EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The Committee for Advanced Therapies (CAT): procedures and support to developers

CAT-DGTI Workshop – 11 September 2014

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CAT Secretariat

Committee for advanced therapies (CAT)

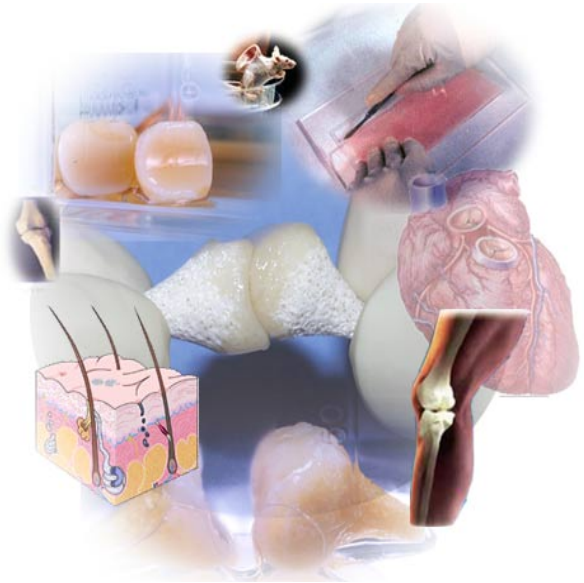
Regulation (EC) No. 1394/2007

First meeting in January 2009 – monthly meetings

Composition:

- 27 national experts and alternates with 5 joint members from the committee for human medicinal products and 2 joint members from the scientific advice working party
- 2 members and alternates represent patients organisations and 2 members and alternates represent clinicians

Tasks of the CAT



- Scientific Advice
- Support to PDCO
- EVALUATION
- Support to CHMP / COMP
- CERTIFICATION
- Interaction with stakeholders
- CLASSIFICATION
- Publications, Guidelines

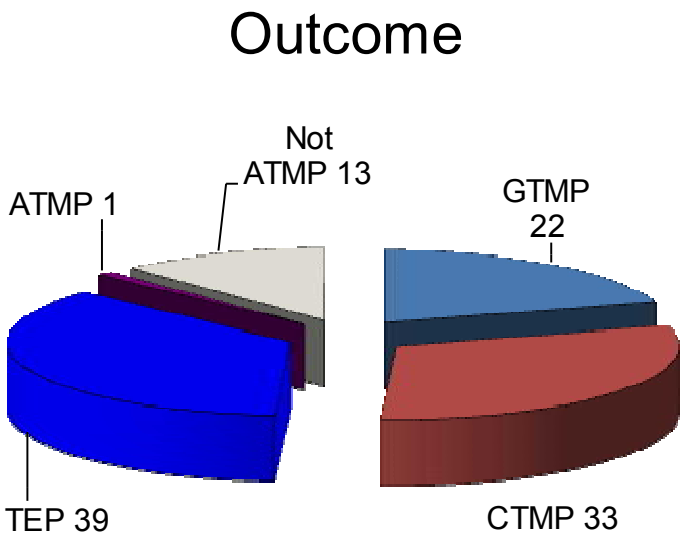
Challenges of ATMP Developers

1. Scientific challenges
2. Developer-related challenges
3. Socio-economical challenges
4. Legal/Regulatory/Political challenges

In the next slides, I will summarise the main support that is available to assist ATMP developers from R&D, through clinical trials and into the marketing authorisation procedure

Support to developers:
1. Classification procedure for ATMPs

- incentive: early / regulatory certainty
- open to all applicants
- scientific recommendation from CAT on the regulatory classification of their ATMP
- 60-day procedure (often shorter)
- publication of summary information on classification
- 108 procedures finalised
- http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2012/04/WC500126681.pdf



(Status July 2014)

Support to ATMP developers
2. ATMP certification

- Incentive: early-late / scientific certainty
- Only for SMEs
- Scientific evaluation by CAT of (early) quality / development data (Module 3) and non-clinical data (Module 4)
- 90 day procedure
- 5 Certification procedures finalised
  - Bone marrow derived progenitor cells for cardiac repair (quality: May 2010)
  - Autologous expanded smooth muscle derived cells for treatment of stress urinary incontinence (quality: Oct 2012 ; non-clinical: Sept 2013)
  - Autologous oral mucosal cells (quality + non-clinical: April 2014)
  - Autologous bone marrow derived MSC (quality: April 2014)

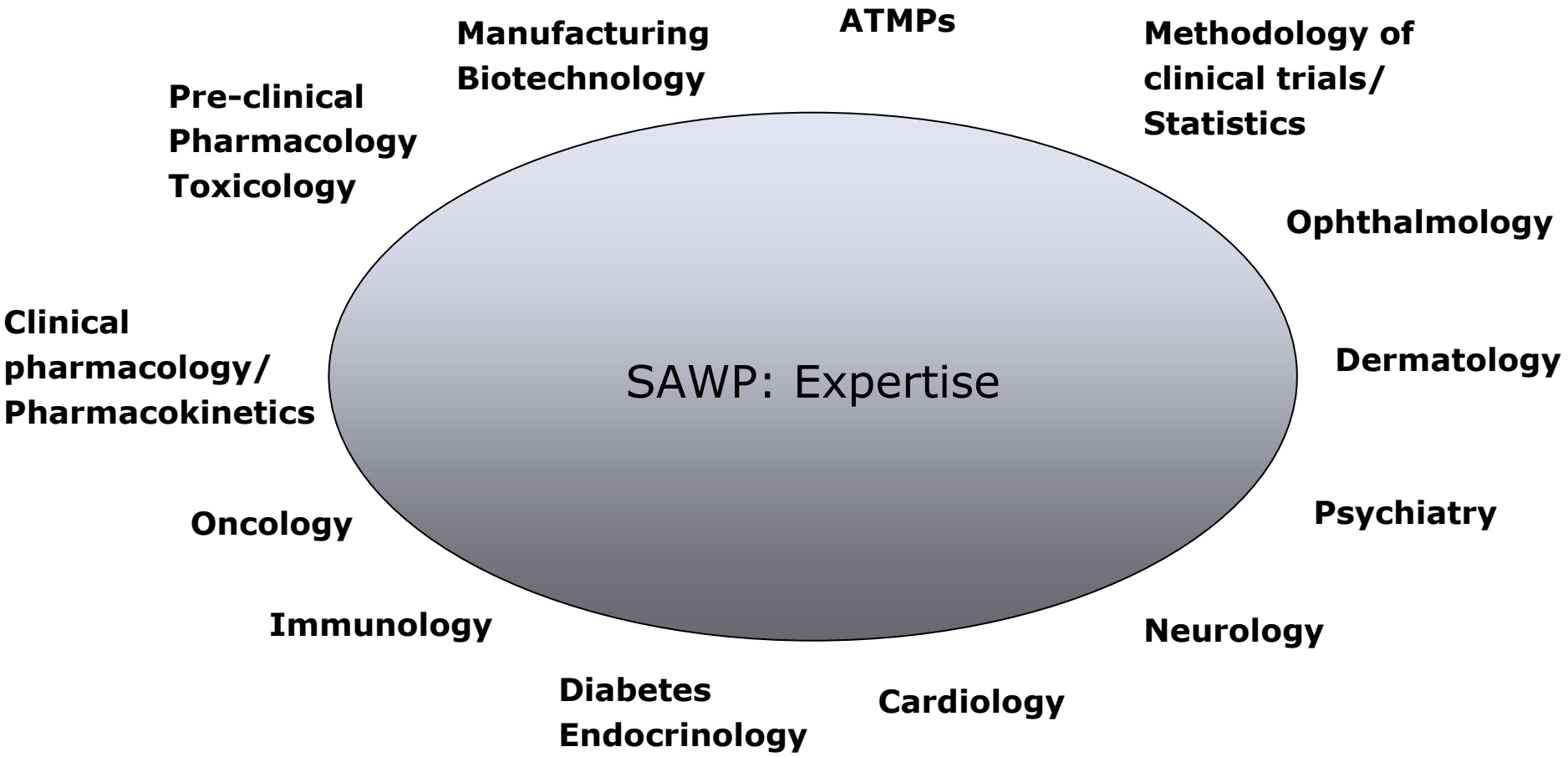
Support to ATMP developers: 3. scientific advice

- Incentive: Early-late, scientific certainty
- Open to all applicants
- Reduced fee for ATMPs
- Scientific advice is given from the scientific advice working party of the CHMP in collaboration with the CAT
- The scientific advice letter contains the consolidated view of the CHMP and the CAT

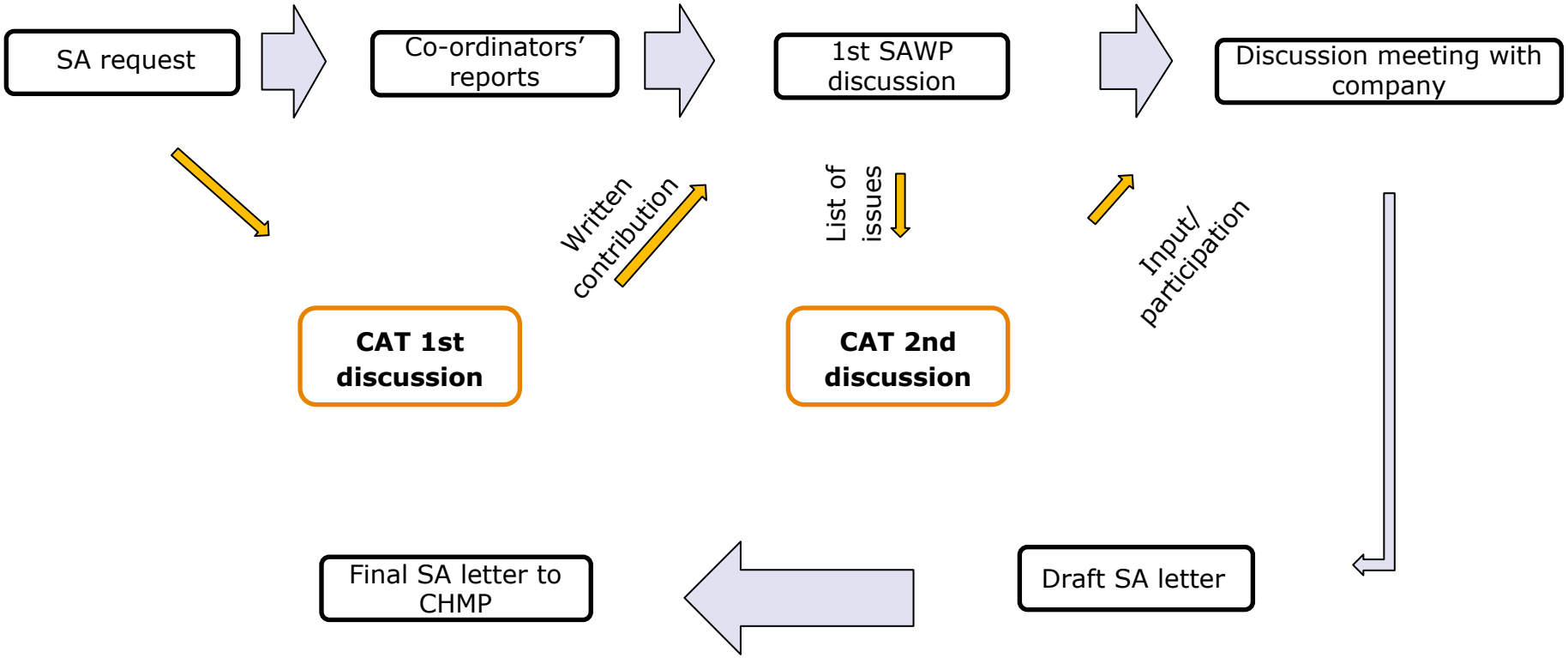
Scientific advice for ATMPs - benefits

- Simple fast procedure: 70 days including face to face meeting with the company at significantly reduced fees!
- Scientific advice is the forum to agree the methodological approach in quality, non-clinical and clinical
- Frequently asked questions for ATMPs:
  - Quality: comparability and potency assays
  - Non-clinical: relevance of animal models for pharmacology and toxicology
  - Clinical: milestones of study design, including e.g. non-feasibility to conduct a comparative trial

SAWP expertise



CAT – SAWP interactions to support ATMP development



Support to ATMP developers
4. Interactions with EMA/CAT

- Briefing meeting with the EMA Innovation Task Force
  - Platform for early dialogue on scientific, regulatory and legal requirements for innovative products (including ATMPs)
  - Informal, not binding
  - EMA staff + members for Committees and working parties
  - Face-to-face or virtual meetings

- Pre-submission meetings for ATMP certification, Scientific Advice, Orphan Designation and Marketing Authorisation

Support to ATMP developers
4. Interactions with EMA/CAT

- ‘One shop shop’ initiative
  - Scientific advice
  - ATMP Classification / Certification
  - Orphan drug designation
  - Paediatric investigation plan
  - Post-authorisation safety studies
  - Post-authorisation efficacy studies
  - Interaction with European health technology assessment bodies via scientific advice

Take home messages (1)

- ATMPs are novel, challenging medicines
  - Additional flexibility is possible: Risk based approach ➔ discuss with authorities
- Consult authorities as early as possible
  - early dialague will increase understanding: (for you) of the expectations of the Regulator and requirements for MAA; (for us) of technical limitations and regulatory hurdles
  - How? Informal meeting with Innovation Task Force (EMA) or national innovation offices, SME office, ATMP classification, Scientific advice (national, EMA), …

Take home messages (2)

- CAT and EMA can help to address your challenges:

Challenge	CAT/EMA help
Scientific challenges	Yes (SA, Certification, Briefing meetings)
Developer-related challenges	Yes (partly), e.g. procedural support to SMEs
Socio-Economical challenges	Yes (partly); e.g. joint SA-HTA scientific advice
Legal/Regulatory/Political challenges	Limited. CAT alert the EC of issues + assist EC if revision of ATMP regulation

Thanks for your attention

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Visit also the Advanced Therapies webpage: www.ema.europa.eu (type in 'Advanced Therapies' in search tool)