38th Workshop of the
EURORDIS Round Table of Companies (ERTC)
9th October 2024
09.00-17.00 CET
Barcelona

Bridging Perspectives: Preparing for Success in Joint Clinical Assessments in EU HTA

Overarching objective and scope

The next workshop of the EURORDIS Round Table of Companies will focus on Health Technology Assessment (HTA) cooperation in Europe. In the context of the EU Regulation on HTA (HTAR 2282/2021), a framework for joint work between Member States was introduced, including new rules for conducting joint clinical assessments of medicines and medical devices.

In this workshop, participants will hear from HTA experts, patient representatives, public officials and industry representatives on how all need to prepare as the EU Cooperation and Joint Clinical Assessments become mandatory for oncology products and ATMPs starting 12 January 2025, in parallel to CHMP marketing authorisation evaluations. This provision will be applicable to all other orphan medicinal products from 2028 onwards.

Discussions will get participants prepared for this joint assessment approach and invite them to reflect on the conditions towards a successful implementation that ultimately improves transparency of the HTA process and accelerates national decision-making (reimbursement decision).

In addition to sharing perspectives from each stakeholder group on their appropriate involvement in the clinical assessment of medicines, the programme will also provide time for small group discussions. Breakout sessions will cover a range of topics, including methodological guidelines as well as timeframe in joint clinical assessments, novel approaches to assess real-world medical data using new technologies, and voluntary cooperation exploring new models of economic evaluation in HTA. Moderated flash debates will conclude the workshop on other specifics of joint clinical assessments, including the engagement of patient experts in this process.

DRAFT PROGRAMME

Co-Chaired by: **Dr Ana Palma, Senior Director Global Head of Patient Access and Policy, SOBI** **François Houÿez, Director of Treatment Information and Access, EURORDIS**	
9.00 – 9.30	**REGISTRATION**
9.30 – 9.40	**Welcome by the Co-Chairs**
9.40 – 9.50	**Welcome introduction, setting the scene and goals of the workshop** **Dr Virginie Bros Facer,** Chief Executive Officer, EURORDIS
	Joint Clinical Assessments start in three months. How to prepare?
9.50 – 10.10	• **EC HTA Unit: How Joint Clinical Assessments will be performed** **Béla Dajka,** Health Policy Officer, European Commission (remote)
10.10 – 10.25 **10.25 - 10.40**	• **Conditions for successful Joint Clinical Assessments:** ➢ **EURORDIS' opinion** **Julien Delaye**, Patient Engagement Manager - HTA, EURORDIS ➢ **Industry's opinion** **Matteo Scarabelli,** Market Access Associate Director, EFPIA
10.40 - 11.00	• **Q & A**
11.00–11.30	**MORNING COMFORT BREAK**
11.30–13.00	**Break-out parallel sessions:**
	• **PICOS and timelines; EFPIA and EUnetHTA21 simulations** **Anne Willemsen,** Senior Project Manager, Zorginstituut Nederland (remote) **Tanja Podkonjak,** Director, EUCAN Oncology Access and Reimbursement Policy, Takeda

	Moderator: **Isabel Klinnert**, Global Government and Public Affairs, Merck Rapporteur: **Gaetan Duport**, Patient Advocate, European Haemophilia Consortium • **Submissions and patient input** ➢ **Thinking outside the box: other possible data sources** **Dr François Meyer,** Founder, Meyer FMF ➢ **Patient Preferences Studies: eliciting developers' interest. Rare diseases as a case study** **Thomas Desmet,** Clinical Pharmacologist, KU Leuven Moderator: **Walter Atzori**, Global Patient Advocacy Strategy & Engagement Lead, Alexion Rapporteur: **Monica Racovita**, Access and Policy Manager, Myeloma Patients Europe
12.30-13.30	**LUNCH**
	Cooperation in the EU: Tools we need
	Break out parallel sessions:
13.30-14.45	• **Methodological guidelines on indirect comparators/external controls** **Prof. Jörg Ruof,** Secretariat, European Access Academy **Dr Beate Wieseler,** Head of Drug Assessment, Institute for Quality and Efficiency in Health Care (remote) Moderator: **Dr Jan Mol**, Patient Advocate, Hematon Rapporteur: **Davide Marchi**, Director, International Patient Engagement, Vertex Pharmaceuticals • **Innovative Methods in HTA:** ➢ **HTx project: Next Generation HTA main outcomes** **François Houÿez,** Director of Treatment Information and Access, EURORDIS ➢ **Real-World Evidence** **Prof. Bertrand Arnulf,** Head of Immuno-Haematology, APHP (remote) Moderator: **Niko Costantino**, Head of Public Affairs, Cometa ASMME Rapporteur: **Gabriella Almberg**, Global Head of Policy and Public Affairs, Rare Diseases, UCB

	• **HTA voluntary cooperation: can we discuss economic methods?** ➢ **How do we evaluate the risk reducing effect of an outcome-based payment model for ATMPs?** **Dr Douglas Lundin,** Chief Economist, TLV (remote) ➢ **Voluntary Cooperation on HTA economic evaluations** **Prof. Mark Nuijten,** Clinical and Economic Valuation Scientist, A2M Moderator: **Francis Pang,** SVP Global Market Access and International Geographic Expansion, Orchard Therapeutics Rapporteur: **Nora Lazaro,** Patient Engagement Manager, EURORDIS
14.45 – 15.00	**TEA BREAK**
	HTA Cooperation in the EU: flash debates
15:00 – 15:20	**How involved can a patient representative be in product development and still be eligible to participate in a joint HTA?** ➢ From the templates we have, what will they say? ➢ What will readers take from them? **Johan de Graaf,** Chair, Dutch Pituitary Foundation **Dominique Sturz,** Vice-President, Pro Rare Austria **Moderated by Julien Delaye,** Patient Engagement Manager - HTA, EURORDIS
15:20- 15:40	**Should the EU Cooperation work with patient experts or more "naïve" patients in HTA at the European level**? ➢ What do we mean by patient experts? ➢ Who are we looking for? ➢ What methods could be used for patient input? **Florian Innig,** Bundesverband Kleinwüchsige Menschen und ihre Familien e.V. **Dr Monica Racovita,** Access and Policy Manager, Myeloma Patients Europe **Moderated by Julien Delaye,** Patient Engagement Manager - HTA, EURORDIS
16:00 – 16:25	**Quiz and Poll**
16:25 – 16:30	**Farewell from Co-Chairs**