EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Committee for Advanced Therapies (CAT)

Presented by Kieran Been – CAT member

Committee for advanced therapies

- EMA's scientific committee for the evaluation of advanced therapy medicinal products (ATMPs) Regulation (EC) No 1394/2007
  - Provision of scientific recommendations on ATMP classification
  - Contribution to early discussions with developers
  - Contribution to scientific advice for ATMPs via ITF consultations
  - Preparation of scientific guidelines in the fields of gene- and cell-therapy and tissue-engineered products
  - Scientific evaluation of quality and non-clinical data for certification procedures
- Leads discussions among national EU authorities and engages with global regulatory authorities on international standardisation discussions.

Advanced Therapeutic Medicinal Products

Initial Evaluation of Marketing Authorisation Applications (MAA) for ATMP										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Submitted MAAs	3	1	2	3	2	2	1	1	3	18
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	1	10*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	4

Advanced Therapeutic Medicinal Products

Scientific recommendation on advanced therapy classification										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Submitted	22	19	12	22	20	28	61	60	43	287
Adopted	12	27	12	16	23	29	31	87	40	277

Scientific advice procedure for ATMPs										
	2009	2010	2011	2012	2013	2014	2015	2016	2017	Total
Number of procedures	17	19	21	19	23	33	39	46	44	261

CAT workplan 2017/18

- Revision and development of ATMP specific guidelines
- Reflection on the Benefit-Risk assessment of ATMPs
- Reflection on the use of Registry data for the initial evaluation of ATMPs and during the post-authorisation phase.
- Scientific and Regulatory considerations on gene editing technologies
- Considerations on novel scientific and regulatory approaches for making ATMPs more readily available to patients
- Addressing the Environmental Risk assessment of ATMPs containing genetically modified organisms (GMO) / genetically modified micro-organisms (GMM)
- Setting up of an *ad hoc* COMP-CAT working group

Expert meetings

6. September 2017

CAT Expert Meeting
Scientific and regulatory considerations for AAV-based gene therapy

EMA expert meeting on genome editing technologies used in medicinal product development

The European Medicines Agency (EMA) is convening an expert meeting (invitation only) on 18 October 2017 with the aim to discuss scientific and regulatory opportunities and challenges of medicinal product development using genome editing technologies.

Thank you for your attention

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