

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

☐ HCT-related

☐ GT-related

☐ IST-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

☐ 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 $\geq 0.5 \times 10^9/L$):

- ☐ 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 $\geq 20 \times 10^9/L$):

- ☐ 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: ____ <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown	Gene editing efficiency: ____% <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown
B cells	VCN: ____ <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown	Gene editing efficiency: ____% <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown
NK cells	VCN: ____ <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown	Gene editing efficiency: ____% <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown
PMN	VCN: ____ <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown	Gene editing efficiency: ____% <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown
Monocytes	VCN: ____ <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown	Gene editing efficiency: ____% <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown
Other; specify: _____	VCN: ____ <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown	Gene editing efficiency: ____% <input type="checkbox"/> Not evaluated <input type="checkbox"/> 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	<input type="checkbox"/> Not evaluated	<input type="checkbox"/> Unknown
Ferritin	_____ ng/mL	<input type="checkbox"/> Not evaluated	<input type="checkbox"/> Unknown

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)
☐ Unknown

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.

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)
☐ Unknown

Bacterial infection: ☐ No ☐ Yes ☐ Unknown

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 ☐ Unknown

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 ☐ Unknown1) New or ongoing: ☐ Newly developed ☐ Ongoing since previous assessmentStart 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***: _____☐ UnknownResolved: ☐ No ☐ Yes ☐ Unknown

(if patient died)

Contributory cause of death: ☐ No ☐ Yes ☐ Unknown2) New or ongoing: ☐ Newly developed ☐ Ongoing since previous assessmentStart 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***: _____☐ UnknownResolved: ☐ 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 ☐ Unknown

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: ☐ Unknown

(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

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:**☐ No☐ Yes☐ Unknown**Concomitant PBMCs
preserved:**☐ No☐ Yes☐ 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:** ☐ Very High
(*Shannon diversity index*)☐ 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☐ 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

☐ 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:

<input type="checkbox"/> Karnofsky	<input type="checkbox"/> 10	<input type="checkbox"/> 20	<input type="checkbox"/> 30	<input type="checkbox"/> 40	<input type="checkbox"/> 50	<input type="checkbox"/> 60	<input type="checkbox"/> 70	<input type="checkbox"/> 80	<input type="checkbox"/> 90	<input type="checkbox"/> 100
<input type="checkbox"/> Lansky										
<input type="checkbox"/> ECOG	<input type="checkbox"/> 0	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4					



EBMT Centre Identification Code (CIC): ____
Hospital Unique Patient Number (UPN): ____
Patient Number in EBMT Registry: ____

Treatment Type ☐ GT
Treatment Date ____/____/____ (YYYY/MM/DD)

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

☐ 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

☐ 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

Best Response and Disease Status (Disease Specific)

Haemoglobinopathies

Complete only for Thalassemia Disease Status

Patient requires regular transfusions during follow-up period:

<input type="checkbox"/> No;	Occasional transfusions during follow-up period: <input type="checkbox"/> No <input type="checkbox"/> Yes; Number of units: ____ <input type="checkbox"/> Unknown Reason: _____ <input type="checkbox"/> Unknown <input type="checkbox"/> Unknown
<input type="checkbox"/> Yes; <input type="checkbox"/> Return to transfusion dependence after gene therapy or transfusion free period;	Date of first transfusion: ____/____/____ (YYYY/MM/DD) <input type="checkbox"/> Unknown (after gene therapy or transfusion free period)
<input type="checkbox"/> Ongoing transfusion dependence since previous assessment	
Number of units: ____ <input type="checkbox"/> Unknown (during follow-up period)	
Did transfusions stop? <input type="checkbox"/> No <input type="checkbox"/> Yes; Date of last transfusion: ____/____/____ (YYYY/MM/DD) <input type="checkbox"/> Unknown <input type="checkbox"/> Unknown	
<input type="checkbox"/> Unknown	

Sickle cell disease:

Complete only for Sickle cell disease Best Response

<input type="checkbox"/> No return of sickling episodes
<input type="checkbox"/> Return of sickling episodes; Date of first episode: ____/____/____ (YYYY/MM/DD) <input type="checkbox"/> Unknown (after gene therapy)
<input type="checkbox"/> Not evaluated
<input type="checkbox"/> Unknown

Complete only for Sickle cell disease Disease Status

Sickling episodes occur during follow-up period:

<input type="checkbox"/> No	
<input type="checkbox"/> Yes; <input type="checkbox"/> First return of sickling episodes after gene therapy	Date of first episode : ____/____/____ (YYYY/MM/DD) <input type="checkbox"/> Unknown (after gene therapy)
<input type="checkbox"/> Ongoing presence of sickling episodes	
Number of SCD episodes: ____ <input type="checkbox"/> Unknown (during follow-up)	
<input type="checkbox"/> Unknown	



EBMT Centre Identification Code (CIC): ____

Hospital Unique Patient Number (UPN): _____

Patient Number in EBMT Registry: _____

Treatment Type ☐ GT

Treatment Date ____/____/____ (YYYY/MM/DD)

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

Other diagnosis

<input type="checkbox"/> No evidence of disease
<input type="checkbox"/> Improved
<input type="checkbox"/> No response
<input type="checkbox"/> Worse
<input type="checkbox"/> Not evaluated
<input type="checkbox"/> 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)
- Pneumocystis 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

Intra-abdominal infections

- Esophagus or gastric infection
- Liver site infection (including biliary tract and gallbladder)
- Lower gastrointestinal infection
- Other intra-abdominal infection

Skin, soft tissue and muscle infections

- Lymph gland infection
- Skin, soft tissue or muscle infection

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
- Urinary tract infection

Nervous system infection

- Central nervous system infection
- Other nervous system infection

Cardiovascular infections

- Endocarditis infective
- Other cardiovascular infection

Head and neck infections (excluding lymph gland)

- Conjunctivitis infective
- Corneal infection
- Ear infection
- Endophthalmitis infective
- Oral cavity 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
- Tunnel infection
- Phlebitis
- Pocket infection
- Exit site infection
- Bloodstream infection