Enhancing patient involvement in the regulatory area through training and education – a dialogue about emerging needs and challenges
24th March 2023, 09:00-10:10
DIA2023 Open Roundtable Session
Organised jointly by EMA and EUPATI
Session report

Session summary

Session chairs
- Juan Garcia Burgos (EMA)
- David Haerry (EATG)

Speakers
- Giulia Gabrielli, EMA (online)
- Christa Wirthumer-Hoche, AGES, Austria
- Gabriela Zenhäusern, Swissmedic, Switzerland
- Dimitrios Athanasiou, World Duchenne Organisation, Greece (online)
- Larisa Aragon Castro, EUPATI Switzerland

Introduction by Session Chairs

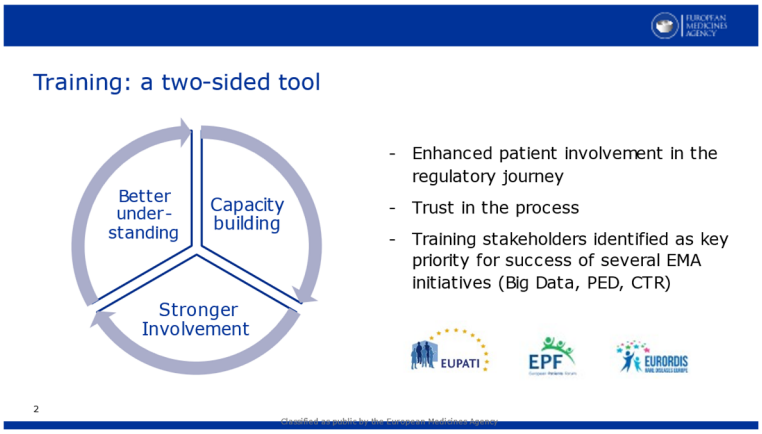
Juan Garcia Burgos (EMA) opened the session by introducing himself and the topic of patient education as an important element in empowering patients to contribute effectively to patient engagement initiatives. It is essential to ensure that patients have the necessary support and resources to engage in a complex environment meaningfully, without feeling intimidated. Juan reiterated the

objective of the session which was to discuss the existing efforts in patient training, identify gaps, and explore opportunities for improvement.

Juan introduced the Co-Chair of the session, **David Haerry (EATG)**, who also emphasized the importance of patient involvement in the regulatory process and highlighted the benefits of patient training.

Patients' and regulators' perspective

Giulia Gabrielli, EMA, discussed the European Medicines Agency's activities and initiatives related to patient education.

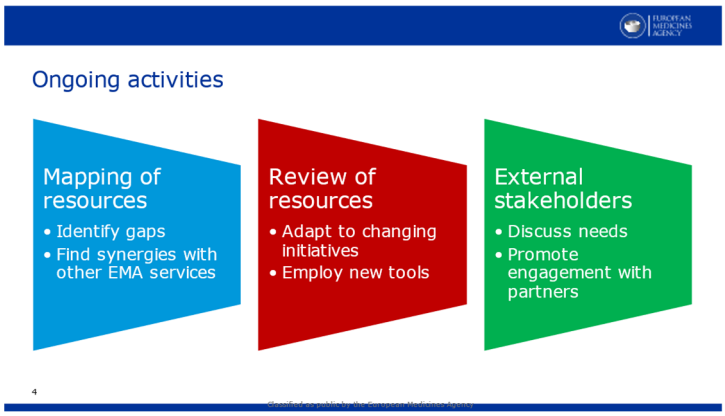


Training plays a crucial role in building capacity and empowering patients to be actively involved in the regulatory process. When patients are more involved and understand the process, it leads to a more consistent and improved regulatory output. Trust between regulators and patients is a valuable currency in building effective relationships. Patients who feel more involved become advocates within their communities, leading to a virtuous cycle of increased engagement.

Training is increasingly recognized as a fundamental ingredient for the success of activities at EMA. It is becoming an integral part of the structural documents and workplans guiding key initiatives, such as theand discussions around the use of patient experience data.

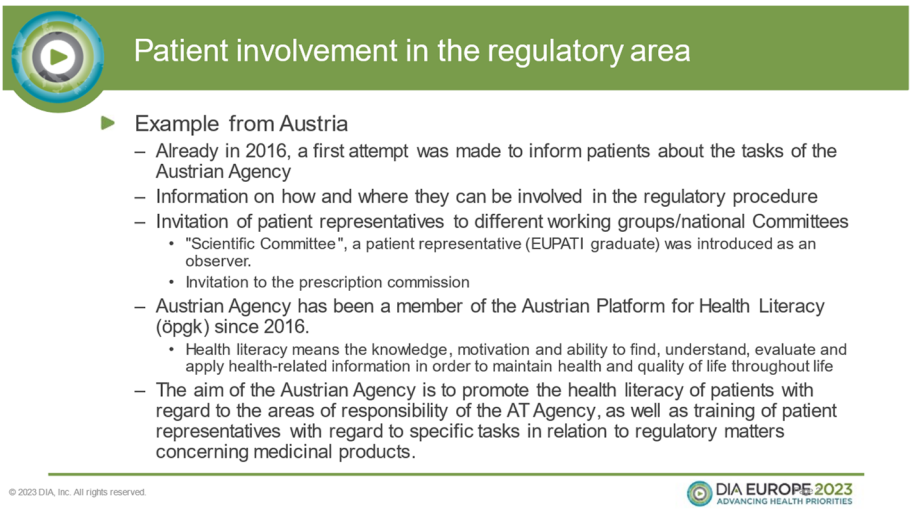
EMA focuses on two main themes for patient training: patient involvement in the regulatory lifecycle of medicine and SMAs (specific medical areas) initiatives. The training tools include '*EMA basics*' videos covering various topics related to the agency's role, available on their website and regularly shared with patients. Annual interaction sessions provide an opportunity for patients interested in EMA work to engage and learn. Webinars address more complex themes and are accessible virtually, with recordings shared online for wider access.

Training is an ongoing process that involves refining tools, improving materials, and exploring new ways to disseminate information. EMA is conducting a mapping of resources to identify gaps and synergies with other services. They are also adapting to changing regulatory environments and utilizing new digital tools to ensure the information is fit for purpose.



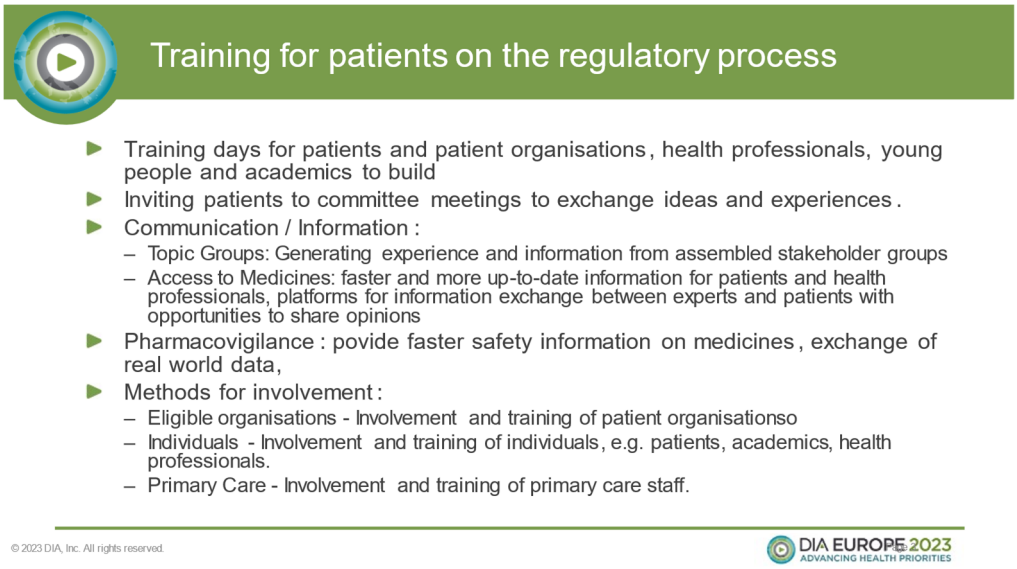
Recognizing the common needs and shared goals, EMA actively participates in discussions with stakeholders and promotes engagement. We understand the importance of working together and believe in fostering collaboration to maximize the benefits of patient training at both the European and national levels.

Christa Wirthumer-Hoche, AGES, described the patient engagement efforts in Austria. In 2016, a meeting was organized to inform patients about the regulatory authorization process. However, there was a larger representation of industry participants than patients, leading to some disappointment. Subsequently, additional discussions were held with patient representatives to explore their involvement in regulatory processes and committee rules.



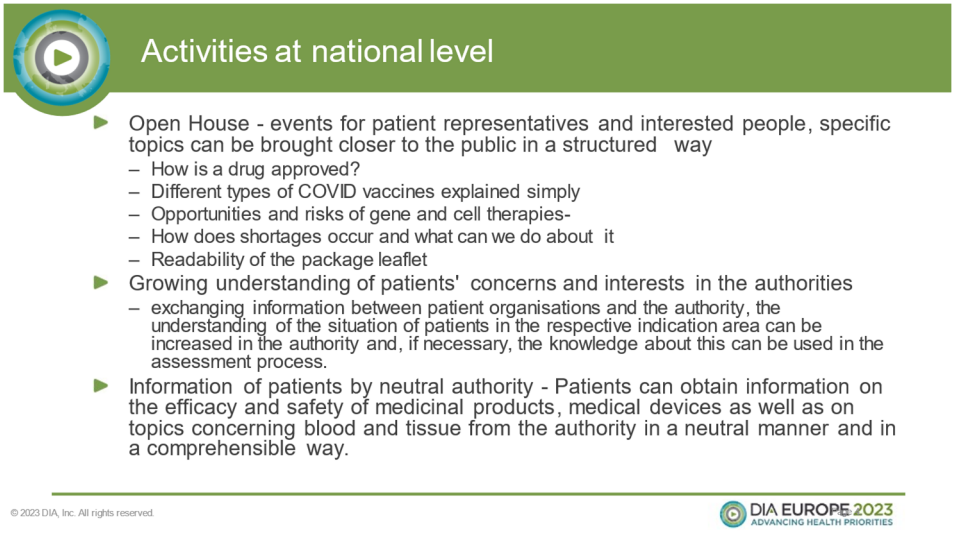
Austria has approximately 1,700 patient groups representing about 250,000 patients, with half of them related to rare diseases. Patient groups associated with rare diseases are generally well-organized, while others often lack structure, making it challenging to connect with them.

Efforts were made to encourage patient representatives to participate as observers in scientific committees responsible for deciding whether a medicinal product should be prescription-only. However, finding willing participants has been a challenge, and issues regarding the declaration of interest have arisen.



The agency participated in the Austria platform for health literacy, recognizing the importance of promoting health literacy in relation to regulatory measures. Training days and information sessions were planned, but low participation led to cancellations.

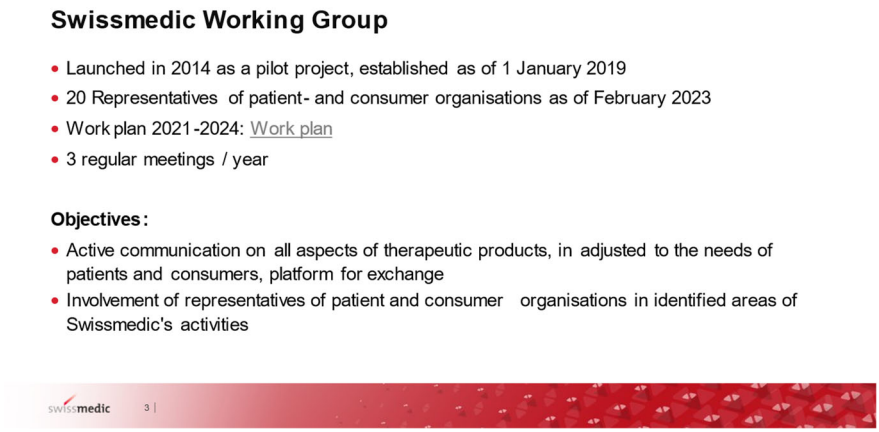
The agency seeks input from patients regarding the information they would like to see on the national website. Topics include pharmacovigilance, ensuring the safety of medicinal products, and publishing signals discussed at the European level to inform patients. Different methods of involvement were discussed, such as involving patient representatives in discussions on general or specific items.



At the national level, an open house event is being planned to encourage dialogue and understanding between patients and the agency. Topics to be discussed include the principle marketing authorization process, information about COVID-19 vaccines, and gene therapy products.

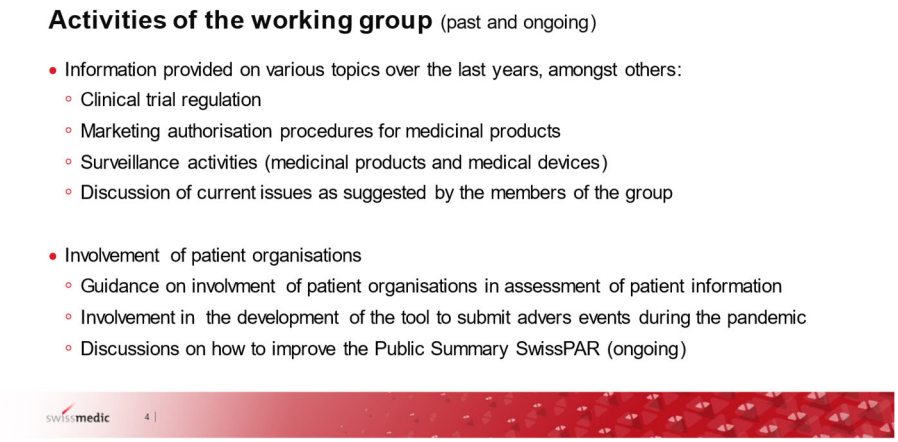
The agency recognizes its role as a neutral platform and emphasizes the importance of providing information. Discussions with patients can help explain the difference between information and training and clarify the responsibilities of national competent authorities.

Gabriela Zenhäusern, Swissmedic, summarised the patient involvement in Switzerland and the collaboration with patient organizations over the past 10 years.



Currently, there are around 20 patient and consumer organizations involved in activities and discussions related to patient engagement in regulatory processes.

The Swissmedic Working Group aims to actively inform patient organizations and involve them in procedures and decision-making. Swissmedic provides information about their procedures to patient organizations, especially during the COVID-19 pandemic. They aim to keep patient organizations up to date and facilitate interactive discussions.



Regular meetings are held to discuss important topics such as including patient representatives in expert committees. The focus is on education, training, effective communication, and understanding the different levels and types of patient involvement.

Several projects have been implemented, including collaboration with patient organizations to gather feedback on specific products or programs before authorization. Additionally, a portal for patients to report adverse events was established with input from patient organizations. Efforts are being made to include patient input in public summary assessment reports.

Swissmedic works closely with other entities such as the federal office of public health and international partner agencies to exchange knowledge and enhance

patient engagement. Transparency and publication of information are priorities for the organization.

References

- Collaboration with patient and consumer organisations (swissmedic.ch)
- Work plan Swissmedic working group with patient and consumer organisations 2021–2024
- Involving patients and consumers in Swissmedic's regulatory processes: From information sharing to participation. In: SCTO Platforms. Regulatory Affairs Watch 6 – PPI. 28 October 2021, doi: https://doi.org/10.54920/SCTO.2021.RAWatch.6.19 .

Dimitrios Athanasiou, World Duchenne Organisation, Greece, discussed the needs for education and training from the patient perspective. There is a need for education and training in patient involvement, including scientific literacy and common language understanding. It is essential to ensure meaningful, impactful, and measurable patient involvement.

There is a lack of common understanding of what patient involvement entails. Clarifying definitions and roles, such as distinguishing between lay patients and patient experts, is important for effective involvement.

Dimitrios emphasised the importance of representativeness. Representing diverse communities and involving patient experts who bring valuable experiences is crucial. Different types of involvement, such as protocol advice and adverse event reporting, require specific considerations.

Cultural perceptions and the role of agencies can influence patient involvement. Building mutual trust, respect, and openness are essential. Overcoming financial burdens and resource limitations is necessary to support patient education and involvement.

Finally, tackling legal and regulatory restrictions is important to enable effective patient involvement. Managing and protecting the system while inspiring trust are key considerations.

Larisa Aragon Castro, EUPATI Switzerland, summarised her views as a patient representative.

Patient education should go beyond simple knowledge transfer and include a holistic approach that addresses health literacy, self-management skills, communication skills, advocacy skills, and critical thinking skills.

A significant percentage of Europeans have a limited understanding of their own health situation, highlighting the importance of prioritizing health literacy to empower patients in making informed decisions.

Patients need to develop effective communication skills to interact with scientific researchers, evaluation panel members, and other stakeholders in the healthcare system.

Patients should be equipped with advocacy skills to advocate for themselves and others. This includes navigating through the regulatory life cycle of medicine products, understanding patient rights and responsibilities, and speaking up when their needs are not being met.

Developing critical thinking skills enables patients to critically assess and improve the current healthcare system.

Building credibility and trust in the pharmaceutical industry and regulatory agencies is essential. Transparency and continuous evaluation of patient engagement efforts are necessary for further improvement.

Making patient engagement a priority involves constructing a comprehensive onboarding process that ensures patients possess the necessary skills to be seen as equal partners in the decision-making process.

Panel discussion and Questions from the audience

Challenges in Patient Training and Education
The panelists shared their experiences and identified gaps and challenges in patient training and education. They emphasized the need to involve patients early on and empower them in the healthcare transformation process. They also highlighted the importance of patient literacy and the need for resources and strategies to ensure effective training.

Streamlining Initiatives and Collaboration
The panelists discussed the potential benefits of establishing patient engagement groups to streamline initiatives between different agencies. They emphasized the importance of collaboration, sharing best practices, and creating a European overview of patient engagement efforts.

Reaching Patients
The panelists explored strategies to reach patients who may not actively seek interaction or engagement. They mentioned the potential of utilizing social media platforms and exploring new channels to ensure inclusivity and diversity in patient engagement.

Conflict of Interest and Training
The panelists addressed the issue of conflicts of interest in patient training and emphasized the need for neutrality and objectivity in training activities. They discussed the importance of managing conflicts of interest effectively and the impact it can have on patient engagement.

Progress and Recommendations
When asked about their desired progress in patient education within a year, the panelists expressed various aspirations. These included having patient representatives involved in expert committees, identifying committed patient

collaborators, gathering data on the value of patient engagement, and advocating for resources and financial support. They also emphasized the need for a commitment to training in action plans and recognizing it as a key pillar of patient engagement.

Concluding remarks

Overall, the session shed light on the importance of patient involvement, challenges in patient training, collaboration among agencies, reaching a wider patient population, managing conflicts of interest, and the desired progress in patient education.

ANNEX

Session description

The importance of greater **patient involvement** in medicines research and development (R&D), as well as in the evaluation and authorisation of new medicines, and the monitoring of their safety, is commonly acknowledged and benefits all involved parties. Patients are directly affected by decisions taken by the regulatory authorities. They also possess unique experience of living with a condition and therefore are in a unique position to inform regulatory processes about the potential positive or negative effects of new and existing health technologies. They can also give substantial input into how adequate and clear information about medicines is in terms of content, format and language.

Training provides patients' with the opportunity to enhance their **knowledge, skills and competencies** throughout the different stages of the medicine lifecycle, e.g. marketing authorization procedures, benefit-risk assessment, pharmacovigilance, pharmacoepidemiology and other relevant aspects, and gain a better understanding of their role in these processes.

Today, the European Medicines Agency (EMA) and the National Competent Authorities increasingly involve patients in their work and also provide information and support for the patient community[1]. Patient organisations, and organisations like the European Patients' Academy on Therapeutic Innovation (EUPATI) also provide training for patients on the regulatory processes.

In order to be effective and beneficial for all stakeholders, a closer look at the current level of patient involvement in the regulatory area, the related **needs for education & training as well as eventual gaps** is needed. It is important that the different actors work together to ensure a joint approach.

Session objectives:

- To open a multi-stakeholder conversation around patient education in the regulatory area
- Summarize what type of capacity building is already available for patients
- Identify needs and gaps and identify opportunities for improvement
- Summarize recommendations for fostering patient education and continued collaboration across the different stakeholders

[1] "To optimise their contribution, EMA provides patients with a training programme to help them understand the Agency's mandate as well as their expected role in the medicine's regulatory process. The training provided is tailored and based on the particular activity where the patient will participate and is complemented by personalised and one-to-one support" (Engagement Framework: EMA and patients, consumers and their organisations, January 2022, p.7).

Speaker bios

Giulia Gabrielli works with patient engagement at the European Medicine Agency (EMA) within the Public and Stakeholder Engagement Department. Earlier at EMA, she has also been involved in medical writing and public outreach. A trained biologist and environmental modeller at Imperial College London, she previously worked for the European Research Council (ERC) in scientific communication. Her work involves daily interactions with patients as they take part in Agency activities, but also monitoring and enhancing the scope of their involvement within the regulatory process.

DI Dr. Christa Wirthumer-Hoche studied biochemistry at the Technical University in Vienna and did her doctoral thesis at the Institute of Medical Physiology in 1983. First as quality assessor at the Austrian Institute for Drug Control (1983 - 1998), from 1998 at the Federal Ministry of Health she was Head of the Marketing Authorisation Department for Medicinal Products. Since the founding of the new Austrian agency on 1 January 2006, she has been Head of the Institute for Market Authorisation and Lifecycle Management of Medicinal Products. Since October 2013, she has been Head of the Austrian Agency for Medicinal Products and Medical Devices at AGES (Austrian Agency for Health & Food Safety).

Dr Gabriela Zenhäusern, Deputy Head Stakeholder Engagement, Swissmedic,
is a pharmacist by training and holds a PhD in Biomedical Research from the Department of Biomedicine in Basel, Switzerland. Gabriela used to work in the sector authorization at Swissmedic, Switzerland from 2010 to 2015 before joining the regulatory systems strengthening team (RSS) at the World Health Organization (WHO) from 2015 to 2019. She joined the Stakeholder Engagement Team in 2019. In her current position, Gabriela is leading the Swissmedic working group with patient and consumer organisations and is being responsible for the coordination of international collaboration.

Dimitrios Athanasiou holds a BA in Business Administration and an MBA in Financial Management and has more than 25 years' experience with international business projects, working in various countries in consulting, developing and reorganizing companies. When his son was diagnosed with Duchenne Muscular Dystrophy, a fatal and incurable rare disease, he become a strong international patient advocate in Duchenne and Rare Diseases. He is a graduate of the EURORDIS Summer School and a EUPATI Fellow, and a Board member of the World Duchenne Organization (WDO) and the European Patient Forum EPF. Since 2017 he serves the Rare Disease Community as PDCO member in European Medicines Agency representing EURORDIS

Larisa Aragon Castro is a patient advocate who is passionate about empowering individuals to better understand medical information. As the co-founder of SumMed.org, she works tirelessly to improve access to medical information for patients and caregivers. Larisa is also a caregiver and cancer survivor, which gives her a unique perspective on the challenges that patients and their families face.
In addition to her work with SumMed, Larisa is a Patient and Public Expert in the Swiss National Science Foundation and an executive board member of EUPATI Switzerland. She is committed to improving the quality of patient care and advocating for the rights of patients to access accurate medical information.

Through her advocacy work, Larisa hopes to empower everyone to take an active role in their healthcare and make informed decisions about their health.