EMA multi-stakeholder workshop:
Qualification of novel methodologies

17 – 18 April 2023

Virtual meeting

Background and objectives

Qualification of novel methodologies is a voluntary pathway allowing developers of innovative methods and tools to request a qualification by European regulators of these instruments for a specific intended use in the context of research and development into pharmaceuticals. Since its introduction in 2008 it has provided a platform for iterative prospective discussion and agreement of evidence generation plans for future qualification (Qualification Advice), as well as for agreement and publication of scientific opinions once a novel methodology has been demonstrated to be valid to inform regulatory decision making (Qualification Opinion). It has also offered publication of 'Letters of Support' aiming at fostering further development when a novel methodology under evaluation cannot yet be qualified but is shown to be promising based on preliminary data.

The EMA Regulatory Science Strategy to 2025 has put forward a clear vision: 'To underpin its mission of protecting human health, EMA must catalyse and enable regulatory science and innovation to be translated into patient access to medicines in evolving healthcare systems', and has laid out strategic goals and core recommendations many of which are facilitated by the qualification of novel methodologies platform, e.g.:

- Enhance early engagement with novel biomarker developers to facilitate regulatory qualification: Critically review the EMA's biomarker validation process, including duration and opportunities to discuss validation strategies in advance, in order to encourage greater uptake and use
- Support the development of robust digital endpoints through qualification, scientific advice, and the establishment of a multi-stakeholder platform to obtain feedback on their utilisation
- Establish an EU framework for data quality and representativeness. Develop guidelines, a strengthened process for data qualification through Scientific Advice, and

promote across Member States the uptake of electronic health records, registries, genomics data, and secure data availability.

Over the last year, a focus group brought together regulators and developers to identify methodologies which will need qualification in the future and explore ways to future proof the process. Discussions on how to foster development of robust novel methodologies and optimise regulatory qualification support have also been ongoing in other fora, e.g. a multi-stakeholder workshop 'Enhancing patient-centric outcome measures and clinical trials with Digital Health Technologies' in December 2022 and in the context of the EMA multi-stakeholder workshop on 'Patient Experience data in medicines development' in September 2022.

This EMA multi-stakeholder workshop will bring together academia, learned societies, public-private-partnerships, consortia, patients, HTA bodies, Notified Bodies, medicines regulators and industry to explore the scope, process and outcomes of the qualification of novel methodologies platform, to share and discuss the focus group learnings, and to identify ways to optimise the process to further support the integration of science and technology in medicines' development while ensuring efficient and robust qualification of methodologies.

The aims of the workshop are to:

- Confirm the future scope of qualification of novel methodologies in light of ever accelerating development of science and technologies, to best support translation of innovation into patient benefit;
- Look at use case examples of different methodologies (Clinical Outcome Assessments, Digital Health Technology and Artificial Intelligence/Machine Learning related methods, Modelling and Simulation and Real World Evidence related methods), share procedural experiences and solicit input from stakeholders to identify recommendations to futureproof the qualification of novel methodologies process and its outcomes.

EMA multi-stakeholder workshop on Qualification of novel methodologies

Chaired by Thorsten Vetter (EMA) and Paolo Foggi (AIFA)

Day 1 - 17 April 2023, 13h00-18h35 (CEST)

12:30 Joining and technical checks

13:00 Welcome and setting the scene

Welcome and workshop objectives **5'**
Michael Berntgen (EMA)

Setting the scene: current procedure and experiences **15'**
Thorsten Vetter (EMA)

13:20 Session 1: From innovation to qualified tools – the scope of qualification of novel methodologies

Chairs: Marjon Pasmooij (CBG-MEB) and Falk Ehmann (EMA)

The journey of innovation **15'**
Ana Drmic (University of Innsbruck)

The Pharma perspective **15'**
Solange Corriol-Rohou (Astra Zeneca)

EU public-private funded project landscape - how to enable a more seamless transition into Qualifications **15'**
Nathalie Seigneuret (IHI)

Panel facilitated Q&A session **45'**
Additional panellists:
Cecile Ollivier (C-Path Institute)
Ralf Herold (EMA)
Jean-Luc Sanne (EC DG-RTD)

14:50 *Coffee break*

14:55 Session 2: Patient-, Observer- and Clinician-reported outcomes (PROs, ObsROs, ClinROs) – key elements of patient centred medicines development

Chairs: Elmer Schabel (BfArM) and Andreas Kirisits (AGES)

Development of a PRO for upper limb function in Duchenne Muscular Dystrophy **15'**
Elizabeth Vroom (World Duchenne Organisation)

Qualifying PROs for rheumatology studies – update from EULAR **15'**
Robert B.M. Landewé (EULAR)

Regulatory perspective **15'**
Elmer Schabel (BfArM)

Panel facilitated Q&A session **45'**
Additional panellists:
Antoine Vanier (HAS France)
Wieneke Mokkink (COSMIN)
Simon Bennett (Biogen)

16:25 *Coffee break*

16:30 Session 3: Methods based on Modelling and Simulation, Digital Health Technologies and Artificial Intelligence/Machine Learning (AI/ML)

Chairs: Joerg Zinserling (BfArM) and Thorsten Vetter (EMA)

Regulatory perspective on qualification of Modelling and Simulation based methods **12'**
Flora Musuamba Tshinanu (FAMHP)

Digital measures of nocturnal scratch **12'**
Cathelijne de Gram (J&J)

AI-based pathology tools to improve clinical evidence generation and patient outcomes **12'**
Katy Wack (PathAI)

Regulatory perspective on qualification of methodologies based on Digital Health Technologies and Artificial Intelligence/Machine Learning **12'**
Joerg Zinserling (BfArM)

The Notified Bodies perspective – opportunity to collaborate with medicine regulators for qualification of novel methodologies? **12'**

Daniela Seneca (BSI Group)

Panel facilitated Q&A session **60'**

Additional panellists:

Bernd Arents (Dutch Association for People with Atopic Dermatitis)
Laurant Servais (University of Oxford)
Abtin Rad (TUV SUD)
Lada Leyens (Roche)
Ieuan Clay (VivoSense)
Elke Stahl (CTCG)
Gabriel Westman (SMPA, MWP)

18:30 Closing remarks day 1

Wrap up **5'**

Thorsten Vetter (EMA)

18:35 End of day 1

Day 2 - 18 April 2023, 13h00-16h45 (CEST)

12:30 Joining and technical checks

13:00 Welcome and summary of day 1

Welcome and intro to day 2 **5'**
Paolo Foggi (AIFA)

13:05 Session 4: Real World Evidence – Qualification of data sources

Chairs: Peter Mol (CBG-MEB) and Juan Jose Abellan Andres (EMA)

A regulatory perspective **15'**
Peter Mol (CBG-MEB, University Groningen)

The Cystic Fibrosis experience **15'**
Lutz Naehrlich (European Cystic Fibrosis Society Patient Registry)

The TREAT-NMD experience **15'**
Neil Bennett (TREAT-NMD global registries)

Panel facilitated Q&A session **45'**
Additional panellists:
Elizabeth Vroom (World Duchenne Organisation)
Julian Isla (Foundation 29, Dravet Syndrome European Federation, COMP)
Álmath Spooner (AbbVie)

14:35 *Coffee break*

14:40 Session 5: Using "Qualification" going forward – ways to optimise the platform

Chairs: Paolo Foggi (AIFA), Pierre Demolis (AFSSaPS/ANSM) and Iordanis Gravanis (EMA)

Panel facilitated working session **120'**

- **Future scope of qualifications and expertise needs**
- **Regulatory guidance and development support**
- **Procedural timelines and flexibility**
- **Patient involvement**
- **Qualification outcome format and communication**
- **Impact, uptake and lifecycle management of qualification opinions**

Additional panellists:
Lutz Naehrlich (European Cystic Fibrosis Society Patient Registry)
Julian Isla (Foundation 29, Dravet Syndrome European Federation, COMP)
Mireille Muller (Novartis)
Klaus Romero (C-Path Institute)
Andreas Kirisits (AGES)
Joerg Zinserling (BfArM)
Peter Mol (CBG-MEB, University Groningen)

16:40 Closing remarks

Wrap up: take-home messages and next steps **5'**
Paolo Foggi (AIFA)

16:45 End of meeting

List of speakers

Bernd Arents	Dutch Association for People with Atopic Dermatitis
Juan Jose Abellan Andres	Statistics Senior Specialist, Data Analytics and Methods Task Force, EMA
Neil Bennett	Global Registries & Research Manager, TREAT-NMD
Simon Bennett	Director, Global Regulatory Policy, European Region, Biogen
Michael Berntgen	Head of Scientific Evidence Generation Department, Human Medicines Division, EMA
Ieuan Clay	Director of Science, VivoSense
Solange Corriol-Rohou	Director of Regulatory Affairs & Policy for Europe, AstraZeneca
Cathelijne de Gram	EMEA Regulatory Policy Lead, Global Regulatory Affairs, Janssen
Pierre Demolis	Scientific Advisor, General Direction, National Agency for the Safety of Medicine and Health Products, France (ANSM) and Vice-chair of EMA's Scientific advice working party (SAWP)
Ana Drmic	Research Assistant at the Institute of Pharmacy at the University of Innsbruck and Collaborating Expert at the Regulatory Science and Innovation Task Force, EMA.
Falk Ehmann	Chair of the Innovation Task Force, EMA
Paolo Foggi	Head of Innovation and Pharmaceutical Strategy Division, Italian Medicines Agency (AIFA) and Chair of EMA's Scientific advice working party (SAWP)
Iordanis Gravanis	Head of Scientific Advice Office, Evidence Generation Department, EMA
Ralf Herold	Senior scientific officer, Regulatory Science and innovation Task Force, EMA

Julian Isla	Scientific Advisor of European Dravet Syndrome Federation, Foundation 29 director and Member of EMA's Committee for Orphan Medicinal Products (COMP)
Andreas Kirisits	Senior Medical Assessor, Department Clinical Assessment of Safety & Efficacy, Austrian Medicines and Medical Devices Agency (AGES) and Member of EMA's Scientific advice working party (SAWP)
Robert B.M. Landewé	Professor at Amsterdam Rheumatology & clinical immunology Center (amC) & Zuyderland MC Heerlen
Lada Leyens	Senior Regulatory Director, Roche
Wieneke Mokkink	Research Associate, Amsterdam UMC and COSMIN
Peter Mol	Senior assessor, Dutch Medicines Evaluation Board (MEB), Professor of Drug Regulatory Science Department Clinical Pharmacy and Pharmacology, UMCG and Member of EMA's Scientific advice working party (SAWP)
Mireille Muller	Executive Regulatory Policy Director, Novartis
Flora Musuamba Tshinanu	Assessor, Federal Agency for Medicines and Health Products, Belgium (FAMHP), Member of EMA's Scientific advice working party (SAWP) and EMA's Methodology working party (MWP)
Lutz Naehrlich	Professor, Pediatric pulmonologist at the University Giessen and medical lead of the German Cystic Fibrosis.
Cecile Ollivier	Managing Director – Europe, C-Path
Marjon Pasmooij	Head Science Department, Dutch Medicines Evaluation Board (MEB)
Abtin Rad	Global Director Functional Safety, Software and Digitization, TUV SUD
Klaus Romero	Chief Science Officer, C-Path Institute
Jean-Luc Sanne	Senior Expert (EC DG-RTD), European Commission

Elmer Schabel	Senior assessor, Federal Institute for Drugs and Medical Devices, Germany (BfArM) and Member of EMA's Scientific advice working party (SAWP)
Nathalie Seigneuret	Senior Scientific Project Manager at the Innovative Health Initiative (IHI)
Daniela Seneca	Regulatory Lead, Artificial Intelligence, BSI
Laurant Servais	Professor of Paediatric Neuromuscular Disease at the University of Oxford
Álmath Spooner	Director of Regulatory Policy and Intelligence, AbbVie
Elke Stahl	Assessor, Federal Institute for Drugs and Medical Devices, Germany (BfArM) and Clinical Trials Coordination Group (CTCG)
Antoine Vanier	Methodologist for the real-world data unit and the health technology assessment department, HAS, France
Thorsten Vetter	Senior Scientific Officer, Scientific Advice Office, Evidence Generation Department, EMA
Elizabeth Vroom	Chair World Duchenne Organization, Director Duchenne Parent Project Nederland
Katy Wack	Vice President, Clinical Strategy, PathAI
Gabriel Westman	Associate Professor (MD, PhD, MSc Eng), Head of Artificial Intelligence, Swedish Medical Products Agency (MPA), Member of Methodology Working Party and EMA/HMA Big Data Steering Group (BDSG)
Joerg Zinserling	Senior statistical assessor, Federal Institute for Drugs and Medical Devices, Germany (BfArM) and Member of EMA's Scientific advice working party (SAWP) and Methodology working party (MWP)