



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 trialPhase:  1  1/2  2  2/3  3Randomised trial:  No  YesTrial 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:****Product name:** Aruvant Sciences Appelis Pharmaceuticals AvroBio Beam Therapeutics Bluebird Bio CRISPR Therapeutics Editas Medicine Graphite Bio Mustang Bio Orchard Therapeutics Rocket Pharmaceuticals Genenta Science Telethon foundation Vertex Other manufacturer; specify: \_\_\_\_\_ 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: \_\_\_\_\_



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## 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  mg/kg  
 Yes; **Total dose\*:** \_\_\_\_\_  mg/m<sup>2</sup>  Unknown  
 Unknown

**Lenograstim:**  No  mg/kg  
 Yes; **Total dose\*:** \_\_\_\_\_  mg/m<sup>2</sup>  Unknown  
 Unknown

**Pegfilgrastim:**  No  mg/kg  
 Yes; **Total dose\*:** \_\_\_\_\_  mg/m<sup>2</sup>  Unknown  
 Unknown

**Plerixafor:**  No  mg/kg  
 Yes; **Total dose\*:** \_\_\_\_\_  mg/m<sup>2</sup>  Unknown  
 Unknown

**Other:**  No  
 Yes; **specify\*\*:** \_\_\_\_\_  
 mg/kg  
**Total dose\*:** \_\_\_\_\_  mg/m<sup>2</sup>  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 occurred:**  Sickling episode  Vaso-occlusive crisis  Other; specify: \_\_\_\_\_



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### 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?

<input type="checkbox"/> No					
<input type="checkbox"/> Yes;	Number of cells collected: _____ <i>(not adjusted for cell viability)</i>	Unit: <input type="checkbox"/> 10 <sup>6</sup> /kg	<input type="checkbox"/> 10 <sup>6</sup>	<input type="checkbox"/> 10 <sup>8</sup> /kg	<input type="checkbox"/> 10 <sup>8</sup>
		<i>(check only one):</i>			
Cell viability (%): _____		<input type="checkbox"/> Unknown			

Was a back-up product collected?

<input type="checkbox"/> No				
<input type="checkbox"/> Yes;	Was the back-up product cryopreserved?	<input type="checkbox"/> No	<input type="checkbox"/> Yes	<input type="checkbox"/> Unknown
<input type="checkbox"/> 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



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## 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?

<input type="checkbox"/> No; Reason(s) why the treatment did not take place: (select all that apply)	<input type="checkbox"/> Production failure
	<input type="checkbox"/> Out of specification product rejected by physician
	<input type="checkbox"/> Disease progression or patient condition worsening
	<input type="checkbox"/> Patient became ineligible for treatment
	<input type="checkbox"/> Patient died
	<input type="checkbox"/> Other reason; specify: _____
<input type="checkbox"/> 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.*



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



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### PREPARATIVE REGIMEN

**Myeloablative conditioning regimen given?**

- No  
 Yes;

<b>Busulfan:</b>	<input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Total dose*:</b> ____ <input type="checkbox"/> mg/kg <input type="checkbox"/> mg/m <sup>2</sup> <input type="checkbox"/> Unknown  <b>Route of administration:</b> <input type="checkbox"/> Oral <input type="checkbox"/> IV <input type="checkbox"/> mg x hr/L (select all that apply) <input type="checkbox"/> micromol x min/L <b>Drug monitoring performed:</b> <input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Total AUC:</b> ____ <input type="checkbox"/> mg x min/mL  <input type="checkbox"/> Unknown
<b>Cyclophosphamide:</b>	<input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Total dose*:</b> ____ <input type="checkbox"/> mg/kg <input type="checkbox"/> mg/m <sup>2</sup> <input type="checkbox"/> Unknown <input type="checkbox"/> Unknown
<b>Fludarabine:</b>	<input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Total dose*:</b> ____ <input type="checkbox"/> mg/kg <input type="checkbox"/> mg/m <sup>2</sup> <input type="checkbox"/> Unknown <input type="checkbox"/> Unknown
<b>Melphalan:</b>	<input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Total dose*:</b> ____ <input type="checkbox"/> mg/kg <input type="checkbox"/> mg/m <sup>2</sup> <input type="checkbox"/> Unknown <input type="checkbox"/> Unknown
<b>Thiotepa:</b>	<input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Total dose*:</b> ____ <input type="checkbox"/> mg/kg <input type="checkbox"/> mg/m <sup>2</sup> <input type="checkbox"/> Unknown <input type="checkbox"/> Unknown
<b>Treosulfan:</b>	<input type="checkbox"/> No <input type="checkbox"/> Yes; <b>Total dose*:</b> ____ <input type="checkbox"/> mg/kg <input type="checkbox"/> mg/m <sup>2</sup> <input type="checkbox"/> Unknown
<b>Other:</b>	<input type="checkbox"/> No <input type="checkbox"/> Yes; <b>specify**:</b> _____ <b>Total dose*:</b> ____ <input type="checkbox"/> mg/kg <input type="checkbox"/> mg/m <sup>2</sup> <input type="checkbox"/> 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



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### 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: \_\_\_\_\_ Unit:  10<sup>6</sup>/kg  10<sup>6</sup>  10<sup>8</sup>/kg  10<sup>8</sup>  
(not adjusted for cell viability) (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**