Forms to be released

- 2814 R4 - Indication Form
- *2003 R1 - Gene Therapy Product
- 2037 R3 - Leukodystrophies Pre-Infusion
- 2137 R3 - Leukodystrophies Post-Infusion
- 2900 R5 – Recipient Death Data
- 2400 R9 – Pre-Transplant Essential Data
- 2450 R6 – Post-Transplant Essential Data

*indicates new form

For information on high level changes to the forms, please select from the list below:

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Indication (2814 R4) Summary

Indication

a. Question 2 – Addition
   Added question to capture the “Event date” for HCT, CT, and marrow toxic injury recipients (moved from those applicable sections).
   Rationale: Allows CIBMTR to capture the necessary data with fewer questions.

Hematopoietic Cellular Transplant (HCT)

b. Questions 2-4 – Removal
   Removed questions asking for the cell source (Qs 2-4 on R3).
   Rationale: The information will be captured on the Pre-TED F2400.

c. Question 4 – Addition
   To ask if the product is genetically modified.
   Rationale: Distinguish gene therapy recipients early on.

d. Question 5 – Removal
   “Planned HCT date” (Q5 on R3) will be captured in what is now Q2.
   Rationale: Capture necessary data with fewer questions.

Cellular Therapy

*no longer a section on R4

e. Question 6 – Removal
   “Planned infusion date” (Q6 on R3) will be captured in what is now Q2.
   Rationale: Capture necessary data with fewer questions.

Marrow Toxic Injury

*no longer a section on R4

f. Question 7 – Removal
   “Event date” (Q7 on R3) will be captured in what is now Q2.
   Rationale: Capture necessary data with fewer questions.

Gene Therapy Product (2003 R1) Highlights

New Form – captures information in following categories
Product processing/manipulation, Product analysis and Product infusion

This form must be completed for all products for Gene Therapy recipients. All patients receiving a Gene Therapy product will be placed into the CRF track. For TED only reporting centers, Form 2003 will also
The Gene Therapy Product (2003) form is designed to capture product specific information for all infusions given to a recipient as a course of gene therapy. A series of collections from the same recipient that uses the same collection method, even if the collections are performed on different days, should be considered a singular gene therapy product if only one set of manufacturing steps are applied to the collected material. If more than one type of gene therapy product is infused, each product type must be reported on a separate Gene Therapy Product (2003) form. A product from the same donor undergoing different processing or manipulations are considered different products and require multiple Gene Therapy Product (2003) forms if each product is infused separately. However, if the cells underwent different processing steps or manipulations and the end result were combined for a single infusion or administration, it will be considered a single product and will require a single Gene Therapy Product (2003) form.

### Leukodystrophies Pre-Infusion (F2037 R3) Summary

#### General Form Updates

- **a.** Updated Key Fields to standard format.  
  **Rationale:** Consistent layout.
- **b.** Updated “HCT” to “infusion”.  
  **Rationale:** Consistent language across forms.
- **c.** Updated “CIBMTR Recipient ID” language to “CIBMTR Research ID”.  
  **Rationale:** Utilize appropriate terminology.

#### Leukodystrophy Diagnosis

- **g.** **Question 1 – Removal**  
  Removed prior Q1 of F2037 R2 “what is the date of diagnosis of leukodystrophy?”.  
  **Rationale:** Already captured in Q1 of the Disease Classification F2402.
- **h.** **Question 1 – Addition**  
  Capture via a question (not a checkbox as seen on R2) if this is a report of a second or subsequent infusion for the same disease.  
  **Rationale:** Ensures for easy querying when needed.
- **i.** **Question 2 – Update**  
  *field auto populated*  
  Added option for “hereditary diffuse leukoencephalopathy with spheroids (HDLS)”.  
  **Rationale:** Relevant to capture, new classification.
- **j.** **Questions 3 & 4 – Addition**  
  Added question to capture the testing performed to establish diagnosis.  
  **Rationale:** Helpful to understand in what way the diagnosis was established in the era of what was recommended.
- **k.** **Questions 5-13 – Update**  
  Captures enzyme / storage activity for all recipients and donors.  
  **Rationale:** Do not need to separate by disease / enzyme.
  **Questions 14-16 – Addition**  
  Obtain information regarding genetic mutational panel results.
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- **Rationale**: Important info. to capture, although results can be difficult to interpret so collecting minimal data.

l. **Question 17 – Updated**
   Made into a new parent question (rather than child question of leukocyte arylsulfatase A enzyme activity) (Q13 on R2).
   **Rationale**: New formatting, in alignment with enzyme / storage activity at diagnosis.

m. **Question 18 – Updated**
   Made into a new parent question (rather than child question of leukocyte arylsulfatase A enzyme activity) (Q16 on R2). Also removed text for “fasting” within the question and added floating text for clarification.
   **Rationale**: New formatting, in alignment with enzyme / storage activity at diagnosis. Not everyone will truly be fasting and this component will never be analyzed as an independent variable.

n. **Questions 18-20 – Removal**
   Removed prior questions 18-20 on R2 regarding “plasma level”.
   **Rationale**: Not relevant.

o. **Question 20 – Update**
   Updated prior Qs 21-23 on R2 and combined into one question.
   **Rationale**: Increase efficiency.

p. **Question 21 – Update**
   Updated prior Qs 24-29 on R2 and combined into two questions. Removed all non-relevant options and added “antecedal cystine”.
   **Rationale**: More efficient & only including relevant options.

**Clinical Status Prior to the Preparative Regimen**

q. **Questions 23-26 – Addition**
   Captures recipient enzyme / storage activity prior to prep.
   **Rationale**: Enzyme / storage activity may be tested again after prep.

r. **Questions 27-44 – Addition**
   Added questions to capture NFS score for ALD recipients.
   **Rationale**: NFS captures the neurological clinical status.

s. **Question 46 – Addition**
   “Were any of the seizures considered nonfebrile?”.
   **Rationale**: Normal children will have febrile seizures, and we don’t want to confuse those that may be related to the disease.

t. **Question 49 – Update**
   Updated CSF results to a “check all that apply” and removed “closing pressure” (Qs 37 & 38 on R2).
   **Rationale**: Improve efficiency and “closing pressure” not relevant.

u. **Questions 54 & 55 – Addition**
   Added questions to capture if gadolinium contrast was used and if enhancement was reported.
   **Rationale**: Recipients are only eligible for transplant if they had gad enhancement, marker of active disease.

v. **Questions 45-48 – Removal**
   Removed prior questions 45-48 on R2 regarding magnetic resonance imaging.
   **Rationale**: Not relevant.

w. **Questions 61-63 - Update**
Updated Mental Development test (Q53 on R2) to “neurocognitive test” and only asking for the date and if documentation was submitted.

Rationale: Use appropriate terminology and only capture relevant information.

**x.** **Additional Removals**

- Vineland Adaptative Behavior Scales (prior questions 63-70 on R2)
- Visual acuity testing (prior questions 71-79)
- Ophthalmologic exam (prior questions 80-83)
- Hearing loss (prior questions 84-93)

Rationale: No longer relevant to capture

**Leukodystrophies Post-Infusion (F2137 R3) Summary**

**General Form Updates**

a. Updated Key Fields to standard format.
   **Rationale:** Consistent layout.

b. Updated “HCT” to “infusion”.
   **Rationale:** Consistent language across forms.

c. Updated “CIBMTR Recipient ID” language to “CIBMTR Research ID”.
   **Rationale:** Utilize appropriate terminology.

**Leukodystrophies Post-Infusion Data**

a. **Question 1 – Update**
   
   *field auto populated*
   
   Added option for “hereditary diffuse leukoencephalopathy with spheroids (HDLS)”.
   **Rationale:** Relevant to capture, new classification.

b. **Questions 2-5 – Update**
   
   Capturing enzyme / storage activity for all recipients.
   **Rationale:** Do not need to separate by disease / enzyme.

**Clinical Status Post-Infusion**

**NOTE:** The rationale for the updates to questions 6-23, 25, 29, 36, 37, 21-25, and 44-46 is to mirror the pre-infusion form.

c. **Questions 6-23 – Addition**
   
   Added questions to capture NFS score for ALD recipients.

d. **Question 25 – Addition**
   
   “Were any of the seizures considered nonfebrile?”.

e. **Question 29– Update**
   
   Updated CSF results to a “check all that apply” and removed “closing pressure” (Q12 on R2).

f. **Question 36 & 37 – Addition**
   
   Added questions to capture if gadolinium contrast was used and if enhancement was reported.

g. **Question 38- Addition**
   
   Added question to capture Loes score for ALD recipients.
   **Rationale:** Important for comparison pre-infusion.

h. **Questions 21-25 – Removal**
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Removed prior questions 21–25 on R2 regarding magnetic resonance imaging.

i. **Questions 29 & 30 – Removal**
   Removed prior questions 29 & 30 on R2 regarding median nerve conduction velocity.
   **Rationale:** Not relevant.

j. **Questions 44-46 - Update**
   Updated Mental Development test (Q33 on R2) to “neurocognitive test” and only asking for the date and if documentation was submitted.

k. **Questions 50 – Addition**
   Captures Clinical Global Impression (CGI), global improvement.
   **Rationale:** Will be documented more frequently moving forward.

l. **Additional Removals**
   - Vineland Adaptative Behavior Scales (prior questions 44-52 on R2)
   - Visual acuity testing (prior questions 53-62)
   - Ophthalmologic exam (prior questions 63-67)
   - Hearing loss (prior questions 68-82)
   **Rationale:** No longer relevant to capture.

m. **Question 51 & 52 – Addition**
   Added questions to capture leukodystrophy-specific therapy given post-infusion.
   **Rationale:** Assess treatment received.

**Recipient Death Data (2900 R5) Summary**

**Recipient Death**

n. **Question 4 – Update**
   - Sub-section headers and options within alphabetized. The five most common causes of death remain at the top of the list.
   - Added new sub-section for “Toxicity” with two new options (neurotoxicity [ICANS] & tumor lysis syndrome).
   - Updated “thromboembolic” option to “thromboembolism”.
   **Rationale:**
   - Alphabetization is standard CIBMTR form format.
   - These can be causes of death for cellular therapy recipients.
   - Utilize correct language.

**Contributing Cause of Death**

o. **Question 6 – Update**
   - Sub-section headers and options within alphabetized. The five most common causes of death remain at the top of the list.
   - Added new sub-section for “Toxicity” with two new options (neurotoxicity [ICANS] & tumor lysis syndrome).
   - Updated “thromboembolic” option to “thromboembolism”.
   **Rationale:**
   - Alphabetization is standard CIBMTR form format.
   - These can be causes of death for cellular therapy recipients.
   - Utilize correct language.
Pre-Transplant Essential Data (2400 R9) Summary

Comorbid Conditions
  p. Questions 90-94 – Addition
     Added questions to capture COVID vaccination data.
     Rationale: to capture vaccination status for transplant recipients.

Post-Transplant Essential Data (2450 R6) Summary

Comorbid Conditions
  a. Questions 52-56 – Addition
     Added questions to capture COVID vaccination data.
     Rationale: to capture vaccination status for transplant recipients.