

BMT #0701 Data Submission - Documentation for Outcomes Dataset

Outcomes dataset has 111 variables for 62 participants on BMT protocol #0701 and each patient has one record. This is the most important dataset in this data submission.

Notes in the last column of below table are provided by BMT CTN DCC to facilitate better understanding of the submitted datasets:

- **CRF** indicates this variable is from EMMES Case Report Form, as reported by the transplant center. The name of the CRF is shown in the column for easy reference.
- **EMMES** indicates this variable is from EMMES Enrollment System, as study implemented per protocol.
- **RECODE** indicates this variable is from computation for analysis purpose based on other data source. Algorithm and computation method are provided for reference.
- **ERC** indicates this variable is from the BMT #0701 Endpoint Review Committee adjudication. ERC adjudicated the data in a blinded manner based on the site-reported data in CRFs as well as some clinical notes from the sites. ERC –adjudicated outcomes should supersede the site-reported data if there would be any discrepancy.
- **CIBMTR** indicates this variable is data retrieval from the CIBTMR data system. CIBMTR data were reviewed by the CIBMTR physicians prior to the data transfer to Emmes DCC.
- **LAB** indicates this variable is the lab-related data retrieval from external source. For this study, Rituxan data was provided by Genentech, serum rituxan samples analyzed by Covance Laboratories Inc.

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
1	cause_death	Char	100			Primary cause of Death	ERC- this is the ERC confirmed primary cause of death (with one last case that CIBMTR provided cause of death)
2	PROJID	Char	5	\$5.	\$5.	Project ID	EMMES - this is the blinded identifier that should be used for any data merge between this dataset and other datasets. This is unique for each patient in BMT CTN
3	enrldate	Num	8	MMDDYY8.		Date of Randomization	EMMES - this is the start date of the patient on this study.
4	DTHDT	Num	8	MMDDYY8.	DATETIME22.3	Date of death	CRF - DTH
5	TXDTP	Num	8	MMDDYY8.	DATETIME22.3	Transplant Date	CRF -TXP
6	CMVSTAT	Char	1	\$CMV.	\$1.	Patient's pre-transplant CMV status	CRF -TXP
7	MAXAGVHD	Char	1	\$AGVH.	\$1.	Maximum aGVHD grade through day 100 post transplant (ERC)	ERC- this is the ERC adjudicated maximum overall grade of acute GVHD
8	AGVH24DT	Num	8	MMDDYY8.	DATETIME22.3	Date of grade 2-4 aGVHD (ERC)	ERC- this is the date of grade 2-4 acute GVHD onset post transplant
9	AGVH34DT	Num	8	MMDDYY8.	DATETIME22.3	Date of grade 3-4 aGVHD (ERC)	ERC- this is the date of grade 3-4 acute GVHD onset post transplant
10	MAXCGVHD	Char	1	\$CGVH.	\$1.	Maximum cGVHD post transplant (ERC)	ERC- this is the ERC adjudicated maximum overall grade of chronic GVHD

Variables in Creation Order in OUTCOME dataset							
#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
11	CGVHDT	Num	8	MMDDYY8.	DATETIME22.3	Date of cGVHD diagnosis (ERC)	ERC- this is the ERC confirmed date of chronic GVHD onset post transplant
12	CGVSEVMX	Char	1	\$SEVC.	\$1.	Maximum cGVHD severity post transplant (ERC)	ERC-this is the ERC adjudicated maximum severity of chronic GVHD
13	THERPYDT	Num	8	MMDDYY8.	DATETIME22.3	Date any not protocol specified therapy received (ERC)	ERC- this is the ERC confirmed date of receiving not protocol specified therapy
14	ENTRYDS	Char	1	\$ENTRY.	\$1.	Disease response at study entry (ERC)	ERC- this is the ERC confirmed disease response at study entry
15	BESTDR	Char	1	\$BESTDR.	\$1.	Best response post transplant (ERC)	ERC- this is the ERC confirmed disease response post transplant
16	BESTDRDT	Num	8	MMDDYY8.	DATETIME22.3	Date of disease response post transplant (ERC)	ERC- this is the date of best disease response post transplant
17	hct_score	Char	100			Comorbidity Index Score	CIBMTR
18	PATHDX	Char	1	\$PATHD.	1.	Patient's pathologic criteria	CRF-ENR
19	PRREGMN	Char	1	\$PRIORREG.	1.	Number of Prior Regimens received	CRF-ENR
20	DXSTATSA	Char	1	\$FOLL.	1.	Follicular lymphoma status at the time of transplantation	CRF-ENR
21	KPS	Char	2	\$KARNOF.	2.	Karnofsky performance score	CRF-ENR
22	DONORTP	Char	1	\$DONOR.	1.	Donor type	CRF-ENR
23	MATCHHLA	Char	3	\$MATCH.	3.	HLA match score	CRF-ENR
24	GENDER	Char	1	\$GENDERF.	1.	Patient's Gender	CRF-DEM

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
25	ETHNIC	Char	3	\$ETHNICF.	3.	Patient's Ethnicity	CRF-DEM
26	age	Num	8			Patient's age at the time of transplant	RECODE- this is the computed age of years based on DOB on DEM form
27	diagnosisDate	Num	8	MMDDYY8.		Date of diagnosis	CIBMTR
28	timetohct	Num	8			Time from diagnosis to allHCT (years)	RECODE-this is the number of years from diagnosis to alloHCT
29	ANCDT1	Num	8	MMDDYY8.	DATETIME22.3	ANC Date 1	CRF- NHM
30	fudate	Num	8	MMDDYY8.		Date of last follow-up	RECODE- this is the last follow up date based on all available data source/CRFs, the last date of all
31	prgrlpdt	Num	8	MMDDYY8.		Date of relapse or progression (ERC)	ERC-this is the ERC confirmed date of progression or relapse
32	osday	Num	8			Overall survival post transplant (days)	RECODE-this is the calculated number of survival days post transplant
33	osmon	Num	8			Overall survival post transplant (months)	RECODE-this is the calculated number of survival months post transplant
34	ossvrcens	Num	8			Overall survival post transplant censor indicator (1 means event)	RECODE- this is the censor indicator for overall survival endpoint

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
35	rfyday	Num	8			Relapse-free survival post transplant (days)	RECODE- this is the calculated number of days from transplant to death, last follow-up, relapse/progression or date patient received any therapy not specified in the protocol, computed for progression-free survival
36	rfsmo	Num	8			Relapse-free survival post transplant (months)	RECODE- this is the calculated number of months from transplant to death, last follow-up, relapse/progression or date patient received any therapy not specified in the protocol, computed for progression-free survival
37	rfssvcens	Num	8			Relapse-free survival post transplant censor indicator (1 means event)	RECODE-this is the censor indicator for progression-free survival endpoint
38	ancday	Num	8			Neutrophil recovery post transplant (days)	RECODE-this is the calculated number of days from date of transplant to date of neutrophil recovery
39	ancoutcome	Char	9			Neutrophil recovery post transplant outcome	RECODE-this is the outcome for neutrophil recovery endpoint
40	anc_CI	Num	8			Neutrophil recovery post transplant indicator for cumulative incidence (1 means event)	RECODE-this is the indicator for cumulative incidence of neutrophil recovery

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
41	relapseday	Num	8			Relapse post transplant (days)	RECODE-this is the calculated number of days from transplant to date of relapse
42	relapsemon	Num	8			Relapse post transplant (months)	RECODE-this is the calculated number of months from transplant to date of relapse
43	relapseoutcome	Char	9			Relapse post transplant outcome	RECODE-this is the outcome for relapse post transplant
44	relapse_CI	Num	8			Relapse post transplant indicator for cumulative incidence (1 means event)	RECODE-this is the indicator for cumulative incidence of relapse
45	TRMday	Num	8			Treatment-related mortality post transplant (days)	RECODE-this is the calculated number of days from transplant to treatment related mortality
46	TRMmon	Num	8			Treatment-related mortality post transplant (months)	RECODE- this is the calculated number of months from transplant to treatment related mortality
47	TRMoutcome	Char	9			Treatment-related mortality post transplant outcome	RECODE-this is the outcome for treatment-related mortality endpoint
48	TRM_CI	Num	8			Treatment-related mortality post transplant for cumulative incidence (1 means event)	RECODE- this is the indicator for cumulative incidence of treatment-related mortality

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
49	agvh234day	Num	8			Acute GVHD grade 2-4 post transplant (days)	RECODE- this is the calculated number of days from transplant date to date of grade 2-4 acute GVHD
50	gvh234outcome	Char	9			Acute GVHD grade 2-4 post transplant outcome	RECODE- this is the outcome of grade 2-4 acute GVHD post transplant endpoint
51	gvh234_CI	Num	8			Acute GVHD grade 2-4 post transplant for cumulative incidence (1 means event)	RECODE-this is the indicator for cumulative incidence of grade 2-4 acute GVHD post transplant
52	agvh34day	Num	8			Acute GVHD grade 3-4 post transplant (days)	RECODE-this is the calculated number of days from transplant date to date of grade 3-4 acute GVHD
53	gvh34outcome	Char	9			Acute GVHD grade 3-4 post transplant outcome	RECODE- this is the outcome of grade 3-4 acute GVHD post transplant endpoint
54	gvh34_CI	Num	8			Acute GVHD grade 3-4 post transplant for cumulative incidence (1 means event)	RECODE-this is the indicator for cumulative incidence of grade 3-4 acute GVHD post transplant
55	cgvhdday	Num	8			Chronic GVHD post transplant (days)	RECODE-this is the calculated number of days from transplant date to date of maximum chronic GVHD
56	cgvhdmon	Num	8			Chronic GVHD post transplant (months)	RECODE-this is the calculated number of months from transplant date to date of maximum chronic GVHD

Variables in Creation Order in OUTCOME dataset							
#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
57	cgvhdoutcome	Char	9			Chronic GVHD post transplant outcome	RECODE-this is the outcome for chronic GVHD post transplant endpoint
58	cgvhd_CI	Num	8			Chronic GVHD post transplant for cumulative incidence (1 means event)	RECODE-this is the indicator for cumulative incidence of chronic GVHD
59	un_ae	Char	3			Unexpected Grade 3-5 AEs reported?	RECODE-this is the indicator to tell if patient reported Grades 3-5 adverse events on the study, computed based on AE data
60	chimerism_d30	Num	8			Donor chimerism on day30 post transplantation	RECODE-this is the T-cell donor chimerism on day 30, computed based on NHM data
61	chimerism_d100	Num	8			Donor chimerism on day100 post transplantation	RECODE-this is the T-cell donor chimerism on day 100, computed based on NHM data
62	NEUTR	Char	3			Grade 3-4 neutropenia?	RECODE-this is the indicator to tell if patient reported any grade 3-4 neutropenia on the study, computed based on T14 data
63	ALT	Char	3			Grade 3-4 ALT ?	RECODE-this is the indicator to tell if patient reported any grade 3-4 ALT on the study, computed based on T14 data

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
64	HYPXI	Char	3			Grade 3-4 hypoxia?	RECODE-this is the indicator to tell if patient reported any grade 3-4 hypoxia on the study, computed based on T14 data
65	PNMTS	Char	3			Grade 3-4 pneumonitis?	RECODE-this is the indicator to tell if patient reported any grade 3-4 pneumonitis on the study, computed based on T14 data
66	tliver	Char	3			Abnormal liver tests?	RECODE-this is the indicator to tell if patient reported any grade 3-4 liver toxicities on the study, computed based on T14 data
67	MAXTOXI	Char	3			At least one grade 3-5 toxicity?	RECODE-this is the indicator to tell if patient reported any grade 3-5 toxicities on the study, computed based on T14 data
68	rituxan_d0	Num	8			Rituxan levels on day 0	LAB-this is the rituxan level at baseline, computed based on data provided by Genentech
69	rituxan_d28	Num	8			Rituxan levels on day 28	LAB-this is the rituxan level on day 28, computed based on data provided by Genentech

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
70	rituxan_d100	Num	8			Rituxan levels on day 100	LAB-this is the rituxan level on day 100, computed based on data provided by Genentech
71	rituxan_d180	Num	8			Rituxan levels on day 180	LAB-this is the rituxan level on day 180, computed based on data provided by Genentech
72	rituxan_d365	Num	8			Rituxan levels on day 365	RECODE-this is the rituxan level on day 365, computed based on data provided by Genentech
73	drop	Char	3			ANC dropped below 500?	RECODE- this is the indicator to tell if patient's ANC dropped below 500, computed based on NHM data
74	pwb_base	Num	8			Physical Well-Being (7 Items) at baseline	RECODE- this indicates the participant's Physical-Well Being at baseline, computed based on seven items of the FCT data
75	swb_base	Num	8			Social / Family Well-Being (7 Items) at baseline	RECODE- this indicates the participant's Social/Family Well-Being at baseline, computed based on seven items of the FCT data
76	ewb_base	Num	8			Emotional Well-Being (6 Items)at baseline	RECODE- this indicates the participant's Emotional-Well Being at baseline, computed based on six items of the FCT data

Variables in Creation Order in OUTCOME dataset							
#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
77	fwb_base	Num	8			Functional Well-Being (7 Items) at baseline	RECODE- this indicates the participant's Functional Well-Being at baseline, computed based on seven items of the FCT data
78	fact_g_base	Num	8			FACT-G Total (27 Items) at baseline	RECODE- this is the FACT-G total score at baseline , computed by adding the participant's Physical, Socail/Family, Emotional and Functional Well-Being
79	bmt_base	Num	8			FACT BMT Concerns (10 Items) at baseline	RECODE- this indicates the FACT BMT concerns at baseline, computed based on twenty-three items of the FCT data
80	fact_total_base	Num	8			FACT-BMT Total (37 Items) at baseline	RECODE- this is the grand total of all items in the FACT-G and BMT modules at baseline
81	fact_toi_base	Num	8			FACT-BMT Trial Outcome Index (24 Items) at baseline	RECODE- this is the FACT-BMT Trial Outcome Index at baseline, computed by adding the FACT-BMT Concerns, the Functional and Physical Well-being
82	pwb_2y	Num	8			Physical Well-Being (7 Items) at 2- years	RECODE- this indicates the participant's Physical-Well Being at 2 years, computed based on seven items of the FCT data

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
83	swb_2y	Num	8			Social / Family Well-Being (7 Items) at 2-years	RECODE- this indicates the participant's Social/Family Well-Being at 2 years, computed based on seven items of the FCT data
84	ewb_2y	Num	8			Emotional Well-Being (6 Items) at 2 years	RECODE- this indicates the participant's Emotional-Well Being at 2 years, computed based on six items of the FCT data
85	fwb_2y	Num	8			Functional Well-Being (7 Items) at 2-years	RECODE- this indicates the participant's Functional Well-Being at 2 years, computed based on seven items of the FCT data
86	fact_g_2y	Num	8			FACT-G Total (27 Items) at 2-years	RECODE- this is the FACT-G total score at 2 years , computed by adding the participant's Physical, Social/Family, Emotional and Functional Well-Being
87	bmt_2y	Num	8			FACT BMT Concerns (10 Items) at 2-years	RECODE- this indicates the FACT BMT concerns at 2 years, computed based on 23 variables of the FCT data
88	fact_total_2y	Num	8			FACT-BMT Total (37 Items) at 2-years	RECODE- this is the grand total of all items in the FACT-G and BMT modules at 2 years, computed based on FCT data

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
89	fact_toi_2y	Num	8			FACT-BMT Trial Outcome Index (24 Items) at 2 years	RECODE- this is the FACT-BMT Trial Outcome Index at 2 years, computed by adding the FACT-BMT Concerns, the Functional and Physical Well-being
90	ht_base	Num	8			RAW SF-36 HEALTH TRANSITION ITEM at baseline	RECODE- this is the participant's raw Health Transition Item at baseline, computed based on SFH data
91	PF_base	Num	8			SF-36 PHYSICAL FUNCTIONING (0-100) at baseline	RECODE- this is the participant's Physical Functioning score at baseline, computed based on SFH data
92	RP_base	Num	8			SF-36 ROLE-PHYSICAL (0-100) at baseline	RECODE- this is the participant's SF-36 Role Physical score at baseline, computed based on SFH data
93	BP_base	Num	8			SF-36 PAIN INDEX (0-100) at baseline	RECODE- this is the participant's Pain Index score at baseline, computed based on SFH data
94	GH_base	Num	8			SF-36 GENERAL HEALTH PERCEPTIONS (0-100) at baseline	RECODE- this is the participant's General Health score at baseline, computed based on SFH data
95	VT_base	Num	8			SF-36 VITALITY (0-100) at baseline	RECODE- this is the participant's Vitality score at baseline, computed based on SFH data

Variables in Creation Order in OUTCOME dataset							
#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
96	SF_base	Num	8			SF-36 SOCIAL FUNCTIONING (0-100) at baseline	RECODE- this is the participant's Social Functioning score at baseline, computed based on SFH data
97	RE_base	Num	8			SF-36 ROLE-EMOTIONAL (0-100) at baseline	RECODE- this is the participant's Emotional score at baseline, computed based on SFH data
98	MH_base	Num	8			SF-36 MENTAL HEALTH INDEX (0-100) at baseline	RECODE- this is the participant's Mental Health Index score at baseline, computed based in SFH data
99	PCS_base	Num	8			STANDARDIZED PHYSICAL COMPONENT SCALE at baseline	RECODE- this is the Physical Component summary at baseline, computed based on SFH data
100	MCS_base	Num	8			STANDARDIZED MENTAL COMPONENT SCALE at baseline	RECODE- this is the Mental Component summary at baseline, computed based on SFH data
101	ht_2y	Num	8			RAW SF-36 HEALTH TRANSITION ITEM at 2-years	RECODE- this is the participant's raw Health Transition Item at 2 years, computed based on SFH data
102	PF_2y	Num	8			SF-36 PHYSICAL FUNCTIONING (0-100) at 2-years	RECODE- this is the participant's Physical Functioning score at 2 years, computed based on SFH data
103	RP_2y	Num	8			SF-36 ROLE-PHYSICAL (0-100) at baseline	RECODE- this is the participant's SF-36 Role Physical score at 2 years, computed based on SFH data

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
104	BP_2y	Num	8			SF-36 PAIN INDEX (0-100) at 2-years	RECODE- this is the participant's Pain Index score at 2 years, computed based on SFH data
105	GH_2y	Num	8			SF-36 GENERAL HEALTH PERCEPTIONS (0-100) at 2-years	RECODE- this is the participant's General Health score at 2 years, computed based on SFH data
106	VT_2y	Num	8			SF-36 VITALITY (0-100) at 2-years	RECODE- this is the participant's Vitality score at 2 years, computed based on SFH data
107	SF_2y	Num	8			SF-36 SOCIAL FUNCTIONING (0-100) at 2-years	RECODE- this is the participant's Social Functioning score at 2 years, computed based on SFH data
108	RE_2y	Num	8			SF-36 ROLE-EMOTIONAL (0-100) at 2-years	RECODE- this is the participant's Emotional score at 2 years, computed based on SFH data
109	MH_2y	Num	8			SF-36 MENTAL HEALTH INDEX (0-100) at 2-years	RECODE- this is the participant's Mental Health Index score at 2 years, computed based in SFH data
110	PCS_2y	Num	8			STANDARDIZED PHYSICAL COMPONENT SCALE at 2-years	RECODE- this is the Physical Component summary at 2 years, computed based on SFH data

Variables in Creation Order in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source/Notes
111	MCS_2y	Num	8			STANDARDIZED MENTAL COMPONENT SCALE at 2-years	RECODE- this is the Mental Component summary at 2 years, computed based on SFH data

Algorithm used for the Recode and ERC Adjudications

Algorithm for Acute GVHD Grade:

- The acute GVHD algorithm calculates the grade based on the organ (skin, GI and liver) stage and etiology/biopsy reported on the weekly GVHD form.
- If none of the etiologies for skin, upper GI, lower GI, or liver are reported as GVHD, then the overall grade is 0
- If multiple etiologies are specified for lower GI or liver, the organ system will be down-staged by 1.
- If an upper GI biopsy is negative, upper GI symptoms are down-staged.
- If GVHD is not listed as an etiology for upper GI then upper GI symptoms are down-staged.
- Each organ contributes to the overall grade; while to get an overall grade, it does not necessarily need all organ symptoms. Different organ/stage determine different grade. Details below:

<p>Grade 0: No skin rash and No protracted nausea and vomiting and No diarrhea or diarrhea < 500 and Bilirubin < 2.0</p>	<p>Grade III: Skin-No rash to Rash > 50% with Diarrhea > 1000 or severe abdominal pain or Bilirubin 3.1 - 15</p>
<p>Grade I: Skin rash 25-50 % and No diarrhea or diarrhea < 500 and Bilirubin < 2.0</p>	<p>Grade IV: Skin-Generalized Erythroderma with Bullus Formation and Desquamation or Bilirubin > 15</p>
<p>Grade II: Skin rash >50% or Diarrhea >500 or Bilirubin 2.0 - 3.0 or Persistent nausea/vomiting</p>	

Algorithm for Chronic GVHD: Limited vs Extensive (Definition from CIBMTR forms)

- Limited – localized skin involvement and/or hepatic dysfunction due to chronic GVHD
- Extensive – one or more of the following:
 1. generalized skin involvement; or,
 2. liver histology showing chronic aggressive hepatitis, bridging necrosis or cirrhosis; or,
 3. involvement of eye: Schirmer's test with < 5 mm wetting; or
 4. involvement of minor salivary glands or oral mucosa demonstrated on labial biopsy; or
 5. involvement of any other target organ

Note on the sample size: Three out of the 65 enrolled participants were excluded from the primary analysis since they did not receive transplant (n=1), withdrew consent (n=1) or were found ineligible due to transformed disease (n=1) leaving 62 participants in the study. The median follow-up time for alive patients is 47 months (range 30-73) post transplant.

Note on Rituxan levels: All values of 499 are equivalent to rituxan levels lower than 500.