Afternoon Workshop - Volunteer engagement across the medicine's lifecycle and healthcare

20'

- How can patient organisations engage with ERNs/EMA?
- What is the role of volunteers in the ERNs/EMA?
- How can you can follow the activities of the ERNs/EMA if you decide not to engage directly.
- Practical aspects (time commitment, skills reimbursement policies, etc.)

60'

- Your peers will reply to participants' questions on the role of volunteers, type of tasks, own experience, and anything else you'd like to know

30'

Coffee break

30'

- Round of unanswered questions

45'

- EURORDIS internal task forces

15'

- Wrap-up

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RARE DISEASES EUROPE

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Engaging with the EMA and ERNs: A Guide for Patient Representatives and Patient Organizations

23 May 2025

European Patient Advocacy Group

What are we covering in the next 20 minutes?

Application & Selection: Understanding eligibility and submission requirements for EMA and ERN engagement

Key Collaboration Partners: Stakeholders with whom you will engage

Volunteer Roles & Responsibilities: Different ways patients contribute and participate

Alternative Engagement Paths: Following activities when direct participation isn't possible

Practical Requirements: Time commitments, training, and support available

Metrics: Involvement and impact of patient representation in ERNs and EMA

Application & Selection Process ERNs

Identify Opportunities

Contact ERN Coordinating team, ePAG advocates or EURORDIS. They will let you know if the ERN is recruiting patient representatives and will inform you about the application process

Submit Application

Complete form with details of Patient Organisation & expertise/experience, motivation of person they would like to designate as an ePAG advocate.

Selection Process

Assessment by the ePAG based on the requirements established in the ERN Terms of Reference for the PO and the patient representative.
Final decision: ePAG or ePAG + Coordinating Team

Representation

ERN partners with the **patient organisation**. PO designates patient representative for disease/condition (European)

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EMA Engagement Framework

Patients representing their community	Patients representing their organisation	Patients as individual experts
• Management Board • Scientific Committee(s) • Emergency Task Force (ETF)	• Patients' and Consumers' Working Party (PCWP) • EMA consultations • Workshops	• Scientific advice / protocol assistance procedures • Scientific advisory / ad hoc expert groups • Scientific committee consultations • Review of documents

https://www.ema.europa.eu/system/files/documents/other/updated_engagement_framework_-_ema_and_patients_consumers_and_their_organisations_2022-en.pdf

Where are patients involved?

EARLY DIALOGUE IN MEDICINES DEVELOPMENT

Orphan designation

- COMP
- PUBLIC SUMMARIES OF ORPHAN DESIGNATION

Advanced therapy classification

- CAT

Paediatric investigation plan

- PDCO

Scientific advice

- SAWP

EVALUATION FOR AUTHORISATION

Marketing authorisation evaluation

- CHMP | CAT | PRAC
- PACKAGE LEAFLET (NEW)
- MEDICINE OVERVIEW

Orphan designation maintenance

- COMP

Scientific advisory/ad hoc expert groups

- CHMP | PRAC

SAFETY MONITORING OF MEDICINES

Post marketing procedures

- CHMP | CAT | PRAC
- PACKAGE LEAFLET (RENEWAL)
- SAFETY COMMUNICATIONS *

Scientific advisory/ad hoc expert groups

- CHMP | PRAC

Public hearings

- PRAC

* produced as needed

COMP: Committee for Orphan Medicinal Products
CAT: Committee for Advanced Therapies
PDCO: Paediatric Committee
CHMP: Committee for Human Medicinal Products
PRAC: Pharmacovigilance and Risk Assessment Committee
SAWP: Scientific Advice Working Party

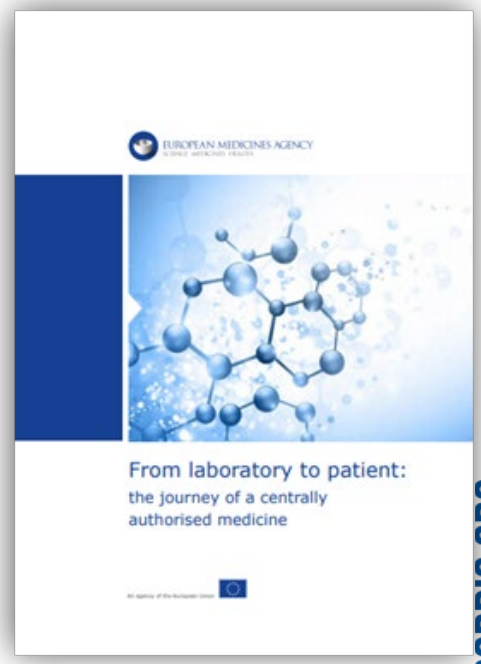
- COMMITTEES & EXPERTS MEETINGS
- DOCUMENTS FOR THE PUBLIC

What is Scientific Advice (SA) / Protocol assistance (PA)

The Scientific Advice Working Party (SAWP) answers specific questions from companies

Scientific advice:

- Requested to EMA at *any* stage of development
- Advice based on documentation provided by companies
- Together with regulators, experts, including patients, and companies
- Recommendations on development for marketing authorisation



Protocol assistance is for medicines with an orphan designation

Scientific advice aims to provide information to ensure that trials are efficient and performed to highest ethical and scientific standards required by regulators.

Identification & selection process
Scientific advice/Protocol Assistance

Identify Opportunities

EMA Scientific Advice Working Party contact EURORDIS

Patient involvement requests for protocol assistance procedures

Patient identification

EURORDIS membership and database (and beyond!)

EMA eligible organisations/**pool of patients (individual experts)**

Individual patients previously participated

Selection Process

Experience with disease as patient or carer

Free from conflict of interests

Ability to speak/read English

Level of experience with medicines development (may vary)

Representation

Individual representation

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Practical Requirements - ERNs

Time Commitment

4-10 hours monthly for meetings and preparation

Technical Requirements

Reliable internet and video conferencing capability

Skills Needed

Communication skills (EN)

Lived experience

and/or knowledge of a condition that falls within the scope of an ERN

Ability to champion the interests champion the diversity of views of the wider patient community relevant for each ERN, and not just of their own condition

- specific ERNs might require additional skills-

Reimbursement Policies

Travel & accommodation expenses to attend ERN meetings covered according to the ERN reimbursement rules.

Practical Requirements EMA

Time Commitment

4-10h/advice depending expertise and the questions

Technical Requirements

Completing the declaration of interests (no interests)

and signing confidentiality agreement

Skills Needed

Knowledge on drug development

English

Reimbursement Policies

Fix amount – 225 euros/advice + travel and accommodation (if f2f meeting)

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https://ec.europa.eu/info/funding-tenders/opportunities/portal/screen/opportunities/tender-details/docs/74ffa4e2-4bda-4465-bbfd-91bb8fe9f82c
CN/.Annex%203A%20Model%20Contract%20for%20Experts%20_Group%201%20MD%20Experts_V1.pdf

Role & Responsibilities - ERNs

2. Relay these insights to clinicians and researchers involved in the ERN to guide activities and strategic planning

3. Share ERNs information, updates and results with your community

1. Capture patient community needs

Be proactive and accountable

- Contribute to develop ePAG annual objectives and work programme
- Participate & contribute to ERN working groups & ERN annual meetings
- Participate in ePAG calls. Report regularly on the progress of the projects in which you are directly involved in the ERN

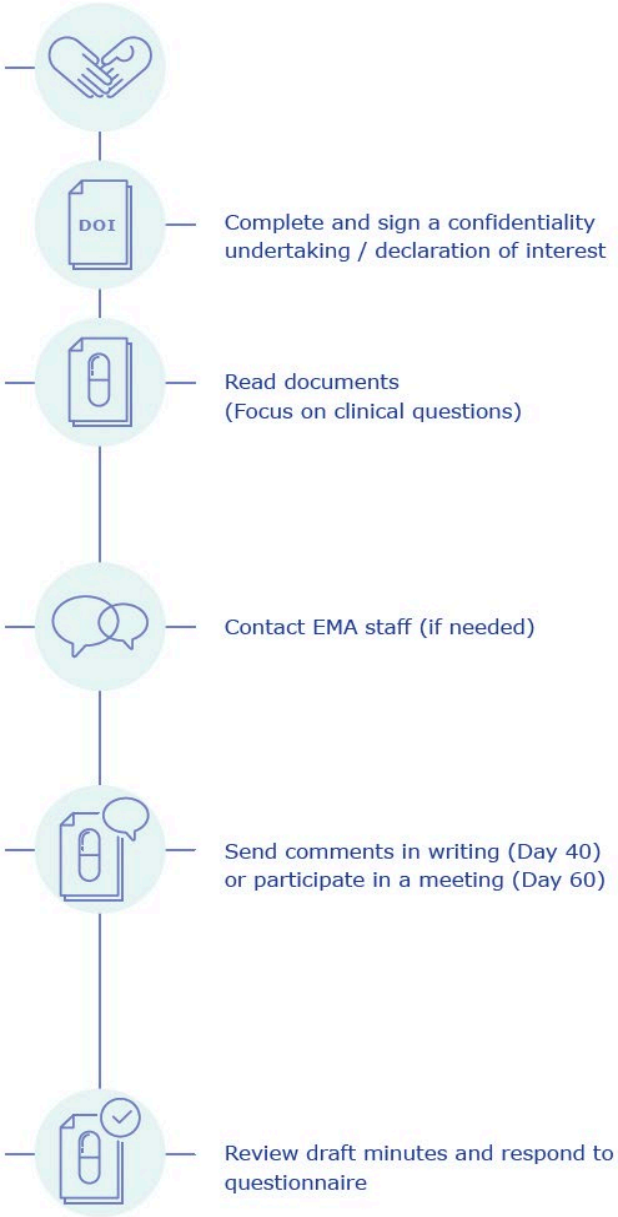
Have a collaborative team spirit

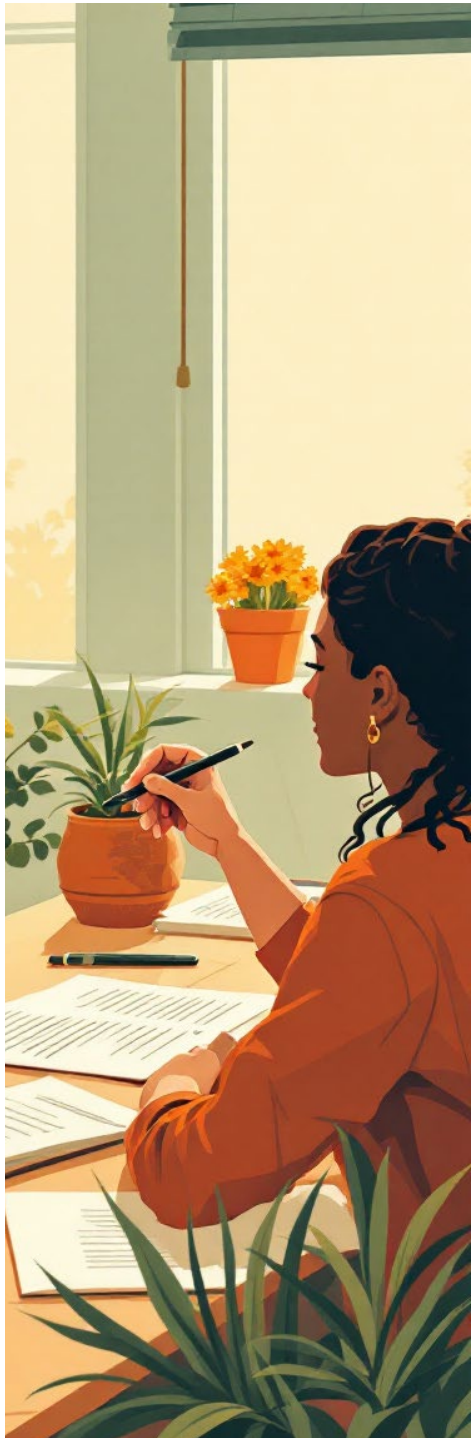
- Collaborate constructively with other patient representatives and clinicians

Be respectful and compliant

- Respect the confidential nature of the discussions
- Comply with the ERN CoI policy, ERN's ToRs and the ePAGs ToRs

Patients





Role & Responsibilities - EMA

- Patients are invited to share their **real-life perspective and experience in relation to a particular medicine in their disease area.**
- Offering a different perspective helping medicines developers and regulators to better understand **what is important to patients.**
- **Raising issues that had not previously been considered.**

- Selection of appropriate **end-points** (most important measures for this study)
- Defining target **population**: inclusion/exclusion **criteria**
- Standard of care and choice of the right **comparator**
- **Quality of life**
- Feasibility of the study: **duration**, treatment administration, **formulation** and **dosage**
- Identification and assessment of **risk** potential
- **Significant benefit** (added-value) over existing therapies
- **Ethical aspects**: Informed consent

Challenges - EMA & ERNs

EMA

- Finding suitable experts that meet requirements and have the time to volunteer (e.g. ultra rare diseases, language barriers)
- Ensuring comprehensive, tailored training to facilitate and enhance participation
- Provide a clear definition of role of experts in the different activities / committees to manage expectations from all angles
- Managing potential conflicts of interest
- Representativeness
- Measuring the value / impact of patient involvement at EMA

European Patient Advocacy Group

- Finding PO that can have a person who can dedicate time to volunteer at EU level (instead of focusing on local matters for example)
- Understanding role as partners of clinicians in the ERNs
- Representativeness and diversity (language barrier, volunteer role)
- Having a European network, the time and capacity to connect with them on ERN matters
- Ensuring support from ERN Coordinating teams & EURORDIS
- Level of maturity of patient partnership in the ERNs differs and this can lead to frustration.
- Maintaining the level of motivation of clinicians and patient representatives
- Measuring the value / impact of patient partnership

Key Collaboration Partners

EMA Collaborations

- EMA Public Engagement Department and EMA elegible patient organisations
- TAG – patients in other committees
- Scientific Committees & PCWP members

ERNs Partnerships

- Clinicians
- ePAG advocates
- Network Coordinator and Coordinating team

External Stakeholders

EMA

- Policy makers – DG Sante
- Regulators from National competent authorities (Member states)
- Healthcare professionals

ERNs

- Policy makers – DG Santé
- ERNs BoMs

Following EMA and ERNs activities if your PO is no directly involved

Connect with volunteers and representatives:
ERNs: Contacts per ERN (here) or reach out to pem-epags@eurordis.org)
EMA: public-engagement@ema.europa.eu

Subscribe to ERNs and EMA newsletters to receive regular updates in your email and follow social media accounts

Attend ERNs or EMA live webinars or listen to recordings

European Public Assessment Reports (EPAR) EMA.
Access published medicine evaluations

Browse EMA & ERNs websites for information on activities

Access published medicine evaluations and training opportunities

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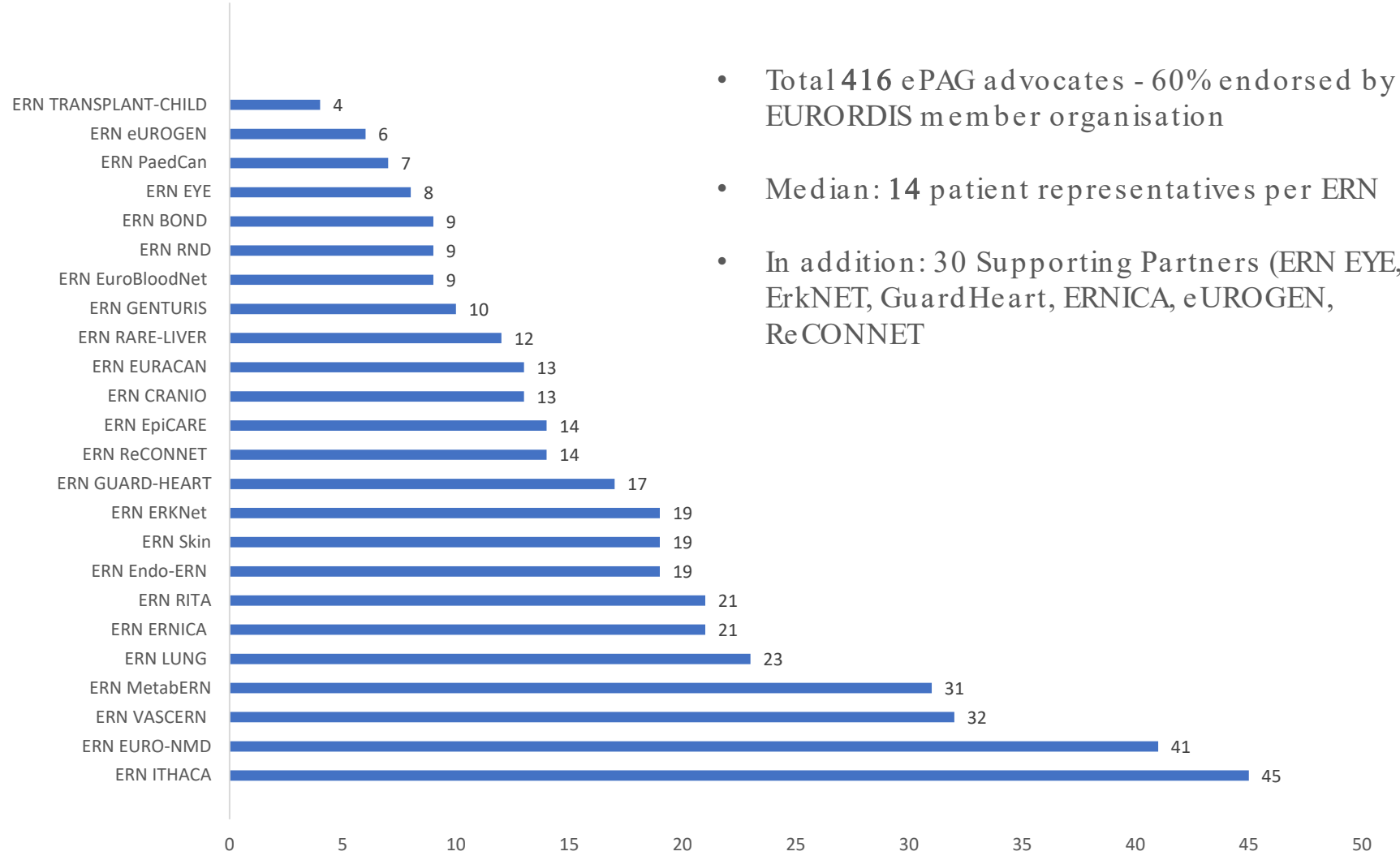
Any Questions so far?

Number of patient representatives per ERN



- Total **416** ePAG advocates - 60% endorsed by a EURORDIS member organisation
- Median: **14** patient representatives per ERN
- In addition: 30 Supporting Partners (ERN EYE, ErkNET, GuardHeart, ERNICA, eUROGEN, ReCONNET

Note: Data for MetabERN, RITA, NMD, EpiCARE, Trasnplantchild, is updated on an annual basis. The rest is updated on a regular basis

Impact of patient involvement in the ERNs

- "Roughly 80% of the ERNs demonstrate a **firm dedication** to involving patient representatives, but barriers remain" (5-Year ERN Evaluation report, 2024)

- There are no public metrics on the **impact** of patient involvement in the ERNs

- Good practices on patient partnership in different areas are available in the Patient Partnership Hub (formats: webinars, factsheets, podcasts)

PATIENT PARTNERSHIP HUB

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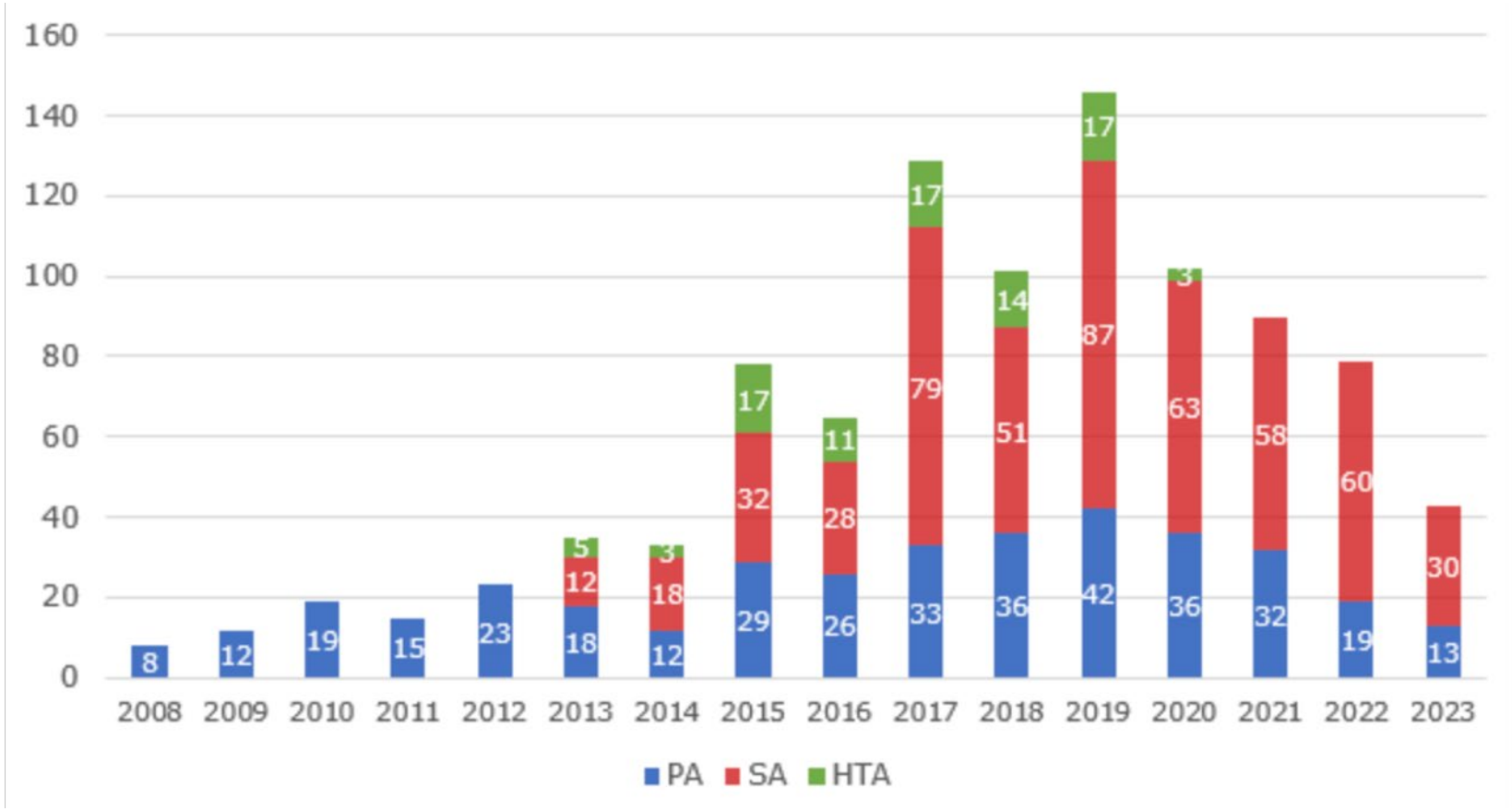
EMA patient involvement

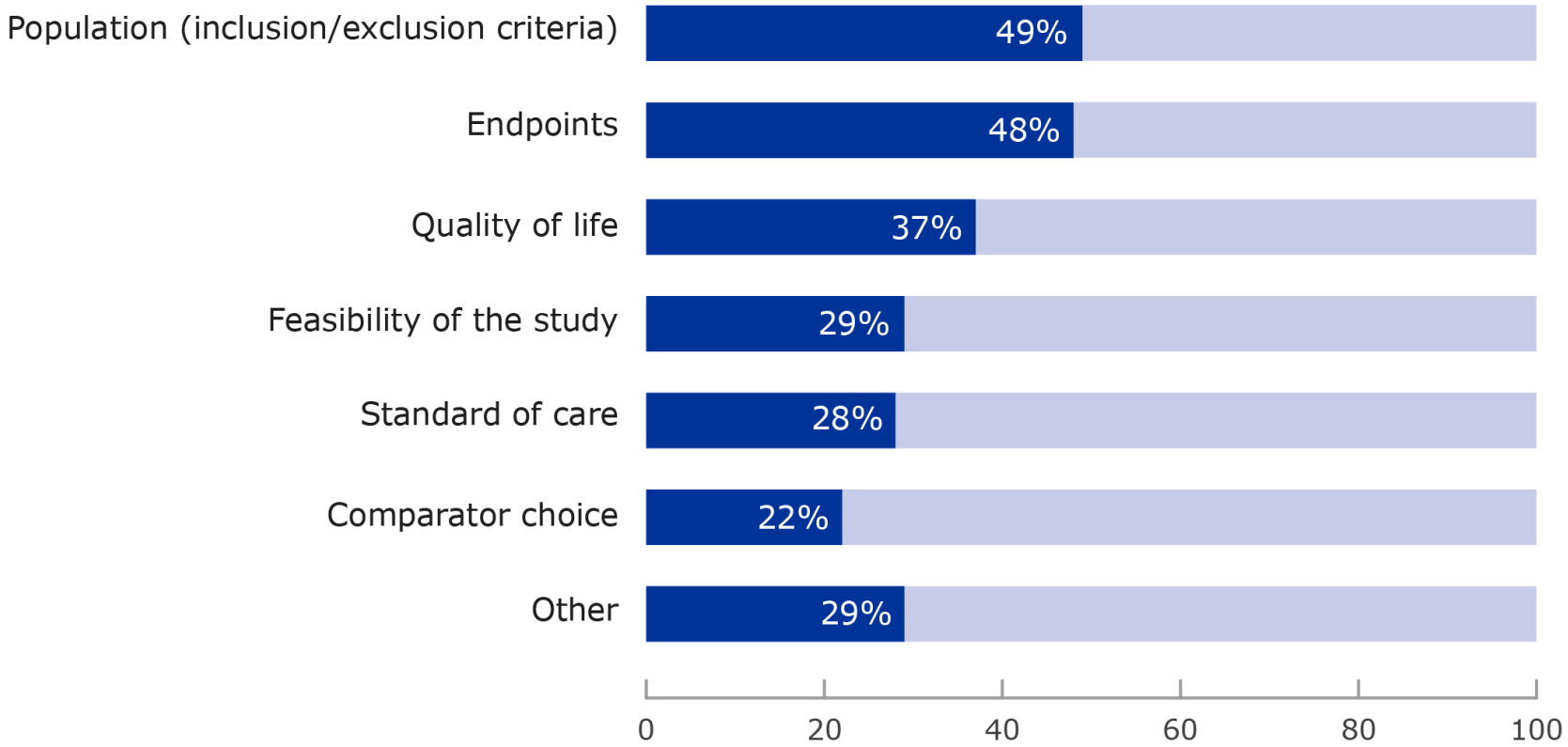


PA - Protocol Assistance

SA - Scientific Advice

HTA – Health Technology Assessment

Where patients gave input



"Other" areas included general insights into the condition, its daily impact and treatment options.

Impact of patient involvement

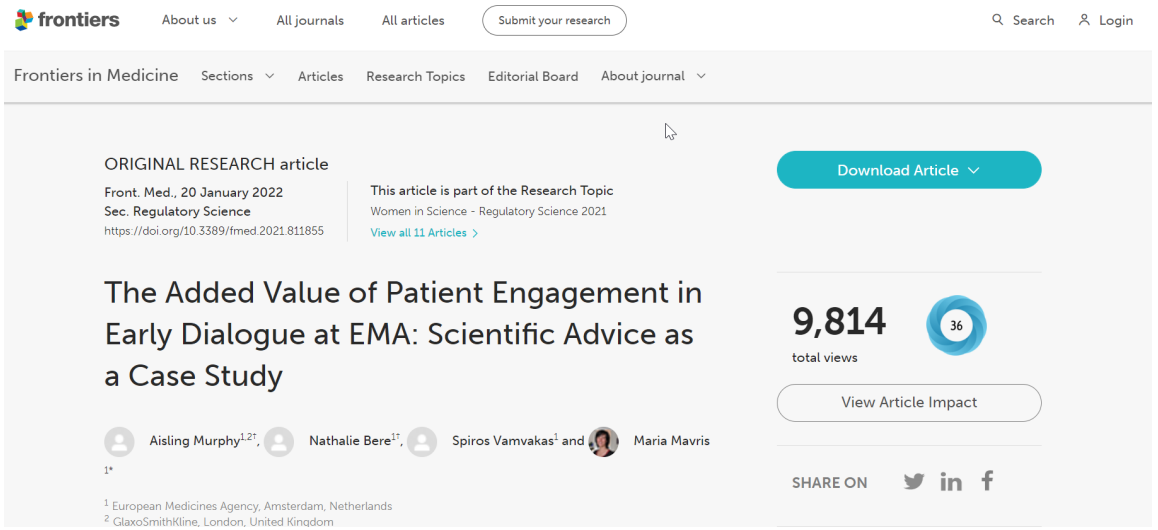
Patient input resulted in modification of advice to company — **20%**

Patient input resulted in further reflection by coordinators —**53%**

'On behalf of EMA and the coordinators, I would like to thank you again for your testimony, which is very moving. This ***gives us*** *additional* ***reassurance*** *that* ***treatment of XX is a high unmet medical need****, and* ***urges us to make the drug available*** *as soon as possible to patients in Europe. The final advice letter reflects this spirit'*

Impact of patient involvement at EMA

- The **added value** of patient input is **not exclusive to scientific advice**
- Patients are involved in other regulatory procedures such as scientific advisory groups and in consultations by EMA committees.
- Demonstrated value of patient inclusion in scientific advice not only supports EMA's continued inclusion of the patient voice throughout the medicine's lifecycle and the diversification of activities where patients participate, but also provides **evidence of impactful importance** of engaging with patients.



The Added Value of Patient Engagement in Early Dialogue at EMA: Scientific Advice as a Case Study
https://www.frontiersin.org/articles/10.3389/fmed.2021.811855/full

Next Steps: Interactive Session

Join a Discussion Table

Connect with volunteer at your assigned table

First 30-Minute Session

Discuss either EMA or ERN experiences

Rotate Tables

Move to new table when prompted

Second 30-Minute Session

Explore the alternative topic (EMA/ERN)

EURORDIS internal taskforces