Contents

Gene Therapy Product Form (2003 R2)

Product Identification

a. **Q2 ("Is the product out of specification? *(only for commercially available products)*")**
   - Addition: Added new question.
   - Rationale: Need to support corporate studies to identify out of specification/non-confirming products.

Product Collection

b. **Q12 ("What agents were used to mobilize the recipient for this HCT? *(check all that apply)*"), Q13 ("Specify other agent")**
   - Addition: Copied questions from the Pre-TED (2400) form.
   - Rationale: Need to capture mobilization agents per product since each product will get a separate F2003.

c. **Q14 ("Was more than one day of collection required?")**
   - Update: Added a wording 'day of' to the question text.
   - Rationale: Rephrased question to capture scientific relevant data.

Product Infusion

d. **Q73 ("Was the entire volume of product infused?"), Q74("Specify what happened to the reserved portion"), Q75("Specify other fate")**
   - Addition: Copied questions from Cell Therapy Infusion (4006) form.
   - Rationale: To understand what happens to the portion not infused.

Leukodystrophies Pre-Infusion (2037 R4)

Disease Modifying Therapies

a. **Qs23-29 (Includes questions related to disease modifying therapies)**
   - Addition: Copied "disease modifying therapies" section from 2058/2158
   - Rationale: Leriglitazone is a clinical trial drug that is the first disease modifying therapy.

Clinical Status Prior to Preparative Regimen

b. **Q62 ("Was gadolinium contrast used for this assessment?")**
   - Update: Levelling was updated.
   - Rationale: Both normal and abnormal MRI results need to answer the gadolinium question.

c. **Q69 ("Was a neurocognitive test administered at any time prior to the preparative regimen?")**
   - Update: Added floating text to the "Yes" option as "Also complete Neurocognitive Assessment Form 3503".
   - Rationale: This is the parent question to trigger the F3503.

Marrow Evaluation

d. **Q72 ("Was a marrow aspirate and / or biopsy performed?")**
   - Addition: Added a new question that triggers 'laboratory studies 3502' and marrow surveillance form 3506' when 'yes' is selected.
   - Rationale: Applies to SCD and supports GT registry needs.

Leukodystrophies Post-Infusion (2137 R4)

Clinical Status Post-Infusion

a. **Q37 ("Was gadolinium contrast used for this assessment?")**
   - Addition: Levelling was updated.
   - Rationale: Both normal and abnormal MRI results need to answer the gadolinium question.

b. **Q45 ("Was a neurocognitive test performed?")**
   - Update: Added floating text to the "Yes" option as "Also complete Neurocognitive Assessment Form 3503".
   - Rationale: This is the parent question to trigger the F3503.

Disease Modifying Therapies

c. **Qs 54-61 (Includes questions regarding disease modifying therapies)**
   - Addition: Copied "disease modifying therapies" section and questions from 2058/2158

- Rationale: Leriglitazone is a clinical trial drug that is the first disease modifying therapy.

Marrow Evaluation

d. Q62 ("Was a marrow aspirate and / or biopsy performed?")
- Addition: Added a new question that triggers 'laboratory studies 3502' and marrow surveillance form 3506' when 'yes' is selected.
- Rationale: Applies to SCD and supports GT registry needs.

Thalassemia Pre-Infusion Data (2058 R2)

Transfusion Therapy

a. Q54 on R1 ("Is the number of red blood cell (RBC) transfusion known?")
- Removal: Removed this question from R1
- Rationale: Also applies to beta-thalassemia and supports GT registry needs.

b. Qs 59 ("Does recipient have donor specific antibodies present to the donor chosen for transplant? (mean fluorescence intensity (MFI) >1000 for HLA-A, HLA-B, and DRB1; OR MFI >2000 for HLA-C, DQB1 and DPB1 or positive virtual cross match)", Q60 ("Were measures taken to lower the MFI for the presence of donor antibodies prior to infusion?")
- Addition: Added new questions that are copied from 2030R3 (Q17,18).
- Rationale: Also applies to beta-thalassemia and supports GT registry needs.

Cardiac Assessments

c. Q69 ("Cardiac iron T2 imaging *(found on MRI results)*"), Q70 ("Cardiac iron T2")
- Addition: Added new questions.
- Rationale: Also applies to beta-thalassemia and supports GT registry needs.

d. Q71 ("Was brain natriuretic peptide (BNP) assessed?"), Q72 ("BNP")
- Addition: Added new questions.
- Rationale: Also applies to beta-thalassemia and supports GT registry needs.

Renal Assessments

e. Q74 ("Glomerular filtration rate (GFR) *(only required if the recipient is 19 years of age or older)*"), Q75 ("GFR")
- Addition: Added new questions.
- Rationale: Also applies to beta-thal and supports GT registry needs.

Avascular Necrosis

f. Q76 ("Has avascular necrosis occurred?"), Q77 ("Specify joint(s) affected *(check all that apply)*"), Q78 ("Specify other")

- Addition: Added new questions that are copied from 2030R3 (Q65,66,67).
- Rationale: Also applies to beta-thal and supports GT registry needs.

Other Symptoms

g. Qs 79 ("Have chronic leg ulcers developed?")

- Addition: Added new question that is copied from 2030R3 (Q80).
- Rationale: Also applies to beta-thal and supports GT registry needs.

Marrow Evaluation

h. Q113 ("Was a marrow aspirate and / or biopsy performed?")

- Addition: Added a new question that triggers 'laboratory studies 3502' and marrow surveillance form 3506' when 'yes' is selected.
- Rationale: Supports GT registry needs.

Thalassemia Post-Infusion Data (2158 R2)

Entire Form

a. Qs 35,63,64,74,102 (Includes all questions that has wording since the last reporting period / since the last report)

- Removal: Removed question text "since the last report / since the last reporting period".
- Rationale: To align with data capture best practices.

Treatment

b. Q24 ("Was phlebotomy performed?")

- Update: Removed 'given' from the question text and added 'performed'.
- Rationale: Support GT registry needs.

Splenic Assessments

c. Q31 ("Did the recipient have splenomegaly?")

- Addition: Added new question.
- Rationale: Supports GT registry needs.

Cardiac Assessments

d. Q65 ("Cardiac iron T2 imaging"), Q66 ("Cardiac iron T2")

- Addition: Added new questions.

- Rationale: Supports GT registry needs.

e. Q67 ("Was brain natriuretic peptide (BNP) assessed?"), Q68 ("BNP")
- Addition: Added new questions and are copied from 2130R3 (Q27,28).
- Rationale: Also applies to beta-thal and supports GT registry needs.

Renal Assessments

f. Q72 ("Glomerular filtration rate (GFR) *(if multiple, report the most recent tested)*"), Q73 ("GFR")
- Addition: Added new questions.
- Rationale: Also applies to beta-thal and supports GT registry needs.

Avascular Necrosis

g. Q74 ("Is there a new area affected by avascular necrosis?"), Q75 ("Specify joint(s) affected *(check all that apply)*"), Q76 ("Specify other")
- Addition: Added new questions that are copied from 2130R3 (Q47,48,49).
- Rationale: Also applies to beta-thal and supports GT registry needs.

Other Symptoms

h. Q77 ("Have chronic leg ulcers developed?")
- Addition: Added new question that is copied from 2130R3 (Q61).
- Rationale: Also applies to beta-thal and supports GT registry needs.

Marrow Evaluation

i. Q122 ("Was a marrow aspirate and / or biopsy performed?")
- Addition: Added a new question that triggers 'laboratory studies 3502' and marrow surveillance form 3506' when 'yes' is selected.
- Rationale: Supports GT registry needs.

Indication for CIBMTR Data Reporting (F2814 R5)

Form Name

a. ("Form name")
- Update: The name of the form was from Indication for CRID Assignment to Indication for CIBMTR Data Reporting.
- Rationale: This form has been updated to capture both first and subsequent infusions, thus the name "Indication for CIBMTR Data Reporting" more accurately describes the updated purpose of this form.

Indication

b. Q1 ("Specify the indication for CIBMTR data reporting")

- Update: The question text was updated from "What is the indication for CIBMTR Research ID (CRID) assignment?" to "Specify the indication for CIBMTR data reporting." Response options "HCT" and "Cellular therapy" have been removed and replaced by "Infusion."
- Rationale: The question text was updated to clarify that this form will now be used to capture data on first and subsequent infusions. The response options "HCT" and "Cellular therapy" were replaced by "Infusion" because the answers to the questions on the form will determine the infusion type, removing the burden from data managers. Additionally using the term "infusion" instead of HCT/CT is in keeping with best practices.

c. Q2 ("Date of marrow toxic injury")

- Update: The question text was update from "Event date" to "Date of marrow toxic injury."
- Rationale: Only the date for marrow toxic injuries needs to be captured prior to the rest of the questions on this form; the date of infusions will be captured in Q12.

Infusion

d. Q3-Q5, Q7-12; Q3 (Does the product(s) infused contain CD34+ cells and is there intent to establish / restore hematopoiesis?), Q4 (What was the primary indication for this infusion), Q5 (Specify other indication), Q7 (Was genetic modification performed to repair or correct a genetic defect?), Q8 (Was the infusion a donor lymphocyte infusion (DLI)? (contains more CD3+ cells than CD34+ cells)), Q9 (Number of DLIs in this reporting period), Q10 (Specify donor), Q11 (Has this donor already provided cells for this recipient for a prior infusion?), Q12 (Date of infusion)

- Addition: Questions to capture data about infusions were added to the form. These questions include: donor type; if the donor has already provided cells for the recipient; if the product contains CD34+ cells and if there is an intent to establish/restore hematopoiesis; indication for infusion; if intent of the genetic modification is to repair or correct a genetic defect; if the main component of the infusion is donor lymphocytes; number of DLIs in the reporting period; and the date of the infusion. These same questions were removed from or deleted on the Pre-Infusion forms (F2450, F4100).
- Rationale: These questions will be used to determine the type of infusion (HCT, CT, DLI, GT) given to the recipient and to trigger the correct pre-infusion form.

e. Q6 ("Are any of the products associated with this infusion genetically modified?")

- Update: The question text was updated from "Is the product genetically modified" to "Are any of the products associated with this infusion genetically modified?"
- Rationale: The text was updated to match the language on F4000 R 10, to maintain consistency across forms.

Non-Cellular Therapy

f. Q14 ("Specify BMT CTN study" & Q16 "Specify the ClinicalTrials.gov identification number")

- Addition: Questions to capture the specific BMT CTN study that the participant is enrolled in and the NCT number of the study were added.
- Rationale: These questions were added to capture relevant data.

Post-Transplant Essential Data (F2450 R8)

Subsequent Infusion

a. Q3 ("Did the recipient receive a subsequent infusion?")

- Update: The question text was changed from "Did the recipient receive a subsequent HCT?" to "Did the recipient receive a subsequent infusion?"
- Rationale: The question text was updated to better align with best practices and allow for all infusion types to be captured with one question. Answering "yes" to this question will trigger the newly revised F2814 R5 (see below).

b. Q4 ("Date of subsequent HCT"),Q5 ("What was the indication for subsequent HCT?"), Q6 ("Specify other indication"), Q7 ("Source of HSCs")

- Removal: These questions were removed from the form.
- Rationale: The data elements from these questions will now be answered on the newly revised F2814 R5. Additionally the questions have been reworded to capture all infusion types.

c. Q9 ("Was this infusion a donor lymphocyte infusion?"), Q10 ("Number of DLIs in this reporting period"), Q11 ("Are any of the products, associated with this course of cellular therapy, genetically modified?")

- Removal: These questions were removed from the form.
- Rationale: The data elements from these questions will now be answered on the newly revised F2814 R5.

d. Q8 ("Has the recipient received a cellular therapy since the date of last report?"), Q9 ("Was this infusion a donor lymphocyte infusion?"), Q12 ("Date of cellular therapy")

- Removal: These questions were removed from the form.
- Rationale: The data from these questions are no longer needed.