17 yrs old	32% +/- 11% with single 40% +/- 13% with tandem					
Burke, USA 2007	4060		tandem group NED n=4 after mean f/u of 6.25 yrs (3-10 yrs) DOD n=2 at 0.3 and 3.2 yrs					group single tx DOD at .5 yrs	
Costa, USA, 2008	1710	1							
Drabko, Poland 2005	6680	21	DFS		in CR .63 and not in CR 0				
Fazekas, Austria, 2008	2720	1	DFS	relapsed 4 mos after HSCT (non-pulm); died after palliative CT					

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	2 yr_2	3 yr_2	5 yr_2	Outcome_3	Med (mos)_3
Hawkins, USA 2000	15360		EFS (n=16)			36%		EFS	grp 1 A NED: 66.7 % median 42 mos (27-66) PD 33.3% median 12 mos (6.3-17) grp 2: PD/DOD 28.6 % median 6.7 mos (0.1-26) NED/DOC n=2 (one at 9.6 months and one 31 mos after a 2nd HSCT (allo after auto) for MDS that the pt had prior to the auto tx
Kasper, Germany, 2006	2570	5							
Kogawa, Japan, 2004	8410	1	DFS	60+ months					
Koscielniak Germany 2005	7860	1	PFS	after allo 3.5 yrs					PFS after tandem 8 mos
Laws, Germany, 2003	9450		DFS	30 mos and 6 mos					
lucas, usa 2008	2450	1							
Lucidarme, france, 1998	17610	3							
Meyers, USA, 2001	13670		EFS		20%				

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	2 yr_2	3 yr_2	5 yr_2	Outcome_3	Med (mos)_3
Navid, US and Canada, 2006	5930	HSCT DOD n=2 at 27 and 28 mos A NED n=3 at median 67 mos (57-73)	no HSCT DOD n=2 @ 10 and 16 months A NED n=2 @ 73 and 80 months						
Numata, Japan, 2002	12130	1	DFS	50 + months					
Oberlin, France, 2008	46850	<15 yrs	EFS				46%		
ozkaynak, usa 1998	18540	15	EFS			51% (for pts in ist remission 66% +/- 19%; for 2nd remission 37%)			
pession, italy, 1999	16120	3		DOD 7 months ANED 58+ A w D 53+					
Prete, italy 1998	17210	17	EFS		63%				
Tanaka, Japan, 2002	11770		DFS	n=4 median 48.5 mos (31-74) n=1 NED at 79 mos					

Appendix Table C8 Time to Event Outcomes Comparator Ewing's Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	1 yr	2 yr	3 yr	5 yr	Outcome_2	1 yr_2	2 yr_2	3 yr_2	5 yr_2	Comment
bernstein, USA/Canada 2006	6290	110	77% +/- 4%	46% +/- 5			EFS	65% +/- 5	24% +/- 4%			No statistical difference was seen in EFS or OS between pts with isolated lung mets and or those with other or more than isolated lu mets
Milano, Italy, 2006	43290	36			with ICE/CAV CT 67%+/- 12% with other CT 22%		EFS			with ICE/CAV 74% with other CT 27%		
Sari, Turkey, 2010	42790					27 %	EFS				18%	
van winkle, USA, 2005	43550	22	43 % (SE 11)	33% (SE 10)								

Appendix Table C9: Adverse Events Treatment Ewing's Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Severity of Grade Secondary Malignancies	F/U (mos) SM	% SM	Comments SM
Burdach, Germany and Austria, 2000	14310	28								
Burdach, Germany, 2003	10030	reported engr, TRM, infec compl, sec malig, and major organ tox, but not by age of < or > 17 yrs								
Burke, USA 2007	4060	7	sepsis n=1		0	0				
Costa, USA, 2008	1710	1			0	0	AML at 53 months post HSCT			
Drabko, Poland 2005	6680	21				5% (n=1 day 35 from multiorgan failure secondary to infection)				
Hara , japan 1998	17950	3				0 NR				
Harimaya, Japan, 2003	9850	2			0	0				
Kasper, Germany, 2006	2570	5			0	0				
Koscielniak Germany 2005	7860				0	0				
Kushner, USA, 2001	14240	1 HSCT pt died at 17 mos after HSCT with NED but pulmonary failure								
lucas, usa 2008	2450	1			0	0				
Lucidarme, france, 1998	17610	3				0 (NR)				

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Severity of Grade Secondary Malignancies	F/U (mos) SM	% SM	Comments SM
Meyers, USA, 2001	13670		sepsis leading to death	4% n=1 patient from HSCT group (incl in TRM)		of HSCT group n=23 n=3 13%				
Navid, US and Canada, 2006	5930	9			0	0				
Numata, Japan, 2002	12130				0	0	CML chronic phase	50 months after HSCT		
ozkaynak, usa 1998	18540	15			0 (one patient not assessable secondary to early toxic death)	n=2 ATN day 0 and septic shock day 8				
pession, italy, 1999	16120	3				0 NR				
Prete, italy 1998	17210	17				0				
Tanaka, Japan, 2002	11770			0		0	CML		14 %	not clear if the 35 y/o pt or one of the 6 abstracted pts

Appendix Table C9 Adverse Events Treatment Ewing's Tumors Continued

Study (Investigator, country, record#)	Record Number	% Hepatic veno-occulusive disease (Hepatic Sinusoidal Obstruction)	Comments hVOD	Severity of Grade Serious Hemmorhagic Event	% SHE
Drabko, Poland 2005	6680	10%	moderate to severe		
Meyers, USA, 2001	13670			HSCT pt died from hemorrhagic pericarditis (included in TRM)	4%

Appendix Table C10: Adverse Events Comparator Ewing's Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	Severity of Grade Secondary Malignancies	F/U (mos) SM	% SM
bernstein, USA/Canada 2006	6290		death	5 of 110 (4.5%)	MDS	at 20 mos after dx	1/110 1%
Bhatia,USA, 2007	43210						cumulative incidence of t-MDS/AML of 11% at 5 yrs from dx
Kushner, USA, 1995	21430	24			leukemia dead at 10.5 mos after HSCT in CR from ESFT		4
Meyers, USA, 2001	13670	9 nonHSCT		11% sepsis during induction CT			
Sari, Turkey, 2010	42790	36					0%

Appendix Table C11: Design, Participant Selection and Enrollment Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Kremens, Germany, 2002	11240	malignant NH	Wilms	1st remission n=4 1st relapse n=19	23	Apr 1992-Dec 1998	Case series	23	0	
Saarinen-Pihkala, Finland, 1998	17940	Malig NH	Wilms	1st CR	3		CS	3	0	
Spreafico, Italy, 2008	2380	malig NH	Wilms and one case of CCSK	relapsed, high risk (3 relapsed in prior RT field)	20	Jan 2001-June 2006	CS consecutive cases	20		20 patients enrolled; 5 did not receive tx (3 due to disease progression and 2 at discretion of M.D.).
Campbell, USA, 2004	8570	malig NH	Wilms	relapsed	13	1991-2001	CS	13	0	
Tucci, Brazil, 2007	3910	malig NH	Wilms	resistant, relapsed	1		CR	1	0	
Hempel, Germany, 1998	18100	malig NH	Wilms	relapse s/p 2nd lung metastasectomy	1		CR	1	0	
Termuhlen, USA, 2006	4890	malig NH	Wilms	CR1 n=1 CR2 n=1	2		CS	2	0	
Kullendorff, Sweden, 1997	19290	malig NH	Wilms	2nd relapse	4	1987-1992	CS	4	0	includes one patient with CCSK
Maurer, Austria, 1997	18670	malign NH	Wilms	relapsed during 1st line CT	1		CR	1	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Pein, France, 1998	17570	malig NH	Wilms	recurrent, high risk 2nd CR n=16 3rd CR n=3 5th CR n=1 2nd PR n=4 3rd CR n=5	28	Oct 1988-Oct 1994	CS	28	1 4 mos after HSCT	
Valera, Brazil, 2004	8620	malig NH	Wilms	relapsed, 2nd CR	3		CS	3	0	
Goldman, USA, 2001	13330	malign NH	Wilms	relapsed (6 mos after dx)	1	1994-1998	CR	1	0	
Dagher, USA, 1998	17840	malign NH	Wilms	multiply recurrent	1		CR	1	0	
Hempel, Germany, 1996	20550	malig NH	Wilms	progressive disease, 1st, 2nd or subsequent relapse, metastatic dz	7	April 1992-May 1995	CS	7		one patient was misdxd and had RMS and is not included in the analysis
Fazekas, Austria, 2008	2720	malig NH	Wilms	relapsed	1		CR	1	0	
Park, Korea, 2006	5450	malign NH	Wilms	recurrent	3	1994-2004	CS	3	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Meyers, USA, 2001	13670	malig NH	ESFT	newly diagnosed metastatic to bone and/or BM	32	Feb 1996-Nov 1998	CS	23	9 patients did not proceed to HSCT b/c 4 had progressive dz, 2 secondary to toxicity and 3 who died from toxicity during high-dose phase of the therapy.	
Abu-Ghosh 2002 USA	45610		Wilms		11	1992-1999 37 N Amer centers and 2 S Amer centers	CS	11	0	
Malogolowkin USA 2008	44950	malign NH	Wilms	relapsed, high risk	60	1995-2002	CS	60	0	

Appendix Table C12 Participant Characteristics Treatment Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Campbell, USA, 2004	8570	13		at dx 4.8 years (1-15 years)		31%,69	initial I n=2 II n=1 III n=5 IV n=5	FH n=12 UH n=1	
Dagher, USA, 1998	17840	1		7 years at HSCT		100 F	recurrent	right-sided tumor bed	pt had a left-sided wilms tumor, FH, stage II at age 9 mos and underwent L nephrectomy and CT. At age 6, developed a right kidney wilms tumor for which she underwent R nephrectomy, CT and RT. At 7 yrs of age she had a right-sided recurrence and HSCT

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Fazekas, Austria, 2008	2720	1	5 yrs at tx			100 M	"intermediate risk" not further defined		
Goldman, USA, 2001	13330	1		2 yrs at HSCT		100% M	III	relapse in lungs and abdomen	
Hempel, Germany, 1996	20550	7		at tx 7.3 yrs (3.8-14.7 yrs)		86,14		UH n=1 FH n=6	
Hempel, Germany, 1998	18100	1	11 months			100,0	initial stage 2	"medium" malignancy	
Kremens, Germany, 2002	11240	23		at dx 74 months	11-210 months	52,48	I n=4 II n=4 III n=3 IV n=13 (note does not total 23)	n=14 Intermediate risk n=5 high-risk n=1 completely necrotic	
Kullendorff, Sweden, 1997	19290	4		at dx median 67 months (43-119 mos)		33,66	I n=2 III n=2	FH n=3 UH n=1 site or relapse lung n=2 and bone n=2	
Maurer, Austria, 1997	18670	1	at dx 8 yrs			100 F	IV with lung mets	UH	
Park, Korea, 2006	5450	3		2 yrs (2-3 yrs)		66,33	initial stage: II (n=3)	UH n=2 FH n=1 site of relapse lung n=2, abdomen n=1	
Pein, France, 1998	17570	29		6 yrs (2-16 years)		41,59	at dx: I n=4 II LN + n=5 II LN- n=7 III n=5 IV n=6 V n=2	UH n=6 (3 anaplastic, 3 CCS) FH n=23	
Saarinen-Pihkala, Finland, 1998	17940	3		at dx 46 months (6-60)		66%, 33%	V (n=3) mets to lung n=1	FH n=2 "rhabdomyomatous" n=1	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Spreafico, Italy, 2008	2380	15		at dx 4.1 years (1.1-11.2)		30,70	Initial stage I n=1 II n=2 III n=8 IV n=8	UH n=1 CCSK n=1	
Termuhlen, USA, 2006	4890	2		40.5 mos (21-60 mos)		100 F	V n=2	FH n=2	
Tucci, Brazil, 2007	3910	1							
Valera, Brazil, 2004	8620	3		at dx 7 yrs (3-9 yrs)		66,33	II n=1 III n =1 IV n=1	FH n=1 not reported n=2	

Appendix Table C13: Participant Characteristics Comparator Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Abu-Ghosh 2002 USA	45610	11		at dx 39 months (13-192 mos)		relapsed/recurrent, high-risk stage at initial dx: I 18% II 9% III 36% IV 27% V 9%	FH 82%, UH 18% site of relapse lung 36%, pleura 9%, kidney 18%, kidney and lung 18% and liver 9%	
Malogolowkin USA 2008	44950	60	at dx 0-23 mos n=4 24-47 mos n=21 48+ n=35		47,53	initial stage II n=1 III n=39 IV n=20	FH n=56 focal anapl n=3 diffuse anapl n=1 site of relapse lung only n=33 operative bed +/- lung +/- other n=7 liver +/- other n=6 abd or pelvis +/- lung n=6 lung and other n=6 other n=2	
Park, Korea, 2006	5450	7 (2 lost to f/u)		2 yrs (1-11yrs)	71,29	initial stage: I n=1 II n=3 III n=1 IV n=2 relapsed, high risk	FH n=7 relapse lung n=3, abd n=2, lung and abd n=1, abd, lung and bone marrow n=1	Although 13 pts in this study did not undergo HSCT, only 9 were high risk relapse, 2 were lost to f/u so only 7 abstracted.
Tucci, Brazil, 2007	3910	10		2 years		relapsed		Recurred in a mean time of 13.4 +/- 10 months (range 2-36). One of 10 of the relapsed pts had favorable px factors.

Appendix Table C14: Treatment Characteristics Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Abu-Ghosh 2002 USA	45610	11							ICE	
Campbell, USA, 2004	8570	13	PB	auto	nephrectomy n=13 reinduction CT n=12	T/CPM n=6 T/CPM/C n=2 T/E n=1 C/CPM/Mel n=4				
Dagher, USA, 1998	17840	1	PB	auto	bilateral nephrectomy, CT and RT	E/C/CPM				
Fazekas, Austria, 2008	2720	1		auto						
Goldman, USA, 2001	13330	1	PB	auto	nephrectomy Flank RT	VP-16/T/cytoxan				
Hempel, Germany, 1996	20550	7	PB	auto		MEC				
Hempel, Germany, 1998	18100	1	BM	auto	Nephrectomy lung metastasectomy x 2 RT to lung	C/M/E				
Kremens, Germany, 2002	11240	23	PB	autologous		MEC n=19 M x2 n=1 E, thio and cyc n=1 mel and ifos n=2	hydration, TPN, prophylactic Abx, irradiated blood products			6 patients received RT after HSCT (2 lung consolidation; 4 to palliate recurrence after HSCT
Kullendorff, Sweden, 1997	19290	4	BM n=2 PB and BM n=2	auto		mel/VP-16/C				

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Malogolowkin USA 2008	44950	60						prior nephrectomy, Radiation, CT	CPM, E, C	
Maurer, Austria, 1997	18670	1	PB and BM	sequential double auto	nephrectomy RT to lung mets and surgical removal of lung mets	1st: modified MEC 2nd: EC				
Meyers, USA, 2001	13670	23	PB	auto	local control RT to primary tumor and mets	TBI MEL E	filgrastim			
Park, Korea, 2006	5450	3	PB	auto	nephrectomy; prior first-line CT RT after relapse n=1	MEC	TPN	CT RT if stage III or IV or UH (n=3)	In the early group (treated 1983-1993) doxorubicin was added in cases where patient had initially received 2 drugs. In the late grou[(treated 1983-1993) pts received combinations of CPM/E and C/E.	Groups of pts were divided into two groups (early and late) according to date of relapse.
Pein, France, 1998	17570	29	BM n=28 PB n=1	auto		MEC	"standard"			
Saarinen-Pihkala, Finland, 1998	17940	3	BM	auto	bilateral nephron-sparing nephrectomy	MEL				

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Spreafico, Italy, 2008	2380	14	PB	auto	nephrectomy n=14	MEC n=7 M/T n=2 E/T/CPM n=2 HD-ICE n=1 HD-ICE, M/CPM n=1 MC n=1 E/T/CPM		CT	ICE	M=melphalan T=thiotepa CPM=cyclophosphamide E=etoposide C=carboplatin
Termuhlen, USA, 2006	4890	2	PB n=1 PB and BM n=1	auto	nephron-sparing nephrectomy n=2 RT to 1 kidney n=1	Mel/T/vincristine	dopamine, anti HTN Rx, hydration			
Tucci, Brazil, 2007	3910	1	PB	auto	nephrectomy; lung lesions resected; RT to lung and liver	E,Mel,C		multiagent salvage CT, abdominal RT (n=6), and lung RT (n=3)	cisplatin, C, CPM, E, ifo	
Valera, Brazil, 2004	8620	3	BM	auto	nephrectomy	C/E/M C/E/pulm RT C/E/Ifos				

Appendix Table C15: Outcome Assessment Treatment Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Campbell, USA, 2004	8570	13	EFS OS		median 30 mos (7-120)
Dagher, USA, 1998	17840	1	OS	DFS	
Fazekas, Austria, 2008	2720	1	OS		
Goldman, USA, 2001	13330	1	OS		
Hempel, Germany, 1996	20550	7	DFS OS		
Hempel, Germany, 1998	18100	1	DFS		
Kremens, Germany, 2002	11240	23	OS EFS		58 mos (37-116)
Kullendorff, Sweden, 1997	19290	4	OS		
Maurer, Austria, 1997	18670	1	OS		
Park, Korea, 2006	5450	3	OS EFS		
Pein, France, 1998	17570	28	DFS OS		37 mos (7-96 mos)
Saarinen-Pihkala, Finland, 1998	17940	3	DFS		
Spreafico, Italy, 2008	2380	14	DFS OS		
Termuhlen, USA, 2006	4890	2	OS		
Tucci, Brazil, 2007	3910	1	DFS OS		
Valera, Brazil, 2004	8620	3	DFS		

Appendix Table C16: Outcome Assessment Comparator Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	F/U Frequency/Duration
Abu-Ghosh 2002 USA	45610	11	PFS OS	median f/u 4.3 yrs
Malogolowkin USA 2008	44950	60	EFS OS	
Park, Korea, 2006	5450	7	EFS OS	
Tucci, Brazil, 2007	3910	10	DFS OS	

Appendix Table C17: Time to Event Outcomes Treatment Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	3 yr	4 yr	HR (95%) CI	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	HR (95% CI)_2	Outcome_3
Campbell, USA, 2004	8570	13				73 %	.40-6.86	EFS					60%	.40-6.88	
Dagher, USA, 1998	17840	1	1.8 years					DFS	.5 years						
Fazekas, Austria, 2008	2720	1	A NED at 12 mos												
Goldman, USA, 2001	13330	1	A NED at 16+ mos												
Hempel, Germany, 1996	20550	7	n=6 A NED at median 2.1 yrs (0.5-3.7 yrs) n=1 DOD 19 mos					DFS	n=1 8 mos n=6 A NED at median 2.1 yrs (0.5-3.7 yrs)						
Hempel, Germany, 1998	18100	1	DFS A NED at 32 mos post HSCT												

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	3 yr	4 yr	HR (95%) CI	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	HR (95% CI)_2	Outcome_3
Kremens, Germany, 2002	11240	23	60.9 % (+/- 10.2 %)					EFS 48.2% (+/- 13.6)							OS and EFS in subgroup that received MEC consolidation (n=19) OS 63.2 % (+/- 11.0%) EFS 54.1% (+/- 14.9%)
Kullendorff, Sweden, 1997	19290	4	2 A & W at 17 and 28 mos 2 DOD 33 and 7 mos after HSCT												
Malogolowkin USA 2008	44950	60				42.3		EFS					48		
Maurer, Austria, 1997	18670	1	A & W at 4 years in CR2 yrs												

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	3 yr	4 yr	HR (95%) CI	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	HR (95% CI)_2	Outcome_3
Meyers, USA, 2001	13670	23						EFS		~40 %	24 %				
Park, Korea, 2006	5450	3	median 53+ months (31+-76+)					EFS	median 53 months (31-76 mos)						
Pein, France, 1998	17570	28			60 % +/- 18 %			DFS				50 % +/- 17			
Saarinen-Pihkala, Finland, 1998	17940	3	DFS	median 51 months (40-53 months)											
Spreafico, Italy, 2008	2380	20	median f/u 25 months (14-79)		55 % +/- 13 %			DFS	25 mos (14-79)			56 % +/- 12 %			
Termuhlen, USA, 2006	4890	2	A NED 7 year after HSCT n=1 DOD 6 y 2 m after HSCT n=1		100%	100%									

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	3 yr	4 yr	HR (95%) CI	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	HR (95% CI)_2	Outcome_3
Tucci, Brazil, 2007	3910	1	A WED 84 mos from relapse					DFS A WED 84 mos from relapse							
Valera, Brazil, 2004	8620	3						A & W at 12 mos and 48 mos one patient relapsed after HSCT then underwent CT and is in 3rd CR for 22 mos							

Appendix Table C18: Time to Event Outcomes Comparator Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	1 yr	2 yr	3 yr	4 yr	5 yr	Outcome_2	Med (mos)_2	3 yr_2	5 yr_2
Abu-Ghosh 2002 USA	45610	11		~73 %	63.6 +/- 14.5 %	63.6 +/- 14.5 %	63.6 +/- 14.5 %	63.6 +/- 14.5 %	PFS		63.6 +/- 14.5 %	
Malogolowkin USA 2008	44950											
Park, Korea, 2006	5450	7	DOD n=5 median 15 mos (2-30 mos) A NED n=1 20+ mos A with D n=1 130+ mos						EFS	median 8 months (2-20 mos)		
Tucci, Brazil, 2007	3910	10				83.3 %		42.8 %	DFS		66.6 %	42.8 %

Appendix Table C19: Adverse Events Treatment Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Comment TRM	Severity of Grade Serious Hemmoragic Event	% SHE
Campbell, USA, 2004	8570	13			0%	0%			
Dagher, USA, 1998	17840	1				0			
Fazekas, Austria, 2008	2720	1				0			
Goldman, USA, 2001	13330	1				0			
Hempel, Germany, 1996	20550	7				0			
Hempel, Germany, 1998	18100	1			0%	0%			
Kremens, Germany, 2002	11240	23				0%			
Kullendorff, Sweden, 1997	19290	4				0			
Maurer, Austria, 1997	18670	1 long term renal tubular dysfun ction				0			
Meyers, USA, 2001	13670	23	sepsis (died)	4 %		13%	one for unreporte d reasons	hemorrhagic pericarditis after RT (died)	4

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Comment TRM	Severity of Grade Serious Hemmoragic Event	% SHE
Park, Korea, 2006	5450	3				0			
Pein, France, 1998	17570	29				0			
Saarinen-Pihkala, Finland, 1998	17940	3			0%	0%			
Spreafico, Italy, 2008	2380		died of sepsis 4 months after tx in CR n=1	7 %					
Termuhlen, USA, 2006	4890	2			0	0			
Tucci, Brazil, 2007	3910				0%	0%			
Valera, Brazil, 2004	8620	3				0			

Appendix Table C20: Adverse Events Comparator Wilm's Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	Comment	TRM	% TRM	Severity of Grade Secondary Malignancy
Abu-Ghosh 2002 USA	45610		septic shock	27	reversible	TRM	0	
Malogolowkin USA 2008	44950	60	one patient died of infl B virus and aspergillus infec during maintenance chemo	2%		TRM		MDS n=1 2%

Appendix Table C21: Design, Participant Selection and Enrollment Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Bisogno, Italy, 2009	75340	Malignant solid tumor	Rhabdomyoscarcoma	Metastatic RMS	HSCT (70)	1999-2006	prospective single arm	70	0	
Breneman, USA, 2003	75360	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	Comparator (127)	1991-1997	Case series	127		
Carli, Italy, 1999	16010	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	HSCT (52) Comparator (44)	1989-	single arm study	96		52 transplanted and 44 failed to meet transplant requirements and served as comparators.
Doelken, Germany, 2005	6570	Malignant Solid Tumor	Rhabdomyoscarcoma	Relapsed	HSCT (2)		Case reports	2	0	
Donker, Netherlands, 2009	1420	Malignant Solid tumor	Alveolar Rhabdomyosarcoma	Stage IV RMS	HSCT (1)		case study	1	0	Allo transplant
Grundy, UK, 2001	14200	Malignant solid Tumor	Rhabdomyoscarcoma congenital	Congenital RMS	HSCT (1)		case report	1		This report also details three other children who died by 3 months of age. The paper also details all other known cases (7). In these cases all patients died
Hara, Japan, 1998	17950	malignant Solid tumor	rhabdomyoscarcoma	Stage III or IV or relapsed RMS	HSCT (7)	1993-1997	Case series	7	0	abstracted from a astudy of multiple solid tumors

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Koscielniak, Germany, 1997	19800	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic and recurrent	HSCT (36)	1986-1994	retrospective case series	36	0	This paper contains both Allo and Auto transplants. The data are reported together as they cannot be seperated.
Kuroiwa, Japan, 2009	390	Malignant Solid Tumor	Rhabdomyoscarcoma	Blueberry Muffin	HSCT (1)		case report	1	0	
Kwan, Hong Kong, 1996	20800	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	HSCT (1)		Case Report	1	0	
Lucidarme, France, 1998	17610	Malignant Solid Tumor	Rhabdomyoscarcoma	Refractory or relapsed RMS	HSCT (8)	1987-1995	single arm phase II	HSCT (8)	0	
Matsubara, Japan, 2003	10810	Malignant Solid Tumor	Rhabdomyoscarcoma	High-risk RMS	HSCT (22)	1990-1999	Case series	22	0	There is one patient who is 22 years old. He is included in these results. His survival is similar when compared to a 16 and a 20 year old with a similar site of relapse and status at transplant
McDowell, UK, 2010	75350	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	HSCT (101) Comparator (45)	1998-2005	Two single arms	HSCT (101) Comparator (45)		This is not a comparative study but two single arms within the same study

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Misawa, Japan, 2003	11040	Malignant Solid Tumor	Alveolar RMS	Refractory RMS	HSCT (1)	1997	Case Study	1	0	Allogeneic transplant
Moritake, Japan, 1998	18280	Malignant Solid Tumor	Rhabdomyo scarcoma	relapsed	HSCT (1)	1994	Case report	1	0	
Navid, USA, 2006	5930	Malignant Solid tumor	Rhabdomyo scarcoma	Metastatic RMS	HSCT (8)	1996-2000	case series	8	1 LFU at 78 months post transplant 2 people were removed from the protocol (one due to fungal infection, one due to delayed hem. Recovery) 1 patient was non-compliant	only two were transplanted as they achieved CR, one other achieved CR but was the LFU
Oue, Japan, 2003	10950	Malignant solid Tumor	Rhabdomyo scarcoma	Metastatic RMS	HSCT (1)	1991-2001	case series	1	one patient died prior to surgery due to progressive disease	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Pappo, USA, 1999	48020	Malignant Solid Tumor	Rhabdomyoscarcoma	relapsed RMS	Comparator (605)	1984-1997	retrospective analysis of three single arm studies	605	0	
Pappo, USA, 2001	47860	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	Comparator (48)	1994-1996	Case series	48		
Raney, USA, 2008	2440	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	Comparator (91)	1978-1997	case series	91		
Sandler, USA, 2001	12810	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	Comaprator (152)	1988-1991	Case series	152		
Sato, Japan, 1998	48070	Malignant Solid Tumor	Rhabdomyoscarcoma	Stage III or IV RMS	HSCT (5)	1993-1998	case series	HSCT (5)	HSCT (5)	only abstracted treatment arm as comparator treatment was not specified for two historical controls
Scully, USA, 2000	14580	Malignant Solid Tumor	Rhabdomyoscarcoma	Recurrent RMS	HSCT (1)		Case report	1	0	
Shaw, Israel, 1996	20050	Malignant solid Tumor	Rhabdomyoscarcoma	Stage IV RMS	HSCT (9)		prospective case series	9	0	this was a study with mixed solid tumors
Van Winkle, USA, 2005	43550	Malignant Solid tumor	Rhabdomyoscarcoma	Recurrent/refractory RMS	Comparator(27)	1992-1996	Case series	27	0	these patients were enrolled in three treatment protocols but will be reported together
Walterhouse, USA, 1999	17240	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic RMS	HSCT (8)	1992-1994	case series	8	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Williams, Canada, 2004	9010	Malignant Solid Tumor	Rhabdomyoscarcoma	Metastatic	13 (comparator) 4 (HSCT)	1989-1999	retrospective review	13 (comparator) 4 (HSCT)	0	

Appendix Table C22: Participant Characteristics Treatment Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comm ent
Bisogno, Italy, 2009	75340	HSCT (70)			<1 year (1) <10 years (38) >=10 (32)		47% Male 53% Female	metastatic most common primary sites include head and neck, limbs and abdomen/pelvis	63% Alveolar RMS 36% Embryonal 1% not otherwise spec	
Carli, Italy, 1999	16010	HSCT (52)		31 pts. < 10 21 Pts. > 10				metastatic	Alveolar (44%) Embryonal/NOS (56%) Primary site Extremity, parameningeal , other (75%), Genitourinary tract and Head and Neck (25%)	
Doelken, Germany, 2005	6570	HSCT (2)	Pt 1- 11.5 Pt 2-13				2 males	Pt 1-Stage IV with mets to lung, pancreas and marrow Pt2-initial stage T1b N0M0, metastatic disease at transplant	Both Alvelolar RMS with varrious metastatic sites at transplant	
Donker, Netherlands, 2009	1420	HSCT (1)	8 years			White	Female	Stage IV RMS	extensive local, abdominal and thoracic lymph node metastases, no BM invasion.	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Grundy, UK, 2001	14200	HSCT (1)	diagnosed at birth				Male		congential alveolar RMS/right thigh and multiple skin nodules	
Hara, Japan, 1998	17950	HSCT (7)	6.8 at diagnosis	3	1-18 years			stage III (2), stage IV (3), replased (2)	43% Alveolar 57% Enbryonal	
Koscielniak, Germany, 1997	19800	HSCT (36)		6 at diagnosis	(<1-22)				RMS alveolar (61%) RMS embryonal (36%) Undifferentiated (3%)	Patient population contains at least one patient over the age of 21. 27 patients had metastatic disease and 9 had relapsed disease.
Kuroiwa, Japan, 2009	390	HSCT (1)		<1 at transplant					Alveolar RMS/priamry skin lesions	
Kwan, Hong Kong, 1996	20800	HSCT (1)	14 years				Female	Stage IV/ Group IV	Alveolar RMS/primary site left thenar region/metastatic to the breast	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comm ent
Lucidarme, France, 1998	17610				2-17 for whole study			5/8 had metastatic disease at transplant		
Matsubara, Japan, 2003	10810	HSCT (22)		8.5 at transplan t	2-22 years		14 males (64%) 8 Females (36%)	group III (14) or IV at transplant (8)	Alveolar 7 (32%) and Embryonal 15 (68%) varried primary sites parameningeal was the most common (7)	
Matsubara, Japan, 2005	7580	HSCT (5)	17.6 months at diagnos is	16 months at diagnosis	3-41		20% Male, 80% Female		distant metastasis	
McDowell, UK, 2010	75350	HSCT (101)		HR' 10.6' SR' 4.28	HR' 1.7-17.5' SR' 0.52-9.93		HR' 56% Male SR' 60% Male	Metastatic	21% Embryonal, 64% Alveolar, 8% unspecfied, 6% unknown primary sites include 28% Orbit,	
Misawa, Japan, 2003	11040	HSCT (1)	17 at present ation				Female	Stage I, Clinical Group III undifferentiated RMS	Alveolar RMS	
Moritake, Japan, 1998	18280	HSCT (1)	10 at diagnos is				male	metastatic to BM	Unspecified subtype/ primary nasal tumor	
Navid, USA, 2006	5930	HSCT (8)	15.5	13.1	(1.6-18.7)		38% Male 62% Female	metastatic/	Alveolar RMS/varrious primary sites	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comm ent
Oue, Japan, 2003	10950	HSCT (1)	4.6 years				female	metastatic	primary site Lt. buttock metastatic to the Lt. femur	
Sato, Japan, 1998	48070	HSCT (5)	5.34 at diagnos is	7	.7-10 years		60% Male 40% female	Stage III RMS	60% Embryonal, 40% undifferentiated retroperitoneum (2), parameningeal (1), Femur (1), Orbit (1)	
Scully, USA, 2000	14580	HSCT (1)	~ 5 at transpla nt				Female	local recurrence	Local recurrence of embryonal RMS/upper arm priamry site	
Shaw, Israel, 1996	20050	HSCT (9)	8.5 years		4-15			Stage IV various primary sites		
Taguchi, Japan, 2005	7430	HSCT (1)	4				M	metastatic	maxilla and mandible	
Walterhouse, USA, 1999	17240	HSCT (8)	14	12.5	3-17 years		63% Female 37% Male	Stage IV/group 4 RMS various primary sites	63% Alveolar, 25% Embryonal, 12% unknown various metastatic sites	
Williams, Canada, 2004	9010	HSCT (4)			TX. < 10 ' Comp 7<10' 6>10		TX' 75% Female 25% Male' Comp' 54% Male' 47% Female	Stage IV mets to lung	Embryonal RMS/ primary head and neck, parameningeal, bladder/prostate	

Appendix Table C23: Participant Characteristics Comparator Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Breneman, USA, 2003	75360	Comparator (127)		7	0-19		56% male 44% Female	Stage IV/Catagory IV	36% embryonal, 46% alveolar, 3% undiff most common primary sites extremity (28%), parameningeal (20%) and Trunk (20%) Lung was the most common metastatic site, folowed by bone marrow, and lymph nodes	
Carli, Italy, 1999	16010	Comparator (44)			3 Pts. <1, 27 Pts. <10, 14 Pts >=10				Alveolar (30%) Embryonal/NOS (70%) Primary site Extremity, parameningeal , other (80%), Genitourinary tract and Head and Neck (20%)	
McDowell, UK, 2010	75350	Comparator (45)		4.28	0.52-9.93		60% Male 40% Female	No bone or bone marrow mets parameningeal (22%) and pelvis (31%) most common primary sites	57% Embryonal 33% alveolar 9% unspecificied or unknown 71% had mets to the lung	This is the standard risk group in this two arm risk stratified study.

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Pappo, USA, 1999	48020	Comparator (605)	8 years at diagnosis		0-20	73% White	57 % Male 43% Female	stage I 17%, stage II 8%, Stage III 36%, Stage IV 36%, unknown 3% 9% clinical group I, 9% clinical group II, 45% clinical group III, 37% clinical group IV	botryoid (3%), embryonal (53%), alveolar or undiffer (45%) most common primary tumor sites extremities (26%) Parameningeal (19%) and retroperitoneum (13%)	
Pappo, USA, 2001	47860	Comparator (48)		10 at diagnosis	0-19	70% White, 15% Black, 15% Other	52% Male, 48% Female	Metastatic	29% Embryonal, 48% alveolar, 4% Undifferentiated, 19% Unspecified Primary sites 43% retroperitoneum/perineum/trunk, 23% extremities, 15% GU/bladder/prostate, 19% other	
Sandler, USA, 2001	12810	Comaprator (152)		8.5	(0-19)		58% Male, 42% Female	metastatic	48% Embryonal, 37% alveolar, 15% Other priamry sites include 31% extremity, 18% HN (including orbit and parameningeal), 18% retroperitoneum, 34% other	
Van Winkle, USA, 2005	43550	Comparator(27)	11.3		2.1-20.5		48% Female 52% Male	at recurrence 4% stage I, 0 stage II, 11% stage III, 63% Stage IV, 22% Unknown	37% alveolar 41% Embryonal 11% Undifferentiated 11% unknown	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Williams, Canada, 2004	9010	Comparator (13)			7 patients <10 6 patients >10		54% Male 47% Female	Stage IV mets to all sites	9 patients Alveolar, 3 embryonal,1 mixed-primary site Trunk(6), bladder.prostate (2), extremity (4) Gennitourinary (1)	

Appendix Table C24: Treatment Characteristics Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Bisogno, Italy, 2009	75340	HSCT (70)	PBSC	Auto	surgery + chemo including ifosfamide, vincristine, actinomycin, CY, carboplatin, vincristine, etoposide	thiotepa, melphalan, CY					those with at least a partial response moved onto HD with stem cell support. Patients received three rounds of HDC and stem cell infusion.
Breneman, USA, 2003	75360	Comparator (127)							Chemo +/- RT	melphalan-vincristine + vincristine, dactinomycin and CY (VAC) or VAC + ifosfamide + etoposide	
Carli, Italy, 1999	16010	HSCT (52) Comparator (44)	PBSC or BM	Auto	epirubicin, carboplatin, vincristin, actinomycin, ifosfamide, etoposide	Melphalan				epirubicin, carboplatin, vincristin, actinomycin, ifosfamide, etoposide	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Doelken, Germany, 2005	6570	HSCT (2)	Pt1- PBSC from HLA-identical sibling Pt 2 PBSC from HLA-identical fraternal twin	Pt1- Allogeneic Pt 2- Auto then Allo	pt1-CWS-96 Arm B protocol including ifosfamide, vincristine, carboplatin, epirubicin and actinomycin D, etoposidem and RT Pt2- CWS-91 protocol chemo+ RT, relapsed +2 years, Auto transplant after HD w/ thiotepa and CY, resection and RT lung mets	Pt1- TBI, etoposide, CY Pt2- immunosuppression with treosulfane and fludarabine W/O HD chemo (for Alllo)	Pt 1- cyclosporin A and MTX and prednisolone and CellCept after AGVHD developed				
Donker, Netherlands, 2009	1420	HSCT (1)	Bone Marrow	Allogeneic	SIOP MMT-98 protocol; including vincristine, dactinomycin, ifosfamide, carboplatin, epirubicin, etoposide and CY.	etoposide, CY and TBI	CsA was given				

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Grundy, UK, 2001	14200	HSCT (1)			chemo including vincristine, actinomycin D, CY, doxorubicin, etoposide + amputation of the right leg	melphalan					transplant was followed by more chemo
Hara, Japan, 1998	17950	HSCT (7)	PBSC or BM	Auto	Chemo containing cisplatin, CY, vincristine. Ifosfamide, dactinomycin, etoposide, carboplatin and pirarubicin were administerd in some patients +/- surgery and LRT	Thiotepa, melphalan and busulfan		laminar air flow, total parenteral nutrition and antibiotics, G-CSF			6 patients were transplanted in CR one was not in CR

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Koscielniak, Germany, 1997	19800	HSCT (36)	BM -26 patients PBSC-5 patients Allogeneic - 5 patients	Auto-31 Allo-5	CWS-81, CWS-86, CWS-91, (23 patients), MMT stage IV (12) CWS relapse (1), treatment included vincristine, dactinomycin, CY, doxorubicin, ifosfamide, VP16, carboplatin, epiadriamycin	melphalan, VP16, carboplatin +/- RT		14 received G-CSF or GM-CSF support			
Kuroiwa, Japan, 2009	390	HSCT (1)		Auto	Chemo including vincristine, actinomycin D, CY	ifosfamide-cisplatin-etoposide					
Kwan, Hong Kong, 1996	20800	HSCT (1)	PBSC	Auto	adriamycin and CY + Surgery and post-operative radiation	Vincristine, ifosfamide, actinomycin D HDC with carboplatin, etoposide, melphalan					transplanted in CR

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Lucidarme, France, 1998	17610	HSCT (8)	PBSC or BM	Auto	chemo including CY or ifosfamide +/- sugery +/- RT	Thiotepa		laminar air-flow, right atrial catheters, parenteral nuitrition, broad spectrum anti-biotics, blood products			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Matsubara, Japan, 2003	10810	hsct (22)	PBSC and BM	Auto	treatment varried and included VAC (vincristine, dactinomycin and CY) VAC-THP (pirarubicin + VAC), VCA(vincristine, dactinomycine , CY, docorubicin, VAI (vincristine, dactinomycin, ifosfamind) +/- cisplatin, etoposide or methotrexate and +/- surgery & RT	included Hi-MEC (etoposide, carboplatin, melphalan), Hi-MEC + pirarubicin, etoposide + melphalan + ifosfamide, etoposide + thiotepa, Melphalan alone		intravenous hyperalimentation or blood products as needed. G-CSF was used in 14 patients transplanted after 1993			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
McDowell, UK, 2010	75350	HSCT (101) Comparator (45)	Auto	PBSC	doxorubicin or carboplatin	sequential high dose therapy containing cyclophosphamide, etoposide, carboplatin			chemo and surgery +/- radiotherapy followed by maintenance therapy 9 courses of VAC	ifosfamide, vincristine, actinomycin D, carboplatin, etoposide, and epirubicin (induction) after local therapy patients received 9 cources of VAC (maintenance therapy)	sequential HD therapy was given at 14 day intervals regardless of blood count. Four does were given
Misawa, Japan, 2003	11040	HSCT (1)	PBSC	Allogeneic from HLA-identical sibling	vincristine, CY, pirarubicin alternating with etoposide, ifosfamide, and cisplatin	pirarubicin, etoposide, carboplatin, melphalan	Cyclosporine and methylperdnisolone				
Moritake, Japan, 1998	18280	HSCT (1)	BM	Allogeneic	VCR, actinomycin D, CY, pirarubicin and ifosfamide + RT	etoposide, carboplatin, pirarubicin, melphalan	methotrexate				

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Oue, Japan, 2003	10950	HSCT (1)		Auto-Auto	chemo + RT	Ifosfamide and melphalan (first) Busulfan and thiotepa (second)		G-CSF, blood products			this is a tandem transplant
Pappo, USA, 1999	48020	Comparator (605)							chemo +/- RT	vincristine-Actinomycin (14%), vincristine-Actinomycin-CY or similar (37%), vincristine, doxorubicin, actinomycin, CY +/- other agents (25%), window + other (24%)	
Pappo, USA, 2001	47860	Comparator (48)							Chemo +/- RT	Topotecan + VAC alternating with vincristine, topotecan, CY or topotecan + VAC	
Raney, USA, 2008	2440	comparator (91)							chemo +/- RT	vincristin, actinomycin D, CY +/-doxorubicin, cisplatin, dacarbazine, etoposide and/or ifosfamide	
Sandler, USA, 2001	12810	Comparator (152)							Chemo +/- RT	ifosfamide, doxorubicin and VAC	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Sato, Japan, 1998	48070	HSCT (5)	PBSC	Auto	Chemo +/- surgery and or RT	Hi-MEC +/- pyrarubicin		Hydroxyzine and hydrocortisone and G-CSF			
Scully, USA, 2000	14580	HSCT (1)	PBSC	Auto	prior chemo for initial disease, chemor for recurrence included ifosfamidem carboplatin, etoposide	HDC with CY and carboplatin					tumor was excised after SC rescue and radiation was delivered

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Shaw, Israel, 1996	20050	HSCT (9)	PBSC and BM	Auto	Chemo +/- surgery and or radiation therapy chemo included vincristine, adriamycin, CY etoposide, ifosfamide	carboplatin, melphalan,		parenteral nutrition, antibiotics and anti-fungal therapy was provided based on the patient status. G-CSF or GM-CSF was used in some patients			
Taguchi, Japan, 2005	7430	HSCT (1)			carboplatin and etoposid						
Van Winkle, USA, 2005	43550	Comparator(27)							chemo	Ifosfamide and etoposide	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Walterhouse, USA, 1999	17240	HSCT (8)	PBSC	Auto	Chemo and radiation +/- surgical resection chemo regemis included vincristine, dactinomycin, CY, melphalan, etoposide, ifosfamide and doxorubicin	thiotepa, CY, carboplatin		G-CSF, fluconazole prophylaxis, broad spectrum antibiotics for fever, parenteral nutrition and blood product support			patients achieving a complete resonse were offered HDC with stem cell rescue
Williams, Canada, 2004	9010	HSCT (4) Comaprator (13)		Auto	ifosfamide and etoposide alternating vincristine,CY, doxorubicin and/or actinomycin +/- radiation and surgical resection	etoposide, CY with or without melphalan			Chemo +/- radiation and surgical resection	ifosfamide and etoposide alternating vincristine,CY, doxorubicin and/or actinomycin	13 patients received radiation with curitive intent of these 4 received HDC with Stem cell support

Appendix Table C25: Outcome Assessment Treatment Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Bisogno, Italy, 2009	75340	HSCT (70)	Survival	Toxicity		Children less that 10 had significantly better outcomes that older children 54.3%. Under multivariate modeling RR of 2.59 (1.3-5.1) for children over 10 for the role of age as a prognostic factor for survival
Carli, Italy, 1999	16010	HSCT (52)	Overall Survival	Event Free		who was transplanted ended up being center based not response based
Doelken, Germany, 2005	6570	HSCT (1)	Pt 1- died of progressive disease 146 days post transplant due to disease progression Pt 2- recurred three years after Auto transplant, five years after Auto transplant he received an Allo he died 379 days post Allo transplant due to disease progression.			Transplants were not perfomed on patients in complete remission
Donker, Netherlands, 2009	1420	HSCT (1)	Survival		Patient was followed and has survived 4 yearspost-transplant	Patient had severe ifosfamide tubulopathy that evolved into chronic renal insufficiency but is sAppendix Table 3with conservative therapy. Transplant was completed on a patient in complete remission.
Dunkel, USA, 2000	14610	HSCT (4)	Survival	No major harms reported	100% of patients were alive at a median FU of 57 months (46-80)	
Grundy, UK, 2001	14200	HSCT (1)	Survival		Patient recurred and died at 2 years 3 months of age.	
Hara, Japan, 1998	17950	HSCT (7)	Survival	Harms	4 alive NED median 26.5 months (15-32) 3 DOD median 6 months (3-6) 1 TRM 1 month	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Koscielniak, Germany, 1997	19800	HSCT (36)	Survival	estimated Event Free Survival at 2 years after HDC 36 +/- 7% 2 years after diagnosis 55 +/- 8%' Harms	9 alive NED median FU of 57 (32-108) months after diagnosis, 27 (20-100) months after HDC. 1 alive in 2nd CR after additional loal treatment	
Kuroiwa, Japan, 2009	390	and ifosfamide-cisplatin-etoposide	Survival		Patient is alive with controlled disease at age 3 years 11 months, 46 months after diagnosis	Patient suvived transplant and recurred with metastatic disease after transplant was treated with Chemo
Kwan, Hong Kong, 1996	20800	HSCT (1)	survival		Alive NED 3 months post transplant	
Lucidarme, France, 1998	17610	HSCT (8)	Survival	Harms	One patient was in complete remission at 33 months post-transplant 7 were DOD median 7 months (2-38) post transplant	all patients two patients were transplanted in partial remission and 6 had progressive diease at the time of transplant.
Matsubara, Japan, 2005	7580	HSCT (5)	Survival	harms	60% alive at a median of 107 months FU 40% died at a median of 26 months FU	the two patients who died developed CNS involvement the three others remained non CNS
McDowell, UK, 2010	75350	HSCT (101)	Survival	Harms	16.56 (0.76-101.39)	8 SAE were reported but not further described.
Misawa, Japan, 2003	11040	HSCT (1)	Survival- Paient died of progressive disease 165 days after transplant			Patient was NOT in CR when the transplant was completed
Moritake, Japan, 1998	18280	HSCT (1)	Survival ~21 months after transplant the patient dies due to progressive disease			Patient received donor lukocyte infusion 12 months after transplant as salvage

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Navid, USA, 2006	5930	HSCT (8), 2 transplanted 6 were not	Survival	harms	2 alive NED median 79 months (65-92) post enrollment 1 LFU at 78 months 3 DOD 12 months (9-16) post enrollment 2 Toxic Death median 6.5 (5-8) months post enrollment	Only two patients were transplanted as they reached CR and stayed on study. One who achieved CR was LFU. In a cox model for all those who were transplanted (more than just RMS) there was no evidence that transplantation had an effect on survival
Oue, Japan, 2003	10950	HSCT (1)	Survial	Harms	patient was alive 19 months after disease onset	harms were for non RMS patients as this was a mixed tumor study
Sato, Japan, 1998	48070	HSCT (5)	Event Free Survival		alive without evidence of disease (EFS) of 23.4 months post-transplant	Transplanted in Complete remission
Scully, USA, 2000	14580	HSCT(1)	Survival	Harms	Alive with secondary malignancy approxamatly 3 years after transplant for RMS	
Shaw, Israel, 1996	20050	HSCT (9)	Survival	Harms	44% DOD median 286 days follow-up 44% NED median 745 days FU 11% AWD 350 days FU	
Taguchi, Japan, 2005	7430	HSCT (1)	Survival	NR	19 months	Dead at 19 months after transplant Non CNS group
Walterhouse, USA, 1999	17240	HSCT (8)	Survival	Harms	38% DOD at median of 15 months after diagnosis (not transplanted) 75% of those transplanted are DOD median 12 months post transplant or 20 months post diagnosis 25% alive NED at 53 months post trans patient who declined transplant DOD 30 m from diag	Five patients achieved a complete resonse and were elligible for transplant, one refused

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Williams, Canada, 2004	9010	HSCT (4)	Survival	Event Free Surival		patients with embryonal histology, metastasis confined to the lung, and < 10 had 100% survival at 3 years compared to 0% for the remaining patients

Appendix Table C26: Outcome Assessment Comparator Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Breneman, USA, 2003	75360	Comparator (127)	Survival			
Carli, Italy, 1999	16010	Comparator	Overall Survival	Event Free survival		
Grundy, UK, 2001	14200					
McDowell, UK, 2010	75350	Comparator (45)	Survival	Harms	30.16 months (0.69-105.40)	
Pappo, USA, 1999	48020	Comparator (605)	survival		five year survival botroid 64% (40-88), embryonal 26% (21-31), alveolar/undiff 5% (2-8)	histologic subtype at initial diagnosis associated with survival after recurrence, but survival not affected by site of recurrence.
Pappo, USA, 2001	47860		Survival	Harms		number of metastatic sites influenced survival 1 or 2 vs. +2
Sandler, USA, 2001	12810	Comparator (152)	Survival	Harms		Patients who are < 10 or with embryonal RMS, or a GU primary site, or no nodal disease at presentation and patients lacking bone or bone amrrow involvement at presentation fared significantly better.
Van Winkle, USA, 2005	43550	Comparator (27)	Survival	Harms		Male gender (p=.015), Embryonal histology at recurrence (p=.005), and CR (p=.014) were associated in univerate analysis with improved survival
Williams, Canada, 2004	9010	Comparator (13)	Survival	Event Free Survival		

Appendix Table C27: Time to Event Outcomes Treatment Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	p	HR (95%) CI
Bisogno, Italy, 2009	75340	HSCT (70)	42.3% (30.5-53.6) 3 year survival								
Carli, Italy, 1999	16010	HSCT (52)	3 year 40.0(25.5-54.7)		~86%	~46%	40.0(25.5-54.7)	~40%	~40%	0.2 for three year versus comparator	
Matsubara, Japan, 2003	10810	HSCT (22)	45% at 5 years								
McDowell, UK, 2010	75350	HSCT (101)					23.70%		17.93%	<0.001	2.46 (1.51-4.03)
Williams, Canada, 2004	9010	All 17 together	35% (13-58) FFS 29.4% (18-40)	3 yrs							

Appendix Table C27: Time to Event Outcomes Treatment Rhabdomyosarcoma Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	p_2	HR (95% CI)_2
Bisogno, Italy, 2009	75340	HSCT (70)	Progression free survival at 3 years 35.3 (24.3-46.5)							
Carli, Italy, 1999	16010	HSCT (52)	EFS 29.7 (15.6-43.8)	~46%	~30%	29.7 (15.6-43.8)	~20%	~20%	0.3 for 3 years versus comparator	
Matsubara, Japan, 2003	10810	HSCT (22)	DFS					36%		
McDowell, UK, 2010	75350	HSCT (101)	Event Free Survial			16.53%		14.88%	<0.001	2.68 (1.64-4.37)
Williams, Canada, 2004	9010	All 17 together	Overall Survival HSCT (4) only			100%				

Appendix Table C27: Time to Event Outcomes Treatment Rhabdomyosarcoma Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_3	3 yr_3	5 yr_3	p_3	Comment
Matsubara, Japan, 2003	10810	HSCT (22)	OS (CR) vs. OS (PR) OS Embryonal vs. OS Alveolar OS >8 years vs. OS <8 years		70% vs. 0% 75% vs. 0% 18% vs. 75%	no difference reported 0.015 no difference reported	
McDowell, UK, 2010	75350	HSCT (101)					This study also reported survival differences by induction treatment, however this is beyond the scope of the review.
Williams, Canada, 2004	9010	All 17 together	Failure Free Survival	75% (33-107)			

Appendix Table C28: Time to Event Outcomes Comparator Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr
Breneman, USA, 2003	75360	comparator (127)			~85%	~50%	39% (30-48)	~25%	~25%
Carli, Italy, 1999	16010	Comparator (44)	27.7 (13.3-42.1) 3 year		~66%	~35%	27.7 (13.3-42.1)	~26%	~26%
McDowell, UK, 2010	75350	Comparator (45)					62.14%		47.68%
Pappo, USA, 1999	48020	Comparator (605)	17% (14-21)	4.7 years (.8-12.6)					
Pappo, USA, 2001	47860	Comparator (48)				46% (31-60)			
Sandler, USA, 2001	12810	Comparator (152)			~75%	~43%	~40%	~34%	~37.5%
Van Winkle, USA, 2005	43550	Comparator(27)			56 (10)	26 (8)			
Williams, Canada, 2004	9010	Comparator (13)	15% (-4-35)	3 year					

Appendix Table C28 Time to Event Outcomes Comparator Rhabdomyosarcoma Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Comment
Breneman, USA, 2003	75360	comparator (127)	FFS	~69%	~33%	25% (17-33)	~20%	~20%	FFS influenced by distant metastasis in lymph nodes. OS influenced by number of metastatic sites
Carli, Italy, 1999	16010	Comparator (44)	EFS	~53%	~30%	19.2 (6.8-31.6)	~20%	~20%	
McDowell, UK, 2010	75350	Comparator (45)	Event free survival			54.92%		51.00%	
Pappo, USA, 1999	48020	Comparator (605)							
Pappo, USA, 2001	47860	Comparator (48)	failure Free	~57%	24% (13-36)	~21%	~21%		
Sandler, USA, 2001	12810	Comparator (152)	FFS	~63%	~36%	~28%	~28%	~27%	
Van Winkle, USA, 2005	43550	Comparator(27)							
Williams, Canada, 2004	9010	Comparator (13)	Failure Free survival			15% (-4-35)			

Appendix Table C29: Adverse Events Treatment Rhabdomyosarcoma

Study (Investigator, country, record#)	Record Number	Group (N)	% Infection	Comment	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Comment TRM	% Secondary Malignancy	Comments SM
Bisogno, Italy, 2009	75340		12.7					4.3			
Carli, Italy, 1999	16010				HSCT (52)			1.9 % TRM (1/52)	Sepsis related death		
Hara, Japan, 1998	17950		14% (1/7)	sepsis			1 month	14% (1/7)	on additional non rms patient experienced TRM so in all 2/28 (7.1%)		
Koscielniak, Germany, 1997	19800		2.8			one patient dies due to sepsis					
McDowell, UK, 2010	75350							5.0	It is unclear from the authors description if any of these are within the first 100 days.		
Navid, USA, 2006	5930	HSCT (8)						25% (2/8)	two patients experienced TRM (radiation pneumonitis and disseminated alveolar infection)		

Study (Investigator, country, record#)	Record Number	Group (N)	% Infection	Comme nt	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TR M	Comment TRM	% Secondary Malignancy	Commen ts SM
Oue, Japan, 2003	10950							8.3	Patients from a mixed tumor study. Neither of these patients had RMS		
Scully, USA, 2000	14580	HSCT (1)								one patient	developed precursor T-lymphoblastic lymphoma and early myeloid dysplastic syndrome
Shaw, Israel, 1996	20050							6.6	Patients from a mixed tumor study. Neither patient had RMS		
Walterhouse, USA, 1999	17240			Sepsis in 4 fungal infection in 1							

Appendix Table C30: Adverse Events Comparator Rhabdomyosarcoma

Study (Investigator, country, record.)	Record Number	Group (N)	% Infection	Comment	Group (N) TRM	% TRM	Comment TRM
Carli, Italy, 1999	16010				Comparator (44)	2.3% TRM (1/44)	due to anthracycline related cardiotoxicity
McDowell, UK, 2010	75350	Comparator (45)	2% (1/45)			4.4% (2/45)	
Pappo, USA, 2001	47860	Comparator (48)	8.3% Bacterima (4/48)	at various doses of Topotecan		4.2% (2/48)	died of tracheobronchitis and interstitial pneumonitis. One other patient died on treatment of adult respiratory distress but the authors said this could not with certianty be related to Topotecan.
Sandler, USA, 2001	12810		30.9% Grade IV, and 5.2%) Grade V	28.3% (43/152) Grade IV infection, and 4.6%(7/152) Grade V infection 2.6% (4/152) catheter infection Grade IV, .7 % (1/152) catheter infection Grade V		5.9% (9/152)	seven infection related and two (thrombocytopenia and hemorrhage and one to pulmonary toxicity) It is unclear if these are all within the first 100 days
Van Winkle, USA, 2005	43550					0.6	TRM from infection among 336 courses of ICE

Appendix Table C31: Design, Participant Selection and Enrollment Retinoblastoma

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)
Antoneli, Brazil, 2003	48830	malignant solid tumor	retinoblastoma	extraocular	Comparator (83)	1987-1991 period 1 1992-2000 period 2	case series	83	8 LFU (9.6%)
Chang, Taiwan, 2006	48660	malignant solid tumor	Retinoblastoma	Extraocular	Comparator(15)	1982-2004	retrospective analysis of medical records	15	0
Chantada, Argentina, 1999	16020	Malignant solid tumor	retinoblastoma	Extraocular	Comparator (10)	1995-1998	Case Series	10	0
Cozza, Italy, 2009	70	malignant solid tumor	retinoblastoma	metastatic retinoblastoma	HSCT (3) Comparator (3)	1988-2007	retrospective review	6	0
Dai, Canada, 2008	1410	malignant solid tumor	retinoblastoma	Trilateral retinoblastoma	HSCT (1)		Case report	1	1
Dunkel, USA, 2000	14610	malignant solid tumor	retinoblastoma	metastatic	HSCT (4)	1993-1996	Case series	4	0
Dunkel, USA, 2010	71500	malignant solid tumor	trilateral retinoblastoma	trilateral retinoblastoma	HSCT (13)	1997-2005	Case Series	13	0
Gunduz, Turkey, 2006	5310	malignant solid tumor	retinoblastoma	metastatic retinoblastoma	Comparator (18)	1999-2005	retorspective case series	18	0
Hertzberg et al, Germany, 2001	13810	Malignant Solid Tumors	Retinoblastoma	Metastatic	HSCT (1)	NR	Case Report	1	0

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)
Jubran, USA, 2004	9480	malignant solid tumor	retinoblastoma	metastatic	HSCT (4) Comparator (3) untreated (3)	1991-1999	retrospective review	10	0
Kremens, Germany, 2003	10860	malignant solid tumor	retinoblastoma	metastatic	HSCT (5)	1992-2001	Case series	5	0
Matsubara, Japan, 2005	7580	malignant solid tumor	retinoblastoma	metastic retinoblastoma without CNS involvement	HSCT (5)	1986-2000	Case Series	5	0
Moshfeghi et al, USA, 2002	12230	malignant solid tumor	Retinoblastoma	Metastatic	HSCT (1)	NR	Case Report	1	0
Namouni, France, 1997	18090	Malignant Solid Tumor	Retinoblastoma	Metastatic or relapse or invasion of the cut end of optic nerve	HSCT (34)	1989-1994	Case Series	25, received HSCT' 9 progressed/died before treatment	
Rodriguez-Galindo, USA, 2003	10420	malignant solid tumor	retinoblastoma	metastatic	HSCT (4)		case series	4	0
Schvartzman, Argentina, 1996	49250	malignant solid tumor	retinoblastoma	extraocular	Comparator (41) Stage II(29) Stage III (6) Stage Iv (6)	1987-1993	prospective single arm non-randomized	41	0
Taguchi, Japan, 2005	7430	malignant solid tumor	retinoblastoma	Metastatic	HSCT (1)		Case Report	1	0

Appendix Table C32: Participant Characteristics Treatment Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)
Cozza, Italy, 2009	70	HSCT (3)		440months (diagnosis of metastasis whole group n=6)	18-110 months (n=6)		50% male, 50% female (n=6)		CSF, Pineal, orbit, bone and bone marrow
Dai, Canada, 2008	1410	HSCT (1)	12 months				Female	trilateral retinoblastoma	with CSF involvement
Dunkel, USA, 2000	14610	HSCT (4)	30.5 months at diagnosis	30.5 months at diagnosis	17-44		50% Male, 50 % Female		distant metastasis (BM, Orbit, liver, bone) no CNS envolvement
Hertzberg et al, Germany, 2001	13810	HSCT (1)	7				F	metastic retinoblastoma	lymph nodes, bones and bone marrow
Jubran, USA, 2004	9480	HSCT (4)	12.3 month at diagniosis	11.5 months at diagnosis	2-24				distant no CNS involvement
Kremens, Germany, 2003	10860	HSCT (5)	51.8 months (treatment)	34 months	20-110				bone marrow, extraoccular tumor
Matsubara, Japan, 2005	7580	HSCT (5)	17.6 months at diagnosis	16 months at diagnosis	3-41		20% Male, 80% Female		distant metastasis
Moshfeghi et al, USA, 2002	12230	HSCT (1)	5			White	F	metastatic	bone marrow, righ humerus, both supraorbital bones, and both tibias, ovary

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)
Namouni, France, 1997	18090	HSCT (25)		34 months	(9-125) months		76% Male 14% Female	extraocular retinoblastoma	cut end of optic nerve (5) disruption of ocular globe(1) isolated orbital relapse (7) bone or bone marrow (8) CNS/spinal axis (4)
Rodriguez-Galindo, USA, 2003	10420	HSCT (4)	28.5 age at diangosis	30.5	17-36	75% white 25% hispanic	75% Male 25% female		distant metastis no CNS involvement
Taguchi, Japan, 2005	7430	HSCT (1)	4				M	metastatic	maxilla and mandible

Appendix Table C33: Participant Characteristics Comparator Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Antoneli, Brazil, 2003	48830	Comparator (83)	32.9 months		2-145	62.7% White	53% Male	extraocular retinoblastoma	69 class I/III CCG classification 14 Class IV/V	Class IV CNS involvement
Chang, Taiwan, 2006	48660			26.3 at diagnosis	1.7-89 months			all stages of extraocular retinoblastoma were reported together	most common sites Orbit (7) and CNS (7)	
Chantada, Argentina, 1999	16020	Comparator (10)		2 years	1-7		40% M, 60% F	extraocular	Varrious sites including 3 patients with bone marrow involvement at diagnosis (30%)	
Cozza, Italy, 2009	70	Comparator (3)		41.5 at diagnosis	3-110 at diagnosis		50% male' 50% female		CSF (3)	
Gunduz, Turkey, 2006	5310	Comparator (18)		45 months at diagnosis	13-86				distant and CNS (5) CNS (9) distant ony (4)	
Jubran, USA, 2004	9480	Comparator (6)	31.3 months at diagnosis	17.5 months	1-96				distant no CNS	two patients were not treated for their extraocular disease, one received no treatment at all
Schvartzman, Argentina, 1996	49250	Comparator (41)						Extraocular retinoblastoma	Orbital (29) intracranial (6) these patients had CNS mets hematogenous metastasis (6) three of these patients also had CNS mets	

Appendix Table C34: Treatment Characteristics Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen
Antoneli, Brazil, 2003	48830	Comparator (83)						Chemo +/- radiation	Cisplatin, teniposide, vincristine, doxorubicin, cyclophosphamide (period 1) Cisplatin and teniopside with alternating ifosfamide and etoposide (period 2)
Chang, Taiwan, 2006	48660	Comparator (15)						Chemo +/- radiation	cyclophosphamide, vincristine, adriamycin, intrathecal methotrexate +/- radiation
Chantada, Argentina, 1999	16020	Comparator (10)					GCSF, platelet and RBC transfusions	Idarubicin	10mg/m2/d
Cozza, Italy, 2009	70	HSCT (3) comparator (3)	PBSC	auto	ifosfmide, carboplatin, etoposide, vincristine, doxorubicin cyclophosphamide (some combination of these)	etoposide, thiotepa, cyclophamide +/- radiation		ifosfmide, carboplatin, etoposide, vincristine, doxorubicin cyclophosphamide, thiotepa with methotrexate (some combination of these)	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen
Dai, Canada, 2008	1410	HSCT (1)	PBSC	Auto	cyclosporine-modulated vincristine, etoposide, carboplatin with intraventricular topocan/cytarabine	carboplatin, etoposidem cyclophosphamide			
Dunkel, USA, 2000	14610	HSCT (4)	BM (3) PBSC (1)	Auto	vincristine, doxorubicin, cyclophosphamide, cisplatin, etoposide, carboplatin (some combination)	thiotepa and carboplatin + radiation			
Dunkel, USA, 2010	71500	HSCT (13)	PBSC (6) Marrow (1) PBSC and Marrow (1) Unknown (1)	Auto	vincristine, cicplatin, cyclophosphamine and stoposide (11) carboplatin, etoposide, cyclophosphamide, doxorubicin (1)' single agen cyclophosphamide (1)	thiotepa based (6) cyclophosphamide and melphalan (2) both (one tandem)			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen
Gunduz, Turkey, 2006	5310	Comparator (18)					GCSF was given to those on treatment B	Treatment A cyclophosphamide, doxorubicin, vincristine, carboplatin, etoposide with intrathecal chemo +/- radiation(4) Teatment B- ifosfamide, carboplatin, etoposide +/- radiation (14)	
Hertzberg et al, Germany, 2001	13810	HSCT (1)	PBSC	Auto	vincristine, cyclophosphamise, etoposide, and carboplatin	HDC Thiotepa by etoposide and carboplatin	Platelet and Red Blood cell transfusion		
Jubran, USA, 2004	9480	HSCT (4) Comparator (6)	BM	Auto	Chemo +/- radiation one patient received no treatment	cyclophosphamide, etoposide and thiotepa		three received no treatment for extraocular disease one radiation alone, one chemo alone, one chemo+ radiation	cyclophosphamide, etoposide, vincristine, carboplatin, thiotepa
Kremens, Germany, 2003	10860	HSCT (5)	PBSC	Auto	cisplatin, etoposide, vindesinem vincristine, DTIC, ifosfamine, doxorubicin or cyclophosphamide, etoposide, carboplatin, vincristine	thiotepa, etoposide, carboplatin (4) +/- radiation BCNU, cyclophosphamide, and etoposide (1)	barrier nursing, oral decontamination, oral antifungal, pneumocystis carinii propholaxis, parenteral nutritional support		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen
Matsubara, Japan, 2005	7580	HSCT (5)	PBSC (1) BM (4)	Auto	vincristine, cyclophosphamide, doxorubicin, cisplatin, etoposide, carboplatin (some combination) +/- radiation	melphalan with some combination of cisplatin,cyclophosphamide, etoposide, carboplatin, thiotepa +/- radiation	GCSF		
Moshfeghi et al, USA, 2002	12230	HSCT (1)		Auto	six courses of chemotherapy, local orbital radiation				
Namouni, France, 1997	18090	HSCT (25)	Marrow	Auto	etoposide, carboplatin, cyclophosphamide, vincristine, doxorubicin, 8 patients had irradiation	Carboplatin,Etoposide, cyclophosphamide +/- radiation	parenteral antibiotics, antifungal therapy, platelet and RBC transfusions		
Rodriguez-Galindo, USA, 2003	10420	HSCT (4)	BM	Auto	carboplatin, etoposide, cyclophosphamide, doxorubicin and carboplatin or cisplatin + radiation	carboplatin and etoposide or cyclophosphamide with busulfan and melphalan or etoposide or topocan	antifungal therapy		
Schvartzman, Argentina, 1996	49250	Comparator (41)						Chemo +/- radiotherapy	Cyclophosphamide, Doxyorubicin, Vincristine Stage III and IV also received cisplatin and etoposide
Taguchi, Japan, 2005	7430	HSCT (1)			carboplatin and etoposid				

Appendix Table C35: Outcome Assessment Treatment Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Cozza, Italy, 2009	70	HSCT (3)	Survival	NR	66% alive at median FU of 61.5 months 33% dead at 16 months	
Dai, Canada, 2008	1410	HSCT (1)	Survival	NR	death at 32 months follow-up	she had CNS involvement
Dunkel, USA, 2000	14610	HSCT (4)	Survival	No major harms reported	100% of patients were alive at a median FU of 57 months (46-80)	
Hertzberg et al, Germany, 2001	13810	1	Survival	Harms	Alive 4 years+ post transplant	
Jubran, USA, 2004	9480	HSCT (4)	Survival	No major harms reported	100% dead at median of 25 months FU	
Kremens, Germany, 2003	10860	HSCT (5)	Survival	No major harms reported	100% alive median 57 months (8-107)	
Matsubara, Japan, 2005	7580	HSCT (5)	Survival	harms	60% alive at a median of 107 months FU 40% died at a median of 26 months FU	the two patients who died developed CNS involvement the three others remained non CNS
Moshfeghi et al, USA, 2002	12230	1	Survival	NR	16 months	dead at 16 months
Namouni, France, 1997	18090	cut end of optic nerve/ocular globe (6) Isolated orbital (7) Various metastasis (8) CNS/spinal axis (4)	Overall Survival	Harms	Cut end/globe-83% (NED) at median 33 (8-55) 20% (DOD) 9 months Isolated orbital-86% (NED) at median 51.5 (25-74), 14% (PD) 5 bone or bone marrow-63% (NED) at median 37 (11-70), 37% (DOD) 13(10-20) CNS-75% (DOD) at median 10 (7-26), 25% (NED) 63	all numbers are months the 37% DOD with bone mets developed CNS after transplant
Rodriguez-Galindo, USA, 2003	10420	HSCT (4)	Survival	Harms	50% alive at median FU of 6.5 years (6-7) 50% dead at median FU of 66 months (44-88)	2 who are deceased developed CNS involvement
Taguchi, Japan, 2005	7430	HSCT (1)	Survival	NR	19 months	Dead at 19 months after transplant Non CNS group

Appendix Table C36: Outcome Assessment Comparator Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Antoneli, Brazil, 2003	48830	Comparator (83)	Survival	Harms			
Chang, Taiwan, 2006	48660	Comparator (15)	Survival	No major harms reported			
Chantada, Argentina, 1999	16020	Comparator (10)	Survival	Harms (no majors reported)	toxicity was evaluated using the modified Children's cancer group criteria.	60% (NED) 16 months (4-30) 20% (DOD) 7.5 months (5-10) 10% (dead of parental abuse) 8 months 10% DOD with CNS involvement at 3 months	in document 1 DOD at 3 mon CNS, NON-CNS 75% NED, 25% DOD. The one patient dead of parental abuse was not included.
Cozza, Italy, 2009	70	Comparator (3)	Survival	NR		100% dead at median of 8 months FU	
Gunduz, Turkey, 2006	5310	Comparator (18)	Survival	No majors reported		100% of patients with CNS involvement were dead at mean 24 months fu(4-62), this is 9 with CNS only and 5 with CNS and distant metastasis. 100% with distant metastasis only were alive at a median FU of 28.5 months (9-62)	
Jubran, USA, 2004	9480	Comparator (6)	Survival	No major harms reported		100% of those treateated(3) were dead at median 7 month FU 100% of those not treated(3) were dead at median 2 months FU	4 patients had CNS involvement (3 were untreated)
Schvartzman, Argentina, 1996	49250	Comparator (41)	Survival	No major Harms reported		50 months	

Appendix Table C37: Time to Event Outcomes Treatment Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr
Namouni, France, 1997	18090	HSCT (25)	~70%	22 months	~97%	~70%	~70%	~70%	~70%

Appendix Table C37: Time to Event Outcomes Treatment Retinoblastoma Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Outcome_3	Med (mos)_3	1 yr_3	2 yr_3	3 yr_3	4 yr_3	5 yr_3	Comment
Namouni, France, 1997	18090	HSCT (25)	Intention to treat overall survival n=34	~88 %	~60 %	~57 %	~52 %	~52 %	Event Free intention to treat n=34	37 months	~88 %	~62 %	~57 %	~53 %	~53 %	8 of 9 excluded died due to CNS involvement

Appendix Table C38: Time to Event Outcomes Comparator Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	Comment
Antoneli, Brazil, 2003	48830	Comparator period 1(43) Comparator period 2(40)	Period 1 Class I/III 65.3% Class IV-V 0% Period 2 Class I/III 75.5% class IV/V 20%						no differences in survival between treatment periods was found
Chang, Taiwan, 2006	48660	Comparator (15)	39.2 +/- 14.7 at 5 years						
Schvartzman, Argentina, 1996	49250	Comparator (41)	Stage II 85% (75-97) 29 pts Stage III 0 (CNS) 6 pts Stage IV 50% (11-89) 6 pts	39 months (12-84) of surviving patients	Stage II 85% stage III and IV ~50%	Stage II 85% stage III and IV ~25%	Stage II 85% stage III and IV ~25%	Stage II 85% stage III and IV ~25%	

Appendix Table C39: Adverse Events Treatment Retinoblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Infectious	Severity or Grade	%	Comment	TRM	% TRM	Comment TRM
Dunkel, USA, 2010	71500	HSCT (13)	Infectious				TRM	7.7% (1/13)	Death due to septicemia and multi-organ failure during induction chemo
Rodriguez-Galindo, USA, 2003	10420	HSCT (4)	Infectious	candida albican sepsis	25% (1/4)	sucessfully treated with antifungals	TRM		

Appendix Table C40: Adverse Events Comparator Retinoblastoma

Study (Investigator, country, record.)	Record Number	Group (N)	TRM	% TRM	Secondary Malignancies	% SM	Comments SM
Antoneli, Brazil, 2003	48830	Comparator (83)	TRM	4.2	Secondary Malignancies	3.6 % (3/83)	two osteogenic sarcoma and one nonlymphocytic leukemia

Appendix Table C41: Design, Participant Selection and Enrollment Neuroblastoma

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Berthold, Germany, 2005	6760	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk	295	1997-2002	RCT	212	83	
George, USA, 2006	5440	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk	97	1994-2002	case series	82	8	6 (of 97) pts developed progressive disease during induction; 2 did not receive HSCT because of parental wishes; 82 (of 89) patients underwent tandem HSCT
Hobbie, USA, 2008	1690	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk	35	1997-2001	case series	13	22	Lost to F/U: 18 pts died of progressive disease; 4 pts alive with no disease with no follow-up at centre This study is a sub-group analysis (from Georg, 2006, #5440) of late effects

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Kim, South Korea, 2007	2870	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk	36	1996-2004	retrospective analysis	36	0	
Ladenstein, EGBMT, 2008	1610	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk Relapse Not specified	3571	1978-2006	case series	3421 (3350 for outcomes)	(221 for outcomes given autologous single and tandem HSCT)	80%, consolidate high-risk; 10%, relapse; 10%, specified
Matthay, US, 2009; 1999	6210	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk	560	1991-1996	RCT	539	21	
Pritchard, United Kingdom, 2005	8030	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk	90	1982-1985	RCT	65	35	
Sung, South Korea, 2007	3950	Malignant Non-Hematopoetic	Neuroblastoma	Consolidate high-risk	52	1997-2005	case series	52		

Appendix Table C42: Participant Characteristics Treatment Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)
George, USA, 2006	5440	97		35 mos, at diagnosis	6 mos-18 yrs, at diagnosis		IV, 90%; III, 10%	Abdomen, 37; Adrenal, 54; Cervical/paraspinal, 7; Unknown, 2
Hobbie, USA, 2008	1690	13		22 mos	13 mos-72 mos	M, 85%; F, 15%	IV	
Kim, South Korea, 2007	2870	36		3-yr, at diagnosis	7 mos-121 mos	M, 69%; F, 31%	III, 6%; IV, 94%	Abdomen, 89%; Other, 11%
Ladenstein, EGBMT, 2008	1610	3350		47 months	4-744 months	59% M, 41% F	IV, 89% (n=1,681)	
Sung, South Korea, 2007	3950	52		36 mos, at diagnosis	13 mos-129 mos		IV, 100%; MYCN-amplified, 56%; multi-organ (>=3) metastasis, 38%	Shimada classification: favorable, 27; unfavorable, 71; undetermined, 2 Site: Abdomen, 81; Other, 19

Appendix Table C43: Participant Characteristics Comparator Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Age (Range)	Disease Stage/category	Disease Histology/Site (%)
Berthold, Germany, 2005	6760	149	(< 1 year, 8%; > 1 year, 92%)	I, 1%; II, 1%; III, 5%; IVS, 3%; IV, 90%	
Matthay, US, 2009; 1999	6210	189	(< 1 yr, 3%; 1-2 yr, 23%; > 2 yr, 74%, at diagnosis)	III, 11%; IV, 89%	Favorable, 3%; Unfavorable, 63%; Unknown, 33%
Pritchard, United Kingdom, 2005	8030	32	(6-12 mos, 9%; 13-24 mos, 25%; > 24 mos, 66%, at diagnosis)	III, 19%; IV, 81%	Abdominal, 88%; Other, 12%

Appendix Table C44: Treatment Characteristics Neuroblastoma

Study (Investigator, country, record#)	Disease	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Berthold, Germany, 2005	Neuroblastoma	6760	149	PBSC	single auto	3 cycles of chemo (cisplatin and etoposide); vindesine; 3 cycles of vincristine and dacarbazine; infosfamide; doxrubicin; radiotherapy; surgery	melphalan; etoposide; carboplatin; (dose and drug adjustments in 6 patients)	chimeric monoclonal antibody; retinoic acid after Nov 2002	drugs given to control pain and allergic reactions during immunotherapy	maintanence chemotherapy	oral cyclophosphamide	
George, USA, 2006	Neuroblastoma	5440	82	PBSC	tandem auto auto	5 cycles of chemo (multi-agents); surgery after 4th or 5th cycle; radiotherapy	high-dose chemo (etoposide, cyclophosphamide, carboplatin, melphalan); total body irradiation	13-cis-retinoic acid				

Study (Investigator, country, record#)	Disease	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Hobbie, USA, 2008	Neuroblastoma	1690	13	PBSC	tandem auto auto	5 cycles of chemo; surgery after 4th or 5th cycle; radiotherapy	high-dose chemo (etoposide, cyclophosphamide, carboplatin, melphalan) and total body irradiation	13-cis-retinoic acid				
Kim, South Korea, 2007	Neuroblastoma	2870	36	PBSC	tandem auto auto, 25%; single auto, 75%	4-5 cycles of chemo (cisplatin, VP-16, doxorubicin, cyclophosphamide); surgery; radiotherapy and chemo	MEC (melphalan, etoposide, carboplatin), 65% (N=46 procedures); no total body irradiation	interleukin-2; 13-cis-retinoic acid				single-auto group consisted of CD34+ non-selected arm (n=13, 36%) and CD4+ selected arm (n=14, 39%)

Study (Investigator, country, record#)	Disease	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Ladenstein, EGBMT, 2008	Neuroblastoma	1610	3350	BM, 41%; 3%, BM+PBSC; PBSC, 56% (n=3295)	tandem auto auto, 14%; single auto, 86%	not specified 1-4 cycles of chemo (various agents); surgery; radiotherapy; total body irradiation (33%)	busulphan; melphalan; cyclophosphamide; thiotepa; total body irradiation (14%, n=2,333) 1-4 cycles of chemo (various agents); melphalan (81%); total body irradation (34%)					auto-transplant group
Matthay, US, 2009; 1999	Neuroblastoma	6210	189	BM	single auto	5 cycles of chemo (cisplatin; doxorubicin; etoposide; cyclophosphamide); radiotherapy; surgery	carboplatin; etoposide; melphalan; total body irradiation	retinoic acid (n=50)	growth factors	conventional therapy	3 cycles of cisplatin; etoposide; doxorubicin; ifosfamide; mesna	

Study (Investigator, country, record#)	Disease	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Pritchard, United Kingdom, 2005	Neuroblastoma	8030	32	BM	single auto	vincristine; cyclophosphamide; cisplatin; teniposide; surgery (no radiotherapy)	melphalan		nutritional supplements	no further therapy		

Study (Investigator, country, record#)	Disease	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Sung, South Korea, 2007	Neuroblastoma	3950	52	PBSC	tandem auto auto, 88%; single auto, 12%	1997-2003: 5-7 cycles of chemotherapy; surgery; radiotherapy (if tumor remained post-surgery); 1-3 cycles of chemotherapy if no tumor or 3-5 cycles of chemo if tumor evident 2004-2005: 6 cycles of chemo; surgery; 3-4 cycles of chemo	1997-2003: high-dose chemo 2004-2005: chemo and total body irradiation	13-cis-retinoic acid and interleukin-2				

Appendix Table C45: Outcome Assessment Treatment Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
George, USA, 2006	5440	82	OS; PFS	secondary malignancies	5.6-yr (15.1 mos-9.9-yr)	
Hobbie, USA, 2008	1690	13	Endocrine; Sensory; Musculoskeletal; Pulmonary; GI; Dental; Renal; Cardiovascular; Secondary malignancies		9-yr since diagnosis	
Kim, South Korea, 2007	2870	9 (tandem auto auto)	OS; DFS		27 mos (1-93) from transplant; 42 mos (11-103) from diagnosis	
Ladenstein, EGBMT, 2008	1610	455	OS; EFS		5-yr	tandem auto auto
Sung, South Korea, 2007	3950	50	OS; EFS	SM; TRM; Other	53 mos (19 mos-117 mos)	

Appendix Table C46: Outcome Assessment Comparator Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes
bernstein, USA/Canada 2006	6290	110	EFS	OS
Kushner, USA, 1995	21430	24	PFS	
Milano, Italy, 2006	43290	36	EFS OS	
Sari, Turkey, 2010	42790	36	EFS OS	
van winkle, USA, 2005	43550	22	OS	

Appendix Table C47: Time to Event Outcomes Treatment Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	3 yr	4 yr	5 yr	Test	p	Outcome_2	3 yr_2	5 yr_2	Test_2	p_2
George, USA, 2006	5440	82	time of 1st transplant to death	74 (62-82)		64 (52-74); 7-yr, 54 (38-67)	Kaplan-Meier		PFS, time from date of 1st transplant to progression or relapse of primary tumor or death	61 (50-71)	54 (42-64); 7-yr, 52 (40-63)	Kaplan-Meier	
Kim, South Korea, 2007	2870	9	OS	66.7 (19.3)			Kaplan-Meier	NS compared to CD34+ selected single-auto arm	DFS	50 (20.4)		Kaplan-Meier	p = 0.50 (NS) compared to CD34+ selected single-auto arm
Ladenstein, EGBMT, 2008	1610	455	OS			33 (3)		0.10	EFS		27 (2)		0.19
Sung, South Korea, 2007	3950	52	OS			64.3 (14.3)	Kaplan-Meier (log-rank)		EFS		62.1 (13.7)	Kaplan-Meier	

Appendix Table C48: Time to Event Outcomes Comparator Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	3 yr	5 yr	Test	p	HR (95%) CI	Outcome_2	3 yr_2	5 yr_2	Test_2	p_2	HR (95% CI)_2
Berthold, Germany, 2005	6760	149	death from any cause or until last exam if patient survived	62 (54-70)		Kaplan-Meier (log-rank)	0.09	1.329 (0.958-1.843)	EFS; time until disease progression or relapse, a 2nd neoplastic disease, or death from any cause or until last exam	47 (38-55)		Kaplan-Meier (log-rank)	0.02	1.404 (1.048-1.881)
Kim, South Korea, 2007	2870	14	OS	55.1% (+/-13.9)		Kaplan-Meier			DFS	40.6% (+/-14.7)		Kaplan-Meier		
Ladenstein, EGBMT, 2008	1610	2895	OS		38 (1)				EFS		33 (1)			
Matthay, US, 2009; 1999	6210	189	definition not mentioned		39 (4%)	log-rank	0.39 (compared to controls)		EFS		30 (4)	log-rank	0.04 (compared to controls)	
Pritchard, United Kingdom, 2005	8030	32	time to death from any cause		47 (30-64)	log rank	0.1		EFS		38 (21-54)	log-rank	0.08	

Appendix Table C49: Time to Event Outcomes Regression Modeling Neuroblastoma

Study (Investigator, country, record#)	Record Number	Design/Outcome/Model	Candidate predictors / Methods for Identifying Candidates	Univaraiate Results, Variable (p value)	Selected Predictors/ Methods for Selecting predictors Multivar	Proportional Hazards Assumption Assessed?/Interactions Considere	Multivariate Model Results, Variable (p Value)	Discrimination/Validation Methods/Results
Ladenstein, EGBMT, 2008	1610	Cox proportional hazards	OS: age at transplant (< 2 yr vs. > 2-yr)				Hazards Ratio (95% CI, p-value): 1.6 (1.4-1.9; < 0.0001)	significantly better OS rates in patients less than 2 years of age at diagnosis
Sung, South Korea, 2007	3950	Cox proportional hazards	EFS	EFS (< 0.05)	application of TBI, application of local radiotherapy, longer interval (>= 12 weeks) between 1st and 2nd transplant.	Yes	Hazards Ratio (95% CI, p-value): EFS, 9.66, 7.17, 5.73; 1.31-71.26, 1.69-30.38, 1.32-24.88; 0.026, 0.007, 0.020	EFS, application of TBI and local radiotherapy, and longer interval between transplants being favorable predictors.

Appendix Table C50: Adverse Events Treatment Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Severity of Grade Secondary Malignancies	F/U (mos) SM	% SM	Comments SM
Burdach, Germany and Austria, 2000	14310	28								
Burdach, Germany, 2003	10030	reported engr, TRM, infec compl, sec malig, and major organ tox, but not by age of < or > 17 yrs								
Burke, USA 2007	4060	7	sepsis n=1		0	0				
Costa, USA, 2008	1710	1			0	0	AML at 53 months post HSCT			
Drabko, Poland 2005	6680	21				5% (n=1 day 35 from multiorgan failure secondary to infection)				
Hara , japan 1998	17950	3				0 NR				
Harimaya, Japan, 2003	9850	2			0	0				
Kasper, Germany, 2006	2570	5			0	0				
Koscielniak Germany 2005	7860				0	0				

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Severity of Grade Secondary Malignancies	F/U (mos) SM	% SM	Comments SM
Kushner, USA, 2001	14240	1 HSCT pt died at 17 mos after HSCT with NED but pulmonary failure								
lucas, usa 2008	2450	1			0	0				
Lucidarme, france, 1998	17610	3				0 (NR)				
Meyers, USA, 2001	13670		sepsis leading to death	4% n=1 patient from HSCT group (incl in TRM)		of HSCT group n=23 n=3 13%				
Navid, US and Canada, 2006	5930	9			0	0				
Numata, Japan, 2002	12130				0	0	CML chronic phase	50 months after HSCT		

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Severity of Grade Secondary Malignancies	F/U (mos) SM	% SM	Comments SM
ozkaynak, usa 1998	18540	15			0 (one patient not assessable secondary to early toxic death)	n=2 ATN day 0 and septic shock day 8				
pession, italy, 1999	16120	3				0 NR				
Prete, italy 1998	17210	17				0				
Tanaka, Japan, 2002	11770			0		0	CML		14 %	not clear if the 35 y/o pt or one of the 6 abstracted pts

Appendix Table C50: Adverse Events Treatment Neuroblastoma Continued

Study (Investigator, country, record#)	Record Number	% Hepatic veno-occulusive disease (Hepatic Sinusoidal Obstruction)	Comments hVOD	Severity of Grade SHE	% SHE
Drabko, Poland 2005	6680	10%	moderate to severe		
Meyers, USA, 2001	13670			HSCT pt died from hemorrhagic pericarditis (included in TRM)	4%

Appendix Table C51: Adverse Events Comparator Neuroblastoma

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	Severity of Grade Secondary Malignancies	F/U (mos) SM	% SM
bernstein, USA/Canada 2006	6290		death	5 of 110 (4.5%)	MDS	at 20 mos after dx	1/110 1%
Bhatia,USA, 2007	43210						cumulative incidence of t-MDS/AML of 11% at 5 yrs from dx
Kushner, USA, 1995	21430	24			leukemia dead at 10.5 mos after HSCT in CR from ESFT		4
Meyers, USA, 2001	13670	9 nonHSCT		11% sepsis during induction CT			
Sari, Turkey, 2010	42790	36					0%

Appendix Table C52: Design, Participant Selection and Enrollment Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Agarwal, USA, 2009	72940	Malignant Non-Hematopoetic	Germ cell tumor	Relapsed	37	1995-2005	case series	37	0	
Lazarus, USA, 2007	72950	Malignant Non-Hematopoetic	Germ cell tumor	Relapsed	32	1989-2001	retrospective analysis of CIBMTR data	32	0	20 tandem; 12 single; based on data from the CIBMTR on childhood cohort
De Giorgi, UK, 2005	77240	Malignant non-hematopoetic	Germ cell tumors	Relapsed	18	1987-2003	cohort	18		
Einhorn, USA, 2007	77230	Malignant non-hematopoetic	Germ cell tumors	Relapsed	17	1996-2004	Case series	17	0	Pediatric data from author; N=184

Appendix Table C53: Participant Characteristics Treatment Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)
Lazarus, USA, 2007	72950	20		20	17-20		Testes (100)	NS (67); SM (0); CC (0); EB (33); Other (0)
Einhorn, USA, 2007	77230	17		20	17-21		NS (81); SM (19)	Testes

Appendix Table C54: Participant Characteristics Comparator Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Agarwal, USA, 2009	72940	37	28	9-59	M (92)	Testes (65); Chest/Neck/RP (27); CNS (8)	NS (84); SM (16)	4 (11%) pediatric patients (0-19 yrs)
Lazarus, USA, 2007	72950	12	19	15-20		Testes (90); Extragonadal (10)	NS (53); SM (21); CC (16); EB (5); Other (5)	
De Giorgi, UK, 2005	77240	18	6.5	1-18	M (56)	CNS (39); Sacr (39); Retro (17); Med (6)	NG (94); GM (6)	

Appendix Table C55: Treatment Characteristics Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Comparative Treatment	Comparative Treatment Dose/Regimen
Agarwal, USA, 2009	72940	37	PBSC	single auto	4 cycles of cisplatin-based chemotherapy (n=29); additional chemotherapy (n=8)	etoposide; caboplatin	(G-CSF)		
Lazarus, USA, 2007	72950	32	BM, 14%; PBC, 74%; BM+PBSC, 12%	Tandem auto auto vs. single auto	(n=100) BEP, 66%; EP, 14%; PVB, 5%; VAB, 0%; Other, 5%; no chemotherapy, 10% - (n=102) surgery, 89% (n=102) 1-5 cycles of chemotherapy, 32%; 6-10 cycles, 56%; >= 11 cycles, 7%; no chemotherapy, 1%	3 drugs, 53%; 2 drugs, 45%; 1 drug, 2%		single auto: BM, 30%; PBSC, 61%; BM+PBSC, 9%	Prior treatment: (n=196) BEP, 60%; EP, 15%; PVB, 9%; VAB, 1%; Other, 7%; no chemotherapy, 8% - surgery, 87% (n=197) 1-5 cycles of chemotherapy, 23%; 6-10 cycles, 62%; >= 11 cycles, 12%; no chemotherapy, 1%
De Giorgi, UK, 2005	77240	18	PB; BM	single HSCT	standard-dose chemotherapy	CarboPEC; CE; TE; CarboPETM; Other			
Einhorn, USA, 2007	77230	17	PB	tandem HSCT	standard-dose chemotherapy	2 cycles of carboplatin plus etoposide			

Appendix Table C56: Outcome Assessment Treatment Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration
Lazarus, USA, 2007	72950	20	OS; EFS	TRM; other	No	1-yr; 3-yr; 5-yr
Einhorn, USA, 2007	77230	17	OS; DFS			4 years

Appendix Table C57: Outcome Assessment Comparator Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration
Agarwal, USA, 2009	72940	4	EFS; OS	TRM; 2nd malignancies; other effects		3-yr
Lazarus, USA, 2007	72950	12	OS; PFS	TRM; other effects	No	1-yr; 3-yr; 5-yr
De Giorgi, UK, 2005	77240	18	OS; EFS	TRM; other		1-3-5 yr

Appendix Table C58: Time to Event Outcomes Treatment Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	1 yr	2 yr	3 yr	4 yr	5 yr	Outcome_2	3 yr_2	5 yr_2
Lazarus, USA, 2007	72950	20	OS	67 (34-86)		42 (15-67)		36 (10-59)	EFS	49 (27-72)	
Einhorn, USA, 2007	77230	17	OS	76.5 (59-99.5)		63 (43-92)		63 (43-92)	EFS		52 (11)

Appendix Table C59: Time to Event Outcomes Comparator Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	1 yr	3 yr	5 yr	Outcome_2	1 yr_2	3 yr_2	5 yr_2	Test_2
Agarwal, USA, 2009	72940	4 (0-19 yrs)	OS		50 (7-93)		EFS		50 (7-93)		log-rank
Lazarus, USA, 2007	72950	12	interval between transplant and death from any cause	65 (40-82)	49 (24-68)	49 (24-68)	PFS, survival without recurrence or cancer progression, as measured by exam, radiographs, and/or an increase in serum cancer markers (n=195)	60 (36-78)	49 (26-69)	49 (26-69)	
De Giorgi, UK, 2005	77240	18	OS	67 (45-88)	56 (33-78.5)	49 (25-72)	DFS	50 (26-74.5)	50 (26-74.5)	50 (26-74.5)	

Appendix Table C60: Adverse Events Treatment Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM
Burdach, Germany and Austria, 2000	14310	28				
Burdach, Germany, 2003	10030	reported engr, TRM, infec compl, sec malig, and major organ tox, but not by age of < or > 17 yrs				
Burke, USA 2007	4060	7	sepsis n=1		0	0
Costa, USA, 2008	1710	1			0	0
Drabko, Poland 2005	6680	21				5% (n=1 day 35 from multiorgan failure secondary to infection)
Hara , japan 1998	17950	3				0 NR
Harimaya, Japan, 2003	9850	2			0	0
Kasper, Germany, 2006	2570	5			0	0
Koscielniak Germany 2005	7860				0	0
Kushner, USA, 2001	14240	1 HSCT pt died at 17 mos after HSCT with NED but pulmonary failure				
lucas, usa 2008	2450	1			0	0
Lucidarme, france, 1998	17610	3				0 (NR)
Meyers, USA, 2001	13670		sepsis leading to death	4% n=1 patient from HSCT group (incl in TRM)		of HSCT group n=23 n=3 13%
Navid, US and Canada, 2006	5930	9			0	0
Numata, Japan, 2002	12130				0	0
ozkaynak, usa 1998	18540	15			0 (one patient not assessable secondary to early toxic death)	n=2 ATN day 0 and septic shock day 8
pession, italy, 1999	16120	3				0 NR
Prete, italy 1998	17210	17				0
Tanaka, Japan, 2002	11770			0		0

Appendix Table C60: Adverse Events Treatment Germ Cell Tumor Continued

Study (Investigator, country, record#)	Record Number	Severity of Grade SM	F/U (mos) SM	% SM	Comments SM	Group (N)_7	% Hepatic veno-occulusive disease (Hepatic Sinusoidal Obstruction)	Comments hVOD	Severity of Grade SHE	% SHE
Costa, USA, 2008	1710	AML at 53 months post HSCT								
Drabko, Poland 2005	6680						10%	moderate to severe		
Meyers, USA, 2001	13670								HSCT pt died from hemorrhagic pericarditis (included in TRM)	4%
Numata, Japan, 2002	12130	CML chronic phase	50 months after HSCT							
Tanaka, Japan, 2002	11770	CML		14 %	not clear if the 35 y/o pt or one of the 6 abstracted pts	1 of 7				

Appendix Table C61: Adverse Events Comparator Germ Cell Tumor

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	Severity of Grade Secondary Malignancy	F/U (mos) SM	% SM
bernstein, USA/Canada 2006	6290		death	5 of 110 (4.5%)	MDS	at 20 mos after dx	1/110 1%
Bhatia,USA, 2007	43210						cumulative incidence of t-MDS/AML of 11% at 5 yrs from dx
Kushner, USA, 1995	21430	24			leukemia dead at 10.5 mos after HSCT in CR from ESFT		4
Meyers, USA, 2001	13670	9 nonHSCT		11% sepsis during induction CT			
Milano, Italy, 2006	43290						
Sari, Turkey, 2010	42790	36					0%
van winkle, USA, 2005	43550						

Appendix Table C70: Design, Participant Selection and Enrollment Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)
Chi, USA, 2004	7900	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	21	1997-2003	Case series	21	0
Dhall, USA/Australia/Argentina, 2008	52130	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	21	1991-2002	Case series	21	0
Fangusaro, USA, 2008	3420	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	43	1991-2002	Case series	43	0
Gardner, USA/Australia, 2008	71930	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	13	1992-2002	Case series	13	0
Geyer, USA, 2005	73920	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	299	1993-1997	RCT	284	15
Gidwani, USA, 2008	71940	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	1		Case report	1	0
Packer, USA, 2006	77250	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	421	1996-2000	RCT	379	42
Perez-Martinez, Spain, 2005	70470	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	13	1995-2002	Case series	13	0
Sung, Korea, 2007	4770	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	14 (11 tandem; 3 single)	1999-2005	Case series	14	
Taylor, UK, 2005	52760	Malignant non-hematopoetic	CNS Embryonal Tumors	Initial therapy	68	1992-2000	Case series	68	0

Appendix Table C71: Participant Characteristics Treatment Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)
Chi, USA, 2004	7900	21		38 months	7-119	76% M	M1 (19); M2 (9.5); M3 (71)	MB
Dhall, USA/Australia/Argentina, 2008	52130	21		21 months	5-35 months	50% M	M0	MB
Fangusaro, USA, 2008	3420	43		37 months	0-120 months	51% M	M0 (82); M1-M3 (18)	PNET
Gardner, USA/Australia, 2008	71930	13		35 months	4-52 months	54% M	M0 (77); M1 (8); M3 (15)	AT/RT
Gidwani, USA, 2008	71940	1	(4 months)			100% M	M0	AT/RT
Perez-Martinez, Spain, 2005	70470	13		3 months	1-14 months	61.5% M	M1-M4 (NR)	MB (69); PNET (31)
Sung, Korea, 2007	4770	14		51.5 months	17-198 months	50% M	M0 (64); M1 (7); M3 (29)	MB (79); PNET (21)

Appendix Table C72: Participant Characteristics Comparator Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Age (median)	Age (Range)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)
Geyer, USA, 2005	73920	284		0-36 months	57% M	M0 (75); M1+ (25)	MB (32); PNET (16); AT/RT (10); Other (41)
Packer, USA, 2006	77250	379		36-252 months	59% M	M0	MB
Taylor, UK, 2005	52760	68	94 months	34-197 months	29% M	M2 (19); M3 (81)	MB

Appendix Table C73: Treatment Characteristics Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Supportive Care
Chi, USA, 2004	7900	21	PB	single	surgery; chemotherapy	carboplatin; thiotepa; etoposide	IV antibiotics; anifungal agents
Dhall, USA/Australia/Argentina, 2008	52130	21	BM; PB	single	surgery; chemotherapy	carboplatin; thiotepa; etoposide	
Fangusaro, USA, 2008	3420	43	BM; PB	single	surgery; chemotherapy	carboplatin; thiotepa; etoposide	radiotherapy for greater/= 6 years of age (37%)
Gardner, USA/Australia, 2008	71930	13		single	surgery; chemotherapy	carboplatin; thiotepa; etoposide	31% radiation
Gidwani, USA, 2008	71940	1	PB	tandem	surgery; chemotherapy	carboplatin-thiotepa-etoposide; busulfan-melphaln-thiotepa	
Perez-Martinez, Spain, 2005	70470	13	PB	single	surgery; chemotherapy; radiation	busulfan-melphalan; busulphan-thiotepa	clonazepam; antibiotics; nutritional support
Sung, Korea, 2007	4770	14	PB (92%); BM (8%)	Tandem (79%); Single (21%)	surgery, radiotherapy and/or chemotherapy	cyclophosphamide; melphalan; carboplatin-tiotepa-etoposide for 2nd transplant	43% post-radiotherapy prior to HSCT

Appendix Table C74: Outcome Assessment Treatment Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Chi, USA, 2004	7900	21	OS; EFS	TRM	40-48 months
Dhall, USA/Australia/Argentina, 2008	52130	21	OS; EFS	QOL; TRM	
Fangusaro, USA, 2008	3420	43	OS; EFS	TRM; SM; Other	5 years
Gardner, USA/Australia, 2008	71930	13	OS; EFS	TRM; Other	54 months
Gidwani, USA, 2008	71940	1	OS; DFS	SM; Other	2 years
Perez-Martinez, Spain, 2005	70470	13	EFS	TRM; SM; Other	34 months (5-93)
Sung, Korea, 2007	4770	14	OS; EFS	TRM; SM; Other	up to 5 years

Appendix Table C75: Outcome Assessment Comparator Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Geyer, USA, 2005	73920	284	OS; EFS	TRM; SM; Other	6.6 years
Packer, USA, 2006	77250	379	OS; EFS	TRM; SM; Other	5 years
Taylor, UK, 2005	52760	68	OS; EFS	TRM; Other	7.2 years

Appendix Table C76: Time to Event Outcomes Treatment Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	2 yr	3 yr	4 yr	5 yr	Outcome_2	2 yr_2	3 yr_2	5 yr_2
Chi, USA, 2004	7900	21	OS		60 (36-84)			EFS		49 (27-72)	
Dhall, USA/Australia/Argentina, 2008	52130	21	OS				70 (10)	EFS			52 (11)
Fangusaro, USA, 2008	3420	43	OS			49 (33-62)		EFS			39 (24-53)
Gardner, USA/Australia, 2008	71930	13	OS		23 (11)			EFS		23 (11)	
Gidwani, USA, 2008	71940	1	OS	Alive				DFS	Disease-free		
Perez-Martinez, Spain, 2005	70470	13	OS					EFS	57 (15)		
Sung, Korea, 2007	4770	11	OS	82 (59-100)			82 (59-100)	EFS	73 (46-99)	73 (46-99)	58 (25-91)

Appendix Table C77: Time to Event Outcomes Comparator Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_1	2 yr	3 yr	5 yr	Outcome_2	2 yr_2	3 yr_2	5 yr_2
Geyer, USA, 2005	73920	284	OS			43 (3)	EFS			27 (3)
Packer, USA, 2006	77250	379	OS			86 (9)	EFS			81 (2)
Sung, Korea, 2007	4770	3	OS	67 (13-100)			EFS	67 (13-100)		
Taylor, UK, 2005	52760	68	OS		50 (38-62)	44 (32-56)	EFS		40 (28-51)	35 (23-46)

Appendix Table C78: Quality of Life Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Scale	Domain	F/U	Group	n	mn+/-sd
Dhall, USA/Australia/Argentina, 2008	52130	21	Parent Form of the Child Health Questionnaire	mean intellectual function and QOL	70 months; 124 months	single	4	within average range

Appendix Table C79: Adverse Events Treatment Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Severity of Grade SM	F/U (mos) SM	% SM	Comments SM
Burdach, Germany and Austria, 2000	14310	28								
Burdach, Germany, 2003	10030	reported engr, TRM, infec compl, sec malig, and major organ tox, but not by age of < or > 17 yrs								
Burke, USA 2007	4060	7	sepsis n=1		0	0				
Costa, USA, 2008	1710	1			0	0	AML at 53 months post HSCT			
Drabko, Poland 2005	6680	21				5% (n=1 day 35 from multiorgan failure secondary to infection)				
Hara , japan 1998	17950	3				0 NR				
Harimaya, Japan, 2003	9850	2			0	0				
Kasper, Germany, 2006	2570	5			0	0				
Koscielniak Germany 2005	7860				0	0				
Kushner, USA, 2001	14240	1 HSCT pt died at 17 mos after HSCT with NED but pulmonary failure								
lucas, usa 2008	2450	1			0	0				
Lucidarme, france, 1998	17610	3				0 (NR)				

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	% Engraftment Failure	% TRM	Severity of Grade SM	F/U (mos) SM	% SM	Comments SM
Meyers, USA, 2001	13670		sepsis leading to death	4% n=1 patient from HSCT group (incl in TRM)		of HSCT group n=23 n=3 13%				
Navid, US and Canada, 2006	5930	9			0	0				
Numata, Japan, 2002	12130				0	0	CML chronic phase	50 months after HSCT		
ozkaynak, usa 1998	18540	15			0 (one patient not assessable secondary to early toxic death)	n=2 ATN day 0 and septic shock day 8				
pession, italy, 1999	16120	3				0 NR				
Prete, italy 1998	17210	17				0				
Tanaka, Japan, 2002	11770			0		0	CML		14 %	not clear if the 35 y/o pt or one of the 6 abstracted pts

Appendix Table C79: Adverse Events Treatment Embryonal Tumors Continued:

Study (Investigator, country, record#)	Record Number	% Hepatic veno-occulusive disease (Hepatic Sinusoidal Obstruction)	Comments hVOD	Severity of Grade Serious Hemorrhagic Event	% SHE
Drabko, Poland 2005	6680	10%	moderate to severe		
Meyers, USA, 2001	13670			HSCT pt died from hemorrhagic pericarditis (included in TRM)	4%

Appendix Table C80: Adverse Events Comparator Embryonal Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	Severity of Grade econdary Malignancies	F/U (mos) SM	% SM
bernstein, USA/Canada 2006	6290		death	5 of 110 (4.5%)	MDS	at 20 mos after dx	1/110 1%
Bhatia,USA, 2007	43210						cumulative incidence of t-MDS/AML of 11% at 5 yrs from dx
Kushner, USA, 1995	21430	24			leukemia dead at 10.5 mos after HSCT in CR from ESFT		4
Meyers, USA, 2001	13670	9 nonHSCT		11% sepsis during induction CT			
Milano, Italy, 2006	43290						
Sari, Turkey, 2010	42790	36					0%
van winkle, USA, 2005	43550						

Appendix Table C81: Design, Participant Selection and Enrollment Glial Tumors

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Ayan, Turkey, 1995	74690	Malignant Non-hematopoetic	Glial	Newly diagnosed, high-risk	Anaplastic ependymoma 4	January 1990 - May 1991	Case series	4	1 patient lost to follow up at 9 months, had no response to treatment	
Berger, France, 1998	75380	Malignant non-hematopoietic	glial	Newly Diagnosed	HSCT Choroid plexus tumor (2) Conventional Therapy Choroid plexus tumor (20)	1984-1995	Case series	22	0	
Bertolone, United States, 2003	10380	Malignant Non-Hematopoetic	Glial	Patients with no previous CHM or RT who had been histopathologically confirmed to have a high-grade astrocytoma after surgical resection	18	April 1985 - May 1990	Randomized trial with non randomized infant component	18	0	4 patients were excluded due to cnsensus pathology diagnosis, 1 juvenile pilocytic astrocytoma 2 low-grade astrocytoma and 1 meduloblastoma

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Bouffet, France, 1997	78760	Malignant Non-Hematopoetic	Glial	Recurrent	5	NR	Case Series	5	0	13 children with high grade glioma were enrolled in this study. 8 were newly diagnosed and exclude while 5 were recurrent after induction chemotherapy
Bouffet, France, 2000	78770	Malignant Non-hematopoietic	Glial	Newly diagnosed pontine glioma	36	March 1990-?	Case series	24	12	
Busca, Italy, 1997	73190	Malignant Non-hematopoetic	Glial	Malignant recurrent or progressive CNS tumor	Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1	May 1991 - August 1996	Case series	6	0	Preliminary results of the present study indicate that children with both recurrent and newly diagnosed brain tumors may benefit from high-dose chemotherapy
Conter, France, 2009	73540	Malignant Non-hematopoetic	Glial	Varied	Ependymoma 24	November 1996 - December 2002	Retrospective case series	24	0	
Doireau, France, 1998	55990	Malignant Non-Hematopoetic	Glial	Recurrent or unresecAppendix Table 8tumors	8	May 1992 - January 1998	Case series	8	1 dead of disease	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Dunkel, United States, 1998	78780	Malignant Non-Hematopoetic	Glial	Recurrent	10	NR	Case Series	10	0	16 patients were enrolled in this study, 6 were excluded based on newl diagnosed pontine tumors with no previous therapy
Finlay, United States	1300	Malignant Non-Hematopoetic	Glial	Recurrent Malignant Astrocytoma	27	NR	Quasi-Experimental Study w/ prosective cohort compared to CCG-945 controls	27, 56 control	0	
Grill, France, 1996	73240	Malignant Non-Hematopoietic	Glial	Recurrent	Ependymoma 16	1988 - 1994	Case Series	16	0	authors suggest the high-dose busulfan-thiotepa combination had little if any activity in refracotry or relapsed ependymoma of children. New therapeutic approaches must be evaluated

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Grill, France, 2001	74360	Malignant non-hematopoetic	Glial	Newly Diagnosed, high grade ependymoma	73	June 1990 - December 1998	Case Series	73	0	
Grovas, United States, 1999	16600	Malignant Non-hematopoietic	Glial	Newly Diagnosed High-Risk	11	1993-1995	Case series	11	0	
Grundy, United Kingdom, 2007	73750	Malignant non-hematopoetic	Glial	Newly Diagnosed, 9 pts metastatic	Ependymoma 89	1992 - 2003	case-series	89	0	
Grundy, United States, 2010	51800	Malignant Non-Hematopoetic	Glial	No prior adjuvant drug or radiotherapy	45	March 1993 - July 2003	Case series	41	4	
Gururangan, United States, 1998	18000	Malignant non-hemtopoetic	Glial	recurrent	N=7, 1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma, 1 CPC	1989-1996	Cohort	n=7	0	
Horn, Untied States, 1999	74470	Malignant non-hematopoetic	Glial	Varied	Ependymoma 83	1987-1991	Retrospective case series	83	0	11 center retrospective
Hurwitz, United States, 2001	53330	Malignant Non-Hematopoetic	Glial	Recurrent or Progressive brain tumors	45	June 1993 - March 1998	Case Series	45	0	75 enrolled 45 eligible based on histology

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Jaing, Taiwan, 2004	74030	Malignant Non-hematopoeitic	Glial	Newly diagnosed high and low grade ependymomas	Ependymoma 46			43	3 excluded due to one death in immediate postoperative period and 2 spinal cord tumors. 2 patients were also lost to follow up	
Jakacki, United States, 1999	15920	Malignant non-hematopoietic	glial	High-dose chemotherapy	11	April 1997 - June 1998	Case series	11	0	Study enrollment stopped early due to concerns about radio-pootentiating effects of chemotherapy given concurrently with radiation 1 pt excluded due to age above 21 years
Kobrinsky, United States, 1999	53560	Malignant Non-Hematopoetic	Glial	Recurrent or unresponsive	42	December 1988 - February 1992	Case Series	42	0	99 patients enrolled, 42 eligible based on histology glioma
Korones, United States, 2006	52670	Malignant Non-Hematopoetic	Glial	Recurrent	9	June 2002 - October 2003	Retrospective case series	9	0	2 patients excluded due to being above age 21

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Kuhl, Germany, 1998	17700	Malignant Non-hematopoeitic	Glial	Untreated, newly diagnosed ependymoma	21	1987 - 1991	Phase II trial	10	11	
Macdonald, United States, 2005	55000	Malignant Non-Hematopoetic	Glial	Newly Diagnosed High-Grade	102	1993-1998	Randomized Trial	76	11 pts did not complete HDCT due to toxicities	26 patients excluded after central neuroradiographic review or pathological review
Mahoney, United States, 1996	73250	Malignant Non-hematopoeitic	Glial	Recurrent or Progressive	7	December 1990 - September 1993	case series	7	0	7 of 19 patients included based on tumor diagnosis
Mason, United States, 1998	73180	Malignant Non-hematopoetic	Glial	Recurrent	Ependymoma 15	December 1986 - November 1993	PII trial	15	0	Given the dismal performance of this regimeni n controlling recurrent intracranial ependymoma in children we cannot recommend this approach of infasive chemotherapy with this regimen as an effective strategy for recurrent disease.

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Massimino, Italy, 2005	55220	Malignant Non-Hematopoetic	Glial	Consolidate high-risk	21	August 1996-March 2003	Case series	21	0	*Was in comparator search
Merchant, United States, 2002	74280	Malignant non-hematopoeitic	Glial	Varied	Ependymoma 64	June 1997 -	PII trial	64	0	
Ozkaynak, United States, 2004	7850	Malignant Non-Hematopoietic	Glial	Relapsed/Progressive : Tandem Treatment	6	1995-2002	Case Series	6	0	Tandem Treatment, not on initial indications for abstraction
Robertson, United States, 1998	74630	Malignant non-hematopoetic	Glial	High-risk	ependymoma 20, anaplastic ependymoma 12	May 1986 - June 1992	RCT	32	0	High degree of discordance between local instiutional diagnoses and centralized review (31% concordance)
Shih, United States, 2008	2530	Malignant Non-Hematopoetic	Glial	Recurrent	5	1989-2004	Retrospective case series	5	0	Of 27 initial patients 5 met inclusion criteria for this abstraction (19)
Sio, Italy, 2006	6950	Malignant Non-Hematopoetic	Glial	Relapsed	14	April 1998 - April 2004	Case series (off-label compassionate use)	14		52 total patients, 38 excluded based on histology or age > 21
Thorarinsdottir, United States, 2007	73050	Malignant Non-hematopoetic	Glial	Malignant CNS	6	1998 - 2005	Case series	6	0	6 of 15 patients included based on tumor type

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Wrede, Germany, 2009	75590	Malignant Non-hematopoetic	Glial	Newly Diagnosed	34 CPC	2000-2008	Case series	29 CPC	5	
Yule, United Kingdom, 1997	18960	Malignant Non-hematopoetic	Glial	High-Risk and Recurrent Tandem (?)	5	1993-1995	Case Series	5	0	8 patients excluded based on tumor histology
Zacharoulis, United States, 2007	73020	Malignant Non-hematopoetic	Glial	No previous treatment, confirmed ependymoma	29	1991-1997 (Head Start 1), 1997 - 2002 (Head Start 2)	Cohort study	29	0	

Appendix Table C82: Participant Characteristics Treatment Glial Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Berger, France, 1998	75380	Choroid Plexus Carcinoma (2)			24 and 33 months			0, 2 (0, 100)	One patient had spinal metastases at diagnosis	Both CPC located supratentorially (100)	
Bouffet, France, 1997	78760	5	7	6	3-14		nr	3,2 (60, 40)	All high-grade	1 parieto-occipital, 3 brain stem, 1 thalamus	
Bouffet, France, 2000	78770	24		7 years	3-17 years		NR	15, 21	Diffuse pontine tumor	At least 2/3rd of pts tumor had to be in the pons	these patient characteristics were reported for the whole patient population and not those evaluated by HDC
Busca, Italy, 1997	73190	Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1		11 years for total group of 11 patients,	3-16 years for total group of 11 patients			5, 6 (46, 54%) for total group		Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1	
Dunkel, United States, 1998	78780	10	7.89	7.9	3.5-14.9		nr	7, 3 (70, 30)	10 High-grade glial malignancies	Pons	
Finlay, United States	1300	27	NR	8.5	.2-20.0	NR	NR	15,12 (55,45%)	NR	Glioblastoma Multiform 17 (63%) Aplastic Astrocytoma 10 (37%)	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Grill, France, 1996	73240	Ependymoma 16		3 years	.5 to 15 years			8, 8 (50, 50)	2 patients had tumor cells in CSF. 3 WHO low-grade tumors, 13 WHO high-grade tumors	6 Supratentorial, 10 Infratentorial	
Grovas, United States, 1999	16600	11		12years	5-18years		ne	7,4 (63)		11 Glioblastoma multiform (100)	one patient's GBM arose from pilocytic xanthoastrocytoma
Gururangan, United States, 1998	18000	N=7, 1 cpc, 1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma			Ependymoma 18mo, Anaplastic Astrocytoma 23mo, Glioblastoma multiforme .24, 3.6, 10.8, and 57.6mo		no	Ependymoma 0,1 (0, 100), anaplstic astrocytoma 0, 1 (0,100), Gliblastoma multiforme 2,2 (50, 50)	All patients recurrent	1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma	
Jakacki, United States, 1999	15920	11	7.2 years	7.2 years	3.1-12.6 years		NR	4, 7 (36, 64)	High grade glial tumor or a diffuse pontine tumor	3 GBM (27), 2 AA (18), 6 Pons (55)	
Mahoney, United States, 1996	73250	Anaplastic Astrocytoma 2, Ependymoma 3, Glioblastoma multiforme 1, Brainstem glioma 1		Anaplastic Astrocytoma 12, Ependymoma 5, Glioblastoma multiforme 15.5, Brainstem Glioma 5	AA (8-16), EP (3-7.5), GBM 15.5, BSG 5			AA 2,0 (100, 0) EP 1,2 (33, 67), GBM 1,0 (100, 0), BSG 0,1 (0, 100)		Anaplastic Astrocytoma 2 (29), Ependymoma 3 (43), Glioblastoma multiforme 1 (14), Brainstem glioma 1 (14)	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Mason, United States, 1998	73180	Ependymoma 15	45 months	22 months	5 months - 12 years			8, 7 (53, 47)	9 low-grade ependymoma (60%), 6 anaplastic (40%)	13 posterior fossa (87%), 2 supratentorial (13%)	
Massimino, Italy, 2005	55220	21	NR	10	3.5-19	NR	NR	7, 14 (33, 67%)	NR, All High-Grade	GBM 10 (48), Anaplastic AST 9 (42), Anaplastic oligodendroglioma 2 (10) spine 2 (10), Posterior fossa 2 (10), Supratentorial 17 (80)	
Ozkaynak, United States, 2004	7850	6	11.5	11	4.5-18		nr	3,3 (50, 50)	Progressive 3 (50), Recurrent 3 (50)	AA 2 (33), GBM 1 (17), BSG 2 (33), Ependymoma 1 (17)	
Shih, United States, 2008	2530	5	7.8 yrs	7.4 yrs	.4-16.6 yrs		NR	nr	NR	1 EPD, 2 AA, 2 GBM	
Thorarinsdottir, United States, 2007	73050	Oligodendrogliomma 1, Ganglioma 1, Anaplastic glioma 3, Ependymoma 1		Oligodendrogliomma 27 months, Ganglioma 25 months, Anaplastic glioma 18 months, Ependymoma 6 months	Oligodendrogliomma 27 months, Ganglioma 25 months, Anaplastic glioma (9-29) months, Ependymoma 6 months			Oligodendrogliomma 1 male, Ganglioma 1 male, Anaplastic glioma 2 male, 1 female (67, 33), Ependymoma 1 female	All WHO grade III	Oligodendrogliomma right frontal, Ganglioma temporal, Anaplastic glioma 1 c-spine 1 brainstem and one parietal, Ependymoma IV ventricle	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Yule, United Kingdom, 1997	18960	5		11.5	10.25	5-14	NR	nr		1 analpastic ependymoma (25%), 1 recurrent GBM (25), 1 GBM (25), 1 suprasellar gbm (25)	
Zacharoulis, US, 2007	73020	Ependymoma 29	2.3 years	2.1 years	.7-8.9 years		NR	18, 11 (62, 38)	M0 24 (83), M1 1 (3), M2 0, M3 4 (14)	Posterior fossa 22 (76), supratentorial 7 (24)	

Appendix Table C83: Participant Characteristics Comparator Glial Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Ayan, Turkey, 1995	74690	Anaplastic ependymoma 4		12.5 years	5-15 years			3, 1 (75, 25%)	Anaplastic 4 (100%)	frontal lobe 1 (25), teporoparieto-occipital lobe 1 (25%), Multiple paranchimal menengial lesions 1 (25%), Temporoparietal lobe 1 (25%). CSF cytology positive in one patient (25%)	
Berger, France, 1998	75380	Choroid plexus carincoma (22)		31 mo	4-111 mo			9, 11 (35, 55)	3 patients had metastases at diagnosis (2 spinal/bifocal, 1 bifocal) 4 patients had no metastases and 13 patients had unknown metastates	12 supratentorial (60), 8 infratentorial (40)	
Bertolone, United States, 2003	10380	18		4	<1 year - 16 years		NR	11, 8 (58, 42)		11 Anaplastic Astrocytoma (61), 3 Ependymoma (17), 2 Glioblastoma multiforme (11), 1 Anaplastic mixed glioma (6), 1 anaplastic ganglioglioma (6)	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Conter, France, 2009	73540	Ependymoma 24		8.6 years	5-17 years			16, 8 (67, 33)	Grade 2 13 (57), Grade 3 10 (43)	Supratentorial 4 (17), Infratentorial 20 (83)	
Doireau, France, 1998	55990	8	4.6	3.8	3 mo - 4.5			nr	Six patients had low-grade tumors while two had grade III tumors. All tumors were progressive and three had metastases before chemotherapy.	5 astrocytoma (63), 3 oligoastrocytoma (37)	Ages at diagnosis
Finlay, United States	1300	56	nr	11.1	.1-19.3	nr	nr	29,27 (52,48%)	NR	Glioblastoma Multiform 27 (48%) Aplastic Astrocytoma 29 (52%)	
Grill, France, 2001	74360	Ependymoma 73		27 months	5-62 months			40, 33 (55, 45)	73 Ependymoma 100%	56 (82%) of patients had a high grade tumor, 12 (18%) had a low-grade tumor	5 patients were not assigned a histological grade
Grundy, United Kingdom, 2007	73750	Metastatic ependymoma 9, non-metastatic ependymoma 80		1.93, 1.36	(.05-3.16), (.24-2.25)			54 (67.5 % male), 4 (44 % male)	Non-metastatic 80 (90), Metastatic 9 (10)	Infratentorial 69 (86), Supratentorial 11 (14) Infratentorial 7 (78), Supratentorial 2 (22) WHO II 54 (68), WHO III 26 (32) WHO II 5 (56), WHOIII (44)	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Grundy, United States, 2010	51800	41		High Grade Glioma 1.8 years, Brain Stem Glioma 2.52 years	High Grade Glioma .33-3.09 years, Brain Stem Glioma .68-3.01 years			18, 8 (69)	HGG: Anaplastic Astrocytoma 7, Astroblastoma 1, Anaplastic oliodendrogliom a 2, Glioblastoma 5, unknown 3 Diffuse pontine glioma: diffuse astrocytoma 1, glioblastoma 1, unclassified 1, inoperable 4	High Grade Glioma 19 (73), Brain Stem Tumor 7 (27) HGG metastatic in posterior fossa 2 (11), metastatic in supratentorial 17 (89) Brain Stem Glioma metastatic in Brainstem 7 (100), 15 cpc	
Horn, Untied States, 1999	74470	Ependymom a 83		51.5 mo	8mo - 20 years			50, 33 (60, 40)	M0 61 (85), M1-M3 11 (15)	WHO II grade 2 51 (61), WHO II grade 3 31 (37) Infratentorial 64 (77), Supratentorial 19 (23)	Age ≤ 3 29 *=(35), Age >3 54 (65)
Hurwitz, United States, 2001	53330	45		7.7	4mos-19yr		NR	56, 44%	Recurrent or progressive disease	Astrocytoma 4 (9), Malignant Glioma 13 (29), Brain Stem Glioma 15(33), Ependymoma 13 (29)	Age and Gender reported for entire 75 enrolled pts, not available by histology
Jaing, Taiwan, 2004	74030	Ependymom a 43		6.6 years	8 months to 18 years			25, 18 (58, 42)	Grade II 20 (47), Grade III [anaplastic] (53)	Supratentorial 15 (35), Infratentorial (65)	
Kobrinsky, United States, 1999	53560	42	NR	NR	NR	NR	White 63, Black 12, Hispanic 19, Asian 3, Other/Mixe d 3	Male 54, Female 45	NR	High grade astrocytoma 20 (48), Brain stem glioma 22 (52)	Race and sex statistics reported for the sum total 99 patients

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Korones, United States, 2006	52670	9	12.2	9	5-21		NR	7,2 (77,23%)		5 Glioblastoma, 2 anaplastic astrocytoma 2 brainstem glioma	
Kuhl, Germany, 1998	17700	21			3-16				19 anaplastic (90), 14 infratentorial (67). 29% of patients had microscopic tumor cells in CSF	21 ependymoma (100%)	
Macdonald, United States, 2005	55000	76		11.95yrs	3-20yrs		69.7% white, 14.5% Hispanics, 10.5% Blacks, and 5.3% other	36, 40 (47. 53%)	All patients had histologic verification of high-grade astrocytoma	GMB/GV 40 (53), AA 30 (39), Other 6 (8)supratentorial tumor 86.8%, five patients had metastatic disase	4 patients not evaluable because imaging reports demonstrating residual disease were not available before chemotherapy
Merchant, United States, 2002	74280	Ependymoma 64		3 years	1.1 - 22.9 years			32, 32 (50, 50%)	45 differentiated ependymoma (70), 19 anaplastic ependymoma (30)		
Robertson, United States, 1998	74630	32		7	2-17.3		Caucasian 22 (69), African American 3 (9), Hispanic 4 (13), Other 3 (9)	17, 15 (53, 47)	Anaplastic 12 (38)	posterior fossa 21 (66), supratentorial 11 (34)	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Sio, Italy, 2006	6950	14	9.6	8.4	4.2-19.6		nr	9, 5 (64, 36)		Ependymoma 2 (14), Anaplastic Astrocytoma 3 (21), Brain Stem Glioma 8 (57), Glioblastoma Multiforme 1 (7)	
Wrede, Germany, 2009	75590	34 CPC		2.3 years	.3-17.1 years			17, 17 (50, 50%)	Metastatic 7 (21%)	Lateral Ventricle 30 (88%), Fourth ventricle 4 (12%)	

Appendix Table C84: Treatment Characteristics Glial Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Ayan, Turkey, 1995	74690	Anaplastic Ependymoma 4							"8 in 1" chemotherapy	methylprednisolone, vincristine, lomustine, procarbazine, hydroxyurea, cisplatin, cytosine arabinoside, cyclophosphamide in a targeted 8 courses or until disease progression	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Berger, France, 1998	75380	HSCT CPC (2) Conventional therapy CPC (20)	Peripheral blood	Autologous	Surgical resection	1 HSCT patient received: carboplatine, procarbazine, etoposide, cisplatine, vincristine, cyclophosphamide. 1 patient received etoposide, ifosfamide and carboplatine			Conventional chemotherapy was given to 17 of 20 remaining patients. 2 of the patients who did not receive chemotherapy had radiotherapy, and two had no treatment other than partial surgical resection. Chemotherapy regimen varied by patient.	10 patients had: carboplatine, procarbazine, etoposide, cisplatine, vincristine, cyclophosphamide. 3 patients had etoposide, carboplatine. 1 patient had carboplatin and ifosfamide; 1 patient received monthly lomustine	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Bertolone, United States, 2003	10380	18			Surgical Excision				Standard Chemotherapy Regimen (A) vs. Experimental 8-in-1 Chemotherapy Regimen (B)	(A) 10 week induction with 8 weekly injections of vincristine, 48 week maintenance with 8 cycles of vincristne, CCNU, and prednisone. (B) 10 week induction of two cycles of 8-in-1 chemotherapy followed by 5400 GY radiotherapy	8-in-1 chemotherapy consisted of (Vincristine, CCNU, procarbazine, hydroxyurea, cisplatin, cytarabine, dacarbine, and methylprednisone)

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Bouffet, France, 1997	78760	5	Bone Marrow	Single Autologous	2 VM-BCNU-PCZ with radiotherapy, 2 VM-CDDP-FU-DTIC-CPM-PCZ one with radiotherapy, and 1 VM-BCNU-PCZ						
Bouffet, France, 2000	78770	24	Bone Marrow	Autologous	NR, Newly Diagnosed	RT initiated as soon as possible after post-op recovery in surgery or after radiologic diagnosis. 50-50Gy given over 6 weeks at a rate of 8-9 Gy per week in 5 daily fracs. HDC initiated 40-60 days after RT. HDC consisted of busulfan 150mg/m^2/d on -8,-7,	-6, and -5. And Thiotepa 300 mg/m^2/d -4, -3, and -2. clonazepam .1 mg/kg/day Day -8 to -1. ABM reinfused 48 hours after chemo.				Only 24 of 35 children proceeded to HDC. One child died during RT, 8 other children experienced early disease progression preventing consolidation, two families declined further treatment.

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Busca, Italy, 1997	73190	Ependymoma 2, Anaplastic Astrocytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1	ABMT	Autologous	All pts had maximal surgical resection. 3 pts (50%) had 1st line RT, 1 pt. had 1st line chemotherapy (17%). 3 pts had secondary total resection after relapse (50%), 3 pts had secondary chemotherapy and 1 pt had radiotherapy (17%).	Two regimen: A, BCNU 2x/d for 3 days and etoposide 1x/d for 3 days (n=5). B, Thiotepa and etoposide 1x/d for 3 days (n=6)		HEPA filtered room, low microbial diet, IV acyclovir, oral nonasorbable antibiotics, and contrimoxazole. Broad-spectrum antibiotics were adminsitered to febrile patients. Blood component therapy to keep elevated platelet count			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Conter, France, 2009	73540	Ependymoma 24							Surgical resection followed by radiotherapy	In a complete resction, patients were given 60 Gy HFRT in two daily frac of 1 Gy (photon energy was >8 MeV. For partial removal, second look surgery discussed before RT. If not compelte resection a 6 Gy boost was given to the initial 60Gy	No patients received chemotherapy

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Doireau, France, 1998	55990	8			ventriculo-peritoneal shunt in 1 pt, 2 pts had biopsy alone, and partial excision in 6 patients				chemotherapy	16 month/seven cycle of carboplatin (15 mg/kg), procarbazine (4 mg/kg), etoposide (5 mg/kg), cisplatin (1mg/kg) vincristine (.05 mg/kg), and cyclophosphamide (50 mg/kg)	
Dunkel, United States, 1998	78780	10	Bone Marrow	Single Auto	10 radiotherapy, 5 with chemotherapy, 1 with beta-interferon	6 Thiotepa Etoposide, 2 BCNU Thiotepa Etoposide, 2 Carboplatin Thiotepa Etoposide					

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Finlay, United States	1300	27	Bone marrow	Autologous	NR	ThioTEPA 900 mg/m^2 w/ etoposide 750 or 1,500 mg/m^2 over 3 days (n=11), 600 mg/m^2 over 3 days preceded by carmastine (n=5), or carboplatin 1,500 mg/m^2 over 3 days (n=11) w/ AEUC of 7 mg/ml/min day	NR	NR	Chemotherapy Only		
Grill, France, 1996	73240	Ependymoma 16	Bone Marrow Stem Cells in 15 pts and PBSC in 1 pt.	Autologous	NR. 8 patients received HDCT + autologous SCT as first treament of relapse, 8 patients received ASCT as second or further relapse treatment	Busulfan, Thiotepa,		Isolated laminar air flow rooms with atrial catheters. Parenteral nutirion and broad spectrum antibiotics when needed.			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Grill, France, 2001	74360	73							Resection and Chemotherapy followed by irradiation in the event of progression or relapse	Maximal surgical resection followed by three courses of two different drugs (carboplatin and procarbazine, etoposide and mannitol, and vincristine cyclophosphamide and uromitexan). Irradiation for relapse was 50 Gy 1.8 Gy/frac 5x week	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Grovas, United States, 1999	16600	11	2 PBSC (18), 9 ABMT (82)	single autologous	NR, newly diagnosed	carmustine, thiotepa, and etoposide. Carmustine at dose of 100 mg/m^2 for six doses, Thiotepa 300 mg/M^2/d * 3 , Etoposide 250 mg/m^2/d *3		Corticosteroids for control of tumor mass effect and cerebral edema. Pts not given coritcosteroids had dexamethasone 5 mg/m^2/d *3. 6 Pts given G-CSF on reinfusion (55). All pts received RT on approximately day +42. 30 fracs 180 cGy 5200 cGy w/ 540 boost			1 patient died before radiotherapy
Grundy, United Kingdom, 2007	73750	Ependymoma 89							Chemotherapy w or w/o RT	4 courses alternating myleosuppresive and non-myelosuppressive carboplatin, vincristine, methotrexate, cyclophosphamide and mesna, cisplatin. RT after progression	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Grundy, United States, 2010	51800	41			Surgical Resection: HGG 14 (74), Brain Stem Glioma 0 (0)				Chemotherapy with or without radiotherapy	Four courses with 7 cycles: course 1 vincristine (1.5mg/^2) and carboplatin (550 mg/m^2), course 2 Vincristine (1.5mg/m^2) Methotrexate (8000mg/m^2) and Folinic Acid 15 mg, course 3 Vincristine (1.5 mg/m^2) Cyclophsphamide (1500mg/m^2) and Mesna (1800 mg	Course 4 Cisplatinum continuous infusion for 4 hours (40 mg/m^2 x 2 days), children 10 kg and under were dosed to weight rather than surface area. Six patients completed Chemotherapy

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Gururangan, United States, 1998	18000	N=7,1 cpc, 1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma	Bone marrow	Autologous	Surgury and chemotherapy in all pts except the astrocytoma patient who had biopsy online	Four patients had carboplatin, thiotepa, and etoposide, one patient had thiotepa and etoposide only, and one patient had carboplatin, thiotepa and carmustine		Varied by treatment protocol. Patients received antifungal and antibiotics if febrile and neutropenic. Maintenance of platlet counts. GCSF use varied by protocol.			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Horn, Untied States, 1999	74470	Ependymoma 83							Patients in this multicenter retrospective study were classified as having either surgery alone 6 (7), chemotherapy alone 17 (20), radiation alone 31 (37), or radiation and chemotherapy 29 (35).	Chemotherapy type was broken into: None 37 (45), Nitrosourea based 13 (16), Alkylatin agent based 21 (25), Nitrosourea and alkylating 9 (11), other types 3 (4) No RT 23 (28), Local 36 (41), Local and cranial 5 (6), and craniospinal 21 (25)	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Hurwitz, United States, 2001	53330	45							Chemotherapy	Dexamethasone .25 mg/kg 14 and 7 hours before other drug administration, paclitaxel 1mg/kg 350mg/m^2 over 24 hours every 3 weeks, and diphenhydramine 1mg/kg	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Jaing, Taiwan, 2004	74030	Ependymoma 43							Surgical excision followed by 30 Gy irradiation w/ 20-25 Gy boost to the primary tumor area [spinal mets irradiated with a total dose of 30-45 Gy]. 9 pts did not receive RT due to >3 years old. 13 pts received chemotherapy	Chemotherapy protocols varied between patients [5 protocols, either platinum or nitrosourea or other combinations exclusive of nitrosourea or platinum]	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Jakacki, United States, 1999	15920	11	PBSC	Autologous given in four doses concurrent with chemotherapy and radiotherapy	NR, newly diagnosed	CCNU 130mg/m^2, vincristine 1.5mg/m^2 on day 0 and procarbazine 150 mg/m^/d on 1-7.PBSC infusion was infused 36-72 hrs after procarbazine.RT began 48-72 hrs after PBSC 180cGY (5040-5940 cGy).2nd, 3rd, and 4th chemotherapy regimens started 4 wks after prev		Pts who developed a procarbazine related rash received diphenhydramine prior to subsequent doses			1 pt with spinal cord glioblastoma had 3600 cGy craniospinal radiation therapy with boost to tumor area, all other pts had invovled field RT.4 pts w/ non-brainstem large volume tumors had <4 PBSC and Chemotherapy,due to progression recruitment was stopped

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Kobrinsky, United States, 1999	53560	High grade astrocytoma 20, brain stem glioma 22			Perviously treated with chemotherapy and/or radiation therapy				Etoposide or etoposide/ mannitol	150mg/M^2 Iv over 3h for 5 days	
Korones, United States, 2006	52670	9			3 RT alone, 2 RT and Chemo, 4 BMT and other therapy				Chemotherapy	Temozolomide and VP-16	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Kuhl, Germany, 1998	17700	21							Chemotherapy: procarbazine, ifosfamide, mesna, vp-16, methotrexate, CF-rescue, cisplatin, cytarbine followed by radiotherapy of 35.2 gy in 22 frac and maintenance chemotherapy in some patients (% unknown for EPD)		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Macdonald, United States, 2005	55000	76			Induction, four 3 week cyces in three different regiments: A) carboplatin, VP-16 B) ifosfamide, mensa VP-16 C) Cyclophosphamide, mesna, VP-16.			Corticosteroids used at clinician recommendation; recommended for raised intracranial pressure and adrenal insufficiency restriction	Chemotherapy with Radiotherapy	Interim therapy: one 12-week course Vincristine at 1.5 mg/m^2 (2 mg max) for 8 weeks w/ 6-week RT followed by 4-week rest. Maintenance cycle of eight 4-week cycles 6 weeks after RT consisting of oral CCNU 100mg/m^2 * 1 day and vincristine 1.5 mg/m^2	(Dose information not entered due to char limit - available in paper)

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Mahoney, United States, 1996	73250	7	Bone Marrow	Autologous	Radiation and Chemotherapy	CTX 4 days, Melphalan 3 days following marrow infusion patients were given escalating CTX dose with mesna support		Amino Acid withholding during melphalan treatment. Irradiated CMV for hematocrit level maintenance, Fluconazole, Acyclovir in pts. With positive HSV			
Mason, United States, 1998	73180	Ependymoma 15	ABMR	Autologous	Maximal surgical resection in all pts. 13 pts had radiotherapy (87%), 14 of the pts had prior chemotherapy (93%).	5 patients received thiotepa/etoposide (33), 10 received thioTEPA/etoposide/carboplatinum (67)		platlet counts maintained above 50,000, hemoglobin mainted above 8.0g/dL, febrile neutropenic patients treated with broad-spectrum antibiotics and antifungal agents. Pts received trimethoprim-sulfamethoxazole prophylaxis from day 30			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Massimino, Italy, 2005	55220	21	PBC	Single Auto, in 4 pts two cycles due to residual tumor response after first course	Surgical Excision	CDDP plus VP-16 week 1 and 4; VCR plus CTX and hd-MTX week 7 and 10, hd-Thiotepa and G-CSFT week 13					

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Merchant, United States, 2002	74280	Ependymoma 64							Radiotherapy with three dimensional treatment planning	Conventional fractionation of 1.8 Gy/d to 59.4 Gy. 4 young children with Ependymoma received 54.0 Gy. Dose limiting to upper cervial spinal cord was 54 Gy, optic chiasm 55.8 Gy, optic nerves 50.4 Gy, and optic globe 50.4 Gy	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Ozkaynak, United States, 2004	7850	6	PBSC	Tandem Autologous	2 Surgery XRT (5400 cGY) and Chemotherapy (CTX, CDDP, VP-16, VCR, CCNU), 3 XRT alone (dose NA), 1 surgery and chemo (CCG-9921)	Cyclophoshamide 4-6 g/m^2 with G-CSF 10 ug/kg/d, Thiotepa 240 mg/m^2/d * 3, carboplatin 400 mg/m^2/d * 3,		Rifampin, trimethoprim/sulfamethoxazole, gentamicin, amphotericin-B, fluconazole, acyclovir.			4 of these pts. GBM, Ependymoma, 1 BSG, and 1 AA had only 1 PBSC. 2 were due to parental decision and 2 were due to tumor progression after first course transplant

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Roberts on, United States, 1998	74630	Ependy moma 32							Maximal surgical resection, randomized assignment to one of two treatment arms.	Regimen A: Craniospinal radiotherapy w/ 8 weekly doses of IV vincristine concurrent with radiotherapy. Pts then received 8 6-week courses of vincristine, ccnu, and prednisode. Regimen B: 8-in-1 regimen, followed by RT, and then maintence 8-in-1	8-in-1 regimen consisted of methylprednisone, vincristine, lomustine [ccnu] or carmustine [bcnu], procarbazine, hydroxyurea, cisplatin, cytarabine, and cyclophosphamide

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Shih, United States, 2008	2530	5	Bone Marrow	Single Autologous	1 chemotherapy for EPD, 1 chemotherapy + local RT for AA, 1 craniospinal irradiation for AA, 1 Chemotherapy + craniospinal irradiation for GBM, and 1 craniospinal irradiation for GBM	1 Busulfan and Thiotepa for EPD, 2 Thiotepa and cylophosphamide for AA, 1 carboplatin and etoposide for GBM, and 1 Thiotepa and cyclophosphamide for GBM					

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Sio, Italy, 2006	6950	14			Surgery 3 (21%), Chemotherapy 6 (43%), Radiotherapy 12 (86%), Bone Marrow Transplant 2 (14%), 1 patient had no prior treatment			Authors not explicit; antibiotics, blood products were administered when required and steroid tehray was limited to treatment of raised intracranial pressure or cerebral oedema in brain tumor pts.	Chemotherapy	Temozolomide single oral dose for 5 consecutive days (214 mg/m^2/day in patients with no prior CSI and 180 mg/m^2/day in CSI or BMT) Courses were repeated every 21-28 days. TMZ reduced by 25% in patients with grade 4 toxicity.	
Thorarinsdottir, United States, 2007	73050	6	PBSC	Autologous	Newly Diagnosed	3 cycles induction cisplatin, cyclophosphamide, etoposide, vincristine. 3 cycles consolidation carboplatin, thiotepa					

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Wrede, Germany, 2009	75590	34 CPC			Newly Diagnosed, surgical resection				6 cycles chemotherapy 31 (91%), radiotherapy in children over 3 years of age		3 patients did not receive chemotherapy (9%)
Yule, United Kingdom, 1997	18960	5	BMP	Tandem Autologous	Surgery 2 (50), RT 1 (25), No Chemotherapy	2 dose CTX accomanied by mesna at 160%. Starting dose CTX was 2.5m/m^2/d and escalated at .5m/m^2/d to 2 g/m^2. stem		oral dexamethasone before CTX 10 mg/m^2/d, prophylactic acyclovir 1,500 mg/m^2/d) and ciprofloxacin (10 mg/kg/d), and oral nysatin.			
Zacharoulis, United States, 2007	73020	Ependymoma 29	PBSC	Autologous	Newly diagnosed	Maximal surgical resection followed by induction (vincristine, etoposide, cyclophosphamide w/ mesna, methotrexate) and consolidation (carboplatin, thiotepa, etoposide) chemotherapy with radiotherapy when indicated by tumor response, age, and location		platelet counters were maintatined above 10,00/mm with transfusion as necessary. Febrile neutropenic pts were given broad spectrum IV antibiotics. Pts received PCP pneumonia prophylaxis			

Appendix Table C85: Outcome Assessment Treatment Glial Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Berger, France, 1998	75380	HSCT CPC (2)	Survival, tumor response	toxicity	21 and 25 mo
Bouffet, France, 1997	78760	5	Survival		
Bouffet, France, 2000	78770	24	Survival, EFS	Toxicity	26 months
Busca, Italy, 1997	73190	Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1	OS, PFS, Tumor response	toxicity	
Dunkel, United States, 1998	78780	10	Survival		
Finlay, United States	1300	27	EFS, OS	toxicity	14 months
Grill, France, 1996	73240	Ependymoma 16	Tumor response, outcome, toxicity		1.7 - 66 months
Grovas, United States, 1999	16600	11	Tumor response, toxicity, survival		Study entry, +21, +42, +100 days and then every 2 months until 1 year after ASCR
Gururangan, United States, 1998	18000	N=6, 1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma	Progression, survival	toxicity, but not given by tumor type	NR
Jakacki, United States, 1999	15920	12	OS, PFS, Tumor response	toxicity	5-19months
Mahoney, United States, 1996	73250	7	Toxicity, Tumor Response, PFS, OS		2.6 years
Mason, United States, 1998	73180	Ependymoma 15	Survival, Progression,	Toxicity	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Massimino, Italy, 2005	55220	21	OS, PFS	TAE	Median FU 57mo (13-84)
Ozkaynak, United States, 2004	7850	6	Disease Outcome	Toxicity	
Shih, United States, 2008	2530	5	Time to Progression, OS, Final Status		73-3727 days
Thorarinsdottir, United States, 2007	73050	6	Tumor Response, PFS, OS, Toxicity		median 22 months (8-82 mo)
Yule, United Kingdom, 1997	18960	4	Tumor Response, Outcome, OS	Toxicity	median 27 months (12-34)
Zacharoulis, United States, 2007	73020	29	EFS, OS	toxicity	.6-12+ years FU range

Appendix Table C86: Outcome Assessment Comparator Glial Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Ayan, Turkey, 1995	74690	Anaplastic Ependymoma 4	Tumor Response, Progression, Survival	Toxicity	9-35 mo
Berger, France, 1998	75380	Conventional therapy CPC (20)	OS, tumor response	toxicity	1-72 mo
Bertolone, United States, 2003	10380	18	Survival	Toxicity	5 year
Conter, France, 2009	73540	OS, EFS			87.5 mo (66-90, 95% CI)
Doireau, France, 1998	55990	8	OS	Tumor response	5.5 years
Finlay, United States, 2008	1300	56	EFS, OS	toxicity	nr
Grill, France, 2001	74360	Ependymoma 73	PFS, OS		4.7 years median FU (5 mo - 8 years)
Grundy, United Kingdom, 2007	73750	Ependymoma 89	OS, PFS,	toxicity	median 6 years (1.5-11.3 years) [for pts alive at last FU]
Grundy, United States, 2010	51800	26	OS, PFS		median FU .89 years, (.19-8.04 years)
Horn, Untied States, 1999	74470	Ependymoma 83	EFS, OS		75.5 mo (9 - 121 mo)
Hurwitz, United States, 2001	53330	45	TTP, progressive disease and early death, tumor response	toxicity	
Jaing, Taiwan, 2004	74030	Ependymoma 43	OS, PFS	toxicity	5 year
Kobrinsky, United States, 1999	53560	42	OS		4 yr

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Korones, United States, 2006	52670	9	PFS, tumor response	toxicity	16 months
Kuhl, Germany, 1998	17700	10	PFS, OS, Tumor Response, Toxicity		7 years
Macdonald, United States, 2005	55000	76	OS, EFS, Tumor Response	Toxicity	Physicial and Neurologicial examination every 3 weeks during induction and interim therapy. Then, at 1 year inverals from entry or at time of progressive disease or relapse.
Merchant, United States, 2002	74280	Ependymoma 64	OS, PFS		17 months (3-44 months)
Robertson, United States, 1998	74630	Ependymoma 32	PFS, OS,		6.5 years
Sio, Italy, 2006	6950	14	PFS, OS, status at final follow up	Toxicity	Range 1-41 months based on survival
Wrede, Germany, 2009	75590	CPC	OS, EFS		0-8.2 yrs (2.2 yrs median)

Appendix Table C87: Time to Event Outcomes Treatment Glial Tumors OS

Study (Investigator, country, record#)	Record Number	Group (N)	OS	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Berger, France, 1998	75380	CPC (2)	21 and 25 mo									
Bouffet, France, 1997	78760	5	Total, (1) Parieto-occipital, (3) Brain stem, (1) Thalamus	3, .4, 4, 3								
Bouffet, France, 2000	78770	24	Group OS	10±3.6	~25	~4	0	0	0			
Busca, Italy, 1997	73190	Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1	Two patients died. One AA at 15 months and 1 oligodendrogliom a at 10 months. All other patients alive with no progression or evidence of disease									
Dunkel, United States, 1998	78780	10	range .1-18 months	4								
Finlay, United States	1300	ABMR transplated(N=27), AA (N=10) and GBM (N=17)	AA and GBM @ 4 months 22±7% months AA @ 4 months 40±14% months GBM @ 4 months 12±6%					AA: 40±14 % GBM: 12±6%		Chemo vs. ABMR unstratified and stratified (Cox)	.018, stratified by histology .010	1.9 (1.1-3.1)

Study (Investigator, country, record#)	Record Number	Group (N)	OS	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Grill, France, 1996	73240	Ependymoma 16	5 patients alive at last followup. 1 patient was in second complete response, 1 patient had relapse in the spinal cord, 1 pt had sAppendix Table 8residual mass, 1 pt was alive with hemispheric disseminated disease, and 1 patient was alive without evidence of disea	20 months (1.7-45 mo)								
Gururangan, United States, 1998	18000	N=7, 1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma	2 glioblastoma patients died of disease. One toxic death at .03 months and one dead of disease at 17 mo. Two other patients are alive with no evidence of disease at 40+ and 98+ mo. Ependymoma patient DOD at 25 mo. AA pt alive/NED at 98+, 1 CPC DOD 5mo									

Study (Investigator, country, record#)	Record Number	Group (N)	OS	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Jakacki, United States, 1999	15920	12	Total (12), GBM (4), AA (2), Pons (6) with 1 Pons patient aliev	8.5 (5-19), 15 (6-19), 8 (7-9) ,8 (5-14)								
Mahoney, United States, 1996	73250	Anaplastic Astrocytoma 2, Ependymoma 3, Glioblastoma multiforme 1, Brainstem glioma 1	AA 1 month and 4 months, EPD 7 months 9 months and 25+ months, GBM 7 months, BSG 2 months									
Mason, United States, 1998	73180	Ependymoma 15		4.5 months	33±11%	20±9%						
Massimino, Italy, 2005	55220	21	Total, GBM, other glioma	37, ~12, >60	~74, ~60, ~73	~50, ~40, ~73	~50, ~30, ~73	~44, ~30, ~73	~37, 0, ~73	log-rank	=.008 (GBM vs. other glioma)	
Ozkaynak, United States, 2004	7850	6	3 patients had sAppendix Table 8disease at a median follow up of 62 months. 3 patients were dead of disease at a median follow up of 4 months.									
Shih, United States, 2008	2530	5	Total, EPD (1), AA (2), GBM (2)	3.9, 2.4, 7.1, 63								

Study (Investigator, country, record#)	Record Number	Group (N)	OS	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Thorarinsdottir, United States, 2007	73050	Oligodendroglimoma 1, Ganglioma 1, Anaplastic glioma 3, Ependymoma 1	ODG 8 mo, GG 59 mo, AG 10 22 and 33.5 mo, EPD 37 mo	ODG 8 mo, GG 59 mo, AG 22 mo, EPD 37 mo								
Yule, United Kingdom, 1997	18960	4	1 GBM patient survived with sAppendix Table 8disease at a follow up of 12 months, a recurrent gbm patient died of disease at 6 months follow up, 1 anaplastic ependymoma patient died of disease at 15 months, and 1 suprasellar gbm pt died of toxicity, 1 CPC DOD 11mo									
Zacharoulis, United States, 2007	73020	Ependymoma 29		~48		69%		38	38±10 % (Kaplan meier curve vies 24%?)	Univariate Cox Proportional Hazards liklihood ratio	EFS Unstratified: Age P=.04, Extent of resection p=.49, Site P=.65. OS: Age p=.20, Extent of resection p=.53, Site P=.70	

Appendix Table C87: Time to Event Outcomes Treatment Glial Tumors Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2
Berger, France, 1998	75380	CPC (2)									
Bouffet, France, 1997	78760	5									
Bouffet, France, 2000	78770	24	PFS	~7	~4	~4	0	0	0		
Busca, Italy, 1997	73190	Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1	PFS Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1	Two patients disease progressed . One AA at 11 months and 1 oligodendroglioma at 4 months. All other patients alive with no progression or evidence of disease							
Dunkel, United States, 1998	78780	10									
Finlay, United States	1300	ABMR transplated(N=27), AA (N=10) and GBM (N=17)	EFS: Total ABMR (27)					22±7%		Unstratified comparison EFS ABMR vs CHM (Cox)	.014
Grill, France, 1996	73240	Ependymoma 16	For those who had sAppendix Table 8disease after HDCT (4), PFS lasted 5-8 mo with a median of 7 months								

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2
Gururangan, United States, 1998	18000	N=7, 1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma	PFS	Two patients progressed one GBM at 8 mo and 1 ependymoma at 16 mo. 1 patient died of toxicity before progression							
Jakacki, United States, 1999	15920	12	PFS: Total (12), GBM (4), AA (2), Pons (6) with 1 Pons patient	4.75 (2-12+), 4 (2-7), 4.75 (4.5-5), 7 (3-12+)							
Mahoney, United States, 1996	73250	Anaplastic Astrocytoma 2, Ependymoma 3, Glioblastoma multiforme 1, Brainstem glioma 1	PFS Anaplastic Astrocytoma 2, Ependymoma 3, Glioblastoma multiforme 1, Brainstem glioma 1	PFS evaluated in 3 of 7 patients. GBM 4 mo, BSG 1 mo, EPD 12 mo							
Mason, United States, 1998	73180	Ependymoma 15	PFS	4 months	~22	0					
Massimino, Italy, 2005	55220	21	PFS: Total, GBM, other glioma	~18, ~10, ~12	~55, ~40, ~73	~46, ~20, ~73	~46, ~20, ~73	~46, ~20, ~73	~40, 0, ~73	log-rank	=.04 PFS other vliomas vs PFS glioblastoma
Ozkaynak, United States, 2004	7850	6									
Shih, United States, 2008	2530	5	Time to Progression: Total, EPD (1), AA (2), GBM (2)	2.54, .95, 1.4, 5, 2.5							

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2
Thorarinsdottir, United States, 2007	73050	Oligodendroglimoma 1, Ganglioma 1, Anaplastic glioma 3, Ependymoma 1	PFS	ODG 8 mo, GG 59 mo, AG 3 17 and 33.5 mo, EPD 37 mo	ODG 8 mo, GG 59 mo, AG 17 mo, EPD 37 mo						
Yule, United Kingdom, 1997	18960	4									
Zacharoulis, United States, 2007	73020	Ependymoma 29	EFS	~22		35%		14%	12±6 %		

Appendix Table C87: Time to Event Outcomes Treatment Glial Tumors Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_3	Med (mos)_3	2 yr_3	4 yr_3	5 yr_3	Comment
Berger, France, 1998	75380	CPC (2)						
Bouffet, France, 1997	78760	5						
Bouffet, France, 2000	78770	24						
Busca, Italy, 1997	73190	Ependymoma 2, Anaplastic Astroctytoma 1, Glioblastoma Multiforme 2, Oligodendroglioma 1						
Dunkel, United States, 1998	78780	10						
Finlay, United States	1300	ABMR transplated(N=27), AA (N=10) and GBM (N=17)						
Grill, France, 1996	73240	Ependymoma 16						
Gururangan, United States, 1998	18000	N=7, 1 ependymoma, 4 glioblastoma multiforme, 1 anaplastic astrocytoma						
Jakacki, United States, 1999	15920	12						1 GBM patient was given an alternative treatment of HDC for progression and was not included in OS, but remains alive 18 months after initial treatment
Mahoney, United States, 1996	73250	Anaplastic Astrocytoma 2, Ependymoma 3, Glioblastoma multiforme 1, Brainstem glioma 1						
Mason, United States, 1998	73180	Ependymoma 15						One patient is alive 25+ months post ABMR
Massimino, Italy, 2005	55220	21						
Ozkaynak, United States, 2004	7850	6						
Shih, United States, 2008	2530	5						All Patients Were Dead of Disease as final status

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_3	Med (mos)_3	2 yr_3	4 yr_3	5 yr_3	Comment
Thorarinsdottir, United States, 2007	73050	Oligodendroglimoma 1, Ganglioma 1, Anaplastic glioma 3, Ependymoma 1						
Yule, United Kingdom, 1997	18960	4						
Zacharoulis, United States, 2007	73020	Ependymoma 29	Post-progression surival Ependymoma 22	~28	46%	9%	9%	14 pts (48%) Dead of Disease, 3 Dead of Toxicity (10.3), 2 are alive with progressive disease (7%), 4 are alive with sAppendix Table 8disease (14%), 6 have no evidence of disease at last followup (21)

Appendix Table C88: Time to Event Outcomes Comparator Glial Tumors OS

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Ayan, Turkey, 1995	74690	Anaplastic Ependymoma 4		33 mo (16-35 mo) [1 pt not included due to loss to fu at 9 months. Pt was non responsive to therapy)								
Berger, France, 1998	75380	CPC total (20), CPC partial resection (12), CPC gross total surgical resection (8)	1 patient in the partial resection group (9%) was alive and well at 55mo follow up. 7 patients in the gross total resection group were alive and well at a median 25 mo (3-72mo) follow up	Total median OS was 10 mo (1-41mo). Partial resection OS had a median of 11 mo (3-41 mo). Gross total resection OS was 5 mo in 1 patient.							Kaplan meier survival curves for gross total resection vs. partial resection were significantly different at p=.009	
Bertolone, United States, 2003	10380	18	GBM and AA only non-infants, Infants	~48, ~22	~83, ~52	~64, ~25	~57, ~25	36+-13%, 25+-15%	36+-13%, 25+-15%			
Conter, France, 2009	73540	Ependymoma 24	8 patients died all of neoplastic disease				79.2%		74.8%			

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Doireau, France, 1998	55990	8	87.5% at median 4.8 years F/U									
Finlay, United States, 2008	1300	56	@ 4 months	~.7 months AA ~ .6 monthsGBM ~ 6 monthsBulky ~.7 monthsNon-Bulky ~1.2 months	HSCT, Comparator |AA: ~41%, ~26%, GBM: ~43%, ~22%		HSCT, Comparator |AA: 40+-14%,7+-4% GBM: 12+-6%, 0		HSCT, Comparator |AA: 40+-14%,~4 GBM: 12+-6%, 0	Wilcoxon	.018 overall, by histology .010	1.9 (1.1-3.1)

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Grill, France, 2001	74360	Ependymoma 73, Supratentorial 13, Posterior Fossa 60, Low grade 8, High grade 60, Complete resection 44, incomplete resection 29, no residuum, radiographic residum		~5.2 years for total population	Ependymoma 88%,	Ependymoma 79 (68-87%)	Ependymoma 53%	Ependymoma 73%, Supratentorial 100, Posterior Fossa 50 (37-64), Low grade 58 (26-85%), High grade 61 (47-73), Complete resection 69 (53-82), incomplete resection 46 (28-65), no residuum 74 (59-86), radiographic residum 35	Ependymoma 24%	two-tailed log rank test	RR difference multivar/univar between Age p=.86/.61, Location p=.0004/.013, Grade .97/.89, Surgery p=.92/.22, Imaging p=.0009/.008	
Grundy, United Kingdom, 2007	73750	Non-metastatic ependymoma 80, metastatic ependymoma 9				90, 78		59, 33				

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Grundy, United States, 2010	51800	41	HGG, Brain Stem Tumor, CPC		57.9 (33.2-76.3 CI), 14.3 (.7-46.5), 50.3 (23.1-72.4)		40.5 (18.7-61.5), 0, 21.5 (5.2-45.0)		34.7 (14.6-56.0), 0, 21.5 (5.2-45.0)			
Horn, Untied States, 1999	74470	Ependymoma 83							57.2±5 %			
Hurwitz, United States, 2001	53330	45										
Jaing, Taiwan, 2004	74030	WHO II 20, WHO III 23, Male 25, Female 23, <3 25, >3 34, Supratentorial 15, Infratentorial 28, GTR 18, STR 19, biopsy 6, RT invovled field 31, without RT 12, CHM 13, without CHM 30	WHO II 74%, WHO III 35, Male 49, Female 62, <3 42, >3 57, Supratentorial 57, Infratentorial 52, GTR 82, STR 37, biopsy 33, RT invovled field 58, without RT 48, CHM 54, without CHM 54						Ependymoma total 53.9	Fischer's exact chi-square test	5 year OS: Histology p=.005, Gender p=.425, Age p=.036, Location p=.917, Surgical resection <.001, Leptospinal dissemination .388, Radiotherapy .150, Chemotherapy .279	

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Kobrinsky, United States, 1999	53560	42	Brain Stem Glioma, High Grade Astrocytoma	~5, ~5	9+-5%, 28+-10%,	0, ~9%	0, ~9%					
Korones, United States, 2006	52670	5 (2 BSG, 2 AST, 1 Glioblastoma)	OS: 1 AA pt DOD at 4 mos, 1 AWD at 10+ mo, 1 glioblastoma pt DF at 15+ mo, 2 BSG pts DOG at 9 and 4 mo									
Kuhl, Germany, 1998	17700	10	Anaplastic Ependymoma (11)						62 ± 11,			
Macdonald, United States, 2005	55000	76	Total (76), Regiment A (23), Regimen B (27), Regimen C (26), Anaplastic Astrocytoma (30), Glioblastoma Multiforme (40), Other (6)	~13, ~18, ~19, ~12, ~12, ~14, ~46	~51, ~55, ~55, ~37, ~46, ~45, ~62	~30, ~33, ~39, ~19, ~28, ~28, ~62	~28, ~27, ~39, ~16, ~22, ~22, ~62	~25, ~20, ~39, ~16, ~25, ~24,~40	24±5, 18±8, 39±10, 16±7, 25±8, 22±7, 40±22	Log-rank?	P=.23, P=.47	
Merchant, United States, 2002	74280	Ependymoma 64										
Robertson, United States, 1998	74630	Ependymoma 32			97%		75%		53%			

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Med (mos)	1 yr	2 yr	3 yr	4 yr	5 yr	Test	p	HR (95%) CI
Sio, Italy, 2006	6950	14	OS: , , , Brainstem Glioma	Total 5.5 (n=8), Ependymoma 4.5(n=2), Anaplastic Astrocytoma 5 (n=3), Brainstem Glioma 6 (n=2) 6 Alive with Disease at median 11.5 mos, Brainstem Glioma 11 (n=5), GBM 12 mos (n=1)								
Wrede, Germany, 2009	75590	CPC 34	OS: CPC (N=29)		~82		~70		36	Cox, CPC vs CPP/ APP	P.003	26.4

Appendix Table C89: Time to Event Outcomes Comparator Glial Tumors PFS

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Ayan, Turkey, 1995	74690	Anaplastic Ependymoma 4	PFS, Anaplastic Ependymoma 4	27 mo (only 1 pt evaluated. Other patients had only partial response or no response to treatment)									
Berger, France, 1998	75380	CPC total (20), CPC partial resection (12), CPC gross total surgical resection (8)											

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Bertolone, United States, 2003	10380	18											The infants category includes 1 case excluded by this review of medulloblastoma that coulda not be abstracted seperately
Conter, France, 2009	73540	Ependymoma 24	PFS	median time to first relapse was 22 months (4-46 months)			62.5%		54.2%				
Doireau, France, 1998	55990	8	Event free survival: 50% at 4 years										1 patient died at 32 months
Finlay, United States, 2008	1300	56	EFS CHM unstratified		HSCT, Comparator |AA: ~30, ~10 GBM: 22+-7%, 0%		HSCT, Comparator |AA: ~22+-7, 0 GBM: 22+-7, 0%		HSCT, Comparator |AA: ~22+-7, 0 GBM: 22+-7, 0%				

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Grill, France, 2001	74360	Ependymoma 73, Supratentorial 13, Posterior Fossa 60, Low grade 8, High grade 60, Complete resection 44, incomplete resection 29, no residuum, radiographic residum	PFS Ependymoma 73	Ependymoma total ~ 1.8 years	Ependymoma 56%	Ependymoma 29%	Ependymoma 23 %	Ependymoma 12%	Ependymoma 12%				At time of analysis 31 patients had died of progressive disease from 3 months to 5.8 years (Median 29 months). Age was analyzed in univariate analysis but no difference between strata > 2 years and below 2 years was observed

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Grundy, United Kingdom, 2007	73750	Non-metastatic ependymoma 80, metastatic ependymoma 9	EFS: Non-metastatic ependymoma 80, metastatic ependymoma 9	~34, ~18		64, 33		43, 0		Cox-proportional hazards model	Metastatic OS vs non-metastatic p<.0001	4.1 (2.0-8.7 95% CI)	
Grundy, United States, 2010	51800	41	Event Free Survival HGG, Brain Stem Tumor		52.6 (28.7-71.9),0.0		24.1 (7.8-45.1)		18.1 (4.6-38.6)				7 pts alive at last follow up
Horn, Untied States, 1999	74470	Ependymoma 83	PFS						42.2±5.5%				

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Hurwitz, United States, 2001	53330	45	Time to progression	Astrocytoma 21.2 (1.2-49.3), Malignant glioma 1.4 (.4-7.2), Brain Stem Glioma 1.4 (.5-37.8), Ependymoma 2.1 (.0-30.3)									No astrocytoma patients had progressive disease or early death, 10 malignant glioma (77%) had progressive disease and early death, 9 brainstem glioma (60%), 7 ependymoma (53)

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Jaing, Taiwan, 2004	74030	WHO II 20, WHO III 23, Male 25, Female 23, <3 25, >3 34, Supratentorial 15, Infratentorial 28, GTR 18, STR 19, biopsy 6, RT invovled field 31, without RT 12, CHM 13, without CHM 30	PFS WHO II 68%, WHO III 27, Male 42, Female 52, <3 22, >3 51,Supratentorial 42, Infratentorial 52, GTR 72, STR 31, biopsy 18, RT invovled field 52, without RT 31, CHM 35, without CHM 52						Ependymoma total 45.9	Fischer's exact chi-square test	5 year PFS: Histology p=.002 Gender p=.775, Age p=.005, Location p=.957, Surgical resection <.001, Leptospinal dissemination .663, Radiotherapy .029, Chemotherapy .820		
Kobrinsky, United States, 1999	53560	42											

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Korones, United States, 2006	52670	5 (2 BSG, 2 AST, 1 Glioblastoma)	PFS: 1 BSG pt progressed at 4 mo, 1 GBM progression free at 15+ mo, anaplastic astrocytoma progression free at 10+ mo										
Kuhl, Germany, 1998	17700	10	PFS Anaplastic Ependymoma (11), pts with residual tumor (11), pts with no residual tumor (10)	10 months					52 ± 11, 36±15, 70±14		non-significant statistical difference		

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Macdonald, United States, 2005	55000	76	Event-free Survival: Total, A (23), B (27), C (26), AA (30), GBM (40), Other (6)	~5, ~9, ~3, ~3, ~3, ~3, ~11	~23, ~27, ~10, ~20, ~26, ~15, ~40	~10, ~14, ~8, ~8, ~10, ~8, ~21	~10, ~14, ~4, ~8, ~10, ~8, ~21	~8, ~14, ~4, ~8, ~7, ~8, ~21	8±3, 14±7, 4±4, 8±6, 7±5, 8±4, 21±18	Log-rank?	P=.07, P=.28		Of the 76 patients 56 (74%) died; 52 deaths were disease related, 1 was due to infection, 2 to hemmorrhage, and 1 to AML development. All analysis ITT EFS defined as minimum time from entry to disase progression, relapse a second mal. Neoplasm/death

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Merchant, United States, 2002	74280	Ependymoma 64	PFS			88±6%	71%						6 ependymoma patients suffered recurrent or progressive disease.
Robertson, United States, 1998	74630	Ependymoma 32	PFS		88%		56%		38%		No significant difference between the two chemotherapy groups, p>.2		

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome_2	Med (mos)_2	1 yr_2	2 yr_2	3 yr_2	4 yr TRM	5 yr_2	Test_2	p_2	HR (95% CI)_2	Comment
Sio, Italy, 2006	6950	14	Progression Free Survival: Total, Ependymoma, Anaplastic Astrocytoma, Brainstem glioma, Glioblastoma multiforme	3 (n=14), Ependymoma 11 (n=2), Anaplastic Astrocytoma 3 (n=3), Brain Stem Glioma 1 (n=8), Glioblastoma multiforme 11 (n=1)									
Wrede, Germany, 2009	75590	CPC 34	EFS CPC N=29		~56		~56		~36	Cox, CPC vs. CPP/APP	p<.0001	HR=15.2	

Appendix Table C90: Neurological Outcomes

Study (Investigator, country, record#)	Record Number	Group (N) (NNO)	Comments (NNO)	Group (N) (NDP)	Comments (NDP)	Group (N) (OOI)	Comments (OOI)
Conter, France, 2009	73540		Two patients were placed in a special school, and two were ≥ 2 years behind at school	Ependymoma 16 (living patients)	Mild retardation 2 (13), Severe retardation 2 (13)	Ependymoma 16 (living patients)	Diplopia 5 (32), mild decrease of visual acuity 1 (6), Severe decrease of visual acuity 1 (6)
Grundy, United States, 2010	51800	21 children alive at last follow up (all histologies, 7 of whom were high-grade gliomas; authors do not give histology in toxicity discussion)	5 children required special needs education				
Thorarinsdottir, United States, 2007	73050			Oligodendroglimoma 1, Ganglioma 1, Anaplastic glioma 3, Ependymoma 1	ODG pt had decreased neurologic responsiveness/ blindness, GG pt had ADD, 1 AG patient had L hemiparesis, 1 AG pt had Ataxia, 1 EPD pt had hypotonia/multiple neuropathies GR 2-4 hearing loss/poor speech		

Appendix Table C91: Adverse Events Treatment Glial Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	Comment	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Comment TRM
Bouffet, France, 2000	78770	24	1 Aspergillus fumigatus , 1 cytomegalovirus	4, 4		24	1 VOD, 1 toxic exfoliative dermatiti with acute renal failure, 1 aspergillus fumigatus pneumonia		4, 4, 4	
Finlay, United States	1300					HSCT (27)	5 toxic deaths	median 17 days	19%	Single death in thiotepa/etoposide (9%), 2 with carmustine (40%), and 2 with carboplatin (9%)regimens
Grill, France, 1996	73240	Ependymoma 16	six documented infectious episodes (2 septicemia, 3 pneumonia, 1 viral encephalitis)	38%		Ependymoma 16	1 death at day 50 following ABMT, 1 pt exerpeinced coma w/ seizures during multiorgan failure leading to death		13%	
Gururangan, United States, 1998	18000					N=4 glioblastoma multiforme	1 patient died of treatment related toxicity at .03 months.		25%	
Jakacki, United States, 1999	15920	12	Two patients had interstitial pneumonia which resolved with treatment	17						

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	%	Comment	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Comment TRM
Mahoney, United States, 1996	73250					Ependymoma 3	Death at 32 days after BMT due to pulmonary hemorrhage in pt with mutiple relapsed ependymoma	1 mo	33%	
Mason, United States, 1998	73180					Ependymoma 15	Toxic mortality		5 pts (33%)	Authors state toxic mortality rate was unexpected and unaccepAppendix Table 8
Thorarinsdottir, United States, 2007	73050	Oligodendroglimoma 1, Ganglioma 1, Anaplastic glioma 3, Ependymoma 1	# G+ bacterium: Oligodendroglimoma 2, Ganglioma 3, Anaplastic glioma 0, 1, 2, Ependymoma 4	ODG 100, GG 100, AG 67, EPD 100						
Zacharoulis, United States, 2007	73020	Ependymoma 29	3 cases of sepsis leading to toxic morality.	10.3 %	No toxic deaths since 1998					

Appendix Table C91: Adverse Events Treatment Glial Tumors Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Group (N) Hepatic veno-occulusive disease (Hepatic Sinusoidal Obstruction)	Severity of Grade hVOD	% hVOD	Comments hVOD	Serious Hemmoragic Event	Group (N)_12	Severity of Grade SHE	% SHE
Bouffet, France, 2000	78770	24	24	4 mild-severe, 1 fatal	17, 4	Pt died due to multiorgan failure	Serious Hemmoragic Event			
Grill, France, 1996	73240	Ependymoma 16	Ependymoma 16		3 grade 2 VOD (19%)		Serious Hemmoragic Event	Ependymoma 16	2 pts had severe epistaxis requiring platelet transfusion	13 %

Appendix Table C92: Adverse Events Comparator Glial Tumors

Study (Investigator, country, record#)	Record Number	Group (N)	Infectious	Severity or Grade	%	Comment	Group (N) TRM	TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Serious Hemmorragic Event	Group (N)_12	Severity of Grade SHE	% SHE	Comments SHE
Grundy, United Kingdom, 2007	73750		Infectious				Ependymoma 89	TRM	1 postoperative death		1%	Serious Hemmorragic Event				
Macdonald, United States, 2005	55000	Total (76), A (23), B (27), C (26)	Infectious	3 or 4	6 (8), 2 (9), 3 (11), 1 (4)	1 patient died due to infection (group not given)		TRM				Serious Hemmorragic Event	Total (76)	Death	2 (3)	Group not given for deaths
Robertson, United States, 1998	74630		Infectious				Ependymoma 32	TRM	1 toxic treatment related death	1 death at 14 months	3%	Serious Hemmorragic Event				

Appendix Table C93: Design, Participant Selection and Enrollment Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Arvio M, Finland, 2001	14180	Inherited Metabolic Diseases	aspartylglucosaminuria		7 HSCT, 12 non-HSCT	transplant: 1991-1997follow-up: 1-7.6 yrs	case series	5 HSCT, 12 non-HSCT		2 HSCT with longer follow-up entered under Malm #8490
Autti T, Finland, 1999	15540	Inherited Metabolic Diseases	aspartylglucosaminuria		2 HSCT, 6 non-HSCT, 7 non-diseased	follow-up: 4-7 yrs	quasi-experimental	15	0	
Banjar H, Saudi Arabia, 1998	17920	Inherited Metabolic Disease	Gaucher Type 3		7	follow-up: 2.5-3.5 yrs	case series	3	0	This study combined two disease types, Gaucher Type 1 and Gaucher Type 3. Three of the pts had Gaucher Type 3.
Chan LL, Malaysia, 2002	11330	Inherited Metabolic Disease	Gaucher Type 3		1	treatment: Jun 1996 - May 1998follow-up: 1.8 yrs on treatment, 2.7 yrs after treatment stopped	case report	1	0	
Chen R, Taiwan, 2007	4490	Inherited Metabolic Disease	Gaucher Type 3		1	transplant: Jul 2004follow-up: 1.5 yrs	case report	1	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Coppa GV, Italy, 1999	16350	Inherited Metabolic Disease	MPS II, Hunter disease		1	transplantation: 1995follow-up: 4 yrs	case report	1	0	
Ehlert K, Germany, 2006	4690	Inherited Metabolic Disease	Farber disease		3	follow-up: 0.7-1.3 yrs	case series	3	0	
El-Beshlawy A, Egypt, 2006	5750	Inherited Metabolic Disease	Gaucher Type 3		22	follow-up: 5-26 mos	case series	11	0	This study combined Gaucher Type 1 and Gaucher Type 3 pts, and 11 were Gaucher Type 3.
Erikson A, Sweden, 1995	21630	Inherited Metabolic Disease	Gaucher Type 3		8	follow-up: 2.0-2.4 yrs	case series	3	0	This study included 5 adult pts and 3 pediatric pts.
Goker-Alpan O, US, 2008	1790	Inherited Metabolic Diseases	Gaucher Type 3		32		2 HSCT followed by ERT; 30 ERT only	2	0	
Grewel S, US, 2003	9750	Inherited Metabolic Disease	Mucolipidosis II (I-cell disease)		1	follow-up: 5 yrs	case report	1	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Guffon N, France, 2009	680	Inherited Metabolic Disease	MPS II, Hunter disease		8	transplantations: 1990-2000follow-up: 5-14 yrs	case series	8	0	
Hsu YS, Taiwan, 1999	16540	Inherited Metabolic Diseases	Niemann-Pick Type C		1	follow-up: 0.8 yrs	case report	1	0	
Imaizumi M, Japan, 1994	23220A	Inherited Metabolic Diseases	MPS II, Hunter disease		4	follow-up: 2 yrs	case series	1	0	this study combined diseases, only one was Hunter disease
Imaizumi M, Japan, 1994	23220B	Inherited Metabolic Disease	Mucolipidosis II (I-cell disease)		4	transplant: 1986follow-up: 5.6 yrs	case series	1	0	this case series combined diseases, only 1 in case series had mucolipidosis II
Jacobs JFM, Netherlands, 2005	6740	Inherited Metabolic Disease	Tay-Sachs disease		1	follow-up: 2 yrs	case report	1	0	
Laitinen A, Finland, 1997	19620	Inherited Metabolic Disease	aspartylglucosaminuria		1	follow-up: 4 mos	case report	1	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Lange MC, Brazil, 2006	5690	Inherited Metabolic Disease	MPS III, Sanfilippo syndrome		8	transplant: 1988-2000 (total study pop)follow-up: 3.3-14.2 yrs (total study pop)	case series	1	0	only 1 of 8 pts in study population with Sanfilippo syndrome (MPS III)
Li P, US, 1996	20260	Inherited Metabolic Disease	MPS II, Hunter disease		1	follow-up: 5 yrs	case report	1	0	
Lonnquist T, Finland, 2001	12960	Inherited Metabolic Disease	ceroid lipofuscinosis		3	transplant: Jun 1996 - Oct 1998follow-up: 2-4 yrs	case series	3	0	
Maegawa GHB, Canada, 2009	56590A	Inherited Metabolic Disease	Sandhoff's disease		5	follow-up: 2 yrs	single arm	3	0	This study combined diseases and 3 are Sandhoff's disease.
Maegawa GHB, Canada, 2009	56590B	Inherited Metabolic Disease	Tay-Sachs disease		5	follow-up: 2 yrs	single arm	2	0	This study combined diseases and 2 pts had Tay-Sachs disease.
Malm G, Sweden, 2004	8490	Inherited Metabolic Diseases	aspartylglucosaminuria		2	transplant: 1996follow-up: 5 yrs	case series	2	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
McKinnis EJR, US, 1996	20560	Inherited Metabolic Disease	MPS II, Hunter disease		1	transplant: 1988follow-up: 5.6 yrs	case report	1	0	
Morel CF, Canada, 2007	3010	Inherited Metabolic Diseases	Niemann-Pick Type A		1	follow-up: 2.7 yrs	case report	1	0	
Muenzer J, US, 2006	57160	Inherited Metabolic Disease	MPS II, Hunter disease		96	follow-up: 1 yr	RCT	96	0	Age range of study participants: 5-31 yrs and cannot separate the adult data from the pediatric data.
Muenzer J, US, 2007	57070	Inherited Metabolic Diseases	MPS II, Hunter disease		12	follow-up: 1 yr	RCT for 6 mos, followed by open-label extension for another 6 mos	12	0	
Mullen CA, US, 2000	15300	Inherited Metabolic Disease	MPS II, Hunter disease		1	follow-up: 2.2 yrs	case report	1	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Paciorkowski AR, US, 2008	2980	Inherited Metabolic Disease	Niemann-Pick Type C		1	follow-up: 1 yr	case report	1	0	
Page KM, US, 2008	1280A	Inherited Metabolic Disease	Tay-Sachs disease		19	transplant: Sep 1998 - Apr 2007	case series	1	0	this is one case within a case series which included other diseases
Page KM, US, 2008	1280B	Inherited Metabolic Disease	MPS II, Hunter disease		19	transplantations: Sep 1998 - Apr 2007	case series	2	0	This study combined several diseases, only 2 pts had MPS II.
Patterson MC, US, 2007	56970	Inherited Metabolic Disease	Niemann-Pick Type C		41	enrollment: Mar 2002 - Apr 2004follow-up: 1 yr	randomized controlled trial	12	1	This study included adults. Results presented by grps of <12 (n=12) and >=12 (n=29). Most results were presented for the >=12 grp, but some results were available for the <12 grp.

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Patterson MC, US, 2010	56500	Inherited Metabolic Disease	Niemann-Pick Type C		10	treatment: Aug 2003-Jan 2008follow-up: 1 yr RCT, 1 yr extension study	open label extension study	9	1 withdrew due to adverse event of Crohn disease	12 entered RCT, 10 entered 1 yr extension
Pineda M, Spain, 2009	56560	Inherited Metabolic Disease	Niemann-Pick Type C		66	observational period: 2003 - Jul 2008	retrospective observational	66	0	
Ringden O, Sweden, 1995	22020	Inherited Metabolic Diseases	Gaucher Type 3		6	follow-up: 5-11 yrs	case series	6	0	
Ringden O, Sweden, 2006	5940A	Inherited Metabolic Disease	MPS III, Sanfilippo syndrome		71	follow-up: 0.4-14.0 yrs	case series	2	0	This is a study of HSCT in 71 pts with inborn errors of metabolism; 2 pts have MPS III.
Ringden O, Sweden, 2006	5940B	Inherited Metabolic Disease	Sandhoff's disease		71	follow-up: 0.4-14.0 yrs	case series	1	0	This is a study of HSCT in 71 pts with inborn errors of metabolism; 1 pt has Sandhoff's disease.

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Schiffman R, Netherlands, 2008	56750	Inherited Metabolic Disease	Gaucher Type 3	substrate reduction therapy combined with ERT	30	follow-up: 2 yrs	phase II open-label clinical trial	30	0	Year 1: 21 received substrate reduction therapy, 9 received no treatmentYear 2: all received substrate reduction therapy
Schiffmann R, Netherlands, 1997	58150	Inherited Metabolic Disease	Gaucher Type 3		5	follow-up: up to 5 yrs	case series	5	0	
Seto T, Japan, 2001	13460A	Inherited Metabolic Disease	MPS II, Hunter disease		23	follow-up: up to 7.0 yrs	case series	10	0	This study followed 23 mucopolysaccharidosis pts, 10 had MPS II, 3 of those 10 had HSCT.
Seto T, Japan, 2001	13460B	Inherited Metabolic Disease	MPS IV, Morquio disease		23	follow-up: up to 7 yrs	case series	4	0	This study followed 23 mucopolysaccharidosis pts, 4 had MPS IV and 1 underwent HSCT.
Shield JPH, England, 2005	6720	Inherited Metabolic Disease	GM1 gangliosidosis		1	follow-up: 7 yrs	case report	1	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Sivakumar P, England, 1999	16200	Inherited Metabolic Disease	MPS III, Sanfilippo syndrome		2: 1 HSCT, 1 non-HSCT	follow-up: 7.4 yrs	comparative study	2	0	comparison of one treated sibling with one untreated sibling
Stein J, Israel, 2007	4880	Inherited Metabolic Disease	Wolman disease		1	follow-up: 11 yrs	case report	1	0	
Takahashi, Japan, 2001	14030	Inherited Metabolic Disease	MPS II, Hunter disease		7	follow-up: 1.1 yrs	quasi-experimental	1 HSCT; 2 non-HSCT	0	This study combined several diseases, 3 had MPS II, one of which underwent HSCT, two did not.
Tokimasa, Japan, 2008	1310	Inherited Metabolic Disease	MPS II, Hunter disease		5	transplantation: Sep 2005follow-up: 0.8 yrs	case series	1	0	This study combined several diseases, only one was MPS II.
Tolar J, US, 2009	1370	Inherited Metabolic Disease	Wolman disease		4	follow-up 0.2-11.0 yrs, thru Apr 2008	case series	4	0	
Tsai P, US, 1992	25120	Inherited Metabolic Disease	Gaucher Type 3		1	follow-up: 2 yrs	case report	1	0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Vellodi A, England, 1999	16650	Inherited Metabolic Disease	MPS II, Hunter disease		10	transplantations: 1982-1991follow-up: 7-14 yrs	case series	9	1	4 died <100 days post, 1 died 4 yrs post, 1 died unknown follow-up of GVHD, detailed follow-up on only 3 pts
Vormoor J, Germany, 2004	9420	Inherited Metabolic Diseases	Farber disease		2	follow-up: 0.9-1.2 yrs	case series	2	0	
Yeager AM, US, 2000	14880	Inherited Metabolic Disease	Farber disease		1	follow-up: 2.3 yrs	case report	1	0	

Appendix Table C94: Participant Characteristics Treatment Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category
Arvio M, Finland, 2001	14180	5	3.04	2.75	1.6-5.5	white (100%)	M (40.0%) F (60.0%)	
Autti T, Finland, 1999	15540	2	2.3 yrs		2.0-2.6 yrs	white (100%)	M (100%)	
Chen R, Taiwan, 2007	4490	1	5.8 yrs			Asian (100%)	F (100%)	
Coppa GV, Italy, 1999	16350	1	3 yrs			White (100%)	M (100%)	
Ehlert K, Germany, 2006	4690	3	3.2 yrs	3.8 yrs	2.0-3.9 yrs		M (33.3%) F (66.7%)	Type 2/3, no CNS involvement
Goker-Alpan O, US, 2008	1790	2	1.3 yrs at dx			White (50%), Hispanic (50%)	Male (50%), Female (50%)	
Grewel S, US, 2003	9750	1	1.6 yrs				F (100%)	
Guffon N, France, 2009	680	8	5.8 yrs	4.6 yrs	7-17 yrs		M (100%)	2 attenuated1 intermediate5 severe
Hsu YS, Taiwan, 1999	16540	1	2.5 yrs			Asian (100%)	F (100%)	
Imaizumi M, Japan, 1994	23220A	1	9.8			Asian (100%)	Male (100%)	attenuated form
Imaizumi M, Japan, 1994	23220B	1	0.7 yrs			Asian (100%)	Female (100%)	CNS impairment present
Jacobs JFM, Netherlands, 2005	6740	1	3.8 yrs				F (100%)	aymptomatic
Laitinen A, Finland, 1997	19620	1	1.5 yrs			white (100%)	M (100%)	asymptomatic
Lange MC, Brazil, 2006	5690	1	6 yrs			Hispanic (100%)	F (100%)	
Li P, US, 1996	20260	1	5.0 yrs				M (100%)	severe
Lonnquist T, Finland, 2001	12960	3	0.5 yrs	0.3 yrs	0.3-0.6 yrs	white (100%)	M (33.3%) F (66.7%)	infantile neuronal form: one mildly symptomatictwo asymptomatic
Malm G, Sweden, 2004	8490	2	8.1 yrs		5.8-10.4	white (100%)	M (50%) F (50%)	
McKinnis EJR, US, 1996	20560	1	2.4 yrs				M (100%)	severe

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Race (%)	Gender M, F (%)	Disease Stage/category
Morel CF, Canada, 2007	3010	1	0.25 yrs				F (100%)	
Mullen CA, US, 2000	15300	1	0.8 yrs				M (100%)	Type IIB, mild
Page KM, US, 2008	1280A	1	0.06 yrs					
Page KM, US, 2008	1280B	2	<= 0.25 yrs					
Ringden O, Sweden, 1995	22020	6	3.5 yrs	2 yrs	2-9 yrs		Male (67%); Female (33%)	2 advanced, 2 early, 2 progressive
Ringden O, Sweden, 2006	5940A	2						1 Type A and 1 Type C
Seto T, Japan, 2001	13460A	3	5.7 yrs	6.0 yrs	2.0-9.0 yrs	Asian (100%)	Male (100%)	1 intermediate2 mild
Seto T, Japan, 2001	13460B	1	15 yrs			Asian (100%)	Male (100%)	Type A
Shield JPH, England, 2005	6720	1	0.6 yrs			Asian (100%)	M (100%)	asymptomatic
Sivakumar P, England, 1999	16200	1	0.6 yrs				M (100%)	type IIIA
Stein J, Israel, 2007	4880	1	0.25 yrs			White (100%)	F (100%)	
Takahashi, Japan, 2001	14030	1	4.7 yrs			Asian (100%)		severe
Tokimasa, Japan, 2008	1310	1	5.8 yrs			Asian (100%)	M (100%)	
Tolar J, US, 2009	1370	4	0.8 yrs	1.3 yrs	0.2-2.1 yrs	White (50%)Not reported (50%)	Male (25%), Female (75%)	
Tsai P, US, 1992	25120	1	2 yrs				Female (100%)	
Vellodi A, England, 1999	16650	3	2.5 yrs	1.7 yrs	0.8-5.1 yrs		M (100%)	
Vormoor J, Germany, 2004	9420	2	3.9 yrs		3.8-3.9 yrs	white (100%)	M (50%) F (50%)	type 2/3, no CNS involvement
Yeager AM, US, 2000	14880	1	0.8 yrs				F (100%)	Type I with CNS involvement

Appendix Table C95: Participant Characteristics Comparator Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Arvio M, Finland, 2001	14180	12					white (100%)	M (58.3%) F (41.7%)			
Autti T, Finland, 1999	15540	6 non-HSCT, 7 non-diseased	non-HSCT: 6.0 yrs	non-HSCT: 5.8 yrs	non-HSCT: 3.0-10.0 yrs						
Banjar H, Saudi Arabia, 1998	17920	3	2.6 yrs	2.8 yrs	2.0-3.0 yrs			Male (33%), Female (67%)			
Chan LL, Malaysia, 2002	11330	1	7.6				Asian (100%)	Female (100%)			
El-Beshlawy A, Egypt, 2006	5750	11	6.14 yrs		1-16 yrs						Mean age and range are for the whole study population of 22, which includes 11 Gaucher Type 1 pts.
Erikson A, Sweden, 1995	21630	3	7.4 yrs	4.8 yrs	3.8-13.7 yrs			Male (33%), Female (67%)			
Goker-Alpan O, US, 2008	1790	30			0.2-2.5 yrs at dx		Hispanic (36.7%), Black (6.7%), White (56.7%)	Male (53.3%), Female (46.7%)			

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Maegawa GHB, Canada, 2009	56590B	2	13.1 yrs	13.1 yrs	10.1-16.0 yrs			Female (100%)	juvenile form		
Maegawa GHB, Canada, 2009	56590A	3	15.6 yrs	18 yrs	8.7-20.1 yrs			Male (67%), Female (33%)	juvenile form		

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Muenzer J, US, 2006	57160	96	placebo, n=32: 13.1 +/- 1.22 yrsERT EOW, n=32: 14.4 +/- 1.2 yrsERT wkly, n=32: 15.1 +/- 1.11 yrs		placebo, n=32: 5.0-29.0 yrsERT EOW, n=32: 5.4-30.9 yrsERT wkly, n=32: 6.3-26.0 yrs		placebo: Asian (9.4%), Black (12.5%), White (75.0%), Other (3.1%) ERT EOW: S Amer Ind (6.3%), Asian (6.3%), Black (3.2%), White (84.3%)ERT wkly: S Amer Ind (3.1%), Black (6.3%), White (87.5%), Other (31%)		Disease score (2-6):placebo: 3 (22%), 4 (44%), 5 (28%), 6 (6%)ERT EOW: 2 (6%), 3 (19%), 4 (34%), 5 (28%), 6 (13%)ERT wkly: 2 (6%), 3 (22%), 4 (31%), 5 (31%), 6 (9%)		Age stratification: placebo: 5-11 yrs (46.9%), 12-18 yrs (31.3%), 19-31 yrs (21.9%)ERT EOW: 5-11 yrs (43.8%), 12-18 yrs (31.3%), 19-31 yrs (25.0%) ERT wkly: 5-11 yrs (43.8%), 12-18 yrs (28.1%), 19-31 yrs (28.1%)

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Muenzer J, US, 2007	57070	12	placebo: 16.7 yrsERT .15 mg/kg: 11.0 yrsERT .5 mg/kg: 20.0 yrsERT 1.5 mg/kg: 10.0 yrs	placebo: 17 yrsERT .15 mg/kg: 10 yrsERT .5 mg/kg: 20 yrsERT 1.5 mg/kg: 8 yrs	placebo: 13-20 yrsERT .15 mg/kg: 9-14 yrsERT .5 mg/kg: 20 yrsERT 1.5 mg/kg: 6-10 yrs		White (100%)	Male (100%)	attenuated		
Paciorkowski AR, US, 2008	2980	1	3.3 yrs					Female (100%)			
Patterson MC, US, 2007	56970	12	7.2		4-11	2.5		Male (42%), Female (58%)			
Patterson MC, US, 2010	56500	12	7.2 yrs	7 yrs	4-11 yrs	2.5 yrs		Male (42%), Female (58%)			
Pineda M, Spain, 2009	56560	66	12.8 yrs	<6 yrs: n=206- 11 yrs: n=14>= 12 yrs: n=27	0.6-43.0 yrs	9.5 yrs		Male (47%), Female (53%)			Cannot separate pediatric and adult pt data.

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Schiffman R, Netherlands, 2008	56750	30	substrate reduction therapy (n=21): 10.4 yrsno treatment (n=9): 9.9 yrs			substrate reduction therapy (n=21): 5.1 yrsno treatment (n=9): 4.0 yrs		substrate reduction therapy (n=21): Male (48%)no treatment (n=9): Male (22%)			Age distribution grps:substrate reduction therapy: 2-11 yrs (52%), 12-17 yrs (33%), >=18 yrs (14%)no treatment: 2-11 yrs (88%), 12-17 yrs (0%), >=18 yrs (11%)
Schiffmann R, Netherlands, 1997	58150	5	6.6 yrs	7.5 yrs	3.5-8.5 yrs			Male (75%), Female (25%)	aggressive systemic disease		3 had partial splenectomy prior to ERT
Seto T, Japan, 2001	13460A	7	7 yrs	6 yrs	4-12 yrs		Asian (100%)	Male (100%)	2 severe2 intermediate3 mild		
Seto T, Japan, 2001	13460B	3	11.7 yrs	13 yrs	4-18 yrs		Asian (100%)	Male (66.7%), Female (33.3%)	Type A		
Sivakumar P, England, 1999	16200	1	5 yrs					F (100%)	type IIIA		
Takahashi, Japan, 2001	14030	2	5.9 yrs		5.8-6.0 yrs		Asian (100%)				

Appendix Table C96: Treatment Characteristics Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Arvio M, Finland, 2001	14180	3		allogeneic					natural history of disease		
Autti T, Finland, 1999	15540	2	related HLA-identical bone marrow	allogeneic		busulfancyclophosphamideone pt total nodal irradiation			non-HSCTnon-diseased		
Banjar H, Saudi Arabia, 1998	17920	3							pt 1: 60 units/kg every 2 wks, for 3.2 yrspt 2: 30 units/kg every 2 wks for 3.5 yrspt 3: 30 units/kg every 2 wks for 2.5 yrs		
Chan LL, Malaysia, 2002	11330	1							ERT	20 units/kg/dose every 2 wks	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Chen R, Taiwan, 2007	4490	1	unrelated HLA-matched bone marrow	allogeneic	ERT for 3 yrs prior to HSCT During ERT, growth maintained, hepatosplenomegaly resolved, and hematologic and bone density abnormalities resolved.Daily activity functions were deteriorating and intellectual impairment was developing.	busulfancyclophosphamidetecelac	cyclosporinemethotrexate				Elective splenectomy prior to HSCT is standard for Gaucher disease, but was not done on this pt, and no adverse effects of spleen retention was seen.

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Coppa GV, Italy, 1999	16350	1	unrelated bone marrow	allogeneic		busulfancyclophosphamide	cyclosporinmethotrexate				
Ehlert K, Germany, 2006	4690	3	2 bone marrow 1 peripheral blood	allogeneic		busulfanmyeloablative	cyclosporin Amethotrexate with or without anti-thymocyte globulin				
El-Beshlawy A, Egypt, 2006	5750	11			1 pt had splenectomy prior to ERT				ERT intravenously 1-2 hrs every two wks, 60 microgm/kg body weight		
Erikson A, Sweden, 1995	21630	3			pt 1: partial splenectomy at 10.7 yrspt 2: splenectomy and HSCT at 2.1 yrs from donor father, but no engraftment				ERT	pt 1: high dose 2x wkly, at .6 yrs dose halved, at 1.8 yrs dose increasedpt 2: high dose 2x wkly, at .5 yrs dose halved, at 1.3 yrs dose 1/4pt 3: high dose 2x wkly, at .8 yrs dose halved, at 1.8 yrs dose 1/4, at 2.3 yrs dose increased	
Goker-Alpan O, US, 2008	1790	2	bone marrow	allogeneic					ERT only		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Grewel S, US, 2003	9750	1	related HLA-identical bone marrow	allogeneic		cyclophosphamideantithymocyte globulintotal body irradiation	cyclosporin Aprednisone				
Guffon N, France, 2009	680	8	6 HLA-identical related bone marrow 1 HLA-identical unrelated bone marrow 1 mismatched unrelated bone marrow	allogeneic		busulfancyclophosphamidethymoglobulin when donor unrelated	cyclosporin Amethotrexate	ntravenous polyvalent immunoglobulinspenicillinacyclovirtrimethoprim/sulfamethizole			
Hsu YS, Taiwan, 1999	16540	1	related HLA-identical bone marrow	allogeneic		busulfancyclophosphamide	cyclosporinemethotrexate				
Imaizumi M, Japan, 1994	23220A	1	HLS-matched sibling bone marrow	allogeneic		busulfancyclophosphamide	cyclosporine				
Imaizumi M, Japan, 1994	23220B	1	HLA-matched sibling bone marrow (carrier)	allogeneic		busulfancyclophosphamide	cyclosporine				

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Jacobs JFM, Netherlands, 2005	6740	1	unrelated bone marrow	allogeneic		busulfancyclophosphamideantithymocyte globulin	cyclosporin A				substrate reduction therapy started at 1.5 yrs post HSCT
Laitinen A, Finland, 1997	19620	1	related HLA-identical bone marrow	allogeneic		busulfancyclophosphamide					
Lange MC, Brazil, 2006	5690	1	related bone marrow	allogeneic		busulfancyclophosphamide	cyclosporinemethotrexate				
Li P, US, 1996	20260	1	related HLA-identical bone marrow	allogeneic							
Lonnquist T, Finland, 2001	12960	3	two umbilical cord bloodone bone marrow	allogeneic		busulfancyclophosphamideantilymphocyte globulin	cyclosporin A				
Maegawa GHB, Canada, 2009	56590A	3							substrate reduction therapyorally, 100-200 mg t.i.d., adjusted to body surface area		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Maegawa GHB, Canada, 2009	56590B	2							substrate reduction therapyorally 100-200 mg t.i.d. adjusted to body surface area		
Malm G, Sweden, 2004	8490	2	unrelated bone marrow	allogeneic							
McKinnis EJR, US, 1996	20560	1	related HLA-identical bone marrow	allogeneic		busulfancyclophosphamide	methotrexatecyclosporine				
Morel CF, Canada, 2007	3010	1	umbilical cord blood	allogeneic		busulfancyclophosphamide	cyclosporinmethylprednisone				
Muenzer J, US, 2006	57160	96							ERT	placebo, n=32ERT every other week, n=32ERT weekly, n=32	
Muenzer J, US, 2007	57070	12							ERT	4 grps: placebo, ERT 0.15 mg/kg, ERT 0.5 mg/kg, ERT 1.5 mg/kg	
Mullen CA, US, 2000	15300	1	unrelated umbilical cord blood	allogeneic		busulfancyclophosphamideantithymocyte globulinmethylprednisolone	tacolimusmethotrexate				

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Paciorkowski AR, US, 2008	2980	1							substrate reduction therapy	total dose = (body surface area / 1.73) X adult dose40 mg, 3 times/day, oral liquid	
Page KM, US, 2008	1280 A	1	unrelated umbilical cord blood	allogeneic		busulfancyclophosphamideantithymocyte globulin	cyclosporinmethylprednisone	IV immunoglobulinacyclovir variconozole			
Page KM, US, 2008	1280 B	2	unrelated umbilical cord blood	allogeneic		busulfancyclophosphamideantithymocyte globulinmyeloablative conditioning	cyclosporinemethylprednisone	IV immunoglobulinacyclovir voriconazole			
Patterson MC, US, 2007	56970	12							substrate reduction therapy, dose adjusted to body weight		
Patterson MC, US, 2010	56500	10							substrate reduction therapy	median dose: 350 mg/day (range: 100-600 mg/day)median length of exposure: 1073 days (range: 725-1604 days)	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Pineda M, Spain, 2009	56560	66							substrate reduction therapy	mean daily dose (95% CI):<6 yrs: 197.7 (138.0-257.3) mg6-11 yrs: 350.0 (266.0-433.9) mg>=12 yrs: 464.8 (403.8-525.9) mg	
Ringden O, Sweden, 1995	22020	6	4 HLA-matched related bone marrow 1 HLA-mismatched related bone marrow 1 HLA-matched unrelated bone marrow	allogeneic		pts 1, 2: cyclophosphamide and total body irradiationpts 3-6: busulfan and cyclophosphamide	pt 1: cyclosporinepts 2-6: cyclosporine and methotrexate				pt 4 did not engraft and was put on ERT
Ringden O, Sweden, 2006	5940A	2		allogeneic		busulfancyclophosphamide	cyclosporin	reversed isolation			
Ringden O, Sweden, 2006	5940B	1		allogeneic		busulfancyclophosphamide	cyclosporin	reversed isolation			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Schiffman R, Netherlands, 2008	56750	30			29 of 30 receiving ERT simultaneously1 of 30 had HSCT at 13 and 16 yrs and engrafted				substrate reduction therapy combined with ERT (and one HSCT)	Year 1: 21 pts received substrate reduction therapy, 9 received no treatmentYear 2: all pts received substrate reduction therapypts >=12 yrs received adult dosage of 200 mg 3 times/daypts <12 yrs received lower dosages adjusted to body surface area	
Schiffmann R, Netherlands, 1997	58150	5							ERT	dosage adjusted by severity of disease, infusions weekly or every other week	
Seto T, Japan, 2001	13460A	3	related HLA-matched bone marrow	allogeneic							

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Seto T, Japan, 2001	13460B	1	related HLA-matched bone marrow	allogeneic							
Shield JPH, England, 2005	6720	1	related HLA-matched bone marrow	allogeneic							
Sivakumar P, England, 1999	16200	1	related bone marrow	allogeneic					natural history of disease		
Stein J, Israel, 2007	4880	1	unrelated umbilical cord blood	allogeneic		cyclophosphamideantithymocyte globulintotal body irradiation	cyclosporin Amethylprednisone	difluconazoleacyclovirpolymyxingammaglobulin			
Takahashi, Japan, 2001	14030	1	bone marrow	allogeneic							
Tokimasa, Japan, 2008	1310	1	unrelated umbilical cord blood	allogeneic		busulfancyclophosphamidefludarabineanticonvulsantsmesna	methotrexatetacrolimus	laminar air flow roomparenteral nutritionantibioticsheparin			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Tolar J, US, 2009	1370	4	3 unrelated bone marrow 1 unrelated umbilical cord blood	allogeneic		1 cyclophosphamide, antithymocyte globulin, total body irradiation1 cyclophosphamide, total body irradiation 1 busulfan, fludarabine, total body irradiation1 busulfan, cyclophosphamide, antithymocyte globulin					
Tsai P, US, 1992	25120	1	HLA-matched related bone marrow	allogeneic		anti-thymocyte globulinbusulfancyclophosphamide	methotrexate				

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Vellodi A, England, 1999	16650	10	6 related non-identical bone marrow 2 related identical bone marrow 1 unrelated bone marrow 1 unknown bone marrow source	allogeneic		busulfancyclophosphamide	cyclosporinmethotrexate				
Vormoor J, Germany, 2004	9420	2	one related bone marrow, one unrelated peripheral blood	allogeneic		busulfancyclophosphamideantithymocyte globulin	cyclosporinmethotrexate				
Yeager AM, US, 2000	14880	1	related HLA-matched bone marrow	allogeneic		busulfancyclophosphamide	cyclosporin				

Appendix Table C97: Outcome Assessment Treatment Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Arvio M, Finland, 2001	14180	5	intellectual skillsoverall health	dysmorphic features	
Autti T, Finland, 1999	15540	2	MRI findings:cortex-white matter differentiationthalami signal intensity		
Chen R, Taiwan, 2007	4490	1	neuropsychologic scores	enzyme activityneurodevelopmental milestones	
Coppa GV, Italy, 1999	16350	1	enzyme activityneurocognitive scores		
Ehlert K, Germany, 2006	4690	3	number of subcutaneous nodulesnumber of joints with limited range of motion	GVHDinfectionstoxicity	
Goker-Alpan O, US, 2008	1790	2	neuropsychometric assessments		
Grewel S, US, 2003	9750		neuropsychologic scoresneurodevelopmental milestones		
Guffon N, France, 2009	680	8	enzyme activityneuropsychologic scores		
Hsu YS, Taiwan, 1999	16540	1	neuropsychologic scoresneurodevelopmental milestones	MRI findings	
Imaizumi M, Japan, 1994	23220A	1	enzyme activityneuropsychologic measurementsneurodevelopmental measurements		2 yrs
Imaizumi M, Japan, 1994	23220B	1	enzyme activityneuropsychologic measurementsneurodevelopmental measurements		5.6 yrs
Jacobs JFM, Netherlands, 2005	6740	1	enzyme activityMRI findings:cerebral cortical atrophy		
Laitinen A, Finland, 1997	19620	1	identification of gene mutations	enzyme activity	
Lange MC, Brazil, 2006	5690	1	overall survivalGVHD		
Li P, US, 1996	20260	1	enzyme activityneuropsychologic scoresneurodevelopmental milestones		
Lonnquist T, Finland, 2001	12960	3	neuropsychologic scoresenzyme activity	MRI findings:cerebral cortical atrophyperiventricular white matter hyperintensity	neuropsychologic testing every 0.5 yrs
Malm G, Sweden, 2004	8490	2	neuropsychologic scoresenzyme activity		

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
McKinnis EJR, US, 1996	20560	1	neuropsychologic scoresneurodevelopmental milestonesenzyme activity		
Morel CF, Canada, 2007	3010	1	enzyme activityneurologic measurementsneurodevelopmental milestones		
Mullen CA, US, 2000	15300	1	enzyme activity	adverse events	
Paciorkowski AR, US, 2008	2980				
Page KM, US, 2008	1280B	2	event-free survival	GVHDdevelopment of autoimmune cytopenias	
Page KM, US, 2008	1280A	1	event-free survival	GVHDdevelopment of autoimmune cytopenias	
Patterson MC, US, 2007	56970	12			
Ringden O, Sweden, 1995	22020	6	enzyme activityliver sizeskeletal symptomsgrowth		
Ringden O, Sweden, 2006	5940A	2	cumulative overall survivalcumulative treatment-related mortality	cumulative incidence of cGVHD	
Ringden O, Sweden, 2006	5940B	1	cumulative overall survivalcumulative treatment-related mortality	cumulative incidence of cGVHD	
Seto T, Japan, 2001	13460A	10	MRI findings in MPS pts		
Seto T, Japan, 2001	13460B	4	MRI findings in MPS pts		
Shield JPH, England, 2005	6720	1	MRI findingsneurodevelopmental milestones	enzyme activity	
Sivakumar P, England, 1999	16200	2	neuropsychologic scoresneurodevelopmental milestones		
Stein J, Israel, 2007	4880	1	enzyme activityMRI findings		
Takahashi, Japan, 2001	14030	3	magnetic resonance spectroscopy measurement of mucopolysaccharides in the central nervous system		
Tokimasa, Japan, 2008	1310	1	engraftmentGVHD		
Tolar J, US, 2009	1370	1	overall survivalneuropsychologic scores	enzyme activityGVHD	
Tsai P, US, 1992	25120	1	neurocognitive scoresgrowthenzyme activity		
Vellodi A, England, 1999	16650	10	TRMneurocognitive scores		

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration
Vormoor J, Germany, 2004	9420	2	number of subcutaneous nodulesnumber of joints with limited range of motion		
Yeager AM, US, 2000	14880	1	enzyme activityneuropsychologic scores	MRI findingsjoint measurements	

Appendix Table C98: Outcome Assessment Comparator Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Arvio M, Finland, 2001	14180	12				
Autti T, Finland, 1999	15540	6 non-HSCT7 non-diseased				
Banjar H, Saudi Arabia, 1998	17920	3	pulmonary involvement	skeletal changes		
Chan LL, Malaysia, 2002	11330	1	organomegalygrowth			
El-Beshlawy A, Egypt, 2006	5750	11	skeletal changes			
Erikson A, Sweden, 1995	21630	3	neuropsychologic scoresglucosylceramide levels (lower is better)	growth		
Goker-Alpan O, US, 2008	1790	30	neuropsychometric assessments			
Maegawa GHB, Canada, 2009	56590B	2	neurological assessmentsneuropsychological tests, 2 types depending on severity of impairment		neurological assessments at baseline and every 3 mosneuropsychological tests at baseline and every 6 mos	
Maegawa GHB, Canada, 2009	56590A	3	neurological assessmentsneuropsychological tests, 2 types depending on severity of impairment		neurological assessments at baseline and every 3 mosneuropsychological tests at baseline and every 6 mos	
Malm G, Sweden, 2004	8490	2				
Muenzer J, US, 2006	57160	96	6-minute walk testforced vital capacity		baseline, 18 wks, 36 wks, 53 wks	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Muenzer J, US, 2007	57070	12	change in urinary glucosaminoglycans	liver and spleen size6-minute walk testpulmonary functionjoint mobilityheart size and functionsleep study	baseline, wk 13, wk 24, wk 25, wk 51	
Paciorkowski AR, US, 2008	2980	1	gait analysisneurologic examsgrowth parameters		gait every 6 mosneurologic exams every 3 mos	
Patterson MC, US, 2007	56970	12	change in horizontal saccadic eye movement-alpha (HSEM-alpha)	mini-mental status examinationambulatory indexdifficulty in swallowing		HSEM-alpha is an indicator of disease severity
Patterson MC, US, 2010	56500	10	horizontal saccadic eye movement (correlates well with disease progression)	neurological assessmentsswallowingambulation		
Pineda M, Spain, 2009	56560	66	4 disease disability scales: ambulation, manipulation, language, swallowing (the lower the score, the better)		at diagnosis, at start of treatment, last clinical contact	Psychiatric impairment was not part of the disability scales because most psychiatric impairment in this disease starts in adolescence or adulthood.
Schiffman R, Netherlands, 2008	56750	30	change in vertical saccadic eye movement velocity (VSEM)	neurological assessmentspulmonary functionliver and spleen volumehematological assessmentssafety evaluations		VSEM chosen end point because supranuclear gaze palsy is the only universal neurological symptom of Gaucher Type 3

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Comment
Schiffmann R, Netherlands, 1997	58150	5	neurocognitive scoreslumbar puncture (3 of 5 pts)		lumbar puncture every 3-6 mos for 3 yrs	
Seto T, Japan, 2001	13460A	7	MRI findings in MPS pts			
Seto T, Japan, 2001	13460B	3	MRI findings in MPS pts			
Sivakumar P, England, 1999	16200	1	neuropsychologic scoresneurodevelopmental milestones			
Takahashi, Japan, 2001	14030	2	magnetic resonance spectroscopy measurement of mucopolysaccharides in the central nervous system			

Appendix Table C99: Time to Event Outcomes Treatment Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Comment
Arvio M, Finland, 2001	14180	3	alive:pt 1: 7.6 yrspt 2: 5.4 yrspt 3: 1.8 yrs	
Autti T, Finland, 1999	15540	2	alive:pt 1: 7 yrspt 2: 4 yrs	
Banjar H, Saudi Arabia, 1998	17920			
Chan LL, Malaysia, 2002	11330			
Chen R, Taiwan, 2007	4490	1	alive at 1.5 yrs post	
Coppa GV, Italy, 1999	16350	1	alive at 4 yrs post	
Ehlert K, Germany, 2006	4690		alive:pt 1: 1.2 yrspt 2: 0.5 yrspt 3: 0.7 yrs	
El-Beshlawy A, Egypt, 2006	5750			
Erikson A, Sweden, 1995	21630			
Goker-Alpan O, US, 2008	1790	2	alive at 19-21 yrs	
Grewel S, US, 2003	9750	1	alive at 5 yrs post	
Guffon N, France, 2009	680	8	7 alive at 12.7 yrs avg post1 dead at 6.1 yrs post	cause of death unrelated to transplant
Hsu YS, Taiwan, 1999	16540	1	alive at 0.8 yrs post	
Imaizumi M, Japan, 1994	23220A	1	alive at 2 yrs	
Imaizumi M, Japan, 1994	23220B	1	dead at 5.6 yrs follow-up	died of natural progression of disease
Jacobs JFM, Netherlands, 2005	6740	1	alive at 2.0 yrs post	
Laitinen A, Finland, 1997	19620	1	alive at 4 mos post	
Lange MC, Brazil, 2006	5690	1	alive at 3.3-14.2 yrs post (for total study pop of 8)	follow-up time for single MPS III pt not given
Li P, US, 1996	20260	1	alive at 5.0 yrs post	
Lonnquist T, Finland, 2001	12960	3	alive:pt 1: 4 yrspt 2: 3 yrspt 3: 2 yrs	
Maegawa GHB, Canada, 2009	56590A			
Maegawa GHB, Canada, 2009	56590B			
Malm G, Sweden, 2004	8490	2	alive:pt 1: 5 yrspt 2: 5 yrs	
McKinnis EJR, US, 1996	20560	1	alive at 5.6 yrs post	
Morel CF, Canada, 2007	3010		alive at 2.7 yrs post	
Muenzer J, US, 2006	57160			
Muenzer J, US, 2007	57070			
Mullen CA, US, 2000	15300		alive at 2.2 yrs	
Paciorkowski AR, US, 2008	2980			
Page KM, US, 2008	1280A	1	dead at 4.6 yrs post	cause of death unknown, probably infection
Page KM, US, 2008	1280B	1	1 alive at 5.1 yrs post1 dead at 1.8 yrs post	cause of death: multi-system organ failure
Patterson MC, US, 2007	56970			

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	Comment
Patterson MC, US, 2010	56500			
Pineda M, Spain, 2009	56560			
Ringden O, Sweden, 1995	22020	6	6 alive, 5-11 yrs follow-up	
Ringden O, Sweden, 2006	5940A	2	1 alive at 14 yrs follow-up (Type C), without engraftment1 dead at 0.4 yrs post (Type A)	Cause of death: pneumonia
Ringden O, Sweden, 2006	5940B	1	1 dead, unknown follow-up	Cause of death: progressive disease
Schiffman R, Netherlands, 2008	56750			
Schiffmann R, Netherlands, 1997	58150			
Seto T, Japan, 2001	13460A	3	3 alive, 1 at 3 yrs post, 1 at 8 yrs post, 1 unknown follow-up	
Seto T, Japan, 2001	13460B	1	alive, unknown follow-up	
Shield JPH, England, 2005	6720		alive at 7 yrs post	
Sivakumar P, England, 1999	16200	1	alive at 7.4 yrs post	
Stein J, Israel, 2007	4880	1	alive at 4 yrs post	
Takahashi, Japan, 2001	14030	1	alive at 1.1 yrs post	
Tokimasa, Japan, 2008	1310	1	dead at 0.8 yrs post	cause of death: post-transplant lymphoproliferative disorder
Tolar J, US, 2009	1370	4	2 dead at 0.2 and 0.7 yrs post2 alive at 4 and 11 yrs post	
Tsai P, US, 1992	25120	1	deat at 2 yrs post	s. pneumoniae sepsis
Vellodi A, England, 1999	16650	10	2 alive at 7-14 yrs post1 dead 11.8 yrs post from natural progression of disease4 dead <100 days post, 2 from aGVHD, 2 from sepsis1 dead 4 yrs post from bronchiolitis1 dead unknown follow-up of GVHD	Authors attribute high mortality to poor donor selection.
Vormoor J, Germany, 2004	9420	2	alive:pt 1: 1.2 yrspt2: 0.9 yrs	
Yeager AM, US, 2000	14880	1	dead at 2.3 yrs post	cause of death: pulmonary failure after aspiration pneumonitis (disease-related, not treatment-related)

Appendix Table C100: Time to Event Outcomes Comparator Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Outcome	1 yr	2 yr	3 yr	4 yr	5 yr	Outcome_2	Med (mos)_2	3 yr_2	5 yr_2
Abu-Ghosh 2002 USA	45610	11		~73 %	63.6 +/- 14.5 %	63.6 +/- 14.5 %	63.6 +/- 14.5 %	63.6 +/- 14.5 %	PFS		63.6 +/- 14.5 %	
Park, Korea, 2006	5450	7	DOD n=5 median 15 mos (2-30 mos) A NED n=1 20+ mos A with D n=1 130+ mos						EFS	median 8 months (2-20 mos)		
Tucci, Brazil, 2007	3910	10				83.3 %		42.8 %	DFS		66.6 %	42.8 %

Appendix Table C101: Neurocognitive/neuropsychological Outcomes Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Arvio M, Finland, 2001	14180	3 HSCT 12 non-HSCT			3 HSCT: developmental age was on average 5 yrs lower than real age 12 non-HSCT: developmental age was on average 3.4 yrs lower than real age						
Autti T, Finland, 1999	15540	2 HSCT		both HSCT pts: gross motor clumsiness, slight balance problems			2 HSCT		frequent respiratory and ear infections	no reports of respiratory and ear infections	
Banjar H, Saudi Arabia, 1998	17920	3			No changes in skeletal symptoms were found.		3		all 3 have diffuse reticular pattern on chest x-rays	2 improved and 1 had no change in lung involvement	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Chan LL, Malaysia, 2002	11330	1		height <3rd percentile	improved growth		1		spleen volume: 1592 cu cmliver: 3 cm below coastal marginmild anemiathrombocytopenia	spleen volume: 856 cu cmliver: 2 cm below coastal marginanemia correctedthrombocytopenia corrected	
Chen R, Taiwan, 2007	4490	1			sAppendix Table 9growth and improved bone density						
Coppa GV, Italy, 1999	16350	1		significant joint limitations in hands, knees, elbowssensorineural hearing loss	mild joint limitations at 0.7 yrs postminimal joint limitations at 2.6 yrs post		1		hepatosplenomegalymild mitral and tricuspid insufficiency	hepatosplenomegaly resolved 2.6 yrs postslight improvements in valve abnormalities at 2.6 yrs post	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Ehlert K, Germany, 2006	4690	3		no. subcutaneous nodules:pt 1: 58pt 2: 39pt 3: 18no. joints with limited motion:pt 1: 26pt 2: 24pt 3: 10	no. subcutaneous nodules:pt 1: 8 at 1.2 yrs postpt 2: 14 at 0.5 yrs postpt 3: 0 at 0.7 yrs postno. joints with limited motion:pt 1: 2 at 1.2 yrs postpt 2: 4 at 0.5 yrs postpt 3: 4 at 0.7 yrs post	all 3 pts showed improvement in mobility, less pain, and considerable gain in function					
El-Beshlawy A, Egypt, 2006	5750	11		Grading severity level of marrow involvement:0A level: 3 pts, 2A level: 6 pts, 3A level: 1 pt, 3B level: 1 pt	0A level:0A level: 1 constant and 2 worsened, 2A level: 5 complete improvement and 1 constant, 3A level: 1 constant, 3B level: 1 constant	3B pt had prior splenectomy	11	radiography of both femora	7 no abnormal osseous changes2 single lesions2 complex lesion	7 no abnormal osseous changes remained same2 single lesions had complete improvement 2 complex lesions had 1 remain same and 1 partial improvement	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Erikson A, Sweden, 1995	21630	3	stunted growthskeletal deformities	pt 2: grew 2 cm/yrpt 3: grew 4 cm/yr	pt 2: grew 9 cm 1 yr postpt 3: grew 12 cm 1 yr post	pt 1 had femur deformity, kyphosis, cortex thinning and pt 3 had femur deformity; no change in skeletal deformities found	3	liver sizespleen size	liver: pt 1: 4.3% body wt, pt 2: 6.2% body wt, pt 3: 8.3% body wtspleen:pt 1: 4.6% body wt, pt 2: splenectomy, pt 3: 14.6% body wt	iver: pt 1: 2.7% at 2.1 yrs, pt 2: 3.6% at 2 yrs, pt 3: 4.3% at 1.9 yrsspleen:pt 1: 1.0% at 2.1 yrs, pt 2: splenectomy, pt 3: 3.3% at 1.9 yrs	
Goker-Alpan O, US, 2008	1790	2		bone abnormalities in 1 pt (50%)	bone abnormalities sAppendix Table 9	or whole grp, 2 HSCT followed by ERT, and 30 ERT only: 100% slowing of horizontal saccadic eye movement					

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Grewel S, US, 2003	9750	1		real age: 1.4 yrsdevelopmental age: 0.9 yrs	real age: 3.0 yrs, gross motor age: 1.2 yrsreal age: 3.5 yrs, gross motor age: 1.3 yrsreal age: 4.7 yrs, gross motor age: 1.5 yrsreal age: 5.7 yrs, gross motor age: 1.5 yrsreal age: 6.7 yrs, gross motor age: 1.5 yrs	gross motor skills impairedfine motor skills slowly growing	1		echocardiograph showed trivial aortic insufficiencyfrequent respiratory infections	echocardiograph showed no further progression of cardiac symptomsno respiratory infections during follow-up	surgery for cataracts, bilateral carpal tunnel, 8 trigger digit releases multiple dental extractionsinsertion of bilateral ear tubes
Guffon N, France, 2009	680	8			8 showed improvement in joint stiffness2 no kyphosis5 mild kyphosis1 severe kyphosis2 no carpal tunnel syndrome 6 carpal tunnel syndrome requiring surgery		8		8 valvular abnormalities detected by echocardiography5 hearing problems3 no hearing problems8 hepatosplenomegaly	cardiovascular abnormalities stabilized1 with hearing problems improved7 hearing remain same8 hepatosplenomegaly resolved in 3 mos post	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Hsu YS, Taiwan, 1999	16540	1		1.2 yrs: sat without support and crawled2.4 yrs: became bed-ridden during conditioning phase			1		frequent respiratory infectionshepatosplenomegalylipid-filled foamy cells among hematopoietic cells	chest CT at 0.5 and 0.8 yrs post show resolution of lung infiltrateshepatosplenomegaly resolved at 0.5 yrs postnormal cellular marrow with no foamy cells at 0.5 yrs post	
Imaizumi M, Japan, 1994	23220A	1		moderate to severe joint contracturesnodular hypertrophy present	improved joint contracturesnodular hypertrophy absent		1		cardiac valvular thicknesshepatomegaly at 12 cmmoderate hearing loss	no change in cardiac valvular thicknesshepatomegaly at 4 cmno change in hearing loss	
Imaizumi M, Japan, 1994	23220B	1		moderate to severe joint contracturesmarked short staturedystosis multiplex present	no change in joint contracturesmarked short staturedystosis multiplex still present		1		high dependence on respirator and frequent infectionsleft ventricular hypertrophymild corneal cloudinesshepatomegaly at 6 cm	low dependence on respirator and less frequent infectionsleft ventricular hypertrophy samemild corneal cloudinesshepatomegaly at 0 cm	at 5 yrs, infections began increasing again and at 5.6 yrs post transplant, pt died of pneumonia

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Jacobs JFM, Netherlands, 2005	6740	1			motor skills deteriorating at 0.5 yrs post	Deterioration of this pt similar to deterioration of untreated older sister.	1			opthalmological deterioration at 1.5 yrs post	
Laitinen A, Finland, 1997	19620	1					1		mild hepatomegalyrecurrent respiratory infections	clinically well	
Li P, US, 1996	20260	1		multiple bone abnormalities	improvements in joint range of motionimprovements in fine and gross motor skills		1		hepatosplenomegalycardiac enlargement with normal function	hepatosplenomegaly resolvedcardiac status unimproved	
Lonnquist T, Finland, 2001	12960	3		one pt mildly symptomatic and two pts asymptomatic	all three pts by end of follow-up at 2-4 yrs of age were hypotonic and spastic, with some head control remaining		3		no optic atrophy or retinopathy	optic atrophy: development of one severe and one mildretinopathy: development of one mild	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Maegawa GHB, Canada, 2009	56590A	3		pt 1: muscle wasting, fully dependent for feeding and ambulation pt 2: moderate skeletal muscle weakness, independent ambulation, feeding, bathingpt 3: independent ambulation, feeding, and bathing	pt 1: 3 mos incoordination progressed, 15 mos wheelchair, 21 mos can't standpt 2: at 18 mos gait disturbance progressed & muscle strength reducedpt 3: 6 mos gait disturbance, 16 mos noAppendix Table 9wt loss	pt 2 and pt 3 stopped tx at 21 mos due to excessive weight loss					

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Maegawa GHB, Canada, 2009	56590B	2		pt 1: mild muscle weakness, moderate muscle impairment, independent feeding and ambulation pt 2: needs support for ambulation	pt 1: at 6 mos handwriting deteriorated, at 12 mos fine tremor in hands, from 12-24 mos, progressive muscle atropypt 2: at 15 mos muscle bulk decreased markedly, at 24 mos wheelchair dependent						
Malm G, Sweden, 2004	8490	2			pt 1: can walk, ride bike, dress selfpt 2: can walk, ride bike, drive tractor, some fine motor skills						

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
McKinnis EJR, US, 1996	20560	1		real age: 1.9 yrsdevelopmental age: 1.3-1.5 yrs	persistent skeletal deformities reversion in balance and coordination though can still walk and ride tricycle		1		hearing deficitshepatomegaly	hearing deficits persist, but have not progressedhepatomegaly resolved	
Morel CF, Canada, 2007	3010	1			alert, active, interactive, rolling back to front to back at 0.6 yrs posthead lag and hypotonic at 1 yr postsignificant developmental delay, limited social interaction, unable to sit or stand at 1.7 yrs post		1		hepatosplenomegaly	splenomegaly resolvedcherry red spots and worsening vision at 0.6 yrs postrecurrent respiratory infectionsfailure to thrivegastronomy feeding at 1.3 yrs postsleep apnea at 1.7 yrs postexclusively g-tube fed at 2.3 yrs post	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Muenzer J, US, 2006	57160	96	6-minute walk test, meters	placebo: 392 +/- 19ERT EOW: 401 +/- 18ERT wkly: 392 +/- 19	Changes in 6-minute walk test:placebo: 7.3 +/- 9.5ERT EOW: 30.3 +/- 10.3 (p=0.07)ERT wkly: 44.3 +/- 12.3 (p=0.01)		96	Forced vital capacity (L)	placebo: 1.09 +/- 0.09ERT EOW: 1.17 +/- 0.10ERT wkly: 1.19 +/- 0.10	Changes in forced vital capacity (p-value):placebo: 0.06 +/- 0.03ERT EOW: 0.07 +/- 0.03 (p=0.37)ERT wkly: 0.22 +/- 0.05 (p=0.001)	Liver volume % change: placebo: -0.8 +/- 1.6ERT EOW: -24.0 +/- 1.7 (p<0.0001)ERT wkly: -25.3 +/- 1.6 (p<0.0001)Spleen volume % change: placebo: 7.2 +/- 4.2ERT EOW: -19.8 +/- 3.2 (p<0.0001)ERT wkly: -25.1 +/- 2.4 (p<0.0001)

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Muenzer J, US, 2007	57070	12	6-minute walk test, meters	placebo: 374.7ERT .15 mg/kg: 448.7ERT .5 mg/kg: 324.3ERT 1.5 mg/kg: 439.7	6 mos: no change12 mos: 8 improved, 4 no change	pooled 6-minute walk test, including placebo which received ERT after 6 mos:baseline: 398 +/- 1171 yr: 445 +/- 124 (p=0.013)	12		12 hepatosplenomegaly	pooled 1 yr: 11 reduced liver and spleen size, changes in size not dose-related	forced vital capacity: pooled 1 yr data did not show significant change, measurements difficult and unreliable sleep study:6 of 7 pts eligible experienced decrease in O2 desaturation events/hr (from 19.2 to 2.4)
Mullen CA, US, 2000	15300	1			growing and developing normally		1		hepatomegaly	hepatomegaly resolved	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Paciorkowski AR, US, 2008	2980	1		proximal weakness in extremities ataxic hand tremoratax ic gaitmotion analysis: walked 0.24 m/sec, 62 steps/min	3 mos: hand tremor diminished 9-12 mos: lost ability to walkmotio n analysis at 6 mos: walked 0.12 m/sec, 32.4 steps/min		1		splenomegaly	unchanged splenomegal y	
Patterson MC, US, 2007	56970	12	horizonta l saccadic eye moveme nt-alpha		mean decrease of -0.465 ms/degp= 0.028 for whole grp (including >=12 yr grp)	improve ment in ambulati on seen for whole grp including pts >=12:p= 0.052					

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Patterson MC, US, 2010	56500	10	Standard Ambulation Index	2.0 (0.7-3.3)	1 yr: 2.3 (0.6-4.0)2 yrs: 2.6 (0.7-4.5)	8 of 10 pts are considered sAppendix Table 9in ambulation	9	Horizontal Saccadic Eye Movement, alpha and beta	HSEM alpha mean (95% CI): 2.181 (1.3-3.0)HSEM beta mean (95% CI): 28.96 (13.9-44.0)	1 yr HSEM alpha mean (95% CI): 1.692 (1.0-2.4)2 yr HSEM alpha mean (95% CI): 2.106 (1.3-2.9)1 yr HSEM beta mean (95% CI): 33.66 (18.3-49.0)2 yr HSEM beta mean (95% CI): 33.47 (17.9-49.1)	no improvement, but overall stability of disease
Pineda M, Spain, 2009	56560	57	Disability scale component scores	at diagnosis: ambulation: 0.18 (0.16,0.20) manipulation: 0.27 (0.24,0.30) language: 0.16 (0.14,0.18) swallowing: 0.12 (0.10,0.15) at start of treatment: overall deterioration of scores	at last clinical visit, % with sAppendix Table 9/improved scores:ambulation: 76.6%manipulation: 76.2%language: 77.0%swallowing: 81.0%		57	Annual change in composite score, by age grp		<6 yrs: -0.070 (-0.275,0.136) 6-11 yrs: -0.0157 (-0.394,0.080) >=12 yrs: -0.162 (-0.329,0.006)	most improvement seen in older grps (6-11 yrs and >=12 yrs)

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Ringden O, Sweden, 1995	22020	6		pts 1, 5, 6: kyphosis pts 2, 3, 4: no kyphosis	pts 1, 5, 6: kyphosis pts 2, 3, 4: no kyphosis all experienced growth spurt	pt 4 who did not engraft and is on ERT, has decreased motor skills	6		hepatomegaly	hepatomegaly resolved	
Schiffman R, Netherlands, 2008	56750	26 (4 withdrew during extension phase of study)	vertical saccadic eye movement (VSEM)		No treatment effect on VSEM.	Study may not have been long enough for neurological defects to improve, or neurological defects are irreversible.	30	forced vital capacity	substrate reduction therapy: 75.1 (62.0-88.2)no treatment: 79.5 (61.3-97.6)	81.1 (65.7-96.5)no treatment: 81.3 (62.7-99.9)	No difference between groups detected, but in subgroup analysis on pts with abnormal forced vital capacity, improvement was seen with substrate reduction therapy.

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Seto T, Japan, 2001	13460B	1 HSCT, 3 non-HSCT		HSCT pt: mild bone deformities	HSCT pt: no follow-up post HSCT	3 non-HSCT pts:#1: bone deformities 1 yr, overflexion of neck becoming quadriplegic 9 yrs#2: decreased tendon reflexes 14 yrs#3: increased deep tendon reflexes 4 yrs					
Shield JPH, England, 2005	6720	1			walking at 0.6 yrs postbecame clumsy at 1.7-2.1 yrs postlimited motor skills at 4.0 yrs postwheel chair at 6.0 yrs post						

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Sivakumar P, England, 1999	16200	2: 1 HSCT, 1 non-HSCT			HSCT pt: immobile at 7.4 yrs postnon-HSCT pt: immobile at 10 yrs of age		2: 1 HSCT, 1 non-HSCT			both treated and untreated pts have swallowing dysfunction	
Stein J, Israel, 2007	4880	1		weight, height, and head circumference at <3rd percentile	at 4 yrs post:weight 10th percentileheight 3rd percentilehead circumference 3rd percentile		1		hepatosplenomegaly	hepatosplenomegaly resolved by 0.5 yrs post	
Tokimasa, Japan, 2008	1310										
Tolar J, US, 2009	1370	4		pt 1: considerable developmental delay	pt 1 at 11 yrs post: motor function improvedpt 4 at 4 yrs post: fine motor skills below avg, gross motor skills avg		4		pt 1: hepatosplenomegaly	pt 1: hepatosplenomegaly resolved	

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Tsai P, US, 1992	25120	1		failure to thrive, height < 3rd percentile	height 10th percentile at 9 mos postheight 50th percentile at 2 yrs post		1	expected liver volume based on body wt	300%	225% at .2 yrs post136% at .7 yrs post116% at 1.1 yrs post125% at 2 yrs post	
Vellodi A, England, 1999	16650	3		Griffiths Mental Development Scale:pt 1: locomotor 63, eye-hand 58pt 2: locomotor 55, eye-hand 58pt 3: locomotor 110, eye-hand 93	Griffiths Mental Development Scale:pt 1 at 10 yrs post: locomotor 11, eye-hand 8pt 2 at 2.7 yrs post: locomotor 6.5, eye-hand 2.5	significant neurodevelopmental decline in pts 1 and 2					
Vormoor J, Germany, 2004	9420	2		no. subcutaneous nodules:pt 1: 58pt 2: 39no. joints with limited range of motion:pt 1: 26pt 2: 24	no. subcutaneous nodules:pt 1: 8pt 2: 12no. joints with limited range of motion:pt 1: 2pt 2: 2	dramatic improvement in motor activity	2				

Study (Investigator, country, record#)	Record Number	Group (N) (NDP)	Normal Level (NDP)	Pre-Transplant (NDP)	Post-Transplant (NDP)	Comments (NDP)	Group (N) (OOI)	Normal Level (OOI)	Pre-Transplant (OOI)	Post-Transplant (OOI)	Comments (OOI)
Yeager AM, US, 2000	14880	1		wt, ht, and head circumference:10th-25th percentile	wt, ht, and head circumference:5th percentile at 0.8 yrs post<5th percentile at 1.5 yrs post		1		subcutaneous nodules present	subcutaneous nodules:nodules resolved 0.1 yrs postincreased joint range of motion at 1.5 yrs postswallowing:no swallowing dysfunction at 0.7 yrs postsevere gastroesophageal reflux at 1.3 yrs post	

Appendix Table C101: Neurocognitive/neuropsychological Outcomes Inherited Metabolic Diseases Continued

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Arvio M, Finland, 2001	14180						3 HSCT 12 non-HSCT				Dysmorphic Facial and Body Features remained unchanged following HSCT
Autti T, Finland, 1999	15540	2 HSCT			2 HSCT: reached heterozygous activity level		2 HSCT, 6 non-HSCT		2 HSCT:poor cortex-white matter differentiationdecreased thalami signal intensity	2 HSCT:decline from poor to evident cortex-white matter differentiation improvement in thalami signal intensityimprovement in concentration and cooperation6 non-HSCT:poor cortex-white matter differentiation decreased thalami signal intensity	True clinical effect of HSCT will not be seen until pts reach puberty, which is when rapid mental decline usually occurs with aspartylglucosaminuria.

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Chan LL, Malaysia, 2002	11330						1				Behavioral and learning difficulties developed after stopping ERT. Recurrent seizures occurred 2.6 yrs after stopping ERT.
Chen R, Taiwan, 2007	4490	1	26 +/- 5 nmol/h/mg protein	1.2 nmol/h/mg protein	16.2, 22.7 nmol/h/mg protein at 0.5 yrs, 0.8 yrs post		1		Weschler Intelligence Scales:performance: 67verbal: 69complete: 67	at 1.5 yrs post, Weschler Intelligence Scales:performance: 60verbal: 69complete: 62	
Coppa GV, Italy, 1999	16350	1	2.6-20.4 U/mg	0.2 U/mg	2.3, 1.0, and 3.3 U/mg at 0.25 yrs, 1.6 yrs, and 3.6 yrs post		1		IQ: 72	IQ: 69, 70, and 70 at 0.7 yrs, 2.6 yrs, and 4.0 yrs post	attends kindergarten, sociable, can speak simple sentences, writes a few letters
Ehlert K, Germany, 2006	4690										
El-Beshlawy A, Egypt, 2006	5750	11	1-5 micromol/hr/gm protein	mean: 0.4 +/- 0.3 micromol/hr/gm proteinrange: 0.0-0.9 microml/hr/gm protein		measurements for whole grp, Gaucher Type 1 and 3 combined					

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Erikson A, Sweden, 1995	21630	3	level of accumulated glucosylceramide in plasma (micromol/liter plasma) (lower is better)	pt 1: 15pt 2: 21pt 3: 13	pt 1: 8,10,8,9,8,9,10 at 3,6,9,12,13,15,18 mospt 2: 11,6,11,12,11,13,12 at 3,6,9,12,15,18,21 mospt 3: 11,5,8,7,7,7,8 at 3,6,9,12,18,21,23 mos	normal levels (5-10) were reached by those with intact spleen	3	EEGWechsler Intelligence Scale and Griffith mental development scale	EEG normalpt 1: 82-88 on Wechsler Scalept 2: 82-88 on Griffith Scalept 3: 104-111 on Griffith Scale	EEG normalpt 1: 89-96 on Wechsler Scale at 1.3 yrs postpt 2: 74-81 on Griffith Scale at 1 yr postpt 3: 97-103 on Griffith Scale at 1 yr post	all 3 pts became more active and needed less sleeppts 2 and 3 were tired and slow and became active pre-schooler post treatment
Goker-Alpan O, US, 2008	1790						2		nr	borderline mental retardation	for whole grp, 2 HSCT followed by ERT, and 30 ERT only: 12.5% cognitive and neurological decline

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Grewel S, US, 2003	9750	1		6.5% of control	20-24% of control at 0.2 yrs, 0.3 yrs, and 0.5 yrs post		1		real age: 1.4 yrsdevelopmental age, expressive language, and receptive language: 0.9 yrs	real age: 3.0 yrs, developmental age: 1.6 yrsreal age: 3.5 yrs, developmental age: 2.1 yrsreal age: 4.7, developmental age: 3.3 yrsreal age: 5.7 yrs, developmental age: 4.3 yrsreal age: 6.7 yrs, developmental age: 5.3 yrs	attends school with individualized education program; slow progress in communication, daily living, socialization, and expressive language; mild to moderate cognitive impairment
Guffon N, France, 2009	680	8		<= 1% of day control	at latest evaluation: 6 100% of day control1 57% of day control1 50% of day control	The two pts at <100% enzyme activity had carrier donors.	8		IQ/DQ:pt 1: 125pt 2: 72pt 3: 87pt 4: 70pt 5: 70pt 6: 65pt 7: 100pt 8: 100	IQ/DQ:pt 1: 110, normal languatept 2: 60, very poor languagept 3: 65, poor languagept 4: <50, no languagept 5: <50, speech loss 3 yrs postpt 6:<50, speech loss 8 yrs post, pt 7: 100, normal languagept 8: <50, poor language	2 attend normal schools5 attend special schools1 attends special apprenticeship3 very poor social adjustment2 poor social adjustment2 good social adjustment1 very good social adjustment

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Hsu YS, Taiwan, 1999	16540	1		40% of normal control			1		real age: 2.4 yrsdevelopmental age: 0.8-1.2 yrs	developmental age decreasing steadily:real age: 2.6 yrs, developmental age: 0.4-0.7 mosreal age: 2.9 yrs, developmental age: 0.3-0.4 yrsreal age: 3.3 yrs, developmental age: 0.2-0.3 yrs	MRI pre-transplant showed normal myelination and no obvious brain atrophyMRI 0.5 yrs post-transplant showed normal myelination and evident brain atrophy
Imaizumi M, Japan, 1994	23220A	1		4% of normal controls	34.9% of normal controls		1		no CNS involvement, attends normal school	no change	
Imaizumi M, Japan, 1994	23220B	1		not detecAppendix Table 9	63.3% of normal control		1		severe psychomotor retardation	severe, gained development of 4-8 month old: sit by self, use walker, exhibited emotional expressions	

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Jacobs JFM, Netherlands, 2005	6740	1		44-50 nmol/h/mg protein	919, 728, 133, 115, 126, and 128 at 0.1 yrs, 0.5 yrs, 1.25 yrs, 1.3 yrs, 1.6 yrs, and 1.9 yrs post		1		MRI shows cerebral atrophy at 0.5 yrs postworsening neuropsychological tests at 0.5 yrs postspeech deterioratin g at 0.5 yrs post		Deterioration of this pt similar to deterioration of untreated older sister.
Laitinen A, Finland, 1997	19620	1		0% of normal control	30.8%, 19.0%, 21.0% of normal control at 6 wks, 3 mos, 4 mos		1		mild global delay		
Lange MC, Brazil, 2006	5690						1			no significant neuropsychological improvement	
Li P, US, 1996	20260	1	4-18 cpm x 1000/hr/mg protein	0 cpm x 1000/hr/mg protein	0.3, 2.5, 2.4 cpm x 1000/hr/mg protein at 1.0, 3.0, 4.0 yrs post		i		IQ: 44	IQ: 44 at 3 yrs post	

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Lonnquist T, Finland, 2001	12960	3	in leukocytes: 24-100 nmol/h/mgin cerebrospinal fluid: 8-24 nmol/h/mg	in leukocytes in all 3 pts: decreasedin cerebrospinal fluid in 1 pt: decreased	in leukocytes in all 3 pts: normalin cerebrospinal fluid: 1 normal, 2 decreased		3			cerebral cortical atrophy: from moderate to severe in one pt, from not detecAppendix Table 9to moderate in two pts periventricular white matter hyperintensity: from mild to severe in one pt, from not detecAppendix Table 9to moderate in two pts	

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Maegawa GHB, Canada, 2009	56590A						3		pt 1: severe cognitive dysfunction, hallucinations, agitation, scores 1.5 yrs below agept 2: episodic psychosis, cognitive function well-preserved, works part timept 3: 2 episodes of psychosis, IQ=75	pt 1: neuropsych scores unchangedpt 2: 18 mos post, neuropsych scores sAppendix Table 9, speech less intelligible, hallucinations reduced, anxiety ongoingpt 3: at 16 mos post, spasticity developed, anxiety aggravated, neuropsych scores sAppendix Table 9	
Maegawa GHB, Canada, 2009	56590B						2		pt 1: mild cognitive impairment, attends regular school with assistancept 2: severe cognitive impairment, generalized seizures	pt 1: at 15 mos acute psychotic eventpt 2: at 15 mos marked increase in seizures, alertness deteriorated, at 24 mos spasticity increased	

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Malm G, Sweden, 2004	8490	2	0.8-2.2 microkat /kg	pt 1: 0.10 microkat /kgpt 2: 0.09 microkat /kg	pt 1: 0.98, 1.10 at 3 mos, 60 mospt 2: 1.59, 0.80 at 3 mos, 60 mos		2		pt 1: developmental age 4.7 yrs below real age	pt 1 and 2: developmental age stabilizing at 5 yrs over time	pt 1 and 2: mentally retarded, speaks in sentences, understands Swedish and Finnish words
McKinnis EJR, US, 1996	20560	1	23-45 units/mg protein	undetec Appendix Table 9	38 units/mg protein		1			intelligence ratio (age equivalent/real age): 0.68, 0.51, 0.48, 0.54, 0.50, 0.42, 0.29, 0.17, 0.12, 0.09 at 2.8, 3.3, 3.4, 3.9, 4.2, 5.0, 5.9, 6.0, 6.9, 8.0 yrs of age	Along with decreasing intelligence ratio, pt went from mild behavioral difficulties pre-transplant to increased behavioral problems post-transplant. Reversion in language, communication, concentration, cooperation, and attention span also seen.
Morel CF, Canada, 2007	3010	1	0.6-1.8 nmol/h/mg protein	0.1	5.7, 1.3, 8.4, 2.2, 1.4, 0.6, 1.7 at 0.2 yrs, 0.4 yrs, 0.5 yrs, 0.6 yrs, 1 yr, 1.3 yrs, 2.7 yrs post		1		neurologically intact	neurological regression:bilateral cerebral atrophy at 0.6 yrs postseizure disorder developed at 1.7 yrs post	

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Muenzer J, US, 2006	57160	96	Urine GAG levels (in micrograms/mg creatinine)	placebo: 419 +/- 34ERT EOW: 338 +/- 21ERT wkly: 326 +/- 26	Percent change (p-value):placebo: 21.4 +/- 11.6ERT EOW: -44.7 +/- 4.0 (p<0.0001)ERT wkly: -52.5 +/- 5.3 (p<0.0001)						
Muenzer J, US, 2007	57070	12	Urine GAG, in micrograms/mg creatinine	placebo: 371.3ERT .15 mg/kg: 386 +/- 124ERT .5 mg/kg: 364+/- 50ERT 1.5: 445 +/- 101	6 mos post:ERT .15 mg/kg: 230 +/- 76ERT .5 mg/kg: 211 +/- 110ERT 1.5: 168 +/- 611 yr post:ERT .15 mg/kg: 203 +/- 55ERT .5 mg/kg: 209 +/- 98ERT 1.5 mg/kg: 178 +/- 32	pooled urine GAG measurements, including placebo which received ERT after 6 mos:baseline: 398 +/- 946 mos: 203 +/- 821 yr: 200 +/- 18					

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Mullen CA, US, 2000	15300	1		< 1% of normal control	8%, 22%, and 55% of normal control at 0.2 yrs, 0.7 yrs, and 2.2 yrs post		1			growing and developing normally	
Paciorkowski AR, US, 2008	2980						1		modest cognitive abilities	3 mos: some improvement in adaptive social domains6 mos: regression, speech decline12 mos: <0.1 percentile in developmental scales	
Patterson MC, US, 2007	56970						29	mini-mental status examination		mean change in score:1.2 for treatment grp-0.3 for non-treatment grpp=0.165	only results for those >=12 provided
Pineda M, Spain, 2009	56560						57	Annual change in composite score, by age grp		in subset with neurological disease (n=43): -0.210 (-0.336,0.085)in whole grp: -0.125 (-0.235,0.115)	A greater treatment effect was seen in subset of those with neurological disease.

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Ringden O, Sweden, 1995	22020	6			5 who engrafted were within normal ranage		6	Weschler Intelligence Scale	pt 1: stanine 7	pt 1: stanine 7, 5, 6, 7, 7 at 1 yr, 3 yrs, 5 yrs, 8 yrs, and 10 yrs follow-up; IQ=112-120pt 2: stanine 7 at 6 yrspt 3: 3 at 4 yrspt 4: below agept 5: at agept 6: below age at 1 yr	
Schiffman R, Netherlands, 2008	56750						30				No statistically significant differences between study grps using Purdue Peg Board test, Wechsler Scale, Benton visual retention test, Rey auditory verbal learning test, d2 test of attention, continuous performance test, and Trail Making Test.

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Schiffmann R, Netherlands, 1997	58150	5	0.585 nmol/ml (0.399-0.764)		0.741 nmol/ml (0.04-2.363)		5		3 mild-moderate mental retardation2 normal IQ	no change in IQ, 1 showed clinical function deterioration	4 sAppendix Table 9or slightly improved, 1 deterioratedce rebrospinal fluid measurements showed that glucocerebrosidase delivery to the cerebrospinal fluid was minimal (not significantly different)
Seto T, Japan, 2001	13460A						10: 3 HSCT, 7 non-HSCT		HSCT:#1: lesions in white matter and corpus callosum#2: enlargement of perivascular spaces at basal ganglia, intensity changes in periventricular white matter#3: lesions in parietal and occipital lobes, intensity in white matter	HSCT:#1: no follow-up MRI#2: no change at 7 yrs post#3: lesions slightly diminished at 2.5 yrs post	non-HSCT:#1: cortical atrophy, white matter lesions & intensity#2: brain atrophy 9 yrs, cerebrum atrophy 15 yrs#3: brain atrophy 12 yrs#4: white matter lesions#5: ventricular dilation, white matter intensity 18 yrs#6: normal 18 yrs#7: normal 16 yrs

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Seto T, Japan, 2001	13460B						1 HSCT, 3 non-HSCT		HSCT pt: no pathological findins in brain or spinal cord	HSCT pt: no MRI following HSCT	3 non-HSCT pts:#1: brain image normal, intellect fairly good, severe spinal cord compression 19 yrs#2: normal intellect, spinal cord compression 13 yrs#3: spinal cord compression, remainder CNS normal 4 yrs
Shield JPH, England, 2005	6720	1		8 nmol/h/mg protein	246 nmol/h/mg protein at 7 yrs post		1			normal language development at 0.6 yrs postlanguage declining at 1.7-2.1 yrs postdemyelination and diffuse cerebral function at 2.4 yrs postno language at 4.0 yrs post	

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Sivakumar P, England, 1999	16200	1 HSCT pt	875-1716 pmol/h/mg protein	4 pmol/h/mg protein	280-666 pmol/h/mg protein over 7 yrs follow-up	no enzyme data for 1 non-HSCT pt	2: 1 HSCT, 1 non-HSCT			developmental quotient scores for HSCT pt: 99, 72, 55, 43, 24, 11, 6 at 1.5, 2.5, 3.5, 4.5, 6, 7, 8, 10 yrs of agedevelopmental quotient scores for non-HSCT pt: 25, 21, 10, 5 at 6, 7, 8, 10 yrs of age	developmental quotients decreasing with age for both treated and untreated pts
Stein J, Israel, 2007	4880	1	8 nmol/mg protein/h	0	7 nmol/mg protein/h at 1.5 yrs post		1		MRI showed 0.5 yr delay in myelination	MRI showed appropriate myelination for age at 1 yr post	at 4 yrs post, pt has normal intellectual development, attends regular school, and speaks Russian and Hebrew

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Takahashi, Japan, 2001	14030	1 HSCT, 2 non-HSCT		1 HSCT pt: non-detecAppendix Table 92 non-HSCT pts: non-detecAppendix Table 9	1 HSCT pt: 6.7 and 6.7 nmol/hr/mg protein at 0.5 and 1.1 yrs post	measurements for non-HSCT given only once	1 HSCT, 2 non-HSCT		DQ:1 HSCT pt: 722 non-HSCT pts: 94 and 124	DQ:1 HSCT pt: 61 and 54 at 0.5 yrs and 1.1 yrs post2 non-HSCT pts: no follow-up measurement	MRI findings:1 HSCT pt:ventricular dilatation present pre-transplant and worsened post-transplant lesions in white matter present both pre-transplant and post-transplant2 non-HSCT pts:no ventricular dilatationlesions in white matter
Tokimasa, Japan, 2008	1310						1		mental retardation		

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Tolar J, US, 2009	1370	4		pt 1: 0pt 2: 6% of normalpt 3: 3.8% of normalpt 4: 12.5% of normal	pt 1: 50%, 80-90%, 100% of normal at 0.1, 2.0, 3.5 yrs postpt 2: 50% of normalpt 3: 4.1 (normal)pt 4: 5.9 (normal)		4			pt 1 at 11 yrs post: mildly impaired cognitive abilities, sustained visual attention impaired, verbal fluency avg, attends special school, mostly homeschooled, no behavior problems, English and Spanish language development	pt 4 at 4 yrs post: cognition improved from baseline, receptive and expressive language high avg, adaptive skills avg, emotional and social behavior avg, attends special education preschool, speaks 3 languages
Tsai P, US, 1992	25120	1			near normal	measured in liver, lung, lymph nodes, brain	6		RA: 22 mos; DA: 15 mos;DQ=68	RA: 33 mos; DA: 21 mos;DQ=64 RA: 39 mos; DA: 25 mos; DQ=64bilingual at 1.6 yrs post	

Study (Investigator, country, record#)	Record Number	Group (N)	Normal Level	Pre-Transplant	Post-Transplant	Comments	Group (N) (NNO)	Normal Level (NNO)	Pre-Transplant (NNO)	Post-Transplant (NNO)	Comments (NNO)
Vellodi A, England, 1999	16650	3			pt 1: consistently reduced compared to control, donor was carrierpt 2: normal reference at 6.5 yrs postpt 3: normal reference range		3		Griffiths Mental Development Scale:pt 1: social 61, speech 61pt 2: social 71, speech 71pt 3: social 93, speech 93	Griffiths Mental Development Scale:pt 1 at 10 yrs post: social 10, speech 10pt 2: social 2, speech 2pt 3: full IQ 78, verbal IQ 80 performance IQ 81	steady deterioration in pts 1 and 2pt 3 attends mainstream school, has difficulty with concentration, but is otherwise doing well
Vormoor J, Germany, 2004	9420	2					2				
Yeager AM, US, 2000	14880	1		6% of normal control	44%, 53%, 52%, 18%, 41%, 47%, 48%, and 53% at 0.1 yrs, 0.3 yrs, 0.5 yrs, 0.7 yrs, 0.8 yrs, 1.1 yrs, 1.4 yrs, and 2.3 yrs		1		normal myelination at 0.75 yrs	normal myelination at 0.3 yrs postloss of grey and white matter differentiation at 0.7 yrs postpoor grey and white matter contrast at 1.3 yrs post	Bayley Scales of Infant Development: developmental age and real age equivalent at time of transplant (0.75 yrs)development age plateaued at 0.6 yrs at real age of 1.3 yrs and 2.1 yrs

Appendix Table C102: Adverse Events Treatment Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	F/U (mos)	%	Group (N) AGVHD	Severity or Grade AGVHD	F/U (mos)_2	%_2
Arvio M, Finland, 2001	14180	3				3			
Autti T, Finland, 1999	15540	2				2			
Chen R, Taiwan, 2007	4490	1	staphylococcus epidermis sepsis		1 (100 %)	1	grade 1		1 (100 %)
Coppa GV, Italy, 1999	16350	1			0 (0%)	1			0 (0%)
Ehlert K, Germany, 2006	4690	3	cytomegalovirus (2 pts)mucositis (2 pts)colitis (1 pt)clostridium difficile enteritis (1 pt)		3 (100 %)	3	grade I (1 pt)grade II (2 pts)		3 (100 %)
Grewel S, US, 2003	9750					1	grade 2 gastrointestinal		1 (100 %)
Guffon N, France, 2009	680					8			0 (0%)
Hsu YS, Taiwan, 1999	16540	1			0 (0%)	1	grade 1	0.5 yrs	1 (100 %)
Imaizumi M, Japan, 1994	23220A					1			0 (0%)
Imaizumi M, Japan, 1994	23220B					1			0 (0%)
Laitinen A, Finland, 1997	19620	1				1			
Lange MC, Brazil, 2006	5690					1			0 (0%)
Lonnquist T, Finland, 2001	12960	3				3			
Malm G, Sweden, 2004	8490	2	shingles		1 (50%)	2	pt 1: severe skin, gastrointestinal, liverpt 2: grade I skin		2 (100 %)
McKinnis EJR, US, 1996	20560					1			0 (0%)
Morel CF, Canada, 2007	3010					1	skin		1 (100 %)

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	F/U (mos)	%	Group (N) AGVHD	Severity or Grade AGVHD	F/U (mos)_2	%_2
Mullen CA, US, 2000	15300	1	2 episodes of gram-positive bacteremia, one of limited gastrointestinal bleeding while thrombocyopenic, and one mucositis requiring total parenteral nutrition for several wks		1 (100 %)	1	grade 3 skin and grade 2 gastrointestinalskin rash	2 wks post17 wks post	1 (100 %)
Page KM, US, 2008	1280A					1	grade 2		1 (100 %)
Ringden O, Sweden, 1995	22020	6	pt 2: pneumococcal meningitis at 7 mospt 3: pneumonia, 3 mos in hospitalpt 4: strep septicemia at day 10pt 5: septicemia day 6		4 (67%)	6	grade I		4 (67%)
Sivakumar P, England, 1999	16200					1	mild		1 (100 %)
Stein J, Israel, 2007	4880	1	cytomegalovirusantig enemia		1 (100 %)	1	mild skin rash	0.2 yrs	1 (100 %)
Takahashi, Japan, 2001	14030								
Tokimasa, Japan, 2008	1310	1	septicemia (MRSA)		1 (100 %)	1	stage 1		1 (100 %)
Tolar J, US, 2009	1370	4	sepsis	0.2 and 0.7 yrs	2 (50%)	4	pt 1: grade 3 skin, liverpt 3: grade 3 skin, liverpt 4: grade 3 skin		3 (75%)
Tsai P, US, 1992	25120	1	bilateral pneumonia	18-21 mos	1 (100 %)	1			0 (0%)
Vellodi A, England, 1999	16650	3	rotavirus gastroenteritis leading to severe hypoalbuminaenemia and cerebral oedema	1 mo	1 (33.3 %)	3	moderate		1 (33.3 %)

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infection	F/U (mos)	%	Group (N) AGVHD	Severity or Grade AGVHD	F/U (mos)_2	%_2
Vormoor J, Germany, 2004	9420	2	mucositis:pt 1: grade 2pt 2: grade 3		2 (100 %)	2	pt 1: grade IIpt 2: grade I		2 (100 %)
Yeager AM, US, 2000	14880					1			0 (0%)

Appendix Table C102: Adverse Events Treatment Inherited Metabolic Diseases Continued

Study (Investigator, country, record#)	Record Number	Group (N) CGVHD	Severity or Grade CGVHD	F/U (mos)_3	%_3	Comment_3
Arvio M, Finland, 2001	14180	3				
Autti T, Finland, 1999	15540	2				
Chen R, Taiwan, 2007	4490	1			0 (0%)	
Coppa GV, Italy, 1999	16350	1			0 (0%)	
Ehlert K, Germany, 2006	4690	3			0 (0%)	
Guffon N, France, 2009	680	8	grade 1, lung		1 (12.5%)	chronic pulmonary disease developed
Imaizumi M, Japan, 1994	23220A	1			0 (0%)	
Imaizumi M, Japan, 1994	23220B	1			0 (0%)	
Lange MC, Brazil, 2006	5690	1			0 (0%)	
Malm G, Sweden, 2004	8490	2			0 (0%)	
McKinnis EJR, US, 1996	20560	1			0 (0%)	
Mullen CA, US, 2000	15300	1	severe hemolytic anemia	0.75 yrs post	1 (100%)	
Page KM, US, 2008	1280A	1			0 (0%)	
Ringden O, Sweden, 1995	22020	6	pancreatitis		1 (17%)	
Sivakumar P, England, 1999	16200	1			0 (0%)	
Stein J, Israel, 2007	4880	1			0 (0%)	
Tolar J, US, 2009	1370	4			2 (50%)	pt 1 and pt 4
Vellodi A, England, 1999	16650	3			0 (0%)	
Yeager AM, US, 2000	14880	1			0 (0%)	

Appendix Table C102: Adverse Events Treatment Inherited Metabolic Diseases Continued

Study (Investigator, country, record#)	Record Number	Group (N) Engraftment Failure	Severity or Grade_4	F/U (mos)_4	%_4	Comment_4
Arvio M, Finland, 2001	14180	5			3 (60.0%)	1 was re-transplanted and was successful
Autti T, Finland, 1999	15540	2			0 (0%)	
Chen R, Taiwan, 2007	4490	1			0 (0%)	
Coppa GV, Italy, 1999	16350	1			0 (0%)	
Ehlert K, Germany, 2006	4690	3			0 (0%)	
Grewel S, US, 2003	9750	1			0 (0%)	
Guffon N, France, 2009	680	8			0 (0%)	
Hsu YS, Taiwan, 1999	16540	1			0 (0%)	
Imaizumi M, Japan, 1994	23220A	1			0 (0%)	
Imaizumi M, Japan, 1994	23220B	1			1 (100%)	engraftment incomplete for 1st 4 yrs, then complete engraftment at 5 yrs
Jacobs JFM, Netherlands, 2005	6740	1			0 (0%)	
Laitinen A, Finland, 1997	19620	1			0 (0%)	
Lange MC, Brazil, 2006	5690	1			0 (0%)	
Li P, US, 1996	20260	1			0 (0%)	
Lonnquist T, Finland, 2001	12960	3			1 (33.3%)	second transplant in engraftment failure pt was successful
Malm G, Sweden, 2004	8490	2			0 (0%)	
McKinnis EJR, US, 1996	20560	1			0 (0%)	
Morel CF, Canada, 2007	3010	1			0 (0%)	
Mullen CA, US, 2000	15300	1			0 (0%)	
Page KM, US, 2008	1280A	1			0 (0%)	
Ringden O, Sweden, 1995	22020	6	was put on ERT	rejected bone marrow at 3 mos	1 (17%)	
Ringden O, Sweden, 2006	5940A	2			2 (100%)	
Seto T, Japan, 2001	13460A	3			0 (0%)	
Seto T, Japan, 2001	13460B					

Study (Investigator, country, record#)	Record Number	Group (N) Engraftment Failure	Severity or Grade_4	F/U (mos)_4	%_4	Comment_4
Sivakumar P, England, 1999	16200	1			1 (100%)	
Stein J, Israel, 2007	4880	1			0 (0%)	
Takahashi, Japan, 2001	14030	1			0 (0%)	
Tokimasa, Japan, 2008	1310					
Tolar J, US, 2009	1370	4			1 (25%)	pt 3 had 2 failures at 1.6 yrs and 1.7 yrs prior to successful engraftment at 2.1 yrs
Vellodi A, England, 1999	16650	10			2 (20.0%)	
Vormoor J, Germany, 2004	9420	2			0 (0%)	
Yeager AM, US, 2000	14880	1			0 (0%)	

Appendix Table C102: Adverse Events Treatment Inherited Metabolic Diseases Continued

Study (Investigator, country, record#)	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Comment TRM	Group (N) Secondary Malignancies	Severity of Grade SM	F/U (mos) SM	% SM	Comments SM	Record Number
Arvio M, Finland, 2001	3			0 (0%)		3					14180
Autti T, Finland, 1999	2			0 (0%)		2					15540
Chen R, Taiwan, 2007	1			0 (0%)							4490
Coppa GV, Italy, 1999	1			0 (0%)							16350
Ehlert K, Germany, 2006	3			0 (0%)							4690
Grewel S, US, 2003	1			0 (0%)		1	EBV-positive B-cell lymphoma	0.7 yrs	1 (100 %)	successfully treated by withdrawal of immunosuppression and one donor lymphocyte infusion	9750
Guffon N, France, 2009	8			0 (0%)							680

Study (Investigator, country, record#)	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Comment TRM	Group (N) Secondary Malignancies	Severity of Grade SM	F/U (mos) SM	% SM	Comments SM	Record Number
Hsu YS, Taiwan, 1999	1			0 (0%)							16540
Imaizumi M, Japan, 1994	1			0 (0%)							23220A
Imaizumi M, Japan, 1994	1			0 (0%)							23220B
Jacobs JFM, Netherlands, 2005	1			0 (0%)							6740
Laitinen A, Finland, 1997	1			0 (0%)		1					19620
Lange MC, Brazil, 2006	1			0 (0%)							5690
Li P, US, 1996	1			0 (0%)							20260
Lonnquist T, Finland, 2001	3			0 (0%)		3					12960
Malm G, Sweden, 2004	2			0 (0%)		2					8490
McKinnis EJR, US, 1996	1			0 (0%)							20560
Morel CF, Canada, 2007	1			0 (0%)							3010
Mullen CA, US, 2000	1			0 (0%)							15300
Page KM, US, 2008	1				dead at 4.6 yrs post, unknown cause, probable infection						1280A
Ringden O, Sweden, 2006	2			1 (50%)	Type A pt, died of pneumonia 0.4 yrs post						5940A
Seto T, Japan, 2001	3			0 (0%)							13460A

Study (Investigator, country, record#)	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Comment TRM	Group (N) Secondary Malignancies	Severity of Grade SM	F/U (mos) SM	% SM	Comments SM	Record Number
Seto T, Japan, 2001	1			0 (0%)							13460B
Sivakumar P, England, 1999	1			0 (0%)							16200
Stein J, Israel, 2007	1			0 (0%)							4880
Takahashi, Japan, 2001	1			0 (0%)							14030
Tokimasa, Japan, 2008	1	PTLD		1 (100 %)		1	post-transplant lymphoproliferative disease	0.8 yrs	1 (100 %)	cause of death	1310
Tolar J, US, 2009	4		0.2 and 0.7 yrs	2 (50%)	pt 2: hepatorenal failure, pulmonary failure, coagulopathy, sepsispt 3: sepsis and liver failure						1370
Tsai P, US, 1992	1	s. pneumoniae sepsis		1 (100 %)							25120
Vellodi A, England, 1999	10			4 (40%)	4 < 100 days post, 2 of sepsis, 2 of aGVHD						16650
Vormoor J, Germany, 2004	2			0 (0%)		2					9420
Yeager AM, US, 2000	1			0 (0%)							14880

Appendix Table C103: Adverse Events Comparator Inherited Metabolic Diseases

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infectious	F/U (mos)	%	Comment	Group (N) AGVHD	Severity or Grade_2	%_2	Group (N) CGVHD	Severity or Grade_3	%_3	Group (N) Engraftment failure	%_4
Arvio M, Finland, 2001	14180	12												
Banjar H, Saudi Arabia, 1998	17920													
Chan LL, Malaysia, 2002	11330													
El-Beshlawy A, Egypt, 2006	5750													
Erikson A, Sweden, 1995	21630													
Muenzer J, US, 2006	57160													
Muenzer J, US, 2007	57070													
Paciorkowski AR, US, 2008	2980	1	viral infection (fever, transient leukopenia, thrombocytopenia)	11 mos	1 (100%)									
Page KM, US, 2008	1280B	2				other complications (not infectious):2 developed autoimmune hemolytic anemia 2 thrombocytopenia	2	1 grade 11 grade 2	2 (100%)	2	1 extensive cytopenia 1 limited cytopenia (skin)	2 (100%)	2	0 (0%)
Patterson MC, US, 2007	56970													

Study (Investigator, country, record#)	Record Number	Group (N)	Severity or Grade Infectious	F/U (mos)	%	Comment	Group (N) AGVHD	Severity or Grade_2	%_2	Group (N) CGVHD	Severity or Grade_3	%_3	Group (N) Engraftment failure	%_4
Patterson MC, US, 2010	56500													
Pineda M, Spain, 2009	56560													
Schiffman R, Netherlands, 2008	56750													
Schiffmann R, Netherlands, 1997	58150													

Appendix Table C103: Adverse Events Comparator Inherited Metabolic Diseases Continued

Study (Investigator, country, record#)	Record Number	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Group (N)Developmental Delay	Severity of Grade DD	% DD	Comments DD	Group (N)_9	Severity of Grade SSGR	% SSGR
Arvio M, Finland, 2001	14180											
Banjar H, Saudi Arabia, 1998	17920	3			0 (0%)							
Chan LL, Malaysia, 2002	11330	1			0 (0%)							
El-Beshlawy A, Egypt, 2006	5750	11			0 (0%)							
Erikson A, Sweden, 1995	21630	3			0 (0%)							
Muenzer J, US, 2006	57160	96			0 (0%)							
Muenzer J, US, 2007	57070											
Paciorkowski AR, US, 2008	2980											
Page KM, US, 2008	1280B	2	1 multi-system organ failure	1.8 yrs	1 (50%)							
Patterson MC, US, 2007	56970	12			0 (0%)	12	lethargy, memory impairment, depression	1 (8%)	withdrew from study	12	severe weight loss	3 (25%)
Patterson MC, US, 2010	56500	10			0 (100%)							
Pineda M, Spain, 2009	56560	66			0 (0%)							

Study (Investigator, country, record#)	Record Number	Group (N) TRM	Severity or Grade TRM	F/U (mos) TRM	% TRM	Group (N)Developmental Delay	Severity of Grade DD	% DD	Comments DD	Group (N)_9	Severity of Grade SSGR	% SSGR
Schiffman R, Netherlands, 2008	56750	30			0 (0%)							
Schiffmann R, Netherlands, 1997	58150					5		1 (20%)	One pt experienced precocious puberty due to human chorionic gonadotropin used in ERT preparation			

Appendix Table C104: Design, Participant Selection and Enrollment Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Brunner et al, Austria, 2002	11910	Non-hematologic autoimmune	Systemic lupus erythematosus (SLE)	Refractory	Case report	WHO class IV nephritis, pneumonitis, cutaneous vasculitis	Case report	1		
Chen et al, China, 2005	7790	Non-hematologic autoimmune	Systemic lupus erythematosus	Refractory	SLE (2)	class III or IV lupus nephritis (1996, 2001)	case reports	2		
Connor et al, UK, 2008	2220	Hematologic autoimmune	Evans syndrome	Refractory	Evans syndrome case report					
Couri et al, Brazil, 2009	290	Non-malignant autoimmune	Type 1 diabetes mellitus	Newly diagnosed	18	Inclusion: both sexes, age 13-21 yrs, clinical and laboratory diagnosis of type 1 DM during previous 6 wks Exclusion: positive serology for HIV, HBV, HCV, underlying disease precluding HSCT, pregnancy (11/2003-04/2008)	Prospective phase I/II	18	0	
Crino et al, Italy, 2005	62110	Non-hematologic autoimmune	Type 1 diabetes mellitus	Newly diagnosed	nicotinamide plus intensive insulin therapy (25) intensive insulin therapy (27)	recent onset type 1 diabetes mellitus (< 4 wks duration from dx) (NR)	retrospective	25 27	0 0	

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Daikeler et al, Switzerland, 2009	740	Hematologic autoimmune	Evans syndrome	Refractory	Evans syndrome (5)	unselected (1984-2007)	EBMT registry report	5	0	All cases reported to EBMT registry 1984-2007
Daikeler et al, Switzerland, 2009	740A	Hematologic autoimmune	Autoimmune hemolytic anemia	Refractory	Autoimmune hemolytic anemia (7)	unselected (1984-2007)	EBMT registry reports	7		All cases reported to EBMT registry 1984-2007
de Kleer et al, Netherlands, 2004	8350	Non-hematologic autoimmune	Juvenile idiopathic arthritis	Refractory	Juvenile idiopathic arthritis (34)	all patients with refractory JIA who underwent autologous HSCT since 1997 29 systemic 5 polyarticular	Registry	34 of 41 pts	7 without sufficient data to allow evaluation	
De Stefano et al, Italy, 1999	16180	Hematologic autoimmune	Autoimmune hemolytic anemia	Refractory	Autoimmune hemolytic anemia case report					
Elhasid et al, Israel, 2004	9050	Non-hematologic autoimmune	Diffuse calcinosis	Refractory, severe disease	1		case report	1		

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Fagius et al, Sweden, 2009	1270	Non-hematologic autoimmune	Multiple sclerosis (MS)	Refractory, malignant progressive MS of short duration	MS (2)	very frequent (> 4/yr) and severe EDSS > 6.0) relapses; disease duration < 1.5 yrs; absence of irreversible CNS damage with documented recent improvement suggesting HSCT can benefit patient	case series	2 pediatric cases of 9 total cases		
Farge et al, France, 2004	8600	Non-hematologic autoimmune	Systemic sclerosis (SSc)	Refractory	SSc (5)	Rapidly progressing, early diffuse life-threatening SSc (< 3 yrs duration) or limited SSc if life-threatening	open, multicenter phase I/II	5 pediatric cases out of total 41 cases		Patients included from record #s 11400, 13740, 16270
Huhn et al, USA, 2003	11550	Hematologic autoimmune	Autoimmune thrombocytopenia	Refractory	Autoimmune thrombocytopenia case report					
Jones et al, USA, 2004	9110	Non-hematologic autoimmune	Overlap syndrome	Refractory, severe	1	1998	case report	1		
Kimiskidis et al, Greece, 2008	3020	Non-hematologic autoimmune	Multiple sclerosis (MS)	Refractory, malignant progressive MS of short duration	MS (1)		case report	1		

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Kishimoto et al, Japan, 2003	9500	Non-hematologic autoimmune	Juvenile idiopathic arthritis	Refractory	Juvenile idiopathic arthritis (3)	Not reported	Case reports	3	0	Japanese experience
Lisukov ey al, Russia, 2004	9190	Non-hematologic autoimmune	Systemic lupus erythemastosus (SLE)	Refractory	SLE (4)	refractory WHO class III-IV glomerulonephritis, CNS, lung, heart involvement, life-threatening cytopenias	case series	4		4 of 6 were pediatric pts
Mancardi et al, Italy, 2005	7110	Non-hemaotlogic autoimmune	Malignant multiple sclerosis (MS)	Life-threatening, progressive, refractory	MS (2)	Malignant life-threatening MS, refractory to alternative therapies	case reports	2		
Mastrandrea et al, USA, 2009	40050	Non-hematologic autoimmune	Type 1 diabetes mellitus	Newly diagnosed	etanercept plus intensive insulin therapy	10/2002-10/2007	RCT	10		Using one arm of an RCT
Musso et al, Italy, 2001	13570	Non-hematologic autoimmune	Systemic lupus erythematosus (SLE)	Refractory	SLE (2)	life-threatening severe, refractory disease, SLICC/ACR damage index score < 3,	case series	2		
Nakagawa et al, Japan, 2001	13910	Non-hematologic autoimmune	Juvenile idiopathic arthritis	Refractory	Juvenile idiopathic arthritis (1)	1998	Case report	1		

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Oyama et al, USA, 2005	7570	Non-hematologic autoimmune	Crohn's Disease (CD)	Refractory	CD (4)	Clinical and histologic evidence of CD, < 60 yrs old, failed treatment with corticosteroids, mesalamine, metronidazole, azathiprine, 6-mercaptopurine, infliximab, CDAI of 250-400	Case series	4		
Paillard et al, 2000, France	14650	Hematologic autoimmune	Autoimmune hemolytic anemia	Refractory	Autoimmune hemolytic anemia case report					
Rabusin et al, Italy, 2000	13940	Non-hematologic autoimmune	Juvenile idiopathic arthritis	Refractory systemic or polyarticular disease	Juvenile idiopathic arthritis (5)	1996-2000	Case series	5		
Raetz et al, USA, 1997	18920	Hematologic autoimmune	Evans syndrome	Refractory	Evans syndrome case report					
Statkute eta, USA, 2005	7370	Non-hematologic autoimmune	Systemic lupus erythematosus (SLE)	Refractory	SLE (9)	SLE refractory to pulse cyclophosphamide and > 20 mg prednisone daily, , 4 of 11 ACR criteria for SLE, class III or IV GN, lung, CNS, or visceral involvement (1997-2004)	Case series	9		9 of 28 in the series were pediatric age

Study (Investigator, country, record#)	Record Number	Indication	Disease	Therapeutic Setting	Group (N)	Participant Selection (Treatment Period)	Design	n, Evaluated	n, Withdrawn (Lost to F/U)	Comment
Strober et al, USA, 2009	230	Non-hematologic autoimmune	Myasthenia gravis (MG)	Refractory	MG (1)		case report	1		
Trysberg et al, Sweden, 2000	15570	Non-hematologic autoimmune	Systemic lupus erythematosus (SLE)	Refractory	1	CNS lupus, bilateral opticus neuritis, transverse myelitis	case report	1		
Urban et al, Austria, 2006	5970	Hematologic autoimmune	Evans syndrome	Refractory	Evans syndrome case report					
Wulffrat et al, Netherlands, 2001	13970	Non-hematologic autoimmune	Systemic lupus erythematosus (SLE)	Refractory	Case reports (2)	Severe, WHO class IV glomerulonephritis, polyarthritis, malar rash	case reports	2		

Appendix Table C105: Participant Characteristics Treatment Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Brunner et al, Austria, 2002	11910	SLE case report	18 yrs					F			
Chen et al, China, 2005	7790	SLE (2)			13, 18 yrs				severe, refractory to corticosteroids, 6-mercaptopurine, cyclophosphamide		
Connor et al, UK, 2008	2220	Evans syndrome case report	7 yrs					F			
Couri et al, Brazil, 2009	290	18		18	13-21		white (75)	67, 33			
Daikeler et al, Switzerland, 2009	740	Evans syndrome (5)		11	2-21 yrs			M 5 (100)			All cases reported to EBMT registry 1984-2007
Daikeler et al, Switzerland, 2009	740A	Autoimmune hemolytic anemia (7)		7	2-14			5 M (71)			All cases reported to EBMT registry 1984-2007
de Kleer et al, Netherlands, 2004	8350	Juvenile idiopathic arthritis (34)	8.9 yrs		4-18 yrs	3.6 yrs	NR	19 M, 15 F (56/44)	refractory	29 systemic5 polyarticular	

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
De Stefano et al, Italy, 1999	16180	Autoimmune hemolytic anemia case report	6 yrs					M			
Elhasid et al, Israel, 2004	9050	Diffuse calcinosis (1)	15 yrs					F	Severe, progressive		
Fagius et al, Sweden, 2009	1270	MS (2)	9, 16 yrs					1 M, 1 F	EDSS 4.0, 8.0; annualized relapse rate 15, 18, respectively		
Farge et al, France, 2004	8600	SSc (5)		12 yrs	9-17 yrs			F 4, M 1		scleroderma lung disease, 4 diffuse, 1 limited	
Huhn et al, USA, 2003	11550	Autoimmune thrombocytopenia case report	17 yrs					M			
Jones et al, USA, 2004	9110	1	10					F	Severe, refractory with small vessel vasculitis		
Kimiskidis et al, Greece, 2008	3020	MS (1)	17 yrs					M	Malignant MS, EDSS score 5.0		
Kishimoto et al, Japan, 2003	9500	Juvenile idiopathic arthritis (2)	3, 13, 21 yrs					1 M, 2 F	Systemic disease, refractory to conventional therapies		
Lisukov ey al, Russia, 2004	9190	SLE (4)	19 yrs		15-21 yrs	2.8 yrs		F (100)	refractory to pulse cyclophosphamide, corticosteroids, azathioprine		

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (median)	Age (Range)	Age (SD)	Race (%)	Gender M, F (%)	Disease Stage/category	Disease Histology/Site (%)	Comment
Mancardi et al, Italy, 2005	7110	MS (2)	16, 18 yrs					1 M, 1 F		Paralyzing lesions within CNS	
Musso et al, Italy, 2001	13570	SLE (2)	17, 20 yrs					F (100)	severe refractory		
Nakagawa et al, Japan, 2001	13910	Juvenile idiopathic arthritis (1)	15 yrs						Refractory disease		
Oyama et al, USA, 2005	7570	CD (4)	17 yrs		15-21 yrs	2.7 yrs	white	M (n = 3)	severe refractory		
Paillard et al, 2000, France	14650	Autoimmune hemolytic anemia case report	8 yrs					M			
Rabusin et al, Italy, 2000	13940	Juvenile idiopathic arthritis (5)	14.6 yrs		9-20 yrs	3.9 yrs		1 M (20)	Refractory, 3-12 yrs duration		
Raetz et al, USA, 1997	18920	Evans syndrome case report	5 yrs					M			
Statkute eta, USA, 2005	7370	SLE (9)	19 yrs		15-21 yrs	2yrs		F (100)	Refractory		
Strober et al, USA, 2009	230	MG (1)	17 yrs					M	Severe, refractory		
Trysberg et al, Sweden, 2000	15570	SLE (1)	18 yrs					F	Severe, refractory		
Urban et al, Austria, 2006	5970	Evans syndrome case report	2 yrs					M			
Wulffrat et al, Netherlands, 2001	13970	SLE (2)	14 yrs					1 M, 1 F	Severe, refractory		

Appendix Table C106: Participant Characteristics Comparator Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Group (N)	Age (mean)	Age (Range)	Age (SD)	Gender M, F (%)
Crino et al, Italy, 2005	62110	nicotinamide plus intensive insulin therapy (25) intensive insulin therapy (27)	14.7 14	NR	5 4.3	
Mastrandrea et al, USA, 2009	40050	etanercept plus intensive insulin therapy (10)	12.5 yrs	3-18 yrs	3.3 yrs	8 (80)

Appendix Table C107: Treatment Characteristics Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Brunner et al, Austria, 2002	11910	SLE case report	PB	autologous	corticosteroids, azathioprine, cyclophosphamide, immunopheresis	cyclophosphamide plus ATG	N/A		N/A		
Chen et al, China, 2005	7790	SLE (2)	PB	autologous	corticosteroids, cyclophosphamide	ATG, cyclophosphamide	N/A	bactrim, IVIG, G-CSF in 1 pt	N/A		
Connor et al, UK, 2008	2220	1 case report	NR	allogeneic	cortocosteroids, IVIG, cyclosporine, mycophenolate mofetil, rituximab	alemtuzumab, fludarabine, melphalan	cyclosporine and mycophenolate mofetil	NR	NA	NA	
Couri et al, Brazil, 2009	290	18	peripheral blood	autologous nonmyeloablative	None	cyclophosphamide, antithymocyte globulin		dexchlorpheniramine, G-CSF			

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Crino et al, Italy, 2005	62110	nicotinamide plus intensive insulin therapy (25) intensive insulin therapy (27)			None				nicotinamide plus intensive insulin therapy	nicotinamide 25 mg/kg daily, plus 3-4 injections per day of regular plus intermediate-acting insulin 3-4 injections per day of regular plus intermediate-acting insulin both groups 55% carbohydrate diet	
Daikeler et al, Switzerland, 2009	740	Evans syndrome (5)	3 BM, 1 PB, 1 CB	allogeneic	Standard therapy (NR)	various combinations, including cyclophosphamide, fludarabine, busulfan, thiotepa, ATG, TBI	cyclosporine A with either methotrexate or mycophenolate mofetil	NR	NA	NA	5 cases reported to EBMT registry between 1984 and 2007
Daikeler et al, Switzerland, 2009	740A	Autoimmune hemolytic anemia (7)		allogeneic	Standard therapy (NR)	various combinations, including cyclophosphamide, fludarabine, busulfan, thiotepa, ATG, TBI	cyclosporine A with either methotrexate or mycophenolate mofetil	NR	NA	NA	5 cases reported to EBMT registry between 1984 and 2007

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
de Kleer et al, Netherlands, 2004	8350	Juvenile idiopathic arthritis (34)	BM 25, PB 9	autologous	various combinations of corticosteroids, methotrexate, cyclosporin A, azathioprine, NSAIDs, sulphasalazine, cyclophosphamide, gold im, IVIG, hydroxychloroquine, anti-TNF agents	3 different regimens used: A = ATG, cyclophosphamide, low dose TBI B = ATG, cyclophosphamide C = fludarabine, cyclophosphamide, methylprednisolone					
De Stefano et al, Italy, 1999	16180	Autoimmune hemolytic anemia case report	bone marrow	allogeneic	autologous HSCT, splenectomy, prednisone, azathioprine, cyclosporine A,	busulfan, fludarabine, thiotepa	cyclosporine A, methotrexate, ATG	NR	NA	NA	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Elhasid et al, Israel, 2004	9050	Diffuse calcinosis (1)	PB	autologous	corticosteroids, cyclophosphamide, azathioprine, methotrexate, hydroxychloroquine, thalidomide	BEAM	N/A		N/A		
Fagius et al, Sweden, 2009	1270	MS (2)	PB	autologous	methylprednisolone, plasma exchange, beta-IFN	cyclophosphamide in 1 case, BEAM (BCNU, etoposide, ara-C, melphalan) in the second	N/A	ATG, acyclovir, bactrim	N/A		
Farge et al, France, 2004	8600	SSc (5)	PB	autologous	NR	Combinations including cyclophosphamide alone, cyclosphosphamide plus ATG and TBI, cyclophosphamide plus CAMPATH-1H	N/A	NR	N/A		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Huhn et al, USA, 2003	11550	Autoimmune thrombocytopenia case report	PB	autologous	prednisone, splenectomy, IVIG, azathioprine, danazol. Interferon-alpha, plasmapheresis	cyclophosphamide		MESNA, G-CSF, fluconazole, Bactrim			
Jones et al, USA, 2004	9110	1	BM	allogeneic	methotrexate, cyclophosphamide, corticosteroids, nifedipine, enalapril, amitriptyline, celecoxib	nonmyeloablative, fludarabine, cyclophosphamide, TBI	mycophenolate mofetil, cyclosporine A	methylprednisolone, IVIG	N/A		
Kimiskidis et al, Greece, 2008	3020	MS (1)	PB	autologous	iv methylprednisolone, IFN-beta	busulfan, ATG	N/A	G-CSF,	N/A		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Kishimoto et al, Japan, 2003	9500	Juvenile idiopathic arthritis (2)	1 BM, 2 PBSC	autologous	corticosteroids, cyclosporine A, NSAIDs, methotrexate, cyclophosphamide	cyclophosphamide and ATG (n = 1), etoposide, thiotepa, ATG (n = 2)	N/A	Not reported	N/A		
Lisukov ey al, Russia, 2004	9190	SLE (4)	PB or BM (not specified)	autologous	corticosteroids, azathioprine, cyclophosphamide	various dose regimens of BEAM with ATG, cyclophosphamide with ATG, etoposide plus melphalan, all with methylprednisolone	N/A	anti-emetics, analgesia, ciprofloxacin, fluconazole, acyclovir, G-CSF, bactrim	N/A		
Mancardi et al, Italy, 2005	7110	MS (2)	PB	autologous	high-dose corticosteroids, cyclophosphamide, plasma exchange, IFN-beta	BCNU, ara-C, etoposide, melphalan, with or without ATG		IV cyclosporine A	N/A		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Mastrandrea et al, USA, 2009	40050	etanercept plus intensive insulin therapy (10)							etanercept plus intensive insulin therapy	etanercept 0.4 mg/kg twice weekly up to max dose of 25 mg/kg three-injection insulin regimen with Humalog and NPH before breakfast	
Musso et al, Italy, 2001	13570	SLE (2)	PB	autologous	corticosteroids, cyclophosphamide, IVIG, azathioprine, plasma exchange	cyclophosphamide plus ATG and prednisolone	N/A	ciprofloxacin, bactrim, acyclovir, itraconazole	N/A		
Nakagawa et al, Japan, 2001	13910	Juvenile idiopathic arthritis (1)	PB	autologous	corticosteroids, methotrexate, NSAIDs	ALG, cyclophosphamide,	N/A	IVIG, acyclovir, antipruritic drugs	N/A		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Oyama et al, USA, 2005	7570	CD (4)	PB	autologous	corticosteroids, mesalamine, metronidazole, azathioprine, 6-mercaptopurine, infliximab	cyclophosphamide, ATG	N/A	mesna, methylprednisolone, G-CSF, low microbial diet, ciprofloxacin, fluconazole, valacyclovir, pentamidine, bactrim	N/A		
Paillard et al, 2000, France	14650	Autoimmune hemolytic anemia case report	PB	autologous	autologous HSCT, prednisone, IVIG, plasmapheresis, splenectomy, ATG	BCNU, etoposide, ara-C, melphalan, ATG	NA	cyclosporine A	NA	NA	
Rabusin et al, Italy, 2000	13940	Juvenile idiopathic arthritis (5)	NR, all cells treated in vitro with vincristine and methylprednisolone	autologous	Corticosteroids, NSAIDs, methotrexate, cyclosporine A, cyclophosphamide	ATG, plus cyclophosphamide or fludarabine	N/A	hyperhydration, uromitexane, cortisone, antihistamine, cyclosporine A	N/A	N/A	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Raetz et al, USA, 1997	18920	Evans syndrome case report	umbilical cord blood	allogeneic	prednisone, IVIG, 6-mercaptopurine, azathioprine, anti-D, cyclosporine A, vincristine	cyclophosphamide and TBI	cyclosporine A	G-CSF	NA	NA	
Statkute eta, USA, 2005	7370	SLE (9)	PB	autologous	pulse cyclophosphamide, > 20 mg prednisone daily	ATG, cyclophosphamide	N/A	G-CSF, pentamidine, fluoroquinolone, acyclovir or valacyclovir, bactrim	N/A		

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Strober et al, USA, 2009	230	MG (1)	PB	allogeneic	pyridostigmine, IVIG, thymectomy, corticosteroids, mycophenolate mofetil, azathioprine, plasmapheresis, rituximab, high-dose cyclophosphamide	alemtuzumab, busulfan, fludarabine	methotrexate, cyclosporine A		N/A		
Trysberg et al, Sweden, 2000	15570	SLE (1)	PB	autologous	corticosteroids, cyclophosphamide, warfarin, aspirin, ATG,	cyclophosphamide and TBI	N/A	cyclosporin A, low dose corticosteroids, anti-herpes, anti-fungal, antibiotics	N/A		
Urban et al, Austria, 2006	5970	Evans syndrome case report	umbilical cord blood	allogeneic	2 autologous HSCT corticosteroids, IVIG, rituximab, vincristine	busulfan, ATG, thiotepa, etoposide	prednisone, cyclosporine A	NR	NA	NA	

Study (Investigator, country, record#)	Record Number	Group (N)	Stem Cell Source	Type of HSCT	Prior Treatment	Conditioning Regimen	Immunosuppressive therapy for GVHD prophylaxis	Supportive Care	Comparative Treatment	Comparative Treatment Dose/Regimen	Comment
Wulffrat et al, Netherlands, 2001	13970	SLE (2)	BM	autologous	corticosteroids, cyclophosphamide, azathioprine, hydroxychloroquine	cyclophosphamide, ATG, low-dose TBI	N/A	NR	N/A		

Appendix Table C108: Outcome Assessment Treatment Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Brunner et al, Austria, 2002	11910	SLE case report	Complete remission			Complete drug-free resolution of SLE at 21 mos F/U, KPS 100%	
Chen et al, China, 2005	7790	SLE (2)	pre-post SLEDAI score~ drug-free clinical remission			Pt 1: SLEDAI 6, 0~ Pt 2: SLEDAI 12, 0~ Pt 1 in complete clinical and laboratory remission 44 mos posttransplant; Pt 2 in complete clinical and laboratory remission until 9 mos, when she was lost to F/U	
Connor et al, UK, 2008	2220	1 case report	survival			at 10 mos she was weaning immunosuppression, with full donor chimerism and no evidence of GVHD	
Couri et al, Brazil, 2009	290	18	AUC of C-peptide levels during mixed-meal tolerance test, 0,24, 36 mos~ TRM total insulin free post-HSCT (%) time free from exogenous insulin			74.5 +/- 24.8 nmol/L, 260.0 +/- 30 nmol/L, 241.0 +/-48 nmol/L (p = 0.001, 0 vs 24 mos) 0 16 of 18 (89%), range 7-52 months	AUC data includes 7 patients > 20 yrs old

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Daikeler et al, Switzerland, 2009	740	Evans syndrome (5)	survival			3 alive at 36, 85 and 113 mos 1 dead from disease at 59 mos 1 dead from interstitial pneumonitis at 6 mos	
Daikeler et al, Switzerland, 2009	740A	Autoimmune hemolytic anemia (7)	survival			4 alive at 3.9, 86, 112, 124 mos 3 dead at 0.7, 1.4, 5.2 mos	Survival reported as alive or dead at follow-up time; not Kaplan-Meier curves

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
de Kleer et al, Netherlands, 2004	8350	Juvenile idiopathic arthritis (34)	Complete drug-free response (%) partial response (%) no response (%) OS EFS	TRM other adverse events	CR = 53%~ PR = 18%~ NR = 21%~ OS = 79% at 5 yrs~ EFS = 54% at 5 yrs~ TRM = 9%		Five of 6 rheumatological outcomes (VAS - wellbeing, CHAQ-pain, disability, active joint count, ESR) improved within 3 mos from pre-HSCT values (p < 0.04); EPM-ROM did not decline. JIA among those who relapsed was as severe and refractory as prior HSCT
De Stefano et al, Italy, 1999	16180	Autoimmune hemolytic anemia case report	survival			patient alive and well 18 mos posttransplant, weaned off immune suppressive thepy, full donor chimerism, normally functioning immune system	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Elhasid et al, Israel, 2004	9050	Diffuse calcinosis (1)	activities of daily living~ clinical disease			At 2 yrs post-HSCT, patient is free from laboratory and clinical evidence of disease, is able to stand, sit, and walk unaided	
Fagius et al, Sweden, 2009	1270	MS (2)	EDSS score pre-post HSCT~ clinical condition			Pt 1: 4.0, 0.0; Pt 2: 8.0, 1.0~ both patients reported without disease-modifying treatments and sAppendix Table 10at 28 and 35 mos	Results except EDSS reported as a group
Farge et al, France, 2004	8600	SSc (5)	Outcomes for 5 patients were reported in scant detail. All 5 were alive, with 4 CR, 1 PR. TRM was reported in a 6th patient. 1 patient relapsed at about 9 mos after initial CR.			median 38 mos (range 14-68 mos)	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Huhn et al, USA, 2003	11550	Autoimmune thrombocytopenia case report	response to therapy (self-sustained platelet count > 100,000/mm3, reduced bleeding complications and transfusion requirements			no response at 39 mos follow-up	
Jones et al, USA, 2004	9110	1	complete drug-free remission~ activities of daily living			Patient's cushingoid features resolved, all immune suppressant therapies were stopped, grew 17.7 cm in 3 yrs, full-time student in a regular classroom	
Kimiskidis et al, Greece, 2008	3020	MS (1)	EDSS pre-post HSCT~ clinical remission			EDSS = 3.5 at 1 mo, 1.0 at 12 mos~drug-free clinical remission at 62 mos, able to finish college and work	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Kishimoto et al, Japan, 2003	9500	Juvenile idiopathic arthritis (3)	Disease response~ Survival			Pt 1: No response to AHSCT, subsequent allogeneic transplant was followd by patient death 48 days posttransplant~ Pt 2: Disease flares at 11 and 23 mos, medication-free at 39 mos~ Pt 3: drug-free clinical remission at > 35 mos	
Lisukov ey al, Russia, 2004	9190	SLE (4)	Complete remission (SLEDAI < 3, prednisolone dose < 10 mg daily, absence of other immunosuppressive therapy) (%)			1 pt (25%) achieved CR with F/U > 60 mos; 1 pt improved functionally but did not achieve primary endpoint	
Mancardi et al, Italy, 2005	7110	MS (2)	EDSS score pre-post HSCT~ neurological improvement~ mobility			Pt 1: 7.4, 4.0; Pt 2; 9, 4.5~ Pt 1 could walk and perform activities of daily living independently at 29 mos F/U~ Pt 2 neurological condition improved dramatically (not described) at 14 mos F/U	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Musso et al, Italy, 2001	13570	SLE (2)	Corticosteroid-free complete remission~ KPS pre- and posttransplant			2 of 2 (100) at > 30 mos and > 3.8 mos F/U~ Pt 1: 40, 100; Pt 2: 60, 100	Both patients reported drug-free at F/U
Nakagawa et al, Japan, 2001	13910	Juvenile idiopathic arthritis (1)	Medication-free survival~ growth rate			15 mos posttransplant~ 16 cm/yr compared to 2 cm/yr in preceding 3 yrs	
Oyama et al, USA, 2005	7570	CD (4)	Clinical drug-free remission (%)~ survival (%)~ KPS pre-post HSCT~CDAI pre-post HSCT~ disease manifestations post-HSCT			100%~ 100% at 37, 36, 16, 7 mos F/U~ 40, 100; 50, 100; 40, 80; 60, 90~ 337, 51; 293, 59; 250, 78; 274, 74~ 2 asymptomatic (50%), 2 (50%) with occasional abdominal pain or diarrhea	
Paillard et al, 2000, France	14650	Autoimmune hemolytic anemia case report	survivaL			Patient in hematological remission 20 mos posttransplant	
Rabusin et al, Italy, 2000	13940	Juvenile idiopathic arthritis (5)	Complete drug-free response 6 mos (%)~ Partial response 6 mos (%)~ Relapse (%)			CR = 4 of 5 (80) at 3 mos, 3 of 5 (60) at 6 mos~ PR = 1 of 5 (20) at 3 mos~ Relapse = 5 of 5 (100) between 6 and 18 mos (mn = 10 +/- 5.1 mos)	Disease evolution followed according to Giannini, including joint-swelling scores, pain scores, and ESR

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Raetz et al, USA, 1997	18920	Evans syndrome case report	survival			patient dead 289 days posttransplant of fulminant liver failure	
Statkute eta, USA, 2005	7370	SLE (9)	SLE drug-free remission (%)~			7 of 9 (78), remission maintained for median 29 mos (rng 12-78 mos)	
Strober et al, USA, 2009	230	MG (10	Activities of daily living			At 40 mos post-HSCT pt if free of all immune suppressant and MG therapies, plays basketbal, and is completely independent	
Trysberg et al, Sweden, 2000	15570	SLE (1)	CNS deficit, mobility			Neurological deficits improved promptly after HSCT, patient was able to read again, walk freely, with MRI observed regression of brain lesions	Required corticosteroids,
Urban et al, Austria, 2006	5970	Evans syndrome case report	survival			at 18 mos patient in good clinical condition, with 100% donor chimerism, no evidence of GVHD, weaned off immune suppressive therapy	

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	Secondary Outcomes	Independent Response Asessor	F/U Frequency/Duration	Comment
Wulffrat et al, Netherlands, 2001	13970	SLE (2)	Pre-post SLEDAI score~ complete drug-free clinical remission			Pt 1: 20, 0; Pt 2: 27, 8~ 2 (100) drug-free complete remission at 18 and 12 mos F/U	Post-HSCT SLEDAI score of 8 in Pt 2 is due to the presence of permanent vasculitic retinal lesions

Appendix Table 10D: Outcome Assessment Comparator Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Group (N)	Primary Outcomes	F/U Frequency/Duration
Crino et al, Italy, 2005	62110	nicotinamide plus intensive insulin therapy (25) intensive insulin therapy only (27)	glycosylated hemoglobin (%) 0, 12, 24 mos fasting C-peptide (nmol/L) 0, 12, 24 mos	nicotinamide plus intensive insulin therapy 9.6+/-2.2, 5.4+/-0.8, 6.1+/-0.9, 1.9+/-0.15, 0.25+/-0.2, 0.19+/-0.2 intensive insulin therapy 10.5+/-2.2, 6.5+/-0.9, 7.0+/-0.9, 0.16+/-0.12, 0.21+/-0.2, 0.19+/-0.13
Mastrandrea et al, USA, 2009	40050	etanercept plus intensive insulin therapy (10)	glycosylated hemoglobin (%) 0, 24 wks meal stimulated C peptide AUC (ng/mL/hr) 0, 24 wks	12.8+/-3.2, 5.9+/-0.5 3.1+/-1.2 ng/mL/hr, 3.9+/-1.6 ng/mL/hr

Appendix Table C109: Time to Event Outcomes Treatment Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Disease	Outcome Asessment Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Time to Event Outcomes Group (N)	Outcome	Outcome_2
Brunner et al, Austria, 2002	11910	Systemic lupus erythematosus (SLE)	SLE case report	Complete remission		Complete drug-free resolution of SLE at 21 mos F/U, KPS 100%			
Chen et al, China, 2005	7790	Systemic lupus erythematosus	SLE (2)	pre-post SLEDAI score~ drug-free clinical remission		Pt 1: SLEDAI 6, 0~ Pt 2: SLEDAI 12, 0~ Pt 1 in complete clinical and laboratory remission 44 mos posttransplant; Pt 2 in complete clinical and laboratory remission until 9 mos, when she was lost to F/U			
Connor et al, UK, 2008	2220	Evans syndrome	1 case report	survival		at 10 mos she was weaning immunosuppression, with full donor chimerism and no evidence of GVHD			
Couri et al, Brazil, 2009	290	Type 1 diabetes mellitus	18	AUC of C-peptide levels during mixed-meal tolerance test, 0,24, 36 mos~ TRM total insulin free post-HSCT (%) time free from exogenous insulin		74.5 +/- 24.8 nmol/L, 260.0 +/- 30 nmol/L, 241.0 +/-48 nmol/L (p = 0.001, 0 vs 24 mos) 0 16 of 18 (89%), range 7-52 months	16	100	

Study (Investigator, country, record#)	Record Number	Disease	Outcome Asessment Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Time to Event Outcomes Group (N)	Outcome	Outcome_2
Daikeler et al, Switzerland, 2009	740	Evans syndrome	Evans syndrome (5)	survival		3 alive at 36, 85 and 113 mos 1 dead from disease at 59 mos 1 dead from interstitial pneumonitis at 6 mos			
Daikeler et al, Switzerland, 2009	740A	Autoimmune hemolytic anemia	Autoimmune hemolytic anemia (7)	survival		4 alive at 3.9, 86, 112, 124 mos 3 dead at 0.7, 1.4, 5.2 mos			
de Kleer et al, Netherlands, 2004	8350	Juvenile idiopathic arthritis	Juvenile idiopathic arthritis (34)	Complete drug-free response (%) partial response (%) no response (%) OS EFS	TRM other adverse events		Juvenile idiopathic arthritis (34)	OS, 1-5 years 79%	EFS, 1-5 years approx 1-54%
De Stefano et al, Italy, 1999	16180	Autoimmune hemolytic anemia	Autoimmune hemolytic anemia case report	survival		patient alive and well 18 mos posttransplant, weaned off immune suppressive thepy, full donor chimerism, normally functioning immune system			
Elhasid et al, Israel, 2004	9050	Diffuse calcinosis	Diffuse calcinosis (1)	activities of daily living~ clinical disease		At 2 yrs post-HSCT, patient is free from laboratory and clinical evidence of disease, is able to stand, sit, and walk unaided			
Fagius et al, Sweden, 2009	1270	Multiple sclerosis (MS)	MS (2)	EDSS score pre-post HSCT~ clinical condition		Pt 1: 4.0, 0.0; Pt 2: 8.0, 1.0~ both patients reported without disease-modifying treatments and sAppendix Table 10at 28 and 35 mos			

Study (Investigator, country, record#)	Record Number	Disease	Outcome Asessment Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Time to Event Outcomes Group (N)	Outcome	Outcome_2
Farge et al, France, 2004	8600	Systemic sclerosis (SSc)	SSc (5)	Outcomes for 5 patients were reported in scant detail. All 5 were alive, with 4 CR, 1 PR. TRM was reported in a 6th patient. 1 patient relapsed at about 9 mos after initial CR.		median 38 mos (range 14-68 mos)			
Huhn et al, USA, 2003	11550	Autoimmune thrombocytopenia	Autoimmune thrombocytopenia case report	response to therapy (self-sustained platelet count > 100,000/mm3, reduced bleeding complications and transfusion requirements		no response at 39 mos follow-up			
Jones et al, USA, 2004	9110	Overlap syndrome	1	complete drug-free remission~ activities of daily living		Patient's cushingoid features resolved, all immune suppressant therapies were stopped, grew 17.7 cm in 3 yrs, full-time student in a regular classroom			

Study (Investigator, country, record#)	Record Number	Disease	Outcome Asessment Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Time to Event Outcomes Group (N)	Outcome	Outcome_2
Kimiskidis et al, Greece, 2008	3020	Multiple sclerosis (MS)	MS (1)	EDSS pre-post HSCT~ clinical remission		EDSS = 3.5 at 1 mo, 1.0 at 12 mos~drug-free clinical remission at 62 mos, able to finish college and work			
Kishimoto et al, Japan, 2003	9500	Juvenile idiopathic arthritis	Juvenile idiopathic arthritis (3)	Disease response~ Survival		Pt 1: No response to AHSCT, subsequent allogeneic transplant was followd by patient death 48 days posttransplant~ Pt 2: Disease flares at 11 and 23 mos, medication-free at 39 mos~ Pt 3: drug-free clinical remission at > 35 mos			
Lisukov ey al, Russia, 2004	9190	Systemic lupus erythema stosus (SLE)	SLE (4)	Complete remission (SLEDAI < 3, prednisolone dose < 10 mg daily, absence of other immunosuppressive therapy) (%)		1 pt (25%) achieved CR with F/U > 60 mos; 1 pt improved functionally but did not achieve primary endpoint			
Mancardi et al, Italy, 2005	7110	Malignant multiple sclerosis (MS)	MS (2)	EDSS score pre-post HSCT~ neurological improvement ~ mobility		Pt 1: 7.4, 4.0; Pt 2; 9, 4.5~ Pt 1 could walk and perform activities of daily living independently at 29 mos F/U~ Pt 2 neurological condition improved dramatically (not described) at 14 mos F/U			

Study (Investigator, country, record#)	Record Number	Disease	Outcome Asessment Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Time to Event Outcomes Group (N)	Outcome	Outcome_2
Musso et al, Italy, 2001	13570	Systemic lupus erythematosus (SLE)	SLE (2)	Corticosteroid-free complete remission~ KPS pre- and posttransplant		2 of 2 (100) at > 30 mos and > 3.8 mos F/U~ Pt 1: 40, 100; Pt 2: 60, 100	SLE (2)		
Nakagawa et al, Japan, 2001	13910	Juvenile idiopathic arthritis	Juvenile idiopathic arthritis (1)	Medication-free survival~ growth rate		15 mos posttransplant~ 16 cm/yr compared to 2 cm/yr in preceding 3 yrs			
Oyama et al, USA, 2005	7570	Crohn's Disease (CD)	CD (4)	Clinical drug-free remission (%)~ survival (%)~ KPS pre-post HSCT~CDAI pre-post HSCT~ disease manifestations post-HSCT		100%~ 100% at 37, 36, 16, 7 mos F/U~ 40, 100; 50, 100; 40, 80; 60, 90~ 337, 51; 293, 59; 250, 78; 274, 74~ 2 asymptomatic (50%), 2 (50%) with occasional abdominal pain or diarrhea			
Paillard et al, 2000, France	14650	Autoimmune hemolytic anemia	Autoimmune hemolytic anemia case report	survivaL		Patient in hematological remission 20 mos posttransplant			
Rabusin et al, Italy, 2000	13940	Juvenile idiopathic arthritis	Juvenile idiopathic arthritis (5)	Complete drug-free response 6 mos (%)~ Partial response 6 mos (%)~ Relapse (%)		CR = 4 of 5 (80) at 3 mos, 3 of 5 (60) at 6 mos~ PR = 1 of 5 (20) at 3 mos~ Relapse = 5 of 5 (100) between 6 and 18 mos (mn = 10 +/- 5.1 mos)			

Study (Investigator, country, record#)	Record Number	Disease	Outcome Asessment Group (N)	Primary Outcomes	Secondary Outcomes	F/U Frequency/Duration	Time to Event Outcomes Group (N)	Outcome	Outcome_2
Raetz et al, USA, 1997	18920	Evans syndrome	Evans syndrome case report	survival		patient dead 289 days posttransplant of fulminant liver failure			
Statkute eta, USA, 2005	7370	Systemic lupus erythematosus (SLE)	SLE (9)	SLE drug-free remission (%)~		7 of 9 (78), remission maintained for median 29 mos (rng 12-78 mos)			
Strober et al, USA, 2009	230	Myasthenia gravis (MG)	MG (10	Activities of daily living		At 40 mos post-HSCT pt if free of all immune suppressant and MG therapies, plays basketbal, and is completely independent			
Trysberg et al, Sweden, 2000	15570	Systemic lupus erythematosus (SLE)	SLE (1)	CNS deficit, mobility		Neurological deficits improved promptly after HSCT, patient was able to read again, walk freely, with MRI observed regression of brain lesions			
Urban et al, Austria, 2006	5970	Evans syndrome	Evans syndrome case report	survival		at 18 mos patient in good clinical condition, with 100% donor chimerism, no evidence of GVHD, weaned off immune suppressive therapy			
Wulffrat et al, Netherlands, 2001	13970	Systemic lupus erythematosus (SLE)	SLE (2)	Pre-post SLEDAI score~ complete drug-free clinical remission		Pt 1: 20, 0; Pt 2: 27, 8~ 2 (100) drug-free complete remission at 18 and 12 mos F/U			

Appendix Table C110: Time to Event Outcomes Comparator Autoimmune Disease

Study (Investigator, country, record#)	Record Number	Disease	Primary Outcomes	F/U Frequency/Duration
Crino et al, Italy, 2005	62110	Type 1 diabetes mellitus	glycosylated hemoglobin (%) 0, 12, 24 mos fasting C-peptide (nmol/L) 0, 12, 24 mos	nicotinamide plus intensive insulin therapy 9.6+/-2.2, 5.4+/-0.8, 6.1+/-0.9, 1.9+/-0.15, 0.25+/-0.2, 0.19+/-0.2 intensive insulin therapy 10.5+/-2.2, 6.5+/-0.9, 7.0+/-0.9, 0.16+/-0.12, 0.21+/-0.2, 0.19+/-0.13
Mastrandrea et al, USA, 2009	40050	Type 1 diabetes mellitus	glycosylated hemoglobin (%) 0, 24 wks meal stimulated C peptide AUC (ng/mL/hr) 0, 24 wks	12.8+/-3.2, 5.9+/-0.5 3.1+/-1.2 ng/mL/hr, 3.9+/-1.6 ng/mL/hr

Appendix D: Disease-Free/Event-Free Survival

ESFT: event-free/disease-free survival

EFS or DFS was reported or generated in 10 HSCT studies (Oberlin, 2008 #2020; Meyers, 2001, #13670; Drabko, 2005, #6680; Prete, 1998, #17210; Hawkins, 2000 ,#15360; Ozkaynak, 1998, #18540; Laws, 2003, #9450; Yaniv, 2004, #9100; Kushner, 2001, #14240; Lucas, 2008, #9450;) and 4 comparative studies (Bernstein, 2006, #6290; Sari, 2010 #42790; Kushner,1995, #44560; Milano, 2006,#5960;)

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TableD1. Event-free survival (DFS; PFS) for treatment (Single and Tandem Auto HSCT) and comparison (Conventional Chemotherapy +/- Radiation) groups

	Intervention Single (%; ± 95% CI)	**Comparator Chemo (%; ± 95% CI)**	**Study**
1 year	54% (25-84)*		Yaniv, Israel, 2004 (n=11) #9100
	20% (0-45)*		Kushner, USA, 2001(n=10) #14240
	50% (0-100%)*		Laws, Germany, 2003 (n=2) #9450
	Stable disease 9 mos after HSCT		Lucas, USA, 2008 (n=1) #2450
		65% +/- 5% [isolated lung mets vs other and more than isolated lung mets 72% +/-7% and 62% +/-6%; p=.39]	Bernstein, USA/Canada 2006 (n=110) #6290
		83% (67-98%)	Kushner, USA, 1995 (n=24) #44560
2 year	20%		Meyers, USA, 2001 (n=32) #13670
	63%		Drabko, Poland, 2005 (n=21) #6680
	63%		Prete, Italy, 1998 (n=17) #17210
	50% (0-100%)*		Laws, Germany, 2003 (n=2) #9450
		24% (+/-4%) [31% +/-7% for pts with isolated lung mets and 20% +/-5% for pts with more widespread dz; p=.39]	Bernstein, USA/Canada 2006 (n=110) #6290
3 year	36%		Hawkins, USA, 2000 (n=16) #15360
	51% for all pts		Ozkaynak, USA, 1998 (n=15) #18540

	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	Study
	66% +/-19% for 1st remission 37% for 2nd remission		
	18% (0-41%)*		Yaniv, Israel, 2004 (n=11) #9100
	20% (0-45)*		Kushner, USA, 2001 (n=10) #14240
		75% (55-95%)	Kushner, USA, 1995 (n=24) #44560
		74%	Milano, Italy, 2006 (n=18) #5960
4 year	A NED (ESFT) at 50 mos after HSCT[a]		Numata, Japan, 2006 (n=1) #12130
5 year	46%		Oberlin, France, 2008 (n=61) #2020
	32% (+/- 11%) in HyperME with median f/u 146 mos (98-190) and 40% (+/-13%) in TandemME with median f/u 68 mos (28-88 mos)		Burdach, Germany and Austria, 2003 (n=32) #10030
	18% (0-41%)*		Yaniv, Israel, 2004 (n=11) #9100
	20% (0-45)*		Kushner, USA, 2001 (n=10) #14240
	A NED 60 months after surgery		Kogawa, Japan, 2004 (n=1) #8410
		18%	Sari, Turkey, 2010 (n=36) #42790
		75% (55-95%)	Kushner, USA, 1995 (n=24) #44560

[a]Numata (#12130)-pt dxd with CML, chronic phase at 50 mos

Wilms

Event-free/disease-free survival

Fourteen studies reported event or disease-free survival (Spreafico, 2008, #2380; Malogolowkin, 2008, ##44950; Tucci, 2007, ##3910; Park, 2006, ##5450; Campbell, 2004, #8570; Valera, 2004, ##8620; Kremens, 2002, ##11240; Abu-Ghosh, 2002, #45610; Saarinen-Pihkala, 1998, ##17940; Pein,1998, ##17570; Dagher, 1998, #17840; Hempel, 1998, ##18100; Hempel, 1996, #20550; Kullendorff,1997, #19290).

Table D2. Event-free survival (DFS; PFS) for treatment (Single Auto HSCT) and comparison (Conventional Chemotherapy +/- Radiation) groups

	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	Study
1 year	1 yr 52% [32-73] (n=23)		Kremens, 2002, #11240
		1 yr ~73% (n=11)	Abu-Ghosh, 2002, #45610
	1 yr 86% [60-100]* (n=7)		Hempel, 1996, #20550
	1 yr 75% [33-100]* (n=4)		Kullendorff, 1997, #19290
	1 yr 67% [13-100]* (n=3)		Valera, 2004, #8620
	.5 yrs (n=1)		Dagher, 1998, #17840
1 year PFS range across studies	52%-86% (Kremens, Spreafico, Hempel, Kullendorff, Valera)	~73% (Abu-Ghosh)	
2 year	2 yr 75% [33-100]* (n=4)		Kullendorff, 1997, #19290
	2 yr 86% [60-100]* (n=7)		Hempel,1996, #20550
	alive at 32 months after HSCT (n=1)		Hempel, 1998, #18100

	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	Study
3 year	3 yr 50% +/- 17 (n=28)		Pein, 1998, #17570
	3 yr 52% [32-73] (n=23)		Kremens, 2002, #11240
	3 yr 56% +/-12% (n=20)		Spreafico, 2008, #2380
		3 year 66.6% (n=10)	Tucci, 2007, #3910
		3 yr 64% (n=11)	Abu-Ghosh, 2002, #45610
	3 yr 67% [13-100]* (n=3)		Valera, 2004, #8620
4 year	4-year 60% (n=13)		Campbell, 2004, #8570
	median 51 months (40-53 months) (n=3)		Saarinen-Pihkala, 1998, #17940
		4 yr 48% (n=60)	Malogolowkin, 2008, #44950
5 year	5 yr 52% [32-73]* (n=23)		Kremens,2002, #11240
	A NED at 7 yr (n=1)	5 year 42.8% (n=10)	Tucci, 2007, #3910
		5 yr 64% (n=11)	Abu-Ghosh, 2002, #45610

Event-free survival

Data on intermediate outcomes were reported in eleven studies and calculated from the raw data from two additional studies (Hara, 1998 #17950; Lucidarme, 1998 #17610). Event free survival estimates are presented below.

Table D3. Event-free survival (DFS; PFS) for treatment (Single Auto HSCT) and comparison (Conventional Chemotherapy +/- Radiation) groups

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
Metastatic Auto	1 year	~46% at 1 year (n=52)	~53% at 1 year (n=42)		Carli, Italy, 1999 #16010
			~63% at 1 year (n=152)		Sandler, USA, 2001 #12810
			~69% at 1 year (n=127)		Breneman, USA, 2003 #75360
Mixed Tumor Stage Auto		12.5 (4,35) at 1 year (n=8)			Lucidarme, France, 1998± #17610
		66.7 (28.9,100) at 1 year (n=7)			Hara, Japan, 1998± #17950
Metastatic Auto	3 year	29.7 (15.6,43.8) at 3 years (n=52)	19.2 (6.8-31.6)at 3 years (n=42)	0.3	Carli, Italy, 1999 #16010
		16.5 at 3 years (n=101)	54.9 at 3 years (n=45)		McDowell, UK, 2010 #75350
		75% (33-107) at 3 years (n=4)	15% (-4-35) at 3 years (n=13)		Williams, Canada, 2004 #9010
		35.3% (24.3-46.5) at 3 years (n=70)			Bisogno, Italy, 2009 #75340
			~28% at 3 years (n=152)		Sandler, USA, 2001 #12810
			25% (17-33) at 3 years (n=127)		Breneman, USA, 2003 #75360

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
Mixed Tumor Stage Auto		66.7 (28.9,100) at 3 years (n=7)			Hara, Japan, 1998± #17950
Metastatic Auto	5 year	14.9 at 5 years (n=101)	51.0% at 5 years (n=45)		McDowell, UK, 2010 #75350
			~20% at 5 years (n=127)		Breneman, USA, 2003 #75360
			~27% at 5 years (n=152)		Sandler, USA, 2001 #12810
Mixed Tumor Stage		36% at 5 years (n=22)			Matsubara**, Japan, 2003 #10810
Cranial Parameningeal			32% (22-42) at 10 years (n=91)		Raney, USA, 2008 #2440
Metastatic (summary)	EFS range for 3 years for studies with > 20 patients	29.7-35.3% (Carli #16010, Biosgno #75340)	19.2-28% (Carli #16010, Sandler #12810, Breneman #75360)		This range does not Include the McDowell #75350 study as the patients in the treatment arm are not comparable to other studies due to their higher risk category.
Mixed Tumor stage (summary)	EFS range for 3 years for studies with > 5 patients	66.7% (Hara #17950)	No comparator		

Retinoblastoma

Two studies reported event free survival (Namouni, 1997 #18090; Kremens, 2003 #10860; Dunkle, 2010 #28560), and EFS was calculated from the raw data from two studies (Galindo, 2003; Matsubara, 2005 #7580). These studies were all single arm case series. At five years the event free survival for patients without CNS involvement ranges from 66.7 to 85.7.

Table D4. Event-free survival (DFS; PFS) for treatment (Single Auto HSCT) and comparison (Conventional Chemotherapy +/- Radiation) groups

Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
Event Free no CNS	Isolated orbital disease (n=7) 85.7 (59.8-100) at 1-5 years			Namouni, 1997± #18090
	100% at 1-3 years 75% at 4-5 years[b] (n=4)			Galindo, 2003± #10420
	100% at mean Follow-up of 38 months (n=3)			Matsubara, 2005 #7580
	66.7% at mean follow-up of 8.9 years (n=5)			Kremens, 2003 # 10860
Event Free mixed	~88% at 1 year[a] ~ 62% at 2 years ~57% at 3 years ~53% at 4-5 years (n=34)[b]			Namouni, 1997 #18090
	Patients with Trilateral retinoblastoma (n=13) ~68% at 1 year ~38% at 2-5 years			Dunkle, 2010 #28560
EFS range for 5 + years for studies with > 2 patients without CNS involvement not including trilateral retinoblastoma	66.7-85.7% (Kremens 2003 # 10860,Galindo 2003 #10420, Namouni 1997 #18090)	NR		
EFS range for 5 + years for studies with > 2 trilateral retinoblastoma	~38% (Dunkle, 2010 #28560)	No comparator study identified		

[a] estimated preceded by a ~ were estimated from published Kaplan-Meier curves. [b] this includes all patients including those who died prior to treatment. ± survival curves were constructed using the raw data published in the articles.

Neuroblastoma

Data on intermediate outcomes were reported in all seven primary studies. Five studies reported data as event-free survival (EFS), one study as disease-free survival (DFS), and another as progression-free survival (PFS). No significant differences between treatment groups in either three-year DFS or five-year EFS were identified in the two comparative studies.(Kim, 2007 [2870]; Ladenstein, 2008 [1610]) Multivariate analysis of the Sung et al. (2007) data showed the application of total body radiation and local radiotherapy during the treatment regimen, and a longer interval (≥ 12 weeks) between the first and second transplant to be independent favorable predictors for EFS (HR, 9.66, 7.17, 5.73; 95% CI, 1.31-71.26, 1.69-30.38, 1.32-24.88; p = 0.026, 0.007, 0.020, respectively).(Sung, 2007 [3950]) It should be noted that five studies (71%) did not define *a priori* these outcomes.

Table D5. Event-free survival (DFS; PFS) for treatment (tandem HSCT) and comparison (single HSCT) groups

Outcome	Intervention Tandem (%; ± 95% CI; SE) [N]	Comparator Single (%; ± 95% CI; SE) [N]	P-value	Study (record #)
3 year rate	50 (20.4) [9]	40.6 (14.7) [27]	0.50	Kim, 2007 (2870)
	61 (50-71) [82]			George, 2006 (5440)
		47 (38-55) [149]		Berthold, 2005 (6760)
5 year rate	27 (2) [455]	33 (1) [2,895]	0.19	Ladenstein, 2008 (1610)
	54 (42-64) [82]			George, 2006 (5440)
	62.1 (13.7) [52]			Sung, 2007 (3950)
		38 (21-54) [32]		Pritchard, 2005 (8030)
		30 (4) [189]		Matthay, 2009 (6210)
> 5 year rate	52 (40-63) [82]			George, 2006 (5440)
EFS range for ≥5 years for studies with > 10 patients	27-62	30-38		

CI, confidence interval; DFS, disease-free survival; N, number of patients; PFS, progression-free survival; SE, standard error

Germ-Cell Tumor

Data on intermediate outcomes were reported in all four studies. The CIBMTR cohort reported data as progression-free survival (PFS) and the remaining three studies either as disease-free survival (Einhorn 2007, De Giorgi 2005) or event-free survival (Agarwal). Data were available to compute three-year rates across all studies, and five-year rates for three studies. For the CIBMTR cohort, there was a trend toward a lower probability of PFS at one-year in the tandem group compared to the single HSCT group (36% vs. 60%), although no p-values were computed due to the small number of cases. PFS did not differ between treatment groups across studies. For the CIBMTR cohort, PFS at 5 years for the tandem group remained at 36% (11%-63%) compared to 49% (26%-69%) in the single HSCT group. PFS was defined as survival without recurrence (or cancer progression) as measured by exam, radiographs, and/or an increase in serum marker levels.(CIBMTR, 2010)

Table D6. Event-free survival (DFS; PFS) for treatment (tandem HSCT) and comparison (single HSCT) groups

Outcome	**Intervention Tandem (%; ± 95% CI) [N]**	**Comparator Single (%; ± 95% CI) [N]**	**P-value**	**Study (record #)**
1-year rate	36 (11-63)	60 (36-78)	NR	CIBMTR, 2010
	59 (39.5-88)			Einhorn, 2007 (77230)
		50 (26-74.5)		De Giorgi, 2005 (77240)
3 year rate	36 (11-63)	49 (26-69)	NR	CIBMTR, 2010
	59 (39.5-88)			Einhorn, 2007 (77230)
		50 (7-93)		Agarwal, 2009 (72940)
		50 (26-74.5)		De Giorgi, 2005 (77240)
5 year rate	36 (11-63)	49 (26-69)	NR	CIBMTR. 2010
	59 (39.5-88)			Einhorn, 2007 (77230)
		50 (26-74.5)		De Giorgi, 2005 (77240)
EFS range for 5 years for studies with > 10 patients	36-59	49-50		

[a] EFS for stage IV patients; CI, confidence interval; DFS, disease-free survival; N, number of patients; NR, not reported; PFS, progression-free survival

CNS/Embryonal Tumors

Data on intermediate outcomes were reported in all 10 studies. For comparisons between tandem vs. single HSCT, data were available to compute two-year, three-year, and five-year rates for three studies. For Sung et al. (2007), EFS at 2 years for the tandem group was 73% (46%-99%) compared to 67% (13%-100%) in the single HSCT group [4770] The AT/RT patient reported in Gidwani et al. (2008) was disease free for two years following tandem HSCT.[71940] EFS was defined as the interval between diagnosis to progression/relapse or death from any cause.

For the conventional-care group of studies, data were available to compute three-year rates for one study and five-year rates for three studies. There were no comparative studies between single HSCT vs. conventional care. For Geyer et al. (2005) on multiple tumor types, overall five-year EFS was 27% (3%) for children under three years of age; for MB, PNET and AT/RT, the corresponding rates were 32% (5%), 17% (6%), and 14% (7%), respectively.[49990] Similar trends to OS above were observed in EFS rates between studies.

Table D7: Event-free survival (DFS; PFS) for treatment (tandem HSCT) and comparison (single HSCT) groups

Outcome (Tumor type)	Intervention Tandem (%; ± 95% CI; SE) [N]	Comparator Single (%; ± 95% CI; SE) [N]	P-value	Study (record #)
2 year rate				
(MB-PNET)	73 (46-99) [11]	67 (13-100) [3]	NR	Sung, 2007 (4770)
(AT/RT)	[One patient remained disease-free]			Gidwani, 2008 (71940)
(MB-PNET)		57 (15) [13]		Perez-Martinez, 2005 (7650)
3 year rate				
(MB-PNET)	73 (46-99) [11]	NA		Sung, 2007 (4770)
(MB)		49 (27-72) [21]		Chi, 2004 (7900)
(AT/RT)		23 (11) [13]		Gardner, 2008 (71930)
5 year rate				
(MB-PNET)	58 (25-91) [11]	NA		Sung, 2007 (4770)
(PNET)		39 (24-53) [43]		Fangusaro, 2008 (3420)
(MB)		52 (11) [21]		Dhall, 2008 (52130)
EFS range for 5 years for studies with > 10 patients	58	39-52		

AT/RT, atypical teratoid/rhabdoid tumor; CC, conventional care; CI, confidence interval; DFS, disease-free survival; EFS, event-free survival; MB, medulloblastoma; N, number of patients; NA, not available; PFS, progression-free survival; PNET, supratentorial primitive neuro-ectodermal tumors; SE, standard error

Table D8: Event-free survival (DFS; PFS) for treatment (single HSCT) and comparison (conventional care) groups

Outcome (Tumor type)	Intervention Single (%; ± 95% CI; SE) [N]	Comparator CC (%; ± 95% CI; SE) [N]	P-value	Study (record #)
3 year rate				
(MB)	49 (27-72) [21]			Chi, 2004 (7900)
(MB)		40 (28-51) [68]		Taylor, 2005 (52760)
(AT/RT)	23 (11) [13]			Gardner, 2008 (71930)
5 year rate				
(MB)	52 (11) [21]			Dhall, 2008 (52130)
PNET	39 (24-53) [43]			Fangusaro, 2008 (3420)
(MB)		81 (2) [379]		Packer, 2006 (77250)
(MB-PNET-AT/RT-Other)		27 (3) [284]		Geyer, 2005 (49990)
(MB)		35 (23-46) [68]		Taylor, 2005 (52760)
EFS range for 5 years for studies with >10 patients	39-52	27-81		

AT/RT, atypical teratoid/rhabdoid tumor; CC, conventional care; CI, confidence interval; DFS, disease-free survival; EFS, event-free survival; MB, medulloblastoma; N, number of patients; NA, not available; PFS, progression-free survival; PNET, supratentorial primitive neuro-ectodermal tumors; SE, standard error

Glial Tumors

Data on intermediate outcomes were reported in twenty-nine studies and calculated from the raw data. Event free survival estimates are presented below.

Table D9: Event-free survival (DFS; PFS) for treatment (Single Auto HSCT) and comparison (Conventional Chemotherapy +/- Radiation) groups

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
Astrocytoma	**1 year**	AA/GBM PFS: ~30% (These HSCT and chemotherapy estimates are grouped EFS for astrocytoma and glioblastoma multiforme) (N=27)	AA/GBM year PFS ~10% (N=56)	Chemo versus ABMR unstratified comparison of event-free survival: P=0.014	Finlay, 2008
			2 patients progressed at 1.5 and 8.5 mo (N=2)		Shih, 2008
			3 patients progressed at 3,3, and 8 mo (N=3)		Sio, 2006
			1 astrocytoma patient progressed at 4 months, and one patient was progression free at 10 months (N=2)		Koronoes, 2006
		Other Glioma ~ 73 (9 AA and 2 Oligodendroglioma) (N=11)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005
			Median AA PFS 21.2mo (1.2-49.3) (N=4)		Hurwitz, 2001
			1 OA patient progressed shortly after chemotherapy and received irradiation (33% of OA) (N=6)		Doireau, 1999
		2 patients progressed at 4.5 and 5.5 months (N=2)			Jakacki, 1999
		1 patient progressed at 11 mo (N=1)			Busca, 1997

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
	3 Year	AA/GBM PFS: 22±7% (These HSCT and chemotherapy estimates are grouped EFS for astrocytoma and glioblastoma multiforme) (N=27)	AA/GBM year PFS 0% (N=56)	Chemo versus ABMR unstratified comparison of event-free survival: P=0.014	Finlay, 2008
		Other Glioma ~ 73 (9 AA and 2 Oligodendroglioma) (N=11)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005
	5 Year	AA/GBM PFS: 22±7% (These HSCT and chemotherapy estimates are grouped EFS for astrocytoma and glioblastoma multiforme) (N=27)	AA/GBM year PFS 0% (N=56)	Chemo versus ABMR unstratified comparison of event-free survival: P=0.014	Finlay, 2008
		Other Glioma ~ 73 (9 AA and 2 Oligodendroglioma) (N=11)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005
Glioblastoma Multiforme	**1 Year**	AA/GBM PFS: 22±7% (These HSCT and chemotherapy estimates are grouped EFS for astrocytoma and glioblastoma multiforme) This AA PFS remained constant up to 5-years follow up (N=27)	1 AA/GBM year PFS 0% (N=56)	Chemo versus ABMR unstratified comparison of event-free survival: P=0.014	Finlay, 2008
			2 patients progressed at 1 and 4.2 mo (N=2)		Shih, 2008
			Median 6 mo (5-12) (86) 1 patient is alive without progression at 15+ mo (N=5)		Korones, 2006
			1 patient progressed at 11mo (N=1)		Sio, 2006
		~40 (N=10)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005
		64±14 (N=11)			Grovas, 1999

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
		4 patients progressed at 2, 3, 4 and 7 months (N=4)			Jakacki, 1999
		1 patient was alive with no progression at last FU (N=1)			Busca, 1997
	3 Year	AA/GBM PFS: 22±7% (These HSCT and chemotherapy estimates are grouped EFS for astrocytoma and glioblastoma multiforme) This AA PFS remained constant up to 5-years follow up (N=27)	1 AA/GBM year PFS 0% (N=56)	Chemo versus ABMR unstratified comparison of event-free survival: P=0.014	Finlay, 2008
		~20 (N=10)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005
		2 year PFS: 46±14 (N=11)			Grovas, 1999
		1 patient progressed at 34months (N=1)			Mahoney, 1996
	5 Year	AA/GBM PFS: 22±7% (These HSCT and chemotherapy estimates are grouped EFS for astrocytoma and glioblastoma multiforme) This AA PFS remained constant up to 5-years follow up (N=27)	1 AA/GBM year PFS 0% (N=56)	Chemo versus ABMR unstratified comparison of event-free survival: P=0.014	Finlay, 2008
		0 (N=10)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
Anaplastic Ependymoma	**1 Year**		91% (76-100%) (N=12)	Neurosurgical estimate of GTR vs. < GTR, P=.0001 Post-op radiographic residual tumor 1.5cm^2 vs < 1.5cm^2, P<.0001 No difference found for anaplastic vs. non-anaplastic progression	Robertson, 1998
		1 patient progressed at 27 mo (25) (N=4)			Ayan, 1995
	3 Year		65% (38-93%) (N=12)	Neurosurgical estimate of GTR vs. < GTR, P=.0001 Post-op radiographic residual tumor 1.5cm^2 vs < 1.5cm^2, P<.0001 No difference found for anaplastic vs. non-anaplastic progression	Robertson, 1998
	5 Year		35.2±11.0% (N=23)	Grade II vs. anaplastic P=.005	Jaing, 2004
			~14% (N=31)		Horn, 1999
			Complete resection 70% (± 14) (N=10) incomplete resection 36% (± 15) I (N=11) 52% (±11) all anaplastic ependymoma (N=21)	Resection: P=.09	Kuhl, 1998

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
			47% (17-61%) (N=12)	Neurosurgical estimate of GTR vs. < GTR, P=.0001 Post-op radiographic residual tumor 1.5cm^2 vs < 1.5cm^2, P<.0001 No difference found for anaplastic vs. non-anaplastic progression	Robertson, 1998
Non-anaplasitc, mixed, or unspecified Ependymoma	**1 Year**		~88 (N=23)	Complete vs. partial resection not significant	Conter, 2009
			2 patients progressed at 1 mo and 1 at 1.4 mo (N=2)		Shih, 2008
			Non-metastatic: ~87% (N=80) Metastatic ~62.5% (N=9)		Grundy, 2007
		~63% (N=29)		EFS across the three age groups: <18 months, 18–35 months and 36 months significant difference P=0.04 GTR vs <GTR not significant	Zacharoulis, 2007
			2 patients progressed at 2 and 3 mo (N=2)		Sio, 2006
			~92% (N=64)		Merchant, 2002
				Median 2.1mo (.0-30.3) (N=13)	Hurwitz, 2001

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
			56% (N=73)	Posterior Fossa Tumor RR 2.1 (1-2.5) P=.05 Postoperative radiologic documented residuum RR 2.9 (1.6-5.1) P=.0004	Grill, 2001
		~22 (N=15)			Mason, 1998
		57 (31-83 95% CI) (N=14)			Grill, 1996
		1 patient progressed at 12 mo (N=7)			Mahoney, 1996
		75% (56-95%) (N=20)		Neurosurgical estimate of GTR vs. < GTR, P=.0001 Post-op radiographic residual tumor 1.5cm^2 vs < 1.5cm^2, P<.0001 No difference found for anaplastic vs. non-anaplastic progression	Robertson, 1995
	3 Year		3 year PFS: 62.5 54.2 (N=23)	Complete vs. partial resection not significant	Conter, 2009
			metastatic: ~46% (N=80) Metastatic 0% (N=9)		Grundy, 2007
		~28% (N=29)		EFS across the three age groups: <18 months, 18–35 months and 36 months significant difference P=0.04 GTR vs <GTR not significant	Zacharoulis, 2007

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
			:~71% (N=64)		Merchant, 2002
			23% (N=73)	Posterior Fossa Tumor RR 2.1 (1-2.5) P=.05 Postoperative radiologic documented residuum RR 2.9 (1.6-5.1) P=.0004	Grill, 2001
		0% (N=15)			Mason, 1998
		27 (0-55 95% CI) (N=14)			Grill, 1996
		54% (31-76%) (N=20)		Neurosurgical estimate of GTR vs. < GTR, P=.0001 Post-op radiographic residual tumor 1.5cm^2 vs < 1.5cm^2, P<.0001 No difference found for anaplastic vs. non-anaplastic progression	Robertson, 1995
	5 Year		54.2% (N=23)	Complete vs. partial resection not significant	Conter, 2009
			Non-metastatic: ~39% (N=80) Metastatic 0% (N=9)		Grundy, 2007

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
			Grade II: 67.5±11.0% (N=20) Age<3 (N=9) 22.2±13.9 Age>3 (N=34): 52.2±9% GTR (N=18): 71.8±10.7% STR (N=19): 30.7±11.3 Bioposy (N=6): 16.7±15.2% RT involved field (N=31) 52.3±9.3% RT without involved field (N=12): 31.3±14%	Gr II vs. Anaplastic P=.002 Age: P=.005 Surgical Resection: P<.001 Radiotherapy: P=.029	Jaing, 2004
			12% (N=73)	Posterior Fossa Tumor RR 2.1 (1-2.5) P=.05 Postoperative radiologic documented residuum RR 2.9 (1.6-5.1) P=.0004	Grill, 2001

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
			Overall: 42.2±5.5% (N=83) <3 years (N=29) ~18 >3 years (N=54): ~56 <GTR (N=48): ~23 GTR (N=35): ~71 Grade II (N=51): ~53	Age<3, Age >3: P<.01 Gender: P<.01 GTR: P<.01 Residual Disease by scan: P<.01 Histology GrII vs GrIII: P<.01	Horn, 1999
			54% (31-76%) (N=20)	Neurosurgical estimate of GTR vs. < GTR, P=.0001 Post-op radiographic residual tumor 1.5cm^2 vs < 1.5cm^2, P<.0001 No difference found for anaplastic vs. non-anaplastic progression	Robertson, 1998
		2 patients alive with no disease progression at last follow up (N=2)			Busca, 1997
		14 (0-37 95% CI) (N=14)			Grill, 1996
CPC	**1 Year**		28.9% (9.0-48.0)% (N=15)		Grundy, 2010
			~56% (N=29)	CPC vs. CPP/APP HR=15.2, P<.0001 Chemotherapy yes vs. no, HR=6.4, P=.004	Wrende, 2009

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
		1 patient progressed at 4 months (N=1)			Gururangan, 1998
	3 Year		28.9% (9.0-48.0)% (N=15)		Grundy, 2010
			~56% (N=29)	CPC vs. CPP/APP HR=15.2, P<.0001 Chemotherapy yes vs. no, HR=6.4, P=.004	Wrende, 2009
	5 Year		21.7(5.3-45.1)% (N=15)	CPC vs. CPP/APP HR=15.2, P<.0001 Chemotherapy yes vs. no, HR=6.4, P=.004	Grundy, 2010
			~36% (N=29)	28 (7-100)% (N=29)	Wrende, 2009
Other Glioma	**1 Year**		HGG: 52.6 (33.2-763) (N=19)		Grundy, 2010
		1 patient with oligodendroglioma progressed at 8 mo. (N=1) 1 patients with anaplastic glioma progressed at 3 mo(N=3)			Thorarinsdottir, 2007
			BSG Median 1 mo (0-5 mo 95% CI) BSG 13 (0-35% 95% CI) (N=8)		Sio, 2006
			1 BSG patient progressed at 4 months, one at 8 months (N=2)		Korones, 2006
		1 year PFS: Other Glioma ~ 73 (9 AA and 2 Oligodendroglioma) (N=11)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
			BSG median 2.9mo (.1-19.8) (N=15) Malignant glioma Median 1.4mo (.4-7.2) (N=13) Miscellaneous glioma median 2.1mo (.6-12.9) (N=12)		Hurwitz, 2001
		1 year PFS Pontine glioma ~3 (N=35)			Bouffet, 1999
		5 Pontine glioma patients progressed Median 5 mo (3-12) (N=6) 1 patient was progression free at 12 months. (N=6)			Jakacki, 1999
		1 ODG patient was alive and progression free at last follow up (N=1)			Busca, 1997
		1 BSG patient progressed at 1 mo (N=1)			Mahoney, 1996
	3 Year		HGG: 24.1(7.8-45.1) (N=19)		Grundy, 2010
		2 patients with anaplastic glioma progressed at 17, and 33.5 mo (N=3)			Thorarinsdottir, 2007
			BSG 0		Sio, 2006
		3 year PFS: Other Glioma ~ 73 (9 AA and 2 Oligodendroglioma) (N=11)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005
	5 Year		HGG: 18.1 (4.6-38.6) (N=19)		Grundy, 2010
		1 patient with ganglioma progressed at 59 mo (N=1)			Thorarinsdottir, 2007

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
		5 year PFS: Other Glioma ~ 73 (9 AA and 2 Oligodendroglioma) (N=11)		PFS for glioblastoma multiforme compared to other histotypes (AA and ODG) were significantly worse P=.008	Massimino, 2005
			Malignant Glioma 36 ± 10 (N=22)		Kuhl, 1998
Astrocytoma	**1 year PFS N ≥ 10**	**Recurrent/Progressive:** ~30% (Finlay N=27) [This estimate includes glioblastoma multiforme tumor types] Measured from time of myleoablative chemotherapy	**Recurrent/Progressive:** ~10 (Finlay N=56) [This estimate includes glioblastoma multiforme tumor types] Finlay et al measured from time of tumor recurrence		
Astrocytoma	**1 year PFS N ≥ 10**	**Newly Diagnosed: ~73%* (Massimo, 2005) [*This study included 9 Anaplastic Astrocytoma patients and 2 lower-grade oligodendroglioma patients.] Massimo measured from time of diagnosis**			
Glioblastoma Multiforme	**1 year PFS N ≥ 10**	**Recurrent/Progressive:** ~30% (Finlay N=27*) [*This estimate includes anaplastic astrocytoma tumor types] Measured from time of myleoablative chemotherapy	**Recurrent/Progressive:** ~10-42% (Finlay* N=56, Korones** N=7) [*This estimate includes glioblastoma multiforme tumor types, ** 1 patient DOD before progression] Finlay et al measured from time of tumor recurrence Korones time measurement uncertain		

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
Glioblastoma Multiforme	**1 year PFS N ≥ 10**	**Newly Diagnosed:** Grovas measured from time of stem cell rescue Massimo considered OS from date of chemotherapy 64 grovas (N=11) ~40 Massimo (N=10)	**Recurrent/Progressive:** **(Korones N=7)** **14%**		
Ependymoma	**5 year PFS for studies with N ≥10 patients**	**Newly Diagnosed** 12% (Zacharoulis (N=29)) Zacharoulis estimated PFS from date of diagnosis	**Newly Diagnosed Non-anaplastic, mixed, or unspecified Ependymoma:** 12-67% (Conter (N=23), Grill,2001 (N=73), Horn (N=51), Jaing (N=20), Robertson (N=20)) Conter and Jaing estimated OS from date of surgery, Grill measured from date of chemotherapy, Robertson measured from date of randomization, and horn measured from date of diagnosis. **Newly Diagnosed Anaplastic Ependymoma**: 14-52% (Jaing (N=23) , Horn (N=31), Kuhl (N=21), Robertson (N=12)) Jaing used date of surgery for PFS calculation, Horn used date of diagnosis, Kuhl used date of chemotherapy, and Robertson used date of randomization		Grundy *et al*. was not included in this estimate because the study stratified by metastasis finding a 5 year PFS of 0% for metastatic ependymoma and 46% for non-metastatic disease and measured the PFS from date of surgery.

Setting	Outcome	Intervention Single (%; ± 95% CI)	Comparator Chemo (%; ± 95% CI)	P-value	Study
Ependymoma	**5 year PFS for studies with N ≥10 patients**	**Recurrent/Progressive:** 14% (Grill, 1996 (N=14)) Grill measured PFS from date of autologous bone marrow transplant			Grundy *et al*. was not included in this estimate because the study stratified by metastasis finding a 5 year PFS of 0% for metastatic ependymoma and 46% for non-metastatic disease and measured the PFS from date of surgery.
CPC	**5 Year PFS All studies:**	1 patient progressed at 4 mo (Gururangan (N=1)) Gururangan assessed EFS after myleoablative chemotherapy	21.7-36% (Grundy (N=15) and Wrede (N=29)) Wrede measured EFS from date of diagnosis and Grundy used date of surgery		
AA= anaplastic ependymoma, AWD= Alive with disease, BSG, Brain stem glioma; CPC, Choroid plexus carcinoma; DOD=Dead of disease, GBM=Glioblastoma multiforme; HGG, high-grade glioma					

Appendix E: Neurodevelopmental and Neurocognitive Outcomes

Table E1: Neurocognitive and neurodevelopmental outcomes for treatment (HSCT) of Inherited Metabolic Diseases with Rapid Progression

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
Wolman disease	nr	pt 1 at 11 yrs post: mildly impaired cognitive abilities, sustained visual attention impaired, verbal fluency avg, attends special school, mostly homeschooled, no behavior problems, English and Spanish language development pt 4 at 4 yrs post: cognition improved from baseline, receptive and expressive language high avg, adaptive skills avg, emotional and social behavior avg, attends special education preschool, speaks 3 languages	pt 1: considerable developmental delay pt 4: nr	pt 1 at 11 yrs post: motor function improved pt 4 at 4 yrs post: fine motor skills below avg, gross motor skills avg	Tolar J, US, 2009 (1370), HSCT, case series (N=4)
	nr	nr	failure to thrive	nr	Gramatges MM, US, 2009 (83290), HSCT, case report (N=1)
	MRI showed 0.5 yr delay in myelination	MRI showed appropriate myelination for age at 1 yr post at 4 yrs post, pt has normal intellectual development, attends regular school, and speaks Russian and Hebrew	weight, height, and head circumference at <3rd percentile	at 4 yrs post: weight 10th percentile, height 3rd percentile, head circumference 3rd percentile	Stein J, Israel, 2007 (4880), HSCT, case report (N=1)

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
Niemann-Pick Type A	neurologically intact	neurological regression: alert, socially engaged, verbalizing appropriately for age at 0.6 yrs post brain CT shows bilateral cerebral atrophy at 0.6 yrs post limited social interaction , seizure disorder developed at 1.7 yrs post	nr	alert, active, interactive, rolling back to front to back at 0.6 yrs post head lag and hypotonic at 1 yr post significant developmental delay, unable to sit or stand at 1.7 yrs post	Morel CF, Canada, 2007 (3010), HSCT, case report
	pt 1 and 2: normal MRI/CAT, EEG pt 1: Denver Developmental Exam, 2-3 mos (real age 10 mos) pt 2: Gessell Schedules, appropriate for age at 4 mos	pt 1 and 2: neurological deterioration seen in MRI/CAT, EEG pt 2 at 6 mos post: mild developmental delay, cognitive skills at 7-8 mos (real age 11 mos) pt 2 at 12 mos post: moderate developmental delay, cognitive skills 12 mos (real age 167mos)	pts 1 and 2: hyponic, depressed reflexes	pt 2 at 6 mos post: moderate developmental delay, motor skills at 6 mos (real age 11 mos) pt 2 at 12 mos post: severe developmental delay, motor skills at 6 mos (real age 17 mos)	Bayever E, US, 1995 (25460), HSCT, case series (N=2)
Mucolipidosis II (I-cell disease)	nr	nr	failure to thrive	nr	Li CK, China, 2004 (9070), HSCT, case series (n=1)
	real age: 1.4 yrs expressive language and receptive language: 0.9 yrs	real age: 3.0 yrs, developmental age: 1.6 yrs real age: 3.5 yrs, developmental age: 2.1 yrs real age: 4.7, developmental age: 3.3 yrs real age: 5.7 yrs, developmental age: 4.3 yrs real age: 6.7 yrs, developmental age: 5.3 yrs attends school with individualized education program; slow progress in communication, daily living, socialization, and expressive language; mild to moderate cognitive impairment	real age: 1.4 yrs developmental age: 0.9 yrs	real age: 3.0 yrs, gross motor age: 1.2 yrs real age: 3.5 yrs, gross motor age: 1.3 yrs real age: 4.7 yrs, gross motor age: 1.5 yrs real age: 5.7 yrs, gross motor age: 1.5 yrs real age: 6.7 yrs, gross motor age: 1.5 yrs gross motor skills impaired fine motor skills slowly developing	Grewel S, US, 2003 (9750), HSCT, case report

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	nr	exhibiting emotional expressions	moderate to severe joint contractures, marked short stature, dystosis multiplex severe psychomotor retardation	no change in joint contractures still severe psychomotor retardation, but gained 4-8 mo-old skills of sitting up and using walker	Imaizumi M, 1994, Japan, (23220B), HSCT, case series (n=1)

Table E2: Neurocognitive and neurodevelopmental outcomes for treatment (HSCT) and comparators (ERT, substrate reduction therapy) of Inherited Metabolic Diseases with Slow Progression

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
MPS II (Hunter's disease)	**Severe form:** IQ/DQ: pt 2: 72 pt 4: 70 pt 5: 70 pt 6: 65 pt 8: 100	**Severe form:** IQ/DQ: pt 2: 60, very poor language pt 4: <50, no language pt 5: <50, speech loss 3 yrs post pt 6:<50, speech loss 8 yrs post, pt 8: <50, poor language all 5 attend special schools	**Severe form:** nr	**Severe form:** 2 no motor problems 3 bedridden	Guffon, France, 2009 (680), HSCT, case series (N=8)
	Attenuated form: IQ/DQ: pt 1: 125 pt 3: 87 pt 7: 100	**Attenuated form:** IQ/DQ: pt 1: 110, normal language pt 3: 65, poor language pt 7: 100 2 attend mainstream school, 1 attends special apprenticeship 3 sociable	**Attenuated form:** nr	**Attenuated form:** 3 no motor problems	
	Form not specified: nr	**Form not specified:** nr	**Form not specified:** nr	**Form not specified:** nr	Page, US, 2008 (1280B), HSCT, case series (n=2)
	Form not specified: real age: 5.9 yrs mental age: 2.3 yrs MRI: brain atrophy	**Form not specified:** real age: 6.5 yrs mental age: 2.5 yrs at autopsy: brain cells distended from accumulation of substrate	**Form not specified:** nr	**Form not specified:** nr	Tokimasa, Japan, 2008 (1310), HSCT, case series (n=1)
	Attenuated form: pt 1: lesions in white matter and corpus callosum pt 2: enlargement of perivascular spaces at basal ganglia, intensity changes in periventricular white matter pt 3: lesions in parietal and occipital lobes, intensity in white matter	**Attenuated form:** pt 1: no follow-up MRI pt 2: no change at 7 yrs post pt 3: lesions slightly diminished at 2.5 yrs post	**Attenuated form:** nr	**Attenuated form:** nr	Seto, Japan, 2001 (13460A), HSCT, case series (n=3)

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	Severe form: DQ and MRI findings: 1 HSCT pt: 72, ventricular dilatation present, white matter lesions 2 non-HSCT pts: 94 and 124, no ventricular dilatation, lesions in white matter	**Severe form:** DQ and MRI findings: 1 HSCT pt: 61 and 54 at 0.5 yrs and 1.1 yrs post, ventricular dilatation worsened, white matter lesions 2 non-HSCT pts: no follow-up measurement	**Severe form:** nr	**Severe form:** nr	Takahashi, Japan, 2001 (14030), HSCT, comparative study (n=1)
	Attenuated form: nr	**Attenuated form:** "developing and growing normally"	**Attenuated form:** nr	**Attenuated form:** "growing and developing normally"	Mullen, US, 2000 (15300), HSCT, case report
	Attenuated form: IQ: 72	**Attenuated form:** IQ: 69, 70, and 70 at 0.7 yrs, 2.6 yrs, and 4.0 yrs post	**Attenuated form:** significant joint limitations in hands, knees, elbows	**Attenuated form:** mild joint limitations at 0.7 yrs post minimal joint limitations at 2.6 yrs post	Coppa, Italy, 1999 (16350), HSCT, case report
	Form not specified: Griffiths Mental Development Scale: pt 1: social 61, speech 61 pt 2: social 71, speech 71 pt 3: social 93, speech 93	**Form not specified:** Griffiths Mental Development Scale: pt 1 at 10 yrs post: social 10, speech 10; steady deterioration pt 2: social 2, speech 2, steady deterioration pt 3: full IQ 78, verbal IQ 80 performance IQ 81; attends mainstream school, trouble with concentration	**Form not specified:** Griffiths Mental Development Scale: pt 1: locomotor 63, eye-hand 58 pt 2: locomotor 55, eye-hand 58 pt 3: locomotor 110, eye-hand 93	**Form not specified:** Griffiths Mental Development Scale: pt 1 at 10 yrs post: locomotor 11, eye-hand 8 pt 2 at 2.7 yrs post: locomotor 6.5, eye-hand 2.5 pt 3: nr	Vellodi, England, 1999 (16650), HSCT, case series (N=9)
	Severe form: IQ: 44	**Severe form:** IQ: 44 at 3 yrs post	**Severe form:** multiple bone abnormalities	**Severe form:** improvements in joint range of motion improvements in fine and gross motor skills	Li, US, 1996 (20260), HSCT, case report

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	Severe form: Mild behavioral difficulties	**Severe form:** Decreasing intelligence ratio (age equivalent/real age) from 0.68 at 2.8 yrs of age to 0.09 at 8.0 yrs of age Increased behavioral problems, reversion in language, communication, concentration, cooperation, and attention span.	**Severe form:** real age: 1.9 yrs developmental age: 1.3-1.5 yrs	**Severe form:** persistent skeletal deformities reversion in balance and coordination though can still walk and ride tricycle	McKinnis, US, 1996 (20560), HSCT, case report
	Form not specified: DQ: normal	**Form not specified:** DQ not reported	**Form not specified:** stiff joints dystosis multiplex	**Form not specified:** joint mobility improved dystosis multiplex stabilized	Hoogerbrugge PM, Netherlands, 1995 (21780B), HSCT, case series (n=1)
	Form not specified: Brunet-Lezine scales: mental age: 25 mos real age: 31 mos good socialization	**Form not specified:** Brunet-Lezine scales: at 3 mos post: mental age: 2 yrs real age: 3 yrs worsening of verbal capabilities, measured at 10 mos level at 20 mos post: mental age: 2.5 yrs real age: 4.5 yrs no change in verbal capabilities	**Form not specified:** mild flexion contractures good motor capabilities	**Form not specified:** joint mobility improved growth in ht and wt	Coppa GV, Italy, 1995 (21950), HSCT, case report
	Attenuated form: Stanford-Benet scales: within normal range, attends regular school	**Attenuated form:** no decrease in school performance, has plans for college	**Attenuated form:** moderate mobility impairment due to deformed bones, joints energy & activity normal ht: <5th percentile, wt: 5th-10th percentile, head circumference: 98th percentile	**Attenuated form:** moderate improvement in joint flexibility growth spurt	Bergstrom SK, US, 1994 (22650), HSCT, case report

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	Attenuated form: no CNS involvement, attends regular school	**Attenuated form:** no change	**Attenuated form:** moderate to severe join contractures nodular hypertrophy present	**Attenuated form:** improvement in joint contractures nodular hypertrophy absent	Imaizumi, Japan, 1994 (23220A), HSCT, case series (n=1)
	Attenuated form: nr	**Attenuated form:** nr	**Attenuated form:** 6-minute walk test: placebo: 374.7 ERT .15 mg/kg: 448.7 ERT .5 mg/kg: 324.3 ERT 1.5 mg/kg: 439.7	**Attenuated form:** Changes in 6-minute walk test: 6 mos: no change 12 mos: 8 improved, 4 no change	Muenzer, US, 2007 (57070), ERT, open label extension (N=12)
	Attenuated form: nr	**Attenuated form:** nr	**Attenuated form:** 6-minute walk test: placebo: 392 +/- 19 ERT EOW: 401 +/- 18 ERT wkly: 392 +/- 19	**Attenuated form:** Changes in 6-minute walk test: placebo: 7.3 +/- 9.5 ERT EOW: 30.3 +/- 10.3 (p=0.07) ERT wkly: 44.3 +/- 12.3 (p=0.01)	Muenzer, US, 2006 (57160), ERT, RCT (N=96)
MPS III (Sanfilippo disease)	nr	nr	nr	nr	Ringden, Sweden, 2006 (5940B), HSCT, case series (n=1)
	nr	no significant neuropsychological improvement	nr	nr	Lange, Brazil, 2006 (5690), HSCT, case series (n=1)
	nr	developmental quotients decreasing with age from 99 at 1.5 yrs of age to 6 at 10 yrs of age	nr	pt immobile at 7.4 yrs post, wheelchair bound like sibling who was untreated	Sivakumar, England, 1999, (16200), HSCT, comparative study (n=1)
	DQ: pt 1: 72, hyperactive pt 2: 80, monosyllabic pt 3: 38, dystonic, dysarthric	DQ: pt 1:41, dysarthric pt 2: 50, hypotonic pt 3: 26, no speech	nr	nr	Hoogerbrugge PM, Netherlands, 1995 (21780A), HSCT, case series (n=3)

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	Ruth Griffiths Mental Development: pt 1: 82 pt 2: 95 functioning in low-average range Social skills: pt 1 and 2: normal	Ruth Griffiths Mental Development: pt 1: 35-40 pt 2: 15-25 significant developmental delays, both attend special school though scores decreased, it is unknown if the decreases would have been greater without HSCT; untreated brothers are severely retarded Social skills: pt 1: normal pt 2: anti-social	Overall growth: pt 1 and 2: normal	Overall growth: pt 1: pubertal development pt 2: has no pubertal development Untreated brothers are wheelchair-bound; unclear if neurodevelopment better for treated sisters due to treatment or if differences due to heterogeneic variations of disease	Vellodi A, England, 1992, (25600), HSCT, case series (N=2)
MPS IV (Morquio syndrome)	no pathological findings in brain or spinal cord MRI	nr	mild bone deformities	nr	Seto, Japan, 2001 (13460B), HSCT, case series (n=1)
	nr	nr	aortic stenosis left ventricular dilatation	no change	Gatzoulis MA, England, 1995 (21610), HSCT, case series (n=1)
Gaucher Type 3	nr	borderline mental retardation in 2 pts	bone problems in 1 pt	bone problems stable in 1 pt	Goker-Alpan 2008, US, HSCT followed by ERT, case series (n=2)
	Weschler Intelligence Scales: performance: 67 verbal: 69 complete: 67	Weschler Intelligence Scales at 1.5 yrs post: performance: 60 verbal: 69 complete: 62	nr	stable growth and improved bone density	Chen R, Taiwan, 2007 (4490), HSCT, case report

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	Weschler Intelligence Scales: pt 1: stanine 7	Weschler Intelligence Scales: pt 1: stanine 7, 5, 6, 7, 7 (at 1 yr, 3 yrs, 5 yrs, 8 yrs, 10 yrs post); IQ=112-120 pt 2: stanine 7 (at 6 yrs post) pt 3: stanine 3 (at 4 yrs post) pt 4: below age (did not engraft, on ERT) pt 5: RA**: 5; DA**: 5 pt 6: RA: 5; DA: 3 (at 1 yr post) pre-transplant data not given for pts 2, 5, and 6, but authors state psychological development was excellent in these 3 pts	Skeletal involvement: pts 1, 5, 6: kyphosis pts 2, 3, 4: no kyphosis Growth: below average	Skeletal involvement pts 1, 5, 6: kyphosis pts 2, 3, 4: no kyphosis Growth: All 6 have growth spurt, even pt 4 who did not engraft and is on ERT	Ringden O, Sweden, 1995 (22020), HSCT, case series (N=6)
	RA**: 22 mos; DA**: 15 mos Developmental Quotient=68	RA: 33 mos; DA: 21 mos; DQ=64 at 0.7 yrs post RA: 39 mos; DA: 25 mos; DQ-=64 at 1.1 yrs post bilingual at 1.6 yrs post	Failure to thrive, height < 3rd percentile	Height at 10th percentile at 9 mos post Height at 50th percentile at 2 yrs post	Tsai P, US, 1992, (25120), case report
	nr	No statistically significant differences between study grps using Purdue Peg Board test, Wechsler Scale, Benton visual retention test, Rey auditory verbal learning test, d2 test of attention, continuous performance test, and Trail Making Test.	nr	No treatment effect on Vertical Saccadic Eye Movement Study may not have been long enough for neurological defects to improve, or neurological defects are irreversible.	Schiffman R, Netherlands, 2008 (56750), substrate reduction therapy with ERT, RCT (N=30)
	nr	nr	Grading severity level of marrow involvement: 0A level: 3 pts 2A level: 6 pts 3A level: 1 pt 3B level: 1 pt	0A level: 1 constant and 2 worsened 2A level: 5 complete improvement and 1 constant 3A level: 1 constant 3B level: 1 constant	El-Beshlawy A, Egypt, 2006 (5750), ERT, case series (n=11)
	nr	Behavioral and learning difficulties developed after stopping ERT Recurrent seizures 2.6 yrs after stopping ERT	height <3rd percentile	improved growth	Chan LL, Malaysia, 2002 (11330), ERT, case report

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	nr	nr	nr	no change in skeletal deformities	Banjar H, Saudi Arabia, 1998 (17920), ERT, case series (n=3)
	3 mild-moderate mental retardation 2 normal IQ	no change in IQ 1 showed clinical function deterioration cerebrospinal fluid measurements showed that glucocerebrosidase delivery to the cerebrospinal fluid was minimal (not significantly different)	nr	nr	Schiffmann R, Netherlands, 1997 (58150), ERT, case series (n=5)
	EEG normal for all pts Weschler Intelligence Scales: pt 1: 82-88 Griffith Scale: pt 2: 82-88 pt 3: 104-111	EEG normal for all pts Weschler Intelligence Scales: pt 1: 89-96 at 1.3 yrs post Griffith Scale: pt 2: 74-81 at 1 yr post pt 3: 97-103 at 1 yr post all 3 pts became more active and needed less sleep pts 2 and 3 were tired and slow and became active pre-schooler post treatment	pt 1: femur deformity, kyphosis, cortex thinning pt 2: grew 2 cm/yr pt 3: grew 4 cm/yr, femur deformity	pt 1: no change in skeletal deformities pt 2: grew 9 cm 1 yr post pt 3: grew 12 cm 1 yr post, no change in skeletal deformities	Erikson A, Sweden, 1995 (21630), ERT, case series (n=3)
Aspartyl-glucos-aminuria	pt 1: developmental age 4.7 yrs below real age pt 2: nr	pt 1 and 2: developmental age stabilizes at 5 yrs pt 1 and 2: mentally retarded, speaks in sentences, understands Swedish and Finnish words	nr	pt 1: can walk, ride bike, dress self pt 2: can walk, ride bike, drive tractor, some fine motor skills	Malm G, Sweden, 2004 (8490), HSCT, case series (N=2)
	nr	5 HSCT: developmental age was on average 5 yrs lower than real age 12 non-HSCT: developmental age was on average 3.4 yrs lower than real age HSCT pts may have lower developmental ages because 2 pts with more severe disease were chosen for HSCT	nr	Dysmorphic Facial and Body Features remained unchanged following HSCT	Arvio M, Finland, 2001 (14180), HSCT, comparative study (n=5)

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	2 HSCT: poor cortex-white matter differentiation, decreased thalami signal intensity 6 non-HSCT: poor cortex-white matter differentiation, decreased thalami signal intensity	2 HSCT: slight improvement from poor to evident cortex-white matter differentiation, improvement in thalami signal intensity, improvement in concentration and cooperation	both HSCT pts: gross motor clumsiness, slight balance problems	nr	Autti T, Finland, 1999 (15540), HSCT, comparative study (n=2)
	mild global delay	nr	nr	nr	Laitinen A, Finland, 1997 (19620), HSCT, case report
Niemann-Pick Type C	real age: 2.4 yrs developmental age: 0.8-1.2 yrs developmental regression began prior to transplant, no speech development in previous yr MRI pre-transplant showed normal myelination and no obvious brain atrophy	real age: 2.6 yrs, developmental age: 0.4-0.7 yrs real age: 2.9 yrs, developmental age: 0.3-0.4 yrs real age: 3.3 yrs, developmental age: 0.2-0.3 yrs MRI 0.5 yrs post-transplant showed normal myelination and evident brain atrophy	1.2 yrs: sat without support and crawled 2.4 yrs: pt became bed-ridden during conditioning phase	6-9 mos post: head lag, could not raise body	Hsu YS, Taiwan, 1999 (16540), HSCT, case report
	MRI: normal brain activity	MRI: developing neurologically, but with delayed speech	mildy hyptonic normal developmental milestones (standing)	fine motor coordination (can hold pencil, draw) at 1.7 yrs post tolerates normal activity walks independently	Bonney DK, England, 2009, (81700) HSCT, case report
	nr	nr	Standard ambulation index: 2.0 (0.7-3.3)	Standard ambulation index: 1 yr: 2.3 (0.6-4.0) 2 yrs: 2.6 (0.7-4.5) 8 of 10 pts are considered stable in ambulation	Patterson MC, US, 2010 (56500), substrate reduction therapy, open label extension (N=12)

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study treatment, study design (N)
	nr	Change in composite disability score combined pediatric and adult pts: greater treatment effect was seen in subset of those with neurological disease	at diagnosis, mean scores: ambulation: 0.18, manipulation: 0.27, language: 0.16, swallowing: 0.12 at start of treatment: overall deterioration of scores	at last clinical visit, % with stable/improved scores: ambulation: 76.6%, manipulation: 76.2%, language: 77.0%, swallowing: 81.0%	Pineda M, Spain, 2009 (56560)*, substrate reduction therapy, retrospective cohort (N=66)
	modest cognitive abilities	3 mos: some improvement in adaptive social domains 6 mos: regression, speech decline 12 mos: <0.1 percentile in developmental scales	proximal weakness in extremities ataxic hand tremor motion analysis: walked 0.24 m/sec, 62 steps/min	3 mos: hand tremor diminished 9-12 mos: lost ability to walk motion analysis at 6 mos: walked 0.12 m/sec, 32.4 steps/min	Paciorkowski AR, US, 2008 (2980), substrate reduction therapy, case report
	nr	Mini-mental status examination data only provided for pts >=12 yrs: difference between treated and untreated groups, p=0.165	nr	Ambulatory index data only provided for pts >=12 yrs: difference between treated and untreated groups, p=0.052	Patterson MC, US, 2007 (56970)*, substrate reduction therapy, RCT (n=12)

*cannot separate adult and pediatric data within this study
**RA: real age, DA: developmental age

Table E3: Neurocognitive and neurodevelopmental outcomes for treatment (HSCT) and comparators (ERT, substrate reduction therapy) of Inherited Metabolic Diseases with both Rapid and Slow Progression

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study (record #), treatment, study design (N)
Farber disease	**Type 2/3, with no CNS involvement** nr	**Type 2/3, with no CNS involvement** nr	**Type 2/3, with no CNS involvement** # subcutaneous nodules: pt 1: 58 pt 2: 39 pt 3: 18 # joints with limited motion: pt 1: 26 pt 2: 24 pt 3: 10	**Type 2/3, with no CNS involvement** # subcutaneous nodules: pt 1: 8 at 1.2 yrs post pt 2: 14 at 0.5 yrs post pt 3: 0 at 0.7 yrs post # joints with limited motion: pt 1: 2 at 1.2 yrs post pt 2: 4 at 0.5 yrs post pt 3: 4 at 0.7 yrs post	Ehlert K, Germany, 2006 (4690), HSCT, case series (N=3)
	Type 2/3, with no CNS involvement nr	**Type 2/3, with no CNS involvement** nr	**Type 2/3, with no CNS involvement** # subcutaneous nodules: pt 1: 58 pt 2: 39 # joints with limited motion: pt 1: 26 pt 2: 24	**Type 2/3, with no CNS involvement** # subcutaneous nodules: pt 1: 8 pt 2: 12 # joints with limited motion: pt 1: 2 pt 2: 2	Vormoor J, Germany, 2004 (9420), HSCT, case series (N=2)
	Type 1, with CNS involvement normal myelination at 0.75 yrs Bayley Scales of Infant Development: developmental age and real age equivalent at time of transplant (0.75 yrs)	**Type 1, with CNS involvement** normal myelination at 0.3 yrs post, decrease in grey and white matter differentiation at 0.7 yrs post, poor grey and white matter contrast at 1.3 yrs post development age plateaued at 0.6 yrs at real age of 1.3 yrs and 2.1 yrs	**Type 1, with CNS involvement** wt, ht, and head circumference: 10th-25th percentile	**Type 1, with CNS involvement** wt, ht, and head circumference: 5th percentile at 0.8 yrs post <5th percentile at 1.5 yrs post	Yeager AM, US, 2000 (14880), HSCT, case report
	Type 1, with CNS involvement mental regression	**Type 1, with CNS involvement** mental regression worsened, cerebral atrophy seen in brain imaging	**Type 1, with CNS involvement** unable to stand decreased tendon reflexes	**Type 1, with CNS involvement** regression of motor abilities increasing tremor	Hoogerbrugge, PM, Netherlands, 1995 (21780D), HSCT, case series (n=1)

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study (record #), treatment, study design (N)
GM_1 ganglio-sidosis	**juvenile form:** nr	**juvenile form:** normal language development at 0.6 yrs post language declining at 1.7-2.1 yrs post demyelination and diffuse cerebral function at 2.4 yrs post no language at 4.0 yrs post	**juvenile form:** nr	**juvenile form:** walking at 0.6 yrs post became clumsy at 1.7-2.1 yrs post limited motor skills at 4.0 yrs post wheelchair at 6.0 yrs post	Shield JPH, England, 2005 (6720), HSCT, case report
Tay-Sachs disease	**form not specified:** nr	**form not specified:** nr	**form not specified:** nr	**form not specified:** nr	Page KM, US, 2008 (1280A), HSCT, case series (n=1)
	form not specified: mental regression brain imaging showed widened subarachnoidal spaces	**form not specified:** vegetative state no brain imaging follow-up	**form not specified:** psychomotor retardation myoclonic jerks	**form not specified:** vegetative state	Hoogerbrugge PM, Netherlands, 1995 (21780C), HSCT, case series (n=1)
	juvenile form: nr	**juvenile form:** MRI shows cerebral atrophy at 0.5 yrs post worsening neuropsychological test scores at 0.5 yrs post speech deteriorating at 0.5 yrs post	**juvenile form:** nr	**juvenile form:** motor skills deteriorating at 0.5 yrs post Deterioration of this pt similar to deterioration of untreated older sister	Jacobs JFM, Netherlands, 2005 (6740), HSCT with substrate reduction therapy added at 2 yrs post, case report
	juvenile form: pt 1: mild cognitive impairment, attends regular school with assistance pt 2: severe cognitive impairment, generalized seizures	**juvenile form:** pt 1: at 15 mos acute psychotic event pt 2: at 15 mos marked increase in seizures, alertness deteriorated, at 24 mos spasticity increased	**juvenile form:** pt 1: mild muscle weakness, moderate muscle impairment, independent feeding and ambulation pt 2: needs support for ambulation	**juvenile form:** pt 1: at 6 mos handwriting deteriorated, at 12 mos fine tremor in hands, from 12-24 mos, progressive muscle atropy pt 2: at 15 mos muscle bulk decreased markedly, at 24 mos wheelchair dependent	Maegawa GHB, Canada, 2009 (56590B), substrate reduction therapy, single arm (n=2)

Disease	Neurocognitive Pre-Intervention	Neurocognitive Post-Intervention	Neurodevelopmental Pre-Intervention	Neurodevelopmental Post-Intervention	Study (record #), treatment, study design (N)
ceroid lipo-fuscinosis	cerebral cortical atrophy: moderate in one pt, not detectable in 2 pts periventricular white matter hyperintensity: mild in 1 pt, not detectable in 2 pts	cerebral cortical atrophy: moderate became severe in one pt, not detectable became moderate in two pts periventricular white matter hyperintensity: mild became severe in one pt, not detectable became moderate in two pts	one pt mildly symptomatic and two pts asymptomatic	all three pts by end of follow-up at 2-4 yrs of age were hypotonic and spastic, with some head control remaining	Lonnquist T, Finland, 2001 (12960), HSCT, case series (N=3)
Sandhoff's disease	nr	nr	nr	nr	Ringden O, Sweden, 2006 (5940B), HSCT, case series (n=1)
	pt 1: severe cognitive dysfunction, hallucinations, agitation, scores 1.5 yrs below age pt 2: episodic psychosis, cognitive function well-preserved, works part time pt 3: 2 episodes of psychosis, IQ=75	pt 1: neuropsych scores unchanged pt 2: 18 mos post, neuropsych scores stable, speech less intelligible, hallucinations reduced, anxiety ongoing pt 3: at 16 mos post, spasticity developed, anxiety aggravated, neuropsych scores stable	pt 1: muscle wasting, fully dependent for feeding and ambulation pt 2: moderate skeletal muscle weakness, independent ambulation, feeding, bathing pt 3: independent ambulation, feeding, and bathing	pt 1: 3 mos incoordination progressed, 15 mos wheelchair, 21 mos can't stand pt 2: at 18 mos gait disturbance progressed & muscle strength reduced pt 3: 6 mos gait disturbance, 16 mos notable wt loss pt 2 and pt 3 stopped tx at 21 mos due to excessive weight loss	Maegawa GHB, Canada, 2009 (56590A), substrate reduction therapy, single arm (n=3)

Appendix F: C-Peptide and HbA1c Outcomes

In all studies, serum C-peptide levels were measured using radioimmunoassay. To accommodate differences in data presentation and analysis, they are presented in the tables as a percentage change from values at study entry.

C-peptide Level

Data on C-peptide levels were reported in all three studies included in this review, at follow-up times that range from 6 months in one IIT study (Crino et al, 2005, rec#23080) to more than 4 years in the HSCT study (Couri et al, 2009, rec#290) (Table F1). The proportional change in C-peptide levels in the HSCT study refer only to patients who remained continuously insulin free.

Table F1. C-Peptide Levels Following Autologous HSCT or IIT in Pediatric Patients

Outcome	Intervention % Δ in Mean Value	Comparator % Δ in Mean Value	p-value (vs Mean Baseline Value)	Study (rec#)
6 months	+ ~ 113 (n = 11)		< 0.001	Couri et al, 2009 (290)
1 year	+ ~ 100 (n = 12)		0.001	
2 year	+ 249 (n = 8)		< 0.001	
3 year	+ 224 (n = 3)		0.001	
4 year	NR (n = 1)		NR	
1 year		+ 131 (n = 27)	NS	Crino et al, 2005 (23080)
2 year		+ 119 (n = 27)		
6 months		- 20 (n = 8)	0.05	Mastrandrea et al, 2009 (40050)

The data in Table F1 show that C-peptide levels following a mixed-meal tolerance test were significantly increased above baseline values in the HSCT study (Couri et al, 2009, rec#290) for more than two years in 8 of 12 patients who became continuously insulin free following the procedure. In one IIT study, mean fasting C-peptide levels were not changed significantly at 1 or 2 years following initiation of treatment from that at study entry (Crino et al, 2005, rec#23080). In the second IIT study, the mean C-peptide level following a Boost meal test was slightly lower at 6 months following initiation of treatment than that at study entry (Mastrandrea et al, 2009, rec#40050).

Hemoglobin A1C Levels

Table F2 shows HbA1C levels in patients treated with nonmyeloablative autologous HSCT (Couri et al, 2009, rec#290) or IIT (Crino et al, 2005, rec#23080; Mastrandrea et al, 2009, rec#40050).

Hemoglobin A1C (HbA1C) levels pretransplant ranged from 5.4% to 11.6% (mean 8.4 ± 1.6%) among 18 pediatric patients in the HSCT study (Couri et al, 2009, rec#290). Among those who became continuously insulin-free, HbA1C declined from a mean 8.0% to 5.7%, 5.7%, 5.5%, and 6.0%, respectively, at 12, 24, 36, and 48 months after transplantation ($p < 0.001$ at all time points versus pretreatment value).

In one IIT study, HbA1C did not change significantly from a mean 10.5 ± 2.2% at diagnosis to 5.4 ± 0.8% and 6.5 ± 0.9% at 12 and 24 months, respectively (Crino et al, 2005, rec# 23080). In the second IIT study, mean HbA1C at study entry (12.4 ± 2.5%) declined to an average 7.0 ± 1.2% (p-value NR) (Mastrandrea et al, 2009, rec#40050).

Table F2. HbA1C Levels Following Autologous HSCT or IIT in Pediatric Patients

Outcome	Intervention % Δ in Mean Value	Comparator % Δ in Mean Value	p-value (vs Mean Baseline Value)	Study (rec#)
3 months	- 32		< 0.001	Couri et al, 2009 (290)
1 year	- 29			
2 year	- 29			
3 year	- 31			
4 year	- 25			
1 year		- 48	NS	Crino et al, 2005 (23080)
2 year		- 38		
6 months		- 44	NR	Mastrandrea et al, 2009 (40050)