EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Session 1: Initiatives to improve ATMPs access to patients

Agency initiatives to foster the development and registration of ATMPs and improve patient access

EMA-EBE "Optimising the development of ATMPs to meet patient needs"
16 December 2016, CCT Venues, Canary Wharf, London, UK

Presented by Ana Hidalgo-Simon
Head of Specialised Scientific Disciplines Department, European Medicines Agency

ATMP landscape in Europe (2009- 2016)

EUROPEAN MEDICINES AGENCY

~ 500 clinical trials using ATMPs in EU
(2009-2016)

~ 230 ATMP classifications
(2009-2016)

~ 215 scientific advice requests
(2009-2016)

15 MAAs reviewed
(2009-2016)

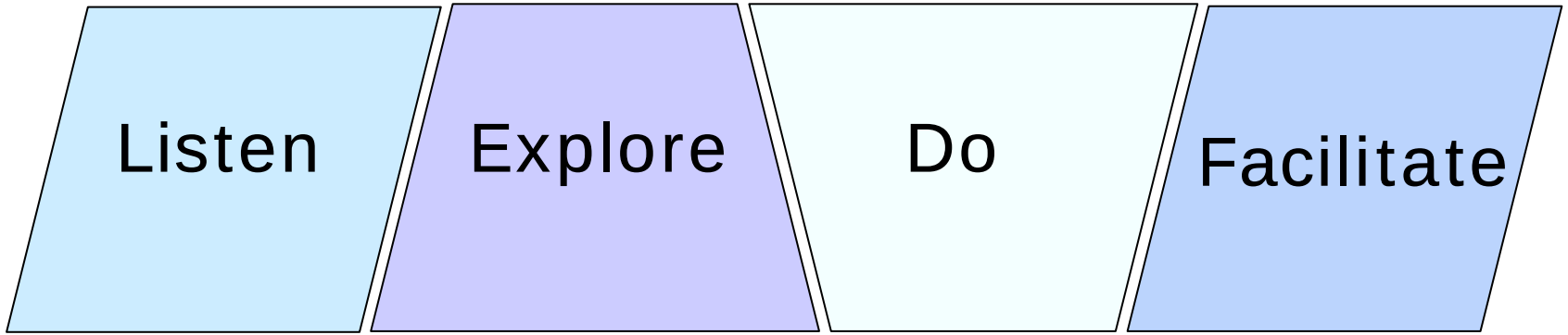
8 ATMPs approved
(2009-2016)

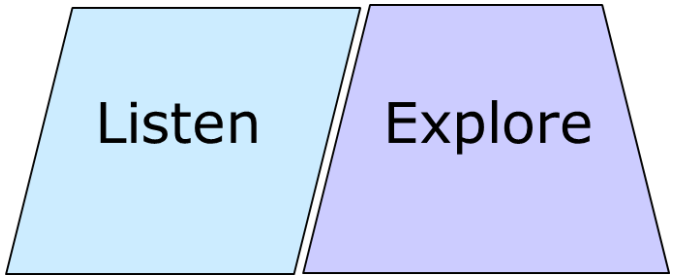
3 withdrawn

market

5
licensed ATMPs

What can the EMA (in collaboration with CAT and the network) do to support ATMP development and patient access?





EMA's **Multi-stakeholders ATMP Expert** meeting of 27th May 2016

- **Listen** to the experience of various stakeholders: Academia, Patients, Treating Physicians, Hospital Pharmacists, Consortia, Incubators, Investors, HTAs and Industry.
- **Explore** solutions to
  - stimulating innovation,
  - facilitating European R &D and
  - accelerating patient's access to high quality, safe and efficacious ATMPs

Outcome of Workshop summarises concrete proposals

http://www.ema.europa.eu/docs/en_GB/document_library/Report/2016/06/WC500208080.pdf

Research and Development

Regulatory Process

Moving from hospital exemption to marketing authorisation

Funding, investments and market access solutions

Outcome of Workshop summarises concrete proposals

http://www.ema.europa.eu/docs/en_GB/document_library/Report/2016/06/WC500208080.pdf

Research and Development

Pragmatic approach on requirements for ATMPs (e.g. GMP, innovative manufacturing models, GMO requirements, guidance)

Moving from hospital exemption to marketing authorisation

Transparency in implementation and use of hospital exemption across Member States & standardised data collection of clinical experience

Outcome of Workshop summarises concrete proposals

http://www.ema.europa.eu/docs/en_GB/document_library/Report/2016/06/WC500208080.pdf

Regulatory Process

Streamline EMA internal regulatory processes +
Promote use of early access tools +
Harmonise and guide on application documents & national requirements +

Raise awareness of regulatory process,
early engagement and collaboration,
develop different models for reimbursement and payment

Funding, investments and market access solutions

Summary and next steps

- Recurring themes touched upon at the meeting by the stakeholders include the need for **early interaction** and **guidance from regulators**, **more transparency** and **information sharing**, **greater harmonisation between Member States** in various aspects of ATMP regulation and **measures to tackle inequalities in patient access to ATMP treatments**.
- The **European Commission**, **national competent authorities**, and **EMA** have already started discussing the **feasibility, responsibilities** and **priorities** of these proposals. Regulators, including HTAs, welcome the contribution from stakeholders and will **decide on appropriate actions**.

Do

What can EMA do?

- Support the Committee for Advanced Therapies to evaluate ATMP applications and develop new concepts and guidance (CAT work plans 2016 & 2017)
- Increase the focus on ATMPs of existing Agency initiatives (e.g. ITF, PRIME, parallel scientific advice)

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

22 January 2016
EMA/CAT/514252/2015
Procedure Management & Business Support Division

CAT (Committee for Advanced Therapies) work plan 2016
Adopted by the Committee on 22 January 2016

Table of Content

1. Evaluation activities for human mec
1.1. Pre-authorisation activities .....
1.1.1. Requirements for ATMPs in clinical trials
1.1.2. Requirements for minimally manipulatec
1.1.3. Simplification of procedures and require
1.1.4. Reflection on similarity of orphan ATMPs
1.1.5. Extrapolation .....
1.2. Initial Evaluation activities.....
1.2.1. Simplification of procedures for ATMP m
1.2.2. Assessor training .....
1.3. Pharmacovigilance activities .....
1.3.1. Simplification of procedures and require
phase .....
1.4. Other specialised areas and activities.....
1.4.1. Development of cell-based cancer immu
1.4.2. Scientific and regulatory considerations

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

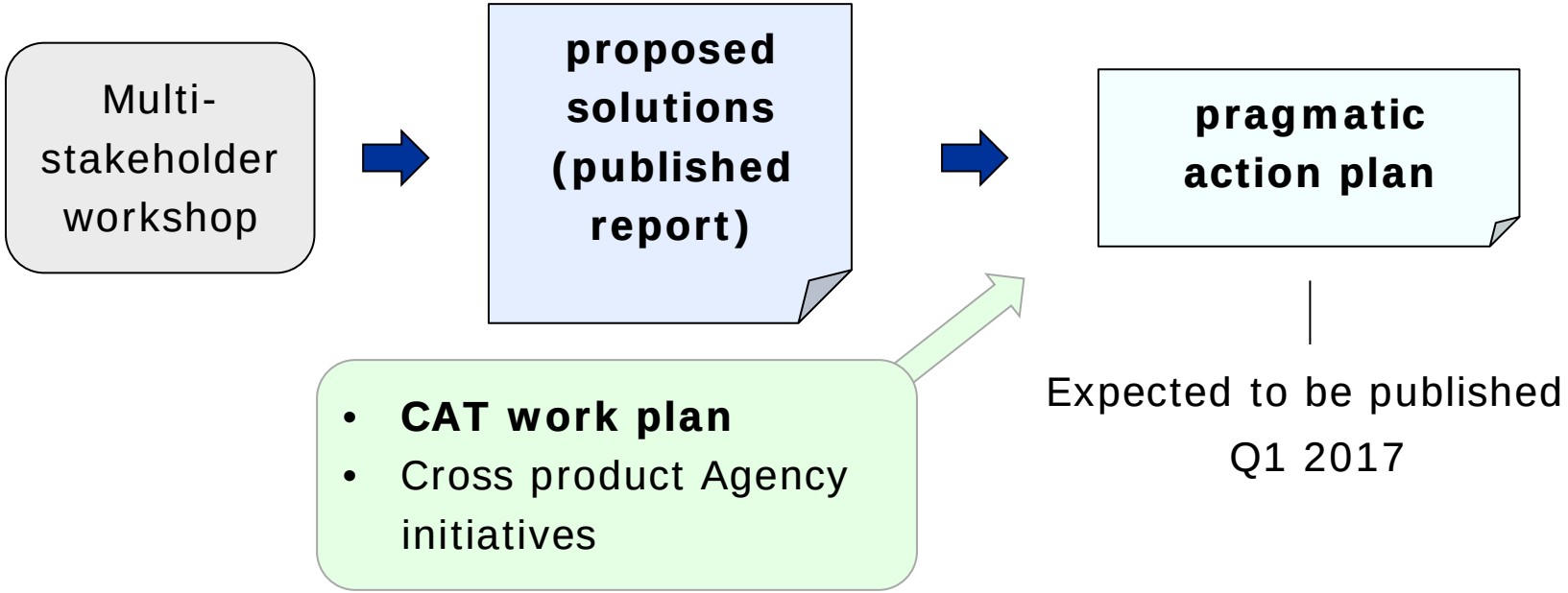
7 December 2016
EMA/378101/2015
Inspections, Human Medicines Pharmacovigilance & Committees

CAT work plan
2017 – for adoption by the Committee on 20 January 2017

Table of Content

1. Evaluation activities for human medicines 2
1.1. Pre-authorisation activities 2
1.2. Initial-evaluation activities 4
1.3. Other specialised areas and activities 6
2. Horizontal activities and other areas 10
2.1. Committees and working parties 10

Develop a pragmatic, forward looking action plan that considers stakeholder's input



proposed solutions (published report)

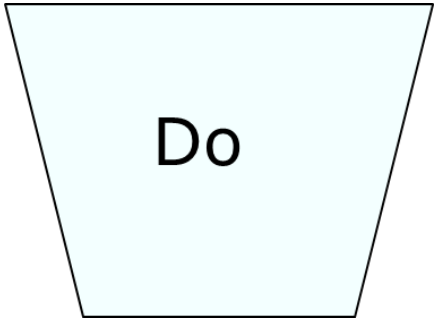
prioritisation

pragmatic action plan

Prioritisation

- Actions starting in 2017 and beyond
- EMA is in the lead on certain topics with EMA legal remit
- Have been assessed to be of ‘high priority’ and ‘feasible’
- Actions that do not require changes to the legal framework on ATMPs

Action plan to be revised periodically and updated with further actions/relevant network and stakeholders interactions



Actions within legal EMA remit

some examples under discussion

- Streamline of review procedures for ATMPs and committee interaction
- Develop guidance (e.g. investigational ATMPs, minimally manipulated cells)
- Explain the risk-based approach
- Raise awareness on early access tools relevant to ATMPs
- Disseminate ATMP related information to the network

Facilitate

Actions where EMA can support with the European Commission, EU Network, relevant stakeholders

some examples under discussion

- Facilitate transparency on ERA/GMO assessment
- Support member states in working towards greater transparency of hospital exemption
- Share and provide information to all relevant stakeholders
- Contribute to orphan similarity as applied to ATMPs

Next steps

- Plan with actions to improve the regulatory and scientific environment for ATMPs under development – publication early 2017
- Continue to listen to all stakeholders
- Monitor the scientific and regulatory environment for ATMPs in Europe (and globally)
- Re-visit suggestions from the workshop not taken in the current plan
- Revise actions as new information becomes available

Thank you for your attention

Further information

ATMP microsite:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000294.jsp&mid=WC0b01ac05800241e0

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**