BMT CTN #0401 Bexxar Data Submission - Documentation for Outcomes Dataset

Outcomes dataset has 77 variables for 224 patients on BMT protocol #0401 Bexxar 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 #0401 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.

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
1	RANDID	Char	10			Randomized Subject ID	Created by BioLINCC
2	MAX	Num	8			Maximum Mucositis Score by Day 21	RECODE - This calculation is based on CRF-MUC data that collected twice weekly for the first three weeks post-transplant. The mucositis score is the sum of average ulceration score and average erythema score. This variable is the maximum of all computed mucositis scores by Day 21 post-transplant.
3	AVG	Num	8			Average Mucositis Score by Day 21	RECODE – There can be up to six computed mucositis scores for each patient based on CRF-MUC data. This is the average of derived mucostis scores.
4	PROT	Char	5	$5.	$5.	BMT CTN Protocol #	EMMES - this indicates the protocol number in BMT CTN
5	PRCHEMBX	Char	1	$1.	$1.	Number of Prior Regimens of Chemotherapy	CRF - ENRA
6	DXSTATBX	Char	1	$PRIMDZF.	$1.	Disease Status at	CRF - ENRA

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
						Transplantation	
7	BILIVLBX	Num	8	5.1	5.1	Most Recent Bilirubin at Study Entry	CRF - ENRA
8	ALTVLBX	Num	8	4.	4.	Most Recent ALT at Study Entry	CRF - ENRA
9	ASTVLBX	Num	8	4.	4.	Most Recent AST at Study Entry	CRF - ENRA
10	DLCOVLBX	Num	8	4.	4.	Most Recent DLCO at Study Entry	CRF - ENRA
11	KPSBX	Char	2	$PSA.	$2.	Karnofsky Performance Score at Baseline	CRF - ENRA
12	TXDTTXP_DAYS	Num	8			Date of Transplant (days since enrollment)	CRF - TXP
13	CMVSTAT	Char	1	$CMV.	$1.	Pre-transplant CMV Status	CRF - TXP
14	GENDER	Char	1	$GENDERF.	$1.	Gender	CRF - DEM
15	DOB	Num	8	MMDDYY8.	DATETIME22.3	Date of Birth	CRF - DEM

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
16	ETHNIC	Char	3	$ETHNICF.	$3.	Ethnicity	CRF - DEM
17	DateofDiagnosis_days	Num	8			Date of Diagnosis (days since enrollment)	CIBMTR - diagnosis data retrieved from CIBMTR database
18	age_txp	Num	8			Age at Transplant	RECODE - this is derived from date of transplant (*TXDTTXP*) from CRF-TXP and date of birth (*DOB*) from CRF-ENRA
19	agegroup	Char	4			Age at Transplantation (>=50|<50)	RECODE - this is based on age at transplant and dichotomized into two groups for multivariate analysis
20	diagtxp	Num	8			Interval from Diagnosis to Transplantation (months)	RECODE - this is based on transplant date from CRF-TXP and date of diagnosis from CIBMTR
21	diaggroup	Char	7			Interval From Diagnosis to Transplantation (>=15 month|<15 month)	RECODE - this is based on computed time interval from diagnosis to transplant and dichotomized into two groups for multivariate analysis
22	raceA	Char	1	$RACEF.		Race (Recoded)	RECODE - this is based on race and secondary race reported on CRF-

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
							DEM form and combine into several big race categories.
23	PSgroup	Char	4			Dichotomized Karnofsky Performance Score (>=90|<90)	RECODE - this is based on Karnofsky Performance Score (*KPSBX*) from CRF-ENRA and dichotomized into two groups for multivariate analysis
24	chemgroup	Char	3			Dichotomized number of prior chemotherapy regimens (<3|=3)	RECODE - this is based on prior chemotherapy regimens (*PRCHEMBX*) from CRF-ENRA and dichotomized into two groups for multivariate analysis
25	dsgroup	Char	5			Disease Status at Transplantation (CR|NonCR)	RECODE - this is based on disease status (*DXSTATBX*) from CRF-ENRA and dichotomized into two groups for multivariate analysis
26	ethnicgroup	Char	6			Ethnic Group (Non-Hispanic|Others)	RECODE - this is based on ethnicity (*ETHNIC)* from CRF-DEM and dichotomized into two groups for multivariate analysis
27	racegroup	Char	5			Race (Black|White|Other)	RECODE - this is based on

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
							computed race (*RACEA*) and dichotomized into two groups for multivariate analysis
28	biligroup	Char	5			Dichotomized Bilirubin (>=0.4|<0.4)	RECODE - this is based on bilirubin (*BILIVLBX*) from CRF-ENRA and dichotomized into two groups for multivariate analysis
29	altgroup	Char	4			Dichotomized ALT (>=27|<27)	RECODE - this is based on ALT (*ALTVLBX*) from CRF-ENRA and dichotomized into two groups for multivariate analysis
30	astgroup	Char	4			Dichotomized AST (>=25|<25)	RECODE - this is based on AST (*ASTVLBX*) from CRF-ENRA and dichotomized into two groups for multivariate analysis
31	dlcogroup	Char	4			Dichotomized DLCO (>=80|<80)	RECODE - this is based on DLCO (*DLCOVLBX*) from CRF-ENRA and dichotomized into two groups for multivariate analysis
32	TRTTRUE	Char	100	100.	100.	Treatment Arm	EMMES - this is based on randomization and indicates the

Variables							
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							assignment upon enrollment.
33	age_at_enrlmnt	Num	8			Age at Enrollment	Created by BioLINCC
34	eligible	Char	1			Eligible for Study (1=Yes, eligible; 2=No, not eligible)	RECODE - this is the ERC adjudicated data based on patient's eligibility for the study
35	fudate_final_days	Num	8			Date of Follow-up (days since enrollment)	RECODE - this is the last follow up date based on all available data source including Emmes CRFs and CIBMTR follow-up data, the last date of all.
36	dthdt_final_days	Num	8			Date of Death (days since enrollment)	RECODE - this is based on all available data source including Emmes CRFs, CIBMTR follow-up data and ERC adjudicated data, the most updated death date.
37	cod_final	Char	35			Cause of Death	RECODE - this is based on all available data source including Emmes CRFs, CIBMTR follow-up data and ERC adjudicated data, the most updated cause of death.

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
38	relapsedate_final_days	Num	8			Date of Relapse/Progression (days since enrollment)	RECODE - this is based on all available data source including Emmes CRFs, CIBMTR follow-up data and ERC adjudicated data, the most updated disease progression/relapse date.
39	PLTDT1IR_DAYS	Num	8			Date of 1st platelet count recovered to >=20,000/uL (days since enrollment)	CRF - IMR
40	ANCDT1IR_DAYS	Num	8			Date of 1st ANC recovered to >500/uL (days since enrollment)	CRF - IMR
41	ose_srvtm	Num	8			Overall Survival Post Randomization (days)	RECODE – this is the days from randomization to the death or last follow up, computed for survival analysis
42	ose_outcome	Char	9			Overall Survival Post Randomization Outcome	RECODE - this is the outcome for overall survival endpoint
43	ose_srvcens	Num	8			Overall Survival Post Randomization Censor Indicator (event=1)	RECODE - this is the censor indicator for overall survival endpoint

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
44	pfs_srvtm	Num	8			Progression Free Survival Post Randomization (days)	RECODE - this is the days from randomization to the death, relapse or last follow up, computed for progression-free survival
45	pfs_outcome	Char	9			Progression Free Survival Post Randomization Outcome	RECODE - this is the outcome for progression-free survival endpoint
48	pfs_srvcens	Num	8			Progression Free Survival Post Randomization Censor Indicator (event=1)	RECODE - this is the censor indicator for progression-free survival endpoint
46	ose_srvtm_mon	Num	8			Overall Survival Post Randomization (months)	RECODE - this is the months from randomization to the death or last follow up, computed for survival analysis
47	pfs_srvtm_mon	Num	8			Progression Free Survival Post Randomization (months)	RECODE - this is the months from randomization to the death, relapse or last follow up, computed for disease progression-free
48	TRMeday	Num	8			Treatment-related Mortality Post Randomization (days)	RECODE - this is the days from randomization to date of treatment-related mortality

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
49	TRMemon	Num	8			Treatment-related Mortality Post Randomization (months)	RECODE - this is the months from randomization to date of treatment-related mortality
50	TRMeoutcome	Char	9			Treatment-related Mortality Post Randomization Outcome	RECODE - this is the outcome for treatment-related mortality endpoint
51	TRMe_CI	Num	8			Treatment-related Mortality Post Randomization 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.
52	relapseeday	Num	8			Relapse Post Randomization (days)	RECODE - this is the days from randomization to date of relapse
53	relapseemon	Num	8			Relapse Post Randomization (months)	RECODE - this is the months from randomization to date of relapse
54	relapseeoutcome	Char	9			Relapse Post Randomization Outcome	RECODE - this is the outcome for relapse post randomization
55	relapsee_CI	Num	8			Relapse Post Randomization Indicator for Cumulative	RECODE - this is the indicator for cumulative incidence of relapse

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
						Incidence (event=1)	(0=End Study, 1=Relapse, 2=Death) Death is considered a competing risk in the cumulative incidence analysis for relapse.
56	ancday	Num	8			Neutrophil Recovery Post Transplant (days)	RECODE, this is the days from transplant to date of neutrophil recovery
57	ancoutcome	Char	9			Neutrophil Recovery Post Transplant Outcome	RECODE - this is the outcome for neutrophil recovery endpoint
58	anc_CI	Num	8			Neutrophil Recovery Post Transplant Indicator for Cumulative Incidence (event=1)	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.
59	pltday	Num	8			Platelet Recovery Post Transplant(days)	RECODE, this is the days from transplant to date of platelet recovery

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
60	pltoutcome	Char	9			Platelet Recovery Post Transplant Outcome	RECODE - this is the outcome for platelet recovery endpoint
61	plt_CI	Num	8			Platelet Recovery Post Transplant Indicator for Cumulative Incidence(event=1)	RECODE - this is the indicator for cumulative incidence of platelet recovery (0=End Study, 1=Engraft, 2=Death) Death is considered a competing risk in the cumulative incidence analysis for platelet recovery.
62	maxtoxyn	Char	3			Maximum Toxicity Grade 3-5	RECODE - this is to indicate if patient experienced any NCI CTCAE Version 3.0 grades 3-5 toxicities during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
63	smnln	Char	3			Grade 3-5 of Somnolence	RECODE - this is to indicate if patient experienced any grades 3-5 somnolence during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.

Variables							
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64	cysti	Char	3			Grade 3-5 of Hemorrhagic Cystitis	RECODE - this is to indicate if patient experienced any grades 3-5 hemorrhagic cystitis during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
65	hypot	Char	3			Grade 3-5 of Hypotension	RECODE - this is to indicate if patient experienced any grades 3-5 hypotension during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
66	crdar	Char	3			Grade 3-5 of Cardiac Arrhythmia	RECODE - this is to indicate if patient experienced any grades 3-5 cardiac arrhythmia during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
67	lvent	Char	3			Grade 3-5 of Left Ventricular Systolic Dysfunction	RECODE - this is to indicate if patient experienced any grades 3-5 left ventricular systolic dysfunction during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity

Variables							
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							grade was 0-2.
68	hypxi	Char	3			Grade 3-5 of Hypoxia	RECODE - this is to indicate if patient experienced any grades 3-5 hypoxia during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
69	dyspn	Char	3			Grade 3-5 of Dyspnea	RECODE - this is to indicate if patient experienced any grades 3-5 dyspnea during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
70	hemrg	Char	3			Grade 3-5 of Hemorrhage	RECODE - this is to indicate if patient experienced any grades 3-5 hemorrhage during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
71	dic	Char	3			Grade 3-5 of HUS/TTP/thrombotic microangiopathy	RECODE - this is to indicate if patient experienced any grades 3-5 HUS/TTP/thrombotic microangiopathy during the study,

Variables							
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							computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
72	vaslk	Char	3			Grade 3-5 of Vascular Leak Syndrome	RECODE - this is to indicate if patient experienced any grades 3-5 vascular leak syndrome during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
73	szgrd	Char	3			Grade 3-5 of Recent Seizure	RECODE - this is to indicate if patient experienced any grades 3-5 seizure during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
74	diarr	Char	3			Grade 3-5 of Diarrhea	RECODE - this is to indicate if patient experienced any grades 3-5 diarrhea during the study, computed based on CRF-TX6 data. If no, it indicates that patient's maximum toxicity grade was 0-2.
75	scr	Char	3			Mucositis WHO Grade 3-5	RECODE - this is to indicate if patient had maximum grade of

Variables							
#	**Variable**	**Type**	**Len**	**Format**	**Informat**	**Label**	**Data Source / Notes**
							Grades 3-5 mucositis based on WHO toxicity grade, computed based on CRF-MUC data. If no, it indicates that patient's maximum toxicity grade was 0-2.
76	ERF_Response_Day_100	Char	2			Disease Response at Day100 Per ERC Adjudication	ERC - this is the ERC adjudicated disease response at Day 100
77	ERF_Response_Day_730	Char	2			Disease Response at Day730 Per ERC Adjudication	ERC - this is the ERC adjudicated disease response at Day 730

Algorithm used for the Recode and ERC Adjudications

Disease Response:

Complete Remission (CR) is defined as disappearance of all evidence of disease.
Partial Remission (PR) is defined regression of measurable disease and no new sites.
Stable Disease (SD) is defined as failure to attain CP/PR or relapsed disease (PD).
Relapsed disease or PD is defined as any new lesion or increase by ≥ 50% of previously involved site from nadir
If not sufficient data or information to evaluate the response, it is Not Evaluable (NE).

Mucositis Score:

Mucositis severity was recorded twice weekly for the first three weeks post-transplant using a modified OMAS tool. The OMAS scale evaluates eight sites of the oral cavity for erythema (none=0, mild-moderate =0.5, or severe=1) and the presence of ulcerations (Yes =1 and No=0). The eight sites are maxillary labial mucosa, mandibular labial mucosa, right cheek, left cheek, right lateral & ventral tongue, left lateral & ventral tongue, floor of mouth, soft palate. The mucositis score is the sum of average ulceration score (total of all site ulceration scores/number of evaluable sites) and average erythema score (total of all site erythema scores/number of evaluable sites), and ranges from 0 to 2.0, with a score of 1 or greater considered to be consistent with severe mucositis.

Notes on CIBMTR long-term follow-up data

The patients on the study were followed for 2 years post-ASCT per protocol. Among 224 patients randomized on the study, 212 of them proceeded to transplant. The long-term follow-up data beyond 2 years were already retrieved and incorporated into the final analysis for the primary publication. The median follow up months for the study survivors by the final data cut-off date was 38.2 months post-transplant with a range 3.7 to 76.3 months. Two patients lost to follow-up before they reached the 2-year follow up.