



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

14 July 2021  
EMA/CAT/349953/2021  
Human Medicines Division

## Committee for Advanced Therapies (CAT)

Minutes of the meeting on 16-18 June 2021

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

### Disclaimers

Some of the information contained in these minutes are considered commercially confidential or sensitive and therefore not disclosed. Regarding 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, these 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 ongoing 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).



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## 1. Introduction

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

The Chairperson opened the meeting by welcoming all participants. Due to the current coronavirus (COVID-19) outbreak, and the associated EMA Business Continuity Plan (BCP), the meeting was held remotely.

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. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

### 1.2. Adoption of agenda

The CAT agenda for 16-18 June 2021 meeting was adopted with 2 additions under AOB.

### 1.3. Adoption of the minutes

The CAT minutes for 10-12 May meeting were adopted

## 2. Evaluation of ATMPs

### 2.1. Opinions

#### 2.1.1. Idecabtagene vicleucel - Orphan - EMEA/H/C/004662

Celgene Europe BV; treatment of multiple myeloma

Scope: Opinion

**Action:** for adoption

List of Outstanding Issues adopted on 19.02.2021, 04.12.2020. List of Questions adopted on 11.09.2020.

The CAT Rapporteur presented the assessment of the responses to the list of outstanding issues.

CAT discussed the product information. The agreed indication is: for the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody and have demonstrated disease progression on the last therapy. A presentation was given on the orphan maintenance and the major therapeutic advantage. The CAT Rapporteur presented the outcome of his analysis of comprehensiveness using the comprehensiveness criteria agreed at the Lisbon SRLM (see 7.6.1). CAT agreed that the risk benefit is positive and that a conditional marketing authorisation can be granted  
The CAT draft opinion and CAT assessment report were adopted.

## 2.2. Oral explanations

No items

## 2.3. Day 180 list of outstanding issues

No items

## 2.4. Day 120 list of questions

No items

## 2.5. Day 80 assessment reports

No items

## 2.6. Update on ongoing initial applications

### 2.6.1. Lisocabtagene maraleucel / lisocabtagene maraleucel - Orphan - EMEA/H/C/004731

Bristol-Myers Squibb Pharma EEIG; treatment of large B-cell lymphoma, diffuse large B-cell lymphoma (DLBCL), primary mediastinal large B-cell lymphoma (PMBCL) and follicular lymphoma grade 3B (FL3B)

Scope: MAA's request (dated 28.05.2021) for a clock-stop extension

**Action:** for adoption

List of Questions (LoQ) adopted on 06.11.2020. List of Outstanding Issues (LoOI) adopted on 16.04.2021

CAT agreed with the extension of a clock stop.

## 2.7. New applications

### 2.7.1. Ciltacabtagene autoleucel - Orphan - EMEA/H/C/005095

#### **Accelerated assessment**

Janssen-Cilag International NV; treatment of multiple myeloma

Scope: Timetable for assessment

**Action:** for adoption

## 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

No items

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

### 2.11.1. Zynteglo - betibeglogene autotemcel - Orphan - EMEA/H/C/003691/II/0025

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bluebird bio (Netherlands) B.V

Rapporteur: Carla Herberts

Scope: Quality. Request for supplementary information (RSI)

**Action:** for adoption

The RSI was adopted.

## 2.12. Extension applications

No items

## 2.13. Other Post-Authorisation Activities

### 2.13.1. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/ANX/003.5

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Novartis Europharm Limited

Rapporteur: Rune Kjekken, CHMP Coordinator: Ingrid Wang, PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: PhV

Study CCTL019B2401:

Non-interventional post-authorisation safety study (PASS): In order to further characterise the safety – including long-term safety – of Kymriah, the applicant should conduct and submit a study based on data from a disease registry in ALL and DLBCL patients. Third semi-annual report (EBMT data only).

**Action:** for adoption

The post-authorisation measure (PRAC led) was adopted.

### 2.13.2. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/P46/012

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Novartis Europharm Limited

Rapporteur: Rune Kjekken, CHMP Coordinator: Ingrid Wang



Scope: Clinical

Paediatric studies submitted in accordance with Article 46 of Regulation (EC) No1901/2006, as amended. FINAL STUDY REPORT, Study no. CTL019B2001X, EudraCT no. 2016-001991-31 (Phase IIIb study for relapsed/refractory paediatric/young adult acute lymphoblastic leukaemia patients to be treated with CTL019).

**Action:** for adoption

The post-authorisation measure was adopted.

### 2.13.3. Libmeldy - atidarsagene autotemcel - Orphan - EMEA/H/C/005321/ANX/002

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Orchard Therapeutics (Netherlands) BV

Rapporteur: Carla Herberts, CHMP Coordinator: Johann Lodewijk Hillege. PRAC Rapporteur: Brigitte Keller-Stanislawski

Scope: clinical and PhV. Submission of the long TERM-MLD (metachromatic leukodystrophy) study protocol: In order to further characterise the long-term efficacy and safety of Libmeldy in children with late infantile or early juvenile forms of MLD, the MAH shall conduct and submit the results of a prospective study based on data from a registry, according to an agreed protocol.

**Action:** for adoption

CAT was informed that during the review of the protocol of post-authorisation efficacy study (PAES), the input from the patient representatives was sought. The patient representative also joined the CAT discussion and provided his views. There was a short discussion on the examinations and testing proposed in the protocol (such as MRI, nerve conduction velocity testing), which were considered as the normal clinical practice to follow disease progression. The post-authorisation measure was adopted.

### 2.13.4. Strimvelis - autologous CD34+ enriched cell fraction that contains cd34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence - Orphan - EMEA/H/C/003854/ANX/004.3

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Orchard Therapeutics (Netherlands) BV

Rapporteur: Sol Ruíz, CHMP Coordinator: Maria Concepción Prieto Yerro, PRAC Rapporteur: Menno van der Elst

Scope: Clinical and PhV. Second Interim Patient Registry / STRIM-0003: Adenosine Deaminase Severe Combined Immunodeficiency (ADA-SCID) Registry for Patients Treated with Strimvelis (or GSK2696273) Gene Therapy: Long-Term Prospective, Non-Interventional Follow-up of Safety and Effectiveness [Interim reports submitted every 2 years].

**Action:** for adoption

The post-authorisation measure (PRAC led) was adopted.

### 2.13.5. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/010

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Kite Pharma EU B.V.

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

Scope: From IB/0020:

In frame of on-going process verification the MAH is asked to provide batch data within 6 months of manufacturing start added at TCF04.

**Action:** for adoption

The post-authorisation measure was adopted.

## 2.13.6. Zynteglo - betibeglogene autotemcel - Orphan - EMEA-H-20-1504-C-003691-0023

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bluebird bio (Netherlands) B.V

CAT-PRAC group: CAT: Carla Herberts (Rapporteurs) and Violaine Closson-Carella (Co-Rapporteur), Alessandro Aiuti; PRAC: Brigitte Keller-Stanislawski, Menno van der Elst

Scope: referral procedure under Article 20 PhV. Feedback from PRAC discussion

**Action:** for information

The CAT Rapporteur informed the CAT of the PRAC discussion on the Zynteglo referral. CAT noted the questions asked by PRAC. A short discussion took place on the proposal for increase monitoring.

PRAC will continue the review of the referral, and expect to conclude at their July meeting.

## 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. Autologous population of selected renal cells (SRC)

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Intended for the treatment of chronic kidney disease (CKD)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.2. Autologous adipose mesenchymal stem cells (MSCs)

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Indicated for cartilage defects of degenerative origin and for the treatment of osteoarthritis

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.3. Allogeneic natural killer cells armed with anti-CD20 monoclonal antibody

Intended for the treatment of B-Cell Non-Hodgkin lymphoma

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.4. Recombinant serotype 9 adeno-associated virus encoding a codon-optimised human galactosylceramidase transgene [ssAAV9/CBA-hsaGALCopt2-SV40p (AAV9-hGALC)]

Intended for the treatment of Krabbe disease

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.5. Minimally manipulated autologous pancreatic islets

Intended for the treatment of chronic pancreatitis and recurrent acute pancreatitis immediately following pancreatectomy

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.6. Extracellular matrix and non-viable osteogenic cells derived from human adipose-derived stem cells, associated with hydroxyapatite/beta-tricalcium phosphate (HA/ $\beta$ TCP) particles

Intended to stimulate bone regeneration in pathological hypoxic and/or necrotic bone conditions

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.7. HEK293 cells transfected with a lentiviral vector to express the tumour-specific antigen, WT1 and the antigen presenting molecule, cluster of differentiation 1d (CD1d).

Intended for the treatment of WT1-expressing tumours

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.8. Ribonucleoprotein (RNP), a complex of Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) Cas 9 and sgRNA, delivered by a novel synthetic non-viral vector, for the excision of exon 80 of the human COL7A1 gene

Intended for the treatment of recessive dystrophic epidermolysis bullosa (RDEB)

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.9. Isolated CD31+ cells

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Intended for the treatment of erectile dysfunction

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.10. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

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Intended for the treatment of rheumatoid arthritis, unspecified

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.11. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

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Intended for the treatment of systemic lupus erythematosus, unspecified

Scope: appointment of CAT Coordinator and adoption of timetable

**Action:** for adoption

The CAT coordinator was appointed.

#### 4.1.12. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

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Intended for the treatment of systemic sclerosis, unspecified

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. Nanoparticle consisting of non-pseudotyped (bald) lentiviral vector encoding for a CD19 CAR, encapsulated

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Intended for the treatment of CD19+ B-cell malignancy

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 2 July 2021.

#### 4.2.2. Autologous T cells genetically modified ex vivo using a synthetic chromosome encoding CCR6, IL-2, a truncated version of CD34 and two independent inducible safety switches

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Intended for treatment of patients with solid tumours for which a draining lymph node can be identified. Initially the product will be developed for colon cancer and urinary bladder cancer

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 2 July 2021.

#### 4.2.3. Live human mesenchymal stem cells derived from allogeneic Wharton's jelly

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Intended for treatment of atherosclerosis of the arteries of the lower extremities

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 2 July 2021.

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

No items

### 4.4. Finalisation of procedure

#### 4.4.1. Allogeneic, expanded, engineered E4ORF1+ human umbilical cord endothelial (CD31+) cells

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Intended to treat organ vascular niche injuries caused by myeloablative, non-central nervous system penetrating high-dose chemotherapy (HDT) to prevent the development of severe regimen-related toxicities (SRRT) in patients diagnosed with aggressive systemic lymphoma

Scope: the European Commission raised minor comments. ATMP Scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.2. Allogeneic human Wharton's jelly derived mesenchymal stem cells

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Intended for the treatment of idiopathic pulmonary fibrosis (IPF), pulmonary fibrosis after COVID-19

Scope: the European Commission raised no comments. ATMP Scientific recommendation

**Action:** for information

The information was noted.

#### 4.4.3. *Ex-vivo* expanded autologous cryopreserved Wharton's Jelly derived mesenchymal stem cells

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Intended for the treatment of Bronchopulmonary Dysplasia (BPD) for preterm infants  
Scope: the European Commission raised no comments. ATMP Scientific recommendation  
**Action:** for information  
The information was noted.

#### 4.4.4. Allogeneic corneal endothelial cells in a confluent monolayer adhering to a cornea-shaped sheet of cross-linked collagen

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Intended for the treatment of corneal dysfunction  
Scope: the European Commission raised minor comments. ATMP Scientific recommendation  
**Action:** for information  
The information was noted.

#### 4.4.5. Proliferation arrested myelomonocytic leukemic cell line-derived cells with a mature dendritic cell phenotype

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Intended for the treatment of acute myeloid leukaemia  
Scope: the European Commission raised no comments. ATMP Scientific recommendation  
**Action:** for information  
The information was noted.

## 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 Peer-reviewers

#### 5.1.1. Ongoing scientific advice procedures – Appointment of CAT Peer-Reviewers

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Timetable:

-Start of the procedure at SAWP:	07-10.06.2021
-Appointment of CAT Peer-reviewers:	18.06.2021
-SAWP first reports:	28.06.2021
-CAT Peer-Reviewer's comments:	02.07.2021
-Discussion at SAWP:	05-08.07.2021
-Discussion at CAT and feedback from SAWP:	17.07.2021

#### 5.1.2. Scientific advice procedures starting at the next SAWP meeting

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Timetable:

-Start of procedure at SAWP:	05-08.07.2021
-Appointment of CAT Peer-Reviewers:	16.07.2021
-SAWP first reports:	23.08.2021
-CAT Peer-Reviewer's comments:	27.08.2021
-Discussion at SAWP:	30 Aug.-02 Sept.2021
-Discussions at CAT and feedback from SAWP:	10.09.2021

- 5.2. Procedures discussed at SAWP – 1<sup>st</sup> reports, D40 JRs, LoIs**
- 5.3. Finalisation of D70 procedures – feedback from the discussion meeting**
- 5.4. Final Advice Letters for procedures finalised the previous month**

No items

## **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**
- 6.2. ITF briefing meetings in the field of ATMPs**
- 6.3. Priority Medicines (PRIME) – Eligibility requests**

### **6.3.1. Month 0 - Start of the procedure**

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Timetable for assessment:	
Procedure start:	10.06.2021
SAWP recommendation:	08.07.2021
CAT recommendation:	16.07.2021
CHMP adoption of report and final recommendation:	22.07.2021

### **6.3.2. Month 1 – Discussion of eligibility**

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No items

### **6.3.3. Month 2 – Recommendation of eligibility**

### **6.3.4. Ongoing support**

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No items

## 7. Organisational, regulatory and methodological matters

### 7.1. Mandate and organisation of the CAT

#### 7.1.1. CAT membership

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Finland – Olli Tenhunen – membership mandate (alternate) ended 14.05.2021

**Action:** for information

The information was noted.

#### 7.1.2. Strategic Review & Learning meeting (SRLM) under the Portuguese presidency of the European Union – 27<sup>th</sup> May 2021, Lisbon, Portugal

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CAT: Bruno Sepodes, Maria-Isabel Vieira

Scope: feedback from the meeting that took place on 27<sup>th</sup> May 2021

**Action:** for information

See also 7.6.1

A short feedback was given from the SRLM that was held virtually on 27 May 2021.

#### 7.1.3. Strategic Review & Learning (virtual) meeting (SRLM) under the Slovenian presidency, 20-21 October 2021, Ljubljana (Slovenia)

---

CAT: Metoda Lipnik-Štangelj, Martina Schuessler-Lenz

Scope: initial discussion on agenda content for the joint CHMP/CAT meeting

**Action:** for discussion

The date of the next SRLM that will take place in Ljubljana.

#### 7.1.4. Planning estimates of forthcoming ATMP MAAs

---

Scope: Q2/2021 update of the business pipeline report for the human scientific committees

**Action:** for information

The information was noted

### 7.2. Coordination with EMA Scientific Committees

#### 7.2.1. CHMP learnings that impact CAT decisions

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CAT: Jan Mueller-Berghaus, Romaldas Mačiulaitis, John-Joseph Borg, Bruno Sepodes, Sol Ruíz

Scope: CHMP learnings with relevance to CAT

**Action:** for information

Two CHMP learning were presented: 1) data exclusivity and the use of data from other medicinal products; 2) major therapeutic benefit.



## 7.2.2. CAT-COMP Working Group

---

CAT: Martina Schuessler-Lenz

Scope: feedback from the CAT-COMP working group meeting that took place on 14 June 2021

**Action:** for information

Feedback from CAT-COMP working group was provided during the discussion (see 2.1.1).

## 7.2.3. Scientific Coordination Board (SciCoBo) – meeting of 29<sup>th</sup> June 2021

---

CAT: Martina Schuessler-Lenz

Scope: CAT feedback is sought on innovative developments and technologies in the ATMP field that are to be taken forward in the 'Horizon Scanning' activity

**Action:** for discussion

CAT members were asked to identify innovative ATMPs that would value an in-depth review (Horizon scanning).

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

### 7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

---

Scope: draft agenda of the PCWP/HCPWP joint meeting that took place on 01-02 June 2021

**Action:** for information

The information was noted.

### 7.3.2. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

---

Scope: minutes on the PCWP/HCPWP joint meeting that took place on 3-4 March 2021

**Action:** for information

Note: the CAT Chair - Martina Schuessler-Lenz - took part on the topic of: '*Timely patients' access to advanced therapy medicinal products in the EU*'

The information was noted.

### 7.3.3. Scientific advice (SA) procedure for ATMPs

---

Scope: Update of sub-headings in the CAT agenda and MMD.

**Action:** for information

A short presentation was given to CAT members.

## 7.4. Cooperation within the EU regulatory network

### 7.4.1. Revision of the EU legislation on blood, tissues and cells (BTC)

---

CAT: Martina Schuessler-Lenz

Scope: feedback from the workshop of 9 June 2021 on 'Borderlines with Other Regulated Frameworks: Classification Advice and Interplay'

**Action:** for discussion

Feedback was given from the discussion during the BTC workshop on 9 June 2021. In the presentation on the ATMP classification procedure (during the ATMP-BTC breakout session), the CAT chair stressed the robustness of the system, the clear definitions that determine the borderline (i.e. substantial manipulation, non-homologous use), the international recognition of these criteria, and data demonstrating that the classification procedure is open to / used by all type of applicants<sup>1</sup>.

The CAT members that attended the workshop (either representing CAT or representing their national agencies) expressed disappointment on the level of the discussion: the predefined (closed) questions did not allow an open exchange of views. No clarity was obtained on the impact of the revision of the BTC legislation on the ATMP regulatory framework.

#### 7.4.2. EU-Innovation Network Borderline Classification Group (BLCG)

---

Scope: identification of CAT member to take part in BLCG

**Action:** for discussion

A short presentation was given on the BLCG group (same presentation as given by EMA during the BTC workshop of 9 June 2021, see 7.4.1).

Following CAT members will take part in the activities of this group: Ilona Reischl, Lisbeth Barkholt. Relevant discussions in the BLCG on ATMP related topics will be reported back to CAT.

#### 7.4.3. EU questionnaire on GMO assessment for medicinal products for human use

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**Action:** for information

Note: the questionnaire has been sent out to the national competent authorities

The European Commission representative provided information on the questions that were put to the national competent authorities. She reiterated the importance of receiving feedback on issues / questions that members states have encountered on ERA / GMO aspects, so that this can be adequately taking into account in the Pharma Strategy exercise.

### 7.5. Cooperation with international regulators

#### 7.5.1. ATMP cluster teleconference with US-FDA, Health Canada and PMDA (Japan)

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CAT: Martina Schuessler-Lenz *et al*

Scope: feedback on the teleconference that took place on 20 May 2021

**Action:** for information

Note: following CAT members/experts attend the ATMP cluster: Egbert Flory, Isabel Vieira, Belaïd Sekkali, Violaine Closson-Carella, Maura O'Donovan, Rune Kjekken, Marie Lüttgen, Alessandra Renieri, Roland Pochet, Ivana Haunerova, Maja Sommerfelt, Heli Suila, Carla Herberts, Rocio Salvador Roldan, Brigitte Keller Stanislawski, Brigitte Anliker (expert), Atilla Sebe (expert) and Beate Mosl (expert)

Feedback was provided from the discussions in the May ATMP cluster.

#### 7.5.2. Ad-hoc teleconference between CAT and US-FDA

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CAT: Maura O'Donovan

Scope: teleconference took place 17 May 2021

**Action:** for information

Note: CAT discussed the scientific advice for this product during its January 2021 meeting

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<sup>1</sup> Over 2018-2020, 29 % of request for classification were from tissue establishments, 14% from hospitals/academia.

Feedback was provided from the discussion in this ad-hoc teleconference with FDA.

### 7.5.3. International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) ICH S12

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CAT members: Rune Kjekken, Claire Beuneu - EU Rapporteurs

Scope: feedback on the ICH S12 guideline: non-clinical biodistribution studies for gene therapy products definition of gene therapy medicinal products'

**Action:** for information

**Note:** ICH S12 was endorsed and signed by the ICH Assembly on 3<sup>rd</sup> June 2021. The document will go for adoption to the CHMP at its PROM (formerly known as ORGAM) meeting on 14 June 2021 before being published for a four-month public consultation.

Rune Kjekken (EU Rapporteur) presented the ICH S12 guideline (step 2) that will now go out for public consultation. There was a short discussion on the difference between the gene therapy definition in this guideline and the EU GTMP definition.

CAT discussed how best to inform our stakeholders of the existence of this first ICH guideline for ATMPs. This could be included as an agenda topic in a CAT interested parties meeting (that would take place in the 2<sup>nd</sup> half of this year) and/or presented during the virtual European Society of Gene and Cell Therapy (ESGCT) meeting scheduled on 22 October 2021.

## 7.6. CAT work plan

### 7.6.1. Comprehensiveness of clinical data in marketing authorisations

---

CAT: Martina Schuessler-Lenz

Scope: criteria for comprehensiveness of clinical data in marketing authorisation applications

**Action:** for adoption

**Note:** discussed at the SRLM of 27 May 2021 - see also: 7.1.2

The criteria were adopted by CAT by consensus, with some editorial changes. CAT subsequently discussed how to implement these criteria in the clinical assessment template. It was agreed that the Rapporteurs start using them during the assessment of new marketing authorisation applications for ATMPs. In a couple of months, a review will be done, when sufficient experience with applying these criteria has been obtained from CAT and CHMP.

## 7.7. Planning and reporting

None

## 7.8. Others

### 7.8.1. Research and innovation workstream

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Scope: horizon scanning report on genome editing

**Action:** for information

**Note:** a report on genome editing has been finalised by the EU Innovation network (EU IN; EMA Rapporteur; contributions by experts from the EU Network, Committees and Working parties). It covers the current status and activities, as well as specific challenges and opportunities, leading to recommendations for genome editing medicinal product development and regulation in the next decade.

The information was noted.

### 7.8.2. Patient engagement in MAAs - pilot

---

Scope: the aim is to enable patients to share their experience, concerns and needs related to their condition with the Rapporteurs/committee so that this can be considered in a timely manner during the assessment process, if appropriate.

**Action:** for information

EMA presented the pilot for patient engagement during the review of MAAs. CAT agreed that the MAA for ciltacabtagene autoleucel (see 2.7.1) would be a good candidate for such patient engagement.

### 7.8.3. DIA Global Annual meeting 2021 – Session on Gene Therapy, 18 June 2021

---

CAT: Martina Schuessler-Lenz

Scope: 'Gene Therapy: Getting Back on Track After COVID-19'. Learning objective: discuss the effect of the COVID-19 pandemic on the development of gene therapies; Identify the need for new policy initiatives to help expedite the gene therapies both in the US and globally. Moderator: Janet Lynch Lambert (ARM). Panellists: Peter W. Marks (CBER-FDA), Adora Ndu (Biomarin) and Martina Schuessler-Lenz

**Action:** for information

Note: the session was pre-recorded on 8<sup>th</sup> June 2021.

Link: [Gene Therapy: Getting Back on Track After COVID-19 \(diaglobal.org\)](https://diaglobal.org)

Topic postponed to the next meeting.

### 7.8.4. CASSS: Cell and Gene Therapy Products: Manufacturing, Quality and Regulatory Considerations, 8-10 June 2021

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CAT: Heli Suila

Scope: feedback from the meeting

**Action:** for information

Topic postponed to the next meeting.

### 7.8.5. European Health Forum Gastein (EHFG) – Health Talks: 'Transforming the future of healthcare – do cell and gene therapies hold the key?', 15 June 2021

---

CAT: Ilona Reischl

Scope: feedback from the meeting

**Action:** for information

Topic postponed to the next meeting.

### 7.8.6. 6<sup>th</sup> Industry Stakeholder Platform on R&D support, 4th June 2021

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Scope: feedback from industry observations of the Q&A on similarity for ATMPs

**Action:** for information

A short feedback was provided.

## 8. Any other business

### 8.1. Alliance for Regenerative Medicine (ARM) - chemistry, manufacturing and controls (CMC) virtual workshop for 21<sup>st</sup> September 2021

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CAT: Martina Schuessler-Lenz, Ilona Reischl

Scope: speaking opportunity to present EMA's PRIME toolbox

**Action:** for nomination of a CAT speaker

CAT members interested to give this talk should inform CAT secretariat.

### 8.2. Alliance for Regenerative Medicine (ARM) - chemistry, manufacturing and controls (CMC) virtual workshop for pluripotent stem cell derived therapeutics, 16<sup>th</sup>-17<sup>th</sup> November 2021

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CAT: Martina Schuessler-Lenz, Ilona Reischl

Scope: speaking opportunity on the quality development aspect of pluripotent stem cell-derived medicinal products

**Action:** for nomination of a CAT speaker

CAT members interested to give this talk should inform CAT secretariat.

### 8.3. Polish Supreme Medical Chamber takes action on cell-based therapies

---

CAT: Dariusz Sladowski

Scope: Feedback from a meeting at the Polish Supreme Medical Chamber

**Action:** for information

Dariusz Śladowski informed the Committee about the activity in Poland. On 17<sup>th</sup> of June the kick-off meeting of the expert group on cell therapies took place at the Polish Supreme Chamber in Warsaw. The activity is closely connected with the EMA letter, warning against using unproven cell-based therapies. The group consists of 17 members representing medical doctors with distinctive knowledge in the field of cell therapies, Main Pharmaceutical Inspectorate (supervising production of ATMP in Poland), Office for Registration of Medicinal Products and Cell and Tissue Authorities. Current situation of the use of cell-based products in Poland has been discussed. Issues have been identified and action plans agreed. The group will review actual cellular therapies available in Poland, their potentials and limitations. This knowledge will be used in planned educational courses for medical doctors and patients. The group will also support Competent Authorities responsible for regulation of cell therapies with the special attention to ATMP products used under Hospital Exemption.

### 8.4. Novel Therapies & Therapies Working Party (NTWP)

---

CAT: Dariusz Sladowski

Scope: Feedback from the first meeting of the NTWP

**Action:** for information

A new veterinary working party for novel therapies, including biologics, synthetic peptides, cell, gene and bacteriophage therapies has been established. The first meeting took place on 19<sup>th</sup> of May. During the meeting the work plan 2021/2022 have been drafted. The working party will focus on drafting guidelines, extend ADVENT Q&A, classification of a novel veterinary products. The information was noted by the Committee.

Date of next CAT meeting:

14-16/07/2021

## 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

MNAT: Multinational assessment team

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  
 QRD: Quality review of documents  
 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: Safety Working Party  
 SME: Small and medium size enterprises  
 SmPC: Summary of Products Characteristics  
 TT: Timetable

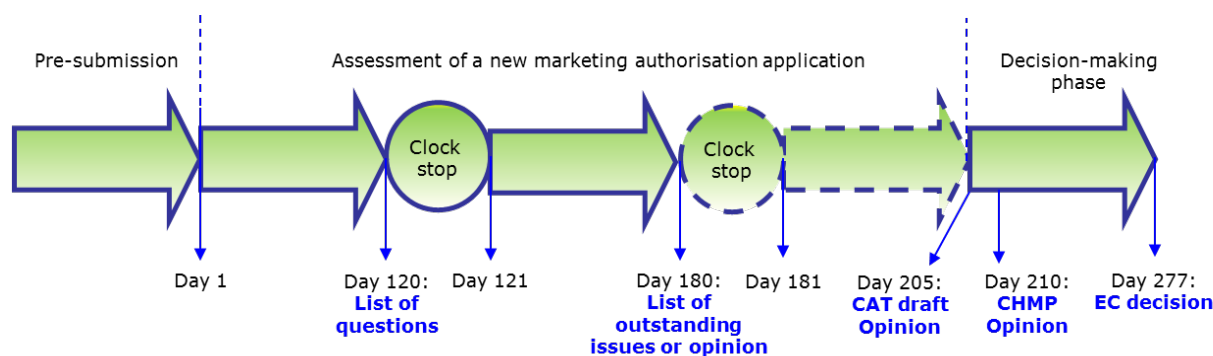
## Evaluation of ATMPs (section 2)

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 16-18 June 2021 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 (Vice-Chair)	Austria	No interests declared	
Silke Dorner	Alternate	Austria	No interests declared	
Claire Beuneu	Member	Belgium	No interests declared	
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	
Petra Sokol	Alternate	Croatia	No interests declared	
Rafaella Pontou	Member	Cyprus	No interests declared	
Isavella Kyriakidou	Alternate	Cyprus	No interests declared	
Ivana Haunerova	Member	Czechia	No interests declared	
Tomas Boran	Alternate	Czechia	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	
Jan Mueller-Berghaus	Member (CHMP co-opted member)	Germany	No interests declared	
Egbert Flory	Alternate (to CHMP representative)	Germany	No interests declared	
Maria Gazouli	Member	Greece	No interests declared	
Angeliki Rompoti	Alternate	Greece	No interests declared	
Katalin Lengyel	Member	Hungary	No interests declared	
Maura O'Donovan	Member	Ireland	No interests declared	
Concetta Quintarelli	Member	Italy	No interests declared	
Barbara Bonamassa	Alternate	Italy	No restrictions applicable to this meeting	
Una Riekstina	Member	Latvia	No interests declared	
Romaldas Mačiulaitis	Member (CHMP member)	Lithuania	No interests declared	
Raimondas Benetis	Alternate (to CHMP representative)	Lithuania	No interests declared	
Nancy De Bremaeker	Member	Luxembourg	No interests declared	
John J. Borg	Member (CHMP member)		No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	
Carla Herberts	Member	Netherlands	No interests declared	
Babs Fabriek	Alternate	Netherlands	No interests declared	
Rune Kjeklen	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)	Portugal	No interests declared	
Silviu Istrate	Member	Romania	No interests declared	
Alexandra Padova	Alternate	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	
Maria Luttgén	Alternate	Sweden	No restrictions applicable to this meeting	
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	
Alessandro Aiuti	Member	Healthcare Professionals' Representative	Restrictions applicable to this meeting	2.13.3 & 2.13.4
Kerstin Sollerbrant	Member	Patients' Representative	No interests declared	
Lydie Meheus	Alternate	Patients' Representative	No interests declared	
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	
Tomask Grybek	Expert - via telephone *	PL - Foundation of Borys the Hero	No interests declared	
Susanne Mueller	Expert - via telephone *	PEI-DE	No interests declared	
Hans Ovelgonne	Expert - via telephone*	NL - CBG/MEB	No interests declared	
A representative from the European Commission attended the meeting				

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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Meeting run with support from relevant EMA staff

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