EURORDIS
RARE DISEASES EUROPE

38TH ERTC WORKSHOP

BRIDGING PERSPECTIVES: PREPARING FOR SUCCESS IN JOINT CLINICAL ASSESSMENTS IN EU HTA

9th October 2024
09.00-17.00 CET, Barcelona

Overarching objective and scope

This edition of the EURORDIS Round Table of Companies Workshop 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 on 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.

WORKSHOP CO-CHAIRS

Dr, Ana Palma

Senior Director Global Head of Market Access Policy, SOBI

Ana Palma has over 24 years' experience in the Pharmaceutical Industry, more than half of it with rare diseases and orphan drugs. Ana is Senior Director Global Head of Patient Access & Policy at SOBI, since 2014. In her role, Ana is responsible globally for patient access and access policy, including activities related to pricing and reimbursement strategies' development and implementation across SOBI's portfolio (haematology, oncology, hemophilia and immunology) and in all SOBI territories (North and Latin America, Europe, Asia Pacific, Middle East, North Africa).

Ana is also active on various projects at EU level in the context of orphan drugs and rare diseases and where she engages with a broad set of stakeholders including policy-makers, health care professionals, government officials, regulators, patient organisations, HTA bodies and payers. Ana is a member of the EU HTA Stakeholder Network.

Prior to joining SOBI, Ana was leading Market Access in fifteen European countries at Shire, and prior to that she was leading the European Market Access Network at Genzyme, always with a particular focus on orphan drugs and rare diseases. She has also worked ten years at Johnson & Johnson having held various scientific roles in the field of technology transfers, validation and QA.

Ana is a Pharmaceutical Doctor and also holds an MBA.

François Houÿez

Director of Treatment Information and Access, EURORDIS

François Houÿez has worked as a patient advocate since the early 1990s (HIV/AIDS, Act Up -Paris and EATG) and joined EURORDIS in May 2003. He now works as Information & Access to Therapies Director & Health Policy Advisor.
He represents EURORDIS at the Patients' and Consumers' Working Party at the European Medicines Agency (EMA). He also represents EURORDIS at the Health Technology Assessment Network, and in CIOMS Working Group XI on Patient Involvement in the Development and Safe Use of Medicines.

François supervises EURORDIS' programme for Community Advisory Boards (EuroCAB) and the European Network of Rare Diseases Help Lines.

He pioneered patient advocacy with the European Medicines Agency as part of the first patients' delegation that engaged in dialogue with the Agency back in 1996 and has continuously been involved in the agency's activities during the last 26 years.
François compiles trend information, and regularly fields questions from rare disease patients having issues with access to treatments (especially marketing authorisations, health technology assessment/pricing/reimbursement, compassionate use, shortages, and pharmacovigilance).
François is also a patient.

WELCOME INTRODUCTION, SETTING THE SCENE AND GOALS OF THE WORKSHOP

Dr Virginie Bros-Facer

Chief Executive Officer, EURORDIS

Virginie returned to EURORDIS-Rare Diseases Europe in March 2024 to take on the role of Chief Executive Officer, after having previously served as the organisation's Scientific Director.

Virginie received her PhD in Neurosciences from King's College London, UK followed by several postdoctoral research projects at the Institute of Neurology, UCL, London focused on testing therapeutic strategies for Amyotrophic Lateral Sclerosis. After leaving the lab, she worked for several research funding organisations in the UK including the National Institute for Health Research, the Medical Research Council and as Medical Director for Sparks, a medical research charity focusing on rare paediatric diseases.

Upon subsequently joining EURORDIS as Scientific Director, she took the lead on project development and patient engagement in rare disease research projects representing the voice of rare disease patients, including within the International Rare Disease Research Consortium (IRDiRC). Virginie led in the creation of the EURORDIS Winter School on Scientific Innovation and translational research, aimed at empowering people living with a rare disease to fight for their rights to a better and healthier future.

In January 2022, Virginie joined Illumina as Associate Director for Medical Affairs, Europe, where she engaged key opinion leaders and centres of excellence to develop clinical evidence for genetic testing of rare and undiagnosed patients to drive clinical adoption of next-generation sequencing and implementation in patient care.

Until joining EURORDIS, Virginie had been a member of the Diagnostic Scientific Committee of the International Rare Diseases Research Consortium (IRDiRC), which coordinates a dedicated working group on real-world applications and technologies for newborn screening.

JOINT CLINICAL ASSESSMENTS START IN THREE MONTHS. HOW TO PREPARE?

EC HTA UNIT: HOW JOINT CLINICAL ASSESSMENTS WILL BE PERFORMED

Béla Dajka, Speaker (remote)

Health Policy Officer, European Commission

Béla Dajka is a policy officer at the HTA Secretariat of the European Commission. He supports the work of the Member State Coordination Group on HTA and manages the HTA Stakeholder Network. Béla joined the European Commission in 2008 and led the corporate communication team in the Directorate-General for Communication before starting his work on health systems in 2020 in the Directorate-General for Health and Food Safety.

Prior to joining the European Commission, he worked at the British Broadcasting Corporation for 10 years. He has a background in health economics, management, media and communication.

CONDITIONS FOR SUCCESSFUL JOINT CLINICAL ASSESSMENTS: EURORDIS AND INDUSTRY'S OPINION

Julien Delaye, Speaker

Patient Engagement Manager HTA, EURORDIS

Julien Delaye joined EURORDIS in September 2019 as an intern and moved to the position of Public Affairs Assistant on the Rare 2030 project. He then became Public Health Policy Junior Manager and now holds the position of Patient Engagement Manager in HTA.

Working closely with the Director of Information & Access to Therapies & Health Policy Advisor, Julien is responsible for organising the participation (awareness, training, monitoring) of people living with a rare disease and their representatives in the European Cooperation on HTA and in national HTA procedures, as well as participating in research activities in the HTA field where EURORDIS is invited to take a role.

Prior to EURORDIS, he worked as an event coordinator for European Calcified Tissue Society (ECTS), was a volunteer for Osteogenesis Imperfecta Federation Europe (OIFE) and was a marketing intern at Sanofi – Copenhagen.

Julien holds two bachelor's degrees, in Marketing (Liège – Campus Guillemins) and in European Public Health (Maastricht University), and a master's degree in Public Policy and Administration (Dublin – UCD).

He speaks French, English and a bit of Dutch.

Matteo Scarabelli, Speaker

Market Access Associate Director, EFPIA

Matteo Scarabelli has a PhD in Philosophy and primary experience in EU public affairs. He has started building an expertise in the pharmaceutical market, being responsible for the involvement of patients in regulatory approval and access decisions for orphan drugs.

He then contributed by designing his strategy of rare disease organisations across Europe for the new EU HTA, also participating in the genesis and in the inter-institutional negotiations of the HTA Regulation.

MORNING COMFORT BREAK TO FOLLOW

PICOS AND TIMELINES: EFPIA AND EUNETHTA21 SIMULATIONS

Anne Willemsen, Speaker (remote)

Senior Project Manager, Zorginstituut Nederland

Anne Willemsen holds double Master's Degrees in Health Sciences, specialising in both Health Technology Assessment and Health Policy. She joined the Dutch National Healthcare Institute in 2016. Until 2023 she has worked for the European Network for HTA (EUnetHTA) and this led her through research and project management roles.

In her last role within EUnetHTA she has been spearheading the Secretariat responsible for coordinating Joint Clinical Assessments (JCA) and the joint workplan between HTA bodies and the European Medicines Agency. Furthermore, she was also involved in developing procedures and templates for involvement of patients into these JCA and she was part of the Conflict of Interest Committee. In April 2023, she was elected as the co-chair of the JCA subgroup of the HTA Regulation's Coordination Group.

Tanja Podkonjak, Speaker

Director, EUCAN Oncology Access and Reimbursement Policy, Takeda

Tanja Podkonjak, Head of Access Intelligence and Readiness, Takeda Oncology. Tanja joined Takeda Pharmaceuticals in 2011 and has held various roles in market access, policy, and strategy across multiple countries.

In her current role, Tanja focuses on collaborating with internal and external stakeholders to shape the evolving access landscape for oncology medicines in Europe and Canada. Her main areas of focus are the EU Joint HTA Regulation and access to combination medicines. Tanja is a Steering Committee member of EFPIA's Oncology Platform, where she is actively involved in EU Joint HTA Advocacy projects and a contributing member to EFPIA's EU HTA working groups on JCA process, methods and advocacy.

She holds an honors Bachelor of Sciences degree and an MBA from McMaster University (Canada) and has completed an MSc in Health Economics from the University of the City of London(UK).

PICOS AND TIMELINES: EFPIA AND EUNETHTA21 SIMULATIONS

Isabel Klinnert, Moderator

Global Government and Public Affairs, Merck

Isabel is an accomplished healthcare professional with extensive experience in the pharmaceutical industry, specialising in strategic health and market access policies at both European and global levels.

She holds a Master's in Health Policy Planning and Financing from the London School of Economics (LSE) and a Master's in European Public Policy from University College London (UCL), complemented by a Bachelor's in International Relations.

Currently, she serves as the Director of Global Government and Public Affairs at Merck KGaA, where she leads cross-functional initiatives aimed at enhancing healthcare policy in Neurology. Her previous roles at Biogen, Teva Pharmaceuticals, and Medicines for Europe involved successfully implementing strategies to navigate complex policy landscapes and improve healthcare systems.

Gaetan Duport, Rapporteur

Patient Advocate, European Haemophilia Consortium

Gaetan (/ga.e.tan/) Duport is a key figure in the haemophilia community, leading the Medication Group at the French Haemophilia Association (AFH) and overseeing the implementation of HTA (Health Technology Assessment) regulations within the community. He plays a vital role in informing and training patients and their families about new therapies, ensuring they understand and access the latest innovations.

Gaetan has been instrumental in establishing support programmes that facilitate the use of innovative treatments, improving patient access to cutting-edge therapies both in France and across Europe.

For the past four years, he has served as a member of the economic body of the French HTA at the Haute Autorité de Santé (HAS), where he brings invaluable patient expertise to public health decision-making. His work includes conducting semi-structured interviews that allow patient associations to contribute throughout the administrative process of evaluating medications. In addition to his work in France, Gaetan represents the European Haemophilia Consortium within the HTA Stakeholder Network created by the 2021 HTA Regulation. His contributions extend to various publications on rare bleeding disorders, where he addresses critical issues like access to care for patients across Europe.

Formerly a physiotherapist, Gaetan holds a degree in Public Health from Maastricht University in the Netherlands and a degree in European Affairs from the College of Europe in Belgium. His interdisciplinary expertise uniquely positions him at the intersection of healthcare, policy, and patient advocacy.

SUBMISSIONS AND PATIENT INPUT

- THINKING OUTSIDE THE BOX: OTHER POSSIBLE DATA SOURCES
- PATIENT PREFERENCES STUDIES:ELICITING DEVELOPERS' INTEREST - RARE DISEASES AS A CASE STUDY

Thomas Desmet, Speaker

Clinical Pharmacologist, KU Leuven

Thomas performs his research at the KU Leuven research unit as well as in collaboration with the Vlerick Business School. He focuses on the regulatory, HTA and reimbursement processes of advanced therapy medicinal products (ATMPs) intended to advance access to treatments that improve patients' lives and address their needs. His PhD builds on frameworks that drive valuation and deepen stakeholder engagement in national and European market access. In doing so, the topic is tackled from three points of view. First, by investigating the value of gene therapies for patients and their acceptance of clinical uncertainties, next to analysing how real-world evidence can tackle some of these uncertainties. Second, developing a holistic value assessment framework for gene therapies, which considers these patient preferences and follow-up real-world evidence, as well as analysing managed entry agreements (MEAs) in Belgium to understand how innovative therapies are valued. Third, elaborating on this understanding and establishing sustainable payment models for innovative therapies by investigating the use and ways to implement newly proposed payment models (e.g., outcome-based spread payments).

Lastly, to potentially improve durable access and affordability of innovative therapies, industry-manufactured and academic-developed CAR-T therapies will be compared from a health-economic business perspective.

Dr François Meyer, Speaker

Founder, Meyer FMF

François Meyer is a medical doctor with an MD degree from the University of Montpellier Medical School in France. He spent 10 years as a physician in the Endocrinology and Internal Medicine units of the Montpellier teaching hospitals.
He then worked for 5 years in the R&D department of an international pharmaceutical company. From 1997 to 2005, he held various positions at the French Medicines Agency, including Deputy Director of the Medicines Evaluation Department and Secretary General of the Transparency Committee.
In 2005, the Haute Autorité de Santé (HAS, French National Authority for Health) was created by law and Dr Meyer was responsible for setting up its Health Technology Assessment (HTA) department, in charge of HTA for pharmaceuticals, medical devices, interventional and diagnostic procedures as well as public health actions and programs. In 2011, he was appointed advisor to the President of the HAS in charge of international affairs.
Dr Meyer has been a member of the Board of Directors of HTAi, the international society for HTA. He has been involved in European cooperation in HTA since the start of the EUnetHTA project in 2006.
Since 2023, Dr Meyer is an independent consultant in the field of HTA and health policy.

SUBMISSIONS AND PATIENT INPUT

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Walter Atzori, Moderator

Global Patient Advocacy Strategy & Engagement Lead, Alexion

Walter Atzori has been with Alexion since 2017, currently serving as Global Patient Advocacy Strategic Lead. In this capacity he leads strategy and execution of global patient advocacy plans and engagements across the all Alexion therapeutic areas, including pipeline programs. He oversees relationships with key International and / or pan-European rare disease groups, ensuring their meaningful engagement in key initiatives across the therapeutic innovation cycle.

Prior to joining the pharmaceutical industry, Walter worked 7 years at the European Patients' Forum where he advocated for the recognition of the value of meaningful patient involvement in medicines' R&D, contributing to the shaping and implementation of flagship projects and initiatives in this area such as EUPATI and PARADIGM.

Dr Monica Racovita, Rapporteur

Access and Policy Manager, Myeloma Patients Europe

Monica holds a PhD in Global Social Sciences from Doshisha University, Japan and a Master of Engineering in Applied Chemistry and Biotechnology from Nagoya University, Japan. In her undergraduate degree, she studied Biomedical Engineering at the University of Pharmacy and Medicine in Iasi, Romania.

After her PhD she worked on science and health policy issues in academia, international development or consultancy in Italy, Austria and the UK. Subjects included the biosafety evaluations of genetically modified crops, Responsible Research and Innovation, Artificial Intelligence and the Internet of Things, and public health.

A desire to work for non-profit organisations and in particular for one representing patients led her to MPE, which she joined in January 2024. Monica comes from Romania and currently resides in the UK. She speaks Romanian, English and Italian and has a working/partial understanding of French and Spanish.

LUNCH BREAK TO FOLLOW

METHODOLOGICAL GUIDELINES ON INDIRECT COMPARATORS / EXTERNAL CONTROLS

Prof. Jörg Ruof, Speaker

Secretariat, European Access Academy

Jörg Ruof holds a degree in Medicine (University of Witten/Herdecke), Public Health (Hanover Medical School), and Business Administration (Edinburgh Business School). He is a Professor of Health Outcomes and Management at Hanover Medical School and has 20 years executive experience in 'big pharma' with a focus on Market Access.

In 2017 he founded r-connect (www.r-connect.org), a strategic consulting organisation located in Basel, Switzerland. Since 2018 he's heading the `German Platform on Benefit Assessment', a Collaboration with 'Springer Medicine'. In 2021 he founded the 'European Access Academy', EAA (www.euaac.org).

Dr Beate Wieseler, Speaker

Head of Drug Assessment, Institute for Quality and Efficiency in Health Care

Dr. Beate Wieseler is Head of the Department of Drug Assessment at the German Institute for Quality and Efficiency in Health Care (Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, IQWiG). At IQWiG she is responsible for the scientific assessment of pharmaceuticals, the development of assessment methods, as well as in the Institute's collaboration with German and international external parties.

With regard to the HTA Regulation, Beate Wieseler is a member of the HTA Coordination Group and the Chair of the Methodological and Procedural Guidance Subgroup.

Prior to joining IQWiG in 2005, Beate Wieseler worked in clinical research and regulatory affairs for about 10 years. Beate Wieseler holds a Diploma in Biology from the University of Bonn and a Dr. rer. nat. from the University of Freiburg, Germany

METHODOLOGICAL GUIDELINES ON INDIRECT COMPARATORS / EXTERNALCONTROLS

Dr Jan Mol, Moderator

Patient Advocate, Hematon

Dr Jan Mol is a retired cell biologist of Utrecht University and patient advocate for people with various forms of blood cancer. Dr Mol graduated from Wageningen University (Molecular Sciences) and did his PhD at Erasmus University Rotterdam on thyroid hormone metabolism. Thereafter he became principal investigator at Utrecht University in the field of endocrinology, oncology and functional genomics of companion animals. He was project leader of the programme on Comparative and Translational Oncology which is part of the Regenerative Medicine programme of Utrecht Life Sciences.

The projects focused around the role of cancer stem cells and the growth hormone receptor (GHR) in mammary gland carcinogenesis. Hallmarks are the discovery of the local mammary expression of growth hormone (GH) and IGF-I, the unique role of the canine progesterone receptor-B and Wnt-signaling in mammary carcinogenesis. After being cured from an aggressive diffuse large B-cell lymphoma (DLBCL) he became board member and patient advocate for Stichting Hematon and member of the global lymphoma coalition. Jan Mol is member of the advisory boards of the Dutch Cancer Society KWF and Oncode Accelerator, and patient expert for EMA. His passion is that the patients of today and tomorrow have faster access to truly effective medicines at a socially acceptable price.

Davide Marchi, Rapporteur

Director, International Patient Engagement, Vertex Pharmaceuticals

Davide has been leading the engagement with patient communities at Vertex for over 6 years across multiple serious diseases, most of which are rare. Davide's work is dedicated to connecting with organisations representing people living with a disease and their carers, to better understand their needs and acknowledge their lived experience, as well as establishing meaningful collaborations. Davide became passionate about patient advocacy and engagement during his previous public affairs roles that focused on healthcare policy (at EuropaBio and Hanover Comms), following his studies in Diplomacy and International Relations in Italy, Sweden and Belgium.

INNOVATIVE METHODS IN HTA:

- HTX PROJECT: NEXT GENERATION HTA MAIN OUTCOMES
- REAL-WORLD EVIDENCE -

François Houÿez, Speaker

Director of Treatment Information and Access, EURORDIS

François Houÿez has worked as a patient advocate since the early 1990s (HIV/AIDS, Act Up -Paris and EATG) and joined EURORDIS in May 2003. He now works as Information & Access to Therapies Director & Health Policy Advisor.
He represents EURORDIS at the Patients' and Consumers' Working Party at the European Medicines Agency (EMA). He also represents EURORDIS at the Health Technology Assessment Network, and in CIOMS Working Group XI on Patient Involvement in the Development and Safe Use of Medicines.

François supervises EURORDIS's programme for Community Advisory Boards (EuroCAB) and the European Network of Rare Diseases Help Lines.

He pioneered patient advocacy with the European Medicines Agency as part of the first patients' delegation that engaged in dialogue with the Agency back in 1996 and has continuously been involved in the agency's activities during the last 26 years.
François compiles trend information, and regularly fields questions from rare disease patients having issues with access to treatments (especially marketing authorisations, health technology assessment/pricing/reimbursement, compassionate use, shortages, and pharmacovigilance).
François is also a patient.

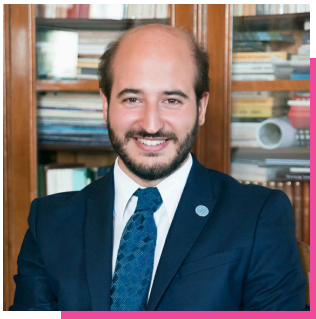
Prof. Bertrand Arnulf, Speaker (remote)

Head of Immuno-Haematology, APHP

Prof. Bertrand Arnulf is the head of the Immuno-Haematology Department of Saint Louis Hospital dedicated to Monoclonal gammopathies and related disorders including Multiple Myeloma, Waldenströml Macroglobulinemia, Monoclonal gammopathies of Clinical significance and AL amyloidosis. Constitutive National Reference center for AL amyloidosis and other monoclonal gammopathy of clinical significance
He is also an active member of IFM board, Coordinator of the Myeloma part of the DESCAR-T registry and the CAR-T program of IFM.
He has worked on both basic and translational research focused on novel immunotherapies in Multiple Myeloma and involved in main CAR T studies in development.

INNOVATIVE METHODS IN HTA:

- HTX PROJECT: NEXT GENERATION HTA MAIN OUTCOMES
- REAL-WORLD EVIDENCE



Niko Costantino, Moderator

Head of Public Affairs, Cometa ASMME

Niko Costantino is a professional with extensive experience in international relations, and a strong background in public diplomacy and political strategy. He holds a Master of Arts in International Relations with Political Communication from the University of Kent's Brussels School of International Studies and a Bachelor's degree in International Relations with Language Studies from Ca' Foscari University of Venice.

Currently, Niko serves as Head of Public Affairs of an Italian patient organization for Inborn Metabolic Diseases, Cometa ASMME, and is member of the EURORDIS HTA Task Force, where he contributes to EURORDIS' position on health technology assessment, aiming at improving patient quality of life, effectiveness and equality in access to health technologies. Niko also serves as an expert at the European Medicines Agency and holds key roles in cultural diplomacy with UNESCO. He has overseen the organization of international conferences and negotiations in the field of rare diseases and cultural cooperation, which has brought him to collaborate with figures of global impact.

Niko has worked in crisis communication, advocacy, and international cooperation, contributing to Europe-wide initiatives on rare diseases and healthcare policy at the European Parliament, focusing on patient access to innovation and public policy participation. He adeptly mediates between patients, healthcare professionals, and policymakers, leveraging his cultural competence and conference management skills to navigate multilingual, intercultural settings.

Niko also contributes to HuffPost and researches for a foreign policy think tank.

Gabriella Almberg, Rapporteur

Global Head of Policy and Public Affairs, Rare Diseases, UCB

Gabriella Almberg heads up the Global Policy & Public Affairs team at UCB, bringing over 15 years of extensive experience in policy, public affairs, and government affairs within the life science sector. Her career spans both trade associations and leading companies, where she has honed her expertise in navigating complex regulatory landscapes and advocating for impactful health policies.

For the past five years, Gabriella has been particularly focused on rare disease policy, driven by a deep commitment to improving the lives of patients with rare conditions. Her work is characterized by a strong collaboration with patient communities, to guide impactful policy discussions and to inform the work of the company to better address the gaps and needs.
Based in Copenhagen with her family, Gabriella's professional journey has equipped her with a broad understanding of the European markets, as well as experience in the US and Japan.

HTA VOLUNTARY COOPERATION:
CAN WE DISCUSS ECONOMIC METHODS?

- HOW DO WE EVALUATE THE RISK REDUCING EFFECT OF AN OUTCOME-BASED PAYMENT MODEL FOR ATMPS?
- VOLUNTARY COOPERATION ON HTA ECONOMIC EVALUATIONS

Dr, Douglas Lundin, Speaker

Chief Economist, TLV

Douglas Lundin is chief economist at the Dental and Pharmaceutical benefits Agency since 2011. He has a PhD in economics from Uppsala University. He has experience in working within the Swedish Competition Authority, where he studied how well competition works in different areas, such as in Sweden's food pricing.

Mark Nuijten, Speaker

Clinical and Economic Valuation Scientist, A2M

Mark is a medical doctor, health economist, valuation expert, and healthcare journalist, recognized as a leading figure in health policy and economics. With over 200 publications and active roles in scientific societies, he has shaped the field for more than two decades. Mark served as Board Director of ISPOR and Chair of the Management Board of Value in Health from 2002 to 2004. He earned his PhD in health economics in 2003 from Erasmus University, Rotterdam, and has extensive experience in clinical research and business, including as a VP at MEDTAP International and Managing Director at IQVIA Quintiles Netherlands.
Mark is the founder of A2M (Ars Accessus Medica) and a founding partner of the Minerva International Health Economic Network. His expertise includes global pricing, reimbursement consultancy, and biotechnology market potential. His work has contributed to several positive reimbursement decisions for innovative drugs across Europe, including involvement with NICE in England.

As an academic, Mark has supervised PhD programs at Maastricht and Groningen universities and is currently a Visiting Professor at Ben-Gurion University in Israel. He pioneered the application of Discounted Cash Flow methodologies for valuing biotechnology innovations, such as orphan drugs, and has developed models like the Integrated Valuation Model, addressing constraints from both payers and biotech companies. He also created a Short Course on Valuation of Innovative Drugs for ISPOR congresses in 2023 and 2024.

HTA VOLUNTARY COOPERATION:

CAN WE DISCUSS ECONOMIC METHODS?

- HOW DO WE EVALUATE THE RISK REDUCING EFFECT OF AN OUTCOME-BASED PAYMENT MODEL FOR ATMPS?
- VOLUNTARY COOPERATION ON HTA ECONOMIC EVALUATIONS

Francis Pang, Moderator

SVP Global Market Access and International Geographic Expansion, Orchard Therapeutics

Francis Pang is Senior Vice President, Global Market Access and International Geographic Expansion at Orchard Therapeutics and has more than 20 years of experience in pricing and reimbursement, market access, health economics and corporate development, encompassing leadership roles at Shire Human Genetic Therapies, Biogen and Amicus Therapeutics.

Francis served as a founding member of the NICE Highly Specialised Technologies (HST) Committee as the Healthcare Industry Representative and Economic Lead for 10 years. Francis currently chairs the EU Access and Value Recognition Committee for the Alliance for Regenerative Medicine (ARM).

Prior to joining the biopharmaceutical industry, Francis was the inaugural Pharmacoeconomics Research Fellow at the Centre for Health Economics, University of York and Monbusho Scholar at Kyoto University. Francis's advanced degree background allows him to operate at the intersection of science (University of Cambridge), business administration (INSEAD) and economics (University of York).

Nora Lazaro, Rapporteur

Patient Engagement Manager, EURORDIS

Nora joined EURORDIS in October 2023 as Patient Engagement Manager within the European Reference Networks (ERNs) and Healthcare team based in Barcelona. She works to support patient involvement and representation in the ERN's. Particularly, she is responsible for knowledge management and communication related activities of the European Patient Advocacy Groups (ePAGs).

Before joining EURORDIS, she worked at the European Medicines Agency (EMA) facilitating the participation of patient representatives in scientific dialogue.
She holds a Bachelor's degree in Law (Universidad de Zaragoza), a Certificate in Political Studies (SciencePo Lille) and a Master in International Cooperation and Development Policies (University Sorbonne Paris).

She is a native Spanish speaker and speaks English and French.

TEA BREAK TO FOLLOW

EURORDIS
RARE DISEASES EUROPE

FLASH DEBATE 1 : HOW INVOLVED CAN A PATIENT REPRESENTATIVE BE IN PRODUCT DEVELOPMENT AND STILL BE ELIGIBLE TO PARTICIPATE IN A JOINT HTA?

Moderated by Julien Delaye, EURORDIS

Johan de Graaf, Panelist

Chair, Dutch Pituitary Foundation

Johan de Graaf is a patient advocate who has been deeply involved in the field of endocrinology, particularly focusing on hypothalamic and pituitary conditions. Diagnosed with a tumour in the hypothalamic and pituitary region, Johan's personal experience led him to become an advocate for others facing similar health challenges.

He has been the Chair of the Dutch Pituitary Foundation since 2015 and co-chairs the patient board of the European Society for Endocrinology. Johan also plays a significant role in the European Reference Network for rare endocrine conditions (Endo-ERN) and has been elected to the EURORDIS Board of Directors in 2024. He is also involved in the HTA Taskforce of Eurordis.
He is also contributing to medical research by ascertaining the unmet needs of patients with endocrine conditions, which have led to a number of scientific publications in cooperation with health care professionals.

Dominique Sturz, Panelist

Vice-President, Pro Rare Austria

Dominique's journey as a Patient Advocate started, after her daughter was diagnosed deaf at age one in 1997, as a consequence of our positive experiences with early bilateral cochlear implantation and audio verbal hearing and speech development.

After an additional clinical diagnosis of RP (Retinitis pigmentosa) and Usher Syndrome at age 9 in 2005 and due to a lack of expertise for this rare diseases in her country, she started to connect to relevant patient organisations and leading research institutions and researchers, scientists and clinicians in Europe and the US and has acquired expert knowledge which contributes in her role as a Patient Advocate to various institutions in her country and at international level (see affiliations below).

Her daughter's diagnosis of USH1b was genetically confirmed at age 16 in 2012, as a consequence, she has expanded the focus of her patient advocacy from the CI and hearing community to the wider Retina and Rare Disease space.

In her role as Vice-President of the Austrian Rare Diseases Alliance and member of EURORDIS HTA Taskforce, she has followed the EU HTA legislation process very closely and has engaged in relevant patient advocacy at EU and national level, since she sees joint HTA, if done right, as one of the most powerful tools for equal access to innovative treatments.

FLASH DEBATE 2 : SHOULD THE EU COOPERATION WORK WITH PATIENT EXPERTS OR MORE "NAÏVE" PATIENTS IN HTA AT THE EUROPEAN LEVEL?

Moderated by Julien Delaye, EURORDIS

Florian Innig, Panelist

Patient represenrative, BKMF e.V.

Florian Innig has long been associated with the topic of dwarfism and works for BKMF e.V., the German association for short statured people and their families. In recent years, in addition to his work for the association, he has also been involved in the evaluation of medicinal products.

Since 2019, Florian is a permanent patient representative in the Federal Joint Committee's Pharmaceuticals Sub-Committee. Medicinal products are also his focus in the Board of ACHSE e.V., the German umbrella organization for rare diseases. He is in two task forces of EURORDIS.

Dr Monica Racovita, Panelist

Access and Policy Manager, Myeloma Patients Europe

Monica holds a PhD in Global Social Sciences from Doshisha University, Japan and a Master of Engineering in Applied Chemistry and Biotechnology from Nagoya University, Japan. In her undergraduate degree she studied Biomedical Engineering at the University of Pharmacy and Medicine in Iasi, Romania.

After her PhD she worked on science and health policy issues in academia, international development or consultancy in Italy, Austria and the UK. Subjects included the biosafety evaluations of genetically modified crops, Responsible Research and Innovation, Artificial Intelligence and the Internet of Things and public health.

A desire to work for non-profit organizations and in particular for one representing patients led her to MPE, which she joined in January 2024. Monica comes from Romania and currently resides in the UK. She speaks Romanian, English and Italian and has a working/partial understanding of French and Spanish.

QUIZ AND POLL

FAREWELL AND CLOSING REMARKS FROM CO-CHAIRS