



EBMT Centre Identification Code (CIC): \_\_\_\_

Hospital Unique Patient Number (UPN): \_\_\_\_\_

Patient Number in EBMT Registry: \_\_\_\_\_

Treatment Type  GT

Treatment Date \_\_\_\_/\_\_\_\_/\_\_\_\_ (YYYY/MM/DD)

## 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)

<input type="checkbox"/> Relapse or progression/persistent disease	
<input type="checkbox"/> Secondary malignancy	
<input type="checkbox"/> CT-related  <input type="checkbox"/> HCT-related  <input type="checkbox"/> GT-related  <input type="checkbox"/> IST-related	<p><b>Select treatment related cause:</b> <i>(select all that apply)</i></p> <input type="checkbox"/> Graft versus Host Disease <input type="checkbox"/> Non-infectious complication <input type="checkbox"/> Infectious complication: <i>(select all that apply)</i> <input type="checkbox"/> Bacterial infection <input type="checkbox"/> Viral infection <input type="checkbox"/> Fungal infection <input type="checkbox"/> Parasitic infection <input type="checkbox"/> Infection with unknown pathogen
<input type="checkbox"/> Other; specify: _____	
<input type="checkbox"/> 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*)



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**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
- Unknown
- Not evaluated

**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
- Unknown
- Not evaluated

**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	<b>VCN:</b> ____ <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated	<b>Gene editing efficiency:</b> ____% <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated
B cells	<b>VCN:</b> ____ <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated	<b>Gene editing efficiency:</b> ____% <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated
NK cells	<b>VCN:</b> ____ <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated	<b>Gene editing efficiency:</b> ____% <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated
PMN	<b>VCN:</b> ____ <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated	<b>Gene editing efficiency:</b> ____% <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated
Monocytes	<b>VCN:</b> ____ <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated	<b>Gene editing efficiency:</b> ____% <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated
Other; specify: _____	<b>VCN:</b> ____ <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated	<b>Gene editing efficiency:</b> ____% <input type="checkbox"/> Unknown <input type="checkbox"/> Not evaluated

 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:** \_\_\_\_\_



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Treatment Type  GT  
Treatment Date \_\_\_\_/\_\_\_\_/\_\_\_\_ (YYYY/MM/DD)

### CURRENT HAEMATOLOGICAL FINDINGS

Haemoglobin \_\_\_\_\_ g/dL  Not evaluated  Unknown

Ferritin \_\_\_\_\_ ng/mL  Not evaluated  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)

#### 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



EBMT Centre Identification Code (CIC): \_\_\_\_  
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 Patient Number in EBMT Registry: \_\_\_\_\_

Treatment Type  GT  
 Treatment Date \_\_\_\_/\_\_\_\_/\_\_\_\_ (YYYY/MM/DD)

**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

Isolation precautions or surveillance

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

Isolation precautions or surveillance

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  
 Isolation precautions or surveillance  
 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  
 Isolation precautions or surveillance  
 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
- Isolation precautions or surveillance
- 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
- Isolation precautions or surveillance
- 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  
 Isolation precautions or surveillance  
 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  
 Isolation precautions or surveillance  
 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  
 Isolation precautions or surveillance  
 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  
 Isolation precautions or surveillance  
 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*

<b>Did insertional mutagenesis occur?</b>	
<input type="checkbox"/> No	
<input type="checkbox"/> Yes:	
<b>Integration site; specify</b> _____	<input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown
<b>Integration site clonal diversity:</b> <i>(Shannon diversity index)</i>	<input type="checkbox"/> Very High <input type="checkbox"/> High <input type="checkbox"/> Moderate <input type="checkbox"/> Low <input type="checkbox"/> Very Low <input type="checkbox"/> Not evaluated <input type="checkbox"/> Unknown
<input type="checkbox"/> Not evaluated	
<input type="checkbox"/> 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:

<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*  
*Not applicable for Inborn Errors*

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





EBMT Centre Identification Code (CIC): \_\_\_\_

Treatment Type  GT

Hospital Unique Patient Number (UPN): \_\_\_\_\_

Patient Number in EBMT Registry: \_\_\_\_\_

Treatment Date \_\_\_\_/\_\_\_\_/\_\_\_\_ (YYYY/MM/DD)

**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;	<b>Occasional transfusions during follow-up period:</b> <input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Number of units:</b> ____ <input type="checkbox"/> Unknown <b>Reason:</b> _____ <input type="checkbox"/> Unknown <input type="checkbox"/> 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**

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

**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 <b>Date of first episode :</b> ____/____/____ (YYYY/MM/DD) <input type="checkbox"/> Unknown (after gene therapy)
<input type="checkbox"/> Ongoing presence of sickling episodes
<b>Number of SCD episodes:</b> ____ <input type="checkbox"/> Unknown (during follow-up)
<input type="checkbox"/> Unknown



EBMT Centre Identification Code (CIC): \_\_\_

Treatment Type  GT

Hospital Unique Patient Number (UPN): \_\_\_\_\_

Patient Number in EBMT Registry: \_\_\_\_\_

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"/> Unknown
<input type="checkbox"/> Not evaluated

## 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 other spp (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
- Chlamydia
- 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](https://ctep.cancer.gov/protocoldevelopment/electronic_applications/ctc.htm#ctc_50)

**Respiratory tract**

- Bronchial infection
- Lung infection
- Laryngitis infective
- Pleural infection
- Tracheitis infective
- Upper respiratory infection

**Uro-genital tract infections**

- Cystitis infective
- Cervicitis infective
- Kidney infection
- Ovarian infection
- Scrotal infection
- Penile infection
- Prostate infection
- Urethral infection
- Urinary tract infection
- Uterine infection
- Vaginal infection
- Vulval infection

**Skin, soft tissue and mucosal surfaces**

- Breast infection
- Folliculitis infective
- Lymph gland infection
- Nail infection
- Mucosal infection
- Papulo/pustular rash
- Paronychia
- Skin infection
- Soft tissue infection
- Wound infection

**Intra-abdominal infections**

- Anorectal infection
- Appendicitis infective
- Appendicitis with perforation infective
- Biliary tract infection
- Cecal infection
- Duodenal infection
- Enterocolitis infective
- Esophageal infection
- Gallbladder infection
- Gastritis infective
- Hepatic infection
- Pancreas infection
- Pelvic infection
- Peritoneal infection
- Splenic infection
- Stoma site infection
- Small intestine infection
- Typhlitis infective

**Muscles and bones**

- Bone infection
- Myositis infective
- Joint infection

**Head and neck**

- Conjunctivitis infective
- Corneal infection
- Endophthalmitis infective
- Retinitis
- Gum infection
- Lip infection
- Oral cavity infection
- Otitis externa infective
- Otitis media infective
- Periorbital infection
- Salivary gland infection
- Sinusitis infective
- Tooth infection

**Nervous system infection**

- Cranial nerve infection
- Encephalitis infective
- Encephalomyelitis infective
- Meningitis infective
- Myelitis infective
- Peripheral nerve infection

**Cardiovascular infections**

- Arteritis infective
- Endocarditis infective
- Mediastinal infection
- Phlebitis infective

**Others**

- Device related infection (other than Intravascular catheter)
- Febrile Neutropenia
- Fever of unknown origin (FUO)
- Sepsis

### 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