EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

GMO/ERA in marketing authorisation applications of ATMPs containing GMOs

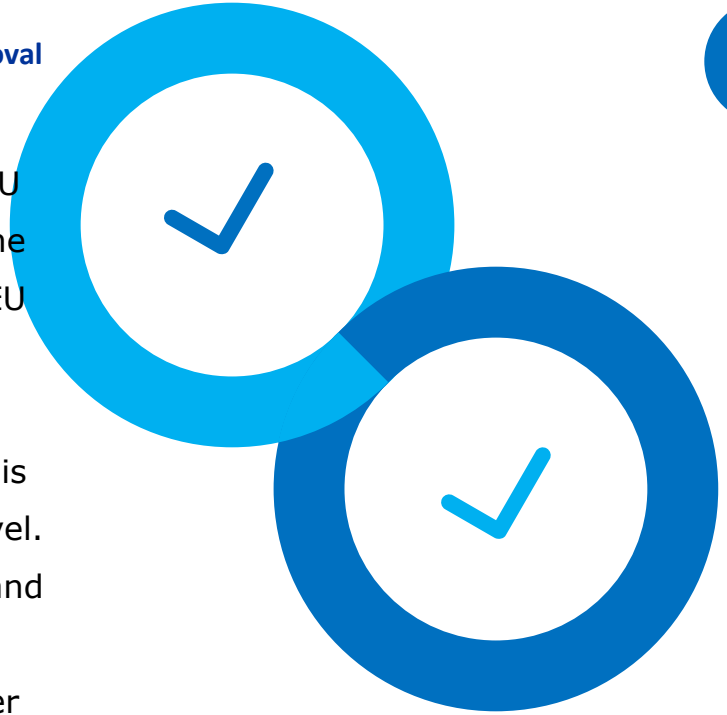
CAT Interested Parties meeting with Industry (26 October 2021)

Presented by Patrick Celis
ATMP Office

ERA evaluation

At the time of CT approval

The EU Clinical Trial Directive (EU CTD, 2001/20/EC)2 as well as the future Clinical Trial Regulation (EU CTR, Regulation (EU) No 536/2014)3 do not cover environmental aspects, and this is therefore legislated at country level. Consequently, the requirements and administrative procedures for Clinical trial approval might differ between member states



At the time of MAA

The environmental aspects at the time of the medicine approval are addressed during the review of the marketing authorisation by the European Medicines Agency (EMA) in a joint review between all member states.

Initiatives to remove hurdles for the development of medicines containing GMOs

Requirements 01

Centralised source of information for key requirements in EC website for CT and EMA website at the time of marketing authorisation

Guidelines 02

Updated guidelines, templates, streamlined procedures have been revised and published by the EC and EMA to guide developers and applicants

Application form 03

A common application form in all Member States to facilitate the assessment at the time of a clinical trial and later on at the time of MAA

Specific ERAs 04

Specific ERAs for products using AAV vectors or human cells genetically modified by means of retro/lentiviral vectors where more experience is available

Main guidelines and reference documents:

•Pre-authorisation procedural advice for users of the centralised procedure. What should I submit if my medicinal product contains or consists of genetically modified organisms (GMOs)? Rev. Feb 2020 (Doc. Ref. EMA/821278/2015)

•Guideline on environmental risk assessment for medicinal products containing, or consisting of, genetically modified organisms (Doc. Ref. EMEA/CHMP/BWP/473191/2006 – Corr).

•Guideline on scientific requirements for the environmental risk assessment of gene therapy medicinal products (Doc. Ref. EMEA/CHMP/GTWP/125491/2006).

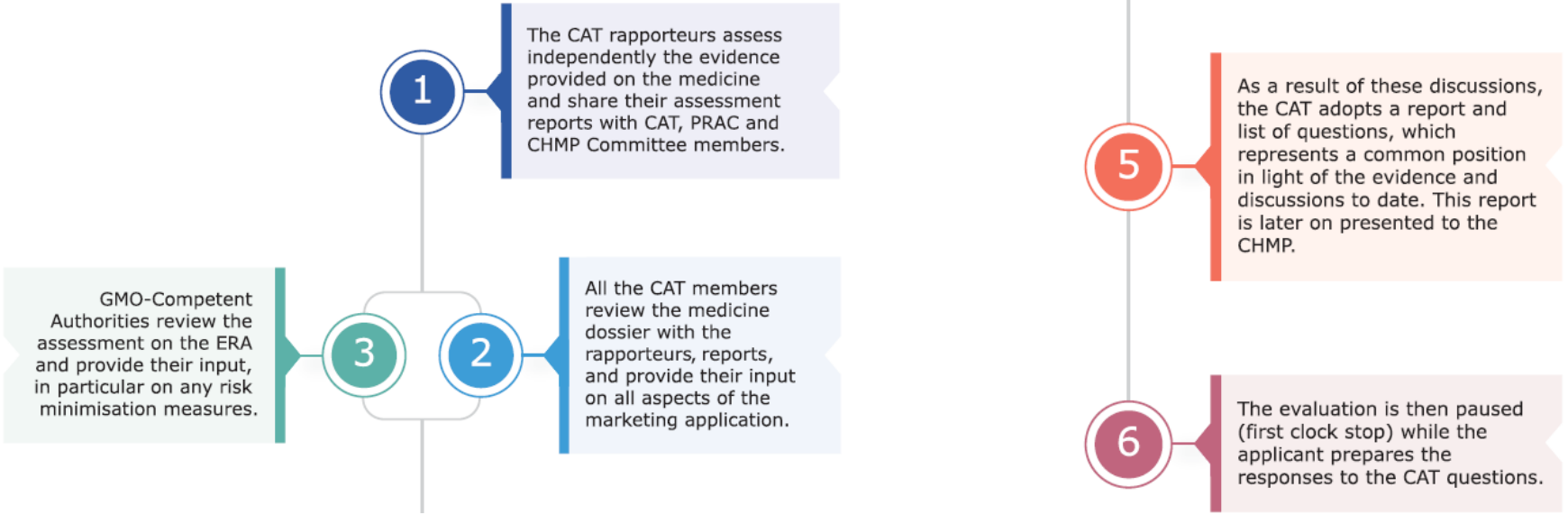
•Guideline *on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells (*EMA/CAT/GTWP/671639/2008 Rev. 1)

ERA submission during MAA

- Selfstanding ERA (Module 1.6.2): content
  - A copy of the CA's written consent to the deliberate release into the environment of the GMOs for research and development purposes
  - Technical dossier → Common application form (as developed for GMOs in clinical trials)
  - Environmental risk assessment → Specific ERA (AAV, GM-cells)
  - Results of studies relevant for ERA (biodistribution or shedding studies)

- Developers to identify need for control measures during reconstitution, handling & administration, personal protective equipment, decontamination & cleaning, waste management, recommendation to patients on donation of blood, tissues, cells

New consultation process with environmental competent authorities



Rhys Whomsley et all
Brit Jnl Clinical Pharma. 2021;87:2450–2458.

Any questions?

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