EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The Committee for Advanced Therapies (CAT)

Dr Kieran Breen

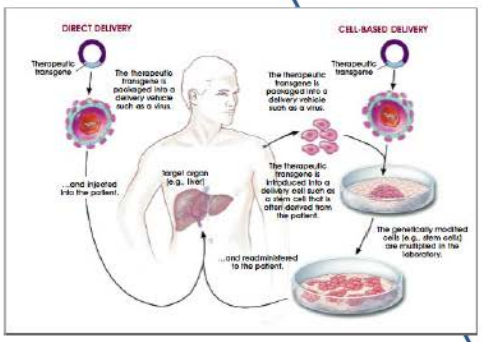
Advanced therapy medicinal products (ATMPs)

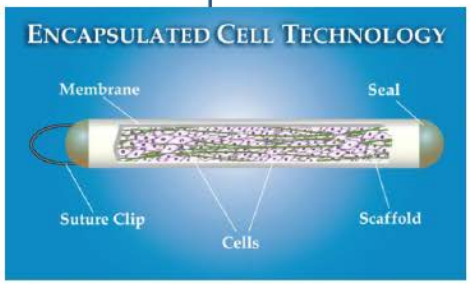
Gene Therapy Medicinal Products

Somatic Cell Therapy Medicinal Products

Tissue Engineering Products

Genetically modified cells






Severe burn victim before and 6 months after treatment with Dermagraft.

medical device + ATMP → combined ATMP

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	1	16
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
Ongoing MAAs										2

*** Corresponding to 9 ATMPs**

[i] Same product (Cerepro)

[ii] Same product (Glybera)

[iii] CAT adopted two negative draft opinions for the same product (Heparesc)

2017 - recommended granting a marketing for a new product (ATMP) to treat adult patients who have knee cartilage defects
- Developed from hospital exemption programe

June 2017

CAT procedures

Scientific recommendation on advanced therapy classification										
Adopted	12	27	12	16	23	29	31	87	16	253

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	24	241

June 2017

The PRIME programme

PRIME builds on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment

To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data.

Once selected for PRIME, the Agency will:

- appoint a **rapporteur** from CHMP and from CAT for ATMPs to provide continuous support and **ahead of a marketing-authorisation application;**
- organise a **kick-off meeting** with the CHMP/CAT rapporteur to provide **guidance on the overall development plan and regulatory strategy;**
- provide **scientific advice at key development milestones**, to facilitate quicker access for patients to the new medicine;

Prime Eligibility for ATMPs

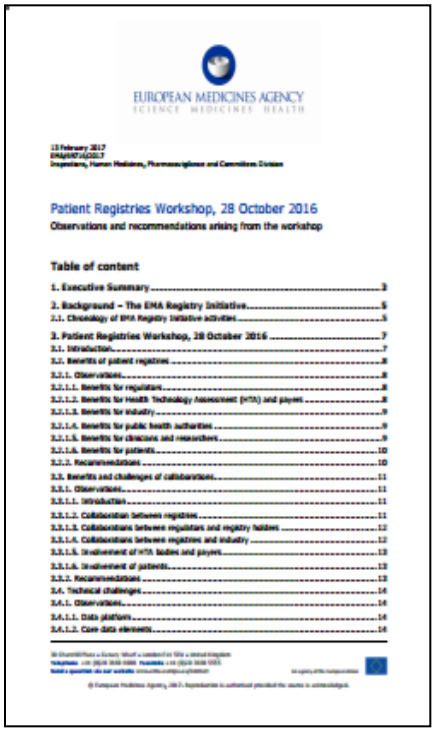
	2016	2017							Total
Discussed	22	9							31
Granted	8	4							12

Additional CAT activities for 2017

- Revision of the **Guideline** on quality, non-clinical and clinical aspects of medicinal products containing **genetically modified cells**
- Reflection on the **Benefit-Risk assessment of ATMPs**
- Scientific and regulatory considerations on **gene editing** technologies
- Contribution to the European Commission discussions on **GMO related issues**
- CAT discussion on the **use of registry data** for the approval and post-marketing follow-up of ATMPs (with cross-committee Patient Registry Initiative)

28th October 2016 - Patient Registries workshop recommendations

- **Common core data elements for registries**
- **Specific data elements for treatment classes / innovative products**
- **Real world evidence**
- **Safety aspects (e.g. reporting of ADEs)**
- **Data quality**
- **Governance for interactions between stakeholders**
  - data ownership, data analysis, data sharing/ registry interoperability
- Facilitation of **interactions** between regulators and registry holders
- Development of an EMA registries strategic initiative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Patient Registries Workshop, 28 October 2016

Observations and recommendations arising from the workshop

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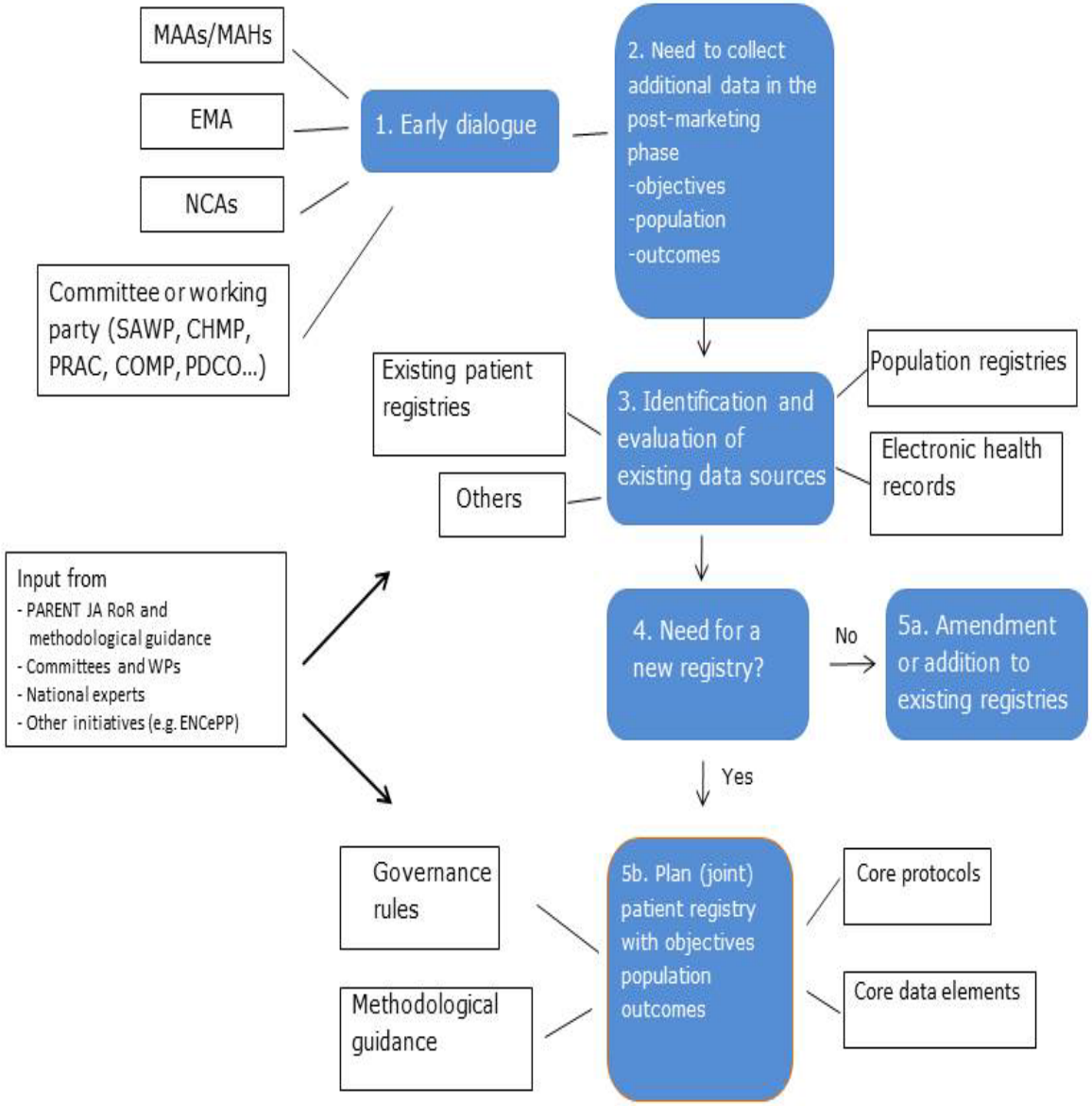
MAAs/MAHs
EMA
NCAs
Committee or working party (SAWP, CHMP, PRAC, COMP, PDCO...)
1. Early dialogue
2. Need to collect additional data in the post-marketing phase
-objectives
-population
-outcomes
Existing patient registries
Others
3. Identification and evaluation of existing data sources
Population registries
Electronic health records
Input from
- PARENT JA RoR and methodological guidance
- Committees and WPs
- National experts
- Other initiatives (e.g. ENCePP)
4. Need for a new registry?
No
5a. Amendment or addition to existing registries
Yes
Governance rules
Methodological guidance
5b. Plan (joint) patient registry with objectives population outcomes
Core protocols
Core data elements

Activities for 2017

To scope the development of an EU collaborative framework for patient registries that would **facilitate** the use of **existing patient registries**, and **setting-up new registries if none is available or adequate**, in order to collect and analyse **high quality data informing regulatory decisions** and the evaluation of the benefit-risk profile of medicinal products.

Thank you!