Qualification of Novel Methodologies (QoNM)

Patient experience data in medicines development and regulatory decision making – A multistakeholder workshop

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The presenter does not have any conflict of interests.
Novel Methodologies

- Clinical Outcome Assessments (PRO, CRO, ObsRO)
- Patient-Preference-Studies
- Biomarkers
- Imaging Markers
- Symptom Scales
- Statistical Methods
- Real-world-evidence generation tools / data sources / registries
- Innovative Clinical Trial methodology (e.g. master protocols)
- Digital/AI based methods
- Etc.
EMAsupport tod evelopers of novel methodologies

• **Innovation Task Force (ITF)**
  • Early informal dialogue with opinion leaders on
    • Scientific, legal and regulatory issues
    • Products, methodologies and technologies
  • Brainstorming “style” on innovation in areas without existing guidance
  • Free of charge

• **Scientific Advice and Qualification of Novel Methodologies (QoNM)**
  • Based on scientific questions to inform and agree development/qualification plan
  • **Scientific Advice** – as part of developments for specific medicinal products
  • **Qualification of Novel Methodologies** – targeting publication of a
    **Qualification Opinion** endorsing the use of a methodology in a specific context of
    use for evidence generation for regulatory decision making
CHMP Qualification of Novel Methodologies

**Vision**
Speed up/optimise drug development and utilisation, improve public health

**Who can apply?**
Consortia, Networks, Public/Private partnerships, Learned societies, Pharma, CROs, Software developers,...

**CHMP Qualification Opinion** *(Publicly Available)*
on the acceptability of a specific use of the proposed method in an R&D context based on the assessment of submitted data

**CHMP Qualification Advice** *(Confidential)*
on future protocols and methods for further method development towards qualification – Letter of support is possible
Qualification team

2 Coordinators (SAWP or CHMP)

Experts multidisciplinary, min 4

Patient Representative

External experts if needed

Therapeutic area experts

Statistics

Project Manager (EMA)

Context of use: e.g. non-clinical safety testing, translational research

Technology platform supporting the development of the novel methodology: e.g. genomics, proteomics, ultrasound, MRI imaging

Multidisciplinary, min 4 external experts if needed
Innovative uses of digital technology to collect data in clinical research to assess and monitor benefit/risk of medicines throughout the life-cycle.
Potential benefits of DHTs in evidence generation for regulatory decision making

• New clinical outcomes assessments
• Observe patient functioning in real-world setting
• Decrease missing data/enable automated checks/passive long-term data acquisition
• Access and structured analysis to real-world data, e.g. use of electronic health records
• Off-site and remote data capture in clinical trials
• Enrol/monitor patients in distant locations
• Increase adherence to therapies
QoNM matters to patients – patients matter to QoNM

• QoNM allows confirming that new methods are fit-for-purpose and generate evidence to reliably assess benefits and risks, and provide relevant information to patients

• IMI-PREFER QO as an example of immediate patient focus and relevance, qualifying a comprehensive reference framework for planning and conducting patient preference studies to complement regulatory decision making

• Patient input to Qualification Team is crucial ensuring that information relevant to patients is collected, that measurements are feasible in daily life and that novel methods offer an advantage over established measures, e.g.:
  • Practical aspects of application of the methodology, e.g. device use during night and different day periods, frequency of measurements
  • Ability to detect change and minimal important difference
  • Anchoring to a clinically recognised instrument relevant to patients
Thank you for your attention

Further information

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DHT related experience in QoNM

- Published Qualification Opinions on DHTs:
  - Stride velocity 95th centile as a secondary endpoint in Duchenne Muscular Dystrophy
  - ProActive in COPD
  - eSource: capture of clinical study source data electronically by investigator site staff complying with GCP requirements for RCTs
  - Ingestible sensor for treatment adherence measurement

- Letter of Support:
  - IMI-Mobilise D: development of Digital Mobility Outcomes (DMOs)

- Examples of methodologies having received Qualification Advice:
  - Actigraphy based measures for e.g. mobility or scratch
  - AI/ML based image analysis and adjudication of biopsy/histology specimens
  - AI based adjudication of clinical events
  - Digital endpoints using smartphone apps