EBMT Centre Identification Code (CIC): _ _ _
Hospital Unique Patient Number (UPN): _ _ _ _ _ _ _ _ _ _ _
Patient Number in EBMT database: _ _ _ _ _ _ _ _ _

Treatment Type ☐ GT
Treatment Date _ _ _ _/ _ _/ _ _ *(YYYY/MM/DD)*
*Embargo end date _ _ _ _/ _ _/ _ _ (YYYY/MM/DD)*

# AUTOLOGOUS HEMATOPOIETIC GENE THERAPY (GT)

**Indication diagnosis for this gene therapy:**_____
*(make sure the indication diagnosis has been registered first, using the relevant diagnosis form)*

## PRE-INFUSION

### BASIC INFORMATION ON THE PLANNED GENE THERAPY

**Setting:** *(check only one)*

☐ As per market authorisation / Standard of care / Institutional guidelines

☐ Accelerated access

☐ Investigational drug product (IDP) / Clinical trial

Phase: ☐ 1 ☐ 1/2 ☐ 2 ☐ 2/3 ☐ 3

Randomised trial: ☐ No ☐ Yes

Trial number: (select all that apply)
☐ EudraCT; **Number**: _____
☐ USA NCT; **Number**: _____
☐ UMIN CT; **Number**: _____

### PLANNED GENE THERAPY INFUSION PRODUCT(S)

**Is the planned gene therapy infusion product a commercial product?** ☐ No ☐ Yes

**Identification:**

**Name of manufacturer:**
☐ Aruvant Sciences
☐ Appelis Pharmaceuticals
☐ AvroBio
☐ Beam Therapeutics
☐ Bluebird Bio
☐ CRISPR Therapeutics
☐ Editas Medicine
☐ Graphite Bio
☐ Mustang Bio
☐ Orchard Therapeutics
☐ Rocket Pharmaceuticals
☐ Vertex
☐ Other manufacturer; specify:_____

**Product name:**
☐ Libmeldy (Atidarsagene autotemcel)
☐ Zynteglo (Betibeglogene autotemcel)
☐ Skysona (Elivaldogene autotemcel)
☐ Casgevy (Exagamglogene autotemcel)
☐ Strimvelis (*autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence)*
☐ Other product; specify: _____

## PLANNED GENE THERAPY INFUSION PRODUCT(S) continued

**Will the planned gene therapy infusion product consist of more than one infusion unit?**

☐ No
☐ Yes; **Number of infusion units:** _____
☐ Unknown

**Tissue source** *(check all that apply)***:**

☐ Bone marrow
☐ Peripheral blood
☐ Umbilical cord blood
☐ Other; specify: _____

**Cell type:**

☐ CD34+ hematopoietic stem cells
☐ T cells (other than CAR-T cells)
☐ Other; specify: _____

## MOBILISATION

**Mobilisation drugs given?**

☐ No

☐ Yes; complete for every apheresis:

**Start date of mobilisation**: _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)* ☐ Unknown

**G-CSF:**

**Filgrastim:** ☐ No ☐ Yes; **Total dose***:_____ ☐ mg/kg ☐ mg/m² ☐ Unknown ☐ Unknown

**Lenograstim:** ☐ No ☐ Yes; **Total dose***:_____ ☐ mg/kg ☐ mg/m² ☐ Unknown ☐ Unknown

**Pegfilgrastim:** ☐ No ☐ Yes; **Total dose***:_____ ☐ mg/kg ☐ mg/m² ☐ Unknown ☐ Unknown

**Plerixafor:** ☐ No ☐ Yes; **Total dose***:_____ ☐ mg/kg ☐ mg/m² ☐ Unknown ☐ Unknown

**Other:** ☐ No ☐ Yes; **specify****: _____ **Total dose***:_____ ☐ mg/kg ☐ mg/m² ☐ Unknown

**CD34+ cell count at apheresis :** _____ 1/mL
(in peripheral blood)

*Report the total prescribed cumulative dose as per protocol. Multiply daily dose by the number of days

**Please consult the **LIST OF CHEMOTHERAPY DRUGS/AGENTS AND REGIMENS** on the EBMT website for drugs/regimens names

*Copy and paste the section above as often as necessary for every apheresis*

**Complications after mobilisation?**

☐ No

☐ Yes: **Select all that occured:** ☐ Sickling episode ☐ Vaso-occlusive crisis ☐ Other; specify: _____

## COLLECTED CELLS

**First date of successful collection**: _ _ _ _ / _ _ /_ _ *(YYYY/MM/DD)* ☐ Unknown

**Total number of collection cycles:**_____ ☐ Unknown

**Is the exact number of collected cells available?**

☐ No

☐ Yes; **Number of cells collected:** _____ *(not adjusted for cell viability)*
**Unit**: *(check only one):* ☐ $10^6$/kg ☐ $10^6$ ☐ $10^8$ /kg ☐ $10^8$

**Cell viability:** _____**%** ☐ Unknown

**Was a back-up product collected?**

☐ No

☐ Yes; **Was the back-up product cryopreserved?** ☐ No ☐ Yes ☐ Unknown

☐ Unknown

## PREVIOUS THERAPIES
(before gene therapy)

**Did the patient receive a previous HCT?**

☐ No

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

**Type:** ☐ Autologous HCT

☐ Allogeneic HCT

**For same indication as the gene therapy?** ☐ No

☐ Yes

**END OF PRE-INFUSION SECTION**

*PLEASE PROCEED WITH THE MAIN TREATMENT SECTION TO COMPLETE*

*THE GENE THERAPY TREATMENT REPORTING*

# GENE THERAPY
# Main Treatment

**Date of (planned) gene therapy infusion:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)*

**Centre where infusion took place (CIC):** ____
*(if the product was not infused, report the center where the infusion would have taken place)*
**Patient UPN for this treatment:** _____

**Team or unit where treatment took place *(select all that apply):***

☐ Adults ☐ Pediatrics ☐ Haematology ☐ Oncology ☐ Allograft ☐ Autograft ☐ Other; specify: _____

**Unit number:** _____ ☐ Not applicable

**Was the gene therapy product infused during this treatment/procedure?**

☐ No; **Reason(s) why the treatment did not take place**: *(select all that apply)*
- ☐ Production failure
- ☐ Out of specification product rejected by physician
- ☐ Disease progression or patient condition worsening
- ☐ Patient became ineligible for treatment
- ☐ Patient died
- ☐ Other reason; specify: _____

☐ Yes

## GENE THERAPY INFUSION UNIT(S)

**Was more than one gene therapy infusion unit administered during this treatment?**

☐ No

☐ Yes; **Number of different gene therapy infusion units that were part of this treatment**: _____

**Unique ID of the product:** _____

**Batch number:** _____

**Identification of the gene therapy infusion unit given by the centre:** _____
*(If there is only one gene therapy infusion unit enter "1")*

**Was the infused gene therapy product consistent with the specifications?**

☐ No: **Specify the difference from specifications**: _____
☐ Yes
☐ Unknown

**Was this product cryopreserved prior to infusion?**

☐ No
☐ Yes
☐ Unknown

*If more than one gene therapy infusion unit please copy and fill-in this section for each one of them.*

## GENE THERAPY INFUSION PRODUCT(S)
### Manipulation

*Complete only for non-commercial products. If more than one gene therapy infusion unit please copy and fill-in this section for each one of them.*

**Identification of the gene therapy infusion unit** (given by the centre)**:** _____
*(If there is only one gene therapy infusion unit enter "1")*

**Manipulation:**

**Processing/Manufacturing facility:**

☐ Onsite, by local cell processing facility

☐ Offsite, by a commercial or non-commercial facility

**Gene manipulation type:**

**Gene transfer:** ☐ No

☐ Yes: **Vector:** ☐ Adenoviral vector

☐ Adeno-associated virus (AAV)

☐ Lentiviral vector

☐ Retroviral vector

☐ Transposon

☐ Other vector; specify: _____

**Vector copy number (VCN):** ___

**Transgene:** ☐ ABCD1

☐ Beta globin

☐ Gamma globin

☐ shRNA/siRNA to BCL11A

☐ Suicide gene; specify: _____

☐ Other; specify: _____

**Gene editing:** ☐ No

☐ Yes: **Manipulation technique:** ☐ CRISPR-Cas9

☐ Transcription activator-like effector nucleases (TALEN)

☐ Zinc finger nucleases (ZFN)

☐ Other; specify:_____

**Manipulated gene:** ☐ BCL11A

☐ Beta globin

☐ CCR5

☐ Gamma globin

☐ Other gene; specify: _____

**% of the gene-edited cells:**___

**Other:** ☐ No

☐ Yes; specify: _____

## PREPARATIVE REGIMEN

**Myeloablative conditioning regimen given?**

☐ No

☐ Yes;

| | |
|---|---|
| **Busulfan:** | ☐ No<br>☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m² ☐ Unknown<br>**Route of administration:** (select all that apply) ☐ Oral ☐ IV<br>**Drug monitoring performed:** ☐ No ☐ Yes; **Total AUC:** _____ ☐ mg x hr/L ☐ micromol x min/L ☐ mg x min/mL<br>☐ Unknown |
| **Cyclophosphamide:** | ☐ No<br>☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m² ☐ Unknown<br>☐ Unknown |
| **Fludarabine:** | ☐ No<br>☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m² ☐ Unknown<br>☐ Unknown |
| **Melphalan:** | ☐ No<br>☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m² ☐ Unknown<br>☐ Unknown |
| **Thiotepa:** | ☐ No<br>☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m² ☐ Unknown<br>☐ Unknown |
| **Treosulfan:** | ☐ No<br>☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m² ☐ Unknown |
| **Other:** | ☐ No<br>☐ Yes; **specify**:** _____<br>**Total dose***:____ ☐ mg/kg ☐ mg/m² ☐ Unknown |

*Report the total prescribed cumulative dose as per protocol. Multiply daily dose by the number of days

**Please consult the **LIST OF CHEMOTHERAPY DRUGS/AGENTS AND REGIMENS** on the EBMT website for drugs/regimens names

## GENE THERAPY INFUSION(S)
Description

*If more than one gene therapy infusion please copy and fill-in this section for each one of them.*

**Date of gene therapy infusion:** _ _ _ _ / _ _ / _ _ *(YYYY/MM/DD)*

**Did the patient receive concomitant therapy?**

☐ No

☐ Yes; **specify**: _____

**Treatment given:** ☐ Simultaneously to the gene therapy
☐ After the gene therapy was finished

**If more than one unit was used, indicate the identification of the gene therapy infusion given by the centre as described in the 'Gene Therapy Infusion Unit' section** *(This item is mandatory if more than one infusion unit was used.)*: _____

**Is the exact number of cells infused available?**

☐ No

☐ Yes: **Number of cells**: _____ *(not adjusted for cell viability)*
**Unit:** ☐ $10^6$/kg ☐ $10^6$ ☐ $10^8$/kg ☐ $10^8$ *(check only one)*:

**Cell type:** ☐ CD34+ ☐ T-cells (other than CAR-T cells) ☐ Other; specify: _____

**Cell viability:** _____ **%** ☐ Unknown

**Was the back-up product infused?**

☐ No

☐ Yes; **Reasons for using the back-up product:** *(select all that apply)*

☐ Compromise of the gene therapy product after initiation of conditioning and before infusion
☐ Primary engraftment failure
☐ Loss of engraftment after infusion
☐ Other; specify:_____

***END OF THE GENE THERAPY DAY 0 REPORT***

***proceed to form DISEASE STATUS AT HCT/CT/GT/IST***