EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Scene setting on the EMA Qualification of novel methodologies (QoNM) for medicine development

Alexis Nolte, EMA Head of Human Division

Bruno Sepodes, National Authority Of Medicines And Health Products, Portugal, Vice-chair of CHMP

Joint HMA/EMA multi-stakeholder workshop on Patient Registries
12 & 13 February 2024

What is the "Qualification of novel methodologies" (QoNM)?

Purpose

Seek **scientific evaluation** on the **acceptability and suitability** of an innovative drug development **methods and tools** for use in a specific context of use related to medicines development

Scope

Focus on the **use of novel methodologies*** developed by consortia, networks, public/private partnerships, learned societies and pharmaceutical industry for a specific intended use **in pharmaceuticals R&D**

Outputs

→ Issued by the Committee for Medicinal Products for Human Use (CHMP) based on recommendations from the Scientific Advice Working Party (SAWP)

Qualification advice

Letters of support published to encourage data sharing, facilitate future Qualification, and to increase the visibility / transparency of promising methodology developments.

Qualification opinion

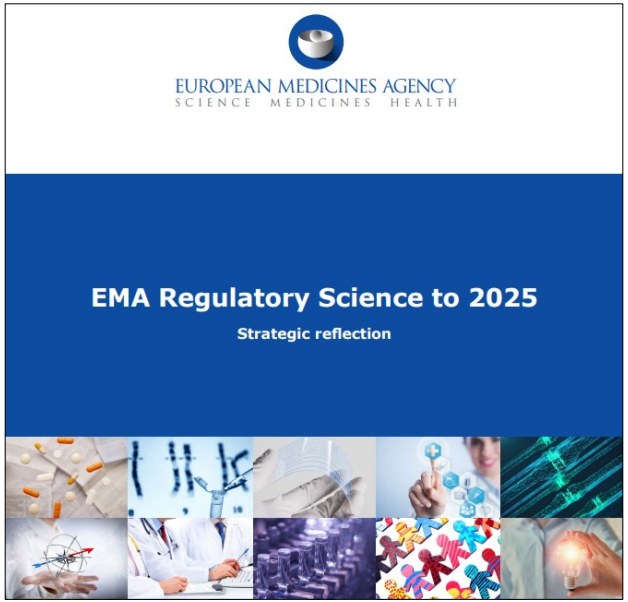
Public consultation of Draft CHMP evaluation before publication of Final CHMP qualification opinions

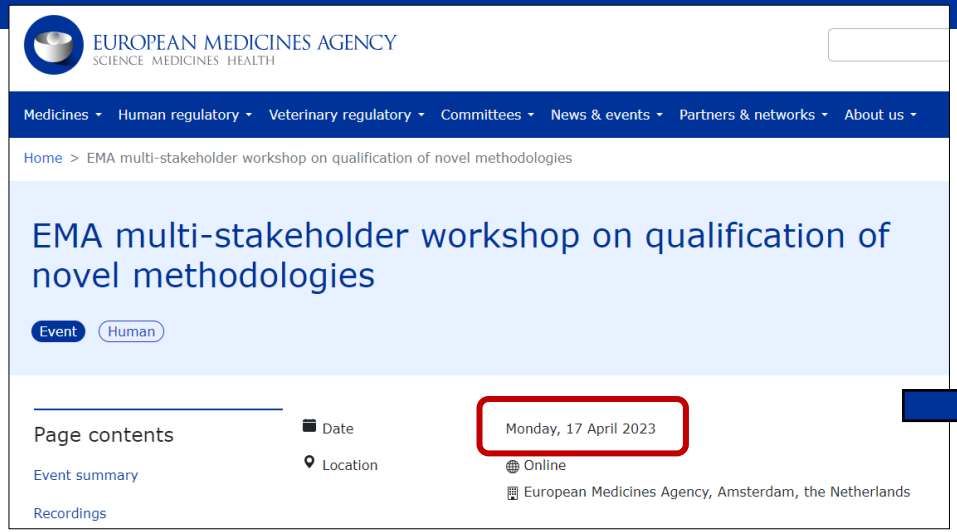
The Qualifications platform is **key** to achieving objectives of EMA Regulatory Science Strategy to 2025 (‘*RSS to 2025*’)

Several strategic goals, including:

Establish an **EU framework** for **data quality** and **representativeness**

Develop **guidelines** and a **strengthened process** for **data qualification** through Scientific Advice





Session 4: Real World Evidence – Qualification of data sources

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

A regulatory perspective *Peter Mol (CBG-MEB, University Groningen)*	**15'**
The Cystic Fibrosis experience *Lutz Naehrlich (European Cystic Fibrosis Society Patient Registry)*	**15'**
The TREAT-NMD experience *Neil Bennett (TREAT-NMD global registries)*	**15'**
Panel facilitated Q&A session Additional panellists: *Elizabeth Vroom (World Duchenne Organisation)* *Julian Isla (Foundation 29, Dravet Syndrome European Federation, COMP)* *Álmath Spooner (AbbVie)*	**45'**

Aims

- **Confirm the future scope** of qualification of novel methodologies to best support translation of innovation into patient benefit;
- Share procedural experiences from use cases to identify recommendations to **future proof the qualification process and its outcomes**.

Outcome of session on RWE – Qualification of data sources

- Clear **definition of contexts of use**

- Clear consideration of **data quality framework**
  - ✓ Develop a checklist to help data sources prepare relevant documentation
  - ✓ Streamline communication to avoid misunderstanding and manage expectations
  - ✓ Explore how to increase agility of the process

- Need for **lifecycle management** to re-confirm registries reliability/relevance
  - ✓ Foster trialogues between registry holders, medicine developers and regulators