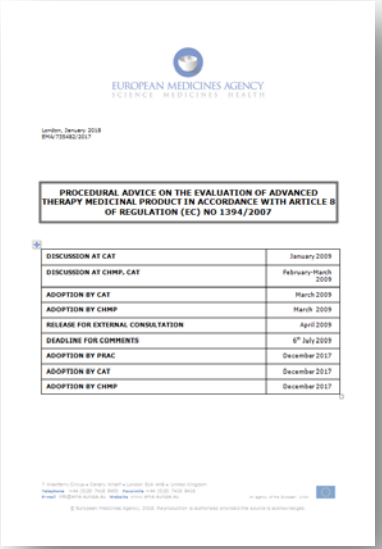
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Adoption of the revised guidance concerning Procedural Advice on the Evaluation of Advanced Therapy Medicinal Products


EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

London, January 2018
EMA/736482/2017

PROCEDURAL ADVICE ON THE EVALUATION OF ADVANCED THERAPY MEDICINAL PRODUCT IN ACCORDANCE WITH ARTICLE 8 OF REGULATION (EC) NO 1394/2007

DISCUSSION AT CAT	January 2009
DISCUSSION AT CHMP, CAT	February-March 2009
ADOPTION BY CAT	March 2009
ADOPTION BY CHMP	March 2009
RELEASE FOR EXTERNAL CONSULTATION	April 2009
DEADLINE FOR COMMENTS	6th July 2009
ADOPTION BY PRAC	December 2017
ADOPTION BY CAT	December 2017
ADOPTION BY CHMP	December 2017

Aim of this presentation

The presentation provides an overview of the points raised in response to the following questions:

1

What is the **Procedural Advice on the Evaluation of ATMPs**?

2

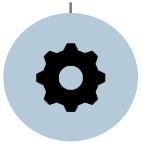
What is **new** in the updated version of the Procedural guidance for ATMPs?

What is the Procedural Advice on the Evaluation of ATMPs?

The procedural advice for ATMPs is a **guidance document**, prepared by EMA and its scientific committees responsible for the evaluation of ATMPs (CAT, PRAC and CHMP).

First drafted in **March 2009.** Required by EU pharmaceutical legislation (Article 8.5 of Regulation (EC) No 1394/2007).

It describes the **procedure for evaluation of ATMPs** for initial marketing authorisation. The same principles apply also for changes during the medicine's lifecycle.



It details the **interactions, the roles and responsibilities** of the committees involved in the evaluation of ATMPs.

Current update: takes into account existing experience evaluating ATMPs, reflects the role of the PRAC, and streamlines processes for evaluation.

What is new in the updated guidance?

1

Streamlines some procedural aspects:

1.a Same processes for adopting questions during the evaluation (List of Question process = List of Outstanding Issues process)

1.b Simplification of the process for oral explanation at committees (oral explanation in front of CHMP only in exceptional circumstances)

2

Strengthens collaboration between EMA's scientific committees

3

Reflects current practice of providing ATMP developers with the possibility of **longer periods ('clock-stops')** to respond to questions raised during the evaluation

4

Describes the role of the Committee for Pharmacovigilance and Risk Management (PRAC) in the **assessment of the ATMPs**

How is EMA organised?

Management Board

Executive Director

EMA staff

CHMP | **CVMP** | **COMP** | **HMPC** | **PDCO** | **CAT** | **PRAC**

7 Scientific Committees
+ 28 working parties
+ 8 scientific advisory groups

National competent authorities
~4000 European experts

EU institutions

Council of the European Union

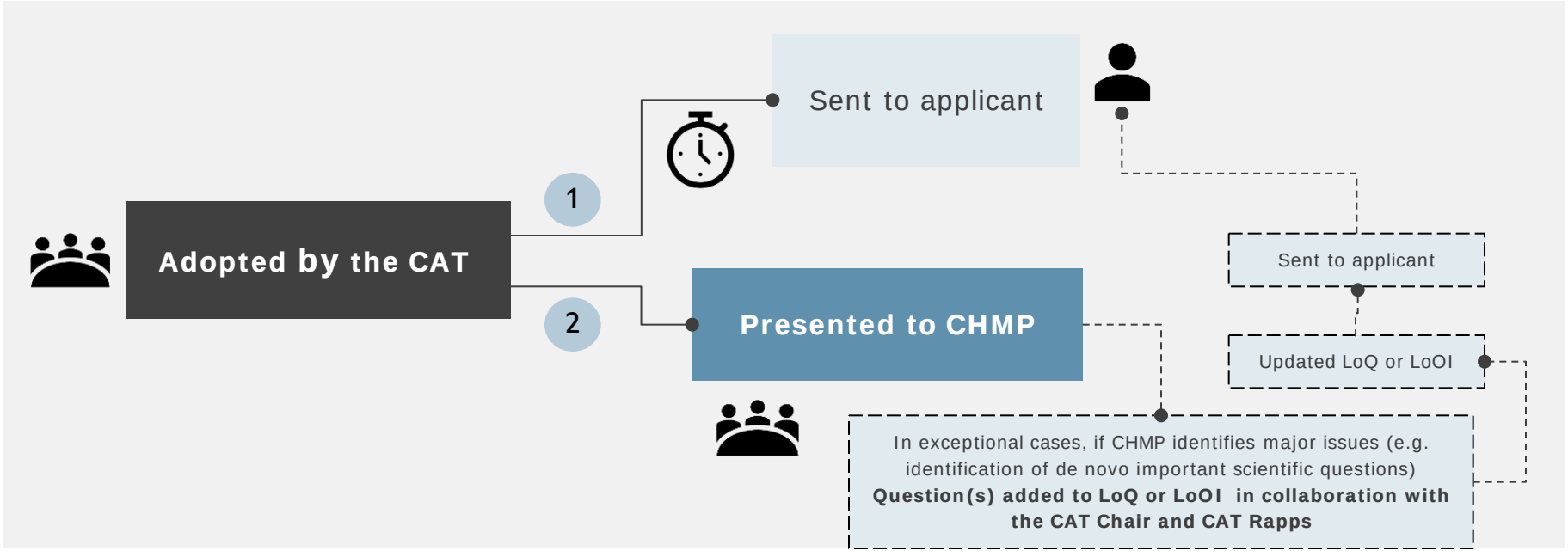
European Commission

European Parliament

1 Streamline of some procedural aspects:

1.a Same processes for adopting questions during the evaluation

List of Question (LoQ) process = List of Outstanding Issues (LoOI) process

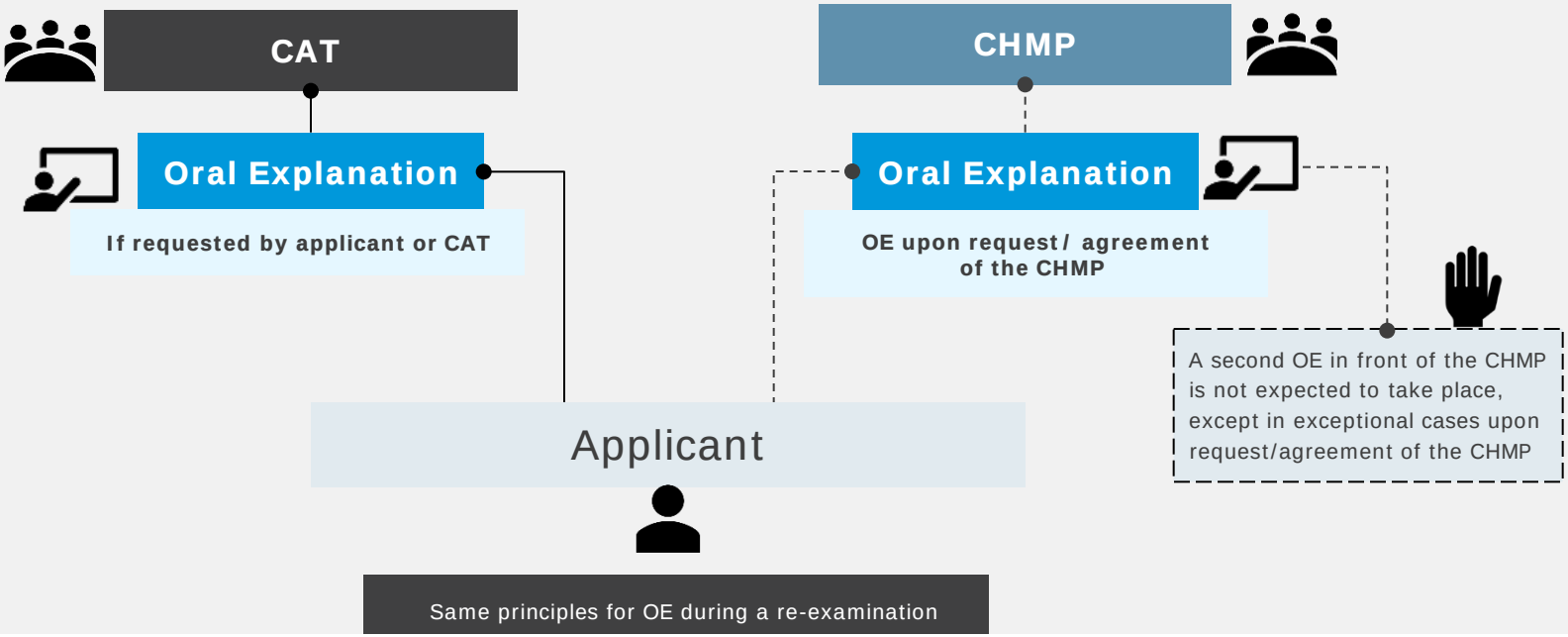


1

Streamlines some procedural aspects:

1.b Simplification of the process for oral explanation (OE)

The oral explanations for ATMPs take place in front of the CAT



2 Strengthening collaboration between the committees

- **CAT and CHMP members in each member state (MS) encouraged to discuss comments at national level** (to send one single list of CAT/CHMP comments per MS, when possible).
- **CAT (Co-)Rapporteurs should liaise with CHMP coordinators** (to ensure a consistent flow of information and facilitate discussions between committees). Similarly, better engagement with PRAC (Co-) Rapporteurs as necessary.
- **CHMP Coordinators to join the discussion at CAT for a given medicine:** to ensure adequate interaction and information flow between committees. Also applied to PRAC (Co-) Rapporteurs.
- **CAT (Co-)Rapporteurs should join discussions at CHMP** on the draft opinion submitted by the CAT.
- **CHMP Coordinators should attend the discussion ('oral explanation')** before the CAT.
- In exceptional cases when a CHMP oral explanation is needed: **CAT Chair and CAT (Co-)Rapporteurs** are expected to support the discussion.

3

Reflects current practice on the possibility of longer periods ('clock-stops') to respond to questions raised during the evaluation

For general guidance on clock stops, applicants can refer to:

"Time allowed for applicants to respond to questions and issues raised during the assessment of new marketing authorisation applications in the centralised procedure".

When justified, **CAT may agree to a longer 'clock-stop' to respond to questions and issues** during the evaluation of ATMPs.

4 *Reflects how PRAC contributes to evaluation of ATMPs*

PRAC plays the same role in the evaluation of any other medicine: provides **recommendations to the CAT on pharmacovigilance and risk management aspects**.

The **role of the PRAC and its scientific experts is detailed** (section 5.8 of the procedural advice).

All the **steps regarding PRAC involvement** are now reflected in the **standard timetable** (section 6.1 of the procedural advice).

Summary slide/take home message

EMA is working to **improve guidance to ATMP developers**, to facilitate development and patient access to these innovative medicines.

This update aims to **streamline some procedural aspects**, **strengthen collaboration** between EMA's scientific committees and **address specific needs** of ATMP developers in the evaluation procedure

- Reinforcement of timely and effective interactions between the applicants, EMA and its committees
- The processes for adopting the list of questions and list of issues by the committees have been streamlined
- More time to respond to questions raised by the Committees by allowing longer clock-stops

Glossary

ATMP: advanced therapy medicinal product: a medicine for human use that is based on genes, cells or tissue engineering.

CAT: Committee for Advanced Therapies: the committee that is responsible for assessing the quality, safety and efficacy of ATMPs, including gene therapy, somatic-cell therapy or tissue-engineered products.

CHMP: Committee for Medicinal Products for Human Use: the committee responsible for preparing EMA scientific opinions on questions concerning human medicines.

Clock stop: A period of time during which the evaluation of a medicine is formally stopped, while the applicant prepares responses to questions from the regulatory authority. The clock resumes when the applicant has sent its responses.

List of outstanding issues: A set of questions addressed to a company during a procedure, such as during the evaluation of a marketing authorisation application.

List of questions: A set of questions addressed to a company during a procedure, such as a marketing authorisation application.

MAA: marketing authorisation application: An application made to a EU regulatory authority for approval to market a medicine within the EU/EEA

Oral explanation: A presentation and discussion in person between representatives of an applicant and an EMA committee.

PRAC: Pharmacovigilance Risk Assessment Committee: the committee that is responsible for assessing all aspects of the risk management of medicines for human use.

(Co-)rapporteur: One of the two members of EMA's committee leading the assessment of an application.

Further information

Send a question to the European Medicines Agency:
www.ema.europa.eu/contact

European Medicines Agency
30 Churchill Place • Canary Wharf • London E14 5EU • United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Follow us on **@EMA_News**