



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

22 April 2020  
EMA/CAT/257145/2020  
Human Medicines Division

## Committee for Advanced Therapies (CAT)

Minutes the meeting on 18-20 March 2020

Chair: Martina Schüßler-Lenz; Vice-Chair: Ilona Reischl

### Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

### Disclaimers

Some of the information contained in these minutes is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the CAT meeting reports once the procedures are finalised.

Of note, the minutes are a working document primarily designed for CAT members and the work the Committee undertakes.

### Note on access to documents

Some documents mentioned in the minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).



## Table of contents

<b>1.</b>	<b>Introduction</b>	<b>6</b>
1.1.	Welcome and declarations of interest of members, alternates and experts.....	6
1.2.	Adoption of agenda .....	6
1.3.	Adoption of the minutes .....	6
<b>2.</b>	<b>Evaluation of ATMPs</b>	<b>6</b>
2.1.	<b>Opinions</b> .....	<b>6</b>
2.1.1.	Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750.....	6
2.2.	<b>Oral explanations</b> .....	<b>7</b>
2.3.	<b>Day 180 list of outstanding issues</b> .....	<b>7</b>
2.4.	<b>Day 120 list of questions</b> .....	<b>7</b>
2.4.1.	Autologous CD34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector encoding the human arylsulfatase A gene - Orphan - EMEA/H/C/005321 .....	7
2.5.	<b>Day 80 assessment reports</b> .....	<b>7</b>
2.6.	<b>Update on ongoing initial applications</b> .....	<b>7</b>
2.7.	<b>New applications</b> .....	<b>7</b>
2.8.	<b>Withdrawal of initial marketing authorisation application</b> .....	<b>7</b>
2.9.	<b>Re-examination of initial application procedures under Article 9(2) of Regulation No. 726/2004</b> .....	<b>8</b>
2.10.	<b>GMP and GCP inspections requests</b> .....	<b>8</b>
2.11.	<b>Type II variations - variation of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008</b> .....	<b>8</b>
2.11.1.	Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0013/G.....	8
2.11.2.	Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0017/G.....	8
2.11.3.	Strimvelis - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ANA cDNA sequence - Orphan - EMEA/H/C/003854/II/0024.....	9
2.11.4.	Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0018.....	9
2.11.5.	Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0019.....	9
2.12.	<b>Extension applications</b> .....	<b>9</b>
2.13.	<b>Other Post-Authorisation Activities</b> .....	<b>9</b>
2.13.1.	Glybera (Expired) - alipogene tiparvovec - Orphan - EMEA/H/C/002145/SOB/001.9.....	9
2.13.2.	Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/006 .....	10
2.13.3.	Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/006.1 .....	10
2.13.4.	Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/009 .....	10

2.13.5.	Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/010 .....	10
2.13.6.	Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/011 .....	11
2.13.7.	Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/012 .....	11

### **3. Certification of ATMPs 11**

<b>3.1.</b>	<b>Opinion</b> .....	<b>11</b>
<b>3.2.</b>	<b>Day 60 Evaluation Reports</b> .....	<b>11</b>
<b>3.3.</b>	<b>New Applications</b> .....	<b>11</b>

### **4. Scientific Recommendation on Classification of ATMPs 12**

<b>4.1.</b>	<b>New requests – Appointment of CAT Coordinator</b> .....	<b>12</b>
4.1.1.	Gene-activated matrix based on octacalcium phosphate and a plasmid carrying VEGF-A gene – H0005629 .....	12
4.1.2.	Leuco platelet enriched plasma – H0005630 .....	12
4.1.3.	Recombinant adeno-associated viral vector rh74 containing the human beta-sarcoglycan gene – H0005631.....	12
4.1.4.	Wharton’s jelly derived mesenchymal stem cell , drug resistant epilepsy .....	12
4.1.5.	Autologous adipose-derived mesenchymal stem cell , diabetic foot syndrome .....	12
4.1.6.	Wharton’s jelly derived mesenchymal stem cell , Behcet disease .....	12
4.1.7.	Wharton’s jelly derived mesenchymal stem cell , choroideremia .....	13
4.1.8.	Wharton’s jelly derived mesenchymal stem cell , foetal alcohol syndrome.....	13
4.1.9.	Wharton’s jelly derived mesenchymal stem cell , frontotemporal dementia .....	13
4.1.10.	Wharton’s jelly derived mesenchymal stem cell , progressive bulbar palsy.....	13
4.1.11.	Wharton’s jelly derived mesenchymal stem cell , vitelliform macular degeneration.....	13
<b>4.2.</b>	<b>Day 30 ATMP scientific recommendation</b> .....	<b>14</b>
4.2.1.	Recombinant chimeric vesicular stomatitis virus carrying the envelope glycoprotein (GP) of the visceral non-neurotropic strain of the lymphocytic choriomeningitis virus – H0005624 .....	14
4.2.2.	Autologous CD34+ cells transduced with CL20-4i-EF1 $\alpha$ -hyc-OPT lentiviral vector – H0005602 .....	14
4.2.3.	Wharton’s jelly derived mesenchymal stem cells, AMN – H0005623 .....	14
<b>4.3.</b>	<b>Day 60 revised scientific recommendation (following list of questions)</b> .....	<b>14</b>
<b>4.4.</b>	<b>Finalisation of procedure</b> .....	<b>14</b>
4.4.1.	Autologous, <i>ex vivo</i> expanded, clonal neoantigen specific tumour infiltrating lymphocytes – H0005575 .....	14
4.4.2.	Autologous adipose derived mesenchymal stem cells, ALS – H0005580.....	15
4.4.3.	Wharton jelly derived mesenchymal stem cells – H0005608.....	15
4.4.4.	Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting CD19 antigen – H0005581 .....	15

4.4.5.	Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting B-cell maturation antigen (BCMA) – H0005582.....	15
4.4.6.	Micronized autologous adipose tissue particles and costal cartilage powder – H0005607...	15
4.4.7.	Human embryonic stem cell-derived otic neural progenitor cells – H0005583.....	16
4.4.8.	Wharton’s jelly derived mesenchymal stem cells, ALS – H0005619 .....	16
4.4.9.	Wharton’s jelly derived mesenchymal stem cell , Huntington’s disease - H0005571 .....	16
4.4.10.	Wharton’s jelly derived mesenchymal stem cell , Lewy body dementia (LBD) - H0005572	16
4.4.11.	Wharton’s jelly derived mesenchymal stem cell , secondary progressive multiple sclerosis (SPMS) - H0005573 .....	16
4.4.12.	Autologous adipose-derived mesenchymal stem cells ex-vivo expanded, alopecia - H0005567 .....	16
4.4.13.	Autologous adipose-derived mesenchymal stem cells ex-vivo expanded, hypertrophic scars - H0005568 .....	17
<b>4.5.</b>	<b>Follow-up and guidance.....</b>	<b>17</b>

## **5. Scientific Advice 17**

<b>5.1.</b>	<b>New requests – appointment of CAT Rapporteurs .....</b>	<b>17</b>
<b>5.2.</b>	<b>CAT reports.....</b>	<b>17</b>
<b>5.3.</b>	<b>List of Issues .....</b>	<b>17</b>
<b>5.4.</b>	<b>Finalisation of SA procedures .....</b>	<b>17</b>

## **6. Pre-Authorisation Activities 17**

<b>6.1.</b>	<b>Paediatric investigation plans.....</b>	<b>17</b>
<b>6.2.</b>	<b>ITF briefing meetings in the field of ATMPs .....</b>	<b>17</b>
<b>6.3.</b>	<b>Priority Medicines (PRIME) – Eligibility requests.....</b>	<b>18</b>
6.3.1.	Month 0 - Start of the procedure .....	18
6.3.2.	Month 1 – Discussion of eligibility .....	18
6.3.3.	Month 2 – Recommendation of eligibility.....	18
6.3.4.	Ongoing support.....	18

## **7. Organisational, regulatory and methodological matters 18**

<b>7.1.</b>	<b>Mandate and organisation of the CAT .....</b>	<b>18</b>
7.1.1.	CAT membership .....	18
7.1.2.	Strategic Review & Learning meeting (SRLM) – Budapest, Hungary, 08 – 10 June 2020 ...	18
7.1.3.	Strategic Review & Learning meeting (SRLM) – Helsinki, Finland, 21 – 22 November 2019	18
7.1.4.	CAT Rules of Procedures – amendments.....	18
<b>7.2.</b>	<b>Coordination with EMA Scientific Committees.....</b>	<b>19</b>
7.2.1.	Committee for Medicinal Products for Human Use (CHMP) .....	19
7.2.2.	CAT-COMP Working Group .....	19
<b>7.3.</b>	<b>Coordination with EMA Working Parties/Working Groups/Drafting Groups .....</b>	<b>19</b>
7.3.1.	Inspectors Working Group (IWG).....	19

7.3.2.	Quality Review of documents (QRD) - agreement on ATMPs.....	19
7.3.3.	Scientific Advice Working Party.....	20
<b>7.4.</b>	<b>Cooperation within the EU regulatory network.....</b>	<b>20</b>
7.4.1.	European Commission’s questionnaire on new genomic techniques.....	20
7.4.2.	Public statement on the use of unregulated/unproven ATMPs .....	20
<b>7.5.</b>	<b>Cooperation with international regulators.....</b>	<b>20</b>
7.5.1.	ATMP cluster teleconference with FDA-USA, Health Canada and PMDA-Japan .....	20
7.5.2.	International Pharmaceutical Regulators Programme (IPRP) – Gene therapy working group.....	20
<b>7.6.</b>	<b>CAT work plan .....</b>	<b>21</b>
<b>7.7.</b>	<b>Planning and reporting .....</b>	<b>21</b>
7.7.1.	Planning estimates of forthcoming ATMP MAAs .....	21
<b>7.8.</b>	<b>Others .....</b>	<b>21</b>
<b>7.9.</b>	<b>Others .....</b>	<b>21</b>
7.9.1.	Draft QWP/BWP guideline on the quality requirements for drug device combinations.....	21
<b>8.</b>	<b>Any other business</b>	<b>21</b>
<b>8.1.</b>	<b>European Conference for Rare Diseases .....</b>	<b>21</b>
<b>9.</b>	<b>Explanatory notes</b>	<b>23</b>
<b>10.</b>	<b>List of participants</b>	<b>27</b>

## 1. Introduction

### 1.1. Welcome and declarations of interest of members, alternates and experts

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and based on the topics in the agenda of the current meeting, the Committee Secretariat announced that no restriction in the involvement of meeting participants in upcoming discussions was identified.

Participants in this meeting were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure and as included in the list of participants. All decisions taken at this meeting were made in the presence of a quorum of members (i.e. 21 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

At the start of the meeting, in the light of the current crisis (Corona pandemic) and as a temporary measure, the participants agreed by consensus to hold the meeting virtual and take decisions remotely.

### 1.2. Adoption of agenda

CAT agenda for 18-20 March 2020 meeting was adopted with 2 additions in section 2.10 and 7.1

### 1.3. Adoption of the minutes

CAT minutes for 19-21 February meeting were adopted with 1 amendment to section 5.4.5

## 2. Evaluation of ATMPs

### 2.1. Opinions

#### 2.1.1. Onasemnogene abeparvovec - Orphan - EMEA/H/C/004750

AveXis EU Ltd; treatment of spinal muscular atrophy (SMA)

Scope: Opinion

**Action:** for adoption

List of Outstanding Issues adopted on 06.12.2019, 21.06.2019. List of Questions adopted on 22.02.2019.

The Rapporteurs presented the outcome of the assessment of the responses to the latest list of outstanding issues. All outstanding points can be resolved as quality commitments, Annex II conditions or Specific Obligations.

The revised product information was discussed and amendments were proposed .

After the discussion, a vote was taken . CAT decided by consensus to recommend granting a conditional marketing authorisation for the following indication:

is indicated for the treatment of:

- patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or
- patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene.

The CAT draft opinion was adopted and will be forwarded to CHMP for adoption.

## 2.2. Oral explanations

No items

## 2.3. Day 180 list of outstanding issues

No items

## 2.4. Day 120 list of questions

- 2.4.1. Autologous CD34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector encoding the human arylsulfatase A gene - Orphan - EMEA/H/C/005321
- 

### **Accelerated assessment**

Orchard Therapeutics (Netherlands) BV; treatment of metachromatic leukodystrophy (MLD)

Scope: Day 120 list of questions

**Action:** for adoption

The CAT Rapporteurs presented their assessment of the marketing authorisation application. The list of questions was discussed. CAT agreed with the BWP report .

The revised list of questions was adopted by CAT.

## 2.5. Day 80 assessment reports

No items

## 2.6. Update on ongoing initial applications

No items

## 2.7. New applications

## 2.8. Withdrawal of initial marketing authorisation application

No items

## 2.9. Re-examination of initial application procedures under Article 9(2) of Regulation No. 726/2004

No items

## 2.10. GMP and GCP inspections requests

## 2.11. Type II variations - variation of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008

### 2.11.1. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0013/G

---

Novartis Europharm Limited

Rapporteur: Rune Kjekken, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: safety. Opinion

Submission of a group of 3 type II variations (C.I.4) to include:

- Long-term efficacy and safety of Kymriah in relapsed/refractory DLBCL based on the 24 months follow-up results from study CCTL019C2201 (update of sections 4.4, 4.8, 5.1 and 5.2 of the SmPC)
- Interim results from study CCTL019B2202 (update of sections 4.4, 4.8, 5.1 and 5.2 of the SmPC)
- Interim results from study CCTL019B2205J (update section 5.2 of the SmPC)

The Marketing authorisation holder (MAH) took the opportunity to clarify the wording of the indication to include patients over 25 years of age and to introduce some minor editorial corrections throughout the SmPC and the Package Leaflet. The MAH also updated sections 4.2, 4.4, 4.8 of the SmPC, Annex II and the Package Leaflet with regards to the acceptability of having 1 dose of tocilizumab per patient per centre for the management of cytokine release syndrome.

In addition, the MAH requested updates to sections 2.2 and 6.5 of the SmPC, the labelling and to the package leaflet to accommodate the administration of additional infusion bags, when applicable.

The requested group of variations proposed amendments to the Summary of Product Characteristics, Annex II, Annex IIIA and Package Leaflet and to the Risk Management Plan (RMP). The RMP version 2.1 has been agreed.

**Action:** for information

Note: additional changes to the SmPC were implemented by CAT and CHMP . The revised Opinion was adopted by written procedure on 4 March 2020

This variation was adopted via a written procedure. The information was noted.

### 2.11.2. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0017/G

---

Novartis Europharm Limited

Rapporteur: Rune Kjekken

Scope: quality. Opinion

**Action:** for adoption

Request for Supplementary Information adopted on 24.01.2020.

The opinion was adopted.



**2.11.3. Strimvelis - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ANA cDNA sequence - Orphan - EMEA/H/C/003854/II/0024**

---

Orchard Therapeutics (Netherlands) BV

Rapporteur: Sol Ruiz, PRAC Rapporteur: Menno van der Elst

Scope: RSI

Safety. Update of sections 4.8 and 5.1 of the SmPC in order to update the safety information following the completion of the STRIM-004 study, which is a non-interventional long term follow up of the subjects who received Strimvelis gene therapy. This study included paediatric patients and is listed as a category 3 study in the RMP. The Package Leaflet is updated accordingly. The RMP version 3.0 has also been submitted. In addition, the Marketing authorisation holder (MAH) took the opportunity to introduce minor administrative changes in the PI.

**Action:** for adoption

The variation was discussed by PRAC, who identified the need for additional information. The RSI was adopted.

**2.11.4. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0018**

---

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality

**Action:** for adoption

The opinion was adopted.

**2.11.5. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0019**

---

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality

**Action:** for adoption

The opinion was adopted.

**2.12. Extension applications**

No items

**2.13. Other Post-Authorisation Activities**

**2.13.1. Glybera (Expired) - alipogene tiparvovec - Orphan - EMEA/H/C/002145/SOB/001.9**

---

uniQure biopharma B.V.

Rapporteur: Egbert Flory, CHMP Coordinator: Jan Mueller-Berghaus

Scope: pharmacovigilance

Long term surveillance programme/ disease registry to collect information on the epidemiology of the disease and the demographics, safety, and the effectiveness outcomes of patients treated with Glybera.

The patients enrolled in clinical studies (CT-AMT-010 -10, CT-AMT 011-01, CT-AMT 011-02) should be followed up in the LPLD registry.

All patients treated with Glybera should be enrolled in the registry and systematic data collection carried out to enrich the database:

- 1) on efficacy data such as biochemical markers as part of normal practice and frequency and severity of pancreatitis and
- 2) on safety including immunogenicity against Glybera and LPL.
- 3) Dietary diary and quality of life data should also be recorded.

The diagnosis of LPLD has to be confirmed by genetic testing.  
[NOTE: 15 years follow-up is recommended for every patient treated.]

**Action:** for adoption

The Rapporteur provide a short feedback. The outcome of the assessment of the post-authorisation measure was agreed.

---

#### 2.13.2. [Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/006](#)

---

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, CHMP Coordinator: Jan Mueller-Berghaus

Scope: quality

**Action:** for adoption

The Rapporteur provided a short feedback. The outcome of the assessment of the post-authorisation measure was agreed.

---

#### 2.13.3. [Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/006.1](#)

---

bluebird bio (Netherlands) B.V

Rapporteur: Carla Herberts, CHMP Coordinator: Paula Boudewina van Hennik

Scope: Quality. MAH's response to REC 006

**Action:** for adoption

The Rapporteur provided a short feedback. The outcome of the assessment of the post-authorisation measure was agreed.

---

#### 2.13.4. [Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/009](#)

---

bluebird bio (Netherlands) B.V

Rapporteur: Carla Herberts, CHMP Coordinator: Paula Boudewina van Hennik

Scope: quality. From Initial MAA

**Action:** for adoption

The Rapporteur provided a short feedback. The outcome of the assessment of the post-authorisation measure was agreed. The post authorisation measure is fulfilled.

---

#### 2.13.5. [Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/010](#)

---

bluebird bio (Netherlands) B.V

Rapporteur: Carla Herberts, CHMP Coordinator: Paula Boudewina van Hennik

Scope: quality

**Action:** for adoption

The Rapporteur provided a short feedback. The outcome of the assessment of the post-authorisation measure was agreed.

2.13.6. [Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/011](#)

---

bluebird bio (Netherlands) B.V

Rapporteur: Carla Herberts, CHMP Coordinator: Paula Boudewina van Hennik

Scope: quality. From Initial MAA

**Action:** for adoption

The Rapporteur provided a short feedback. The outcome of the assessment of the post-authorisation measure was agreed. The post authorisation measure is fulfilled.

2.13.7. [Zynteglo - autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene - Orphan - EMEA/H/C/003691/REC/012](#)

---

bluebird bio (Netherlands) B.V

Rapporteur: Carla Herberts, CHMP Coordinator: Paula Boudewina van Hennik

Scope: quality

**Action:** for adoption

The Rapporteur provided a short feedback. The outcome of the assessment of the post-authorisation measure was agreed.

## 3. Certification of ATMPs

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

### 3.1. Opinion

No items

### 3.2. Day 60 Evaluation Reports

No items

### 3.3. New Applications

No items

## 4. Scientific Recommendation on Classification of ATMPs

### 4.1. New requests – Appointment of CAT Coordinator

#### 4.1.1. Gene-activated matrix based on octacalcium phosphate and a plasmid carrying VEGF-A gene – H0005629

---

Intended to various bone healing indications (sinus lift, non-unions, spinal fusion, etc.)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.2. Leuco platelet enriched plasma – H0005630

---

Intended for the treatment of ulcers, chronic wounds

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.3. Recombinant adeno-associated viral vector rh74 containing the human beta-sarcoglycan gene – H0005631

---

Intended for the treatment of limb-girdle muscular dystrophy type 2E

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.4. Wharton's jelly derived mesenchymal stem cell , drug resistant epilepsy

---

intended for the treatment of drug resistant epilepsy

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.5. Autologous adipose-derived mesenchymal stem cell , diabetic foot syndrome

---

Intended for the treatment of diabetic foot syndrome

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.6. Wharton's jelly derived mesenchymal stem cell , Behcet disease

---

Intended for the treatment of Behcet disease

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.7. Wharton's jelly derived mesenchymal stem cell , choroideremia

Intended for the treatment of choroideremia

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.8. Wharton's jelly derived mesenchymal stem cell , foetal alcohol syndrome

Intended for the treatment of foetal alcohol syndrome

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.9. Wharton's jelly derived mesenchymal stem cell , frontotemporal dementia

Intended for the treatment of frontotemporal dementia

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.10. Wharton's jelly derived mesenchymal stem cell , progressive bulbar palsy

Intended for the treatment of progressive bulbar palsy

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.11. Wharton's jelly derived mesenchymal stem cell , vitelliform macular degeneration

Intended for the treatment of vitelliform macular degeneration (Best disease)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

## 4.2. Day 30 ATMP scientific recommendation

### 4.2.1. Recombinant chimeric vesicular stomatitis virus carrying the envelope glycoprotein (GP) of the visceral non-neurotropic strain of the lymphocytic choriomeningitis virus – H0005624

---

Intended for the treatment of solid tumours, including non-small cell lung carcinoma.

Scope: ATMP scientific recommendation

**Action:** for adoption

CAT discussed the ATMP classification report. Additional information and clarification are needed before concluding on this classification. The list of issues was adopted by CAT and the procedure is stopped awaiting responses from the applicant.

### 4.2.2. Autologous CD34+ cells transduced with CL20-4i-EF1 $\alpha$ -hyc-OPT lentiviral vector – H0005602

---

Intended for the treatment of X-linked severe combined immunodeficiency (XSCID)

Scope: ATMP scientific recommendation

**Action:** for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 3 April 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

### 4.2.3. Wharton's jelly derived mesenchymal stem cells, AMN – H0005623

---

Intended for the treatment of adrenomyeloneuropathy (AMN)

Scope: ATMP scientific recommendation

**Action:** for adoption

CAT discussed the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments by 3 April 2020.

The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

## 4.3. Day 60 revised scientific recommendation (following list of questions)

No items

## 4.4. Finalisation of procedure

### 4.4.1. Autologous, *ex vivo* expanded, clonal neoantigen specific tumour infiltrating lymphocytes – H0005575

---

Intended for the treatment of solid tumours

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.2. [Autologous adipose derived mesenchymal stem cells, ALS – H0005580](#)

---

Intended for the treatment of Amyotrophic Lateral Sclerosis (ALS)

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.3. [Wharton jelly derived mesenchymal stem cells – H0005608](#)

---

Intended for the treatment of spinal cord injury, drug resistant epilepsy and hypoxia ischemia encephalopathy

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.4. [Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting CD19 antigen – H0005581](#)

---

Intended for the treatment of CD19+ haematological malignancies

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.5. [Allogeneic CRISPR/Cas9-mediated genetically modified CAR T cells targeting B-cell maturation antigen \(BCMA\) – H0005582](#)

---

Intended for the treatment of relapsed or refractory multiple myeloma

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.6. [Micronized autologous adipose tissue particles and costal cartilage powder – H0005607](#)

---

Intended for the treatment of cartilage defects

Scope: comments received from the European Commission. Revised ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.7. Human embryonic stem cell-derived otic neural progenitor cells – H0005583

Intended for the treatment of sensorineural hearing loss

Scope: comments received from the European Commission. Revised ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.8. Wharton's jelly derived mesenchymal stem cells, ALS – H0005619

Intended for the treatment of Amyotrophic Lateral Sclerosis (ALS)

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.9. Wharton's jelly derived mesenchymal stem cell , Huntington's disease - H0005571

Intended for the treatment of Huntington's disease

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.10. Wharton's jelly derived mesenchymal stem cell , Lewy body dementia (LBD) - H0005572

Intended for the treatment of Lewy body dementia (LBD)Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.11. Wharton's jelly derived mesenchymal stem cell , secondary progressive multiple sclerosis (SPMS) - H0005573

Intended for the treatment of secondary progressive multiple sclerosis (SPMS)

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.12. Autologous adipose-derived mesenchymal stem cells ex-vivo expanded, alopecia - H0005567

Intended for the treatment of alopecia

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

LoQs adopted on 24 January 2020

The information was noted.



#### 4.4.13. Autologous adipose-derived mesenchymal stem cells *ex-vivo* expanded, hypertrophic scars - H0005568

---

Intended for the treatment of hypertrophic scars

Scope: the European Commission has raised no comments. ATMP scientific recommendation

**Action:** for information

LoQs adopted on 24 January 2020

The information was noted.

#### 4.5. Follow-up and guidance

No items

### 5. Scientific Advice

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

#### 5.1. New requests – appointment of CAT Rapporteurs

#### 5.2. CAT reports

#### 5.3. List of Issues

No items

#### 5.4. Finalisation of SA procedures

### 6. Pre-Authorisation Activities

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

#### 6.1. Paediatric investigation plans

No items

#### 6.2. ITF briefing meetings in the field of ATMPs

No items

## 6.3. Priority Medicines (PRIME) – Eligibility requests

- 6.3.1. Month 0 - Start of the procedure
  - 6.3.2. Month 1 – Discussion of eligibility
  - 6.3.3. Month 2 – Recommendation of eligibility
  - 6.3.4. Ongoing support
- 

## 7. Organisational, regulatory and methodological matters

### 7.1. Mandate and organisation of the CAT

#### 7.1.1. CAT membership

---

Romania: Alina Musetescu – Membership mandate ended on 12 March 2020

**Action:** for information

The information was noted.

#### 7.1.2. Strategic Review & Learning meeting (SRLM) – Budapest, Hungary, 08 – 10 June 2020

---

CAT: Katalin Lengyel

Scope: topics for inclusion in the joint CAT-PDCO agenda and CAT-only agenda to take place on 08-10 June 2020

**Action:** for discussion

CAT members were asked to send proposal for agenda topics to CAT secretariat in advance of the April 2020 CAT meeting.

#### 7.1.3. Strategic Review & Learning meeting (SRLM) – Helsinki, Finland, 21 – 22 November 2019

---

CAT: Heli Suila

Scope: minutes of the meeting that took place on 21-22 November 2019

**Action:** for discussion

CAT members (and especially the speakers) were asked to provide comment on the minutes of the SRLM meeting. Comments to be sent to CAT secretariat until 10 April 2020.

#### 7.1.4. CAT Rules of Procedures – amendments

---

Scope: revision of the CAT Rules of Procedure

**Action:** for adoption

The EMA Management Board (MB), at its meeting on 19 March 2020 adopted amendments to the existing Rules of Procedure of EMA's scientific committees and MB. These amendments are required to enable those bodies to continue their workings in a virtual emergency setting, as well as to ensure the validity of the various output decisions that each committee will adopt in the coming weeks. A change is also introduced in the quorum required for adoption of scientific opinions or recommendations in case of an emergency situation. To add flexibility to the system, irrespective of an emergency situation, the possibility is introduced to give a proxy vote to another member or to the alternate of a member who is present at the relevant meeting of the body concerned.

CAT adopted the amendments to the CAT Rule of Procedure, as adopted by the EMA Management Board.

## 7.2. Coordination with EMA Scientific Committees

### 7.2.1. Committee for Medicinal Products for Human Use (CHMP)

---

Scope: Summary of Outcomes (SoO) for the February 2020 meeting

**Action:** for information

The information was noted

### 7.2.2. CAT-COMP Working Group

---

CAT core members: Kieran Breen, Carla Herbert, Maura O'Donovan, Maja Sommerfelt and Martina Schübler-Lenz

Scope: agenda for the monthly COMP-CAT Working Group to take place on 18 March 2020 from 18:30-19:30hrs

**Action:** for discussion

Feedback was provided on the discussions in the CAT-COMP working group.

## 7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

### 7.3.1. Inspectors Working Group (IWG)

---

Scope: feedback on discussion in the IWG on inspection of viral vector manufacturer and on decentralised manufacture

**Action:** for information

This topic was postponed.

### 7.3.2. Quality Review of documents (QRD) - agreement on ATMPs

---

**Action:** for information

Feedback was provided from the discussion in the QRD on the use of scientific notification in the expression of strength and information on the type of cells (SmPC sections 1 and 2).

The proposal from QRD is to use scientific notification in the expression of strength. The Commission representative asked for additional clarifications regarding the proposed expression of strength (i.e. how would the strength of genetically modified cells be measured).

There was some unclarity on the information of the type of cells to be included in section 1 and 2 of the SmPC, and whether the information in the labelling was scientifically sound.

It was proposed to bring the topic back to the next CAT meeting; this will allow the CAT Rapporteurs to check the product information of authorised cell-based ATMPs.

### 7.3.3. Scientific Advice Working Party

---

Scope: SAWP re-nomination composition, including the CAT nominees.

**Action:** for information

Note: adopted by the CHMP at its March 2020 ORGAM meeting

CAT noted the CAT-nominated SAWP members: Rune Kjekken (with alternate: Maura O'Donovan) and Romaldas Maciulaitis (with alternate: Jan Mueller-Berghaus). Hans Ovelgönne and Olli Tenhunen are nominated as SAWP members by CHMP.

## 7.4. Cooperation within the EU regulatory network

### 7.4.1. European Commission's questionnaire on new genomic techniques

---

Scope: stakeholders' consultation on new genomic techniques

**Action:** for information

[https://ec.europa.eu/food/plant/gmo/modern\\_biotech/stakeholder-consultation\\_en](https://ec.europa.eu/food/plant/gmo/modern_biotech/stakeholder-consultation_en)

Note: the European Commission is carrying out targeted consultations with Member States and EU-level stakeholders to gather information for the Commission study on new genomic techniques.

The European Commission Representative presented the questionnaire on new genomic techniques, including aspects related to GMO containing medicines. CAT members were asked to consider the questionnaire and to provide feedback from the medicines side.

### 7.4.2. Public statement on the use of unregulated/unproven ATMPs

---

Scope: feedback on next steps

**Action:** for information

EMA provided feedback. EMA is very supportive to have an updated public statement on their website: amendments are proposed by the EMA Stakeholder and Communications department to provide a clear message to the different target audiences and to ensure consistency with other communications. The revised statement will be discussed with the CAT chair in the next week. The revised public statement will be circulated to CAT members, for adoption via a written procedure.

## 7.5. Cooperation with international regulators

### 7.5.1. ATMP cluster teleconference with FDA-USA, Health Canada and PMDA-Japan

---

Scope: feedback from the teleconference that took place on 27 February 2020

**Action:** for discussion

Feedback was provided from the discussion at the ATMP cluster TC.

### 7.5.2. International Pharmaceutical Regulators Programme (IPRP) – Gene therapy working group

---

CAT: Pille Säälük

Scope: agenda of the teleconference that will take place on 26 March 2020

**Action:** for discussion

The draft agenda of the upcoming IPRP gene therapy working group was presented.

## 7.6. CAT work plan

None

## 7.7. Planning and reporting

### 7.7.1. Planning estimates of forthcoming ATMP MAAs

---

Scope: Q1/2020 update of the business pipeline report for the human scientific committees

**Action:** for information

The information was noted.

## 7.8. Others

## 7.9. Others

### 7.9.1. Draft QWP/BWP guideline on the quality requirements for drug device combinations

---

Rapporteur: Nicholas Lee (IE, QWP)

CAT: Ilona Reischl

Scope: Presentation of the revised draft following public consultation

**Action:** for discussion

The Rapporteur of this guideline provided an update to CAT. Ilona Reischl highlighted the scope of the guideline, which is relevant for ATMPs. The Commission representative raised comments. Any last comments from CAT are awaited by Monday 23 March.

The draft guideline will now be finalised by the drafting group: the aim is to publish the guideline at the same time when the Medical Device Regulation will come into force.

## 8. Any other business

### 8.1. European Conference for Rare Diseases

Scope: identification of CAT speaker for the session: *Innovation in Advanced Therapy* (Saturday 16<sup>th</sup> May 2020, 11:00 – 12:30)

**Action:** for action

Note: the conference will be conducted virtually. For more information on the conference, see: <https://www.rare-diseases.eu/>

A general discussion took place on CAT participation (as speakers/panellist) to European/international conferences. As the number of requests is increasing, it was proposed to define some criteria for participation 1) to ensure CAT representation at key meetings and 2) to avoid that invitations are sent to multiple CAT members (potentially resulting in more than 1 member attending the same meeting). Also a repository of

presentation will be set up to avoid duplication of work for the speakers when preparing their presentations.

It was agreed that CAT members will forward to the CAT secretariat all recent invitations for meetings and conferences. These will be included in a database/excel sheet. Further discussion on this topic will take place at the next CAT meeting.

CAT members should inform CAT Secretariat by Monday 23 March if they want to present in the ATMP sessions at the European Conference for Rare Diseases.

Date of next CAT meeting:

22-24/04/2020

## 9. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

### Abbreviations / Acronyms

AAV: Adeno-Associated Virus

AR: Assessment Report

ATMP: Advanced Therapy Medicinal Product

BWP: Biologics Working Party

CAT: Committee for Advanced Therapies

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

CTFG: Clinical Trial Facilitation Group

DG: Drafting Group

EC: European Commission

EU NTC: European Union Network Training Centre

ERA: Environmental Risk Assessment

FDA: Food and Drug Administration

FL: Final Letter

GCG: Guideline Consistency Group

GCP: Good Clinical Practice

GLP: Good Laboratory Practice

GMO: Genetically-modified organism

GMP: Good Manufacturing Practice

GTMP: Gene Therapy Medicinal Product

HTA: Health Technology Assessment Bodies

HSPC: Hematopoietic Stem and Progenitor Cells

ITF: Innovative Task Force

JR: Joint Report

LoOI: List of outstanding issues

LoQ: List of questions

MA: Marketing Authorisation

MAA: Marketing Authorisation Application

MAH: Marketing Authorisation Holder

MSC: Mesenchymal stem cells

PDCO: Paediatric Committee

PMDA: Pharmaceuticals and Medical Devices Agency (Japan)

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee #

PRIME: Priority Medicines

RMP: Risk Management Plan  
 RP: Reflection paper  
 RSI: Request for supplementary information  
 SAs: Scientific Advices  
 SAG-O: Scientific Advisory Group Oncology  
 SAWP: Scientific Advice Working Party  
 SR: Summary Report  
 SWP: Scientific Working Party  
 SME: Small and medium size enterprises  
 SmPC: Summary of Products Characteristics  
 TT: Timetable

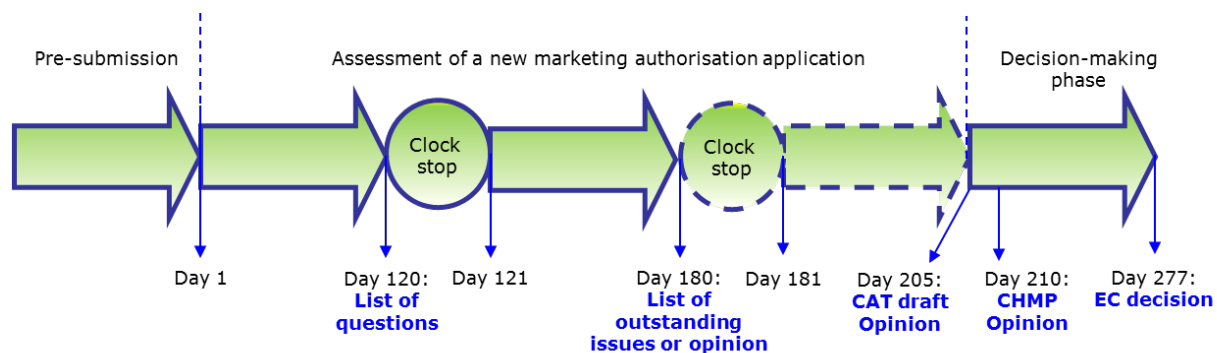
## Evaluation of ATMPs (section 2)

This section lists applications for marketing authorisations of new Advanced Therapy Medicinal Products (ATMPs) that are to be discussed by the Committee. It also lists any ATMP related inspection requests (section 2.9) and Post-authorisation activities (section 2.10).

### *New applications (sections 2.1. to 2.12.)*

Section 2.1 is for ATMPs nearing the end of the evaluation and for which the CAT is expected to adopt a draft **opinion** at this meeting on whether marketing authorisation should be granted. Once adopted, the CAT opinion is transmitted to the CHMP for final adoption. The CHMP opinion will be forwarded to the European Commission for a final legally binding decision valid throughout the EU. More information on the evaluation of ATMPs can be found [here](#).

The other items in the section are listed depending on the stage of the evaluation, which is shown graphically below:



The assessment of an application for a new medicine takes up to 210 'active' days. This active evaluation time is interrupted by at least one 'clock-stop' during which time the applicant prepares the answers to questions from the CAT. The clock stop happens after day 120 and may also happen after day 180, when the CAT has adopted respectively a **Day 120 list of questions** (section 2.3) or a List of outstanding issues to be addressed by the company, which is listed in the agenda under sections 2.7 (**Ongoing evaluation procedures**). Section 2.7 also includes the CAT discussions at any other timepoint of the evaluation procedure of new applications.

### *Oral explanation (section 2.2.)*

Prior to adoption of the CAT opinion, marketing authorisation applicants are normally invited to the CAT plenary meeting to address questions raised by the Committee.

Oral explanations normally relate to ongoing applications, but they can also relate to any other issue for which the CAT would like to discuss with company representatives in person.



### *Re-examination procedures (new applications) under article 9(2) of regulation no 726/2004 (section 2.6.)*

This section lists applications for new marketing authorisation for ATMPs for which the applicant has requested a re-examination of the opinion previously issued by the CHMP. Similar to the initial evaluation of a marketing authorisation of an ATMP, CAT will adopt a draft re-examination opinion, which is transmitted to the CHMP for final adoption.

### *Withdrawal of applications (section 2.7.)*

This section includes information on marketing authorisation applications that are withdrawn by the applicant. Applicants may decide to withdraw applications at any stage during the assessment and a CAT opinion will therefore not be issued. Withdrawals are included in the agenda for information or discussion, as necessary.

### *New applications (section 2.9.)*

In this section, information is included on upcoming marketing authorisation applications for ATMPs, as well as information on appointment of Rapporteurs for new ATMP applications.

### *GMP and GCP Inspections Issues (section 2.10.)*

This section lists inspections that are undertaken for ATMPs. Inspections are carried out by regulatory agencies to ensure that marketing authorisation holders comply with their obligations. Inspection can relate to good manufacturing practice (GMP), good clinical practice (GCP), good laboratory practice (GLP) or good pharmacovigilance practice (GVP).

### *Post-authorisation activities (section 2.12.)*

This section lists type II variations, extension application according to Annex I of Reg. 1234/2008, re-examination procedures for type II variations (including extension of indication applications) for which the applicant has requested re-examination of the opinion previously issued by the CHMP and other issues concerning authorised medicines that are not covered elsewhere in the agenda such as annual reassessments, 5-year renewals, supply shortages, qualify defects. Issues that have been discussed at the previous meeting of the PRAC, the EMA's committee responsible for evaluating and monitoring safety issues for medicines, will also be included here.

## **Certification of ATMPs (section 3)**

This section includes the scientific evaluation by the CAT of quality and non-clinical data that small and medium-sized enterprises have generated at any stage of the ATMP development process. More information on the ATMP certification procedure can be found [here](#).

## **Scientific Recommendation on Classification of ATMPs (Section 4)**

This section includes the scientific recommendation by the CAT on whether medicines based on genes, cells or tissues meet the scientific criteria that define ATMPs. More information on the ATMP classification procedure, including the outcomes of finalised classifications, can be found [here](#).

## **Scientific Advice (section 5)**

This section includes all scientific advice given to companies during the development of an ATMP. Information related to the number of ATMP related scientific advices discussed by CAT can be found in the CAT Monthly reports. Further information on SAWP can be found [here](#).

## **Pre-Authorisation (section 6)**

### *Paediatric Investigation Plan (PIP)*

This section includes the discussion of an ATMP before a formal application for marketing authorisation

is submitted. These cases refer for example to requests for an accelerated assessment for medicines that are of major interest for public health or can be considered a therapeutic innovation: in case of an accelerated assessment the assessment timetable is reduced from 210 to 150 days.

CAT contributes to the evaluation of a Paediatric Investigation Plan (PIPs) for ATMPs by the Paediatric Committee. These PIPs are included in this section of the Agenda.

#### *ITF Briefing meeting in the field of ATMPs*

This section refers to briefing meetings of the Innovation Task Force and International co-operations activities of the CAT

The Innovation Task Force (ITF) is a body set up to encourage early dialogue with applicants developing innovative medicines. Minutes of meetings with applicants developing ATMPs and of other ITF meetings of interest to the CAT are included in this section of the agenda. Further information on the ITF can be found [here](#).

#### *Priority Medicines (PRIME)*

This section includes the new requests for eligibility to PRIME for ATMPs under development, the discussions in CAT of these eligibility requests and the final recommendations for eligibility of ATMPs adopted by CHMP.

CAT will appoint one of its members as the CAT sponsor for each new ATMP eligibility request who will lead the CAT discussion based on the recommendation from the SAWP.

#### **Organisational, regulatory and methodological matters (section 7)**

This section includes topics related to regulatory and procedural guidance, CAT workplan, CAT meeting organisation (including CAT membership), planning and reporting, co-ordination with other committees, working parties and scientific advisory groups.

Furthermore, this section refers to the activities of the CAT drafting groups developing scientific guidelines for gene therapy medicinal products and for cell-based medicinal products, cooperation within the EU regulatory network and international regulators as well as direct interaction with interested parties. It also includes topics of scientific interest for the Committee that are not directly related to the work of the CAT drafting groups or CAT associated working parties.

#### **Any other business (section 8)**

This section is populated with miscellaneous topics not suitable under the previous headings.

More detailed information on the above terms can be found on the EMA website: [www.ema.europa.eu/](http://www.ema.europa.eu/)

## 10. List of participants

including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 18-20 March 2020 virtual meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Martina Schüssler-Lenz	Chair	Germany	No interests declared	
Ilona Reischl	Member	Austria	No interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Belaïd Sekkali	Alternate	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Mirna Golemovic	Member	Croatia	No interests declared	
Petra Sokol	Alternate	Croatia	No interests declared	
Isavella Kyriakidou	Alternate (replacing CAT member)	Cyprus	No interests declared	
Ivana Haunerova	Member	Czech Republic	No interests declared	
Anne Pastoft	Member	Denmark	No interests declared	
Toivo Maimets	Member	Estonia	No interests declared	
Pille Saalik	Alternate	Estonia	No interests declared	
Heli Suila	Member	Finland	No interests declared	
Violaine Closson	Member	France	No interests declared	
Nathalie Morgensztejn	Alternate	France	No interests declared	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Angeliki Rompoti	Alternate (replacing CAT member)	Greece	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Katalin Lengyel	Member	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	
Niamh Curran	Alternate	Ireland	No restrictions applicable to this meeting	
Paolo Gasparini	Member	Italy	No interests declared	
Una Riekstina	Member	Latvia	No interests declared	
Romaldas Mačiulaitis	Member (CHMP member)	Lithuania	No interests declared	
Guy Berchem	Member (to CHMP representative). Replacing CAT member	Luxembourg	No restrictions applicable to this meeting	
Anthony Samuel	Alternate (to CHMP representative). Replacing CAT member.	Malta	No interests declared	
Carla Herberts	Member	Netherlands	No interests declared	
Johannes Hendrikus Ovelgonne	Alternate	Netherlands	No interests declared	
Rune Kjekken	Member	Norway	No restrictions applicable to this meeting	
Maja Sommerfelt Grønvold	Alternate	Norway	No restrictions applicable to this meeting	
Dariusz Śladowski	Member	Poland	No restrictions applicable to this meeting	
Maria Isabel Borba Vieira	Alternate (to CHMP representative). Replacing CAT member.	Portugal	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Gianina-Nicoleta Andrei	Alternate (replacing CAT member)	Romania	No interests declared	
Alexandra Padova	Alternate (replacing CAT member)	Slovakia	No interests declared	
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	
Sol Ruiz	Member (CHMP co-opted member)	Spain	No interests declared	
Marcos Timón	Alternate (to CHMP representative)	Spain	No interests declared	
Lisbeth Barkholt	Member	Sweden	No interests declared	
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	
Alessandro Aiuti	Member	Healthcare Professionals' Representative	Restrictions applicable to this meeting.	
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	
Roland Pochet	Alternate	Patients' Representative	No interests declared	
Barbara Bonamassa	Expert	AIFA-IT	No interests declared	
Hans Hillege	Expert	CBG-MEB-NL	No interests declared	
Tineke va den Hoorn	Expert	CBG-MEB-NL	No interests declared	
Marcel Kwa	Expert	CBG-MEB-NL	No interests declared	
Marcel Hoefnagel	Expert	CBG-MEB-NL	No interests declared	
Emmely de Vries	Expert	CBG-MEB-NL	No interests declared	
Ria Nibbeling	Expert	CBG-MEB-NL	No interests declared	
Marja van de Bovenkamp	Expert	CBG-MEB-NL	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Janneke van Leeuwen	Expert	CBG-MEB-NL	No interests declared	
Jan Span	Expert	CBG-MEB-NL	No interests declared	
Charlotte de Wolf	Expert	CBG-MEB-NL	No interests declared	
Juliane Rau	Expert	PEI-DE	No interests declared	
Beate Mosl	Expert	PEI-DE	No restrictions applicable to this meeting	
Daniela Melchiorri	Expert	AIFA-IT	No restrictions applicable to this meeting	
Svetlana Lorenzano	Expert	AIFA-IT	No restrictions applicable to this meeting	
Antonella Isgro	Expert	AIFA-IT	No interests declared	
Nick Lee	Expert	HPRA-IE	No restrictions applicable to this meeting	
Katrin Féchir	Expert	PEI-DE	No interests declared	
Caoimhin Concannon	Expert	HPRA-IE	No interests declared	
Wiebke Hoppensack	Expert	PEI-DE	No interests declared	

A representative from the European Commission attended the meeting

Meeting run with support from relevant EMA staff

\* Experts were only evaluated against the agenda topics or activities they participated in.