Event Report

Preparing to engage in your first JCA: What the ATMP pipeline means for you

21 October 2025

On October 21, 2025, the *Alliance for Regenerative Medicine* (ARM), *Cancer Patients Europe* (CPE), *Fondazione Telethon,* and the *European Haematology Association (EHA)* co-hosted the third in a continuing series of online discussions on the implementation of the EU Health Technology Assessment (HTA) Regulation and its new Joint Clinical Assessment (JCA) framework for Advanced Therapy Medicinal Products (ATMPs).

The session, titled '***Preparing to engage in your firsts JCA: What the ATMP pipeline means for you',*** gathered 37 representatives from 25+ organisations – including patient organisations, medical societies and developers – for a forward-looking discussion on early experiences from the first JCAs, and how the ATMP community can prepare for those to come.

This event followed two previous joint discussions in 2024 and early 2025 that established an informal advocacy platform around flexibility in evidence requirements within assessments and ensuring meaningful patient and clinician involvement in the JCA.

The latest discussion built on that foundation to:

- **Provide insights on the ATMP pipeline** likely to undergo the JCA in the coming years
- **Share early learnings and hear from stakeholders** that have already or are preparing to engage in the JCA process
- **Raise awareness of stakeholder roles** under the EU HTA Regulation
- **Strengthen patient and clinical experts' readiness** to engage effectively in upcoming JCAs
- **Collaborate on solutions** and shape our shared advocacy for 2026 and beyond

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Setting the scene: the first JCAs in 2025: why it matters?

Paolo Morgese, *Vice President of Public Affairs Europe, ARM*

Paolo Morgese set the scene by highlighting that 2025 marks the first operational year of the EU HTA Regulation, which introduced the Joint Clinical Assessment (JCA) for ATMPs and oncology products. These first assessment will shape how Europe evaluates clinical evidence for innovative therapies and will set precedents that determine whether the new framework will help patients gain faster access to advanced treatments.

The JCA framework was conceived to make access to innovation faster, more predictable, and more coherent across Member States**,** reducing duplication and streamlining the work of developers and national HTA bodies. Its success, however, will depend on whether implementation remains pragmatic and proportionate.

"We do not need more duplication; we need systems that assess which drugs benefit patients in the most efficient way possible."

Reflecting on the advocacy milestones achieved since 2024, **Paolo** highlighted the joint work accomplished:

- **Joint Call to Action** garnering endorsements from 40 leading patient organisations, medical societies, foundations, and developer networks, that urged the HTA Coordination Group (HTA CG) and JCA assessors to adopt a pragmatic approach when assessing ATMPs, one that recognises all types of available evidence and uses the JCA report to describe rather than judge any resulting uncertainty.

- **ARM's CEO Letter to the European Commission,** a joint statement from leading executives across Europe's advanced therapy sector. The letter called for Europe to match its scientific excellence with a policy environment that supports development, access, and investments, urging greater alignment across regulatory and reimbursement frameworks and close coordination between the JCA, the upcoming Biotech Act, and the Life Sciences Strategy to create a coherent pathway from discovery to delivery.

Looking ahead to 2026, **the co-hosts** called for continued collaboration and joint advocacy among stakeholders to ensure the JCA fulfils its purpose as a key enabler, not a barrier, to innovation and access. The priority, he concluded, must be to make the process work for patients, not against them.

The ATMP pipeline: Which indications are being investigated?

Lydia Shotton, *Associate Director Government Affairs Europe, ARM*

Lydia Shotton presented an overview and early insights into the evolving ATMP clinical trial pipeline from an ARM horizon-scanning exercise currently being conducted based on publicly available data.

As a member of the HTA Stakeholder Network, ARM launched this analysis in response to repeated calls from the HTA Coordination Group (HTACG) for improved visibility into upcoming assessments. The objective is to support shared preparedness among developers, patients, clinicians, and assessors ahead of the growing wave of JCAs expected over the next few years.

The analysis identifies nearly 1,000 ongoing clinical trials across the United States and Europe, signalling unprecedented momentum in the field and a significant number of potential JCA submissions in the near term. Because the United States remains a key predictor for the products that will later enter the European market, ARM included US trial data to provide a realistic indication of which therapies are likely to reach Europe in the coming years.

While Europe remains a major contributor to global ATMP development, activity levels vary widely across Member States. When measured per capita, smaller and research-intensive countries such as Denmark and Belgium lead Europe in clinical trial activity, followed by the Nordic and Benelux regions. Larger markets such as Germany, France, and Italy, despite their size and resources, currently lag behind these frontrunners.

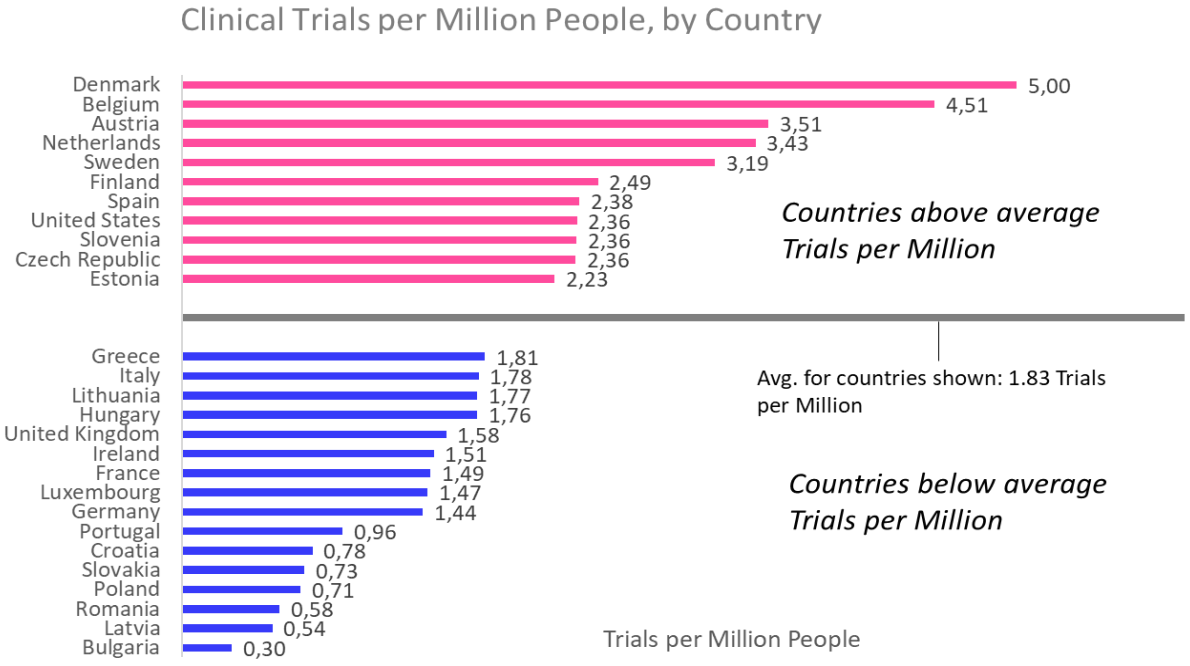

Figure 1*: Clinical Trials per Million People, by Country.* ***Source:*** *ARM's own elaboration based on publicly available data.*

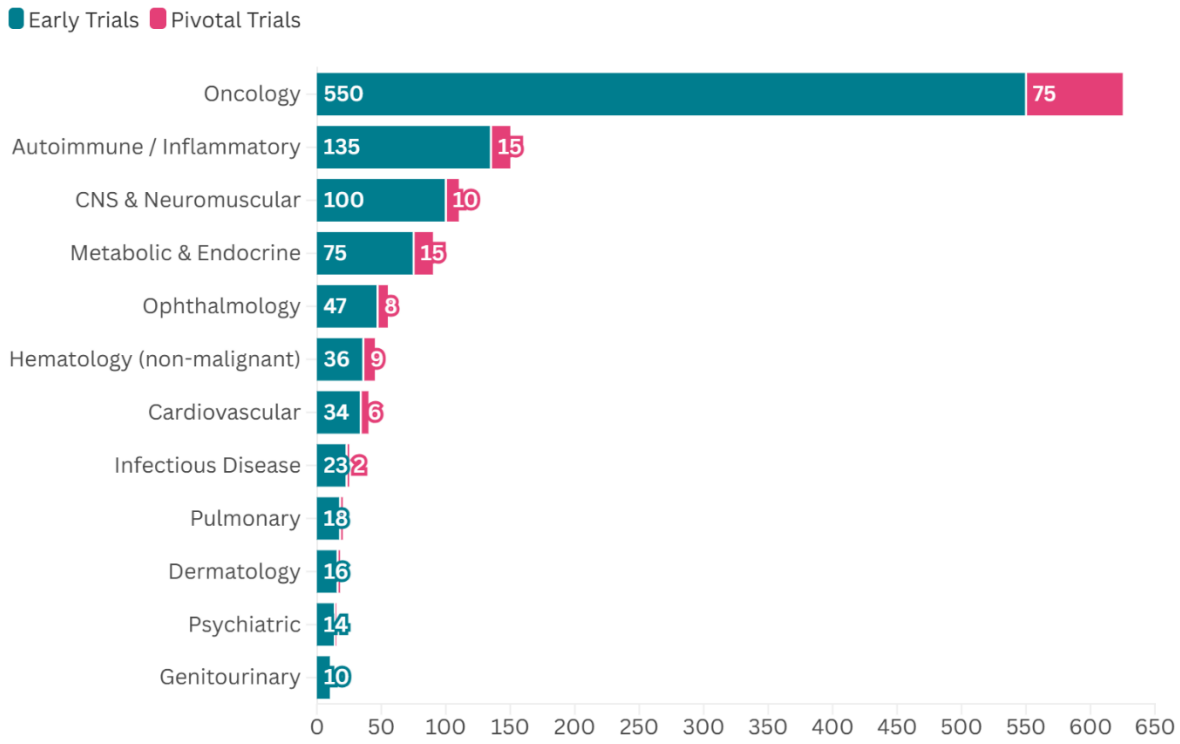

Figure 2. *Early vs Pivotal Trials by Therapeutic Area.* ***Source:*** *ARM's own elaboration based on publicly available data.*

Beyond geographical distribution, ARM's early mapping revealed a broad diversification across therapeutic areas, with ATMPs now being investigated in over 150 disease indications. Oncology remains by far the largest and most mature domain, dominated by haematological cancers but now expanding into solid tumours such as melanoma, lung, and gastric cancers. Other rapidly developing areas include autoimmune and inflammatory disorders, ophthalmology, and metabolic diseases, while early-stage work is growing in neuromuscular, cardiovascular, and infectious diseases.

Approximately two-thirds of current ATMP trials target rare diseases, but early-phase activity in more prevalent conditions is beginning to rise, marking a meaningful shift in the field's evolution. The data also show that the sector remains predominantly early-stage, with a ratio of seven to ten early trials for every pivotal trial, reflecting both the youth of the technology and the coming surge of future marketing applications.

To illustrate where future JCAs are likely to occur, ARM's analysis clustered indications according to the density of pivotal-stage research. Oncology, autoimmune, hematology, and ophthalmology emerged as the most advanced and JCA-ready areas, while other therapeutic classes are still maturing. Within these groups, leading indications include Graft Vs. Host Disease (GVHD), Lupus, and Scleroderma in autoimmune disorders, and Muscular Dystrophy, Multiple Sclerosis, and Parkinson's disease within the neuromuscular and central nervous system category (see Figure 3 for further details).

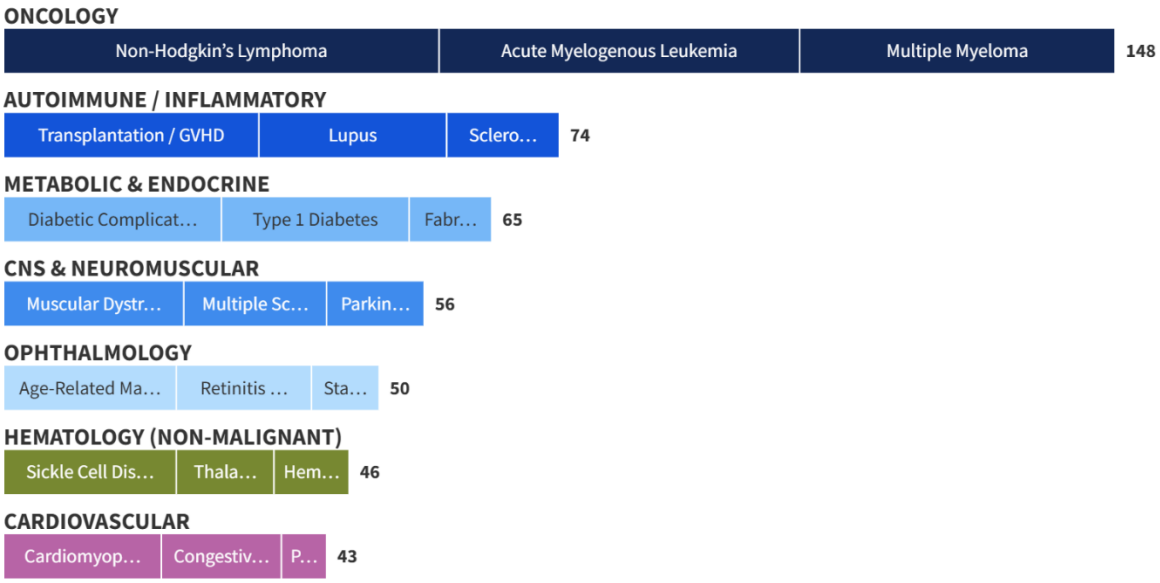

Figure 3. *Leading Indications within ATMP Front-Runners.*
Description: **Oncology**: Non-Hodgkin's Lymphoma, Acute Myelogenous Leukemia, Multiple Myeloma, **Autoimmune / Inflammatory:** Transplantation / GVHD, Lupus, Scleroderma; **Metabolic & Endocrine**: Diabetes complications, Type 1 Diabetes, Fabry Disease; **CNS & Neuromuscular**: Muscular Dystrophy, Multiple Sclerosis, Parkinson's Disease; **Ophthalmology:** Age-related macular degeneration, Retinitis Pigmentosa, Stargardt Disease; **Hematology:** Sickle Cell, Thalassemina, Hemophilia; **Cardiovascular:** Cardiomyopathy, Congestive Heart Failure, Peripheral Arterial Disease.
Source: *ARM's own elaboration based on publicly available data.*

Moderated discussion

Joséphine Mosset, *Senior Policy Officer, Cancer Patients Europe (CPE)*
Robin Doeswijk, *Head of European Affairs, European Hematology Association (EHA)*
Stefano Benvenuti, *Head of Public Affairs, Fondazione Telethon*

Tresja Bolt*, Executive Director, Harwood Levitt Consulting****,*** moderated a discussion centred around several key questions (in ***bold italics***) that explored early experiences from the first live nine JCAs, their practical implications, and what these lessons mean for system readiness.

Tresja Bolt (HLC): How are patient and clinical experts being engaged in practice, and what challenges have emerged so far?

Robin Doeswijk (EHA) explained that medical societies are beginning to play a critical role in identifying and nominating experts for JCAs. EHA had recently been asked by the European Commission to propose experts for the first haematology assessment. The process, however, has highlighted both opportunities and challenges.

"We were asked to nominate experts without knowing much about the disease scope. We can manage, but without clearer information and a pragmatic approach to conflicts of interest, it will be difficult to ensure the best expertise is represented."

He noted that while the HTA Secretariat is making a genuine effort to interact with stakeholder organisations, two recurring issues persist: conflicts of interest and the limited information shared with societies before nominations are due.

Joséphine Mosset (CPE) echoed these concerns. She described how CPE, as part of the HTA Stakeholder Network, had already received multiple "bulk requests" to identify patients for upcoming JCAs, often with little notice.

"The first request we received gave us less than a week to identify patients. The last one gave us three weeks, which was manageable. So it's definitely improving, but there's still work to do."

"One patient told me they had to keep their camera off and stay anonymous. Others said the questionnaires were so generic that anyone could fill them out. Patients need to feel that their contribution matters."

She also pointed to structural issues: once CPE nominates patients, they are no longer involved and receive no feedback or updates, leaving both the organisation and the selected patients without support. In some cases, patients felt their participation was impersonal or overly bureaucratic, with limited opportunity for meaningful input.

Finally, **Joséphine Mosset (CPE)** emphasised that language remains a key barrier: the entire process is currently conducted in English, which risks excluding patients from non-English-speaking countries.

"We believe that a process that is European in nature should also be European in languages"

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Tresja Bolt (HLC): Do you have any tips or advice on how to prepare your membership, or any observations on how to organise internally to make sure you are prepared to meet all the requests?

Robin Doeswijk (EHA) encouraged both medical societies and patient groups to put clear internal processes in place before being contacted by the HTA Secretariat. He advised that organisations define internal responsibilities, establish a contact point known to the Secretariat, and prepare expert groups in advance, as once they get approached they will have very little time to do so. Robin also added that motivation among experts will depend on helping them understand the importance of their contribution to the JCA process.

Laura Gumbert, Healthcare System and Access Manager, SMA Europe, echoed these concerns and described how SMA Europe has begun creating its own internal systems to guide and support members. The organisation established a dedicated webpage with HTA and JCA information and small group meetings to exchange experiences.

"We built a space where our community can share experiences and find information, but since we aren't told who has been selected, we can't properly support them – that shows how closed the process still is."

Adding to these reflections, **Dimitrios Athanasiou**, **Board member, World Duchenne Organisation**, urged patient organisations to **build pools of trained experts** who can be activated quickly and provide feedback internally while respecting confidentiality.

He added that although the patient input forms may appear over-simplistic, they still allow for substantial, evidence-based contribution if used effectively. In many cases, this means submitting detailed 20-page feedback documents, supported with the relevant publications, as evidence within the current system.

"We need our own champions – people trained and protected from conflicts, who can respond fast and share insights afterwards without breaching confidentiality."

Together, the participants agreed that readiness, transparency, and internal coordination will be key to ensuring that both patient and clinical experts can participate effectively in the JCA process and that their contributions are reflected meaningfully in future assessments.

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***Tresja Bolt (HLC):* Has the JCA process influenced the way developers engage with patient organisations?**

Stefano Benvenuti (Fondazione Telethon) explained that the JCA has prompted developers to rethink how they plan and structure patient engagement.

Within Fondazione Telethon, engagement efforts were initially concentrated in Italy or the small number of countries where trials were conducted, but they are increasingly recognising the need for a pan-European approach. This is because in the HTA process, developers must increasingly account for variation in standards of care and patient priorities across Member States when preparing evidence and defining PICOs.

"We realised we need to engage with patients across Europe, not just where our trials take place. Understanding national standards of care and what outcomes matter most to patients has become essential to the JCA."

Stefano Benvenuti (Fondazione Telethon) added that broadening this engagement is resource-intensive but critical to ensure that evidence-generation plans reflect the diversity of care settings across Europe and to avoid misalignment between development plans and HTA expectations.

Simon Dawson, **Country Director Nordics, Ultragenyx,** concurred that the JCA is reshaping how companies approach expert and patient involvement, particularly in ultra-rare diseases where the pool of qualified experts is small.

He emphasised that while early and continuous scientific dialogue with patient experts is indispensable for clinical development, it also raises questions around conflict of interest for future JSC and JCA participation.

"In ultra-rare conditions, most experts have already been involved in clinical trials, which make them ineligible as patient experts for the assessments.

It's a really difficult balance – we can't develop therapies without expert input, but we also risk excluding those same experts later in the process."

Simon Dawson (Ultragenyx) concluded that the solution will depend on closer collaboration between developers, the HTA Secretariat, and stakeholder groups to ensure expert input can continue without disqualifying valuable voices from assessment roles.

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***Tresja Bolt (HLC):* Do the current system's resources match the growing number and complexity of ATMP dossiers?**

Stefano Benvenuti (Fondazione Telethon) raised concerns that access to Joint Scientific Consultations (JSCs) may become a "lottery" due to the very limited number of available slots, especially compared to the volume of protocol assistance requests already seen at EMA. He stressed the need for alternative pathways to obtain scientific guidance for developers unable to access JSCs.

Robin Doeswijk (EHA) echoed these concerns from a clinical standpoint, noting again that while medical societies are actively trying to support the system, conflict of interest rules, tight timelines, and a lack of feedback mechanisms make this difficult. He emphasised that true success will depend on whether HTA bodies will free up the resources and show real willingness to make this collaborative mechanism work, and reiterated the importance of involving top clinical experts, even those with industry ties, to ensure credible assessments.

Joséphine Mosset (CPE) highlighted the resource constraints facing patient groups who are expected to contribute meaningfully to the process. She raised concerns about attitudes within some HTA bodies that prefer "regular patients" over trained advocates, a trend that risks sidelining patient expertise. To help address capacity issues, CPE has hired a dedicated staff member for HTA engagement and is working closely with other patient groups within the HTA Stakeholder Network to pool resources, share tools, and avoid duplication. To close the feedback gap that arises once patients are nominated, and the patient groups are locked out of the process, CPE is setting up a focus group to gather experiences from those involved and channel this back to the HTA Coordination Group. These efforts aim to build sustained, informed participation despite limited access to formal roles in the process.

***Tresja:* While developers and stakeholder organisations may be engaged at EU level, the responsibility for submitting PICO's ultimately sits with Member States. How are medical societies collaborating with national members around the definitions of PICOs?**

Robin Doeswijk (EHA) emphasised that fragmented comparator preferences across Member States risk undermining the efficiency and credibility of the assessments. While medical societies do not hold a formal role in shaping PICO preferences under the legislation, EHA is working through its European and national networks to support alignment on PICO definitions.

"We're still at the awareness-raising stage, but the aim is to help patient groups at national level understand the process and be ready to engage when the opportunity comes."

Drawing on established clinical guidelines and expert consensus within their national networks, EHA is proactively advising HTA subgroups on the appropriate comparators and outcomes to ensure national submissions reflect local realities while still promoting broader alignment.

Joséphine Mosset (CPE) noted that while CPE cannot be directly involved in all Member States, it is working to raise awareness and strengthen national-level readiness for PICO engagement. Many national patient groups, she explained, are still unfamiliar with HTA processes and need support to understand how the European and national dimensions interact.

CPE has also been organising information sessions and events in several countries to build understanding of the new EU HTA Regulation and how PICOs are defined. At the same time, it is collaborating with the **European Patients' Forum (EPF) and HTAi's Patients and Citizens Involvement Interest Group** on a **pilot initiative** to develop a patient-friendly PICO survey and **plain-language summaries** of EU HTA reports. The goal is to make it easier for patients to contribute meaningfully to future JCA processes.

"The ultimate goal is to help HTA bodies converge around pragmatic, clinically grounded PICOs that can support meaningful joint assessments."

David Bertwistle, Global Health Economics & Outcomes Research, Autolus Therapeutics, reflected on the scale of the challenge that PICOs present for developers, particularly smaller companies. He explained that because the framework is designed to capture all relevant treatment comparisons across Europe, in practice the number of possible PICOs per assessment could potentially be very high.
He also flagged the idealistic nature of PICO lists, which are not always grounded in available evidence. Developers also face tight timelines: once the JCA Coordination Group finalises the PICOs, companies have only 100 days to submit their dossiers, with no formal mechanism to challenge or refine the PICO list.

Finally, he warned that any international heterogeneity in PICOs consolidation practices might further complicates planning, especially for indications with many different comparators. He concluded that the success of the system will depend on how **pragmatically** assessors apply the methodology and that availability of real-world treatment pattern data could streamline the process.

"My understanding is that there's no way to challenge the PICOs that you are given. You can have a meeting to discuss the PICO list, but it cannot be changed. And you don't know which specific countries the PICOs are from."

Prof Bernard Wörmann, Department of Hematology, Oncology and Cancer Immunology, Charité - Universitätsmedizin Berlin, Germany emphasised that comparator choices

must reflect not just clinical guidelines, but also real-world availability. In dynamic and fast-evolving disease areas like multiple myeloma, access to treatment varies not just between countries, but also depending on the line of therapy. In some cases, a drug might be available only for third-line treatment in some settings, complicating comparator selection. He also raised concerns about limited national-level engagement with key stakeholders: in many countries, HTA bodies do not engage with either national patient organisations or local clinical experts, even when such expertise is available. This lack of consultation undermines the quality and inclusivity of national-level PICO submissions.

Stefano Benvenuti (Fondazione Telethon) added that while developers hope for pragmatism in the early phase of JCA implementation, evidence expectations remain a serious hurdle, particularly for ATMPs and rare diseases. He acknowledged anecdotal signs that some assessors are being more flexible than the official guidance suggests, but that the proof would be in the pudding and would only be confirmed once the JCA reports are published.

A key technical issue he raised is the requirement for patient-level data. Until now, developers have relied on indirect comparisons using aggregated registry data. However, new guidance from the JCA Secretariat restricts this approach, requiring deeper access to patient-level data, which is often not permitted under current informed consent structures in national registries.

This creates a dilemma: either re-run studies from scratch, which is resource-intensive and duplicative, or attempt to work through registry owners, which raises its own challenges around data quality, analytical pipelines, and trust. He stressed that without more pragmatic workarounds and sustained collaboration with patients and data owners, these requirements may become a structural barrier to meaningful assessments.

Conclusions & Next steps

Lydia Shotton, *Associate Director Government Affairs Europe, ARM*

Lydia Shotton (ARM) closed the discussion by thanking all speakers and participants. She highlighted that the strength of such meetings lies not only in the expert contributions, but also in the openness of attendees to exchange views, raise questions, and share experiences. Through continued dialogue, the stakeholder community has gained a clearer understanding of where key challenges lie and where practical solutions might be found.

Lydia Shotton (ARM) reaffirmed ARM's and the co-hosts commitment to keeping up the momentum and bringing shared concerns and recommendations to the attention of the European Commission and the HTA Coordination Group.

"Our priority is to keep understanding more clearly where the challenges lie and bring those messages to the European Commission and to the HTA Coordination group. We want to ensure the JCA process is inclusive, transparent, and workable for everyone involved."

While the HTA Stakeholder Network provides an important forum, many active groups remain outside of it. Initiatives and discussions like this one, she said, help to broaden the conversation and foster a wider, more vibrant community of practice around the implementation of the EU HTA, particularly as the process evolves.

She closed by expressing appreciation once again to all those who contributed and reiterated the importance of staying connected as a network and continuing to advocate for a JCA process that works for patients, clinicians, developers, and all involved.