EMA
EUROPEAN MEDICINES AGENCY

Patient involvement in the Committee for Advanced Therapies

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Vice-Chair CAT

Nominated by Parkinson's Europe

parkinson's europe

Patients at the EMA

- Management Board
- Committees
  - CAT, PDCO, PRAC, COMP
- Working groups
  - Registries, drafting groups, expert groups
- PCWP
- Scientific advisory groups (SAGs)
- Ad-hoc advisory groups

The Committee for Advanced Therapies

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

→ European Commission

Draft opinion

Final opinion

Authorisation for placing ATMPs on the market in the European Union

Experts from 26 EU member states, Norway, Iceland, Liechtenstein, **Patient and physician representatives**, n=66
Multi-disciplinary expertise

Patient representatives on CAT

- Full membership of the committee
- Vote on all products and procedures
- Stand for chair/vice-chair of CAT
- Can be Rapporteur, Co-Rapporteur, Peer reviewer
- Can be peer reviewer for scientific guidelines and advice
- Can take part of assessment team for:
  - ATMP product classification
  - Certification of Quality/Non-Clinical data
  - MAA for ATMP

Patients' Contribution to EMA Scientific Committees

Expertise: convey a combination of specific education, training and professional experience as a patient organisation nominee.

Experience: convey practical disease knowledge obtained from direct contact with "a disease" (affected person or contact with an affected person).

Advocacy: act on behalf of the affected patients in defence of their rights; provide patient-oriented public health/healthcare policy perspective.

Empowerment: provide access to the information necessary to participate in the decision-making processes on behalf of all patients.

Role of patient organisations' representatives on the CAT

- Act as a patient champion e.g. patient and healthcare briefings in SmPC
- Highlight patient-specific issues at the CAT
- Communicate the role of the CAT to external audiences (including PCWP) and to patient organisations where appropriate
- Educate patients about ATMPs (e.g. CAR T cells) and also unproved cell-based therapies
- Propose patient experts and link with outside Patient Organisations useful for their specific expertise (e.g. nominate patient members to SAGs)
- Represent the CAT at dialogues with patient organisations on the development of products in specific disease domains
- Propose actions beyond the regulatory framework: e.g. proposal for a CAT work programme addressing issues related to ATMPs development

The ATMP lifecycle – patient involvement

ATMP DEVELOPMENT

MARKETING AUTHORISATION

POST-APPROVAL LIFE CYCLE MANAGEMENT, PHARMACOVIGILANCE

Clinical Trials

Clinical trial authorisation

CAT

CHMP

PRAC

Benefit-risk assessment / opinion

(Conditional) Approval

Post-authorisation data generation,

Real World Data/Registries,

Clinical trials, indication extension

HTAs,

Reimbursement

Patient accesss

National Scientific Advices

EMA scientific advice

National HTA advice

EMA/HTA parallel advice

RWD/registries

Scientific assessments and authorisations by experts from NCAs throughout life cycle

Patient registries

- Use observational methods to collect uniform data on a population **defined by a particular disease**, condition, or exposure followed over time.
- A patient registry is established primarily by a **clinician or a patient/consumer organisation**.
- Clinical **information is collected over time** and samples (e.g. blood specimens) may also be collected.
- Patients need to be aware of what information is being collected, **how it will be used and by whom**
- Make valuable contributions to the **evaluation and monitoring of medicines for public health benefit**, especially in relation to their safety

EMA patient registries initiative

- Explore mechanisms for regulators and MAAs to consider where registry data may support benefit-risk evaluations throughout product lifecycles and to interact early with registry holders to determine data availability
- Share and disseminating information on patient registries in specific disease areas
- Recommend governance principles and standards for stakeholder interactions
- Make recommendations on core data elements and quality standards acceptable for regulatory decision-making
- Identifying registry holders' needs for methodological and technical guidance
- Investigate what patient-reported outcomes to collect in registries
- Explore further measures to improve the sustainability of registries

CAT SMA registry study

- The study used data collected in SMA from patients enrolled across 6 patient- and clinician-based registries in 9 European countries as part of the TREAT-NMD network. This is the first of its kind to evaluate natural disease progression, clinical, healthcare management and treatment patterns from multiple specific-disease registries across Europe, in the context of rare SMA disease.

- The results were globally consistent with existing studies evaluating the progression of the SMA disease. Clinically relevant gains in motor function were observed in SMA 1, SMA 2 and SMA 3 treated patients per DMTs.

- Improving the data accuracy and quality, reducing the missingness and identifying important data items could help greatly answering key questions for the SMA community and regulatory decision making.

- Our study exemplified that the use of multiple registries in rare disease provides complementary information and new avenues to answer regulatory research questions.

CAT and the PCWP

- Update other patient organisations on the role of the CAT
- Inform patient organisations about the latest developments in the field of ATMPs
  - CAR-T cell therapies
  - Gene therapies
  - Unproven cell therapies
- Feedback from patient organisations to the CAT on issues relating to medicines availability and cost
  - Liaison with HTA and cost reimbursement
  - Availability of medicines

Patient Communication

Expertise and experience

Patient organisations

Understanding of the regulatory process

Patient Communication

- Liaison with patient organisations
  - The work of the CAT (specifically) and the EMA (in general)
  - Understanding the regulatory process (particularly during COVID)
  - Encouraging patient engagement at all levels – clinician involvement is key
- Promoting the role of patients in the design of clinical trials
- Explaining the decision-making process and, particularly, why products may be rejected (US vs. EU)
- Input into the design of clinical trials based upon MAA assessment
- Highlighting the importance of good quality patient-reported outcomes (PROMs) and patient evidence data (PED) to support clinical outcome data

CAT Public Statement 2020

24 February 2020
EMA/CAT/94295/2020
Committee for Advanced Therapies

Unproven cell-based therapies: a risk to public health and to the development of advanced therapies

Public statement

The ATMP lifecycle – patient communication

ATMP DEVELOPMENT

Clinical Trials

- Recruiting clinicians
- CRAs
- Pharmaceutical industry
- Patient organisations

Indirect interaction

MARKETING AUTHORISATION

CAT → CHMP → European Commission

EMA press release

POST-APPROVAL LIFE CYCLE MANAGEMENT, PHARMACOVIGILANCE

Post-authorisation data generation,
Real World Data,
Clinical trials, indication extension

HTAs,
Reimbursement
Patient accesss

Indirect interaction

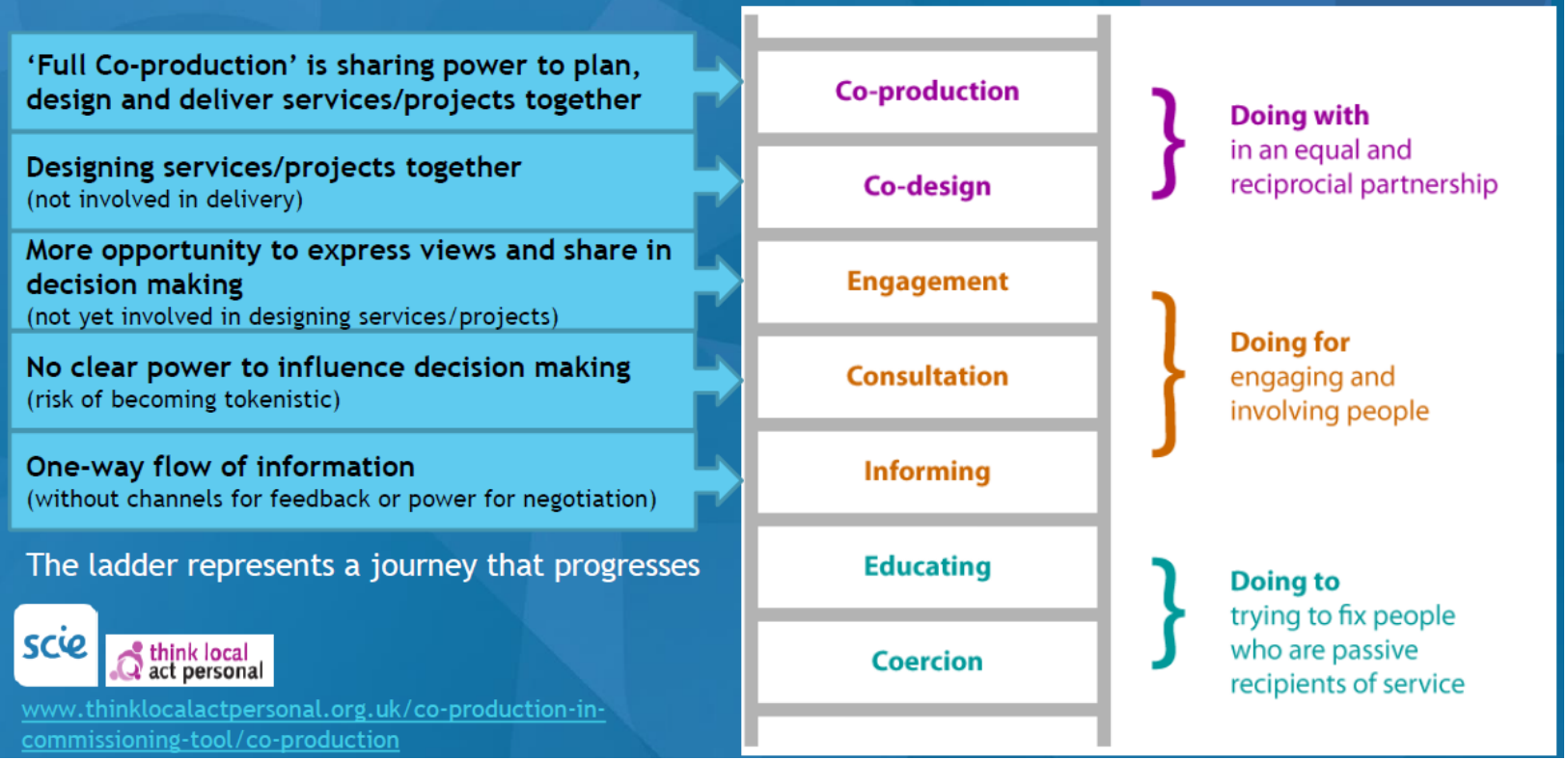
Key points in my journey at the CAT

October 2012	Replied to advertisement on Twitter for patient representative on CAT nominated by EPDA
July 2013	Induction and first meeting
Oct 2014	Joined PCWP as CAT representative
March 2015	ATMP classification
Jun 2015	Peer review scientific advice (AAV2/8 product)
Oct 2015	Joined Cross-Committee Task Force on Patient Registries as CAT representative (EBMT, WFH, RWE, big data steering group)
Oct 2021	CAT SMA registry project initiated
Sept 2023	Appointed CAT vice-chair
Dec 2023	Patient experience data (PED) reflection paper expert group

The next 15 years…

- There will be a greater awareness of the role of patients of the regulatory arena
  - The regulator will work with the patient/consumer to ensure that the best quality healthcare products are made available on the market as efficiently as possible and ensure that they are followed up (PASS and PAES) appropriately
- CHMP is likely to have patient and healthcare professional input under new legislation
- Patient input should be meaningful rather than tokenistic and must have a genuine rationale behind their inclusion
- Clinical trial design should include PROMs and the use of PED, but only where the use of this data is truly meaningful and will add to the benefit/risk analysis
- Patients should be engaged in all stages of medicines development and should have a clear understanding of their role in the process

The Ladder of Co-production



The ladder represents a journey that progresses

scie
think local act personal

www.thinklocalactpersonal.org.uk/co-production-in-commissioning-tool/co-production