Strengthening the European translational research ecosystem for advanced therapy medicinal products (ATMPs) for rare diseases

IHI call 3 – topic 4

Nathalie Seigneuret
13.12.2022 • Online

innovative health initiative

Today’s session

- **Will cover**:
  - Introduction to IHI programme
  - IHI Call Topic
    - Challenge, need for public-private collaborative research
    - Scope, outcomes & impacts, budget
  - Proposal submission, evaluation & preparation tips

- **Will not cover**
  - rules and procedures
  - how to prepare the financial proposal

innovative health initiative

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)

innovative health initiative

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**.

innovative health initiative

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

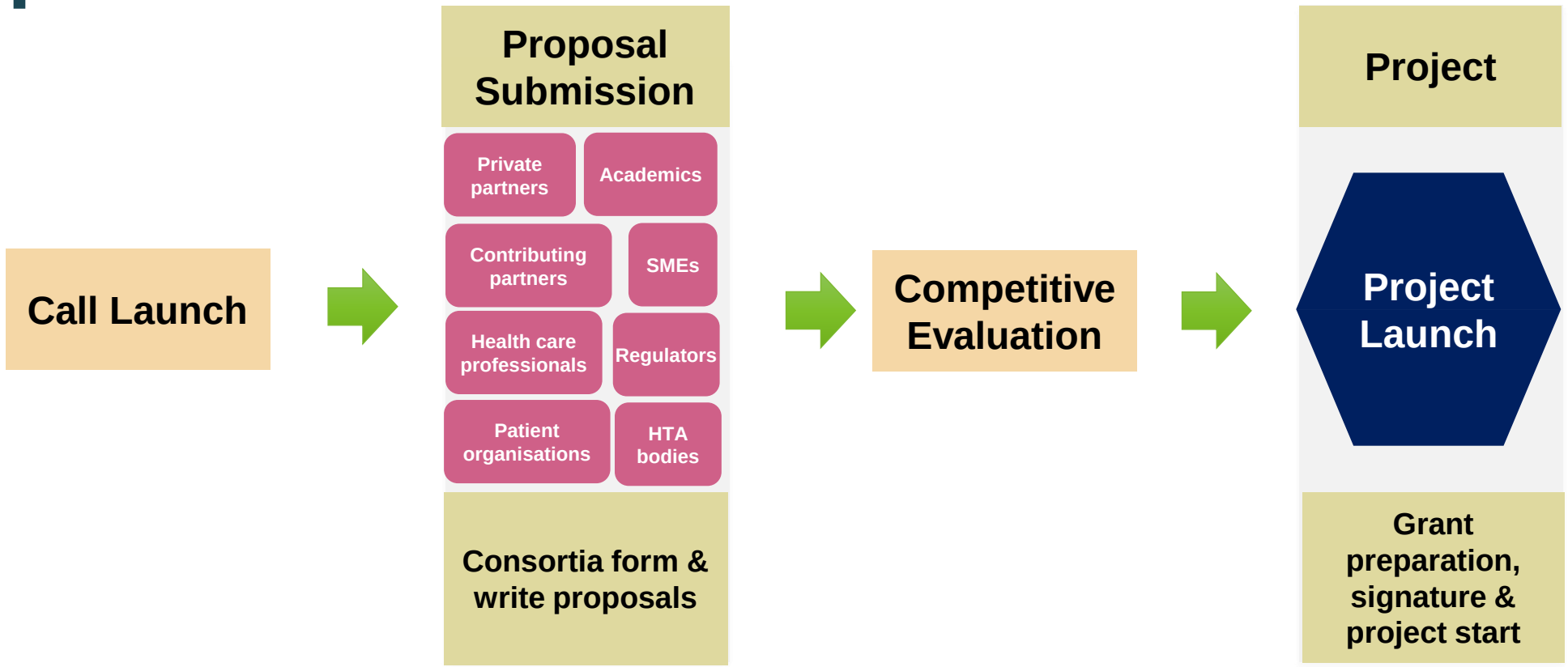
- Lay foundations for development **of safer and more effective health care products or solutions** that respond to **unmet public health needs** and that can be implemented into healthcare systems.

Research supported by IHI should remain at precompetitive level

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

innovative health initiative

How does IHI work? single-stage procedure



innovative health initiative

Strengthening the European translational research ecosystem for advanced therapy medicinal products (ATMPs) for rare diseases

innovative health initiative

The challenge

- Over 7000 rare diseases resulting in 30 million patients in Europe with a rare disease
- Less than 10% of rare disease patients receive treatment and only 1% are managed using an approved treatment
- ATMPs such as gene and cell therapies and other related innovative therapeutic modalities are very promising to treat patients with rare diseases, especially ultra-rare ones

But…

- rely on **complex technologies** where the development process is hampered by a **lack of standardisation, scalability and reproducibility**.

innovative health initiative

Need for public-private, cross-sector collaboration

Cross-sectoral trusted collaboration among all stakeholders is key to drive innovative science and technology solutions and remove key technical bottlenecks.

- Collaboration between the **research institutions, clinics** who often conduct the early research, **biotech, SMEs, pharma** & **medical technology companies** is critical to ensure that the approaches can be translated.

- Bringing on board the unique expertise of **patients and advocates in rare diseases** in this effort is essential.

- Early engagement with **regulators** is fundamental to maximise the impact of these technologies on public health and ensure they are fit for purpose.

- Connection with **clinicians and rare diseases networks** are needed to ensure an integrated development pathway for ATMPs for rare diseases.

innovative health initiative

Scope of the topic (1/4)

Overall aim of the topic:

- **Optimise** and **streamline** the development of ATMPs and other related innovative therapeutic modalities for rare diseases by strengthening the ecosystem that facilitates the transition of early pre-clinical proof-of-concept research to clinical development.

- Focus on the **scientific, technological and regulatory barriers** that are limiting **translational research** into rapid and cost-effective development of ATMPs and other related innovative therapeutic modalities for rare diseases.

innovative health initiative

Scope of the topic (2/4)

To fulfill the overall aim, proposals should:

1. Establish a **network** of **scientific and technical centres of excellence** to enable translational research in ATMPs or other related innovative therapeutic modalities, provide access and advance translatable technologies, share data and build capacity.

2. Develop **tools and methods** and **define key characteristics** of ATMPs, and **quality standards**, in particular those targeting rare diseases with no approved treatment option.
    i. Relevant therapeutic modalities must include appropriate vector systems and innovative modalities such as mRNA and nanoparticles (NPs).
    ii. Technology areas of interest could include targeted delivery, stability, transgene expression, advanced redosing approaches/reduced immunogenicity of gene delivery platforms and other underlying biology relevant to the specific therapeutic modalities.

3. Develop and support the uptake of **standardised analytical assays, methods, design strategies & translation processes to:**
    i. Reduce the timeframe and costs
    ii. Optimise manufacturing processes

innovative health initiative

Scope of the topic (3/4)

4. Demonstrate the **translatability, scalability, and robustness** of technologies which may include process development, mRNA and NP scale-up and stability, vector production, systematic assessment of the biological and mechanistic features and product characterisation, and ensuring accessibility of critical manufacturing materials.

5. Assess the methods and technological platforms developed for their **regulatory validity/utility**. **Define a regulatory pathway** to support the fit-for purpose development of ATMPs.

6. Validate the **performance of the methods and technologies** for addressing the bottlenecks in the development and manufacturing cycles of ATMPs and other related innovative therapeutic modalities. Test the functionality of the centres of excellence through **use cases.** Proposals should include a plan for **open call** to invite third parties to submit use cases at least twice during the lifetime of the project. Use cases must:

    i. showcase the utility and validity of the methods and technologies developed and verify that they are fit for purpose in the context of the current challenges

    ii. measure and adjust the capability and performance of the centres of excellence

innovative health initiative

Scope of the topic (4/4)

For the use cases clinical validation of technological solutions would be in scope (conduct of full randomised controlled trials out of scope).

7. Contribute to **strengthening the European rare disease ecosystem**

8. Define relevant **metrics and measure the use of centres of excellence**

9. Define a **plan for sustainability** beyond the lifetime of the project, including consideration for potential expansion to additional promising technological areas.

Other consideration:

Plan for **synergies and collaborations** other initiatives on rare diseases (e.g. the Accelerating Medicines Partnership Bespoke Gene Therapy Consortium, IMI project ARDAT, the European Joint Programme on Rare Diseases and the future European partnership on rare diseases, or other EU-funded consortia).

innovative health initiative

Expected outcomes

- A **sustainable network** of centres of excellence that should
  - i. advance the most promising, impactful, translatable, quality-controlled technologies that address the bottlenecks in the development of ATMPs such as mRNA, nucleic acid or NPs for delivery for gene editing.
  - ii. make these technologies accessible to all actors involved
  - iii. share information, processes and methods, and build capacity in science and technology, and regulatory awareness of ATMPs
- Consensus reached **on quality standards** and **translation process**
- Strengthened interactions with regulators to enable a more **streamlined and more transparent regulatory pathway**
- **Improved technologies/processes**, analytic tools, methods incl non-clinical, assays useful for the development and support of ATMPs and other related innovative therapeutic modalities, beyond those targeting underserved rare diseases.

innovative health initiative

Expected impact

- **Benefits for patients** both with rare and ultra-rare diseases and who may gain from effective and safe ATMPs and other related innovative therapeutic modalities.

- A better and more cost-effective **development of ATMPs** and other related innovative therapeutic modalities intended for rare diseases and ultra-rare diseases, due to improved scientific and technological processes.

- **Europe** to become **more attractive** for developing ATMPs due to the availability of sustained, interconnected technological centres of excellence with links to clinical networks, including the European Reference Networks (ERNs) on rare diseases and synergies with European Joint Programme on Rare Diseases and the future European partnership on rare diseases.

- Benefits for a **broader range of disorders** beyond the rare disease domain due to a more **robust development** of ATMPs and other related innovative therapeutic modalities as well as **knowledge transfer** across actors in ATMP development.

innovative health initiative

Budget & Duration

Budget

- Total of EUR 30 million public funding available for this topic
- IHI estimates that an IHI financial contribution of around **EUR 20 000 000 to 30 000 000** would allow a proposal to address the topic outcomes appropriately.
- At least **45% of the total budget** of each project must be covered by contributions provided by IHI Private members & Contributing partners

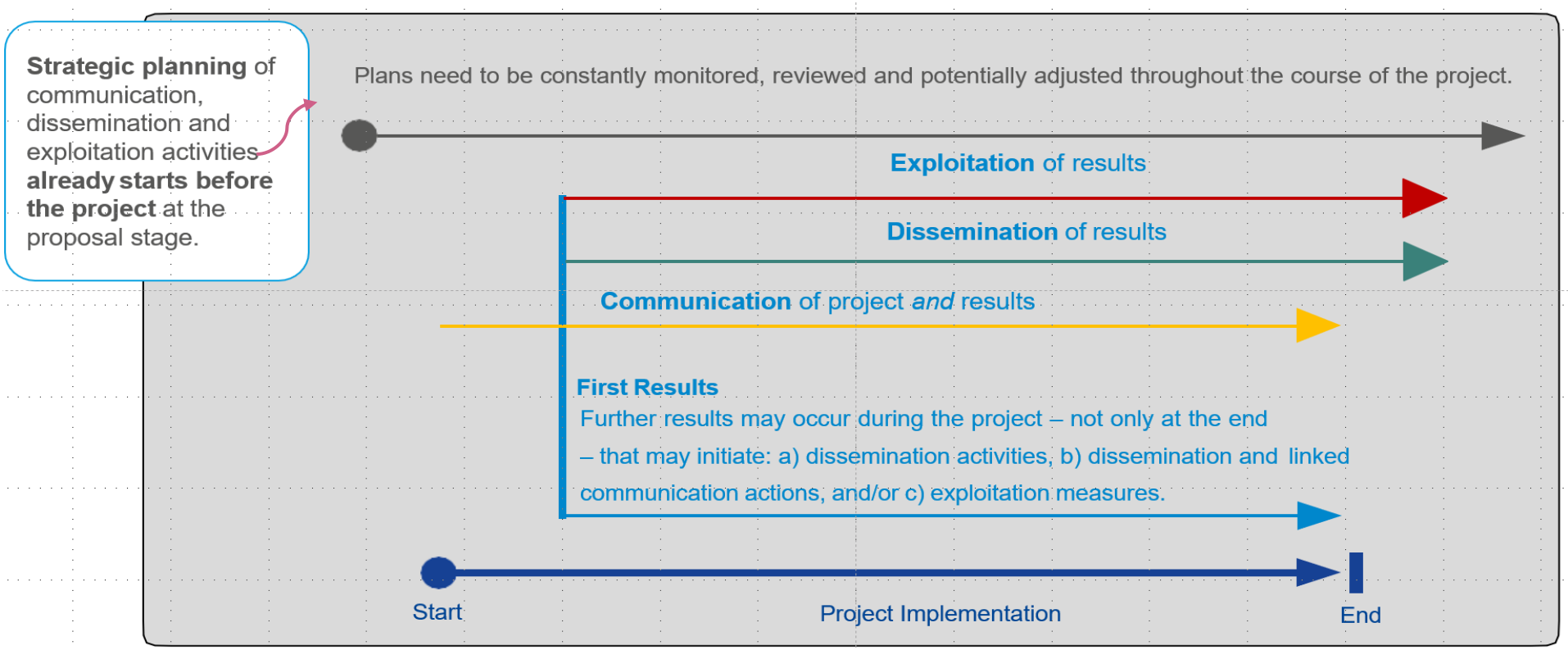
Duration

- Applicants should propose a project duration that matches the project's activities and expected outcomes and impacts.

innovative health initiative

Dissemination, exploitation and communication

- Importance to communicate and disseminate results throughout the full lifespan of the project
- Plan Dissemination & Exploitation measures to maximise the impact, incl. elements in line with the Availability, Accessibility and Affordability (3A) provisions
- Plan communication measures for promoting the project and its findings
- **Short description** of the plan for Dissemination, Exploitation & Communication activities ('PDECA"), together with the impact pathways in the proposal. This is an **admissibility condition**.
- Full-fledged PDECA to be submitted as a deliverable after the first 6 months of the project.



Dissemination, exploitation and communication

SPECIFIC NEEDS
What are the specific needs that triggered this project? ***Example 1*** ***Health solutions need to be better tailored to patients' needs. Novel approaches are needed to capture patients' needs and to involve them in the development a novel health technology.***

EXPECTED RESULTS
What do you expect to generate by the end of the project? ***Example 1*** ***Patient-centric clinical development: Patients perspectives included in design of studies.*** ***Patients' perspective incorporated into the evidence generated for decision making.***

D & E & C MEASURES
What dissemination, exploitation and communication measures will you apply to the results? ***Example 1*** ***Exploitation: Approach to include patients' perspectives is adopted by industry in their novel health technologies development programmes.*** ***Dissemination towards the scientific community and industry: Scientific publication of the results of the demonstration pilot*** ***Communication towards citizens: An event in a shopping mall to show how the outcomes of the action are relevant to our everyday lives.***

TARGET GROUPS
Who will use or further up-take the results of the project? Who will benefit from the results of the project? ***Example 1*** ***Healthcare industry companies: pharmaceutical (including vaccine), biopharmaceutical, medical (and digital) technologies, etc.*** ***Scientific community (clinical research investigations, and testing activities of health solutions)*** ***End-user of the novel health technology: patients and citizens***

OUTCOMES
What change do you expect to see after successful dissemination and exploitation of project results to the target group(s)? ***Example 1*** ***Healthcare industry partners: novel health technologies adapted to patients' needs.*** ***Use of the scientific results published (measured through the bibliometric indicators of the project publication).***

IMPACTS
What are the expected wider scientific, economic and societal effects of the project contributing to the expected impacts outlined in the topic text? ***Example 1*** ***Scientific: New approach to patient engagement in the development of novel health technologies tailored to the patients needs.*** ***Economic/Technological: Health solutions designed with the patients in mind will facilitate the adoption of the health technology by the market / healthcare system*** ***Societal: Patients will benefit from truly patient-centric health technologies (designed from the start based on their needs)***

Make sure you reserve budget / resources for D, E & C activities

innovative health initiative

Proposal Submission & Evaluation

innovative health initiative

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**

- **Annexes:**
  - Participant type
  - Budget details
  - Clinical studies template

innovative health initiative

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, including the underlying concepts, models, assumptions, interdisciplinary approaches, appropriate consideration of the gender dimension in research and innovation content, and the quality of open science practices, including sharing and management of research outputs and engagement of citizens, civil society and end users where appropriate.
- **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.
  - Suitability and quality of the measures to maximise expected outcomes and impacts, as set out in the dissemination and exploitation plan, including communication activities.

innovative health initiative

Evaluation Criteria (2/2)

- **Quality and efficiency of the implementation**
  - Quality and effectiveness of the work plan, assessment of risks, and appropriateness of the effort assigned to work packages, and the resources overall
  - Capacity and role of each participant, and extent to which the consortium as a whole brings together the necessary expertise.

innovative health initiative

Tips for applicants

innovative health initiative

Tips for applicants

- Read all the call-relevant material, especially the **topic text**
  - https://www.ihi.europa.eu/apply-funding/ihi-call-3
  - https://www.ihi.europa.eu/apply-funding/call-documents
- Form your consortium **early**
  - Always think “public-private partnership“
  - Include partners bringing **in-kind contributions**
- 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

innovative health initiative

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

innovative health initiative

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

The platform will be available for meetings from the 22 November 2022 to 20 April 2023 and not only during the IHI Call Days

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

innovative health initiative

Additional Slides

innovative health initiative

Simplified example budget

Participant type	Total eligible costs + IKAA	Requested IHI funding Reimbursed eligible costs	In-kind contributions (IKOP, IKAA, FCs)
'Public' partners (Universities, hospitals, SMEs, patient orgs, regulators…..)	15 million	15 million	0
Private members & contributing partners (requested funding = 0)	15 million	0	15 million
Private members & contributing partners ("Hybrid")	10 million	5 million	5 million
Total	**40 million**	**20 million** (50%) public funds	**20 million** (50%) private funds

innovative health initiative

Pitching Sessions

We are organising individual pitching sessions per call 3 topic
You can join the sessions via the B2Match platform

Monday, 12 December

15:30-16:30 (CET) Digital health technologies for the prevention, and personalised management of mental disorders and their long-term health consequences

Tuesday, 13 December

15:30-16:30 (CET) Strengthening the European ecosystem for Advanced Therapy Medicinal Products (ATMPs) and other innovative therapeutic modalities for rare diseases

Wednesday, 14 December

11:30-13:00 (CET) Screening platform and biomarkers for prediction and prevention of diseases of unmet public health need.

16:00-17:00 (CET) Patient input and patient generated evidence to improve patient outcomes, support decision making, and accelerate innovation

Thursday, 15 December

12:30-13:30 (CET) Combining hospital interventional approaches to improve patient outcomes and increase hospital efficiencies

innovative health initiative

innovative health initiative

Thank you for your attention

ihi.europa.eu

COCIR Advancing Healthcare

efpia

EuropaBio The European Association for Bioindustries

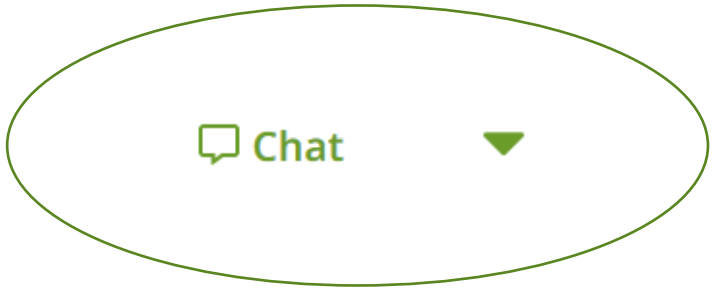
MedTech Europe from diagnosis to cure

Vaccines Europe

Co-funded by the European Union

Questions time

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



innovative health initiative