EUROPEAN MEDICINES AGENCY
PCWP / HCPWP
Joint Meeting

WORLD DUCHENNE ORGANIZATION

AMSTERDAM APRIL 1-2 , 2025
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PCWP / HCPWP

"10 years experience of patient involvement EMA" Opportunities, Challenges and Learnings for meaningful patient involvement

DISCLAIMER

Disclosures

I have no real or apparent relevant financial relationships to disclose

DOI

www.ema.europa.eu/docs/en_GB/document_library/contacts/athanasioud_DI.pdf

47 MEMBER PATIENT ORGANIZATIONS FROM 39 COUNTRIES

Founded in 2005 as UPPMD with a strong focus on:

- Harmonizing Reseach Funding & Knowledge
- Advocacy
- Regulatory work
- Policy

Currenty :

- WDO is Global network of member patient organizations
- WDO is member and contributed in board position in EURORDIS and EPF but also part of RDI.
- WDO's Board Members are involved in FDAs and EMA's EMA Committees, WPs and activities
- Eligible member of the European Medicines Agency

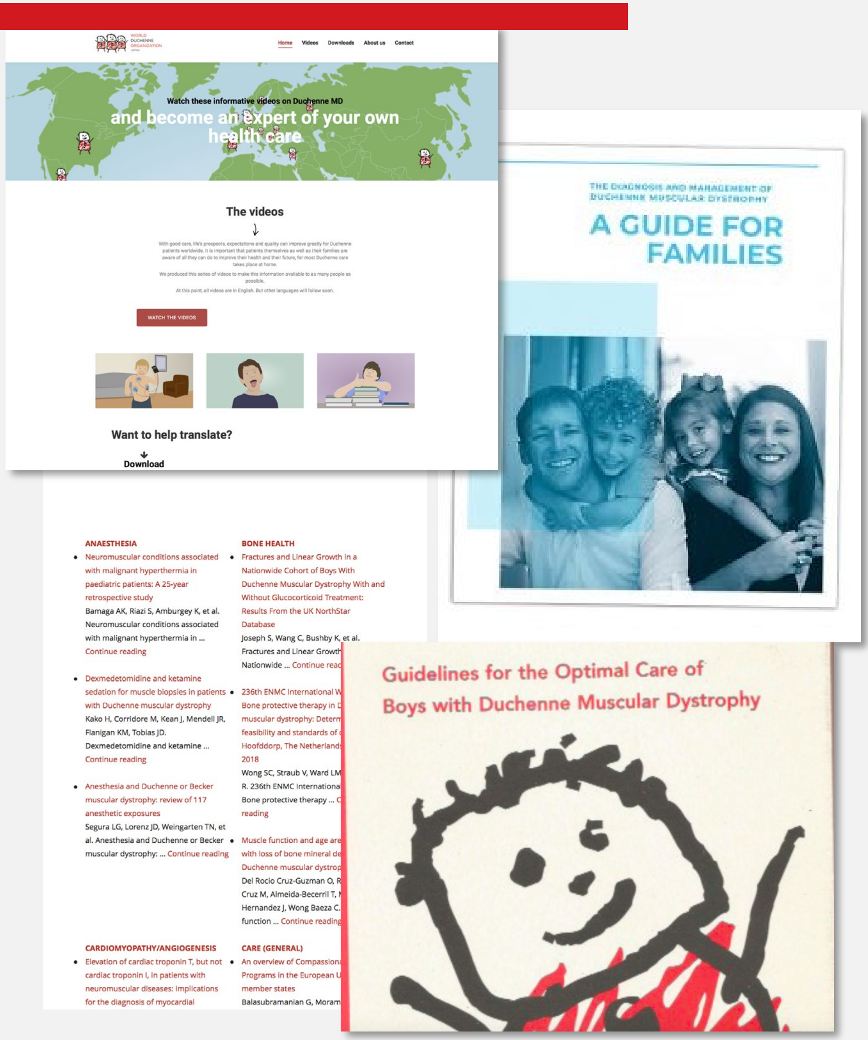
The Community

In Research :

- Multi Millions € invested in Research and Care
- CT increase from 10 to 100+ clinical trails in the last decade
- Patient input to almost all the CTs in DMD via CAB
- Involvement in almost all DMD CTs in EMA, FDA, MHRA etc

In Care and Education :

- International SoC development
- Accredited Duchenne Care Center Globally
- Increase in life expectancy by 7 years every decade
- Global Care Conference (+700 Part from 72 Countries
- Health literacy, almost 100+ patient experts trained per year
- Education, materials reach out to more than 10,000 families



Credit : Chris Barbalis@cbarbalis

The Community

The DMD community is supporting from basic Research to Care and Medicines Development :

Community Advisory Board, Duchenne Patient Academy
- In Silico Development A.I. and Machine learning
- Care Guidelines development
- **Regulatory Guidelines for DMD Developers**
- **Clinical Trials Simulation Tools development**
- **Duchenne Platform Trials development**
- New outcome measures development
- PROMs and PROs development and validation
- **Preclinical research support & Animal models**
- **Virus Development & ATM research like Gene and Cell Therapies,**
- **Gene editing and Exon skipping technologies**
- **Biotech and Spin-off seed funding**
- Exoskeletons and Supportive Digital Applications

Credit : Chris Barbalis@cbarbalis

BETWEEN HYPE AND HOPE

- Still the pathway is marked with successes and failures.
- The boys live longer, have a better a life, but still lose the fight with Duchenne.
- Balancing between Hype and Hope the community still fights to keep the boys alive while keeping DMD and the Rare Diseases in the centre of R&D, Regulatory and Policy discussions.

WDO’s INVOLVEMENT IN THE ECOSYSTEM

DG SANTE

EUROPEAN COMMISSION

HMA
Heads of Medicines Agencies

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HTA CG

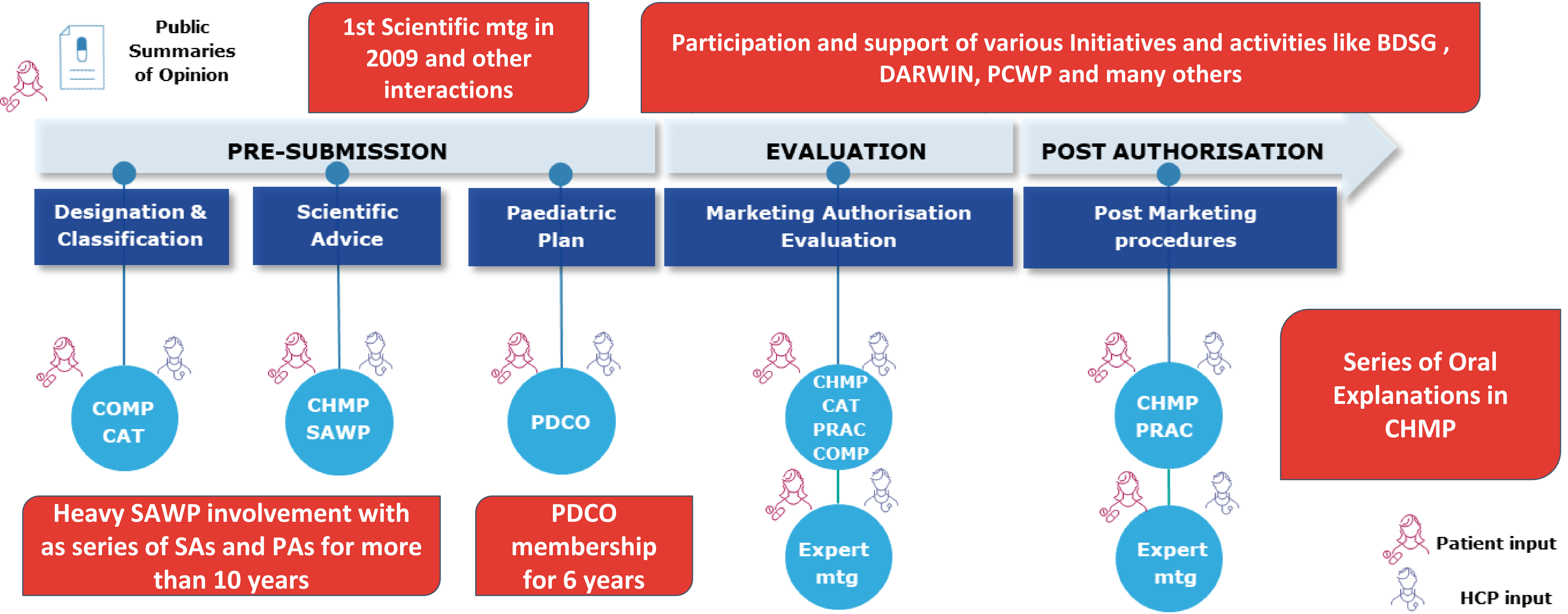
COUNCIL OF EUROPE

EUROPEAN PARLIAMENT

FDA

WDO’s INVOLVEMENT, COMMITMENT & INVESTMENT

EMA Public and Stakeholder Engagement Department



Public Summaries of Opinion

1st Scientific mtg in 2009 and other interactions

Participation and support of various Initiatives and activities like BDSG , DARWIN, PCWP and many others

PRE-SUBMISSION

EVALUATION

POST AUTHORISATION

Designation & Classification
- COMP
- CAT

Scientific Advice
- CHMP
- SAWP

Paediatric Plan
- PDCO

Marketing Authorisation Evaluation
- CHMP
- CAT
- PRAC
- COMP
- Expert mtg

Post Marketing procedures
- CHMP
- PRAC
- Expert mtg

Series of Oral Explanations in CHMP

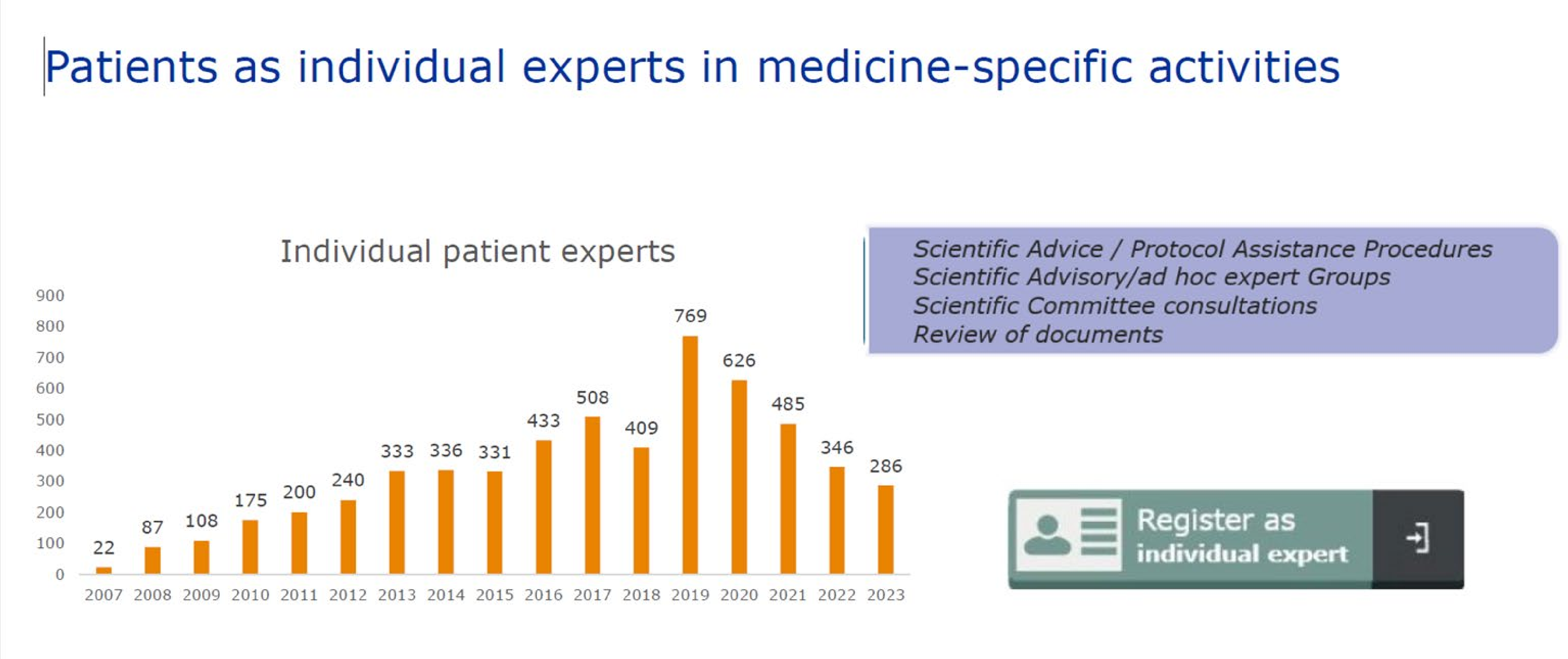
Heavy SAWP involvement with as series of SAs and PAs for more than 10 years

PDCO membership for 6 years

Patient input

HCP input

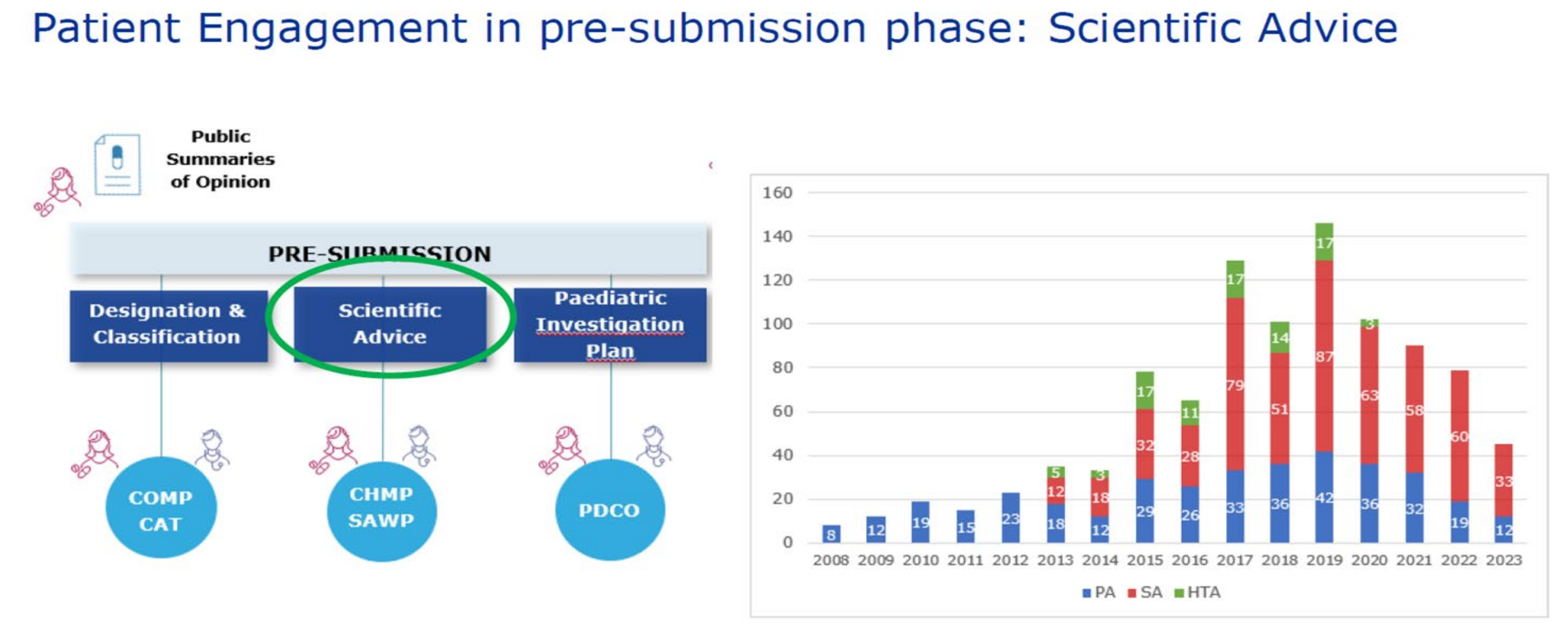
WHERE DO WE STAND TODAY?



Patients as individual experts in medicine-specific activities

Scientific Advice / Protocol Assistance Procedures
Scientific Advisory/ad hoc expert Groups
Scientific Committee consultations
Review of documents

WHERE DO WE STAND TODAY?



Patient Engagement in pre-submission phase: Scientific Advice

Public Summaries of Opinion

PRE-SUBMISSION

- **Designation & Classification**
  - COMP CAT
- **Scientific Advice**
  - CHMP SAWP
- **Paediatric Investigation Plan**
  - PDCO

WHAT THE FUTURE HOLDS?

EU Pharmaceutical Legislation

MEANINGFUL PATIENT INVOLVEMENT

JCA Regulation

Challenges of Patient Involvement and not only BEYOND Public and Stakeholder Engagement Department Efforts

a. Limited Representation & Diversity

- Underrepresentation of marginalized groups and rare disease patients

b. Knowledge & Expertise Gaps and Quality

- Limited understanding of regulatory processes and scientific concepts

c. Resource Constraints and Deterioration in the Quality of Engagement

- Insufficient funding and logistical support for patient involvement
- ***Timing and delays***

d. Conflicts of Interest

- Managing CoI in Small Populations and Rare Diseases

Challenges of Patient Involvement

e. Lack of Training & Support

- Absence of structured training programs for patient representatives + regulators?

f. Communication Barriers or/and lack of understanding?

- Overly technical language hindering effective communication + other factors

g. Inconsistent Engagement

- Limited involvement during early phases like trial design (early and often)?

h. Tokenistic Involvement

- Superficial engagement without genuine consideration of input

i. Mentality and Value

- Gate-keeper Mentality vs Enabler for HUNs with no real buy-in

Ways forward?

Early, Often & More Involvement

- Engaging more patients throughout all regulatory phases

Enhancing Training & Education

- Developing accessible training programs for better understanding

Broadening Representation

- Actively recruiting diverse patients and rare disease representatives.

RESOURCES AND MENTALITY CHANGE

Creating Patient Advisory Panels

- Establishing panels for structured, continuous input.

Transparent Meaningful Engagement

- Ensuring genuine consideration of patient input.

Improving Communication

- Providing plain-language summaries and simplifying technical language

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ORGANIZATION

Q&A and Discussion

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