

## Commentary

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### Author for correspondence:

Giovanni Tafuri,

E-mail: [gtafuri@zinl.nl](mailto:gtafuri@zinl.nl);

[Admin\\_EUNETHTA@zinl.nl](mailto:Admin_EUNETHTA@zinl.nl)

# The fourth edition of the European Network for Health Technology Assessment Forum: highlights and outcomes

Giovanni Tafuri<sup>1</sup> , Chantal BÉlorgey<sup>2</sup>, Carlo Favaretti<sup>3,4</sup>, Edith Frénoy<sup>5</sup>, Flora Giorgio<sup>6</sup>, Marcus Guardian<sup>1</sup>, Ansgar Hebborn<sup>7</sup>, François Houÿez<sup>8</sup>, Jessica Imbert<sup>9</sup>, Denis Lacombe<sup>10</sup>, Jordi Llinares Garcia<sup>11</sup>, Zoe Garrett<sup>12</sup>, Elena Petelos<sup>3,13,14,15</sup> , Ingvil Saeterdal<sup>16</sup>, Ad Schuurman<sup>1</sup>, Anna Lefevre Skjöldebrand<sup>17</sup>, Valentina Strammiello<sup>18</sup>, Daniel Widmer<sup>19</sup>, Anne Willemsen<sup>1</sup> and Niklas Hedberg<sup>20</sup>

<sup>1</sup>Zorginstituut Nederland (ZIN), Willem Dudokhof 1, 1112 ZA Diemen, The Netherlands; <sup>2</sup>Haute Autorité de Santé (HAS), 5 Avenue du Stade de France, 93210 Saint-Denis, France; <sup>3</sup>European Public Health Association (EUPHA), Otterstraat 118, 3513 CR Utrecht, The Netherlands; <sup>4</sup>Centre on Leadership in Medicine, Catholic University of the Sacred Heart, Largo F. Vito 1, 00168 Rome, Italy; <sup>5</sup>European Federation of Pharmaceutical Industries and Associations (EFPIA), Rue du Trône, 1050 Ixelles, Belgium; <sup>6</sup>European Commission, Directorate General for Health and Food Safety, Rue Breydel 4, 1000 Brussels, Belgium; <sup>7</sup>F. Hoffmann-La Roche AG, Pharmaceutical Division, Basel, Switzerland; <sup>8</sup>European Organisation for Rare Diseases (Eurordis), 96 rue Didot, 75014 Paris, France; <sup>9</sup>MedTech Europe, Rue Joseph II 40, 1000 Brussels, Belgium; <sup>10</sup>European Organisation for Research and Treatment of Cancer (EORTC), Avenue E. Mounier 83, 1200 Brussels, Belgium; <sup>11</sup>European Medicines Agency (EMA), Domenico Scarlattilaan 6, 1083 HN Amsterdam, The Netherlands; <sup>12</sup>National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester, M1 4BT; <sup>13</sup>European Forum for Primary Care (EFPC), Otterstraat 118, 3513 CR Utrecht, The Netherlands; <sup>14</sup>Clinic of Social and Family Medicine, Faculty of Medicine, University of Crete, Voutes University Campus, 70013 Heraklion Crete, Greece; <sup>15</sup>Health Services Research Department, Care and Public Health Research Institute (CAPHRI), Faculty of Health, Medicine and Life Sciences, Maastricht University, Minderbroedersberg 4-6, 6211 LK Maastricht, The Netherlands; <sup>16</sup>Norwegian Institute of Public Health (NIPH), PO Box 222 Skøyen, 0213 Oslo, Norway; <sup>17</sup>Swedish Medtech, Sveavägen 63, 113 59 Stockholm, Sweden; <sup>18</sup>European Patients' Forum (EPF), Chaussée d'Etterbeek 180, 1040 Etterbeek, Belgium; <sup>19</sup>European Union of General Practitioners (UEMO), Rue des Deux Églises 37-39, 1000 Brussels, Belgium and <sup>20</sup>Tandvårds & Läkemedelsförmånsverket (TLV), Fleminggatan 18, 112 26 Stockholm, Sweden

The European Network for Health Technology Assessment (EUnetHTA) organizes an annual Forum with stakeholders to receive feedback on its activities, processes, and outputs produced. The fourth edition of the EUnetHTA Forum brought together representatives of HTA bodies, patient organizations, healthcare professionals (HCPs), academia, payers, regulators, and industry. The aim of this paper is to provide an overview of the highlights presented at the 2019 EUnetHTA Forum, reporting the main items and themes discussed in the plenary panel and breakout sessions. The leading topic was the concept of unmet medical need seen from different stakeholders' perspectives. Breakout sessions covered the joint production of assessment reports and engagement with payers, patients, and HCPs. Synergies, pragmatism, and inclusiveness across Member States and stakeholders were emphasized as leading factors to put in place a collaboration that serves the interest of patients and public health in a truly European spirit.

The European Network for Health Technology Assessment (EUnetHTA) organizes an annual Forum with stakeholders to receive feedback on its activities, processes, and outputs produced. This is an opportunity for a diverse network of stakeholders such as healthcare professionals (HCPs), industry, academia, patients, and payer organizations to engage into an open dialogue with representatives from the EUnetHTA Executive Board and the different Work Packages. Participation is free, not restricted to invitations and open to all interested parties.

For its fourth edition, on 11 April 2019 in Amsterdam, the Forum brought together 220 participants. Participants included representatives of HTA bodies, patient organizations, HCPs, academia, payers, regulators, and industry (see [Table 1](#) for a thorough description of participants).

The Forum included a plenary panel, whose primary objective was to engage in a multi-stakeholder discussion on the concept of unmet medical need, and five breakout sessions, structured as facilitated discussions allowing groups of different stakeholders to engage in debates on topics related to EUnetHTA activities of interest.

The aim of this paper is to provide an overview of the highlights presented at the 2019 EUnetHTA Forum in order to transparently share its contents more broadly, reporting the

**Table 1.** EUnetHTA Forum participants

Affiliation	Number of representatives
European HTA body	75
Pharmaceutical company	38
Consulting company	25
Academia	22
Medical device/diagnostics manufacturer	16
Patient organization	10
Non for-profit organization	7
Hospital	7
HCP organization	5
Regulatory agency	3
Payer organization	3
European Commission	2
Other	7
Total	220

main items and themes discussed in the plenary panel and breakout sessions (see Table 2 for key outcomes of the Forum).

## Methods

Agenda items were identified following a process of stakeholder consultation in which EUnetHTA received advice on topics of potential interest from various parties. This resulted in the topic of the concept of unmet medical need as well as the five breakout sessions which focused on the interaction EUnetHTA established with each single stakeholder (patients, HCPs, payers, pharmaceutical, and medical device industry). Breakout sessions were held in parallel and their attendance was predefined based on the affiliation of the Forum participants. For each breakout session two moderators were identified to facilitate the discussion and reported a summary of the main outcomes to the plenary panel. In order to balance the discussion, one moderator was always a representative of the respective stakeholder group and one from EUnetHTA.

## The Concept of Unmet Medical Need: Views and Perspectives from Different Stakeholders

The concept of unmet medical need has important implications for different stakeholders and plays a major role in the priority setting of health interventions. For instance, the level of prioritization and resources public healthcare systems allocate to rare diseases, as opposed to more widespread diseases affecting much larger populations, can greatly vary among European countries (1).

However, its interpretation and definition may vary across stakeholders.

A multi-stakeholder panel discussed the varying interpretations of such concept. The eight panelists reflected a wide range of relevant stakeholders: Health Technology Assessment bodies, regulators, patients, the European Commission, payers, HCPs, and industry. Panelists were affiliated to: the French Haute Autorité de Santé (HAS), the European Medicines Agency, the European Patients' Forum, DG Santé of the European Commission (EC), the

European Social Insurance Platform, the European Forum for Primary Care and the European Public Health Association, the European Federation of Pharmaceutical Industries and Associations, and the trade association for medical device companies in Sweden (Swedish Medtech).

The aim of the panel was to provide an opportunity for enriching the dialogue on the topic of unmet medical need and to create a common understanding of the challenges related to its interpretation.

The European Union (EU) legislation (Regulation 726/2004, article 14a) defines unmet medical need as “a condition for which there exists no satisfactory method of diagnosis, prevention or treatment authorised in the Union or, even if such a method exists, in relation to which the medicinal product concerned will be of major therapeutic advantage to those affected.” It was generally acknowledged by the panel that this definition is extremely broad and can be subject to varying interpretations.

The EC proposal on HTA cooperation at the EU level originally foresaw that unmet medical need would be one of the criteria for identifying medicinal products for joint assessment during the transitional period (2). However, the discussions and negotiations on the text of the proposal – including this point – at the Council of the European Union and European Parliament are still ongoing.

At the regulatory level, the unmet medical need of pharmaceuticals is a criterion for eligibility to enter procedures such as conditional marketing authorization and accelerated assessment and it is used for prioritization of products within the EMA's Priority Medicines (PRIME) scheme (3). The presence of an unmet medical need has to be carefully evaluated by regulators especially when it is related to a subgroup of patients, for example characterized by a specific biomarker, because this may result in a “fragmentation” of diseases for which therapeutic alternatives may exist. This is particularly important because the recognition of an unmet medical need by regulators is likely to have an impact on downstream stakeholders such as HTA bodies and payers.

When applying unmet medical need, regulators use it as a binary concept whereas other stakeholders, such as HTA bodies or payers, may need to quantify the degree of unmet medical need using a scale (e.g., major, moderate, and minor).

At the HTA level in France, unmet need is not considered as a stand-alone criterion and it is one of the elements taken into account in the assessment of the clinical benefit. In addition, in the French system the existence of an inadequately met medical need is one of the elements providing eligibility for the early access programs of medicines such as the compassionate use program (ATU), the “*Forfait Innovation Package*” for medical devices or the RIHN programme (*Référentiel des actes Innovants Hors Nomenclature de biologie et d'anatomopathologie*) which particularly concerns *in-vitro* diagnostic tests (4).

A health technology addressing an unmet medical need can also be eligible for procedures of consultation between manufacturers and HAS during the clinical development (national early dialogues [ED]) as well for fast track assessment and appraisal by HAS for the decisions on pricing and reimbursement which will be taken by the French Ministry of Health.

Furthermore, in France, some technologies addressing an unmet medical need might – in case of major uncertainties – exceptionally receive conditional and temporary appraisal on condition that predefined post-marketing studies will tackle the evidence gaps that exist for the product. Early reassessments will then either

**Table 2.** Highlights

Session	Key outcomes and highlights
Panel discussion on unmet medical need	<ul style="list-style-type: none"> <li>• Discussion facilitated mutual understanding of different stakeholders' perspectives</li> <li>• Unmet medical need is crucial for prioritization and resource allocation as well as an eligibility criterion for regulatory procedures and special appraisal processes for reimbursement</li> <li>• Decisions about what constitutes an unmet medical need should be transparently communicated to the patient community and HCPs</li> </ul>
Breakout session on EUnetHTA JA (for pharmaceuticals and other technologies)	<ul style="list-style-type: none"> <li>• Timely availability of the EUnetHTA JA reports is considered critical</li> <li>• Most HTA bodies complemented the report with additional analyses (e.g., country specific), although some simply translated the reports in their local language without any changes</li> <li>• More transparency about data used, assumptions, and decisions was requested</li> <li>• EUnetHTA methodologies need to keep pace with scientific advances</li> <li>• EUnetHTA needs to pragmatically design a structure that fits the majority of systems</li> </ul>
Breakout session on patient involvement	<ul style="list-style-type: none"> <li>• The recent EUnetHTA policy on conflict of interest poses the basis for further stakeholder engagements</li> <li>• A more robust measurement of the contribution provided by patients to EUnetHTA is needed</li> </ul>
Breakout session on HCP involvement	<ul style="list-style-type: none"> <li>• The HCP community is concerned about the uncertainties persisting at the time of marketing authorization and the lack of data on comparative effectiveness across treatments</li> <li>• The sole use of registries cannot address such uncertainties. Furthermore, difficulties for some Member States to collect Real World Data at the national level are recognized</li> <li>• EUnetHTA's PLEGs can be a relevant starting point</li> </ul>
Breakout session on payer involvement	<ul style="list-style-type: none"> <li>• There is general acknowledgment that the varying definition of payer with different roles and remits in the EU landscape generates obstacles in establishing stronger collaborations between payers and EUnetHTA</li> <li>• Despite such challenges, EUnetHTA and payers have been increasing their interactions and mutual exchanges during the current Joint Action</li> </ul>

BS, breakout session; HCP, healthcare professionals; JA, joint assessments.

confirm or revert the initial opinion, therefore as a consequence the initial decisions on reimbursement could change. It is worth noting that patients and HCPs should receive transparent information about those processes.

However, it was stressed that the concept of unmet medical need should not necessarily be associated with an automatic acceptance of uncertainties by HTA bodies and it should not imply that randomized clinical trials are unfeasible.

Similarly, for payers, unmet medical need is part of a multi-criteria decision making process which takes into account other aspects such as disease severity and the availability of therapeutic alternatives. Most importantly, from a payer perspective, for the purpose of conducting cost effectiveness and budget impact analysis, the population for which an unmet need is identified needs to be well defined, although some uncertainty at the time of introduction of a new intervention seems inevitable.

From a patient perspective, patients should be invited to indicate priorities based on their own perception of unmet medical needs. Furthermore, technologies need to be not only safe and effective, but also affordable and accessible in a timely manner. In general, a key element of value for a new technology is a substantial gain in terms of quality of life. Decision making processes to define the medical need, as well as regulatory and HTA evaluations, must be transparent and clearly communicated to the patient community.

Multi-stakeholder collaboration is crucial also from a pharmaceutical industry perspective, which supports the idea of more systematically including patient and citizen inputs – in particular, preferences regarding unmet medical needs – into drug development, HTA, reimbursement, and treatment decision making processes. According to the industry, tensions between decision makers with different interpretations of the existing unmet need

warrant more thoughtful alignment efforts informed by relevant evidence. With time, this would also lead to a more predictable environment for the development of innovative therapies.

It is worth mentioning that the industry perspective may vary between pharmaceutical and device manufacturers. Indeed, substantial differences exist in terms of regulation, business models, investment requirements, and timelines, as well as in the interpretation of the concept of unmet medical need. The definition provided in the EU legislation is expressly focused on medicinal products<sup>1</sup> which gives rise to the need to broaden the concept of *unmet medical need* for devices. Indeed, the focus on the *diagnosis, prevention, or treatment* aspects in the definition excludes unmet *patient* needs that many medical devices, such as assistive devices, address and that not only relate to body functions, but can also facilitate participation in the everyday life. Furthermore, a new technology for early diagnosis can potentially prevent disease progression or disease onset and can therefore be resource saving for the healthcare system. A new technology can also address unmet societal needs, for example allowing a decentralization from specialized traditional healthcare venues to more sustainable healthcare models outside of the hospital. In conclusion, when it comes to a prioritization situation it may be worthwhile considering not only the unmet *medical* needs, but also the unmet *patient, health care, and societal* needs, for which medical devices may offer solutions.

In Sweden, structured systems for managed introduction of new medicines have been in place for over 10 years at a county/

<sup>1</sup>Any substance or combination of substances which may be used in or administered to human beings either with a view to restoring, correcting, or modifying physiological functions by exerting a pharmacological, immunological, or metabolic action, or to making a medical diagnosis.

regional level and more recently at a national level for hospital drugs. Similarly, a new structure aiming at a managed introduction of medical devices is currently being established. In general, prioritization is based on three key principles: the equal human rights (principle of human dignity), unmet patient need and societal need (principle of need and solidarity), and the unmet healthcare need (principle of cost-effectiveness).

European HCPs elaborated on multiple considerations from the public health perspective, with emphasis on new technologies and the need to consider quality data and evidence generation. The need to move from specialized conventional healthcare service delivery settings to more sustainable healthcare models beyond tertiary care was mentioned. Indeed, emphasis has been placed globally on the importance of quality primary and public health services, as well as on the availability and accessibility of health technologies (as captured in the Sustainable Development Goal 3, i.e., to ensure healthy lives and promote well-being for all at all ages) (5). Unmet medical need largely also pertains to access and affordability and HTA is a key decision making tool to achieve Universal Health Coverage (sub-goal of 3.8 of SDG3) (6).

Similar to other stakeholders, the HCP community advocates conceptual and operational alignment, the need for more systematic efforts to address the heterogeneity in definitions and approaches for qualifying and quantifying unmet need and public health impact. Again, transparency in exchanges and decision making practices, in what needs to be assessed and prioritized, both in terms of methodologies and of policies, is perceived as highly needed.

In particular, unmet medical need is considered crucial for prioritization purposes, with examination of such considerations starting as early as possible, as part of horizon scanning. Multi-stakeholder dialogue is again perceived as essential, in particular at the level of primary care and as part of a continuum of care with regular exchanges and dialogue between HCPs, patients, and carers. New technologies were highlighted as means for wider involvement of the community in deliberative processes, both in terms of prioritization and for determining value, and to safeguard fair and legitimate decisions.

### Breakout Sessions

Breakout session aimed to receive feedback on the activities of the consortium, identify challenges, and possible avenues for improvement.

### Uptake of EUnetHTA Joint Assessments for Pharmaceuticals at a National Level

Overall, 116 participants joined the breakout session (see Supplementary Table 1 for details about the session composition). The purpose of the session was to explore how EUnetHTA pharmaceutical Joint Assessments (JA), also known as joint Relative Effectiveness Assessments (REAs), play a role at the national level, presenting the perspective of EUnetHTA partners and manufacturers. At the time of the Forum, EUnetHTA had published three pharmaceutical JA, however several others were in preparation. The discussion focused on the use of the assessment reports at the national level and whether the information provided in the reports can satisfy the needs of the national HTA bodies.

The discussion showed several topics that influence usability of a JA at the national level: timely availability, relevance of the

report, transparency of information used and decisions made, and methodologies used.

Several participants of the Forum stressed that timely availability of the EUnetHTA JA reports is critical to ensure implementation at the national level. Currently, EUnetHTA is able to publish the report around 3 weeks after the European Public Assessment Report (EPAR) is available.

Participants from both the industry as well as national HTA bodies stated that previous examples showed misalignment between countries and reports were perceived to reflect solely the work of the country acting as first author. EUnetHTA attempted to tackle this issue, by incorporating a so-called “PICO-survey” for all ongoing and future assessments. This survey enables all Member States to reflect on the relevance of the suggested PICO.

HTA bodies requested EUnetHTA to be more transparent about the data used, assumptions, and decisions made. It was argued that earlier access to company submissions is needed to facilitate de-duplication of joint and national assessment work and to support national HTA bodies to work in parallel on the Health Economic modeling. Some HTA bodies may already require less information in national submissions if the topic is being assessed by EUnetHTA.

The content of the EUnetHTA assessment reports is strongly linked to the EUnetHTA methodological guidelines. However, participants underlined that current EUnetHTA methodologies tend to be quickly outdated and need to keep pace with scientific advances. Stronger engagements with academic institutions were advocated by some participants as a platform for debate and scientific exchange.

The importance of linking the work of EUnetHTA to initiatives of joint pricing and reimbursement, such as BeNeLuxA, the Valletta Declaration, and Finose, was discussed. Of note, it was highlighted that the Finose collaboration (between Finland, Norway, and Sweden) has been adopting the EUnetHTA templates and intends to use the EUnetHTA JA for joint negotiations with no duplication of the clinical assessment.

In summary, HTA cooperation is perceived to have the potential to streamline administrative processes and to support timely decision making about patient access to innovative treatments. However, the discussion confirmed that there is no “one-size fits all” approach for use, as this depends on the healthcare system of each country. To achieve the ultimate goal of European joint clinical assessments that are not duplicated and fully integrated in national HTA processes, EUnetHTA needs to pragmatically design a structure that fits the majority of systems. Where possible flexibility should be built in to allow it to accommodate special situations.

### Impact of EUnetHTA Joint Assessments for Other Technologies at a National Level

Overall, 103 participants joined the breakout session (see Supplementary Table 1 for details about the session composition). In line with the previously described session, the aim was to describe the experience with EUnetHTA collaborative assessments from the perspective of EUnetHTA partners and manufacturers. At the time of the Forum, EUnetHTA had published eleven assessments on other technologies (OT) and a further eight were ongoing. Other technologies include non-pharmaceutical technologies, that is, screening technologies, medical and surgical interventions, and diagnostic technologies. As of March 2019, ninety-six uses of the OT assessments had been



reported in total, forty-six were used in national assessment procedures, and fifty-two uses were sharing via dissemination. It emerged that most HTA bodies complemented the report with additional analyses such as budget impact analysis or cost effectiveness analysis, although some simply translated the reports in their local language without any changes. Timing was again identified as a potential hurdle to local use, because EUnetHTA assessments for other technologies may not be aligned with national timelines. Reasons for non-use are also related to the language and to the relevance of the products. EUnetHTA is currently developing recommendations to select products of common interest for the production of JA through its “Task Group on Topic Identification, Selection and Prioritisation” (TISP) (7). Collaboration on EUnetHTA Joint Assessments was seen as a basis to create future collaboration on HTA in Europe, build trust among HTA bodies and potentially save resources by avoiding duplication of work.

### Lessons Learned and Avenues for the Improvement of Patient Involvement in EUnetHTA Processes

This breakout session, focusing on the activities of the EUnetHTA task groups concerning patient engagement, was attended by seventy-three participants (see Supplementary Table 1 for details about the session composition). A recent key EUnetHTA achievement was the finalization of its policy on conflict of interest, which is now publicly available on the official portal (8). This policy posed the basis for further stakeholder engagements, defining a consistent and transparent decision making process for the identification of a conflict. In terms of levels of patient engagement in the EUnetHTA procedures it was reported that at the time of the Forum in thirteen out of seventeen ED and in sixteen out of twenty-six joint clinical assessments patients/patient representatives had been successfully involved in EUnetHTA procedures. This showed an overall patient involvement of about 75 percent for ED and about 60 percent for the joint/collaborative assessments. Discussion also focused on a specific EUnetHTA guidance explaining the different methods to collect patient inputs during the process of REA production, on which the patient community was asked to contribute and is now publicly available (9). A focus on the quality of the EUnetHTA Joint Assessments, in relation to the patient engagement process, was brought in the discussion. A more robust measurement of the contribution and the added value provided by patients along the HTA work is indeed needed.

### Challenges in the Interaction between EUnetHTA and Payer

The focus of this session, attended by seventy-five participants (see Supplementary Table 1 for details about the session composition) was the challenges encountered in including payers' inputs in the current EUnetHTA activities. The starting point of the discussion was the varying definition of payer with different roles and remits in the EU landscape. Indeed, in Europe, based on the different organizations of national healthcare systems, payers may either be identified as Ministries of Health or regional bodies, but also private or public insurers. In addition, in a single Member State, different types of payers may co-exist. This heterogeneity and complexity generate obstacles in establishing stronger collaborations between payers and EUnetHTA, also in light of the fact that the relationship between national HTA bodies and payers varies across the EU Member

States. However, although such challenges as well as the need to better define remits and characteristics of payers were recognized by participants, it was acknowledged that EUnetHTA and payers have been increasing their interactions through regular meetings and opportunities for mutual exchange during the current Joint Action.

### Bridging EUnetHTA and Healthcare Professionals

This session was attended by seventy-two participants (see Supplementary Table 1 for details about the session composition). The underlying question that it tried to address was how EUnetHTA deliverables –JA, ED, Post Launch Evidence Generation (PLEG) – can contribute to address the uncertainties faced in clinical practice by HCPs and on the role and engagement of HCPs in the EUnetHTA processes.

The discussion focused on the uncertainties that both HTA bodies and ultimately HCPs need to deal with following regulatory approvals. Expedited regulatory procedures, the use of surrogate end points, the lack of data on quality of life, and limited long-term outcome data are some of the most recurring issues that downstream decision makers need to face. In particular, the potential failure to confirm clinical benefits during the post-authorization stage is a key concern regarding early access and conditional approvals for new medicines. This topic has been largely debated within the HTA and payer communities (10–12).

Limited knowledge on appropriate use of medicines, on their optimal sequencing, combination, and duration of treatment persist at the time of marketing authorization. Sub-optimal administration of costly treatments may generate unnecessary toxicity for the patients and negatively affect national healthcare budgets. Moderators from the European Organisation for Research and Treatment of Cancer (EORTC) and the European Union of General Practitioners (UEMO) emphasized the importance of comparative effectiveness, although it was acknowledged that this is not within the remits of the EU regulators.

EUnetHTA can play an important role in this respect, facilitating a process of mutual exchanges and understanding between HTA bodies and regulators, as part of its ongoing work plan with EMA (13). In addition, procedures of parallel consultation, during which manufacturers can receive simultaneous feedback from regulators and HTA bodies on the clinical development and in which HCPs and patients can actively be involved, seemed to be a good opportunity to bridge the evidence gaps between the HTA and the regulatory communities.

The discussion then focused on post-marketing data collection and on the use of Real World Data. Representatives from EORTC and UEMO did not support the idea that the sole use of registries can address the uncertainties and unsolved issues existing at the time of marketing authorization. Furthermore, difficulties for some Member States to collect Real World Data at the national level are recognized. It was discussed how EUnetHTA's PLEGs, which aim at defining common data sets for post-marketing data collection for HTA bodies across different Member States, can be a relevant starting point in this respect.

HCPs represent a key stakeholder for EUnetHTA. The recently established EUnetHTA policy on conflict of interest is crucial for such engagement (8). Of note, it foresees – under exceptional circumstances (e.g., lack of experts with no prior industry involvement due to the rarity of a disease) – the possibility to consult with experts with an existing conflict exclusively on a predefined

set of questions elaborated by EUnetHTA and without providing access to confidential documentation.

## Conclusions

The fourth edition of the EUnetHTA Forum brought together HTA bodies, regulators, payers, industry representatives, patients, and patient advocates, as well as researchers and HCPs to share experiences, exchange information, and most importantly contribute to shape the Network in a way that best addresses the needs of patients and safeguards the interest of public health. The wide range of expert opinions and the high attendance provided opportunity for EUnetHTA to discuss processes and outcomes and to identify avenues for improvement.

The Forum has set up the basis for a continuing discussion on the concept of unmet medical need. The relevance of this topic has been confirmed by a recent review which identified sixteen different definitions used across different stakeholders (14). Overall, discussions on the different topics covered during this edition – joint production of assessment reports, engagement with payers, patients, and HCPs – emphasized the importance of synergies, pragmatism, and inclusiveness across Member States and stakeholders to put in place a collaboration that serves the interest of patients and public health in a truly European spirit.

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