BMT CTN #0604 Double UCB Data Submission - Documentation for Outcomes Dataset

Outcomes dataset has 83 variables* for 50 patients on BMT protocol #0604 Double UCB 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 #0603 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.

*Note: The OUTCOMES dataset for the primary publication set has 80 variables. This is because donor chimerism (Marrow, Blood, and T-cell) at Day 365 was not available when the primary manuscript was published.

Variables in Creation Order							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
1	site	Char	5	5.	5.	Site Code	EMMES
2	SITENAME	Char	200	200.	200.	center name	EMMES– this is the name of the transplant center where the recipient was enrolled from
3	PROT	Char	5	5.	5.	BMT CTN Protocol #	EMMES – this indicated BMT CTN Protocol #
4	PATID	Char	15	15.	15.	Patient 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.
5	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.
6	PRIMADDC	Char	2	$DIS.	2.	Primary Disease at Baseline	CRF - ENRA

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
7	PRIORRDC	Char	1	$PRGM.	1.	Prior Chemotherapy Regimens	CRF - ENRA
8	PRFSCODC	Char	10	$PSA.	10.	Performance Status at Baseline	CRF - ENRA
9	PRIOAUDC	Char	1	$YESNO.	1.	Prior Autologous Transplant	CRF - ENRA
10	DOB	Num	8	MMDDYY8.	8.	Date of Birth	CRF - DEM
11	dsstage	Char	50			Disease Stage at Baseline	RECODE - this is computed based on the collected disease stage reported on CRF-ENR form
12	ENRLDATE	Num	8	MMDDYY8.	8.	Enrollment Date	EMMES - this is the start date of the patient on this study.
13	ETHNIC	Char	3	$ETHNICF.	3.	Ethnicity	CRF - DEM
14	GENDER	Char	1	$GENDERF.	1.	Gender	CRF - DEM
15	raceA	Char	1	$RACEF.		Race	RECODE - this is based on race and secondary race reported on DEM form and combine into several big race categories.

Variables in Creation Order							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
16	IFUWEIGH	Num	8	7.1	7.1	CBU Infusion Weight	CRF – IF1
17	if12prenu2	Num	8			Combined Pre-cryopreservation Total Nucleated Cell Count(x10^7/kg)	RECODE - this is sum of pre-cryopreservation total nucleated cell count for cord blood unit infusion #1 and #2 (*IF1PRENU, IF2PRENU*) divided by patient's weight (*IFUWEIGH*) collected on CRF-IF1
18	if12posnu2	Num	8			Combined Post-thaw Total Nucleated Cell Count(x10^7/kg)	RECODE - this is sum of post-thaw total nucleated cell count for cord blood unit infusion #1 and #2 (*IF1POSNU, IF2POSNU*) divided by patient's weight (*IFUWEIGH*) on CRF-IF1
19	fudate	Num	8	MMDDYY8.		Last follow up date	RECODE - this is based on the last follow- up date from all available data sources including CRF, CIBMTR long term data and ERC adjudication
20	DTHDT	Num	8	MMDDYY8.	8.	Date of Death	RECODE - this is based on death date from all available data sources including CRF-DTH, and

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
							CIBMTR long-time data
21	PRPLDATE	Num	8	MMDDYY8.	8.	Date of Relapse	RECODE - this is based on disease progression/relapse date from all available data sources including CRF-PRP, CIBMTR long-term data and ERC adjudicated data
22	cGVHDdate_f	Num	8	MMDDYY8.	8.	Date of Diagnosis of Chronic GVHD	ERC - this is based on CIBMTR long term follow-up data and ERC adjudication on date of cGVHD
23	maxgrade	Num	8			Maximum Grade of Acute GVHD	ERC – This is the ERC adjudicated maximum overall grade of acute GVHD. ERC adjudicated the maximum grade based on the weekly acute GVHD assessment and some clinical notes as needed.
24	maxdate	Num	8	MMDDYY8.		Date of Maximum Acute GVHD	ERC – This is the ERC adjudicated date of maximum acute GVHD. ERC adjudicated the maximum grade based on the weekly acute GVHD

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
							assessment and some clinical notes as needed.
25	ag24date	Num	8	MMDDYY8.		Date of Grades 2 to 4 Acute GVHD	RECODE - this is based on date of maximum acute GVHD and maximum 2 to 4 aGVHD grades
26	ag34date	Num	8	MMDDYY8.		Date of Grades 3 to 4 Acute GVHD	RECODE - this is based on date of maximum acute GVHD and maximum 3 to 4 aGVHD grades
27	toxmax	Num	8			Maximum CTCAE Toxicity Grade	RECODE - this is the maximum grade of Grades 3-5 toxicity that patient experienced, computed based on CRF-T13 data. If null, it indicates that patient's maximum toxicity grade was 0-2.
28	COD_f	Char	18	$18.	$18.	Primary Cause of Death	ERC - this is based on primary cause of death reported on CRF-DTH form and adjudicated by ERC
29	primarygf	Char	12	$12.	$12.	Primary Graft Failure	ERC - this is based on donor cell engraftment and chimerism assay data on CRF-NHM form

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
							and adjudicated by ERC
30	secondgf	Char	3	$3.	$3.	Secondary Graft Failure	ERC - this is based on variables reported on CRF-SGR form and adjudicated by ERC
31	IFUINFDT	Num	8	MMDDYY8.	8.	CBU Infusion Date	CRF – IF1
32	HLACord1	Char	3	$3.	$3.	HLA match score for Cord Unit 1	ERC - this is based on CRF-CB3 (*COR1SISC*) and adjudicated by ERC
33	HLACord2	Char	3	$3.	$3.	HLA match score for Cord Unit 2	ERC - this is based on CRF-CB3 (*COR2SISC*) and adjudicated by ERC
34	HLACord12	Char	3	$3.	$3.	HLA typing match score(1st cord to 2nd cord)	ERC - this is based on CRF-CB3 (*COR3SISC*) and adjudicated by ERC
35	ANCDT1	Num	8	MMDDYY8.	8.	Date of Neutrophil Recovery	CRF - NHM
36	PLT201DT	Num	8	MMDDYY8.	8.	Date of Platelet Recovery to 20K	CRF - NHM
37	PLT501DT	Num	8	MMDDYY8.	8.	Date of Platelet Recovery to	CRF - NHM

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
						50K	
38	age	Num	8	4.1		Age of patient (yrs) at enrollment	RECODE - this is the computed age of years at enrollment based on DOB on CRF-DEM form
39	marrowpct28	Num	8			Donor Chimerism in Marrow at Day28	RECODE - this is computed based on marrow samples (*MRWCHMRS, ASYRSTCB, MCBU1PCT, MCBU2PCT)* from CRF-NHM form for Day 28. If chimerism assay was not performed on marrow samples, used blood sample instead
40	bloodpct28	Num	8			Donor Chimerism in Blood at Day28	RECODE - this is computed based on blood samples (*BLDCHMRS, BDASRTCB, BCBU1PCT, BCBU2PCT)* from CRF-NHM form for Day 28
41	tcellpct28	Num	8			Donor Chimerism in T-Cell at Day28	RECODE - this is computed based on T-cell samples (*TCLCHRSM, TASYRESU, TCBU1PCT, TCBU2PCT)* from CRF-NHM form for Day 28

Variables in Creation Order							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
42	marrowpct56	Num	8			Donor Chimerism in Marrow at Day56	RECODE - this is computed based on marrow samples (*MRWCHMRS, ASYRSTCB, MCBU1PCT, MCBU2PCT)* from CRF-NHM form for Day 56. If chimerism assay was not performed on marrow samples, used blood sample instead
43	bloodpct56	Num	8			Donor Chimerism in Blood at Day56	RECODE - this is computed based on blood samples (*BLDCHMRS, BDASRTCB, BCBU1PCT, BCBU2PCT)* from CRF-NHM form for Day 56
44	tcellpct56	Num	8			Donor Chimerism in T-Cell at Day56	RECODE - this is computed based on T-cell samples (*TCLCHRSM, TASYRESU, TCBU1PCT, TCBU2PCT)* from CRF-NHM form for Day 56
45	marrowpct180	Num	8			Donor Chimerism in Marrow at Day180	RECODE - this is computed based on marrow samples (*MRWCHMRS, ASYRSTCB, MCBU1PCT, MCBU2PCT)* from CRF-NHM form for Day 180. If chimerism assay was not

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
							performed on marrow samples, used blood sample instead
46	bloodpct180	Num	8			Donor Chimerism in Blood at Day180	RECODE - this is computed based on blood samples (*BLDCHMRS, BDASRTCB, BCBU1PCT, BCBU2PCT)* from CRF-NHM form for Day 180
47	tcellpct180	Num	8			Donor Chimerism in T-Cell at Day180	RECODE - this is computed based on T-cell samples (*TCLCHRSM, TASYRESU, TCBU1PCT, TCBU2PCT)* from CRF-NHM form for Day 180
48	marrowpct365	Num	8			Donor Chimerism in Marrow at Day365	RECODE - this is computed based on marrow samples (*MRWCHMRS, ASYRSTCB, MCBU1PCT, MCBU2PCT)* from CRF-NHM form for Day 365. If chimerism assay was not performed on marrow samples, used blood sample instead
49	bloodpct365	Num	8			Donor Chimerism in Blood at Day365	RECODE - this is computed based on blood samples (*BLDCHMRS, BDASRTCB,*

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
							BCBU1PCT, BCBU2PCT) from CRF-NHM form for Day 365
50	tcellpct365	Num	8			Donor Chimerism in T-Cell at Day365	RECODE - this is computed based on T-cell samples (*TCLCHRSM, TASYRESU, TCBU1PCT, TCBU2PCT)* from CRF-NHM form for Day 365
51	Maxcgvhd_f	Char	9			Maximum Grade of Chronic GVHD	RECODE - this is based on CRF-CGV form, CIBMTR long term follow-up data and ERC adjudicated maximum cGVHD grade
52	ost_srvtm	Num	8			Overall Survival Post Transplant(days)	RECODE - this is the days from transplant date to the death or last follow up, computed for overall survival
53	ost_mon	Num	8			Overall Survival Post Transplant (months)	RECODE - this is the months from transplant date to the death or last follow up, computed for overall survival
54	ost_outcome	Char	9			Overall Survival Post	RECODE - this is the outcome

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
						Transplant Outcome	for overall survival endpoint
55	ost_srvcens	Num	8			Overall Survival Post Transplant Censor Indicator	RECODE - this is the censor indicator for overall survival
56	dfs_srvtm	Num	8			Progression-Free Survival Post Transplant (days)	RECODE - this is the days from transplant date to the death, relapse, or last follow up, computed for disease progression-free
57	dfs_mon	Num	8			Progression-Free Survival Post Transplant (months)	RECODE - this is the months from transplant date to the death, relapse or last follow up, computed for disease progression-free
58	dfs_outcome	Char	9			Progression-Free Survival Post Transplant Outcome	RECODE - this is the outcome for disease progression-free endpoint
59	dfs_srvcens	Num	8			Progression-Free Survival Post Transplant Censor Indicator	RECODE - this is the censor indicator for disease progression-free
60	TRMday	Num	8			Treatment-related Mortality	RECODE - this is the months

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
						Post Transplant(days)	from transplant date to date of treatment-related mortality
61	TRMoutcome	Char	9			Treatment-related Mortality Post Transplant Outcome	RECODE - this is the outcome for treatment-related mortality endpoint
62	TRM_CI	Num	8			Treatment-related Mortality Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE - this is the indicator for cumulative incidence of treatment-related mortality (0=End Study, 1=Death, 2=Relapse) Relapse is considered as a competing risk in the cumulative incidence analysis for TRM.
63	relapseday	Num	8			Relapse Post Transplant(days)	RECODE - this is the days from transplant date to date of relapse *(=PRPLDATE - TXDTTXP)*
64	relapseoutcome	Char	9			Relapse Post Transplant Outcome	RECODE - this is the outcome for relapse post transplant
65	relapse_CI	Num	8			Relapse Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE - this is the indicator for cumulative incidence of relapse (0=End Study,

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
							1=Relapse, 2=Death) Death is considered a competing risk in the cumulative incidence analysis for relapse.
66	ancday	Num	8			Neutrophil Recovery Post Transplant(days)	RECODE - this is the outcome for neutrophil recovery endpoint (=*ANCDT1- IFUINFDT*).
67	ancoutcome	Char	9			Neutrophil Recovery Post Transplant Outcome	RECODE - this is the indicator for cumulative incidence of neutrophil recovery (0=End Study, 1=Engraft, 2=Death) Death is considered a competing risk in the cumulative incidence analysis for neutrophil recovery.
68	anc_CI	Num	8			Neutrophil Recovery Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE, this is the days from transplant date to date of platelet recovery to 20K.
69	plt20day	Num	8			Platelet Recovery to 20K Post Transplant(days)	RECODE, this is the days from transplant to date of platelet recovery to 20K (=*plt201dt - IFUINFDT*).

Variables in Creation Order							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
70	plt20outcome	Char	9			Platelet Recovery to 20K Post Transplant Outcome	RECODE - this is the outcome of platelet recovery to 20K.
71	plt20_CI	Num	8			Platelet Recovery to 20K Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE - this is the cumulative incidence indicator of platelet recovery to 20K (0=End Study, 1=Engraft, 2=Death). Death is considered as a competing risk.
72	plt50day	Num	8			Platelet Recovery to 50K Post Transplant(days)	RECODE - this is days from transplant to date of platelet recovery to 50K (=*plt501dt - IFUINFDT*).
73	plt50outcome	Char	9			Platelet Recovery to 50K Post Transplant Outcome	RECODE - this is the outcome of platelet recovery to 50K.
74	plt50_CI	Num	8			Platelet Recovery to 50K Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE - this is the cumulative incidence indicator for platelet recovery to 50K (0=End Study, 1=Engraft, 2=Death). Death is considered as a competing risk.
75	cgvhdday	Num	8			Chronic GVHD Post Transplant(days)	RECODE - this is the days from transplant date to date of maximum chronic GVHD

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
							(=DTDGNCGV –IFUINFDT).
76	cgvhdoutcome	Char	9			Chronic GVHD Post Transplant Outcome	RECODE - this is the outcome for chronic GVHD Post Transplant endpoint.
77	cgvhd_CI	Num	8			Chronic GVHD Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE - this is the cumulative incidence indicator for chronic GVHD post transplant endpoint. (0=End Study, 1=cGVHD, 2=Death) Death is considered a competing risk in the cumulative incidence analysis for chronic GVHD.
78	agvhd24day	Num	8			Grades 2-4 Acute GVHD Post Transplant(days)	RECODE - this is days from transplant date to date of Grade 2-4 acute GVHD (=*ag24date-IFUINFDT*).
79	agvhd24outcome	Char	9			Grades 2-4 Acute GVHD Post Transplant Outcome	RECODE - this is the outcome of Grades 2-4 acute GVHD Post Transplant endpoint
80	aGVHD24_CI	Num	8			Grades 2-4 Acute GVHD Post Transplant Indicator for	RECODE - this is the cumulative incidence indicator for Grades 2-

Variables in Creation Order							
#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
						Cumulative Incidence(event=1)	4 acute GVHD post transplant endpoint. (0=End Study, 1=g24aGVHD, 2=Death) Death is considered a competing risk in the cumulative incidence analysis for acute GVHD.
81	agvhd34day	Num	8			Grades 3-4 Acute GVHD Post Transplant(days)	RECODE - this is days from transplant date to date of Grade 3-4 acute GVHD (=*ag34date-IFUINFDT*).
82	agvhd34outcome	Char	9			Grades 3-4 Acute GVHD Post Transplant Outcome	RECODE - this is the outcome of Grades 3-4 acute GVHD Post Transplant endpoint
83	aGVHD34_CI	Num	8			Grades 3-4 Acute GVHD Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE - this is the cumulative incidence indicator for Grades 3-4 acute GVHD post transplant endpoint (0=End Study, 1=g34aGVHD, 2=Death). Death is considered a competing risk in the cumulative incidence analysis for acute GVHD.

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:

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

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

Notes on the sample size:

There were fifty-four patients enrolled to BMT CTN 0604. The endpoint review committee adjudicated 50 of them to be eligible for the study. In some tables, we collected information for 54 patients.

The study protocol specifies that patients will be followed for one year after transplantation. The median survival post-transplant for primary publication set is 365 days with range [56, 411]. After incorporating CIBMTR long-term data, the median survival turns to 1118 days with range [712, 1538].