Establishing novel approaches to improve clinical trials for rare and ultra-rare diseases

IHI call 4 – topic 4

Nathalie Seigneuret
21.06.2023 • Online

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Important disclaimer

Please be aware that the call topics are still under consultation with the States' Representatives Group and the Science and Innovation Panel (IHI advisory bodies).

All information regarding future IHI call topics is indicative and subject to change until call launch.

You will find the final authorised version of the topic text on the Funding and Tenders Portal at call launch.

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Before we start…

- We are recording this webinar and it will be published on the IHI website and B2Match platform.
- We will also publish the presentation slides

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Today’s webinar

- **Will cover**:
  - Introduction to IHI programme
  - IHI Call 4 Topic 4 presented by lead industry pre-identified consortium
  - Proposal submission & evaluation
  - Tips for writing a successful proposal

- **Will not cover** rules and procedures
  - The webinar on rules and procedures is at IHI website and B2Match platform

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Innovative Health Initiative

EU's new **partnership in health** between:

- the **European Union** represented by the European Commission

&

- **Healthcare industry associations:**
  - **COCIR** (medical imaging, radiotherapy, health ICT and electromedical industries)
  - **EFPIA**, including **Vaccines Europe** (pharmaceutical and vaccine industries)
  - **EuropaBio** (biotechnology industry)
  - **MedTech Europe** (medical technology industry)

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COCIR Advancing Healthcare | efpia | EuropaBio The European Association for Bioindustries | MedTech Europe from diagnosis to cure | Vaccines Europe | Co-funded by the European Union

IHI´s general objectives

- Turn health research and innovation into **real benefits for patients and society**

- Deliver safe, effective health innovations that **cover the entire spectrum of care** – from prevention to diagnosis and treatment – particularly in areas where there is an unmet public health need

- Make Europe’s health industries globally **competitive**.

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Strategic Research & Innovation Agenda

Focus

- **Cross-sectoral approaches** to facilitate creation of new products and services to **prevent, intercept, diagnose, treat and manage diseases** and foster recovery more efficiently.

Goal

- Lay foundations for development **of safer and more effective health care products or solutions** that respond to **unmet public health needs** and that can be implemented into healthcare systems.

Research supported by IHI should remain at precompetitive level

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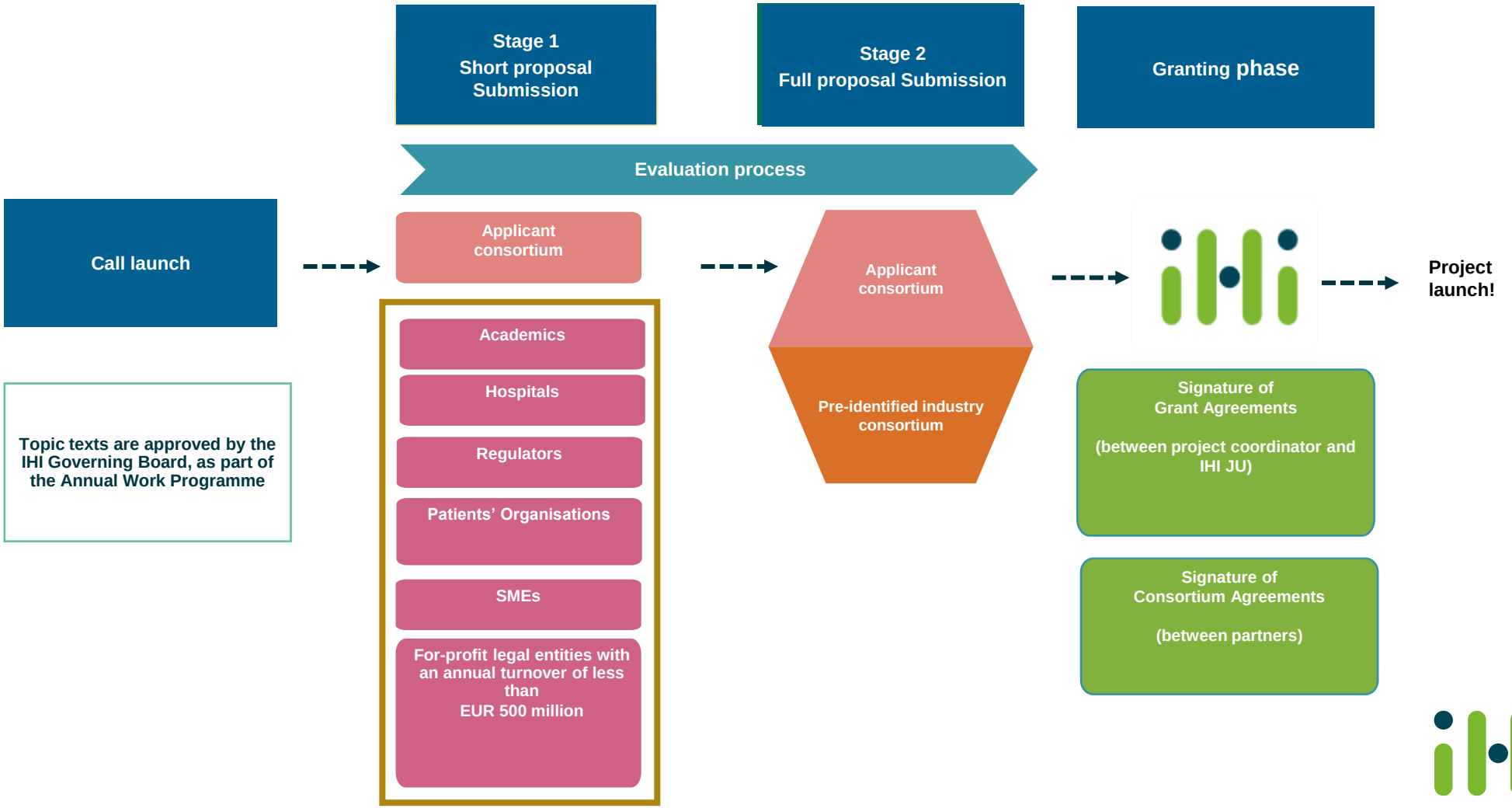
https://www.ihi.europa.eu/about-ihi/research-and-innovation-agenda

Two –stage calls – How does it work?



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Establishing novel methodological approaches to improve clinical trials for rare and ultra-rare diseases

IHI call 4 – topic 4

Solange CORRIOL-ROHOU, MD
21 June 2023• Online

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The challenge: complexities beyond those seen in common conditions

Need for holistic and inclusive solutions to address the root causes of these unmet medical needs and unlock science and development capabilities in R&D white spot areas

- For most rare diseases, disease aetiology, biology and natural history are insufficiently understood, while there are often no established endpoints for use in clinical trials.
- Enrolling, engaging and retaining patients, including patients' who may be far apart geographically.
- Designing and evaluating clinical trials, including using/identifying relevant outcome measures.
- Ensuring the quality of patient data, and enabling re-use of data (e.g., registries).
- Underdeveloped and fragmented clinical trial infrastructure for the conduct of clinical studies, including those using ATMPs and for cell & gene therapies
- An evolving and internationally fragmented global regulatory landscape

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Need for public-private, cross-sector collaboration

A concerted collaborative effort is key to deliver approaches acceptable and accessible to all health and research stakeholders and ensure implementation across Europe

- Industry partners from different sectors, e.g. the pharma, biotech, medical device, diagnostic, data ➔ integration of knowledge, technologies, experience
- Clinical centres experienced in conducting trials
- Academia and SMEs experienced in managing rare diseases
- Patients, caregivers and HCPs are required to ensure the incorporation of their voice and experience
- Regulators, policy makers, HTA experts and payers to guide the development and deployment of the playbooks

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Scope of the topic (1/2)

- Focus on paediatric and adult rare diseases ("**R&D white spots**")
- **Deliver methodological solutions for innovative clinical trial designs and analyses, including regulatory considerations** (basket trials, platform trials, *in silico* trials, RWD, Digital Health Technologies, quantitative approaches, trial with remote elements ...)
- **Identify good practices to address knowledge gaps** including collection of natural history data, development of relevant new endpoints and of patient reported outcomes (PROs)
- **Capacity building** for innovative clinical trials conduct / **education and training**
- Develop **a virtual platform for knowledge and tool sharing**, which could be also used for **Playbooks deployment**.

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Scope of the topic (2/2)

- Identify **certified/qualified scientifically and operationally CT sites** (especially in the areas of ATMPs) with readily available pools of patients ready to be recruited in CTs where appropriate.

- Once developed and established, the **Playbooks and related infrastructures will be pressure-tested** through case studies and modelling, using up to 4 selected paediatric/rare diseases (with at least one ultra-rare disease or clusters of diseases) and different types of interventions (at least one being an ATMP).

In collaboration with other initiatives wherever possible, and in particular with the EU Rare Disease Partnership

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Expected outcomes

Playbooks for novel CTs for rare diseases /clusters of diseases, to be also used for education/training; co-created with and validated by regulators, HTA bodies and patients, including:

- Good practice recommendations and expert advice for multinational innovative studies, study protocols, EHRs driven registries and longitudinal natural history studies;
- Standardized processes across all disease areas, countries and sites for fast and reliable feasibility processes, allowing for example for early feasibility assessment to support design of feasible development programmes. Effectiveness assessment of optimised CT designs as compared to the 'gold-standard' CT design for rare diseases.
- Information to support clinical research network set up to conduct innovative trials including e.g., RWE, remote elements …

Certified/qualified CT sites

Structured and predictable system of referral of patient (physically and virtually) to expert centres

Alignment and complementarity with the EU Rare Diseases Partnership to create synergies and avoid overlaps.

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Expected impact

A direct impact not only on patients with rare/ultra-rare diseases but also on all stakeholders involved in drug development

- New pathways co-created by all interested parties and new rules/best practices for early engagement to facilitate clinical developments in “white spot” areas
- Patients with rare/ultra-rare diseases will directly benefit from cutting edge clinical development of new health innovations
- Patients will have a higher probability of being assigned to active treatment, whether in Phase 2 and/or in Phase 3 registrational trials (especially critical for rare pediatric genetic diseases where window of therapeutic intervention may be relatively narrow).
- Continuum of evidence accelerates authorisation and patient access/treatment/deployment.
- Optimised and predictable referral of patient (physically and virtually) to expert centres, and alignment amongst healthcare providers (Policy makers, Regulators and HTA bodies)
- Europe becomes more attractive for the clinical development of medicines for rare/ultra-rare diseases thanks to the uptake of innovative methodological approaches for conducting successful CTs for rare/ultra-rare diseases.

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Expected contributions of the applicants

Applicant should bring together partners with relevant expertise for:

- development of new endpoints, biomarkers in rare/ultra-rare and paediatric diseases.
- epidemiology and natural history diseases.
- translational science.
- data management and standards
- devices, digital health and registries
- clinical operations
- education and training
- European Research Networks.

SMEs with expertise in clinical development in small populations, in the use of digital health technologies.

Broad geographical representation of EU countries.

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Expected (in-kind) contributions of the industry consortium

- To build solutions components that are sustainable and scalable.
- To generate site standards and quality processes to support build-up and training of research network hubs and expert sites.
- To engage and raise awareness amongst patient groups.
- To provide regulatory expertise, to help with other experts to build playbooks.
- To provide anonymized data that could be used as control arms.
- To support virtual trial platform providers, directly or indirectly.
- To support sustainability of existing trial networks and/or data sources, directly or indirectly.
- To aid centres in building referral networks.
- To provide support for the conduct of natural history studies for ultra-rare disease, identification of standard of care - patient flow, and development of patient registries.
- To seek expert input/advice for ultra-rare disease.

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Budget

Indicative budget: ~ 18 Mio Eur

- The maximum financial contribution from IHI is up to Eur 8.5
- The indicative in-kind and financial contribution from industry partners is Eur 9.1

Duration

Indicative duration: 60 Months

- This duration is indicative only.
- At stage 2, the consortium selected at stage 1 and the predefined industry consortium may jointly agree on a different duration when submitting the full proposal.

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Thank you for your attention

Got questions? Contact applicants@ihi.europa.eu

DO NOT CONTACT THE TOPIC WRITERS!

ihi.europa.eu

COCIR Advancing Healthcare

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EuropaBio The European Association for Bioindustries

MedTech Europe from diagnosis to cure

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Proposal Submission & Evaluation

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Proposal Template - Parts A, B & Annexes

- **Part A** of the proposal is **administrative data** that is entered in webforms through the Funding & Tenders Portal.

- **Part B** of the proposal is the **narrative part** that includes three sections:
  - Excellence
  - Impact
  - Quality and efficiency of the implementation
- **Read instructions** in proposal template **very carefully**

- **Annex:**
  - Participant type

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Evaluation Criteria (1/2)

- **Excellence**
  - Clarity and pertinence of the project's objectives, and the extent to which the proposed work is ambitious, and goes beyond the state of the art.
  - Soundness of the proposed methodology
- **Impact**
  - Credibility of the pathways to achieve the expected outcomes and impacts specified in the work programme, and the likely scale and significance of the contributions due to the project.

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Evaluation Criteria (2/2)

- **Quality and efficiency of the implementation**
  - Quality and effectiveness of the work plan
  - Capacity and role of each participant, and extent to which the consortium as a whole brings together the necessary expertise.

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Tips for applicants

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Tips for applicants

- Read all the call-relevant material, especially the **topic text**
  - www.ihi.europa.eu/apply-funding/open-calls
- Form your consortium **early**
  - Already think “public-private partnership“
- Ensure that **all information requested in the call text and proposal template** is provided to allow the evaluation experts to easily assess your proposal against the evaluation criteria
- Consider & plan for the potential **regulatory impact** of results

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Finding project partners

You'll need to build or join a consortium!

- Network with **your contacts & IHI Call days participants:**
- https://ihi-call-days.ihi.b2match.io/
- Use EU Funding & Tenders portal **partner search tool:**
  - https://europa.eu/!QU87Nx
- Get in touch with your **IHI national contact point**:
  - https://europa.eu/!D7jyMy
- Network on social media:
  - www.twitter.com/IHIEurope
  - be.linkedin.com/company/innovative-health-initiative

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How to book your meetings via the B2Match platform

Book your meetings in 4 easy steps

1. Make yourself available
2. Look for partner on the participants or organisation tab
3. Select date, time, attendees (up to eight per meeting), add message
4. Send the meeting request and wait for the reply

Step by Step guide on how to book meetings: https://europa.eu/!FkjV9n

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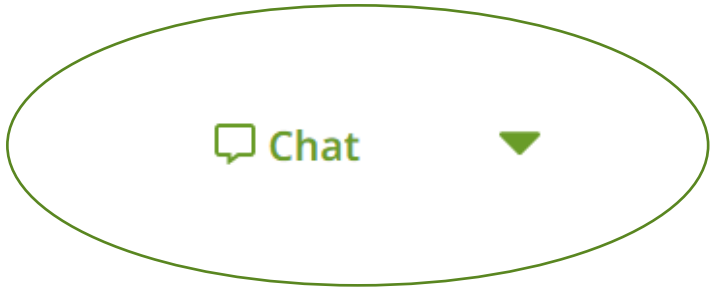
Questions time

If you want to ask a question please use the chat function on the right corner of your screen



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#IHICallDays

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Call 4

19 June	**16:00-17:30**	IHI rules & procedures
20 June	**10:00-11:00**	Expanding translational knowledge in minipigs
20 June	**14:00-15:00**	Patient-centric blood sample collection
21 June	**10:00-11:00**	Inclusive clinical studies
21 June	**15:30-16:30**	Improving trials for rare diseases
22 June	**14:00-15:00**	Safe & sustainable by design packaging & devices
22 June	**16:00-17:00**	Sustainable circular development and manufacture

Online event

COCIR efpia EuropaBio MedTech Europe from diagnosis to cure Vaccines Europe

Co-funded by the European Union

Register now

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Call 5

26 June	15:00-16:30	IHI rules & procedures
27 June	10:00-12:10	Non-animal approaches for health technologies
27 June	14:00-16:10	Theranostics solutions
28 June	14:00-16:10	Stroke management
29 June	10:00-12:10	Synthetic data generation
29 June	14:00-15:30	The financial part of the proposal

Online event

Register now

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