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PATIENT AND CITIZEN INVOLVEMENT INTEREST GROUP

# Embedding Effective Patient Involvement in EU Joint Clinical Assessments

## ISPOR Europe Glasgow 2025: Issue Panel Session

HTAi Patients and Citizen Involvement Interest Group (PCIG)

Monday 10th November

# Embedding Effective Patient Involvement in EU Joint Clinical Assessments

# Panel Overview and Introduction

**Antonella Cardone** *(Cancer Patients Europe – Moderator)*

**Co-Lead, HTAi Patient and Citizen Involvement in HTA interest Group Project**

*To support Patient Stakeholder Input to the JCA Process*

# Embedding Effective Patient Involvement in EU Joint Clinical Assessments

# Panel Overview and Introduction

**Julie Spony**

*(HTA Secretariat, European Commission)*

**Maggie Galbraith**

*(Haute Autorité de Santé (HAS))*

**Jose Diaz**

*(Bristol Myers Squibb)*

# Panel agenda

## Embedding Effective Patient Involvement in EU Joint Clinical Assessments

**01** Introduction and Overview
***Antonella Cardone*** *(Moderator)*

**02** Audience Poll

**03** Panel Perspectives
***Julie Spony,*** *HTA Secretariat, European Commission*
***Maggie Galbraith,*** *National HTA/JCA Sub-Group*
***Jose Diaz,*** *Industry*

**04** Panel/Audience Discussion

**05** Audience Poll

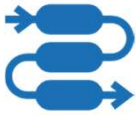
**06** Summary and Close

### Objectives

- To discuss actionable, realistic refinements to enhance meaningful patient involvement and
- To prioritize feasible mechanisms to embed timely and effective patient involvement in JCAs without extending statutory timelines

# Embedding Effective Patient Involvement in EU Joint Clinical Assessments

EU HTA Regulation provides for the involvement of patients in joint clinical assessments (JCA). Understanding **the experiences of patients, their families and carers will be a critical component of the JCA process**

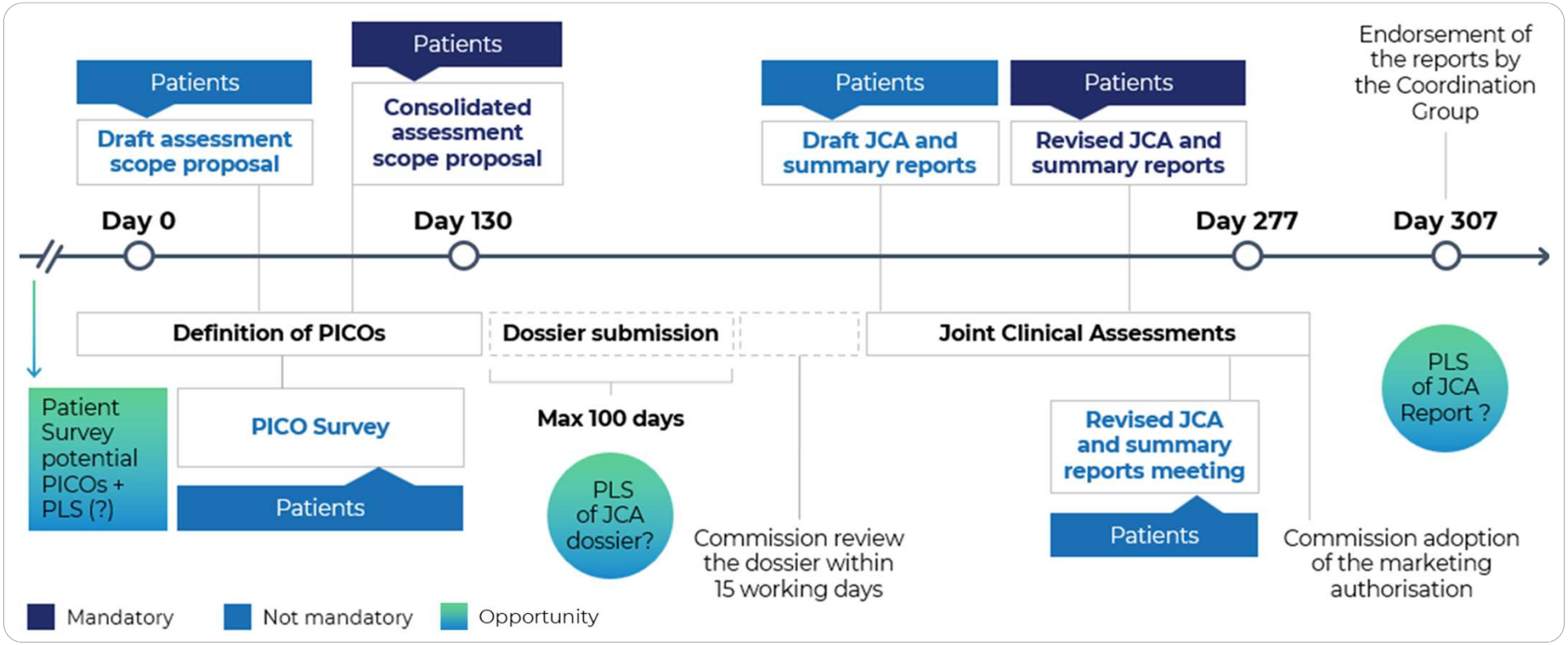


It is therefore vital to have an approach **to gain patient input at the beginning of the JCA process and strengthen the information provided to patient stakeholders during the process**, including at the assessment step and reporting phase

**Issue:**

Patient input remains limited to brief reactions to a near-final scope and a draft report. Is this enough at the right time to include essential patient insights and is there opportunity to support effective patient involvement?

# Embedding Effective Patient Involvement in EU Joint Clinical Assessments



# Embedding Effective Patient Involvement in EU Joint Clinical Assessments

## Four Options Opportunities

**01** Patient-informed survey to capture priority outcomes and inform JCA scoping prior to PICO consolidation and validation.

**02** Plain language summaries of both the manufacturer dossier and the JCA report—produced in plain language in different EU languages.

**03** Gathering patient input of contextual domains (unmet need, treatment acceptability, lived experience) so interpretations reflect real-world priorities.

**04** Patient involvement policy in EU HTA could help to lead best practice across the EU, taking example from well developed processes at agencies like SMC, NICE and HAS.

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# Patient involvement in Joint Clinical Assessments

**Julie Spony**

Project Officer, Health Technology Assessment Unit, DG SANTE, European Commission

*ISPOR issue panel: "Embedding Effective Patient Involvement in EU Joint Clinical Assessments"*

# Joint Clinical Assessments

**One single report** on the relative clinical effectiveness and safety of new medicinal products and certain high-risk medical devices – **starting with new cancer medicines** and advanced therapy medicinal products and including selected high-risk and in vitro diagnostic medical devices in 2026, orphan medicines in 2028, and all centrally approved medicinal products in 2030

**Pooling EU Member States resources and expertise** for high-quality, timely assessments

**Faster access**: Assessments to be completed within 30 days after the medicine is authorised vs. years after in some cases

Supporting EU Member States in making **evidence-based decisions on how to spend healthcare funds** (e.g., setting prices or deciding whether to cover treatments).

# Consultation of patients and clinicians in Joint Clinical Assessments

Input from patients and clinicians **before the finalisation of the assessment scope**

Input from patients and clinicians **before the finalisation of the JCA and summary reports**


**Coming Soon**

At any time during the joint clinical assessment, the JCA Subgroup may seek input on the disease and therapeutic area from patient organisations, healthcare professional organisations or clinical and learned societies via the members of the HTA stakeholder network.



European Commission

# Identification and selection of patients and clinicians in joint work

**HTA Stakeholder Network Working Group** to co-create the process


European Commission

# State of play

- **10 ongoing** joint clinical assessments
- **17 patients/carers** provided input to assessment scope
- **> 200 patients/carers and clinicians** invited to fill in a Declaration of Interest
- **> 40 patients/carers and clinicians selected** for Joint Clinical Assessments and Joint Scientific Consultations

- **New dedicated IT helpdesk** for patients and clinicians
- Additional **communication material and guidance on conflict of interest**
- Upcoming **new Europa website page** on involvement of patients and clinicians

# Experience so far

Need to **raise awareness** about the HTA Regulation to patients, clinicians.

**Clarify conflict-of-interest rules**

Need for **tailored support for patients throughout the process** (Commission, IT support, Brussels Centre for Collaboration in Health)

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# HTA Body Perspective

Maggie Galbraith,
European HTA Project Lead, HAS France

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# Involvement of EU Patients in the JCA

1. Implementing Regulation (EU) 2024/1381 explicitly includes patients (as stakeholders and experts) in the process of a JCA; Patient-Centered Outcomes (PROs) are highlighted as a formal category of evidence in The Guidance on Outcomes for Joint Clinical Assessments

2. Involvement during the JCA Scoping Process
   Input is presented to the entire JCA SG most often resulting in reformulated outcomes based on their input (very helpful for quality of life outcomes/scores)

3. Further involvement during the JCA
   - All experts will be able to review the draft report at the end of the assessment phase (currently no JCA has progressed this far in the process)
   - Document annexed to the final report, thus making them visible publicly

4. Opportunities for progress
   Obtain EU patient input earlier in the scoping phase

# Involvement of French Patients in the JCA Scoping Period

1. **Reminder:** National Involvement is *out of scope* of the HTAR

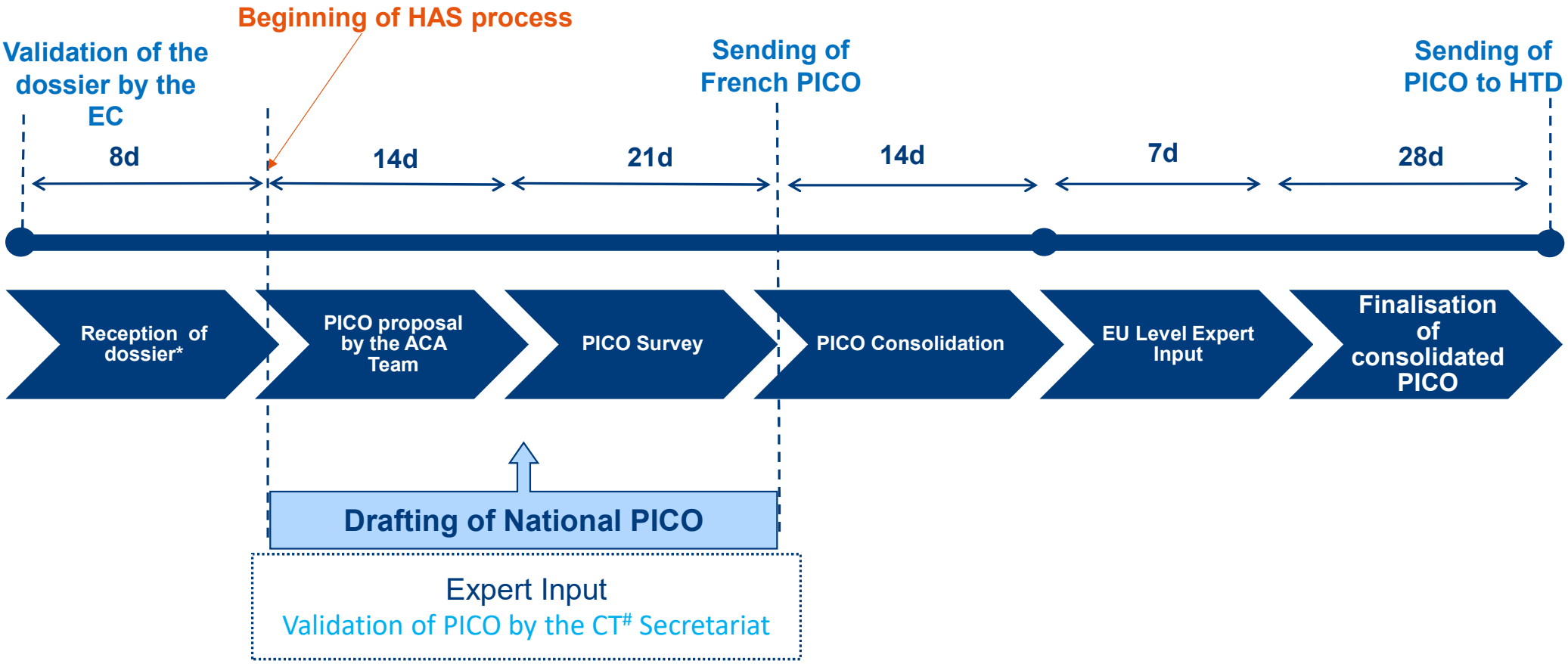
2. Patient involvement is part of HAS's Strategic Priority (2019–2024 and 2025–2030)

3. French Pilot Programme
   - Developed in collaboration with French patient associations
   - Creation of a «pool» of patient experts
     - Focus on oncology and rare diseases
     - 15 participants with 15 in process
     - React to proposed French PICO (and EU PICO proposal, depending on timing)
   - Assessed ~1 year

4. Opportunities for progress

   Scalability of this process

# Development of the PICO




*This initial dossier is akin to the Marketing Authorisation Dossier submitted to EMA. The actual JCA dossier is submitted by the company on the basis of the PICO(s) provided by the Assessor team
#The French Transparency Committee (Commission de la Transparence) of the Haute Autorité de Santé evaluates medicines for reimbursement by assessing their clinical benefit (SMR) and added value (ASMR) and provides scientific opinions to guide public health decisions and promote appropriate use of medicines.

# Comparison French and EU Patient Contribution

| France | European Level |
|---|---|
| National level patients participating as *experts* | European level patient *experts* |
| *Before* French PICO is validated by the Transparency Committee's Secretariat and provided to EU assessor team | Often *after* EU PICO Consolidation and (later) all experts review the draft final report |
| Receive proposed French PICO proposal (and EU proposal (pre-consolidation) depending on timing) | Receive consolidated PICO to react upon |
| Input not included in final JCA report | Input annexed to final JCA report |
| → ! Scalability | → ! Timing of reception of EU Patient input |

| France (post-JCA) |
|---|
| National *stakeholder* input |
| Contribution during the national assessment process (post-JCA) in view of reimbursement in France |
| Written submission via standardised questionnaire online |
| Presentation during TC by patient representative; input published on HAS website along side HAS opinion. |

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# From Procedure to Partnership: Embedding Patient Voice in EU HTA

**Jose Diaz**

Global Health Systems Economics & Value of Innovation

GHEOR

Bristol Myers Squibb®

*The opinions expressed in this presentation and on the following slides are solely those of the presenter and not those of BMS.*

# 1. Why Patient Involvement Matters

EU HTA Regulation calls for patient participation in JCA. To make it meaningful, it must be timely, structured and grounded in lived experience.

**01** EU HTA Regulation aims to **align evidence generation and assessment** across Member States.

**02** **Patient perspectives** (inc. lived experience) improve relevance, legitimacy, and societal accountability of HTA.

**03** **Timely engagement**: Ensure patients help define **what is assessed**, not just comment on how it is reviewed..

**04** **Context matters:** Capture **unmet need** and **lived experience** to anchor assessments in real-world priorities

**05** **Shared journey:** Evidence Generation → EU JCA → National Appraisal → **Access.**

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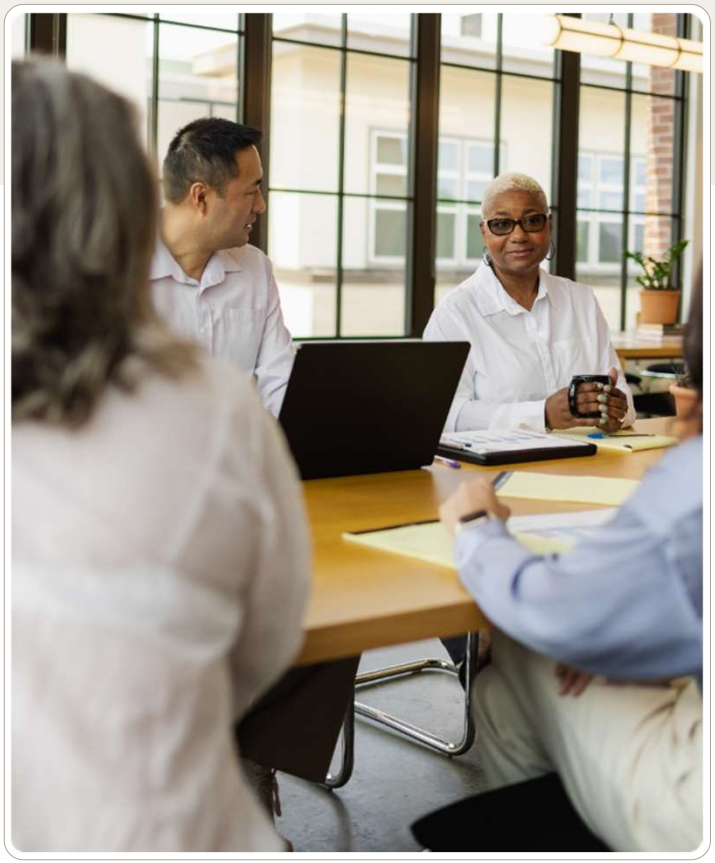
*Timely, structured, and meaningful patient voice that adds value to the process*

# 2. Learning from Proven HTA Practice



Mature HTA systems show that early, well-structured engagement enriches evidence and decision-making without adding complexity.

**01** **NICE:** Early scoping, patient expert input, and lay members; guidance, templates and training enable participation.

**02** **SMC:** PACE meetings and written submissions capture lived experience and unmet need; statements inform decisions.

**03** **HAS:** Structured questionnaires for patient associations, with opportunities for follow-up and dialogue..

**04** **NICE core principles:** Best evidence, expert input, genuine consultation, independent committees, equity and transparency.

*Structured collaboration enhances efficiency, not complexity.*

# 3. Ensuring Trust & Scalability: PLS and Responsible AI


*PLS already mandated for EU clinical trials; HTA is the logical next step*

Plain Language Summaries (PLS) are vital for transparency and participation. Key to ensure clear safeguards are needed to ensure information is accurate, balanced, and trusted.

**01** **PLS inform, not persuade** – A communication tool, not promotion.

**02** **Standardized templates** with fixed sections, plain language standards, and clear references to source data to avoid selective presentation.

**03** **Two-step review:** JCA Secretariat technical check, then independent EU academic review for neutrality and accessibility.

**04** **Transparency and consistency:** Public change logs, declarations of interest, and back-translation across EU languages.

**05** **Generative AI** can draft PLS rapidly; human-in-the-loop remains essential to ensure accuracy and neutrality.

**06** **Outcome:** Accurate, balanced, accessible materials that strengthen confidence among patients, HTA bodies, and the public.



*Proportionate safeguards to ensure clarity and trust*

# 4. The Role of Industry – Partnering for Meaningful Engagement

Industry can help embed patient voice early and collaboratively, ensuring evidence and communication are both patient-relevant and scientifically robust.

**01** **Upstream partnership:** Involve patients in target profiles, trial design and endpoints.

**02** **Evidence generation:** High-quality Patient Experience Data (PED) and preference studies aligned with EMA guidance.

**03** **Plain language summaries**: Integrate PLS from the outset—across trials, submissions, and HTA dossiers—to make evidence accessible, strengthen understanding, and build public trust.

**04** **Capacity building:** Partner with umbrella organisations, including regional PAGs, ISPOR and HTAi, to strengthen participation.

**05** **Early dialogue:** Engage early and collaboratively with HTA bodies and patient groups to align evidence planning with JCA timelines and strengthen meaningful, coordinated dialogue.

# A Shared Call to Action

## Build a trusted, people-centred European HTA that informs better decisions for all.

**01** **Start early, design together:** Involve patients from the very beginning of evidence planning and scoping, co-designing with HTA bodies, regulators, and developers as equal partners.

**02** **Meaningful evidence:** Integrate patient-informed outcomes, contextual domains, and lived experience into assessments.

**03** **Transparent communication:** Provide multilingual, plain-language summaries of dossiers and reports to strengthen understanding and trust.

**04** **Set Europe's benchmark:** Legitimate, efficient, and people-centred HTA that others emulate.

*Design JCAs with patients, not merely for them*

# Discussion

## Panel and Audience Questions

# Embedding Effective Patient Involvement in EU Joint Clinical Assessments

## Summary and Conclusions

- **Panel Discussion**
- **Audience Polls**
- **HTAi Patient and Citizen Involvement in HTA interest Group Project**

  *To support Patient Stakeholder Input to the JCA Process*

# Thank you

Please do not hesitate to get in touch:
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