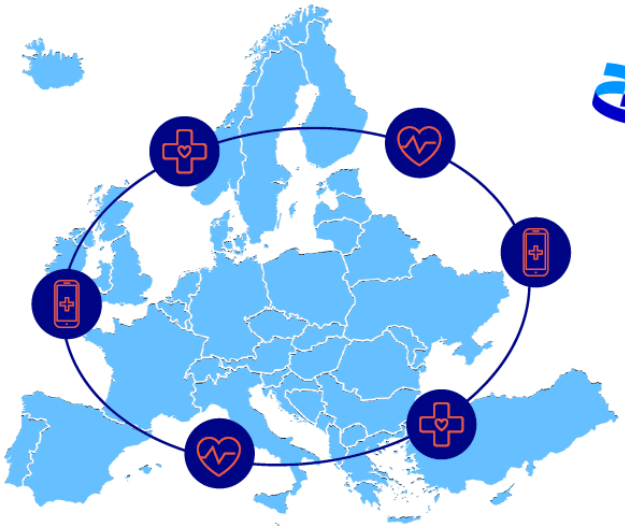
Involving Patients in the Early Stages of HTA

European Patient Advocacy Summit 2021

October 6, 13 and 20
2.30 pm – 5.00 pm (C.E.S.T)
8.30 am – 11.00 am (E.T)



Pfizer

Pfizer Health Technology Assessment (HTA) Advocacy Summit
'Involving Patients in the Early Stages of HTA'

MEETING REPORT
November 2021

PP-PCP-DNK-0002 (January 2022)

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1. Executive summary

- Following the success of Pfizer's 2019 and 2020 Patient Advocacy Summits, Pfizer delivered a further **capability strengthening workshop in 2021** to continue to develop the technical capacity of patient advocacy groups (PAGs) and strengthen relationships between Pfizer and PAGs in Europe. 2021's Advocacy Summit focused on both patient involvement in Health Technology Assessment (HTA) and healthcare policy decision-making more broadly, to ensure continuity of content from previously held Summits.
    - Across the three Advocacy Summits held to-date, 104 PAGs from 20 European countries have received Pfizer's tailored capability training.
    - 19 of these groups have attended two or more of the three Summits.
- **87 patient advocacy group representatives from 14 different countries** registered to take part in three, WebEx based, 2.5-hour sessions from 14:30 – 17:00 CET on Wednesday 06, 13 and 20 of October. **With 59 attendees across the sessions, the conversion rate between registrations and participants was high**. Pfizer representatives from each of the following countries were present (Czech Republic, Denmark, Italy, Greece, Poland, Portugal, Romania, Spain, USA). This year's Summit again took place via a virtual platform rather than face to face to incorporate social distancing requirements related to the ongoing coronavirus pandemic. Given the amount of and technically 'dense' nature of the content, as well as the majority of participants being non-native English speaking, the Summit was delivered across three smaller sessions.
- Capability building sessions were delivered across three sessions with the following themes: 'Why is the patient voice critical to the evolution of healthcare decision-making in Europe?'; 'The European Union (EU)-wide HTA process and how will it be implemented locally?'; 'Providing impactful contributions to healthcare decision-making processes.'
- The sessions were opened by Gary Surmay (Senior Director Corporate Affairs, Internal Medicine, Pfizer), who set the tone by noting the tailored training the sessions would deliver and thanked advocates for attending in order to further build their expertise in the field of advocacy. High-level speakers from organizations representing patients

from across Europe participated: the European Brain Council (EBC), Plataforma de Organizaciones de Pacientes (POP), the European Patients Forum (EPF), the European Coalition for People Living with Obesity (ECPO), the European Federation of Neurological Associations (EFNA) and Pain Alliance Europe (PAE). A representative from EUnetHTA, an organization established to create an effective and sustainable network for HTA across Europe, presented, as well as two leading health policy stakeholders from the EU institutions: Member of European Parliament Tomislav Sokol (Croatia, European People's Party) and Flora Giorgio, Team leader on HTA at the European Commission. Flora specifically commented that the Commission was strongly in favor of Pfizer's training initiative and that it hoped to organize a similar initiative as soon as the EU-wide Regulation is adopted.

- Opportunities for patient advocacy group (PAG) engagement were presented throughout with interactive sessions driven by the online platform *Mentimeter.* The *Chat* function within Webex events was enabled to allow commentary from participants throughout.
- **Participants came from the following 14 European countries: Belgium, Denmark, Finland, France, Greece, Ireland, Italy, North Macedonia, the Netherlands, Poland, Portugal, Romania, Russia and Spain.**

Why is this training program important?

- **Patient involvement in both HTA and healthcare policy decision-making is critical to improve accessibility** to new medicinal products. There is an increasing necessity to effectively demonstrate product value through stakeholder relations and environment shaping activities prior to launch. **Creating space within health budgets for innovative therapies continues to be challenging**, as healthcare budgets are squeezed in a post-pandemic environment and disease areas prioritized by policymakers. The availability of treatment varies dramatically in Europe. On average patients in Europe wait 525 days after a European Medicine's Agency (EMA) marketing authorization before getting access to new treatment. Out of the 172 medicines approved by the EMA between 2015 and 2018, Member States provided their populations with 85 on average (less than 50%).
- **Public and patient involvement in healthcare policy decision-making and HTA is considered a priority by EU healthcare decision-making bodies**, including EMA. Patients are widely recognized as the only stakeholders who have comprehensive knowledge of the direct impact of a treatment or technology on their condition. The EMA states that patient involvement improves transparency and trust and brings the everyday aspects of living with a disease into scientific discussions, bridging the gap between clinical trials and real-world data.
- **However, the scope of patient involvement remains limited in both scope and impact**.
    - **EMA engages with a small network of 35 eligible PAGs throughout the medicine's lifecycle, and groups must be called upon in order to present input**. The impact of the patient voice in EMA's processes has been questioned by leading patient organizations including the EPF.
    - Out of 23 European countries that have an HTA system for the assessment of pharmaceuticals, only nine countries indicate involving patients at the step of advice and decision-making: Estonia, France, Italy, Lithuania, Malta, Netherlands, Poland, Sweden and the UK. **The type and level of patient involvement varies widely, which reflects the different rationale, motivation and approach applied in each country**. Very few HTA agencies and decision-making bodies currently involve and integrate patients' perspectives in their reports and conduct formal evaluation of the impact of patient involvement in HTA.
- **European PAGs have called for the need to guarantee adequate involvement of patients in all HTA activities** (joint consultations, early dialogues, scoping and assessments). The European Organization for Rare Diseases (EURORDIS) launched a statement during the development of this legislation, **calling on EU institutions to adopt measures to fully incorporate meaningful involvement**. This was signed by a range of patient organizations including EPF and EFNA.
    - 'Adequate involvement' is understood as **participation in discussions taking place prior to and during HTA assessments of a new technology**, for example face-to-face meetings (joint consultations), focus groups with assessors for the scoping phase, telephone interviews with assessors, and expert or citizen panels.

- The statement also called for more research to **determine which methods to use** to involve patients in the EU-wide HTA.

- **The upcoming EU-wide HTA legislation**, predicted to be effective as of Q1 2025 (three years after adoption in January 2022), **represents an evolved HTA landscape in Europe and it is essential for PAGs to be equipped** to participate, so as to remain as efficient and visible as possible.
  - The Regulation establishes **rules for joint work between Member States on common scientific and clinical aspects of HTA, to be driven by national HTA bodies**. Key principles for this collaboration are high quality, timeliness and transparency. **Member States remain responsible** for drawing **conclusions on added value to healthcare systems** and taking **decisions on pricing and reimbursement**.
  - **Patient involvement is embedded** within the Regulation. Patients shall **contribute individually as external experts**, alongside clinical and other relevant experts, and provide input based on therapeutic area expertise during Joint Clinical Assessments and Joint Scientific Consultations. Patients will also be consulted on behalf of their **wider stakeholder organization** to provide input on horizontal and strategic issues.
  - **Whilst the EU-wide HTA provides provisions for patient involvement, this process will not be open to all patient representatives**. Patient representatives, if deemed as experts, will only be called upon to take part in Joint Clinical Assessments and Joint Scientific Consultations. Patient involvement and stakeholder engagement is subject to a 'top-down' approach.
- The training was also developed as a **direct response to in-country Corporate Affairs teams who counselled that patient advocacy organizations could benefit from being better equipped** to take part in national HTA processes.

Key learnings

1. **European health policy decision-making is not accessible enough to patient representatives. There are specific structures for patient involvement, in both the EMA and at varying levels across national HTAs, but they are limited in both scope and impact.** Specifically:
   - Patient engagement in EMA is limited to 35 eligible PAGs, and representatives must be called upon to provide input.
   - Patient advocates do not feel as though they receive enough feedback from European policy decision-making bodies on both the value of their input, and how their input is used in policy outcomes. This impacts their ability to participate effectively.
   - Due to a lack of plain language materials, policymaking bodies are not providing enough accessible opportunities for patient advocates to be involved.
2. **Whilst the implementation of an EU-wide HTA looks to shift the health policy landscape, and provide new opportunities for patient involvement, this process will not be as accessible as European PAGs had hoped. The final details for stakeholder engagement will be published in 2022.**
   - Patient involvement and stakeholder engagement in the EU-wide HTA will be subject to a 'top-down' approach. Patient representatives will only be called upon to take part in Joint Clinical Assessments and Joint Scientific Consultations, and only if deemed as 'experts'. Patients will not be included in the general governance structures or have voting rights. It is likely that the approach of the EU-wide HTA to patient involvement will mirror that of EMA.
   - The European Commission will publish implementing acts setting out the rules for patient involvement in the EU-wide HTA process in early 2022. **It is crucial that PAGs are kept up to date on these texts and their implications as they will be decisive for PAGs planning HTA engagement strategies**.
3. **Patient advocates recognize the value and importance of their voice in healthcare decision-making processes; however, they lack both the tools and opportunities to participate**. **Pfizer can support by continuing to deliver high-level capability training programs which guide PAGs on how to approach healthcare policy decision-making bodies and empower them to effectively communicate towards them.**

- Participants of this year's HTA Advocacy Summit told us that there were not enough training opportunities available to them to ensure their full participation in healthcare policy decision-making. Participants stated that more regular trainings by healthcare policy and communications specialists at both a national and EU-level are needed. In addition to more training on HTA and patient involvement in HTA, further ideas for training topics included: social media, communications and campaigning skills, presentation skills and how to present relevant data to HTA bodies.

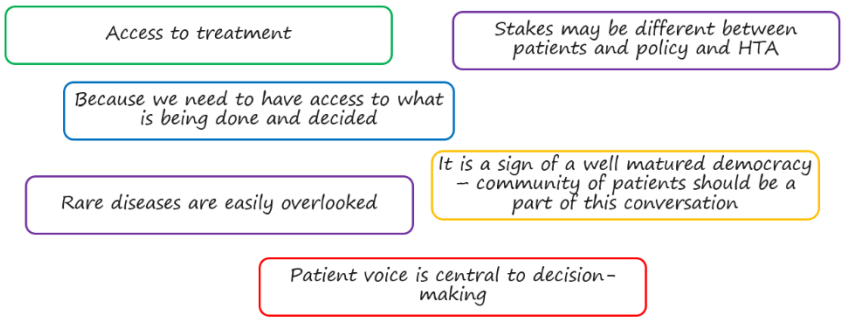
2. Interactive Sessions

To understand more about participant perspectives on patient involvement in both healthcare policy decision-making and health technology assessment, we put together interactive questions using the tool 'Metimeter'. This allowed participants to respond to questions online, via yes and no options, rating statements from high to low, participating in word cloud activities and providing short written comments. All answers were anonymous to encourage feedback. Below is a summary of key feedback received throughout the three sessions.

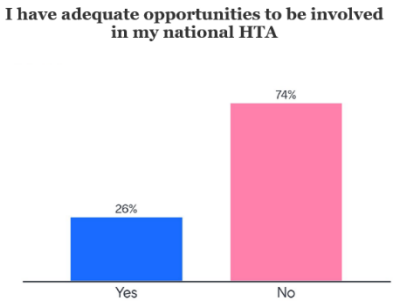
During Session 1, we asked participants about **their perception on the importance of the patient voice** in healthcare policy and HTA. The goal was to set the foundations for the training and understand if PAG representatives were clear that patient input was a priority for health policy decision-makers. All participants scored the importance of patient participation highly.

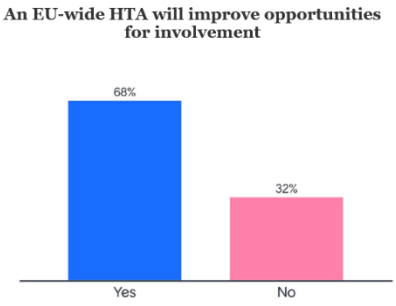
Why is patient involvement important to you and your organization?

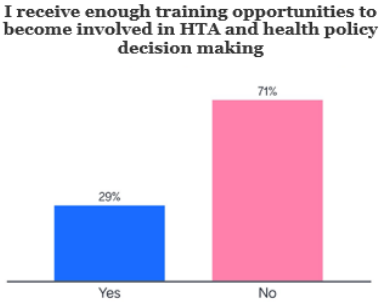
Summary of comments



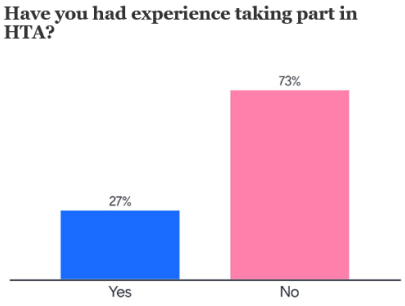
We also asked participants **why patient involvement was important** to them and their organisation. Answers focused on the importance of patient perspectives in decision-making and ensuring access to treatments.

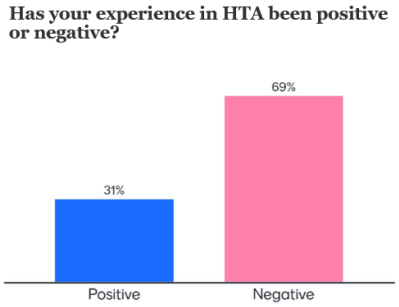




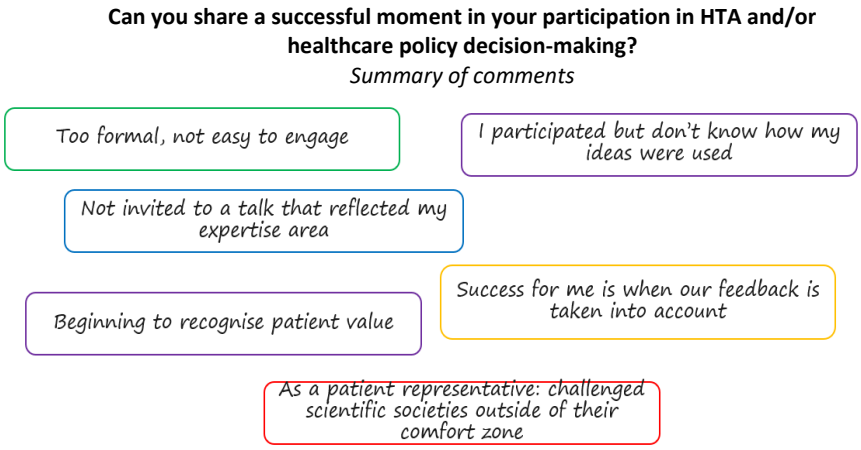


Finally, we asked participants if they felt they had **adequate opportunities to be involved** in their national HTA, and 74% responded with 'no'. The goal was to understand if HTA bodies are providing enough opportunities, or awareness of opportunities, for patient involvement. 68% of participants, however, felt that an EU-wide HTA would improve opportunities for involvement. Furthermore, 71% of participants told us that they do not receive enough training opportunities to become involved in HTA and health policy decision-making.





During Session 2, we learned that only 27% of participants had taken part in an HTA process. 69% of participants who had been involved, said that they had had a **negative experience**.

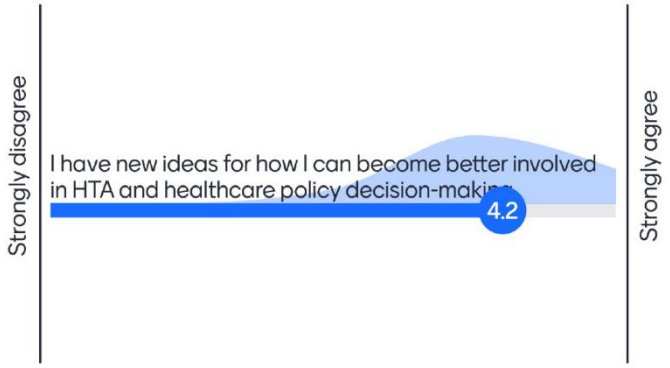


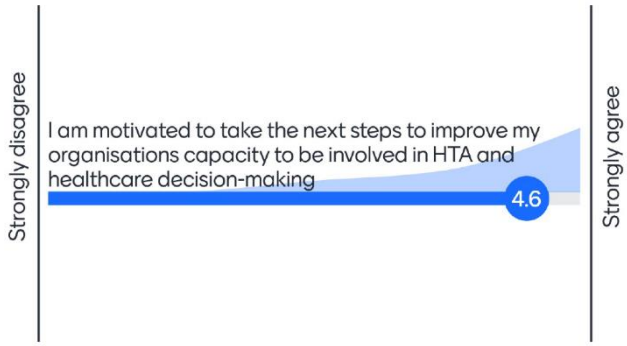
We also asked participants to share **both successful and challenging moments in their experience with HTA**. The goal was to better understand how PAGs perceive their involvement in HTA to date and identify areas where more support could be provided. The majority of feedback received was negative, with participants highlighting that: opportunities to get involved were limited, communication from HTA bodies was poor and there was a lack of plain language materials for complicated processes.

Finally, we asked participants to share **what HTA bodies can do to ensure more input from patients**, so that we could better understand the barriers that PAGs need to overcome to ensure effective involvement**.** Responses focused on improved communication, including informing PAGs of opportunities to participate and use of plain language materials, more transparent processes and providing opportunities for training.

At the end of Session 3 we asked participants for **feedback on content what was learned**, and **ideas for the future.**

All participants indicated that they **had new ideas for how they can become better involved** and were **motivated to take the next steps to improve their organisations capacity to be involved** in HTA and healthcare policy decision-making after the training.





Can you share your ideas for more effective patient involvement?
Summary of comments

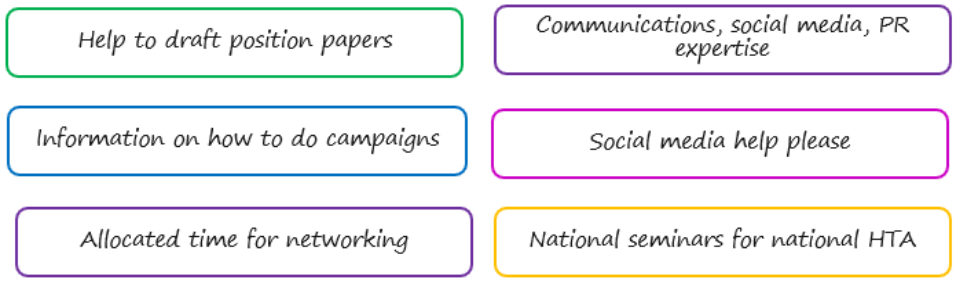
We also asked participants to share **their ideas for more effective patient involvement**. This enabled us to understand what had been learned throughout the Summit. The majority of feedback received was focused on the use of social media, creation of content and assets that can be used in advocacy activities and more training.

What is one action that you are going to do following this training program?
Summary of comments

When also asked what **one action they would take forward** as a result of this training programme, the results were varied. Participants said that they would either contact or try to understand more about their national HTA, discuss prioritization of HTA internally, find partners or explore use of social media.

The next three questions focused on **practical tools to support participants in their next steps** to achieve more effective patient involvement, and how they could be supported to follow these.

What practical tools will you need to support you in your next steps?
Summary of comments

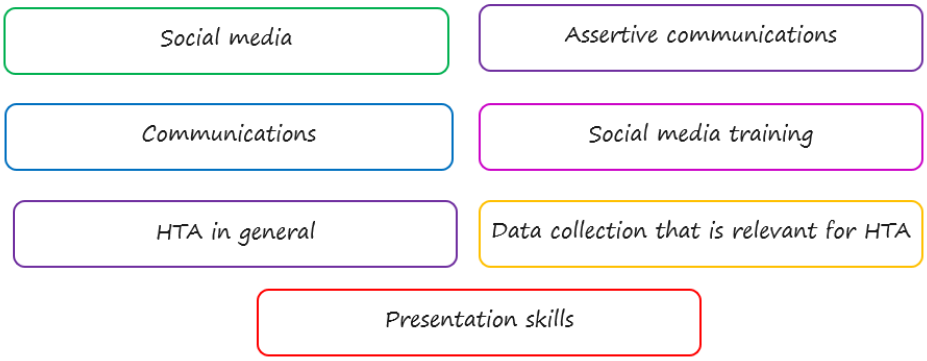


When asked **what practical tools would be needed** to support participants in their next steps, the main emphasis was on communications capability building, including how to effectively use social media.

How can we support you further?
Summary of comments

When asked **how Pfizer could support them in their next steps**, responses were focused around more providing more training opportunities and case studies.

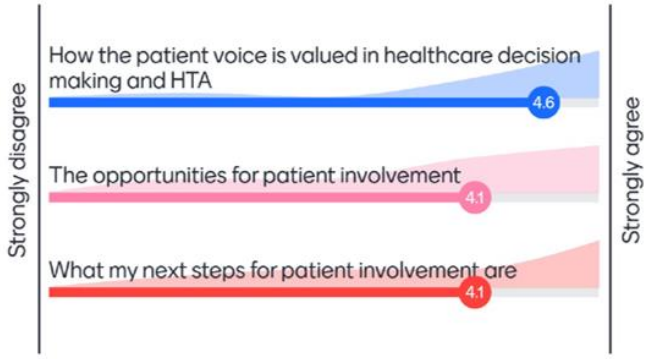
What area would you like to have more training on?
Summary of comments



We asked **what areas PAGs would like to have more training on**, participants focused on communications, social media and national HTA processes.

To close Session 3, we asked participants **for their feedback on the session overall**. The feedback received was overwhelmingly positive.

The training programme has helped me develop a better understanding of...



Feedback showed that participants **strongly felt that Pfizer's third HTA Advocacy Summit helped them** to understand how the patient voice is valued in healthcare policy decision-making and HTA, opportunities for patient involvement and next steps.

Is there any specific feedback that you would like to share with us or the speakers?

Summary of comments

- Everything was super motivating
- Very useful information, thank you!
- Very well done, very good and comprehensible speakers. Very clear
- I enjoyed the case studies a lot, they were super helpful
- Really useful. Italy is unfortunately in a bad moment for this and this is a sort of sunshine for me and my voice as a patient
- Loved the practical perspective

When asked to submit comments on the training overall, participants told us that the **training was useful and motivating**, that **speakers were clear**, and that **practical aspects**, such as sharing of best practice and case studies, were useful to bring back to their organization.

3. Summary of all presentations

Workshop 1, October 06, 14:30 – 17:00 CET

14.30 – 14.40	**Welcome and Introduction** *Gary Surmay, Pfizer*
14.40 – 15.00	**Inspirational Keynote 1: Why is patient involvement important to the development of an EU wide HTA-process?** *Flora Giorgio, Team Leader on HTA, B6 Unit, DG SANTE, European Commission*
15.00 – 15.30	**The patient voice is at the core of Europe's rapidly evolving health policy landscape** *Frédéric Destrebecq, Executive Director, European Brain Council (EBC)*
15.30 – 15.45	***Break***
15.45 – 16.30	**Country experience: How is Spain incorporating the patient perspective into health policy and HTA processes?** *Maria Galvez, CEO, Plataforma de Organizaciones de Pacientes (POP)*
16.30 – 16.50	**Interactive session 1: Share your experience**
16.50 – 17.00	**Recapping day one**

Gary Surmay (Senior Director, Corporate Affairs, Internal Medicine | Pfizer)
Opening remarks

Gary Surmay welcomed participants to Pfizer's third European Patient Advocacy Summit, this year focused on patient engagement in the early dialogue and assessment stages of HTA. Providing context to its development, Gary highlighted how this year's program has been specifically designed to provide continuity to 2019 and 2020's Advocacy Summits as well as respond to feedback from advocates who had expressed their interest in program continuation.

Gary also highlighted that the European Advocacy Summit series has been created as a direct response to calls from European PAGs to guarantee adequate involvement of patients in both healthcare decision-making and all HTA activities. To address this need, this year's program consists of EU and national level expert speakers in health policy, delivering their concepts and considerations across three virtual sessions to empower the patient voice in healthcare decision-making.

Flora Giorgio, Team Leader on HTA| B6 Unit, DG SANTE, European Commission
Inspirational Keynote 1: Why is patient involvement important to the development of an EU-wide HTA process?

Flora Giorgio, Team Leader on Health Technology Assessment in the European Commission's (EC) Health Directorate, DG SANTE, delivered a keynote presentation on why patient involvement is important to the development of an EU-wide HTA process. The presentation was designed to inform participants on the evolution of the EU-wide HTA legislation, it's role within the broader European HTA landscape, and how the EC views patient participation. Participation from the EC further demonstrated the value given to the patient voice in the process and the commitment to its integration.

Flora began her presentation by introducing the new EU-wide Regulation on HTA, to be adopted by EU institutions at the end of 2021. She highlighted that this new legislation does not remove responsibilities from national governments. Whilst part of the HTA process will be done jointly with all 27 Member States, notably the scientific and clinical aspects, EU Member States remain responsible for drawing conclusions on added value for their health system and taking decisions on pricing and reimbursement. She also reminded participants the three core principles for the new HTA Regulation: high quality, timeliness and transparency.

Of interest to participants, Flora explained how patient involvement would be foreseen in this new legislation. Patients will be able to provide input based on their specialized expertise on a therapeutic area during Joint Clinical Assessments and Joint Scientific Consultations. They would be referred to as 'external experts' and work alongside clinical and other

relevant experts. Flora also presented a second type of patient involvement: patients providing input as representatives of their wider PAG on horizontal and strategic issues. In this second scenario, they would be part of a 'Stakeholder Network and Coordination Group' with healthcare professional organizations, insurers and payers, industry associations and other PAGs. Overall, Flora advised patients to be involved within the 'scoping phase', as experts, to provide the most effective contribution.

Flora concluded her presentation with the EU-wide regulation's next steps. The EU institutions intend to adopt the law by the end of 2021, with a three-year transition period between its entry into force and the time it is actually applicable in the EU. This will allow EU institutions to draft implementing and delegated acts to complete the legislation, as well as guidance documents to help all HTA stakeholders participate. Flora highlighted that patient involvement will start as soon as the Stakeholder Group is set up (between 2021 and 2024), with the first EU-wide HTA process starting in December 2024. The Group's work will first target cancer drugs, followed by orphan drugs in 2027.

Audience Question and Answer Session

A question was asked about what type of skills PAGs need to effectively participate in policy decision-making. Flora stated that events such as Pfizer's HTA Advocacy Summit are important as they support PAGs to develop the know-how for future HTA activities. Flora also advised participants to closely monitor the EC's activities, especially the upcoming call to create the Stakeholder Group. This will ensure it represents as many PAGs as possible. Finally, she invited groups to actively appoint HTA experts to ensure contributions are meaningful and the ideas shared are innovative.

Frédéric Destrebecq, Executive Director| European Brain Council (EBC)

The patient voice is at the core of Europe's rapidly evolving health policy landscape

Frédéric Destrebecq, Executive Director of the European Brain Council (EBC), delivered a presentation on why the patient voice should be at the core of Europe's rapidly evolving health policy landscape. The EBC is an international health group comprising of major organizations in the field of brain research and brain disorders in Europe. Its structure involves a vast network of patients, scientists, and doctors, working in partnership with the pharmaceutical and medical devices industries. This presentation provided continuity from 2019's Patient Advocacy Summit, which trained European PAGs on how to become more effectively involved in the broader European health policy decision-making process.

Frédéric started the presentation by providing participants with concrete examples of the EBC's EU-level advocacy work, these included:

1. A consensus statement entitled 'The Need to Expand Brain Research in Europe', which included a preface from Philippe De Backer, former Member of the European Parliament (MEP), input from EBC members and over 100 European scientists and patient representatives. Presenting key data on the economic and social costs of brain disorders, the EBC called on European authorities to cooperate with EU Member States to devise and implement a plan to tackle brain health in an integrated and comprehensive manner. Since then, the statement has been presented during European congresses and policy events. A key outcome of this statement was the publication of the Belgian Brain Council's own national statement, stressing the importance of a Belgian Brain Plan.
2. Studies on Multiple Sclerosis entitled 'Rethinking MS', showing European policymakers that investment in brain research is cost-effective.
3. A 'call to action' in 2020 to EU institutions, signed by 33 PAGs from across Europe, asking for the development of an EU-wide brain health plan.

Frédéric stressed to participants the need for advocacy at a European level, noting the impact of brain disorders is growing, cures for brain diseases are sparse and access to treatment is low.

Frédéric explained why the integration of the patient voice – especially those who represent niche therapy areas such as brain disorders – is so important at every step of the value chain. In November 2018, the European Brain Research Area

(EBRA) was created as a platform for brain research stakeholders (researchers, clinicians, patients, governments, funders, and public institutions) to streamline and better co-ordinate brain research across Europe. Focusing on patient involvement, Frédéric explained how the EBC launched EBRA to support patients to share their opinion on the future of brain research and set out key priorities.

Beyond research, Frédéric also mentioned that the patient voice is essential to the modernization of brain tools and treatment. It allows quality of life to be considered as a criterion within their revisions.

Finally, Frédéric highlighted the importance of integrating the patient voice into the development of the European Health Union in order to address two challenges: low accessibility to treatment and lack of innovation in the post-COVID-19 period. He emphasized that the EU could be doing more to support the evolution of the European Health Union and that the EBC would be working on key priorities such as funding and digital health solutions. To conclude, the EBC emphasized the importance of continued interaction with the European Institutions to build strong European health policies, raise awareness and encourage education on the brain and the repercussions of neurological and mental health conditions on society as a whole.

Audience Question and Answer Session

Two members of the audience asked Frédéric for advice on how to ensure PAGs are considered as valuable stakeholders in policy shaping. He answered that first, patient groups should recognize their real value in health policy and avoid underestimating the value of their lived experiences. He also discussed the power of using digital tools, referencing how the pandemic has changed how medicine is delivered and encouraging patient groups to also leverage digital tools to ensure their voices are heard in health policy.

Fredéric was also asked how to ensure patient voices representing non-priority disease groups were heard at an EU-level. Fredéric stated that whilst the pandemic remains a health policy priority, this does not mean that other disease areas are going to be overlooked, specifically referencing the fact that DG SANTE is keen on making sure that no areas are left behind. He stated that those representing non-priority disease areas must put forward solutions to their challenges and that policymakers no longer need the data but concrete solutions to problems.

Finally, an Italian patient group representative shared an example of best practice in data sharing from Italy. Frédéric acknowledged the achievements of the country and advocated for the promotion of a similar cultural mindset for European healthcare systems.

Maria Galvez, Director| Plataforma de Organizaciones de Pacientes (POP)

Country experience: How is Spain incorporating the patient perspective into health policy and HTA processes?

Maria Galvez, Director at the Plataforma de Organizaciones de Pacientes (POP), delivered a presentation on how Spain incorporates the patient perspective into health policy and HTA processes. POP is a non-profit organization bringing together chronic disease patient organizations from across Spain, with a mission to promote participation and defend the rights of patients in all areas impacting their lives, including public policy. The presentation gave a national level example of how PAGs can advocate towards healthcare systems, directly responding to feedback from 2020's Advocacy Summit, where there was a call from participants for more national examples.

Maria began her presentation by outlining how healthcare is organized in Spain. She explained how the Spanish Constitution of 1978 established a universal healthcare system, and how the General Health Law of 1986 established the framework for today's National Health System. This Constitution and Law form the regulatory framework for the devolution of healthcare services to the 17 Autonomous Regions, who all have complete power regarding public health and planning. However, responsibilities on healthcare financing, organization, provision, and management have devolved to only seven Autonomous Regions. Financial support for health services comes mostly from taxes.

Maria presented best practice case studies of POP's advocacy towards the Ministry of Health, explaining how it was important for the patient voice to be active across all its different functions and activities. Providing examples, Maria explained how POP had worked on Spain's COVID-19 vaccination strategy and advocated for the inclusion of vulnerable groups to be prioritized, and that POP was a member of the communication committee for Spain's vaccine campaign. She also explained how POP was active within committees in the Spanish Agency of Medicines and Medical Devices (AEMPS) and brought the voice of the patient to challenges around medicine shortages.

Another example given was POP's work with the Spanish Secretary of State for Digital Health with regards to the development of eHealth services. The purpose of which, Maria noted, was to ensure that eHealth services reflect the needs of the patient, advocate for the generalization of sharing health information between the 17 Regions and promote the use of data analytics related to health systems. Maria highlighted how the devolution of competencies in Spain means that networking is an extremely important part of PAG networking activities.

Maria then outlined how HTA is organized in Spain, emphasizing its long history with its first institutional initiative taking place in Catalonia in 1984. Whilst HTA organizations and formal activities developed at different speeds across the Spanish Regions, today Spain has a national agency and several regional organizations coexisting and cooperating. HTA is considered critical to support the decision-making process as it ensures the introduction, adoption, and utilization of a health technology is made according to scientifically proven criteria of efficacy, safety, effectiveness, and efficiency.

Looking at patient involvement in HTA in Spain, Maria explained how there is a national declaration urging for a strategy for involving patients. Furthermore, that the Spanish Network of Agencies for Assessing National Health System Technologies and Performance has a methodological framework for patient engagement and is incorporated by six of the eight regional agencies. Input opportunities take a variety of forms, including surveys, focus groups, and telephone or face-to-face interviews. Patients participate in some assessment organizations' expert panels. She noted how the framework is an important step towards overcoming the challenges that stem from Spain's decentralized approach to HTA. Maria also talked about Valtermed, a new shared information system implemented within the Spanish National Health System to assess the real therapeutic value of drugs. The inclusion of a new functionality is being analysed so patients included in Valtermed can enter information related to quality of life. Forms will be designed based on validated surveys.

Overall, patients in Spain are often still limited in their opportunities to engage in HTA. There is limited evidence on how patient input is incorporated into the assessment process and outcomes. Maria explained how the HTA system can be improved to integrate the patient voice. First, the structure for involvement could be improved. Assessment organizations struggle with recruitment and capacity building for patient engagement. Second, plain-language materials should be provided by national HTA bodies to increase patient involvement. Thirdly, there should be more feedback on how the patient voice has been integrated into HTA outcomes.

Maria concluded her presentation by explaining how POP has worked to elevate the patient voice in HTA, including collaboration with a renowned university to create an expert nine-month training program and certificate to improve knowledge about patient engagement in HTA.

<u>Audience Question and Answer Session</u>

The first question from the audience was about advocacy practices established during the pandemic that Maria hoped would say. First, Maria said that she believed that there had been an increased awareness of the importance of PAGs in both society and policy decision-making, and that the pandemic had been an opportunity for groups to show their strengths. She believed that this momentum must be built upon in the future. She also explained how the digital transformation induced by the pandemic had provided opportunities for better collaboration between Spain's 17 Autonomous Regions.

Two members of the audience asked Maria why PAGs should advocate at an EU-level. Maria explained the importance of working within a network and how POP works closely with EPF. She explained how organizations with similar goals must

collaborate and how umbrella organizations have a higher leverage at an EU-level. Maria also emphasised the importance of sharing data with policymakers to support argumentation.

The final question received was about Maria's optimism towards the EU-wide HTA and its inclusion of patient perspectives. Maria told the audience that initiating the obligation that every HTA process needs to include the patient voice at a national level was a positive step forward. She said that from her perspective, PAGs should be involved in every health policy decision taken.

Workshop 2, October 13, 14:30 – 17:00 CET

14.30 – 14.40	**Welcome back and introduction to day 2** *Gary Surmay, Pfizer*
14.40 – 15.00	**Inspirational Keynote 2: How will an EU-wide approach to HTA impact patients in Europe?** *MEP Tomislav Sokol (European People's Party, Croatia)*
15.00 – 15.30	**Key strategies to support patient involvement in HTA** *Valentina Strammiello, Program Manager, European Patients Forum (EPF)*
15.30 – 15.40	***Break***
15.40 – 16.30	**A deep dive on early assessments: how can patient involvement ensure that HTA outcomes remain relevant?** *Anne Willemsen, Senior Project Manager, EUNetHTA*
16.30 – 16.50	**Interactive session 2: How could HTA outcomes be more relevant for you?**
16.50 – 17.00	**Recapping day two**

MEP Tomislav Sokol, Member of European Parliament (Croatia, EPP)

Inspirational Keynote 2: How will an EU-wide approach to HTA impact patients in Europe?

Member of European Parliament (MEP) Tomislav Sokol (Croatia, European People's Party) delivered a keynote presentation on how an EU-wide approach to HTA will impact patients in Europe. This presentation was important to both set the scene for participants on what an EU-wide HTA process would look like, but also by attendance, to demonstrate to patients that their voice and perspectives are valuable to the European Parliament in healthcare policy decision-making.

MEP Sokol started his presentation by reminding participants that health has never been a competence of the EU, and that responsibility for the organization of healthcare systems remains with the individual member state. However, he stated that there is now political will for a competency 'shift', and for the EU to assume more powers in health. He noted the establishment of the EU's Health Emergency Preparedness and Response Authority (HERA) in response to the COVID-19 pandemic as a tangible example of said political will.

Looking specifically at the EU-wide HTA legislation, MEP Sokol reminded participants that the initial legislation had the intent to remove the need to go through the procedures of 27 different member states with different legal frameworks. One single procedure for the EU would have been a positive step for all stakeholders, and it would have meant that a new medicinal product could be applied to all member states simultaneously.

Moving on, MEP Sokol explained how he does not believe that the final EU-wide HTA legislation, diluted through compromise amongst the EU institutions, goes far enough in creating one EU-wide approach to HTA and that the final decision on a new technology will ultimately rest with the Member State. This reminded participants that the EU-wide approach to HTA will not replace existing systems, but instead will be an additional procedure in the overall HTA landscape.

MEP Sokol also reminded participants that the EU-wide HTA will not have the legal strength to impose obligations to provide funding for certain technologies. He shared his belief that decisions on cost-effectiveness should be made at an EU-level, as this would increase the availability of new technologies and would increase transparency.

To conclude, MEP Sokol stated that he believed the EU-wide HTA legislation was a step in the right direction to increase accessibility to new health technologies in Europe.

Valentina Strammiello, Director| European Patients Forum (EPF)

Key strategies to support patient involvement in HTA

Valentina Strammiello, Director at the European Patient's Forum (EPF) presented on key strategies to support patient involvement in HTA. This presentation was important for participants to understand how patient advocacy organizations had worked to shape the EU-wide HTA legislation, and what the scope of patient participation in the new EU-wide HTA process would look like. The EPF is an umbrella organization of 75 patient organizations across Europe, across disease-areas. It has been the leading patient group advocating on behalf of patients in the development of the EU-wide HTA. EPF is also one of the European Medicine's Agency's 35 eligible PAGs.

Valentina began by describing the existing HTA system in Europe, which is characterized by cooperation between national HTA bodies. Whilst this model has led to achievements such as trust building, capacity building, development of joint tools and joint work pilots, it faced significant shortcomings rendering it unsustainable and unable to reach its full potential. For example, it created a heavy burden of administration leading to a duplication of HTA work across EU Member States. Furthermore, differences in procedural frameworks and national methodologies caused significant divergences between national HTA processes regarding reimbursement decisions, and assessment criteria. The intent of the new EU-wide HTA regulation, proposed by the European Commission in 2017, was to address these challenges through the creation of common standards and strengthening evidence-based decision-making at a national level.

In a short outline of the evolution of the EU-wide HTA legislation, Valentina emphasized that due to conflict between the European institutions during the negotiation phases, the final legislation was 'watered-down', and initial wording around 'mandatory update' was removed. This clause had been designed to legally impose Member States to take on HTA research conducted at an EU level. This means that the EU-wide HTA legislation will not replace national HTA processes and there is still a division of competences. The joint work on scientific and clinical aspects of HTA will be conducted at EU-level, driven by national HTA bodies. EU Member States remain responsible for assessing the added value of a technology for their national health system and pricing and reimbursement.

Valentina stated the EPF welcomed the overall intent of the EU-wide HTA legislation, noting that an increase in EU cooperation would lead to a reduction in discriminatory outcomes caused by different HTA methodologies for the patient community. However, whilst the new regulation allows for increased patient involvement in the HTA process, it is disappointing that patient involvement and stakeholder engagement is subject to a 'top-down' approach. Patients are not included in the Coordination Group of the HTA, essential to its functioning, governance structures and they do not have voting rights. Valentina explained how the EPF would like to see patients involved at every step of the process, and that they are anticipating the stakeholder guidelines to be released by the Commission as a part of the implementing acts, expected in early 2022. Whilst the regulation is not ideal, she reaffirmed that it does provide the basis for further work and improvement. The introduction of an EU-wide HTA in itself is a good foundation.

Valentina explained how the EPF ensured inclusion of the patient voice throughout the development of the EU-wide HTA legislation. She explained how the EPF contributed to a public consultation in 2015, produced a policy position statement, connected with multiple MEPs and organized events. This included closed events not accessible to all patient representatives, such as meetings with EU Health Attachés. They also collaborated with other patient organizations to put forward a strengthened collective voice and used social media to ensure the EPF's point of view was made known.

Valentina explained it is important to understand that whilst a European level framework is nice to have, it cannot happen without joint efforts at a national level. Patient representatives at national level should advocate to promote common standards across Member States, including sharing of best practice, insights from impactful engagements, and knowledge from country experiences. Each country has its own methods for patient involvement – some invite PAGs to meetings, others to phone interviews and others to written consultations.

To conclude, Valentina explained now is the time for groups to start getting prepared at a national level for involvement in both national and EU-wide HTA processes. Member States are preparing to update their HTA processes to align with the EU-wide HTA, providing an opportunity to engage with national Ministries of Health to ensure processes are in place for patient representatives to contribute. National groups can also connect with umbrella groups at a European level and contribute to the collection of data. Another useful skill is to be able to mitigate the risk of conflict of interest. Regarding this, EPF runs a project called 'Paradigm' that provides patients with tools to mitigate conflicts of interest, educates on how to engage with companies, clarifies the consequences of engagement and supplies patients with practical tools.

Valentina's take-away messages are:

1. There is a need to educate patient groups and to raise awareness of the HTA process and areas for patient input.
2. Patient groups need to understand how to play the art of diplomacy and resilience in the current HTA, as conflicts of any kind should be prevented to supply the process with maximum patient input.
3. Patient groups need to show solidarity with our similar groups to promote innovative technologies. A winner/looser mindset is of no use.

Audience Question and Answer Session

The first question was about how the EU-wide HTA process can be made representative of variations in Member States and how, amidst this, we can ensure its inclusivity. Valentina responded by saying patient involvement in the HTA process needs to encompass patient perspectives from across the EU. It is, therefore, crucial to ensure EU-wide education of patient organizations. Regarding inclusivity of the process, Valentina mentioned that even if they are not obliged to take on EU conduct, the HTA forces Member States to be transparent on their procedures which make it more inclusive than its precedent, which, she noted, is a good starting point.

The second question critically asks why patients, under the new regulation, have the right to suggest but not the right to vote. Valentina responded by saying it is perceived that this approach was chosen by HTA bodies to keep control of the process. She agrees that the regulation does not provide the right opportunities for patients to share their valuable knowledge and that the overall perception one gets is that patients need to be invited to participate rather than being a firm component of the process. However, she has hope that patients will be able to increase their scope for input in areas such as the prioritization of technological input.

Anne Willemsen, Senior Manager | European Network for Health Technology Assessment (EUnetHTA)

A deep dive on early assessments: how can patient involvement ensure that HTA outcomes remain relevant?

Anne Willemsen, Senior Manager at the European Network for Health Technology Assessment (EUnetHTA), delivered a presentation on how patient involvement can ensure HTA outcomes remain relevant. This presentation outlined concrete approaches to patient involvement in HTA, including where to input in the process to be more influential. EUnetHTA was founded to foster cross-border collaboration between HTAs throughout Europe and to reduce overlaps in assessed topics and wasted resources. Anne also presented in 2020's HTA Advocacy Summit.

Anne began her presentation with a refresher on HTA, clarifying how it presents the scientific and technical side of allowing bringing a new technology to market. It assesses whether a new health technology is better,

how it compares to existing technology and affordability at national level. It supplies policymakers with reliable and timely information about complex research questions and facilitates their decisions on pricing and reimbursement.

Anne presented EUnetHTA's framework as a facilitator of high-quality HTA collaboration in Europe, funded by the European Commission. Its origin lies in a highly inefficient European HTA process, characterized by issue overlap and high cost and resource usage due to multiple independent national processes. The core model of EUnetHTA is to supply Member States with an information package covering the scientific and technical evaluation of a health technology across four domains: health problems and current use of technology; description and technical characteristics; safety; and clinical effectiveness. Anne clarified EUnetHTA does not give recommendations on added value or reimbursement decisions.

Anne explained the key benefits of HTA collaboration are increased efficiency, timeliness, higher quality, and greater consistency among Member States. Anne outlined EUnetHTA's Joint Action 3 initiative (2016-2020), which aims to increase production of high quality HTA joint work, increase uptake and implementation of joint HTA work at national level and support evidence-based, sustainable, and equitable choices in healthcare. This system is currently based on voluntary cooperation between 83 national and regional HTA bodies from more than 30 countries.

Moving on, Anne explained how EUnetHTA includes patient perspectives in HTA processes. EUnetHTA deems patient involvement most important in early dialogues, to form advice based on unique patient experience, and in the production of assessment reports. EUnetHTA recognizes patients have a unique knowledge of living with a specific disease or medical condition.

Anne described how EUnetHTA uses a hybrid model to test multiple approaches to patient involvement in early dialogues, requiring different levels of engagement and expertise. These are: individual patient interview; interview and discussion with local HTA bodies about the submission file *without* the applicant; and interview and discussion with all HTA bodies about the submission file *with* the applicant. After each process, interview minutes are sent to participating patients and EUnetHTA shares its recommendations and receives a patient feedback questionnaire. Participant feedback was positive, expressing how this model allowed ample opportunities to express opinions. Nevertheless, participants signaled a continued need for training.

Moving on to talk about patient involvement in Joint Assessments, Anne explained three specific methods for engagement: a patient input template, one-on-one conversations, and group conversations. All approaches offer opportunities and challenges. Patient input templates allow for general feedback and a synthesized view. One-on-one conversations focus on individual patient experiences whilst a group conversation allows discussion between individuals on specific topics. Whilst patient input is highly valued during this scoping phase, as it ensures EUnetHTA assessments are fit for purpose, it remains difficult to identify patients willing to participate and share experiences, as this can be intimidating to patient representatives. Additionally, patient involvement can be subject to a conflict of interest, for example, if the patient has already assisted in the product development.

Using the interactive tool *Mentimeter*, Anne asked participants how they view patient participation in HTA. The first question, 'what is in your opinion the best method', revealed 39% of the audience preferred 'one-on-one conversations', 25% 'scoping meetings' and 19% for 'group conversations'. For the second question, 'what do you think is the best method according to HTA bodies?', 47% of the audience voted for 'review of assessment report', 33% 'join a scoping meeting' and 13% for 'group conversations'. The final question, 'which method do you think is most feasible?', showed 61% of participants perceived online calls for patient input to be the most effective at encouraging their participation. Anne commented responses revealed that patients would like to be more closely integrated into the process through one-on-one conversations, invitations to a scoping meeting and group conversations, yet the most feasible option remains the open call for patient input.

Anne concluded by stating that EUnetHTA would like to see 'open calls' for patient feedback as a standard approach during HTA. If necessary, this should be complemented by other tactics such as interviews. This is a common approach among many HTA agencies. This open call for patient input is published on the EUnetHTA website, and at the same time, EUnetHTA pro-actively approaches relevant patient organizations who are asked to complete a questionnaire. This combination offers a synthesized view of patient experience both from a national and European perspective and reduces issues around conflict of interest.

Anne's key takeaways messages are:

1. Patient involvement is highly valuable in early dialogues and in scoping the research questions as it ensures that HTA assessments are fit for purpose.
2. Patient involvement is subject to significant challenges regarding patient identification, patient responsiveness, and conflict of interests.
3. Patient involvement is the most effective when patients are sufficiently trained on procedures of HTA and have sufficient knowledge about the technology in question, wherefore training is essential.

Audience Question and Answer Session

The first question asked Anne to give a concrete example of patient involvement best practice in Europe. Anne outlined the example of a treatment for young children's Kawasaki-like disease (KLD). EUnetHTA conducted an open call which asked for patient group input on which outcome is the most important and how to link it into the assessment of treatment for this disease. Three countries responded to the open call showing the method's efficacy for patient involvement.

A second question asked what the most valuable tool would be to ensure the successful functioning of the HTA. Anne responded by saying she would like to see a department solely dedicated to stakeholders in the HTA process, similar to the set-up at the EMA.

Following on from this question, Anne was asked what she perceives as the greatest threat to setting-up the EU-wide HTA process. Anne believes there will be a challenge in adapting vastly different national HTA methodologies to meet the new EU-wide framework, as political institutions are, typically, reluctant to adapt. Anne predicts a long journey to reach a fully collaborative HTA, in which it will be essential to build trust between national and European policymakers, patients, and industry.

Regarding divergences between Member States, the audience asked Anne what the best approach would be to reach a fair assessment in HTA across European countries. She affirmed that inequalities will remain with the current system due to decisions on cost-effectiveness remaining at national level. An HTA framework that minimizes inequalities would be one that integrates not only scientific and technical evaluation but also economic decisions. Member States would be legally obliged to take on procedures and results conducted at the European level.

Workshop 3, October 20, 14:30 – 17:00 CET

14.30 – 14.40	**Welcome back and introduction to day 3** *Gary Surmay, Pfizer*
14.40 – 15.10	**Building an impactful advocacy approach: lessons learned from a patient advocacy group** *Vicki Mooney, Executive Director, European Coalition for People Living with Obesity (ECPO)*
15.10 – 15.20	***Break***
15.20 – 16.00	***What can patient advocacy groups do to generate data needed to provide impactful contributions to HTA?*** *Donna Walsh, Executive Director, European Federation of Neurological Associations (EFNA)*
16.00 – 16.20	**Interactive session 3: Translating learnings into action**
16.20 – 16.50	**Keynote 3: Observations on how the patient voice has been built into pharmaceutical policy and thoughts for the future** *Deirdre Ryan, President, Pain Alliance Europe (PAE)*
16.50 – 17.00	**Recapping day two**

Vicki Mooney, Executive Director, European Coalition for People living with Obesity (ECPO)

Building an impactful advocacy approach: lessons learned from a patient advocacy group

Vicki Mooney, Executive Director of the European Coalition for People living with Obesity (ECPO) delivered a presentation on how to build an impactful advocacy approach, with key lessons learned from ECPO's own experience. ECPO is an independent patient led, managed, and run organization working collaboratively across Europe, representing the voice of the obesity patients. As a nascent PAG, the purpose of this presentation was to highlight the experiences, challenges and key learnings of a PAG building its advocacy strategy and working to integrate its voice in policy decision making process.

Vicki started her presentation by introducing the disease obesity. Obesity is recognized as a global epidemic and the most prevalent metabolic disease world-wide. It is a complex disease embedded in a perpetuating system of many areas of life, such as societal influences, individual psychologies, food consumption and biology. For 70% of the patients, obesity has genetic causes. Besides this, it can find its roots in environmental, socioeconomic, neurological, and psychological causes. Nevertheless, obesity is not commonly spoken about as a disease, but is often considered to be a lifestyle choice. Additionally, access to specialized obesity services is rare and varies significantly across Europe.

Vicki moved on to present ECPO. Launched in April 2019, following its initial position as a working group of the European Association for the Study of Obesity (EASO), ECPO encompasses 50 organizations from 27 countries in Europe. Its strategy is built on four pillars: advocacy, access, education, and capacity. Advocacy lies at the heart of the organization's work and builds the foundation to access, education, and capacity. ECPO's aim is to raise awareness of obesity not only as a disease, but also a gateway to many other medical conditions such as cancer, leukemia, or diabetes type 2. The organization wants to increase access to quality and affordable healthcare across Europe and reduce the stigma around obesity being a lifestyle choice.

To build a European advocacy strategy, ECPO relied on three important aspects:

1. Collaboration with other communities to increase leverage
2. Listen and learn from sister communities on best practices to influence change
3. Empowerment of members through an interactive patient advocacy program to build an advocacy plan

Vicki also outlined three characteristics PAGs need to have at their disposal to succeed:

1. Patience, as building an advocacy strategy takes time
2. Trust in yourself, as this will make you resilient to obstacles that you will face

3. Be prepared to be disappointed and grow back stronger when you are

Vicki continued to outline why the patient voice is key to be included in health policy making. She emphasized patients are critical in influencing change as they are experts on their disease. Additionally, patients have passion to push for change which is drawn from their unique experience of living with their disease. Finally, the impact patients can have through contributing to policy debates should never be underestimated. Patients give highly complex medical discussions a human feel and stir emotions which attract media attention, and through this raise public awareness.

Drawn from personal experience, Vicki continued to outline learnings and challenges that ECPO faced in building their advocacy action plan. She highlighted that patient experience is key to successful advocacy. For patients to share it, they need to be well advised and prepared. Whilst some patients are highly passionate about achieving more equitable and adjusted access to healthcare services relating to their disease, they risk becoming victims of burn out and other conditions related to overwork and stress. Others might need more empowerment to share their experience due to low self-esteem when faced by scientists. At the heart of successful and sustainable advocacy is relationships. Therefore, diplomacy is an important skill to have to overcome cultural differences in how, for example, to approach obesity. Finally, an advocacy plan should be broken down into manageable steps to prevent organizations from feeling overwhelmed by the actions that lie in front of them.

Vicki outlined two case studies on what successful advocacy can look like. First, the Irish Coalition for People living with Obesity managed to hugely extend its advocacy work from a Facebook group for obesity patients in 2010, serving to share experiences, to one of the largest active PAGs in Europe 2021. Their success is due to the groups' engagement with advocacy trainings, policy conferences and congresses, leading to the empowerment of their members. Having launched in 2020, the group's members increasingly reached out to collaborate with other peer organizations such as EASO, and the Association for the Study of Obesity Ireland (ASOI), clinicians and industry, finally making it the winner of the 2021 Award on collaboration at the World Obesity Day.

Second, a PAG in Italy launched a campaign to ensure people experiencing obesity have the necessary support and access to treatment. It advocated for the Italian government to recognize obesity as a disease that falls under the Charter of Fundamental Human Rights, needing regulatory and legislative support. Through strong engagement with a network of scientists and politicians at the national level, the advocacy group was able to publish the Italian Obesity Barometer, containing official data on the burden obesity exercises on the economy and society, as well as an open letter on the protection of people with obesity in COVID-19. Supported by these achievements, the group submitted a motion on recognizing obesity as a disease that needs regulatory and legislative support to the Italian government, which was accepted by 458 of 458 parliamentarians. This ultimately achieved a political commitment of the Italian government to implement a national action plan for the fight against obesity.

Vicki's key takeaway messages:

1. Building a firm advocacy plan and network can be a long process, but change is inevitable and patient organizations need to stay empowered. Empowerment is what creates change.
2. Collaboration, diplomacy, and a firm network of relationships is key to successful advocacy as it increases leverage in the political sphere and allows to share best practices.
3. Teamwork and watching out for each other is important as advocacy can be demanding, disappointing and overwhelming in the short run. Perseverance pays off.

Audience Question and Answer Session

Having presented two national level case studies, Vicki was asked to give an example of successful advocacy at European level. She outlined the 'Open EU' initiative on obesity that was created amidst the COVID-19 pandemic. This initiative seeks to ensure that European stakeholders integrate measures that help support people living with obesity through

concrete institutional strategies and policies. Intermediate results of this ongoing initiative are that the EU Commission referred to obesity as a chronic disease in one of its reports. This represents a door opener to extend the work with MEPs from the European Alliance on Obesity to further call European policymakers to address the needs of people living with obesity.

Donna Walsh, Executive Director, European Federation of Neurological Associations

What can patient advocacy groups do to generate data needed to provide impactful contributions to HTA?

Donna Walsh, Executive Director of the European Federation of Neurological Associations (EFNA), delivered a presentation on what patient advocacy groups can do to generate data needed to provide impactful contributions to HTA. EFNA is a leading umbrella organization of pan-European groups working on neurological disorders, recognized as one of the European Medicine's Agency's 35 eligible PAGs. EFNA includes groups active and influential in the HTA process and aims to strengthen the capacity of patient organizations in advocacy, awareness, empowerment and engagement. EFNA's vision is to improve patient quality of life, acknowledging that the key to achieving this goal is access to treatment.

Donna began her presentation by explaining that HTA has always been on EFNA's radar, workshops about HTA were organized at the London School of Economics in 2018, and by denouncing HTA processes which prohibit patient involvement. Efforts to include patients need to be on both sides: patients need to upskill, and stakeholders need to work for more inclusive processes.

Donna advocated for a more patient-centered approach to the development of medicines, which looks at demographic, gender, ethnicity, and environment. The more personalized approach to healthcare, the better. Donna explained that it is time for this field to incorporate social and emotional functionalities, quality of life, and patient preferences.

Before presenting ways for patient advocates to meaningfully contribute to HTA, Donna reminded participants that each patient is different. For this specific reason, adopting a one-size-fits-all within HTA processes is not effective. Donna did not agree with previous methods to only involve one patient within HTA processes, as the experience shared, although valuable, is not representative of the wider community. To ensure that patient engagement is capturing the voices of the community, Donna proposed to build real-world data and fill-in evidence gaps. This would be achieved though analysis of disease registry, observational studies with patients, and reaching out to communities to learn more about experiences.

Donna gave participants a key piece of advice: national HTA agencies do not usually welcome patients to advocate. Rather, they invite them to share facts, instead of emotions. If each national body is different in terms of evidence needs, a common ground is that evidence should be up to date and supplied by patient organizations as representatives. To Donna, providing these agencies with data is the most effective way to be involved within HTA.

Participants were presented tactics to collect real-time data. Donna insisted patient organizations should not aim for 'perfect' information or provide scientific data, as HTA processes are not clinical trials. Instead, they should present the opinion of their community on the assessed technology. Donna recommended using interactive platforms such as social media to gather such data, where patients provide the most honest point of view. These online platforms are especially useful to gather contributions from patients around the world, who have already had access to a treatment that is not yet available in other countries. Overall, the harnessing of input from social media could complement traditional forms of patient opinion collection. The key for patient organizations, at this point, is to create a safe environment in which peers can share their experience.

Donna presented a case-study conducted in the U.S. to show how using social media can be an effective method for patient data collection. Following the COVID-19 pandemic, many American citizens discussed their symptoms on social

media, which turned out to be a valuable source of information to doctors, regulators, and researchers to address the needs of the community. The FDA, concluded Donna, even encourages the use of social media in some parts of its work.

Donna explained how patients often ask whether it is worth their time to share their perspectives – online or offline. Donna presented results of a patient engagement study in the UK, Germany and France. It found that whilst there were opportunities for patient engagement, contributions had little or no impact on the final decision taken. Donna explained that this is often because there is a lack of shared purpose, representation across the wider patient community, accessibility, capability, and transparency, as patients do not receive feedback following their contribution. In order to prevent these shortfalls, Donna gave participants four pieces of advice:

1. Get to know national HTA bodies: the person responsible for the HTA processes, the type of patient involvement required, methods and expectations.
2. Work with other patient organizations and share best practices, healthcare professionals for guidance, and industry to best prepare.
3. Receive feedback from contributors to learn from mistakes in supplying evidence to HTA bodies.
4. Be involved as early as possible in the development process of a medicine or device, and not just HTA.

Donna concluded her presentation by encouraging patient organizations to get informed. There are platforms for patients to interact between each other and share data, as well as associations such as EUPATI which train patient advocacy groups.

Donna's key-takeaway messages are:

1. Real world data is of increasing importance in regulatory and reimbursement decision making including HTA.
2. Patient organizations are well placed to generate this data using simple, accessible tools such as social media.
3. Data can influence decision-making but more needs to be done to optimize its potential.
4. Patient organizations must engage along the R&D process and beyond to advocate for access incl. in early dialogues.
5. Education and empowerment are key!

Audience Question and Answer Session

A member of the audience asked Donna whether she could enlighten the audience with the most effective patient involvement method that she has experienced. She replied that what matters the most in the HTA process, is the actual influence patient contributions have. She gave the example of Lithuania, a country that publishes tenders for organizations to work alongside the government on the HTA process. Donna applauded this initiative, which allows for a real patient-centered process. She concluded her answer with the following quote: "it is easier to build a new process than change an existing one."

Deirdre Ryan, President Pain Alliance Europe

Keynote 3: Observations on how patient voice has been built into pharmaceutical policy and thoughts for the future

Deirdre Ryan, President of Pain Alliance Europe (PAE), presented her observations on how the patient voice has been built into pharmaceutical policy, alongside her thoughts for the future. PAE is a pan-European umbrella organization of 40 national and European associations in 17 EU countries, representing over 400,000 individual chronic pain patients. It is also one of the European Medicine's Agency's 35 eligible PAGs. Deirdre's presentation represents a credible source in explaining how patient organizations representing deprioritized therapy areas can be included in European policymaking through concrete advocacy plans and strategies.

Deirdre began by introducing 'chronic pain' and the organization PAE. Chronic pain is an unpleasant sensory and emotional experience associated with actual or potential tissue damage, or described in terms of such damage. It persists or recurs for longer than three months. PAE's key initiatives include the 'Societal Impact of Pain' (SIP) platform, a multi stakeholder partnership with the European Pain Federation (EFIC) aiming to raise awareness of pain and change pain policies, and the

MEP Interest Group on Brain, Mind and Pain, in partnership with EFNA. The MEP interest group aims to improve the quality of life of all those living with brain, mind and pain disorders across the EU, as well as their families and carers.

In the context of SIP's newly published position paper on Workplace Integration and Adaption, Deirdre presented specific questions patient organizations need to consider when building such a position paper or advocating for policymakers to consider their point of view. Deirdre remarked that the foundation of SIP's policy paper presents a thorough understanding of the patient's situation, needs, and how to improve these. To do this, it is important to invest a considerable amount of time in acquiring information from reliable and valuable sources. A second step is to develop a concrete strategy to achieve your organization's advocacy goals. This advocacy plan should be built on the identification of the following:

1. Partner organizations that could increase leverage at the EU level and facilitate exchange of best practice.
2. Policy areas that have an impact on the life of people in the advocacy area – in pain, an example beyond health would be employment.
3. MEPs and committees interested in the advocacy area – this gives an advocacy plan a strategic goal.
4. EU funding structures related to the advocacy issue and that provide opportunities for involvement. Participation in research, for example, makes a patient organization familiar with an issue allowing for their effective inclusion in decision-making.

Moving on, Deirdre presented an overview of pain in the EU. Given pain turns chronic after three months, it is shocking that, on average, people take one year to seek medical help for it. A 2017 PAE survey[1] shows 20% of participants waited more than 10 years before getting a diagnosis for chronic pain. These statistics indicate that health literacy and awareness of chronic pain treatment is too low amongst pain patients. A solution to this challenge could be the further implementation of digital health. A trend we are witnessing due to the pandemic. A Pfizer sponsored 2021 PAE survey[2] on COVID-19 and Chronic Pain shows that 49% of patients receive advice or medical appointments via telephone. However, there is an opportunity for improvement as only 10% currently receive video consultations, despite the fact 40% prefer this as a consultation method.

Presenting thoughts for the future, Deirdre stated PAE's advocacy efforts would be directed towards three key areas of focus. First, ensuring that pain becomes a key health quality indicator, second, that care pathways are implemented to support people living with pain, and finally, to improve health literacy and raise awareness of pain to empower people to seek help before it becomes chronic. Deirdre stated that it is important to ensure that EU and national level legislation remains relevant, participation in EU funded research, the continuous gathering of the patient voice in surveys and to increase leverage in under-represented countries through the continuous search for new partners.

Deirdre's key-takeaway messages are:

1. Policy position papers need to be constructed on a thoroughly considered basis of key contributors, key stakeholders, and key policy areas.
2. Health literacy on pain is insufficient amongst patients and policymakers. It can be increased through greater collaboration between national and European patient organizations.
3. Continuous engagement with both the policy environment and partnering with patient organizations representing similar interests is key to increasing leverage at an EU-level.

Gary Surmay (Senior Director, Corporate Affairs, Internal Medicine | Pfizer)
Concluding remarks

To conclude, Gary thanked participants for their attendance, and summarised key messages delivered across the three Summit sessions. First, Gary reminded the audience that patient involvement in HTA is important as it ensures patient relevant parameters are used when judging product efficacy during pricing and reimbursement processes. HTA is a critical

tool in helping healthcare policy decision-makers to determine the added therapeutic value of a product, and whether it should be covered by a national health system or insurer. He reminded participants that the EU-wide HTA aims to further integrate the patient voice into HTA processes and that specific provisions for stakeholder involvement will be published in implementing acts by the European Commission in early 2022. There are predictions that the model for patient involvement in an EU-wide HTA will mirror that of patient involvement in the European Medicines Agency.

To provide continuation from 2019's Advocacy Summit, Gary reminded participants that the patient voice is also important in healthcare policy decision-making more broadly as it ensures patient experience is at the core of decisions relating to healthcare delivery, access, quality of care and health equity. The ongoing development of the European Health Union provides a huge opportunity for the patient voice to shape the future of European health policy.

Gary went on to explain how we had learned across the three Summit sessions that there are different methods for patient involvement in both HTA and EU health policy decision-making. How participants had heard examples of how to build successful advocacy strategies, including working with, and learning from, sister communities, and empowering advocates with toolkits and programmes. That participants also learned about steps that can be taken to ensure impactful involvement in HTA, including integration into early assessments, one-on-one or group conversations with their HTA body, and using social media to generate evidence data.

Gary also reflected on the feedback received from participants throughout the Summit sessions. He reminded participants that we had heard from them that there are challenges to being involved in both HTA and EU health policy decision making. This includes lack of clear information, awareness of opportunities for engagement, and lack of training opportunities.

Finally, Gary concluded by reminding participants that we should never underestimate the power of experience **-** patients are the only stakeholders in healthcare policy decision-making that have first-hand experience and knowledge of living with a disease.

4. Next steps

Looking specifically at Europe's healthcare policy landscape, we anticipate the following activities in 2022 that may provide opportunities for further patient involvement:

1. **Healthcare will remain a top priority for EU-wide policymakers as they strive to build Europe's first ever 'Health Union'.** This historical project aims to better protect the health of citizens, equip Member States to prevent and address future pandemics, and improve the resilience of Europe's healthcare systems. PAGs have an important opportunity to shape ongoing conversations on the development of the Health Union and ensure the patient perspective is included.
2. **The publication of the EU-wide HTA implementing acts by European institutions**. This will include the procedural rules for stakeholder engagement and patient involvement within the process. Specifically, it should make the criteria for patients to be consulted as stakeholders clear. It will be important to closely monitor the publication of these texts and ensure that PAGs remain informed on how they can access and contribute to the process.
3. **Beginning of three-year transition period between the adoption of the new EU-wide HTA Regulation and its application at EU-level.** PAGs will have three-years to prepare for the EU-wide HTA legislation coming into force. With support, this is a window of opportunity for PAGs to get to know the details of the process and develop their skills for effective engagement.
4. **EUNetHTA21, a new HTA consortium, will launch the first open call to pharmaceutical companies to participate in upcoming Joint Scientific Consultations**. HTA stakeholders are already organizing for upcoming joint work. Joint Scientific Consultations will be conducted between EMA and HTA bodies in January 2022, with the EUnetHTA21 stakeholder kick-off meeting to be held online on 3 December. The call is part of the consortium's work to prepare for the application of the new HTA regulation aimed at increasing EU-wide collaboration in this area. Overall, these consultations will occur in parallel to EMA scientific advice. The EUnetHTA21 consortium will run for 24 months,

focusing on methodological issues to support joint HTA work and EU cooperation, for example, developing HTA methodology to be applied to joint clinical assessments and joint scientific consultations. Whilst PAGs are not directly impacted by this call, it is likely EUNetHTA21 will publish calls relating to PAG involvement in the near future. EUNetHTA will remain a key actor facilitating relations between various HTA stakeholders, including patients.

5. Conclusion

Feedback from PAG participants, has demonstrated that this capability building training program has helped PAGs to better understand how the patient voice is valued in healthcare policy decision-making and HTA, opportunities for patient involvement and next steps.

However, given the complex nature of Europe's health policy landscape, and its continued evolution in 2022 and beyond, it can be difficult for groups to stay abreast of new policy developments and maintain and strengthen their technical capacity to participate without regular training opportunities.

At the end of this year's Summit, we asked PAG participants what tools they need to move forward on their patient involvement journey. There was a strong focus on the need for more and regular training opportunities by public affairs experts, with a focus on developing communications capabilities such as effective use of media and social media, how to campaign, how to draft position papers and policy materials and how to present. There was also a desire for more information on HTA processes, particularly with the upcoming implementation of the EU-wide HTA.