EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Facilitating innovation in regulatory science: research needs and multi-stakeholder collaboration

Academic drug development across Europe, regulatory challenges and learnings

PCWP/HCPWP and all eligible organisations meeting

Presented by Ralf Herold, Regulatory Science & Academia Workstream (TRS-ACD),
Regulatory Science and Innovation Task Force (TRS)

Example regulatory science innovation: Adaptive trial designs

Year	Milestone
1989	Bauer "Multistage Testing with Adaptive Designs"
1995	Proschan & Hunsberger "Designed Extension of Studies Based on Conditional Power"
2007	EMA Reflection Paper on "Methodological issues in confirmatory clinical trials planned with an adaptive design"
2010	FDA Draft Guidance (Drugs and Biologics)
2018	FDA Guidance "Adaptive Design Clinical Trials for Drugs and Biologics"
2019	ICH Concept paper E20 "Adaptive Clinical Trials"

Based on König F 2022

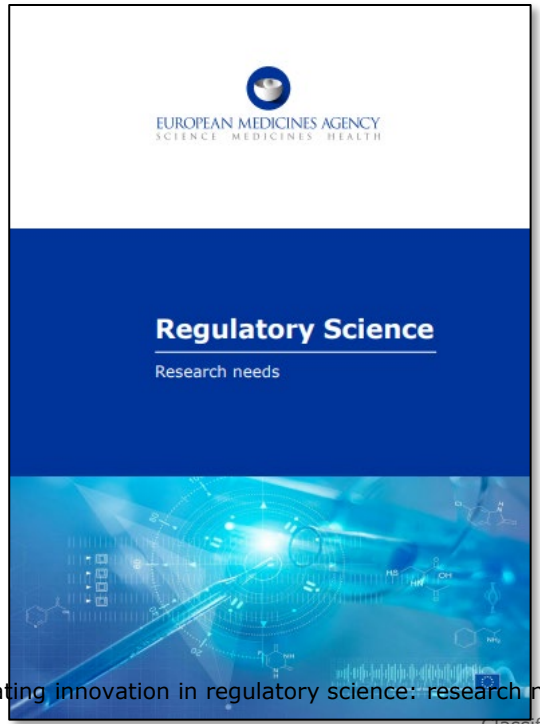
1 Facilitating innovation in regulatory science: research needs and multi-stakeholder collaboration

Address gaps in regulatory science to serve unmet public health needs

Strategic reflection led to regulatory science research needs

RSS Strategic goals	Number RSRN topics
Goal 1: Catalysing the integration of science and technology in medicines development	**12 H + 9 V**
Goal 2: Driving collaborative evidence generation improving the scientific quality of evaluations	**25 H + 16 V**
Goal 3: Advancing patient-centred access to medicines in partnership with healthcare systems	**23 H**
Goal 4 (H) / 3 (V): Addressing emerging health threats and availability/therapeutic challenges	**12 H + 11 V**
Goal 5 (H) / 4 (V): Enabling and leveraging research and innovation in regulatory science	**2 H + 1 V**
	Total: 111

Regulatory Science Research Needs (RSRN) Initiative



- **More than 100 regulatory challenges** expressed as research topics for improving medicine development and evaluation

- Regulatory science comprises the "range of scientific disciplines that are applied to the quality, safety and efficacy assessment of medicinal products and that inform regulatory decision-making throughout the lifecycle of a medicine. […] contributes to the development of regulatory standards and tools." EMA Regulatory Science to 2025 https://www.ema.europa.eu/en/documents/other/regulatory-science-research-needs_en.pdf

Regulatory Science Research Needs (version 1.0)

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Word cloud from list of 111 regulatory science research topics (v1.0)

Example topic in need of interdisciplinary research

Number (click to send email)	Research topic	Objectives	Expected impact
H2.2.1	Understand and provide solutions to the methodological, organisational and regulatory challenges for complex clinical trials that involve multiple companies and products.	To support the development of treatments in conditions where there are candidates but developmental hurdles. To foster the conduct of clinical trials with adaptive models for multiple candidates for a disease where there is no effective treatment but where there is a solid understanding of the mechanism of action. To foster multi-stakeholder dialogue into the feasibility of umbrella trials.	Potential to bring products to market and foster collaborative business models.

Translating and updating regulatory science research needs

Regulatory science research needs
- Committees, Working Parties
- EMA Academia Matrix
- External stakeholders

Engaging research funders
- EU funding institutions (Horizon Europe, ...)
- Other independent funding mechanisms
- Non-for-profit organisations

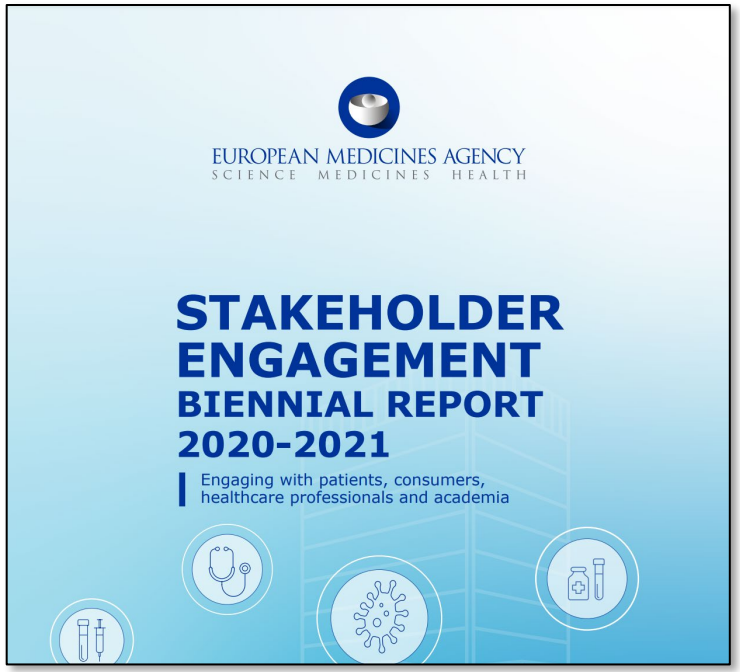
Research activities
- Academic and multi-stakeholder research
- Experts within EMA
- EMA involvement

Results
- Peer-reviewed publications
- Technical reports
- Webinars, presentations, ...

Regulatory translation
- Informing reg. science discussions
- Feed into guideline updates
- Application in SA, ITF, submissions
- Expand pool of external experts

EMA engagement with stakeholders, focus on academia

EMA systematically engages with stakeholders



- A successful model of engagement built between regulators, patients, consumers, academics, and healthcare professionals
- Engagement strengthened in crisis
- Academic stakeholders: >80 EU organisations registered. Many academics and healthcare professionals registered as EU External experts, contribute to regulatory evaluations of medicines
- Academia := higher education establishments awarding academic degrees, non-profit research organisations, international EU interest organisations

https://www.ema.europa.eu/en/documents/report/stakeholder-engagement-report-2020-2021_en.pdf

Examples of externally funded projects with EMA involvement

IMI

VALUE-Dx

EHDEN
EUROPEAN HEALTH DATA & EVIDENCE NETWORK

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

prefer.
PATIENT PREFERENCES

WEB-RADR

FLUCOP

PREMIER
PRIORITISATION AND RISK EVALUATION OF MEDICINES IN THE ENVIRONMENT

VITAL
Vaccinating Elderly for Healthy Ageing

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

conception
SAFETY EVIDENCE ECOSYSTEM

EU-PEARL
EU PATIENT-CENTRIC CLINICAL TRIAL PLATFORMS

H2020

ECRAID

PERMIT
PERsonalised Medicine Trials

EUROPEAN JOINT PROGRAMME RARE DISEASES

AVANT

STARS
STRENGTHENING REGULATORY SCIENCE

Marie Curie

MINDED

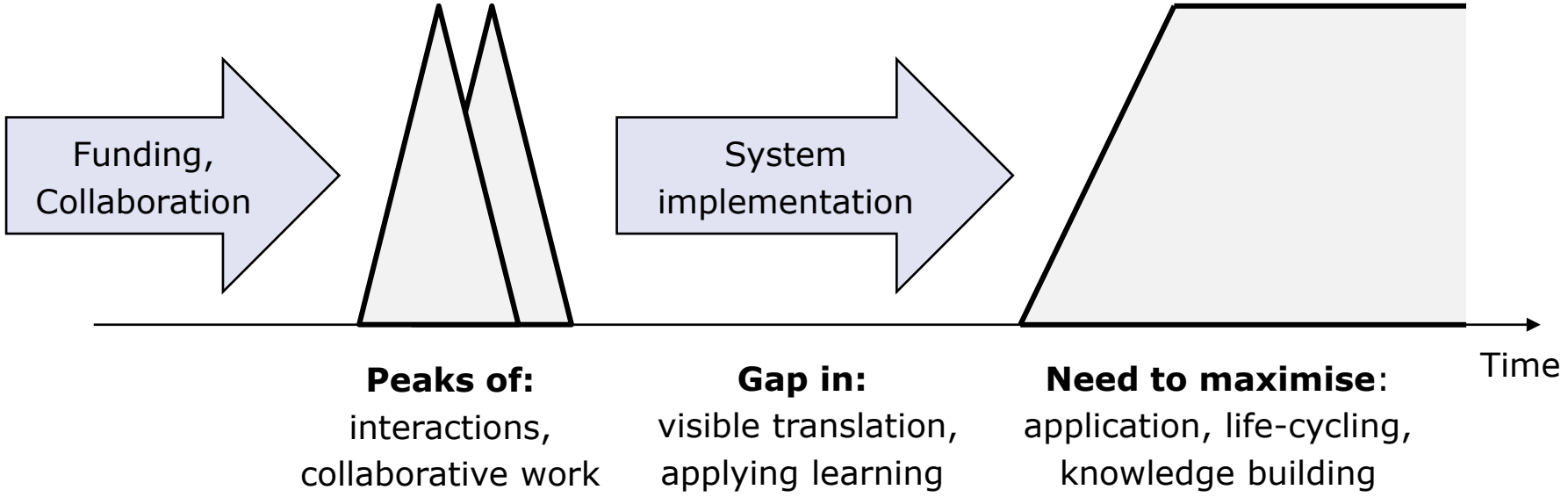
PEARRL

Other

VAC4EU

THE FORUM
For Collaborative Research℠

Common interest: accelerating regulatory science research



Example of a European Partnership: The Innovative Health Initiative

WHAT IS IHI?

FROM IMI TO IHI

IHI PARTNERS

IHI IS EUROPE'S NEW PARTNERSHIP FOR HEALTH

IHI is a **collaboration** between the EU and the biopharmaceutical, biotechnology, digital health and medical technology sectors, as well as academics, patients, regulators and other healthcare professionals.

IHI will build on the extensive experience of **IMI's 14 years and almost 200 projects** to build an interdisciplinary, sustainable, patient-centric health research partnership to help transform patients' lives.

The industry members that make up the new partnership are:

- COCIR
- EFPIA
- EuropaBio
- MedTech Europe
- Vaccines Europe

The public member in the partnership is the European Union, represented by the European Commission

innovative health initiative

IHI vision: contribute to societal challenges through ...

- **Ensure healthy lives** [UN SDG #3]
- **Sustainable industry & innovation** [UN SDG #9]
- **European policy for health and well-being** [WHO Health 2020]

- Translate knowledge into innovations*
- Innovation addressing public health needs**
- Competitive EU health industry***

- Understand health determinants and disease areas
- Knowledge generation and sharing via joint publications
- People-centred, integrated health care solutions
- Improved clinical guidelines
- Health care data management, integration, AI

- Multi-stakeholder involvement
- Cross-sector collaboration
- Engagement with regulators
- Agility of processes

* **IHI General Objective 1:** Contribute toward the creation of an EU-wide health research and innovation ecosystem that facilitates translation of scientific knowledge into innovations

** **IHI General Objective 2:** Foster the development of safe, effective, people-centric and cost-effective innovations that respond to strategic unmet public health needs

*** **IHI General Objective 3:** Drive cross-sectoral health innovation for a globally competitive European health industry

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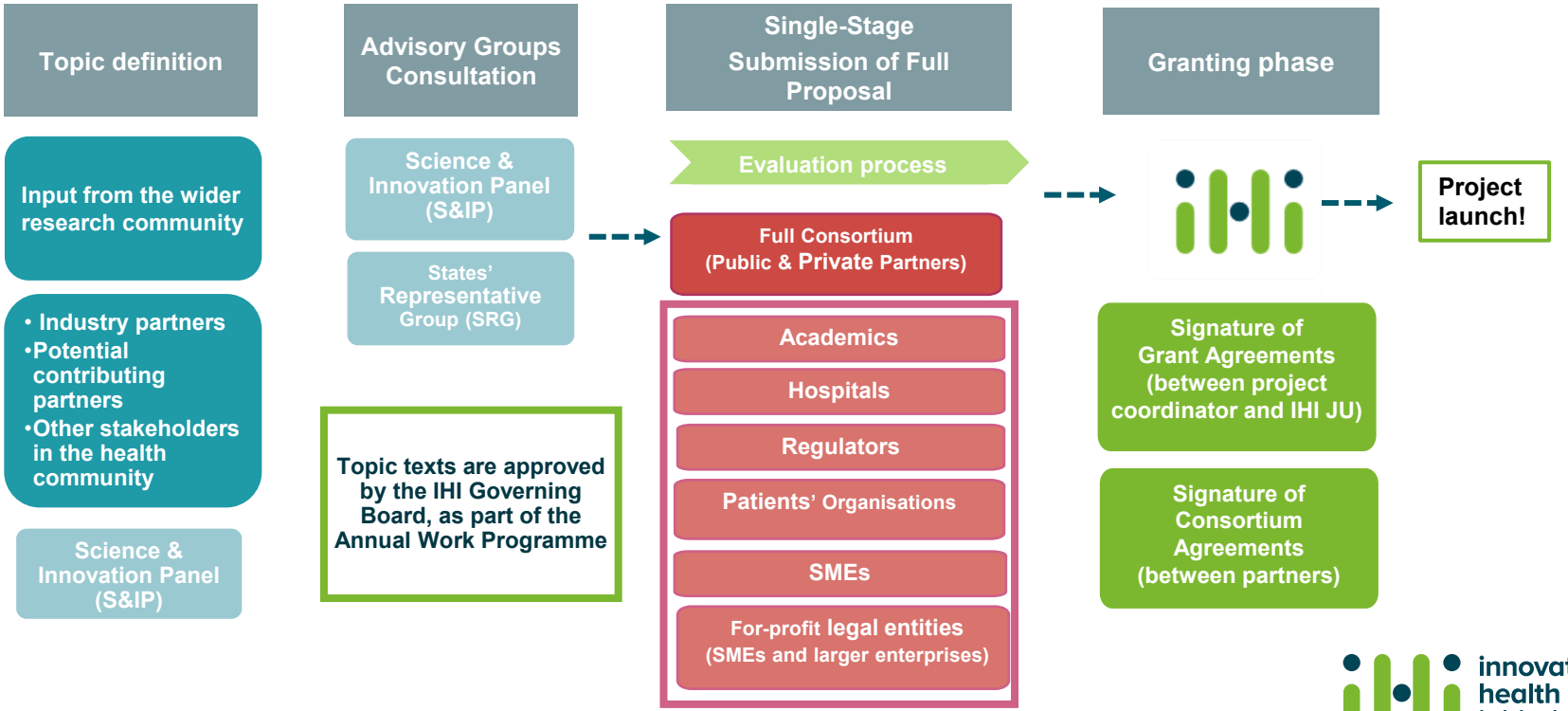
How does IHI work? single-stage procedure



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Patient Pool

Identified patients/ patient informal carers interested in participating in IMI activities both at strategic and operational levels.

Drawing from this Patient Expert Pool, the Programme Office invited expert patients/patient informal carers with the most suitable profile to perform a variety of roles and tasks.

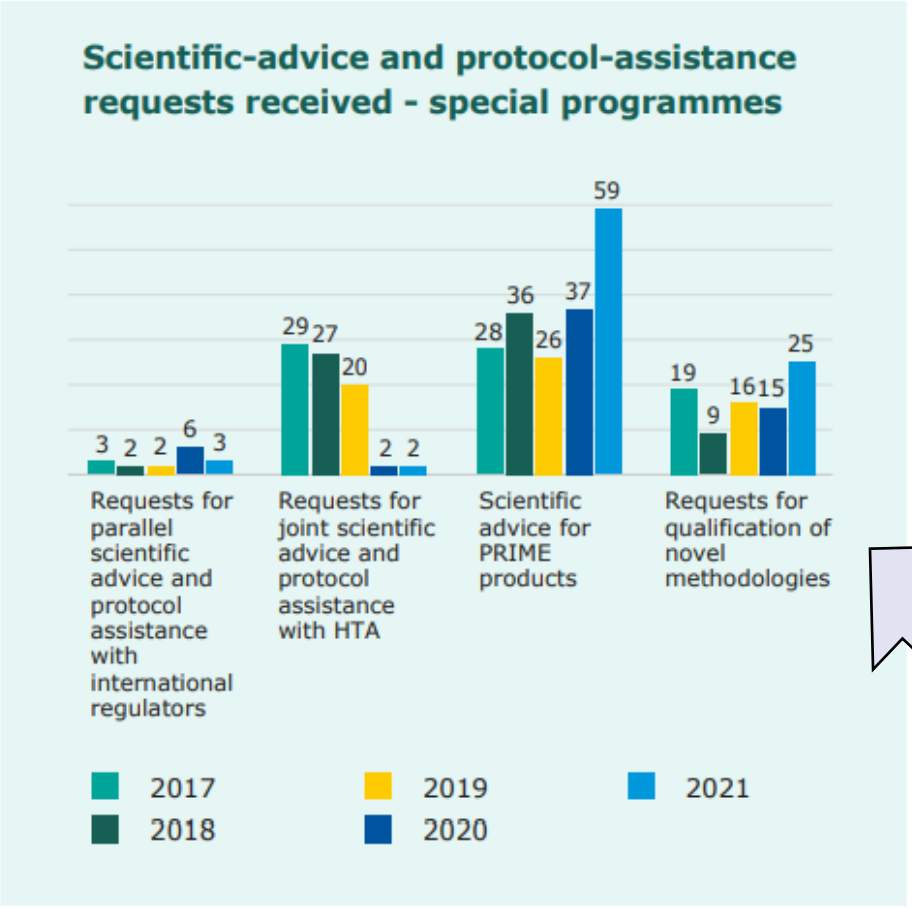
Later in the year we will re-open the patient pool to seek others who can contribute to shaping the IHI activities and improve the quality of IHI projects from a patient perspective through early and meaningful engagement.

innovative health initiative

Send us your ideas for IHI projects

- Many ideas for call topics come from our partners (industry members and the European Commission) and from contributing partners, but **we also welcome ideas from the wider health and research community**.
- All ideas will have to demonstrate
  - how they would help IHI to achieve its goals;
  - how they are aligned with the IHI SRIA;
  - why they need a cross-sectoral public-private partnership.
- Ideas will be assessed by the IHI Programme Office and governance bodies.
- We’re working on a platform and procedures to make it easy for people to submit call topic ideas. Once available, information will be placed on our website.

innovative health initiative



Opinions and letters of support on the qualification of novel methodologies for medicine development

Share

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EMA experience from externally funded projects

EMA may contribute	EMA is interested in
• Guidance on use of EMA support tools • Regulatory knowledge and guidelines • Regulatory data • Experience from previous involvement in other regulatory science projects • Active contribution to work packages • Insights into regulatory thinking and activities • Recommendations derived from consultations with other regulators	• Awareness of innovative research and of its hurdles and opportunities • Learnings from dialogue with innovators • Identification of gaps in regulatory science • New or improved methodologies or standards • Visibility through dissemination of results (publications, conference presentations) • Research network and access to expertise • Outputs tailored to regulators' demands and patients' needs

Saesen-R et al. Frontiers in Medicine 2023
https://doi.org/10.3389/fmed.2023.1181702

Summary

Important **opportunities** for patients and health professionals to engage:

- In consortia that address important needs, questions, opportunities
- Join various multi-stakeholder activities to co-create solutions
- Complement research activities with specific views and experience
- Introduce your organisations' ideas to funders of research and collaboration

Offices of EU Partnerships (and EMA) available for questions

Consider subscribing to newsletters or social media of funders (e.g., https://www.ihi.europa.eu/news-events/newsletter)

Acknowledgements

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- Phil Hines, Workstream Research and Innovation

Thank you for your attention

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