efpia

European Federation of Pharmaceutical Industries and Associations

SESSION 1: From Innovation to Qualified Tools
The Scope of Qualification of Novel Methodologies

The Pharma Perspective






EMA MSH WORKSHOP on QoNM

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Solange Corriol-Rohou, M.D.

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Introduction

- Advances in science and technologies have led to the development of new tools and methods that in turn support the development of new treatments.
- Qualification procedures play an increasingly crucial role in the regulatory landscape.
- The path to qualification of novel methodologies is still rather challenging to most applicants.
- Improvements are key to ensure added value for all stakeholders and the health system.

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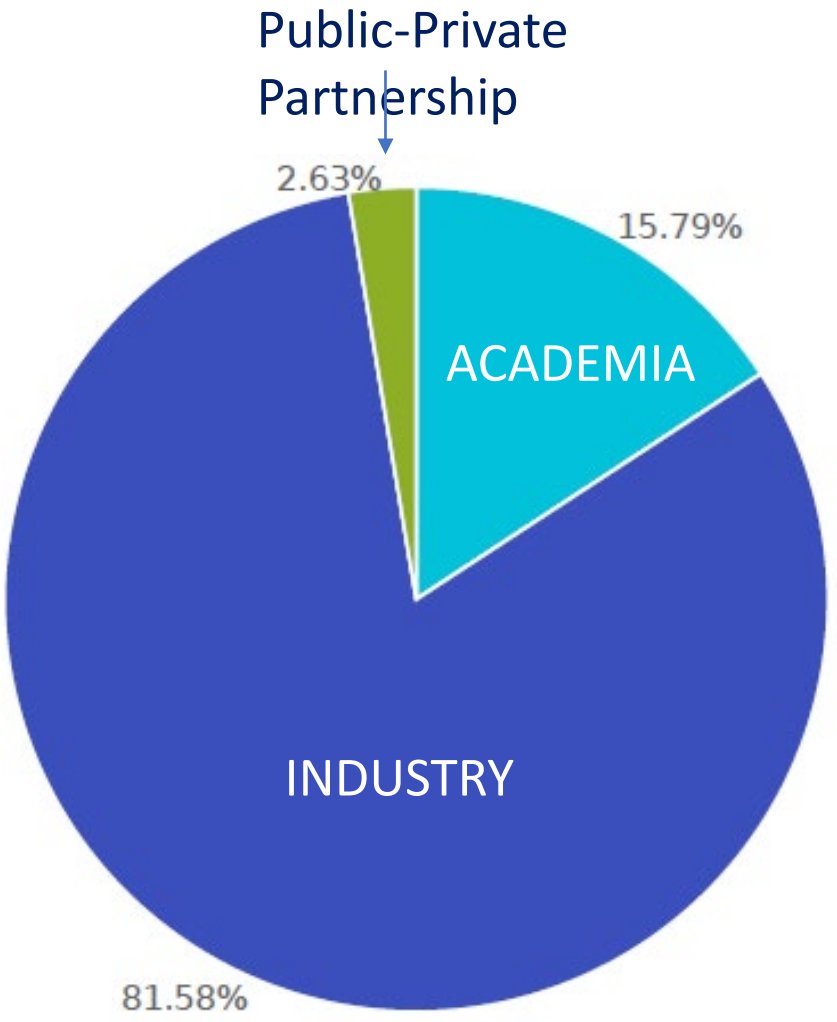
2022 - Horizon Scanning Survey

- Survey including **12 questions**, was released through **all trades**, (AESGP, EFPIA, EUCOPE, EuropaBio, Vaccines Europe, Medicines for Europe), to **Academia by EMA**, and via LinkedIn; closed on July 31, 2022

- 38 responses received and 1199 Survey Visits



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Survey - High level results

31 projects identified so far, in a variety of therapeutic domains

- Quality (CMC)
- Pre-clinical /animal testing
- Biomarkers; surrogate endpoints
- Outcome measures (including digital EPs; Clinician & Patient Reported Outcomes)
- Statistical methods
- Model-based/quantitative approaches
- AMR & virology
- Registries
- PV tools, e.g., automated adjudication of CV outcome events
- Rare disease/unmet medical needs: primary sclerosing; cholangitis; NASH; haemoglobinopathies

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Horizon Scanning Survey - High level results

Consistency in responses

Complex and lengthy process which is **resource and time intensive** and for which there aren't many **incentives to take this additional validation step**

Collaboration among companies and academia might also be required to generate sufficient data to support regulatory validation

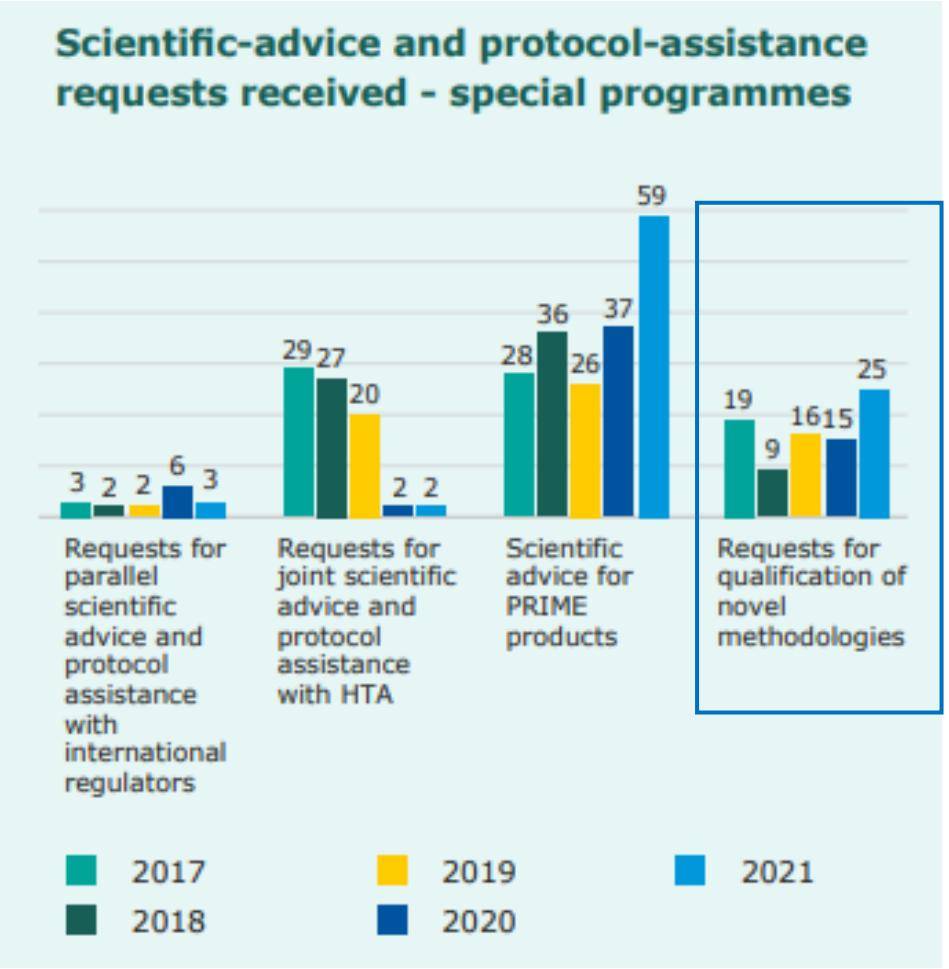
Key take-aways

Value statement to be clarified

Better awareness and additional guidance would be useful

Predictability and timelines

Qualification requests are likely to increase in future



Source: [ANNUAL REPORT 2021 (europa.eu)]

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Our Vision

Make Europe the Centre of Excellence for Regulatory Science

- Make EMA the "go to place" for qualification advice *
- Create additional value for both EMA and other stakeholders / pharma
- Build on what is currently working well and integrate into systems and procedures

Our common mindset

We do not stop at replicating the current system, we co-create and test improvements for both applicants and regulators

* in line with ACT EU goals (accelerating-clinical-trials-eu) and Regulatory Science Research Needs (regulatory-science-research-needs)

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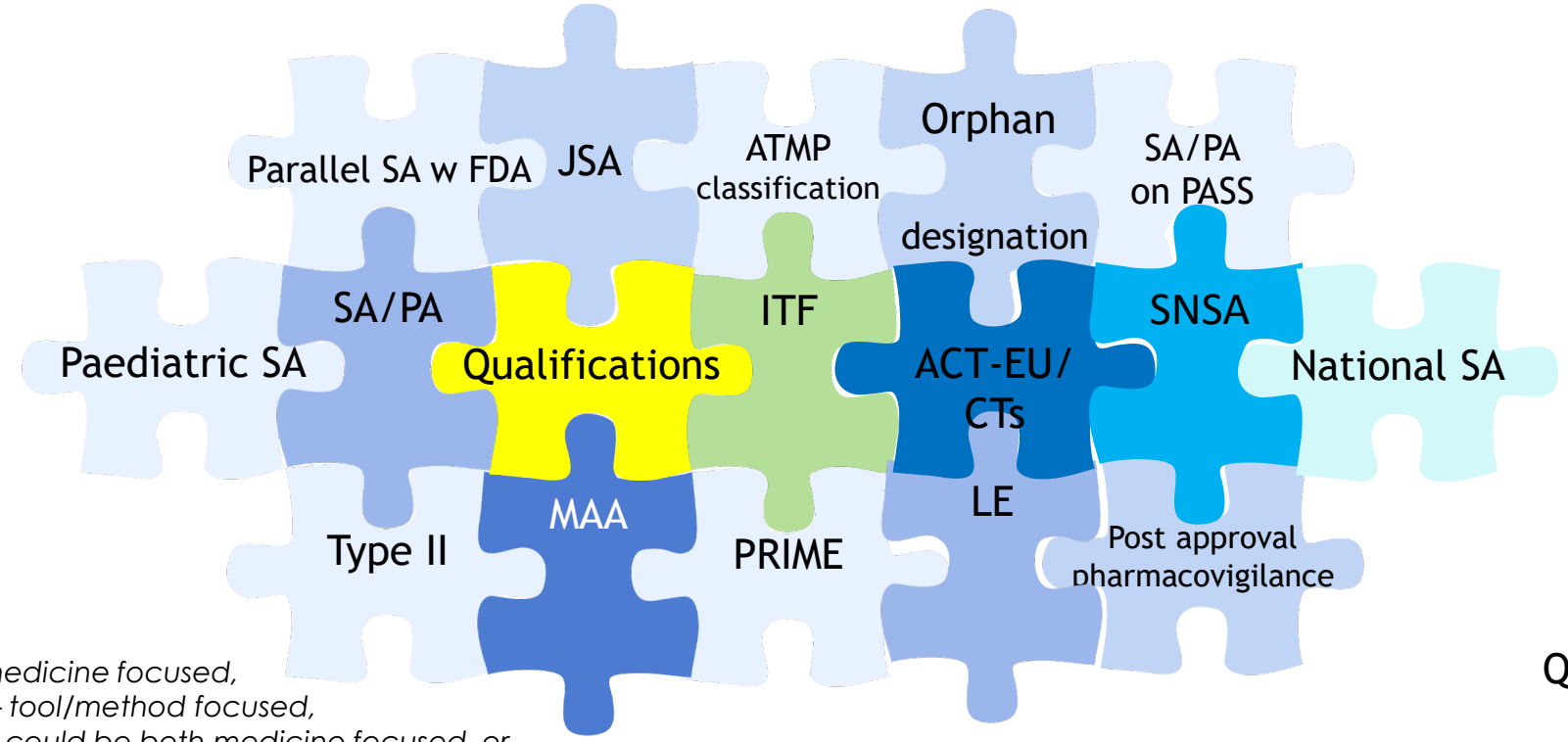
Fitting qualifications in regulatory and healthcare frameworks to ensure a future-proof and agile ecosystem



Blue *– medicine focused,*
Yellow *– tool/method focused,*
Green *– could be both medicine focused, or tool/method focused*

Qualification procedures are a unique platform for collaboration that can lead to substantial scientific and medical progress building beyond [Regulatory Science Research needs]

Qualifications need to fit in the regulatory framework and serve other regulatory procedures

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Scope & Value of the Qualification of Novel Methodologies (QoNM) for medicine development

Fundamental principles:

- Value of QoNM and how it **contributes to scientific and medical progress.**
- Scope of QoNM and how it **fits in the regulatory framework.**
- Clarity and **guidance on QoNM paths** with **clear data package expectations.**

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Scope of qualification – additional considerations (1/3)

Qualification is meant to support innovation and development of new treatments

- What proprietary technology constraints could prevent the wider use of a qualified tool?
- If technology agnostic, IP complications are lessened
  - In the context of an IMI/IHI project, IP aspects should be discussed at the beginning of the project (Field Manual)

Which element(s) of such methods/models could be of public-enough use to be eligible as qualified objects?

Flexibility to re-use qualification package elements (new device/new CoU)

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Scope of qualification – additional considerations (2/3)

Clinical design features should be considered as out of scope for qualification and be preferably discussed at SA level in collaboration with CTCG (linked to Simultaneous National Scientific Advice)

Considering the foreseen future qualifications, are there missing type(s) of expertise that the Agency should try to secure?

- One-stop shop for multistakeholder (e.g., regulators, HTA bodies, NBs) advice would be welcome
- Inclusion of Notified Bodies/NCA device experts, HTAs, CTCG, MDCG and patients' representatives
- Paediatric aspects should be part of qualification

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Scope of qualification – additional considerations (3/3)

Do the evidentiary requirements differ depending on the type of methodology to be qualified?

- A **regulatory guidance or a Q&A document describing standards for each type would be welcome.**
- Alternatively, an interactive scoping meeting (CoU/scope of qualification) could be considered

How could regulatory support be enhanced?

- Early (pre-submission) interactions following appointment of the Qualification Team ➔ a scoping meeting to have a mutual understanding on the PoS of the QoNM
- EMA contact point ➔ concept of stewardship.

How could regulators support pre-competitive collaboration? Should we advertise relevant platforms to increase awareness among prospective methodology developers?

- DEEP platform is one example; IMI/IHI, C-Path and other developers go in the same collaborative direction
- Awareness of ongoing initiatives is low

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Conclusion - A call for a qualification evolution

Novel methodologies can improve drug development and regulatory decision making

- **Qualifications are truly incubators for innovation**
- **Qualification procedures have the potential to inform regulatory decision-making throughout the lifecycle of a medicine**
  - Catalysing the integration of science and technology in drug development
  - Facilitating the translation of medical innovation in regulatory science
- **Drive collaborative evidence generation, wide knowledge exchange with active patient and other stakeholders' participation**
- **EU (EMA and EMRN) to become a qualification centre of excellence**

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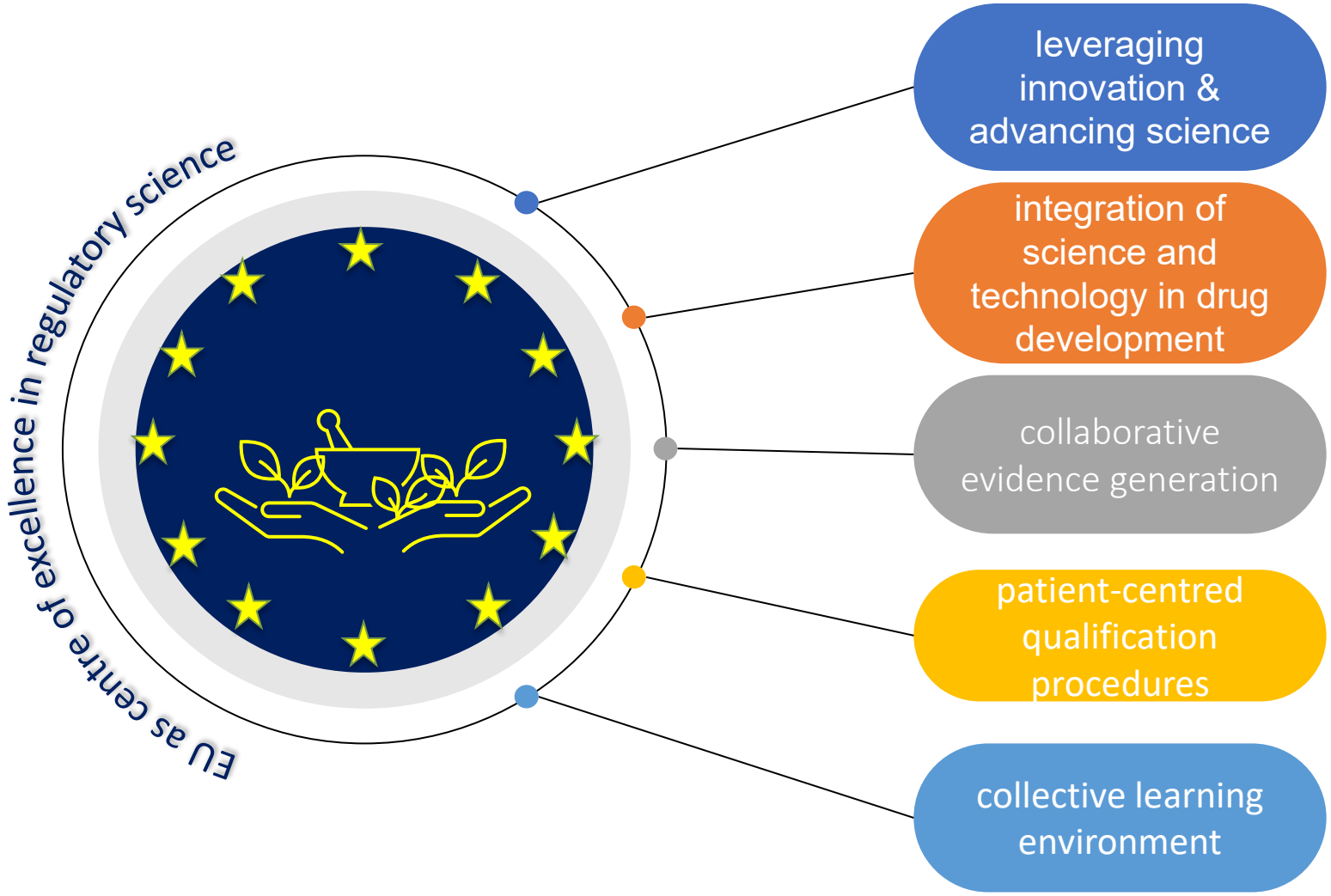
Vision and Value of QoNM



Qualifications enable innovation & foster an innovation friendly ecosystem

Allows to discuss and improve new tools and technologies that can inform drug development

Qualifications are one of the very few opportunities to generate new evidence across multiple stakeholders

Patients have a unique opportunity to take an active role in participating and influencing qualifications

"… a system in which "science, informatics, incentives, and culture are aligned for continuous improvement and innovation …"

Source: IoM 2015; http://www.learninghealthcareproject.org/section/background/learning-healthcare-system

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List of Abbreviations

AESGP	Association of the European Self-Care Industry
AI	Artificial intelligence
AMR	Antimicrobial resistance
BE	Bioequivalence
CMC	Chemistry, Manufacturing, and Controls
CoU	Context of Use
CTCG	Clinical Trial Coordination Group
EFPIA	European Federation of Pharmaceutical Industries & Associations
EMNR	European Medicines Regulatory Networks
EP	Endpoint
HTA	Health Technology Assessment
HTAb	Health Technology Assessment Body
IP	Intellectual Property
ITF	Innovation task force
LE	Line Extension
MAA	Marketing Authorisation Application
MDCG	Medical device Coordination Group
ML	Machine Learning
M&S	Modelling & Simulation
NB	Notified Bodies
PASS	Post Approval Safety Study
PA	Protocol Assistance
PoS	Probability of Success
PV	Pharmacovigilance
Q&A	Questions & Answers
SA	Scientific Advice
SNSA	Simultaneous National Scientific Advice

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