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)*

☐ Early access

☐ 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 / Marketing authorisation holder:

☐ Aruvant Sciences
☐ Appelis Pharmaceuticals
☐ AvroBio
☐ Beam Therapeutics
☐ Bluebird Bio
☐ CRISPR Therapeutics
☐ Editas Medicine
☐ Graphite Bio
☐ Mustang Bio
☐ Orchard Therapeutics
☐ Rocket Pharmaceuticals
☐ Genenta Science
☐ 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)*
☐ Temferon
☐ 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/m^2 ☐ Unknown ☐ Unknown

Lenograstim: ☐ No ☐ Yes; **Total dose***:_____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown ☐ Unknown

Pegfilgrastim: ☐ No ☐ Yes; **Total dose***:_____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown ☐ Unknown

Plerixafor: ☐ No ☐ Yes; **Total dose***:_____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown ☐ Unknown

Other: ☐ No ☐ Yes; **specify****: _____ **Total dose***:_____ ☐ mg/kg ☐ mg/m^2 ☐ 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: ☐ 10^6/kg ☐ 10^6 ☐ 10^8 /kg ☐ 10^8 *(check only one):*

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 ☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown **Route of administration:** ☐ Oral ☐ IV (select all that apply) **Drug monitoring performed:** ☐ No ☐ Yes; **Total AUC:** _____ ☐ mg x hr/L ☐ micromol x min/L ☐ mg x min/mL ☐ Unknown
Cyclophosphamide:	☐ No ☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown ☐ Unknown
Fludarabine:	☐ No ☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown ☐ Unknown
Melphalan:	☐ No ☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown ☐ Unknown
Thiotepa:	☐ No ☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown ☐ Unknown
Treosulfan:	☐ No ☐ Yes; **Total dose***:____ ☐ mg/kg ☐ mg/m^2 ☐ Unknown
Other:	☐ No ☐ Yes; **specify**:** _____ **Total dose***:____ ☐ mg/kg ☐ mg/m^2 ☐ 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