EU public-private funded project landscape
How to enable a more seamless transition into Qualifications

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Contribution to regulatory science

Many of the research projects funded under IMI/IHI aim at the development of novel methodologies relevant for the development, evaluation or use of medicines, vaccines, and as such generate outputs/ results that may have on influence on the regulatory decision making and are contributing to advancement of regulatory science.

Examples of such novel methodologies :

- methods/tools to improve clinical trials/investigations of innovative health technologies and their combination
- biomarkers, endpoints, clinical outcome assessment tool or patient reporting tools,
- modelling tools to facilitate clinical trial design, patient stratification
- pre-clinical tools (e.g., safety biomarker), models for early go/no go decision

Qualification opinions

- IMI 1: 59 projects

1 EMA Qualification opinion issued in April 2018 for the PROactive patient reported outcomes tools in Chronic Obstructive Pulmonary Diseases.

- IMI2: 123 projects

So far 1 EMA Qualification opinion issued in May 2022 for the PREFER framework for patient preferences studies and points to consider document on content & methodological aspects.

- For a number of IMI1/IMI2 projects, EMA letters of support issued for wide range of promising tools.

Letters of Support

- **Necessity** : Sjögren's Tool for Assessing Response (STAR)
- **Macustar** : Intermediate Age-Related Macular Degeneration biomarker and novel clinical endpoint development (2 letters)
- **INNODIA** : Master Protocol for Type 1 diabetes prevention studies
- **Mobilise-D** : Digital mobility outcomes as monitoring biomarkers (2 letters)
- **AIMS 2-Trial**
  - N170 ERP as prognostic biomarker and for composite score measure for adaptive social functioning and its potential to stratify study populations in people with Autism spectrum disorders without intellectual disability
  - VABS-II Adaptive Behavior Composite (VABS-II-ABC) score as measure of adaptive social functioning in people with Autism Spectrum Disorders (ASD) without intellectual disability

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Letters of Support

- **SAFE-T**
  - drug-induced vascular injury (DIVI) biomarker
  - drug-induced renal tubular injury biomarker(s)
- **EU-AIMS**
  - EEG utility to measure deficits in social recognition in people with Autism spectrum disorders (ASD) and its potential to stratify patient groups
  - MRI methodology to be used to stratify populations of people with ASD
  - Eye tracking to be used to stratify populations of people with ASD
  - Measures of executive function and basic emotions to be used to stratify populations of people with ASD and predict clinical outcome
  - Clinical Outcomes Assessments utility to measure clinical symptoms in people with ASD

Interactions with regulators

- A number of projects had or have initiated qualification advice & many have engaged with EMA through the Innovation Task Force (some also interacted with Working Parties).
- A number of projects opted for an EMA FDA HTA parallel advice procedure.
- Some consortia engaged with FDA and EMA in parallel or subsequently through:
  - Critical Path Innovation Meeting (CPIM)
  - FDA qualification Biomarker Programme
    - 1 letter of support issued to IMI-1 SAFE-T consortium and Predictive Safety Testing Consortium (PSTC) in 2016.
    - Letters of intent accepted for Transbioline, Litmus, Tristan

DDTBMQ000113 Innovative Medicines Initiative (IMI) TransBioLine Contact: Lidia D. Mostoyv	Drug-induced liver injury biomarker panel	Safety biomarker panel that aids in identifying clinical trial subjects with potential acute liver injury caused by drugs, in whom dose reduction or dose interruption is warranted.	LOI 2/16/2021	LOI – Accepted 5/14/2021

Raising the awareness

- From IMI's inception, the Office has raised the awareness of consortia of importance of regulatory engagement.
- Contract with C-Path in 2020 to provide a central support to IMI to maximise the potential regulatory impact of the IMI projects results.
- Mapping of relevant IMI projects (mostly IMI1) - analysis & identification of information/data gaps for selected projects.
- Key findings:
  - Defined context of use from the start if data intended to be used for regulatory purposes
  - Clear robust regulatory strategy from the start
  - Data management plan tailored to specific regulatory objectives
  - Regulatory expertise needed to engage with regulatory agencies
  - Plan to pursue post project

Qualification procedure – some thoughts

- Value of qualification
- Complex process
- Timing
- Efforts & evidence required for qualification
- Length of the procedure & limited duration of project
- Prioritisation
- Resources & expertise required (consortium & Regulators levels)
- Costs
- Communication on the qualified methodologies
- Uptake of the qualified methodologies used
- Impact on the development/approval lifecycle (e.g length, efficiency, costs)

Qualification procedure – some thoughts

- Sharing practises/experience
  Some projects have published their experience like Litmus, BEAt-DKD etc.. experience from Regulators' side would also be valuable.
- How to ensure that letters of support will lead to qualification at a certain point?
- Data, new technologies, digitalisation and cross-sectoral collaboration are at the core of IHI projects:

  ➡ interplay between the different regulations/actors and path for regulatory acceptance

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Thank you for your attention

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