EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Qualification of Novel Methodologies

A key regulatory tool to facilitate drug development

ECRD 2018 Vienna

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Vision

- speed up/optimise drug development and utilisation
- improve public health

Qualification

- …on the regulatory validity and acceptability of a specific use of a proposed method in R&D context (in non-clinical and clinical studies)
- Voluntary, scientific pathway for innovative methods or drug development tools not yet integrated in the drug development and clinical management paradigm

One procedure with two outcomes:

- Qualification Advice, OR
- Qualification Opinion

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10 November 2014
EMA/CHMP/SAWP/72894/2008
Revision 1: January 2012[1]
Revision 2: January 2014[2]
Revision 3: November 2014[3]
Scientific Advice Working Party of CHMP

Qualification of novel methodologies for drug development: guidance to applicants

Agreed by SAWP	27 February 2008
Adoption by CHMP for release for consultation	24 April 2008
End of consultation (deadline for comments)	30 June 2008
Final Agreed by CHMP	22 January 2009

Qualification advice

- Confidential
- On future protocols and methods for further method development towards qualification
- The advice is based on the evaluation of the scientific rationale and on the preliminary data submitted to the Agency
- The procedural route is not fixed but will follow the assessment of the data

Letter of support

- Based on qualification advice, when the novel methodology under evaluation cannot yet be qualified but is shown to be promising based on preliminary data.
- Aim to encourage data-sharing and to facilitate studies aimed at eventual qualification for the novel methodology under evaluation.
- A high-level summary of the novel methodology, context of use, available data, and on-going and future investigations. The Agency publishes letters of support on this page, if the sponsors agree.

Letter of support for Patient Data Platform for capturing patient-reported outcome measures for Dravet syndrome

On 09 December 2015 the applicant Dravet Syndrome Foundation Spain requested qualification opinion for Patient Data Platform as an electronic tool for capturing patient reported outcomes in paediatric epilepsies, pursuant to article 57(1)(n) of regulation (EC) 726/2004 of the European Parliament and of the Council.

Qualification opinion

- Publicly available
- on the acceptability of a specific use of the proposed method (e.g. use of a biomarker) in a research and development (R&D) context (non-clinical or clinical studies), based on the assessment of submitted data

Qualification opinion - The European Cystic Fibrosis Society Patient Registry (ECFSPR)

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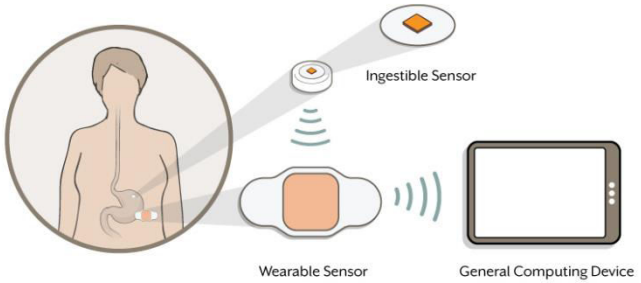
Document(s)	Language	Status	First published	Last updated	Effective Date
Qualification opinion - The European Cystic Fibrosis Society Patient Registry (ECFSPR)	(English only)	draft: consultation open	09/02/2018		

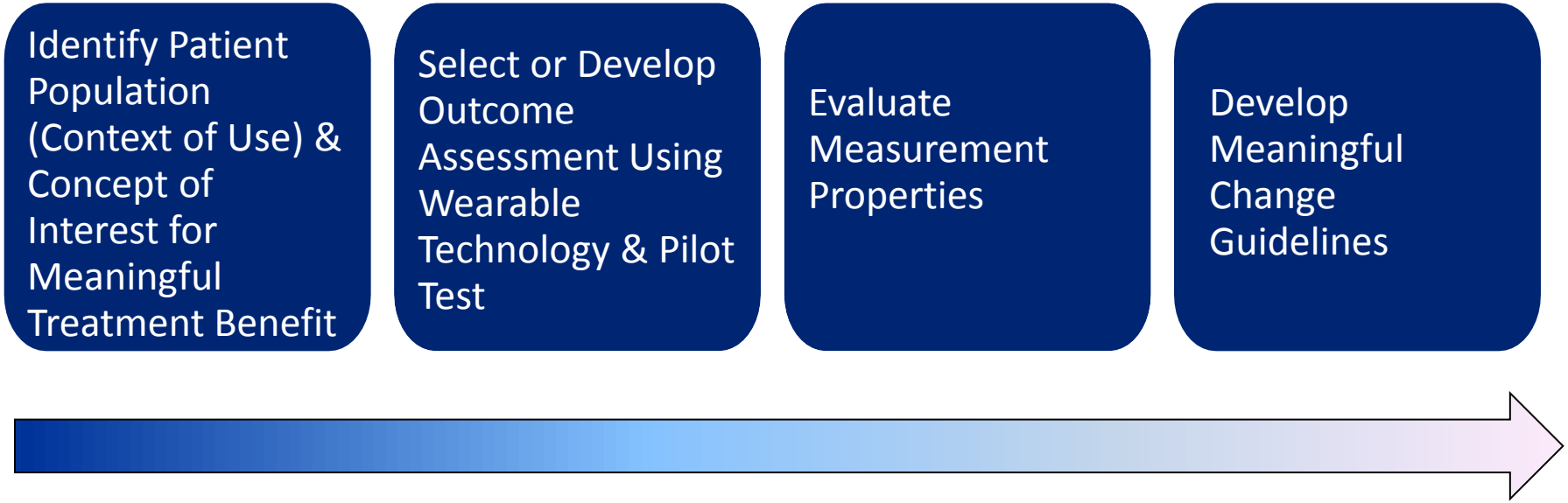
Examples of Novel Methodologies

- Biomarkers (prognostic/diagnostic and predictive)
- Clinical Outcome Assessments (PRO, ClinRO, ObsRO)
- Imaging Markers
- Symptom Scales
- Animal Models
- Statistical Methods



ingestible sensor system for medication adherence as biomarker for measuring patient adherence to medication in clinical trials

mHealth technology data must be linked to **meaningful clinical benefit** (e.g. improved patient function)



Applications throughout life-cycle

Preclinical development

- pharmacological screening
- mechanism of action
- **predict activity/safety**
- PK/PD modelling
- toxicogenomics

Clinical development

- verify MoA
- **dose/exposure-response**
- proof of concept Ph2
- **enrich/stratify population**
- **surrogate endpoint**
- Early detection of safety signals

Drug utilisation

- optimise target population
- guide treatment regimen

Qualification

Fees & Exemptions applicable

Who can apply?

Consortia, Networks, Public/Private partnerships, Learned societies, Pharma, CROs, Software developers, Patient groups etc.

Roles at EMA

Scientific Advice Working Party (SAWP)

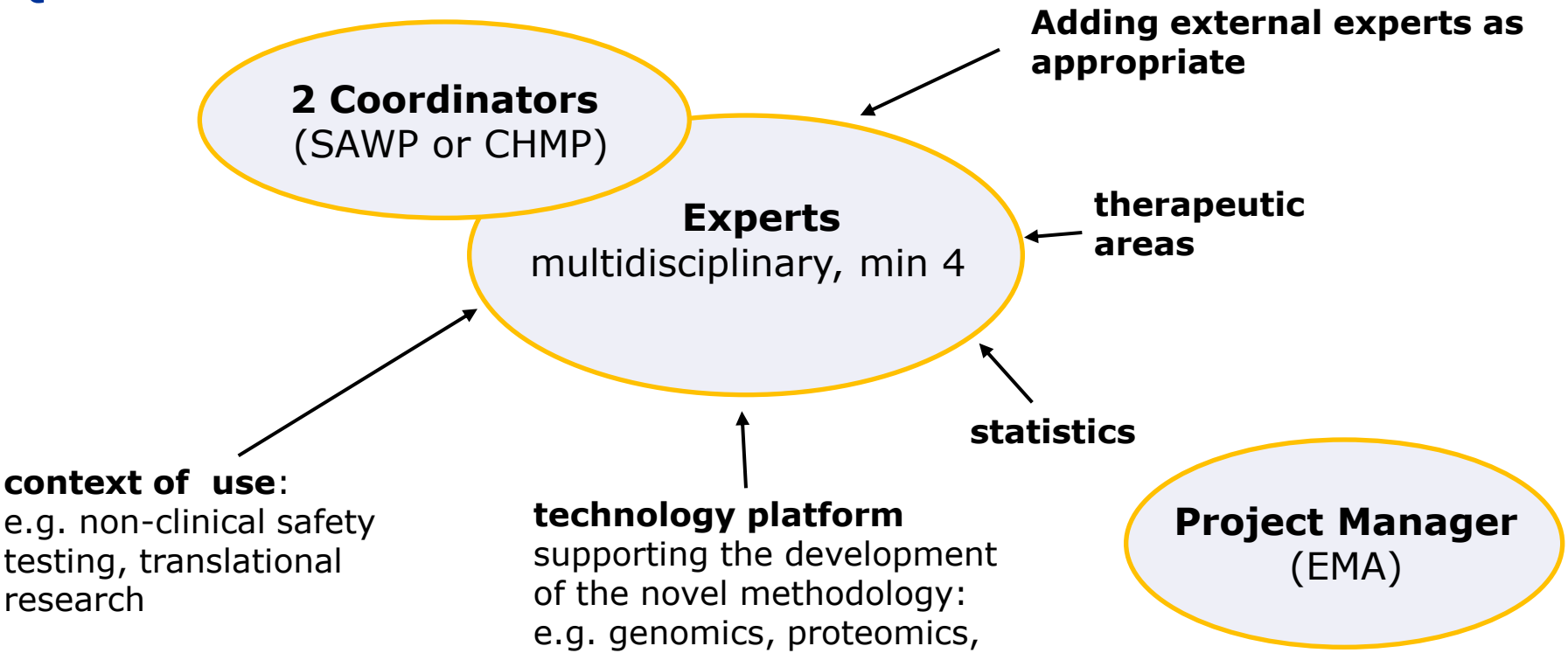
Serves as primary scientific group, allows extensive networking within the Agency (Committees, other working parties and expert groups will be involved as appropriate)

Committee for Medicinal Products for Human Use (CHMP)

CHMP member can be team member; peer review, discussion and adoption of final responses (Advice Letter or Qualification Opinion) by CHMP plenary

Helpful for future CHMP interactions, also in the context of Marketing Authorisation Applications

Qualification team



Timeline

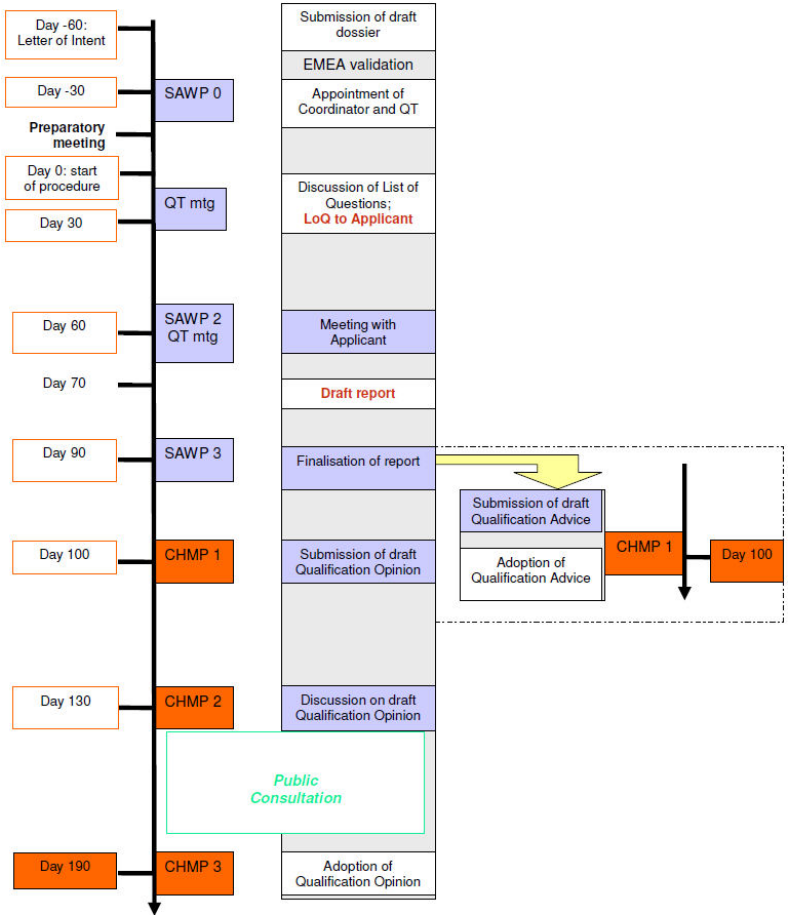
Qualification opinion:

130 days + 60 days public consultation

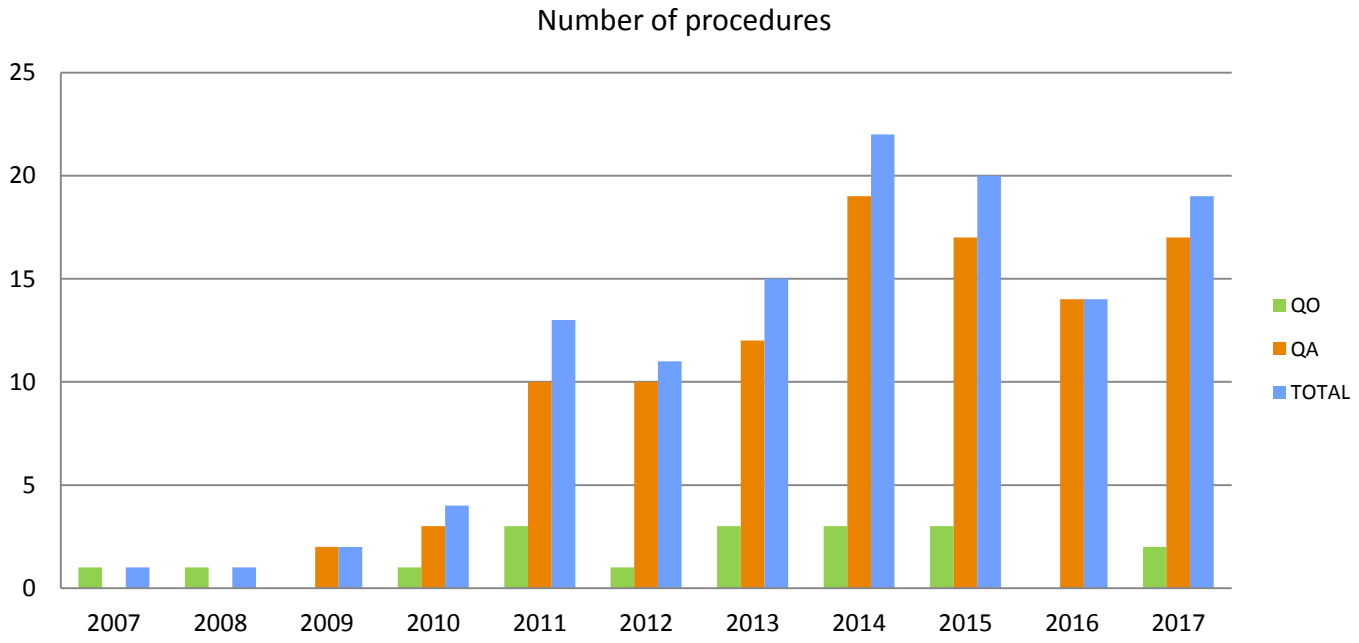
Qualification advice:

100 days

Meetings with applicant adjusted on a **case by case basis.**



Number of qualification opinions and advices



Conclusion

- Qualification is not a trivial exercise but a platform for dialogue
- Regulatory requirements are case dependent and require dialogue
- Many stakeholders (e.g. Regulators, Learned Societies, Patients, Notified Bodies)
- Many scientific disciplines (Analytical Scientists, Pharmacologists, Toxicologists, Modellers, Clinicians, Statisticians)
- Cooperation of international regulators facilitates adequate study designs
- **Long-term benefits from EMA perspective**:
  - Speed-up the time to regulatory acceptance of novel approaches and time to new marketing authorisations
  - improve public health

Thanks to:
Thorsten Vetter
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Francesca Cerreta

Any questions?

Further information

Qualification of novel methodologies for medicine development

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