EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Focus Group on review and strengthening the framework for Qualification of Novel Methodologies (QoNM)

A short EMA perspective

9th Industry stakeholder platform on research and development support

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Background

- FG discussions allowed for highly valuable in-depth exploration and exchange
- Agreement that Qualification of Novel Methodologies platform is a key tool to confirming the validity of innovative methodologies for evidence generation for benefit/risk assessment of Medicinal Products
- Tasks set:

Focus group on review and strengthening the framework for qualification of novel methodologies

The objective of this group is to review the future needs of the qualification framework and on this basis provide recommendations to future proof the procedure. The group should particularly discuss the specific needs of various types of methodologies that could be subject to such qualification, including nonclinical models, novel endpoints (clinical, digital) as well as real word evidence datasets and collection methods. The following outcome is expected:

- Comprehensive overview of types of methodologies for qualification in the future on the basis of horizon scanning by developers;
- Identification of procedural needs for qualification of different types of methodologies, taking into account experience to-date;
- Recommendations to strengthen the process for qualification including options for expected outputs.

The outcome of the Focus group should be reported back at a future R&D platform meeting. Furthermore, it will inform the discussions at a workshop on the qualification process, planned for mid-2022.

Overview of types of Methodologies for Qualification on the basis of ad hoc horizon scanning survey by developers

- Survey feedback limited: 38 responses with 1199 survey visits with not all responders answering all issues raised, limiting robustness
- Identified methodologies largely in line with expectations:
    - BM's, surrogate endpoints
    - PRO/CRO/ObsRO
    - Modelling & Simulation based methods, including for BE demonstration for generics
    - Statistical methodologies
    - RWE generation, Registries
    - Digital Health Technologies, including AI/ML based methods
- Comments on improving procedural flow/duration/predictability will be considered when revising the QoNM procedural guidance after the EMA workshop on QoNM
- Suggestions for shorter procedure duration/facilitation of extensions of QOs are limited by a) the deliberation process in WPs/committees and b) the evidence requirements for QO

Identification of procedural needs

- Strong consensus that enhanced pre-competitive collaboration between developers is needed to facilitate evidence generation for Qualification
  - DEEP was presented as a digital platform which may help improving pre-competitive collaboration amongst developers, exchanging information with regulators and sharing of qualified tools amongst developers
- Procedural agility, fees and timeliness vis-à-vis quality of output
- Consensus that (mandatory) disclosure of Qualification Advice information can be helpful for developers to facilitate collaboration and avoid parallel development and wasting of resources. Implementation will need to be discussed after the EMA QoNM workshop

Procedural needs cont'

- RWE/registries and digital/AI/ML based tools indicate need for life-cycle management of qualified tools; this will be discussed further in the EMA QoNM workshop
- Best setting to qualify data sources/registries and methodologies for RWE generation will need further consideration, topic in the EMA QoNM workshop
- Qualification Advice is best suited for agreeing evidentiary requirements to extend the Context of Use of qualified methods; interest from developers for general guidance on such 'extrapolation/scaling exercises' is acknowledged, but feasibility is limited due to highly specific evidentiary considerations
- Strive to extending involvement of experts for areas/technologies not within immediate EMA remit while respecting applicable regulation (e.g. for MD/software)

Recommendations to strengthen process and outcomes

- **Learnings for implementation**
  - Update of procedural guidance for QoNM
  - Update of Q&A document on Digital Health Technologies Qualification
  - Standardisation of outcome documents (QO, LoS)
  - Improved presentation of QoNM related information on EMA webpage
- **Intermediate implementation options**
  - (mandatory) publication of Qualification Advice high-level summaries
- **Topics requiring further reflection**
  - Best framework for data source qualification/registries
  - Life-cycle management of Qualification Opinions (in particular for RWE and DHT methodologies)
  - Involvement of experts covering areas outside the immediate remit of EMA as members of the Qualification Team (e.g. MD/software)

Next steps

additional input from:

- EFPIA MSHW on digital measures, 12/13 Dec 2022
- DEEP pilot
- EMA workshop on QoNM, March/April 2022

Proposal

- Arrange for 2 additional TCs with FG members:
    a) After EFPIA MSHW to identify learnings and solicit input into EMA QoNM WS preparation
    b) After EMA QoNM WS to discuss/agree key points for strengthening the QoNM platform

EMA workshop QoNM, March/April 2023

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- Final date tbc
- EMA programme committee in place
- Meeting with industry members will be arranged in January 2023 to solicit input

I. From Innovation to qualified tools – the scope of QoNM

II. Qualification of Digital Health Technologies and AI/ML based methods

III. RWE - Qualification of Data sources

IV. PRO, ObsRO, CRO – key elements of patient centred medicines development

V. QoNM procedure going forward – ways to optimise

Thank you for your attention

Further information

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