

BMT CTN #0302 Data Submission - Documentation for Outcomes Dataset

Outcomes dataset has 47 variables for 180 patients on BMT protocol #0302 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 #0302 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 in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
1	RAND_ID	Char	5	\$5.	\$5.	Randomized subject ID	RECODE – randomized ID
2	ETHNIC	Char	3	\$ETHNICF.	\$3.	Ethnicity	CRF - DEM
3	raceA	Char	1	\$RACEF.		Primary Race category (recode)	RECODE - this is based on race and secondary race reported on DEM form and combined into several big race categories.
4	fudate_days	Num	8			Date of Last follow up - days since enrollment	RECODE - this is the last follow up data based on all available data source/CRFs, the last date of all.
5	ADTREAT28_R	Char	12	12.	12.	Secondary Treatment By Day 28? (Per ERC adjudication)	ERC – this is the ERC adjudicated secondary treatment that patient received by Day 28. If null, means did not receive any secondary treatment for GVHD.
6	ADTREAT56_R	Char	32	32.	32.	Secondary Treatment By Day 56? (Per ERC adjudication)	ERC – this is the ERC adjudicated secondary treatment that patient received by Day 56 (after Day 28)
7	SITE	Char	5	\$5.	\$5.	Site Code	EMMES - the code is unique for each site participating in the BMT CTN
8	TRTRUE	Char	800	\$800.	\$800.	Treatment Arm per randomization	EMMES - this is based on randomization and indicates the assignment upon enrollment.
9	GRDCGVHD	Char	1	\$CGVH.	\$1.	Maximum overall grade of chronic GVHD	RECODE - this is the maximum grade of chronic GVHD that patient experienced, computed based on CRF-CGV data.
10	SEVCGVHD	Char	1	\$SEVC.	\$1.	Maximum severity of chronic GVHD	RECODE - this is the maximum severity of chronic GVHD that patient experienced, computed based on CRF-CGV data.

Variables in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
11	TRTSTOP_DAYS	Num	8			Date of discontinuation of immunosuppressive therapy for GVHD - days since enrollment	CRF – CGV, coded to days since enrollment
12	INFSTDT_DAYS	Num	8			First date of severe/life-threatening/fatal infections – days since enrollment	RECODE - this is the first date of severe or life-threatening or fatal infection that patient experienced, computed based on CRF -INF data.
13	MAXTOX	Num	8			Maximum grade of toxicity by Day 56	RECODE - this is the maximum grade of Grades 3-5 toxicity that patient experienced, computed based on CRF-TX4 data. If null, it indicates that patient's maximum toxicity grade was 0-2.
14	grade3date_days	Num	8			Toxicity evaluation date for grade 3 or higher toxicity by Day 56 – days since enrollment	RECODE - this is the associated date for toxicity evaluation that patient reported grade 3 or higher toxicity by Day 56. This is null if no grade 3 or higher toxicity by Day 56.
15	STAGEDT_DAYS	Num	8			Date of acute GVHD onset – days since enrollment	CRF – GVH, this is the date of staging for acute GVHD, coded to days since enrollment
16	MMFUSED	Char	1	\$YESNOF.	1.	Received MMF Previously?	CRF – ENR, this is the variable from enrollment form. This is the important stratification factor for randomization

Variables in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
17	grade0	Num	8			Baseline acute GVHD - overall grade	RECODE – This is the overall acute GVHD grade at study entry, computed based on the four organ involvements (variables on the enrollment form: Skin abnormalities (<i>SKIN_ABN</i>), Upper GI abnormalities (<i>UPPER_GI</i>), Lower GI abnormalities (<i>LOWER_GI</i>), Liver abnormalities (<i>LIVER_AB</i>)). Please refer to the acute GVHD algorithm described in this document.
18	age	Num	8	4.1		Age at enrollment	RECODE - this is the computed age of years at enrollment based on DOB on DEM form
19	dzstage	Char	20			Disease Stage	RECODE – this is the recoded disease stage pre-transplant, based on the data on Baseline form, including AML stage, ALL stage, CML stage, MDS stage, Lymphoma stage and others.
20	PM0302DX	Char	1	\$PRIMDZF.	1.	Primary Disease	CRF – BL1
21	CON0302R	Char	1	\$CONREG.	1.	Conditioning Regimen	CRF – ENR (enrollment form)
22	KA0302SC	Char	2	\$PSA.	2.	Karnofsky/Lansky performance score at transplant	CRF – BL1
23	T0302DT_DAYS	Num	8			Date of Transplant – days since enrollment	CRF – BL1, coded to days since enrollment
24	REL0302U	Char	1	\$DSRC.	1.	Donor source	CRF – BL1
25	T0302TY	Char	1	\$TXPTYPE.	1.	Stem cell type	CRF – BL1

Variables in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
26	CMV0302S	Char	1	1.	1.	CMV status at transplant	CRF – BL1
27	txptype	Char	21			Transplant type (recoded category)	RECODE – combination category of donor source and stem cell type
28	GENDER	Char	1	\$GENDERF.	1.	Gender	CRF – DEM
29	resp14	Char	2	\$RESPF.		GVHD Response - Day 14 assessment (recoded)	RECODE – acute GVHD response at Day 14, based on the weekly GVHD assessment (CRF -GVH) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.
30	resp21	Char	2	\$RESPF.		GVHD Response - Day 21 assessment (recoded)	RECODE – acute GVHD response at Day 21, based on the weekly GVHD assessment (CRF -GVH) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.
31	resp35	Char	2	\$RESPF.		GVHD Response - Day 35 assessment (recoded)	RECODE – acute GVHD response at Day 35, based on the weekly GVHD assessment (CRF -GVH) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.
32	resp42	Char	2	\$RESPF.		GVHD Response - Day 42 assessment (recoded)	RECODE – acute GVHD response at Day 42, based on the weekly GVHD assessment (CRF -GVH) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.

Variables in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
33	resp49	Char	2	\$RESPF.		GVHD Response - Day 49 assessment (recoded)	RECODE – acute GVHD response at Day 49, based on the weekly GVHD assessment (CRF -GVH) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.
34	resp90	Char	2	\$RESPF.		GVHD Response - Day 90 assessment (recoded)	RECODE – acute GVHD response at Day 90, based on the weekly GVHD assessment (CRF -GVH) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.
35	resp120	Char	2	\$RESPF.		GVHD Response - Day 120 assessment (recoded)	RECODE – acute GVHD response at Day 120, based on the GVHD follow-up assessment (CRF -CGV) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.
36	resp180	Char	2	\$RESPF.		GVHD Response - Day 180 assessment (recoded)	RECODE – acute GVHD response at Day 180, based on the GVHD follow-up assessment (CRF -CGV) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.
37	resp270	Char	2	\$RESPF.		GVHD Response - Day 270 assessment (recoded)	RECODE – acute GVHD response at Day 270, based on the GVHD follow-up assessment (CRF -CGV) and algorithm for response, please refer to the acute GVHD response algorithm described in this document.

Variables in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
38	flareday	Num	8			Days to aGVHD flare post randomization(only include flare by Day 120 by Chronic GVHD diagnosis)	ERC – this is the ERC adjudicated days to acute GVHD flare, which only includes GVHD flare by Day 120, and exclude any flare after day 120 or after chronic GVHD diagnosis.
39	rlpday	Num	8			Days to disease relapse/progression post randomization	RECODE – this is the computed days from randomization to the date of disease relapse/progression (= RELAPSDT – ENRLDATE)
40	maxgrade_R	Num	8			Maximum Acute GVHD Overall Grade (Per ERC adjudication)	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.
41	resp28_R	Char	2	\$RESPF.		Response at Day 28 (Per ERC adjudication)	ERC – This is the ERC adjudicated GVHD response at Day 28. ERC adjudicated the GVHD response based on the weekly acute GVHD assessment, some clinical notes as needed and the protocol definitions for GVHD response.
42	grade28_R	Num	8			Acute GVHD Overall Grade at Day 28 (Per ERC adjudication)	ERC – This is the ERC adjudicated overall grade of acute GVHD at Day 28. ERC adjudicated GVHD grade based on the weekly acute GVHD assessment and some clinical notes as needed.

Variables in OUTCOME dataset

#	Variable	Type	Len	Format	Informat	Label	Data Source / Notes
43	resp56_R	Char	2	\$RESPF.		Response at Day 56 (Per ERC adjudication)	ERC – This is the ERC adjudicated GVHD response at Day 56. ERC adjudicated the GVHD response based on the weekly acute GVHD assessment, some clinical notes as needed and the protocol definitions for GVHD response.
44	grade56_R	Num	8			Acute GVHD Overall Grade at Day 56 (Per ERC adjudication)	ERC – This is the ERC adjudicated overall grade of acute GVHD at Day 56. ERC adjudicated GVHD grade based on the weekly acute GVHD assessment and some clinical notes as needed.
45	disctime	Num	8			Days to discontinuation of immunosuppressive therapy post randomization	RECODE – this is the computed days from randomization to the date of discontinuation of immunosuppressive therapy (= TRTSTOP-ENRLDATE)
46	dthday	Num	8			Days to death post randomization	RECODE – this is the computed days from randomization to the date of death (= DTHDT-ENRLDATE)
47	cgvday	Num	8			Days to chronic GVHD post randomization	RECODE – this is the computed days from randomization to the date of chronic GVHD (=DTDGNCGV -ENRLDATE)

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% 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	

Response to acute GVHD:

Complete Response (CR) is defined as resolution of all signs and symptoms of GVHD in all evaluable organs in comparison to Day 1 scoring. For a response to be scored as CR at Day 28 or later, the participant must still be in CR on that day and have had no intervening salvage therapy for an earlier progression, PR or NR.

Partial response (PR) is defined as improvement in one or more organs involved with GVHD symptoms without progression in others. For a response to be scored as PR at Day 28 or later, the participant must still be in PR on that day and have had no intervening salvage therapy for an earlier progression, PR or no response (NR).

Mixed response (MR) is defined as improvement in one or more organs with deterioration in another organ manifesting symptoms of GVHD or development of symptoms of GVHD in a new organ.

Progression is defined as deterioration in at least one organ without any improvement in others.

No response (NR) is defined as absence of any improvement or progression as defined.

Scoring of PR, MR, NR and progression are in comparison to the participant's GVHD status (score) on Day 0 of the study. If not sufficient data or information to evaluate the GVHD response, it is Not Evaluable.

GVHD flare:

Patient achieved CR, later acute GVHD symptoms reappeared, days to the first time that acute GVHD grade above 0 after initial CR.