AUTOLOGOUS HEMATOPOIETIC GENE THERAPY
--- Day 100, 6 Months, Annual & Unscheduled Follow-Up ---

SURVIVAL STATUS

Date of follow-up _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)*
*(*if died: date of death, if lost to follow up: date last seen*)*

Survival status:

☐ Alive
☐ Dead
☐ Lost to follow-up

Main cause of death:
(check only one main cause)

☐ Relapse or progression/persistent disease	
☐ Secondary malignancy	
☐ CT-related	**Select treatment related cause:** *(select all that apply)* ☐ Graft versus Host Disease ☐ Non-infectious complication ☐ Infectious complication: *(select all that apply)* ☐ Bacterial infection ☐ Viral infection ☐ Fungal infection ☐ Parasitic infection ☐ Infection with unknown pathogen
☐ HCT-related	
☐ GT-related	
☐ IST-related	
☐ Other; specify: _____	
☐ Unknown	

Was an autopsy performed?

☐ No
☐ Yes
☐ Unknown

Assessment period covered by this report:

☐ Day 100
☐ 6 months
☐ 12 months (1 year)
☐ 18 months
☐ 24 months (2 years)
☐ Annual or unscheduled Follow-Up (*up to 15 years*)

BEST RESPONSE

Complete only for Day 100 and 6 Months Follow-Up
Only for Sickle cell disease

Best clinical/biological response after this GT* *(observed before any subsequent treatment)*: _____

* Indicate the best clinical/biological response after GT corresponding to indication diagnosis for GT was given by selecting from the list provided in Appendix 1

RECOVERY

Complete only for Day 100 and 6 Months Follow-Up

Absolute neutrophil count (ANC) recovery *(neutrophils ≥ 0.5x10^9/L)*:

- ☐ No: **Date of the last assessment:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown
- ☐ Yes: **Date of ANC recovery:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown
  (first of 3 consecutive values after 7 days without transfusion containing neutrophils)
- ☐ Never below
- ☐ Not evaluated
- ☐ Unknown

Platelet reconstitution *(platelets ≥ 20x10^9/L:)*:

- ☐ No: **Date of the last assessment:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown
- ☐ Yes: **Date of platelet reconstitution:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown
  (first of 3 consecutive values after 7 days without platelet transfusion)
- ☐ Never below
- ☐ Not evaluated
- ☐ Unknown

Date of the last platelet transfusion: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Not applicable *(not transfused)* ☐ Unknown

THERAPY SUCCESS
only for Primary Immunodeficiencies

Engraftment of the modified stem cells assessed?

☐ No

☐ Yes: **Date evaluated:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown

For gene transfer Gene Therapy only *For gene editing Gene Therapy only*

T cells	**VCN:**_____	☐ Not evaluated ☐ Unknown	**Gene editing efficiency:**_____%	☐ Not evaluated ☐ Unknown
B cells	**VCN:**_____	☐ Not evaluated ☐ Unknown	**Gene editing efficiency:**_____%	☐ Not evaluated ☐ Unknown
NK cells	**VCN:**_____	☐ Not evaluated ☐ Unknown	**Gene editing efficiency:**_____%	☐ Not evaluated ☐ Unknown
PMN	**VCN:**_____	☐ Not evaluated ☐ Unknown	**Gene editing efficiency:**_____%	☐ Not evaluated ☐ Unknown
Monocytes	**VCN:**_____	☐ Not evaluated ☐ Unknown	**Gene editing efficiency:**_____%	☐ Not evaluated ☐ Unknown
Other; specify: _____	**VCN:**_____	☐ Not evaluated ☐ Unknown	**Gene editing efficiency:**_____%	☐ Not evaluated ☐ Unknown

☐ Not evaluated

THERAPY SUCCESS
only for Haemoglobinopathies

For gene transfer Gene Therapy only

Vector copy number (VCN):_____ ☐ Not evaluated ☐ Unknown

For gene editing Gene Therapy only

Gene-edited cells:_____% ☐ Not evaluated ☐ Unknown

HbF _____ % ☐ Not evaluated ☐ Unknown

For Sickle Cell Disease only

HbS _____ % ☐ Not evaluated ☐ Unknown

For Bluebird Bio product only

H87q _____ % ☐ Not evaluated ☐ Unknown

Other therapy specific recovery; specify:_____

CURRENT HAEMATOLOGICAL FINDINGS

Haemoglobin	_____ g/dL	☐ Not evaluated	☐ Unknown
Ferritin	_____ ng/mL	☐ Not evaluated	☐ Unknown

Extended dataset

Antimicrobial prophylaxis

Did the patient receive prophylaxis for bacterial, viral or fungal infection during this follow-up period? ☐ No ☐ Yes

If yes, what type of prophylaxis? ☐ Antibacterial ☐ Antifungal ☐ Antiviral
(select all that apply and complete the relevant section)

Antibacterial

Antibiotic *(select all that were administered)*	**Phase** *Day 100 0nly*	**Responses for > 100 days only**
☐ Ciprofloxacin	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Levofloxacin	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Moxifloxacin	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Penicillin	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown

Extended dataset

Antibacterial

Antibiotic *(select all that were administered)*	**Phase** *Day 100 only*	**Responses for > 100 days only**
☐ Non-absorbable antibiotic	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown

Final date antibacterial prophylaxis was discontinued: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Ongoing ☐ Unknown

Extended dataset

Antiviral

Did the patient receive cytomegalovirus (CMV) prophylaxis during this follow-up period?

☐ No

☐ Yes: **Which drugs were used?** *(select all that apply)*

- ☐ High-dose acyclovir
- ☐ High-dose valacyclovir
- ☐ Gancyclovir intravenous
- ☐ Valgancyclovir
- ☐ Foscarnet
- ☐ Other drug

Final date CMV prophylaxis was discontinued: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Ongoing ☐ Unknown

Did the patient receive prophylaxis for varicella-zoster virus (VZV) or herpes simplex virus (HSV) with either acyclovir or valacyclovir during this follow-up period?

☐ No

☐ Yes: **Final date VZV or HSV prophylaxis was discontinued:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Ongoing ☐ Unknown

Did the patient receive rituximab or another anti-CD20 monoclonal drug as prophylaxis for Epstein-Barr virus post-transplant lymphoproliferative disorder (EBV-PTLD) during this follow-up period?

☐ No

☐ Yes

Did the patient receive prophylaxis for hepatitis B virus (HBV) during this follow-up period?

☐ No

☐ Yes:

Which drugs were used? *(select all that apply)*

- ☐ Lamivudine
- ☐ Entecavir
- ☐ Tenofovir
- ☐ Other drug

Final date HBV prophylaxis was discontinued: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Ongoing ☐ Unknown

Extended dataset

Antifungal

Antifungal *(select all that were administered)*	**Phase** *Day 100 0nly*	**Responses for > 100 days only**
☐ Fluconazole	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Voriconazole	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Posaconazole	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Itraconazole	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown

Antifungal

Antifungal *(select all that were administered)*	**Phase** *Day 100 0nly*	**Responses for > 100 days only**
☐ Caspofungin	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Micafungin	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Anidulafungin	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown
☐ Ambisome (IV or inhalations)	☐ Pre-engraftment ☐ Post-engraftment; specify: ☐ Only post-engraftment ☐ Started pre-engraftment and continued into post-engraftment ☐ Started and stopped in pre-engraftment phase and restarted in post-engraftment phase ☐ Unknown	☐ Started in this follow-up period; **Start date:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown ☐ Ongoing since previous follow-up ☐ Unknown

Final date antifungal prophylaxis was discontinued: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Ongoing ☐ Unknown

Extended dataset

Did the patient receive prophylaxis for *Pneumocystis jirovecii* pneumonia (PJP) during this follow-up period?

☐ No

☐ Yes: **Which drugs were used?** *(select all that apply)*
- ☐ Trimethoprim-sulfamethoxazole
- ☐ Dapsone
- ☐ Atovaquone
- ☐ Pentamidine inhaled
- ☐ Pentamidine intravenous
- ☐ Other drug

Final date prophylaxis was discontinued: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Ongoing ☐ Unknown

☐ Unknown

Pre-emptive viral therapy

Did the patient receive pre-emptive therapy for a viral infection during this follow-up period ? ☐ No ☐ Yes

If yes, for what virus? *(select all that apply)* ☐ CMV ☐ EBV

Specify the pre-emptive therapy for each CMV episode that occurred during this follow-up period

CMV treatment start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown

Antiviral(s) used:
(Select all that apply)
- ☐ Valgancyclovir
- ☐ Gancyclovir intravenous
- ☐ Foscarnet
- ☐ Cidofovir
- ☐ Maribavir
- ☐ Specific CMV T-cell
- ☐ Other drug

Was this episode of CMV infection due to a resistant CMV strain?
☐ No ☐ Yes ☐ Unknown

Copy as often as necessary to reflect all episodes that occurred

Specify the pre-emptive therapy for each EBV episode that occurred during this follow-up period

EBV treatment start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown

Antiviral(s) used:
(Select all that apply)
- ☐ Rituximab
- ☐ Specific EBV T-cells
- ☐ Other drug

Copy as often as necessary to reflect all episodes that occurred

COMPLICATIONS SINCE THE LAST REPORT
-- Non-infectious complications --

Do not report complications that were resolved before the Gene Therapy
Do not report complications that were previously reported as resolved, unless they recurred

Did non-infectious complications occur during the follow-up period?

☐ No ((*proceed to 'Complications since the last report - Infectious complications')*
☐ Yes (report in the table below)

Macrophage activation syndrome (MAS)

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date** (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Secondary haemophagocytic lymphohistiocytosis (HLH)

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date** (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Organ toxicity: skin

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date** (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

*Grade 0-2

COMPLICATIONS SINCE THE LAST REPORT
-- Non-infectious complications --

Organ toxicity: liver

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date (***YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Organ toxicity: lung

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date (***YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Organ toxicity: heart

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date (***YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Organ toxicity: kidney

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date (***YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

* Grade 0-2

COMPLICATIONS SINCE THE LAST REPORT
-- Non-infectious complications --

Organ toxicity: gastrointestinal

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date (***YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Other organ toxicity observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Organ specify: _____

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date (***YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Tumour lysis syndrome (TLS)

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date (***YYYY/MM/DD***):** _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

* Grade 0-2

COMPLICATIONS SINCE THE LAST REPORT
-- Non-infectious complications --

Cytopenia

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date** (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Idiopathic pneumonia syndrome

Complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date** (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

Other complication observed during this follow-up period? ☐ No*
☐ Yes: ☐ Newly developed ☐ Ongoing since previous assessment
☐ Unknown

Specify: _____ *Consult appendix 4 for a list of complications that should not be reported*
(Indicate CTCAE term)

Maximum CTCAE grade observed during this period: ☐ 3 ☐ 4 ☐ 5 (fatal) ☐ Unknown

Onset date (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown *Only if newly developed*

Resolved: ☐ No
☐ Yes; **Stop date** (*YYYY/MM/DD*): _ _ _ _ / _ _ / _ _ ☐ Unknown
☐ Unknown

If more other complications occurred, copy and fill-in this table as many times as necessary.

* Grade 0-2

COMPLICATIONS SINCE THE LAST REPORT
-- Infectious complications --

Do not report infections that were already reported as resolved on the previous assessment and did not reoccur.

Did infectious complications occur during the follow-up period?

☐ No *Consult appendix 4 for a list of complications that should not be reported*

☐ Yes (*report all infectious complications below*)

Bacterial infection: ☐ No ☐ Yes

1) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* *only if newly developed*

☐ Gram-positive ☐ Gram-negative ☐ Other

Pathogen*: _____

Infection with clinical implications: ☐ No ☐ Yes: *(select all that apply during this period)*

☐ Symptoms/signs of disease

☐ Administration of pathogen-directed therapy

☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term):** _____

Localisation 2 (CTCAE term):** _____

Localisation 3 (CTCAE term):** _____

Intravascular catheter-related infection: ☐ No ☐ Yes; specify***: _____ ☐ Unknown

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)

Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

2) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* *only if newly developed*

☐ Gram-positive ☐ Gram-negative ☐ Other

Pathogen*: _____

Infection with clinical implications: ☐ No ☐ Yes: *(select all that apply during this period)*

☐ Symptoms/signs of disease

☐ Administration of pathogen-directed therapy

☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term):** _____

Localisation 2 (CTCAE term):** _____

Localisation 3 (CTCAE term):** _____

Intravascular catheter-related infection: ☐ No ☐ Yes; specify***: _____ ☐ Unknown

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)

Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

If more than 2 bacterial infections, copy and fill-in this table as many times as necessary.

* Indicate the pathogen and sub-type (if applicable) by choosing from the list of pathogens provided in Appendix 2
** Indicate CTCAE term by choosing from the list provided in Appendix 3
*** If intravascular catheter-related infection, specify it by choosing from the list provided in Appendix 5

COMPLICATIONS SINCE THE LAST REPORT
-- Infectious complications -- continued

Viral infection: ☐ No ☐ Yes

1) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

Pathogen*: _____

If the pathogen was CMV/EBV: **Was this infection a reactivation?** ☐ No ☐ Yes

Infection with clinical implications: ☐ No ☐ Yes: *(select all that apply during this period)*
☐ Symptoms/signs of disease
☐ Administration of pathogen-directed therapy
☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term):** _____

Localisation 2 (CTCAE term):** _____

Localisation 3 (CTCAE term):** _____

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)
Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

2) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

Pathogen*: _____

If the pathogen was CMV/EBV: **Was this infection a reactivation?** ☐ No ☐ Yes

Infection with clinical implications: ☐ No ☐ Yes: *(select all that apply during this period)*
☐ Symptoms/signs of disease
☐ Administration of pathogen-directed therapy
☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term):** _____

Localisation 2 (CTCAE term):** _____

Localisation 3 (CTCAE term):** _____

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)
Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

If more than 2 viral infections, copy and fill-in this table as many times as necessary.

* Indicate the pathogen and sub-type (if applicable) by choosing from the list of pathogens provided in Appendix 2
** Indicate CTCAE term by choosing from the list provided in Appendix 3
*** If intravascular catheter-related infection, specify it by choosing from the list provided in Appendix 5

COMPLICATIONS SINCE THE LAST REPORT
-- Infectious complications -- continued

Fungal infection: ☐ No ☐ Yes

1) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

☐ Yeasts ☐ Moulds

Pathogen*: _____

Infection with clinical implications: ☐ No ☐ Yes: *(select all that apply during this period)*

☐ Symptoms/signs of disease

☐ Administration of pathogen-directed therapy

☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term)**: _____

Localisation 2 (CTCAE term)**: _____

Localisation 3 (CTCAE term)**: _____

Intravascular catheter-related infection: ☐ No ☐ Yes; specify***: _____ ☐ Unknown

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)

Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

2) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

☐ Yeasts ☐ Moulds

Pathogen*: _____

Infection with clinical implications: ☐ No ☐ Yes: *(select all that apply during this period)*

☐ Symptoms/signs or disease

☐ Administration of pathogen-directed therapy

☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term)**: _____

Localisation 2 (CTCAE term)**: _____

Localisation 3 (CTCAE term)**: _____

Intravascular catheter-related infection: ☐ No ☐ Yes; specify***: _____ ☐ Unknown

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)

Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

If more than 2 fungal infections, copy and fill-in this table as many times as necessary.

* Indicate the pathogen and sub-type (if applicable) by choosing from the list of pathogens provided in Appendix 2
** Indicate CTCAE term by choosing from the list provided in Appendix 3
*** If intravascular catheter-related infection, specify it by choosing from the list provided in Appendix 5

COMPLICATIONS SINCE THE LAST REPORT
-- Infectious complications -- continued

Parasitic infection: ☐ No ☐ Yes

1) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

☐ Protozoa ☐ Helminths

Pathogen*: _____

Infection with clinical implications: ☐ No
☐ Yes: *(select all that apply during this period)*
☐ Symptoms/signs or disease
☐ Administration of pathogen-directed therapy
☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term)**: _____

Localisation 2 (CTCAE term)**: _____

Localisation 3 (CTCAE term)**: _____

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)

Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

2) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

☐ Protozoa ☐ Helminths

Pathogen*: _____

Infection with clinical implications: ☐ No
☐ Yes: *(select all that apply during this period)*
☐ Symptoms/signs or disease
☐ Administration of pathogen-directed therapy
☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term)**: _____

Localisation 2 (CTCAE term)**: _____

Localisation 3 (CTCAE term)**: _____

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)

Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

If more than 2 parasitic infections, copy and fill-in this table as many times as necessary.

* Indicate the pathogen and sub-type (if applicable) by choosing from the list of pathogens provided in Appendix 2
** Indicate CTCAE term by choosing from the list provided in Appendix 3
*** If intravascular catheter-related infection, specify it by choosing from the list provided in Appendix 5

COMPLICATIONS SINCE THE LAST REPORT
-- Infectious complications -- continued

Infection with unknown pathogen: ☐ No ☐ Yes:
(for clinical infections without microbiological documentation, like pneumonia, cellulitis, etc.)

1) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

Infection with clinical implications: ☐ No
☐ Yes: *(select all that apply during this period)*
☐ Symptoms/signs or disease
☐ Administration of pathogen-directed therapy
☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term)*: _____

Localisation 2 (CTCAE term)*: _____

Localisation 3 (CTCAE term)*: _____

Intravascular catheter-related infection: ☐ No
☐ Yes; specify**: _____
☐ Unknown

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)
Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

2) **New or ongoing:** ☐ Newly developed ☐ Ongoing since previous assessment

Start date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD) only if newly developed*

Infection with clinical implications: ☐ No
☐ Yes: *(select all that apply during this period)*
☐ Symptoms/signs or disease
☐ Administration of pathogen-directed therapy
☐ Unknown

Indicate at least 1 location involved during this period:

Localisation 1 (CTCAE term)*: _____

Localisation 2 (CTCAE term)*: _____

Localisation 3 (CTCAE term)*: _____

Intravascular catheter-related infection: ☐ No
☐ Yes; specify**: _____
☐ Unknown

Resolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)
Contributory cause of death: ☐ No ☐ Yes ☐ Unknown

If more than 2 infections with unknown pathogen, copy and fill-in this table as many times as necessary.

* Indicate CTCAE term by choosing from the list provided in Appendix 3
** If intravascular catheter-related infection, specify it by choosing from the list provided in Appendix 5

COMPLICATIONS SINCE THE LAST REPORT
-- Infectious complications -- continued

Extended dataset

SARS-CoV-2 RELATED QUESTION

Did the patient receive a vaccination against SARS-CoV-2 during this follow-up period?

☐ No

☐ Yes: Number of doses: _____ ☐ Unknown

Date of the last dose: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown

☐ Unknown

SECONDARY MALIGNANCIES AND AUTOIMMUNE DISORDERS

Did a secondary malignancy or autoimmune disorder occur during this follow-up period?

☐ No

☐ Yes: **Diagnosis:** _____

Date of diagnosis: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)*

Histologic type *(if applicable)*: _____

Location *(if applicable)*: _____

Secondary malignancy material preserved:	**Concomitant PBMCs preserved:**
☐ No	☐ No
☐ Yes	☐ Yes
☐ Unknown	☐ Unknown

☐ Unknown

Viral vectors: *For gene transfer Gene Therapy only*

Did insertional mutagenesis occur?

☐ No

☐ Yes:

Integration site; specify_____ ☐ Not evaluated ☐ Unknown

Integration site clonal diversity: *(Shannon diversity index)*

- ☐ Very High
- ☐ High
- ☐ Moderate
- ☐ Low
- ☐ Very Low
- ☐ Not evaluated
- ☐ Unknown

☐ Not evaluated

☐ Unknown

ADDITIONAL CELL INFUSIONS

Did the patient receive an (salvage infusion) autologous boost?

☐ No

☐ Yes: **Date of the (salvage infusion) autologous boost**: _ _ _ _/ _ _/ _ _ *(YYYY/MM/DD)* ☐ Unknown

RECURRENCE OF DISEASE

only for Haemoglobinopathies

Was there a recurrence of disease since last follow-up? *(detected by any method)*

☐ No

☐ Yes; *for every recurrence complete the question below*

Date of recurrence: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown

copy and fill-in this table as many times as necessary.

HOSPITAL ADMISSION

Complete only for Day 100 and 6 Months Follow-Up.

Was inpatient admission and care needed since the last follow-up?

☐ No

☐ Yes: **Number of days in hospital**: _____

☐ Unknown

Was the patient transferred to the intensive care unit (ICU) since the last follow-up?

☐ No

☐ Yes: **Number of days in ICU**: _____

☐ Unknown

PATIENT STATUS

Performance status at the last assessment *(choose only one)*:

Type of scale used:	Score:									
☐ Karnofsky ☐ Lansky	☐ 10	☐ 20	☐ 30	☐ 40	☐ 50	☐ 60	☐ 70	☐ 80	☐ 90	☐ 100
☐ ECOG	☐ 0	☐ 1	☐ 2	☐ 3	☐ 4					

DISEASE STATUS

Disease specific

Disease status at this follow-up or at time of death*: _____

* Indicate the disease status at this follow-up or at time of death corresponding to indication diagnosis by selecting from the list provided in Appendix 1

PREGNANCY AFTER GENE THERAPY

Complete only after 6 Months

Has patient become pregnant or impregnated another person since last follow-up?

☐ No;

Extended dataset
Was there an attempted pregnancy since last follow-up? ☐ No ☐ Yes ☐ Unknown

☐ Yes: **Did the pregnancy result in a live birth?**

- ☐ No; **Date of spontaneous or induced termination**: _ _ _ _ / _ _ / _ _ (YYYY/MM/DD) ☐ Unknown
- ☐ Yes; **Year of birth:** _ _ _ _ (YYYY) **Month of birth:** _ _ (MM) ☐ Unknown
- ☐ Still pregnant at time of follow-up
- ☐ Unknown

Extended dataset
Conception method: ☐ Natural ☐ Assisted ☐ Unknown

☐ Unknown

END OF GENERAL FOLLOW-UP REPORTING

TO COMPLETE FOLLOW-UP REPORTING, PLEASE FILL IN THE APPLICABLE DIAGNOSE-SPECIFIC QUESTIONS ATTACHED TO THIS FORM

Appendix 1
Disease Status

Extended dataset

Immunomodulatory treatments
(Only for Inborn errorrs of immunity)

Select the immunomodulatory treatments the patient received within 3 months prior to follow-up.

Only report treatments administered in the 3 months before this follow-up. Do not report treatments for GT related complications, only for the underlying disease

- ☐ No treatment given
- ☐ IVIG
- ☐ SCIG
- ☐ Steroids (>0.5 mg/kg/day prednison equivalent)
- ☐ Cyclosporine A
- ☐ Tacrolimus
- ☐ Sirolimus
- ☐ Ruxolitinib
- ☐ Baricitinib
- ☐ Other JAK-inhibitor, specify: _____
- ☐ Leniolisib
- ☐ Abatacept
- ☐ Anakinra
- ☐ Canakinumab
- ☐ Etoposide
- ☐ Interferon gamma
- ☐ Etanercept
- ☐ Infliximab
- ☐ Vedolizumab
- ☐ Dupilumab
- ☐ Emapalumab
- ☐ PEG-ADA
- ☐ Other drug; specify: _____

Appendix 1
Disease Status

Extended dataset

Patient status post GT
Inborn errors only

Patient height: _____ **cm** ☐ Not evaluated ☐ Unknown

Patient weight: _____ **kg** ☐ Not evaluated ☐ Unknown

Patient is attending:

☐ Regular school/work
☐ Alternative school/adapted work
☐ Patient is not able to attend work/school
☐ Unknown

(Only for Inborn errors of Immunity)

Immune profiling done during this follow-up period: ☐ No ☐ Yes ☐ Unknown

Test date: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown

Cell type and test results		**Units** *(for CD4 and CD8, select unit)*
CD3 T-cells: _____	☐ Not evaluated ☐ Unknown	Cells/µl
CD4 T-cells: _____	☐ Not evaluated ☐ Unknown	Cells/µl
CD8 T-cells: _____	☐ Not evaluated ☐ Unknown	Cells/µl
B-cells (i.e. CD19): _____	☐ Not evaluated ☐ Unknown	Cells/µl
NK-cells (CD16/CD56): _____	☐ Not evaluated ☐ Unknown	Cells/µl
Naive CD4 T-cells (CD4/CD45RA): _____	☐ Not evaluated ☐ Unknown	☐ % of CD4 ☐ Cells/µl
Naive CD8 T-cells (CD8/CD45RA): _____	☐ Not evaluated ☐ Unknown	☐ % of CD8 ☐ Cells/µl
IgG: _____	☐ Not evaluated ☐ Unknown	Gram/l
IgA: _____	☐ Not evaluated ☐ Unknown	Gram/l
IgM: _____	☐ Not evaluated ☐ Unkown	Gram/l

Extended dataset

Patient status post GT
Inborn errors of Immunity only

Indicate in the table below if the comorbidities de novo, resolved, improved, stabilised or worsened since the treatment .

Inflammatory bowel disease	Crohn's disease or ulcerative colitis	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated
Rheumatologic	SLE, RA, polymyositis, mixed CTD or polymyalgia rheumatica	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated
Renal: moderate/severe	Serum creatinine > 2 mg/dL or >177 µmol/L, on dialysis, or prior renal transplantation	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated
Hepatic: mild	Chronic hepatitis, bilirubin between Upper Limit Normal (ULN) and 1.5 x ULN, or AST/ALT between ULN and 2.5 × ULN	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated
Hepatic: moderate/severe	Liver cirrhosis, bilirubin greater than 1.5 × ULN, or AST/ALT greater than 2.5 × ULN	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated
Chronic lung disease	Bronchiectasis, interstitial pneumonitis, GLILD, oxygen dependency, structural lung disease (e.g. pneumatoceles)	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated
Pre-GT malignancy	Leukaemia, lymphoma, myelodysplastic syndrome (MDS)	☐ No ☐ Yes: ☐ In remission ☐ Stable disease ☐ Relapsed ☐ Not evaluated ☐ Not evaluated
Failure to thrive	Weight <3rd percentile or requirement for (par)enteral feeding	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated
Active infection at GT	Any infection requiring therapy in the immediate pre GT period	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ Not evaluated
Lymphoproliferation	I.e. splenomegaly, organ specific lymphoproliferation	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ De novo ☐ Not evaluated

Appendix 1
Disease Status

Extended dataset

Patient status post GT
Inborn errors of Immunity only

Indicate in the table below if the comorbidities de novo, resolved, improved, stabilised or worsened since the treatment .

Pre-GT organ impairment	Infectious or non-infectious (including neurologic)	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ Not evaluated
Autoimmunity/ autoinflammation	Pre GT (includes patients in remission but on immunomodulatory treatment within 3 months before GT)	☐ No ☐ Yes: ☐ Resolved ☐ Improved ☐ Stabilised ☐ Worsened ☐ Not evaluated

Was the patient admitted to ICU during this follow-up period? ☐ No ☐ Yes ☐ Unknown

Appendix 1
Best Response and Disease Status (Disease Specific)

Haemoglobinopathies

Complete only for Thalassemia Disease Status

Patient requires regular transfusions during follow-up period:

☐ No;
Occasional transfusions during follow-up period: ☐ No
☐ Yes; **Number of units:** ____ ☐ Unknown
Reason: _____ ☐ Unknown
☐ Unknown

☐ Yes; ☐ Return to transfusion dependence after gene therapy or transfusion free period; **Date of first transfusion:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown
(after gene therapy or transfusion free period)

☐ Ongoing transfusion dependence since previous assessment

Number of units: ____ ☐ Unknown
(during follow-up period)

Did transfusions stop? ☐ No
☐ Yes; **Date of last transfusion:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown
☐ Unknown

☐ Unknown

Sickle cell disease:

Complete only for Sickle cell disease Best Response

☐ No return of sickling episodes
☐ Return of sickling episodes; **Date of first episode:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown *(after gene therapy)*
☐ Not evaluated
☐ Unknown

Complete only for Sickle cell disease Disease Status

Sickling episodes occur during follow-up period:

☐ No
☐ Yes; ☐ First return of sickling episodes after gene therapy **Date of first episode :** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown (after gene therapy) ☐ Ongoing presence of sickling episodes **Number of SCD episodes**: ____ ☐ Unknown (during follow-up)
☐ Unknown

Appendix 1
Best Response and Disease Status (Disease Specific)
continued

Other diagnosis

☐ No evidence of disease
☐ Improved
☐ No response
☐ Worse
☐ Not evaluated
☐ Unknown

Appendix 2
-- Pathogens as per EBMT Registry database --

**As defined by the IDSA (Mermel LA, Allon M, Bouza E, Craven DE, Flynn P, O'Grady NP, et al. Clinical practice guidelines for the diagnosis and management of intravascular catheter-related infection: 2009 Update by the Infectious Diseases Society of America. Clin Infect Dis. 2009;49(1):1-45)*

Bacterial infections

Gram-positive:
- Clostridioides difficile
- Enterococcus faecalis (vancomycin-susceptible)
- Enterococcus faecalis (vancomycin-resistant)
- Enterococcus faecium (vancomycin-susceptible)
- Enterococcus faecium (vancomycin-resistant)
- Listeria monocytogenes
- Nocardia spp (specify)
- Staphylococcus aureus MRSA (methicillin-resistant)
- Staphylococcus aureus MSSA (methicillin-susceptible)
- Staphylococcus aureus VISA (vancomycin-intermediate, MIC 4-8 µg/ml)
- Staphylococcus aureus VRSA (vancomycin-resistant, MIC ≥ 16 µg/ml)
- Staphylococcus coagulase-negative spp (at least two positive blood cultures)
- Streptococcus pneumoniae
- Streptococcus viridans
- Streptococcus other spp (specify)
- Gram-positive bacteria other spp (specify)

Gram-negative:
- Acinetobacter baumannii
- Campylobacter jejuni
- Citrobacter freundii
- Enterobacter cloacae
- Enterobacter other spp (specify)
- Escherichia coli
- Haemophilus influenzae
- Helicobacter pylori
- Klebsiella aerogenes (carbapenem-susceptible)
- Klebsiella pneumoniae (carbapenem-susceptible)
- Klebsiella (any species) (carbapenem-resistant) (specify)
- Legionella pneumophila
- Morganella morganii
- Neisseria gonorrhoeae
- Neisseria meningitidis
- Proteus vulgaris
- Providencia spp
- Pseudomonas aeruginosa (carbapenem-susceptible)
- Pseudomonas aeruginosa (carbapenem-resistant)
- Salmonella spp (specify)
- Serratia marcescens
- Shigella spp
- Stenotrophomonas maltophilia
- Treponema pallidum
- Gram-negative bacteria other spp (specify)

Other bacteria:
- Chlamydia spp
- Chlamydophila
- Mycobacterium other spp (specify)
- Mycobacterium tuberculosis
- Mycoplasma pneumoniae
- Rickettsia spp
- Bacteria other (specify)

Viral infections:

- Adenovirus
- Gastrointestinal viruses:
  - o Norovirus
  - o Rotavirus
- Hepatotropic viruses:
  - o HAV
  - o HBV
  - o HCV
  - o HEV
- Herpes group:
  - o CMV
  - o EBV
  - o HHV6
  - o HHV7
  - o HHV8
  - o HS
  - o VZ
- HIV
- Human papilloma viruses (HPV)
- Parvovirus
- Polyomaviruses:
  - o BK
  - o JC
  - o Merkel cell
  - o Other polyomavirus (specify)
- Respiratory viruses:
  - o Enterovirus
  - o Human coronavirus
  - o Influenza A
  - o Influenza B
  - o Metapneumovirus
  - o Parainfluenza
  - o Rhinovirus
  - o RSV
  - o SARS-CoV-2
  - o Respiratory virus other (specify)
- Viruses other (specify)

Appendix 2
-- Pathogens as per EBMT Registry database -- continued

**As defined by the IDSA* *(Mermel LA, Allon M, Bouza E, Craven DE, Flynn P, O'Grady NP, et al. Clinical practice guidelines for the diagnosis and management of intravascular catheter-related infection: 2009 Update by the Infectious Diseases Society of America. Clin Infect Dis. 2009;49(1):1-45)*

Fungal infections:

Yeasts:
· Candida albicans
· Candida auris
· Candida other (specify)
· Cryptococcus neoformans
· Trichosporon (specify)
· Pneumocytis jiroveci
· Yeasts other (specify)

Moulds:
· Aspergillus flavus
· Aspergillus fumigatus
· Aspergillus other spp (specify)
· Aspergillus terreus
· Fusarium other spp (specify)
· Fusarium solani
· Lomentospora prolificans (formerly Scedosporium prolificans)
· Order Mucorales (specify)
· Dematiaceous fungi (Phaeohyphomycosis) (specify)
· Scedosporium spp (specify)
· Moulds other spp (specify)
· Mould infection diagnosed based on positive galactomannan only, without microbiological confirmation
· Blastomyces spp
· Histoplasma spp (specify)
· Coccidioides spp
· Paracoccidioides spp

Parasitic infections:

Protozoa:
· Babesia spp (specify)
· Cryptosporidium
· Giardia spp
· Leishmania spp (specify)
· Plasmodium spp (specify)
· Toxoplasma gondii
· Trypanosoma cruzi
· Protozoa other spp (specify)

Helminths:
· Strongyloides stercoralis
· Other helminths

Appendix 3
-- CTCAE term --

CTCAE terms related to infections and infestations (version 5.0.)
https://ctep.cancer.gov/protocoldevelopment/electronic_applications/ctc.htm#ctc_50

Respiratory tract infections
· Pneumonia
· Other respiratory tract infections, please specify:

· Upper respiratory tract infection
·Tracheobronchitis
.Pleural infection

Intra-abdominal infections
· Esophagus or gastric infection
· Liver site infection (including biliary tract and gallbladder), please specify:

· Biliary tract or gallbladder infection
· Liver infection

· Lower gastrointestinal infection, please specify:

· Anorectal infection
· Appendicitis infective
· Duodenal infection
· Enterocolitis infective
· Small intestine infection
.Typhlitis infective

· Other intra-abdominal infection, please specify:

.Pancreas infection
.Peritoneal infection
.Splenic infection

Skin, soft tissue and muscle infections
. Lymph gland infection
. Skin, soft tissue or muscle infection, please specify:

· Breast infection
· Muscle infection
· Papulo/pustular rash
· Periorbital infection
. Skin infection (other than periorbital)
. Soft tissue infection (other than periorbital)

Blood infections
· Bacteremia
· Fungemia
· Viremia (including DNAemia)
. DNAemia for parasitic infection

Other infections
. Device-related infection (other than intravascular catheter)

Uro-genital tract infections
· Genital infection, please specify:

. Deep genital infection(including cervicitis infective, ovarian/ pelvic/ prostate/ uterine infection)
. Superficial genital infection(including penile/ scrotal / vaginal / vulvai infection)

· Urinary tract infection, please specify:

· Cystitis or urethritis infective
. Upper urinary tract infection (e.g. kidney infection)

Nervous system infection
· Central nervous system infection, please specify:

· Encephalitis infective (including abscess)
. Isolated meningitis infective

· Other nervous system infection, please specify:

· Cranial nerve infection
. Myelitis infective

Cardiovascular infections
. Endocarditis infective
. Other cardiovascular infection, please specify:

· Arteritis infective
. Mediastinal infection

Head and neck infections (excluding lymph gland)
· Conjunctivitis infective
· Corneal infection
. Ear infection
· Endophthalmitis infective
· Oral cavity infection, please specify:

· Salivary gland infection
. Other oral cavity structure infection

· Retinitis infective
· Sinusitis infective

Osteoarticular infections
· Joint infection
· Bone infection

Appendix 4

-- Non-infectious Complications CTCAE term -- No Reporting Required

Non-infectious complications

- Allergic reaction
- All laboratory abnormalities
- All types of pain
- Alopecia
- Blurred vision
- Diarrhoea (enteropathy)
- Dry mouth
- Dyspepsia
- Dysphagia
- Edema
- Esophageal stenosis
- Fatigue
- Flashes
- Gastritis
- Hematologic toxicities
- Hematoma
- Hypertension
- Injection site reaction
- Malaise
- Mucositis
- Sore throat
- Tinnitus
- Vertigo
- Weight loss

Infectious complications

- Minor ophthalmologic bacterial infections
- External otitis treated topically
- Otitis media treated with oral antibiotics
- Isolated lip herpes simplex
- Bacterial tonsillitis or pharyngitis treated orally
- Laryngitis without viral identification managed at home by inhalations or without any intervention
- URTI without viral/bacterial identification managed at home
- Bilateral cervical lymph node enlargement concurrent with URTI that resolved without specific treatment, together with the resolution of URTI
- Local superficial wound infection resolved under topical antibiotics (incl. impetigo)
- Minor skin bacterial infections
- Minor fungal skin infection
- Diaper rash treated with local antifungals
- Candidal balanitis treated topically
- Vaginal candidiasis treated topically or with a single oral dose
- Asymptomatic bacteriuria due to a pathogen not multi-resistant
- Single low urinary tract infection treated orally without need for hospitalisation
- Phlebitis following peripheral intravascular infusion that resolved after intravascular removal without treatment with antibiotics
- Any isolate that is considered part of the normal flora of the place (oral cavity, vagina, skin, stools) except if it carries an antimicrobial resistance that has clinical implications (induce isolation precautions or a pathogen-directed therapy)
- Positive culture without clinical implications

Appendix 5

-- Intravascular catheter-related infections --

CVC infections:

- Catheter colonization
- Phlebitis
- Exit site infection
- Tunnel infection
- Pocket infection
- Bloodstream infection