4000: Cellular Therapy Essential Data Pre-Infusion

> ! This form must be completed for all recipients of cellular therapy (non-HCT) where it is the first indication for treatment (no prior hematopoietic cell transplant), when a cellular therapy event is reported on an HCT follow up form, or when cellular therapy (non-HCT) is reported as a new indication following a marrow toxic injury (RITN patient), or a non-cellular therapy (e.g. chemotherapy, immunotherapy) patient receives cellular therapy.
> For recipients of hematopoietic stem cell transplants, complete a form 2400 – Pre-Transplant Essential Data.

This form reflects baseline recipient data and indications for cellular therapy. At this stage only non-HCT cellular therapies are being collected on this form. However, some indications reflect donor cellular infusions (DCI/DLI) done post-transplant. The intent in the near future is to report any cellular therapy, excluding HCT, through the cellular therapy essential data forms.

The use of cellular therapy is expanding and treatment strategies include isolation and transfer of specific stem cell populations, administration of effector cells (e.g. cytotoxic T-cells), induction of mature cells to become pluripotent cells, and reprogramming of mature cells (e.g. CAR T-cells).

Manual Updates:

Sections of the Forms Instruction Manual are frequently updated. In addition to documenting the changes within each manual section, the most recent updates to the manual can be found below. For additional information, select the manual section and review the updated text.

Date	Manual Section	Add/ Remove/ Modify	Description

4/17/17	4000: Cellular Therapy Essential Data Pre-Infusion	Add	Added instructions, in red below, regarding how to report the start date for multiple infusions (question 31). *Report the intended start date of the infusion for the instance being reported. If multiple infusions are planned within the first 100 days, each infusion must be reported as a separate instance / copy of questions 31-35 and the planned infusion date, question 31, will correspond to the specific infusion being reported. The planned infusion date of the earliest infusion must match the planned infusion date reported on one of the following forms:* • Indication for CRID Assignment Form (Form 2814) – This form will be used to generate a Cellular Therapy Essential Data Form (Form 4000) when the recipient has not received a HCT. • Post-Transplant Essential Data Form (Form 2450) – This form will generate a Cellular Therapy Essential Data Form (Form 4000) when the recipient has received an HCT followed by a donor cellular infusion (excluding subsequent HCTs) or other cellular therapy. • Post-HCT Follow-Up Data Form (Form 2100) – This form will generate a Cellular Therapy Essential Data Form (Form 4000) when the recipient has received an HCT followed by a donor cellular infusion (excluding subsequent HCTs) or other cellular therapy.
4/17/17	4000: Cellular Therapy Essential Data Pre-Infusion	Add	Added further clarification, in red below, to the instructions for question 34: *The intent of question 34 is to determine whether an HLA Form (Form 2005) has already been completed for this donor so that duplicate reporting may be avoided. For infusions using an NMDP donor or cord blood unit, the donor's HLA typing is reported on NMDP Form 22 (Confirmation of Donor HLA Typing) and the recipient's HLA typing is reported on NMDP Form 117 (Final Recipient HLA Typing). Please contact your CRC if you think the Form 2005(s) should be removed.*
4/4/17	4000: Cellular Therapy Essential Data Pre-Infusion	Modify	Instruction change for questions 120-122: Age range for Lansky Scale has been updated from *recipients less than 16 years old* to *recipients one year old to less than 16 years old.* If the recipient is less than one year old, questions 120-122 should be left blank.
3/17/17	4000: Cellular Therapy Essential Data Pre-Infusion	Add	Added the following note box to the instructions for question 1: **Reporting Consent Status for DCI** If this form is being completed for a DCI reported on a Post-TED Form (Form 2450) or Post-HCT Follow-Up Data Form (Form 2100), report "Not applicable" for question 1. The consent status will be reported on the Pre-TED Form (Form 2400) and should not be re-reported here. If the recipient's consent status has changed since the Pre-TED Form was completed, update the consent status on the Pre-TED Form.

2/22/17	4000: Cellular Therapy Essential Data Pre-Infusion	Add	Added instructions to question 34 (bold text): Indicate if the allogeneic unrelated or related donor reported in question 32 was used for prior cellular therapies **or HCT** for this recipient. Do not answer this question for autologous donors. **The intent of question 34 is to determine whether an HLA Form (Form 2005) has already been completed for this donor so that duplicate reporting may be avoided.**
7/29/16	4000: Cellular Therapy Essential Data Pre-Infusion	Add	Version 1 Released
2/15/17	4000: Cellular Therapy Essential Data Pre-Infusion	Add	Added note box above question 19: **Questions 19-27 HCT History**

Q1-8: Recipient Data

! To be compliant with Federal Regulations for human research subject protection, centers must obtain IRB-approved informed consent from recipients and donors (if applicable) to allow data submitted to the CIBMTR to be used for observational research. The NMDP/CIMBTR has written protocols and informed consent documents for the Observational Database. All centers must have local IRB approval for the Observational Database protocol. The NMDP IRB has approved these protocols and consent forms, and the documents are provided to participating sites to include with their local IRB submissions. **International Centers** must obtain consent of each patient participating in the Observational Database in a manner consistent with the laws and regulations of that country.

Question 1: Has the recipient signed an IRB / Ethics Committee-approved consent form for submitting research data to the CIBMTR?

✻ **Reporting Consent Status for DCI**
If this form is being completed for a DCI reported on a Post-TED Form (Form 2450) or Post-HCT Follow-Up Data Form (Form 2100), report "Not applicable" for question 1. The consent status will be reported on the Pre-TED Form (Form 2400) and should not be re-reported here. If the recipient's consent status has changed since the Pre-TED Form was completed, update the consent status on the Pre-TED Form.

When a recipient consents to participate in the Observational Database, their data is available in the CIBMTR's Observational Research Database and used for research. The database includes recipient baseline and outcome data for related and unrelated allogeneic transplants from any cell source, and for autologous transplants. The Observational Research Database will now include data on recipients of cellular therapy.

The primary purpose of the Observational Research Database is to have a comprehensive source of data that can be used to study hematopoietic cellular transplantation and cellular therapy. Studies using this data include:

- How well recipients recover from their transplants.
- How recovery after transplantation can be improved.
- What the long-term outcomes are after transplantation and cellular therapies.
- How access to transplantation for different groups of recipients can be improved.
- How well donors recover from collection procedures.

- The application and success of transplantation in the management of marrow toxic injuries.
- Cellular therapy.
- Better understand new complications seen with infusion of certain cellular therapy products.
- Compare outcomes of transplantation and cellular therapies between each other and to other therapies.

Indicate if the recipient has signed an IRB-approved consent form to participate in the Observational Research Database. If "yes (patient consented)," continue with question 2. If "no" (patient declined)", "Not approached", or "Not applicable" continue with question 3. If the patient declines consent, any data reported will not be used in observational studies.

When to use the "Not Approached" option for the Research Database Consent
CIBMTR expects all transplant centers to approach all patients for the Research Database consent. The "not approached" option should only be used in the rare event when the physician feels it would be in the best interest of the patient not to be consented.

Question 2: Date form was signed:

Report the date the research database consent form was signed by the recipient. Do not report the date that the witness or health care professional signed the consent form.

Question 3: Is the recipient participating in a cellular therapy clinical trial?

Indicate if the recipient is a registered participant with BMT-CTN, RCI-BMT, USIDNET, COG, a Corporate/ Industry trial and/or another clinical trial sponsor that uses CIBMTR forms to capture outcomes data. If "yes," continue with question 4. If "no," continue with question 9. If the participant is enrolled in multiple studies, even if from the same sponsor, report each study separately.

- BMT-CTN: Blood and Marrow Transplant Clinical Trials Network
- RCI-BMT: Resource for Clinical Investigation in Blood and Marrow Transplant
- USIDNET: United States Immunodeficiency Network
- COG: Children's Oncology Group
- Corporate / Industry

Cellular therapy clinical trials
There are no clinical trials for cellular therapy at this time. Questions 4-7 are currently unable to be answered in FormsNet3^{SM}. As studies are opened in the future, study options will be added to the form.

Questions 4-8 Reporting Participation in More Than One Study

FormsNet3SM application: Complete questions 4-8 for each study the recipient is participating in by adding an additional instance in the FormsNet application.
Paper form submission: Copy questions 4-8 and complete for each study in which the recipient is participating.

Question 4: Study sponsor:

Select the study sponsor of the clinical trial the recipient is participating in.

If the study sponsor is reported as “BMT-CTN” or “RCI-BMT,” continue with question 7.

If the study sponsor is reported as “USIDNET” or “COG,” continue with question 8.

If the study sponsor is reported as “Corporate/Industry”, specify in question 5 and continue with question 8.

If “other sponsor” is reported, specify the study sponsor in question 6 and continue with question 8.

Question 5: Specify corporate / industry sponsor name:

Report the name of the Corporate or Industry sponsor.

Question 6: Specify other sponsor:

If the recipient is participating in a clinical trial and the study sponsor is not listed, report in question 6 and continue with question 8.

Question 7: Study ID Number:

Specify the identification number of the recipient of a cellular therapy product under the clinical trial, if known.

Question 8: Specify the ClinicalTrials.gov identification number:

All clinical trials required to be registered on the clinicaltrials.gov website and will have an associated identification number.

Q9-30: Cellular Therapy and HCT History

Question 9: Is this the first application of cellular therapy (non-HCT)?

Indicate if this is the recipient's first cellular therapy application. "First application" is defined as the first application the recipient ever receives, not the first application the recipient receives at your facility.

If "yes", continue with question 19. If "no", continue with question 10.

Question 10: Were all prior cellular therapies (non-HCT) reported to the CIBMTR?

If "yes", continue to question 19. If "no", continue with question 10. If "unknown", continue with question 19.

Question 11: Specify the number of prior cellular therapies:

Enter the number of prior cellular therapies for the recipient. A "cellular therapy event" is defined as the infusion or administration of a cellular therapy product for treatment of a specific indication(s). Each infusion or administration of a cellular product should be counted separately.

Questions 12-18 Reporting Prior Cellular Therapies

FormsNet3SM application: Complete questions 12-18 to report all prior cellular therapies that have not yet been reported to the CIBMTR by adding an additional instance in the FormsNet application.

Paper form submission: Copy questions 12-18 and complete for each prior cellular therapy that has not yet been reported to the CIBMTR.

Question 12: Date of the prior cellular therapy:

Report the date of prior cellular therapy.

Question 13: Was the indication for the prior cellular therapy the same as the current cellular therapy?

If the indication for the prior cellular therapy is the same indication as the current cellular therapy for which this form is being completed, select "yes" and continue with question 16. If the indication is not the same, select "no" and continue with question 14.

Question 14: Specify the indication for the prior cellular therapy:

Select the indication for the prior cellular therapy being reported in this instance.

Question 15: Specify other indication:

If the indication for the prior cellular therapy is not listed, specify the indication.

Question 16: Was the cellular therapy performed at a different institution?

Indicate if the last cellular therapy was performed at another institution. If “yes”, continue with question 17. If “no”, continue with question 18.

Question 17: Specify the institution that performed the prior cellular therapy:

Report the name, city, state, and country of the institution where the recipient’s last cellular therapy was performed. This data is used to identify and link the recipient’s existence in the database and, if necessary, obtain data from the other institution where the previous treatment was administered.

Question 18: What was the cell source for the prior cellular therapy?

Indicate the cell source for the prior cellular therapy being reported.

An autologous product has cells collected from the recipient for his/her own use.

An unrelated donor (allogeneic, unrelated) is a donor who shares no known ancestry with the recipient. Include adoptive parents/children or step-parents/children.

A related donor (allogeneic or syngeneic, related) is a blood-related relative. This includes monozygotic (identical twins), non-monozygotic (dizygotic, fraternal, non-identical) twins, siblings, parents, aunts, uncles, children, cousins, half-siblings, etc.

Questions 19-27 HCT History
For scenarios where both HCT and CT forms will be submitted at the same time, there are duplicate questions across the F2100 and F4000. To reduce the reporting burden, duplicated questions on the Cell Therapy forms are disabled. This includes HCT History reported in Q19-27.

Question 19: Has the recipient ever had a prior HCT?

If “yes”, continue with question 20. If “no”, continue with question 28.

Question 20: Were all prior HCTs reported to the CIBMTR?

If “yes”, continue with question 28. If “no”, continue with question 21.

Questions 21-27 Reporting Prior HCTs

FormsNet3SM application: Complete questions 21-27 to report all prior HCTs that have not yet been reported to the CIBMTR by adding an additional instance in the FormsNet application.

Paper form submission: Copy questions 21-27 and complete for each prior HCT that has not yet been reported to the CIBMTR.

Question 21: Date of the prior HCT:

Report the date of the prior HCT for which questions 21-27 are being completed.

Question 22: Was the HCT performed at a different institution?

Indicate if the last HCT was performed at another institution. If “yes” continue with question 23. If “no” continue with question 24.

Question 23: Specify the institution that performed the prior HCT:

Report the name, city, state, and country of the institution where the recipient’s last HCT was performed. This data is used to identify and link the recipient’s existence in the database and, if necessary, obtain data from the previous transplant center.

Question 24-27: Specify the HSC source(s) for the prior HCT:

Indicate the cell source for the prior HCT being reported in this instance.

An autologous product has cells collected from the recipient for his/her own use.

An unrelated donor (allogeneic, unrelated) is a donor who shares no known ancestry with the recipient. Include adoptive parents/children or step-parents/children.

A related donor (allogeneic or syngeneic, related) is a blood-related relative. This includes monozygotic (identical twins), non-monozygotic (dizygotic, fraternal, non-identical) twins, siblings, parents, aunts, uncles, children, cousins, half-siblings, etc.

Question 28: Is a subsequent HCT part of the overall treatment protocol?

This question intends to capture instances where the cellular therapy is administered in association with a HCT, either planned or dependent upon the response to the cellular therapy. If a subsequent HCT is part of the overall treatment plan, indicate “yes”, continue with question 29. If “no”, continue with question 31.

Question 29: Specify the HCT type:

Specify the type of the subsequent HCT that is planned as part of the overall treatment protocol as either “autologous” or “allogenic”.

Question 30: Specify the circumstances in which the subsequent HCT will be performed:

Specify the reason for which the subsequent HCT will be performed as “regardless of response to cellular therapy”, “only if the patient responds to cellular therapy” or “only if the patient fails to respond or has an incomplete response”.

Q31-35: Planned Infusions in First 100 Days

Questions 31-35 Reporting Planned Infusions in First 100 Days
FormsNet3SM application: Complete questions 31-35 to report all infusions to be given within the first 100 days, as part of this course of cellular therapy, by adding an additional instance in the FormsNetSM application.
Paper form submission: Copy questions 31-35 and complete for each infusion planned in the first 100 days.

Question 31: Planned infusion date:

Report the intended start date of the infusion for the instance being reported. If multiple infusions are planned within the first 100 days, each infusion must be reported as a separate instance / copy of questions 31-35 and the planned infusion date, question 31, will correspond to the specific infusion being reported. The planned infusion date of the earliest infusion must match the planned infusion date reported on one of the following forms:

- Indication for CRID Assignment Form (Form 2814) – This form will be used to generate a Cellular Therapy Essential Data Form (Form 4000) when the recipient has not received a HCT.
- Post-Transplant Essential Data Form (Form 2450) – This form will generate a Cellular Therapy Essential Data Form (Form 4000) when the recipient has received an HCT followed by a donor cellular infusion (excluding subsequent HCTs) or other cellular therapy.
- Post-HCT Follow-Up Data Form (Form 2100) – This form will generate a Cellular Therapy Essential Data Form (Form 4000) when the recipient has received an HCT followed by a donor cellular infusion (excluding subsequent HCTs) or other cellular therapy.

Question 32: Specify the cell source:

Select the cell source for the infusion date reported in question 31.

An autologous product has cells collected from the recipient for his/her own use. Continue with question 35.

An unrelated donor (allogeneic, unrelated) is a donor who shares no known ancestry with the recipient. Include adoptive parents/children or step-parents/children. Continue with question 34.

A related donor (allogeneic or syngeneic, related) is a blood-related relative. This includes monozygotic (identical twins), non-monozygotic (dizygotic, fraternal, non-identical) twins, siblings, parents, aunts, uncles, children, cousins, half-siblings, etc. Continue with question 33.

Question 33: Specify the related donor type:

Indicate the relationship and match between the recipient and the donor.

Syngeneic:
Includes: Monozygotic (identical) twins. Occurs when a single egg is fertilized to form one zygote, which then divides into two separate embryos.
Does not include: Other types of twins or HLA-identical siblings (see below).

HLA-identical sibling:
Includes: Non-monozygotic (dizygotic, fraternal, non-identical) twins. Occurs when two eggs are fertilized by two different sperm cells at the same time. This category also includes siblings who aren't twins, but have identical HLA types.
Does not include: Half-siblings (report as "HLA matched other relatives" if their HLA typing is a match, or "mismatched relative" if it does not match).

HLA-matched other relative:
Includes: All blood-related relatives, other than siblings, who are HLA matched (e.g., parents, aunts, uncles, children, cousins, half-siblings).
Does not include: Adoptive parents/children or stepparents/children who are HLA matched.

HLA-mismatched relative:
Includes: Siblings who are not HLA-identical and all other blood-related relatives who have at least one HLA mismatch (e.g., parents, aunts, uncles, children, cousins, half-siblings).
Does not include: Adoptive parents/children or stepparents/children.

Question 34: Was this donor used for any prior cellular therapies?

Indicate if the allogeneic unrelated or related donor reported in question 32 was used for prior cellular therapies or HCT for this recipient. **Do not answer this question for autologous donors.**

The intent of question 34 is to determine whether an HLA Form (Form 2005) has already been completed for this donor so that duplicate reporting may be avoided. For infusions using an NMDP donor or cord blood unit, the donor's HLA typing is reported on NMDP Form 22 (Confirmation of Donor HLA Typing) and the recipient's HLA typing is reported on NMDP Form 117 (Final Recipient HLA Typing). Please contact your CRC if you think the Form 2005(s) should be removed.

Question 35. Is the product genetically modified?

Genetically modified products include any product that was manipulated to alter its gene expression through the insertion of different genes, or editing of genes. An example of a genetically modified product is the manipulation of T-lymphocytes to express Chimeric Antigen Receptors (CAR T-cells) directed towards specific tumor targets (antigens).

Q36-82: Indication for Cellular Therapy

Question 36: What was the indication for performing treatment with cellular therapy?

From the list provided, select the indication for which the recipient is receiving the cellular therapy and continue with question 37 (Date of diagnosis), question 90 (Was the disease assessed prior to cellular therapy?) or question 116 (cellular therapy).

If the indication is any in the list below and the cell therapy is being given post-HCT, no additional consent is required from the patient:

- Promote stem cell engraftment (e.g. co-infusion with HCT)
- Suboptimal donor chimerism (post-HCT)
- Immune reconstitution (post-HCT)
- GVHD prophylaxis (with HCT)
- GVHD treatment (post-HCT)
- Prevent disease relapse (post-HCT)
- Relapsed, persistent or progressive disease (post-HCT)

If the indication for cellular therapy is relapsed, persistent or progressive disease (post-HCT), there must be documented relapse on the appropriate post-HCT follow up form.

Question 37: Date of diagnosis:

Report the date of the first pathological diagnosis (e.g. bone marrow or tissue biopsy) of the disease for which the patient is receiving cellular therapy. Enter the date the sample was collected for examination. If the indication is infection, report the date of diagnosis as the collection date for the first positive microbiology culture. If the diagnosis was determined at an outside center, and no documentation of a pathological or laboratory assessment is available, the dictated date of diagnosis within a physician note may be reported. Do not report the date symptoms first appeared.

If the recipient was diagnosed prenatally (in utero) or if the indication is a congenital disorder, report the date of birth as the date of diagnosis.

If the exact pathological diagnosis date is not known, use the process described in General Instructions, Guidelines for Completing Forms.

Disease Classification Questions
The newest versions of the TED Forms use the World Health Organization (WHO) disease classifications. The Disease Classification questions contain all of the established WHO disease types and subtypes. The "other, specify" category should be used only if the recipient's disease is not one of the listed options. For more information regarding disease classification, consult a transplant physician, contact your center's CIBMTR CRC, or visit the WHO website.

Malignant vs. Non-Malignant
Malignant diseases involve cells dividing without control that can spread to other parts of the body through blood and lymph systems. These diseases are usually characterized by unlimited, aggressive growth, invasion of surrounding tissues, and metastasis.

Non-malignant diseases involve cell overgrowth, but lack the malignant properties of cancer.

The CIBMTR database disease codes are represented in parentheses after the disease subtype on the Disease Classification questions and can be helpful in mapping diagnosis [e.g. Myeloid Sarcoma (295)], and determining if the disease is malignant or non-malignant. Disease codes (10-299) indicate a malignant disease, with the exception of Paroxysmal Nocturnal Hemoglobinuria (PNH) (56). A disease code of (300) or above indicates a non-malignant disease, with the exception of disease code (900), which could indicate either a malignant or non-malignant disease.

Question 38-40: Specify autoimmune disease:

If autoimmune disease is the indication for cellular therapy, indicate the specific disease in question 38. If "other autoimmune bowel disorder", specify in question 39. If "other autoimmune disease", specify in question 40. Continue with question 116.

Question 41-43: Specify cardiovascular disease:

If cardiovascular disease is the indication for cellular therapy, indicate the specific disease in question 41. If "other cardiovascular disease", specify in question 42. If "other peripheral vascular disease", specify in question 43. Continue with question 116.

Question 44-45: Specify musculoskeletal disorder:

If musculoskeletal disorder is the indication for cellular therapy, indicate the specific disorder in question 44. If "other musculoskeletal disorder", specify in question 45. Continue with question 116.

Question 46 & 47: Specify neurologic disease:

If neurologic disease is the indication for cellular therapy, indicate the specific disease in question 46. If "other neurologic disease", specify in question 47. Continue with question 116.

Question 48 & 49: Specify solid tumor:

If solid tumor is the indication for cellular therapy, indicate the specific solid tumor in question 48. If "other solid tumor", specify in question 49. Continue with question 90.

Question 50 & 51: Specify malignant hematologic disorder:

Indicate the broad malignant hematologic disorder in question 50 for which the recipient received cellular therapy. If "other malignant hematologic disorder", specify in question 51.

Question 52: Specify the AML classification:
If "acute myeloid leukemia (AML)" is the broad malignant hematologic disorder, report the sub-type in Q52. Continue with question 90.

Question 53: Specify the ALL classification:
If "acute lymphoblastic leukemia (ALL)" the broad malignant hematologic disorder, report the sub-type in question 53.

Question 54 & 55: Specify other acute leukemia classification:
If "other acute leukemia" is the broad malignant hematologic disorder, report the sub-type in question 54. If "other acute leukemia", specify in question 55. Continue with question 90.

Question 56: Specify CML classification:
If "Chronic myelogenous leukemia (CML)" is the broad malignant hematologic disorder, report the sub-type in question 56. Continue with question 90.

Question 57: What was the MDS / MPN subtype?
If "MDS/MPN" is the broad malignant hematologic disorder, report the sub-type in question 57. Continue with question 90.

Question 58: Specify the CLL classification:
If "Chronic lymphocytic leukemia" is the broad malignant hematologic disorder, report the sub-type in question 58. Continue with question 90.

Question 59: Specify Hodgkin lymphoma classification:
If “Hodgkin lymphoma” is the broad malignant hematologic disorder, report the sub-type in question 59. Continue with question 90.

Question 60 & 61: Specify Non-Hodgkin lymphoma classification:
If “non-Hodgkin Lymphoma” is the broad malignant hematologic disorder, report the sub-type in question 60. If “other lymphoma”, specify in question 61. Continue with question 90.

Question 62 & 63: Specify the multiple myeloma/plasma cell disorder (PCD) classification:
If “multiple myeloma / plasma cell disorder (PCD)” is the broad malignant hematologic disorder, report the sub-type in question 62. If “other plasma cell disorder”, specify in question 63. Continue with question 90.

Question 64: Specify non-malignant hematologic disorder
Indicate the broad non-malignant hematologic disorder in question 64 for which the recipient received cellular therapy.

If “Hemophilia A or B” is the broad non-malignant hematologic disorder, continue with question 116.

Question 65 & 66: Specify severe aplastic anemia classification:
If “Severe aplastic anemia” is the broad non-malignant hematologic disorder, report the sub-type in question 65. If “other acquired cytopenic syndrome”, specify in question 66. Continue with question 116.

Question 67-69: Specify inherited abnormalities of erythrocyte differentiation or function classification:
If “Inherited abnormalities of erythrocyte differentiation or function” is the broad non-malignant hematologic disorder, report the sub-type in Q67. If “other constitutional anemia”, specify in question 68. If “other hemoglobinopathy”, specify in question 69. Continue with question 116.

Question 70-72: Specify disorder of immune system classification:
If “Disorder of immune system” is the broad non-malignant hematologic disorder, report the sub-type in question 70. If “other SCID”, specify in question 71, if “other immunodeficiency”, specify in question 72. Continue with question 116.

Question 73 & 74: Specify inherited abnormalities of platelets classification:
If “Inherited abnormalities of platelets” is the broad non-malignant hematologic disorder, report the sub-type in question 73. If “other inherited platelet abnormality”, specify in question 74. Continue with question 116.

Question 75 & 76: Specify inherited disorders of metabolism classification:
If “Inherited disorders of metabolism” is the broad non-malignant hematologic disorder, report the sub-type in question 75. If “other inherited metabolic disorder”, specify in question 76. Continue with question 116.

Question 77 & 78: Specify histiocytic disorder classification:
If “histiocytic disorder” is the broad non-malignant hematologic disorder, specify in question 77. If “other histiocytic disorder”, specify in question 78. Continue with question 116.

Question 79:
If “Other non-malignant disorder” is the broad non-malignant hematologic disorder, specify in question 79. Continue with question 116.

Question 80: Ocular

If “ocular” is the indication for which the recipient is receiving the cellular therapy reported in question 36, specify in Q80.

Question 81: Pulmonary

If “pulmonary” is the indication for which the recipient is receiving the cellular therapy reported in question 36, specify in Q81

Question 82: Other

If “other” is the indication for which the recipient is receiving the cellular therapy reported in question 36, then specify in Q82.

Q83-89: Infection

Question 83-89: Specify organism:

Indicate the fungal or viral organism for which the patient is receiving a cellular therapy. Only one instance is required, the other instances may be skipped.

Organism:

From the table entitled "Codes for Commonly Reported Organisms," select the code corresponding to the identified infection as reported on the microbiology, laboratory report, or other physician documentation. Report the code in the boxes provided. If the specific organism is not listed, use the "other, specify" code (259 – fungus, 329 – virus, etc.) and report the name of the organism in the space provided.

Fungal infections: Note the inclusion of Pneumocystis (formerly found under parasites). The most commonly found fungal infections are Candida (C. albicans, C. tropicalis, C. glabrata [also known as Torulopsis glabrata], C. parapsilosis, C. krusei), Aspergillus (A. fumigatus), Fusarium sp., and Zygomycetes.

- For fungal species marked with a section symbol (§), also complete a Fungal Infection Form (2146).

Viral infections: Caused by exposure to a new virus or reactivation of a dormant virus already present in the body. The most common viral infections are due to HSV (Herpes simplex), VZV (Varicella zoster, shingles), and CMV (Cytomegalovirus). If the site of CMV is the lung, confirm whether the patient had interstitial pneumonitis rather than CMV pneumonia.

- For hepatitis infections marked with a dagger symbol (†), also complete a Hepatitis Form (2147).
- For HIV infections marked with a currency symbol (¤), also complete an HIV Infection Form (2148).

Q90-115: Disease Assessment at Last Evaluation Prior to Cellular Therapy

These questions are for malignant disease indications or relapsed, persistent or progressive disease only.
This section is not completed if indication for cellular therapy is "Promote stem cell engraftment" (e.g. co-infusion with HCT), "Suboptimal donor chimerism" (post-HCT), "Immune reconstitution" (post-HCT), "GVHD prophylaxis (with HCT)", "GVHD treatment (post-HCT)", or "Prevent disease relapse (post-HCT)". Continue with question 116.

Specify the method(s) of disease detection below. For each method used, if the result was positive report the first date the disease was detected; if the result was negative report the last date the method was used prior to cellular therapy.

Question 90: Was the disease assessed prior to the cellular therapy?

Indicate if the disease status was assessed prior to the cellular therapy. If "yes', continue with question 91. If "no", continue with question 116.

Disease Assessment Method:
This section should be completed for every malignant disease. Not all diseases have molecular and/or cytogenetic/FISH abnormalities identified to monitor disease status. If a disease assessment was done, but has always been normal, check "not applicable." In some circumstances, disease may be detected by molecular or cytogenetic testing, but may not be considered a relapse or progression. Test results should still be reported.

Question 91: Was the disease status assessed by molecular testing (e.g. PCR)?

Molecular assessment involves determining whether a molecular marker for the disease exists in the blood or bone marrow. Molecular assessment is the most sensitive method of detection, and can indicate known genetic abnormalities associated with the disease for which the cellular therapy was performed. RFLP testing (with PCR amplification) is an example of a molecular test method used to detect BCR/ABL.

Select "yes" if a molecular method was used to determine disease status at the last evaluation prior to cellular therapy and continue with question 92. If a molecular method was not used to determine disease status, check "no" and continue with question 95. If a molecular method was used to evaluate the disease status, but has never been positive, check "not applicable" and continue with question 95.

Question 92: Date sample collected:

Indicate the date the sample was collected for disease assessment by molecular method.

For more information regarding reporting partial or unknown dates, see General Instructions, General Guidelines for Completing Forms.

Question 93: Was disease detected?

If molecular markers for disease were found, check “yes” and continue with question 94.

If molecular markers for were not found, check “no” and continue with question 95.

Question 94: Was the status considered a disease relapse or progression?

If the physician believes the test results indicate disease relapse or progression, check “yes.” If the recipient has a positive test result, but the physician does not believe the result represents relapse or progression (e.g., a recipient transplanted for CML exhibits such a low level of BCR-ABL positivity post-HSCT that the physician does not believe is disease), check “no.” Continue with question 95.

Question 95: Was the disease status assessed via flow cytometry (immunophenotyping)?

Flow cytometry is a technique that can be performed on blood, bone marrow, or tissue preparations where cell surface markers can be quantified on cellular material.

Select “yes” if flow cytometry was used to determine disease status at the last evaluation prior to cellular therapy and continue with question 96. If flow cytometry was not performed or could not be used to determine disease status, check “no” or “not applicable”, respectively and continue with question 99.

Question 96: Date sample collected:

Indicate the date the sample was collected for disease assessment by flow cytometry.

For more information regarding reporting partial or unknown dates, see General Instructions, General Guidelines for Completing Forms.

Question 97: Was disease detected?

If flow cytometry detected disease, check “yes” and continue with question 98.

If flow cytometry did not detect disease, check “no” and continue with question 99.

Question 98: Was the status considered a disease relapse or progression?

If the physician believes the test results indicate disease relapse or progression, check “yes.” If the recipient has a positive test result, but the physician does not believe the result represents relapse or progression, check “no.” Continue with question 99.

Question 99: Was the disease status assessed by cytogenetic testing (karyotyping or FISH)?

Cytogenetic assessment involves testing blood or bone marrow for the presence of a known cytogenetic abnormality that reflects the recipient’s disease. FISH is categorized with cytogenetics. Although often used for finding specific features in DNA, FISH is not as sensitive as molecular methods, even though the markers identified may be the same.

Select “yes” if cytogenetic testing was used to determine disease status at the last evaluation prior to cellular therapy and continue with question 100. If cytogenetic testing was not performed or was not used to determine disease status, check “no” or “not applicable”, respectively and continue with question 108.

Question 100: Was the disease status assessed by karyotyping?

Select “yes” if karyotyping was used to determine disease status at the last evaluation prior to cellular therapy. If karyotyping was not performed or used to determine disease status, check “no” or “not applicable”, respectively and continue with question 101.

Question 101: Date sample collected:

Indicate the date the sample was collected for disease assessment by karyotyping.

For more information regarding reporting partial or unknown dates, see General Instructions, General Guidelines for Completing Forms.

Question 102: Was disease detected?

If disease was detected by karyotyping, check “yes” and continue with question 103.

If disease was not detected by karyotyping, check “no” and continue with question 104.

Question 103: Was the status considered a disease relapse or progression?

If the physician believes the test results indicate disease relapse or progression, check “yes.” If the recipient has a positive test result, but the physician does not believe the result represents relapse or progression, check “no.”

Question 104: Was the disease status assessed by FISH?

Select “yes” if FISH was used to determine disease status at the last evaluation prior to cellular therapy. If FISH was not performed or used to determine disease status, check “no” or “not applicable”, respectively and continue with question 105.

Question 105: Date sample collected:

Indicate the date the sample was collected for disease assessment by FISH.

For more information regarding reporting partial or unknown dates, see General Instructions, General Guidelines for Completing Forms.

Question 106: Was disease detected?

If FISH markers for disease were found, check “yes” and continue with question 107.

If FISH markers for disease were not found, check “no” and continue with question 108.

Question 107: Was the status considered a disease relapse or progression?

If the physician believes the test results indicate disease relapse or progression, check “yes.” If the recipient has a positive test result, but the physician does not believe the result represents relapse or progression, check “no.”

Question 108: Was the disease status assessed by radiological assessment? (e.g. PET, MRI, CT)

Select “yes” if a radiologic assessment was used to determine disease status at the last evaluation prior to cellular therapy. If a radiologic assessment was not performed or used to determine disease status, check “no” or “not applicable”, respectively and continue with question 109.

Question 109: Date assessed:

Indicate the date the disease was assessed by radiological assessment.

For more information regarding reporting partial or unknown dates, see General Instructions, General Guidelines for Completing Forms.

Question 110: Was disease detected?

If radiologic evidence of disease was found, check “yes”.

If radiologic evidence of disease was not found, check “no”.

Question 111: Was the disease status assessed by clinical / hematologic assessment?

Clinical/hematologic assessment is the least sensitive method of disease detection. Examples include circulating blasts in the bloodstream for AML, and enlargement of a malignant mass for lymphoma or a solid tumor, as determined physically. Every recipient who has an evaluation by a physician has a “clinical” assessment. If “yes”, continue with question 112. If “no”, continue with question 114.

Question 112: Date assessed:

Indicate the date the disease was assessed by clinical / hematologic method.

For more information regarding reporting partial or unknown dates, see General Instructions, General Guidelines for Completing Forms.

Question 113: Was disease detected?

If clinical/hematologic evidence of disease was found, check “yes.”

If clinical/hematologic evidence of disease was not found, check “no.”

Question 114: What was the recipient’s disease status immediately prior to the cellular therapy?

Based on the results reported above and in conjunction with established disease criteria, indicate if the disease status immediately prior to the cellular therapy was “Complete Remission” or “Not in Complete Remission”.

Question 115: Date assessed:

Indicate the date assessed of the disease status reported in question 114.

For more information regarding reporting partial or unknown dates, see General Instructions, General Guidelines for Completing Forms.

Q116-119: Therapy Prior to Cellular Therapy

Question 116: Was systemic therapy given immediately prior to cellular therapy as part of the cellular therapy protocol?

Indicate “yes” if the recipient received systemic therapy prior to the cellular therapy infusion or “no” if they did not. Systemic therapy may include intravenous or oral chemotherapy with the intent to deplete circulating lymphocytes, reduce tumor burden or other. If “yes”, continue with question 117. If “no, continue with question 120.

Question 117: Date started:

Indicate the date the systemic therapy started.

Question 118: Specify the reason for which the systemic therapy was given per protocol:

If systemic therapy was given as “Lympho-depleting therapy” or for “Reduction of tumor burden”, continue with question 120. If systemic therapy was given for another reason, specify in question 119.

Question 119: Specify other indication:

If a reason other than “Lympho-depleting therapy” or “Reduction of tumor burden” for the systemic therapy, specify in question 119.

Q120-122: Functional Status

Specify the functional status of the recipient immediately prior to the cellular therapy

Question 120: What scale was used to determine the recipient's functional status prior to the cellular therapy?

The CIBMTR uses the Karnofsky/Lansky scale to determine the functional status of the recipient immediately prior to the start of the cellular therapy. The Karnofsky Scale is designed for recipients aged 16 years and older, and is not appropriate for children under the age of 16. The Lansky Scale is designed for recipients one year old to less than 16 years old. For recipients less than one year old, questions 120-122 should be left blank.

Select the appropriate performance scale, Karnofsky or Lansky, based on the recipient's age.

Question 121 & 122: Performance score prior to the cellular therapy:

Recipient performance status is a critical data field that has been determined to be essential for all outcome-based studies. The CIBMTR uses the Karnofsky/Lansky scale to determine the functional status of the recipient immediately prior to the start of the cellular therapy. **For the purposes of this manual, the term "immediately prior" represents approximately one month prior to the cellular therapy infusion.**

Using the appropriate scale as selected in question 120, select the score (10-100) that best represents the recipient's activity status immediately prior to the start of the preparative regimen. For an example of the Karnofsky/Lansky scale, see Appendix L.

If a Karnofsky/Lansky score is not documented in the source documentation (e.g., inpatient progress note, physician's clinic note), data management professionals should not assign a performance score based on analysis of available documents. Rather, a physician should provide documentation of the performance score.

The CIBMTR recognizes that some transplant centers prefer to collect and use the ECOG performance score as opposed to the Karnofsky/Lansky score. Although the ECOG and Karnofsky/Lansky performance score systems are based on similar principles, the scales are not the same. For example, the Karnofsky/Lansky scale is described in 11 categories, whereas the ECOG performance status is reported in six categories. Due to the overlap between the two systems, an ECOG score of "one" can represent either "80" or "90" on the

Karnofsky/Lansky scale. For centers that collect only an ECOG performance score, CIBMTR will make the following accommodations when auditing the source data: Centers collecting ECOG scores should do so using standard practices to ensure accuracy. For the purposes of CIBMTR reporting, conversion of ECOG to Karnofsky/Lansky should follow a standard and consistent practice. This practice should be clear and reproducible.

For more information regarding converting an EGOG score to a Karnofsky/Lansky score, see Appendix L.