Novel endpoints for osteoarthritis (OA) by applying big data analytics

IHI call 8 – topic 2

Geraldine Joanny, IHI Scientific Officer
20.06.2024 • Online

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Before we start…

- We are recording this session and it will be published on the IHI website. We will also publish the presentation slides.
- The call will be launched shortly and all links and details of how to apply will be published on the IHI website and the Funding and Tenders Portal.
- If you want to ask a question please use the chat function on the right corner of your screen.

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Today’s webinar

- **Will cover**:
  - Introduction to IHI programme
  - IHI call 8 topic 2 presented by lead of the pre-identified industry consortium
  - Proposal submission & evaluation
  - Tips for writing a successful proposal

- **Will not cover**
  - Rules and procedures - Presentation & Recording

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Innovative Health Initiative

EU **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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IHI projects are…

Created via open and competitive calls for proposals

Cross sectorial public private partnerships leveraging:

- Contributions from industrial partners from the IHI industry associations (COCIR, EFPIA including Vaccines Europe, EuropaBio, MedTechEurope)
- if relevant, contributions from contributing partners (must be approved by IHI GB)

and

- Public funding via European Commission (Horizon Europe)

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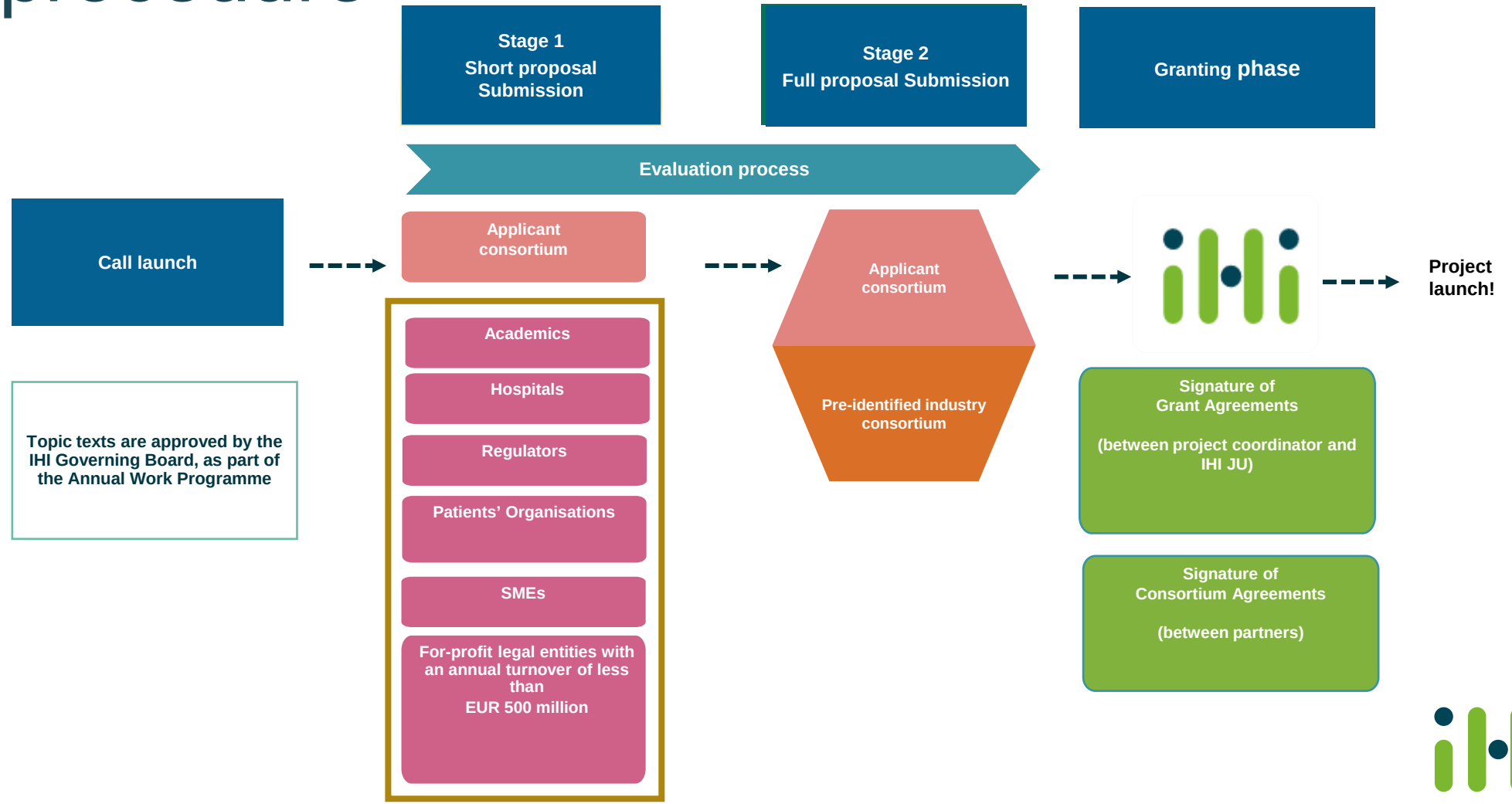
https://www.ihi.europa.eu/about-ihi/research-and-innovation-agenda

How does IHI work? Two-stage procedure



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Novel Endpoints for Osteoarthritis (OA) by applying Big Data Analytics

IHI call 8 – topic 2

Matthias Schieker, Gunther Jansen, Franziska Saxer, Gwen Nicolas
20 June 2024 • Online

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Osteoarthritis – The challenge

- Osteoarthritis (OA) affects the lives of more than 57mn people in Western Europe* and 500mn people worldwide, significantly reduces quality of life, is associated with increased mortality and has relevant personal financial and health economic impact.
- OA prevalence is expected to increase due to the prevalence of risk factors such as ageing, obesity and knee injuries and annual health care costs in key European countries are up to 7bn €*
- Key challenge in drug development for OA is to measure treatment effects in a slowly progressive disease with a heterogeneous pathomechanistic background and different disease trajectories
- Various development efforts over the years have failed to provide a disease modifying treatment and it is crucial to stratify the different patient groups and target treatments to their needs.
- Concerted action of various fields of expertise (clinical practice, AI-based image analytics, data science, bioanalytics, epidemiology, risk modelling, early and late-stage drug development, movement science, (epi)genetics and patient advocacy) can unravel and link the hidden insights from the plethora of existing data to generate tangible strategies to develop OA treatments

*Berenbaum et al, The unrecognised burden of osteoarthritis: unmet needs in Europe, The Economist Intelligence Unit Limited 2021

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Need for public-private, cross-sector collaboration

- Efforts to develop insights into disease drivers and to develop disease modifying pharmacological treatments that address pain, function and joint survival have been fragmented and futile for decades.
- Typically small sample sizes in early trials, the lack of stratification, the insensitivity of traditional biomarkers and outcome measures such as conventional x-ray as well as the vulnerability to confounders specifically of patient reported outcomes for pain have contributed to this failure.
- In view of increasing patient numbers and the devastating impact from OA, it is time to assemble an interdisciplinary team of of clinical and scientific experts, health technology innovators, affected patients, their caregivers, HTA bodies and regulators to tackle this complex pathology leveraging AI that finally allows for the management and analytics of an important amount of data.
- Only concerted action and cross-sectoral public private partnership incorporating various fields of expertise across various (health) industry sectors can bring together the necessary skills to unravel and link the hidden insights from the plethora of existing data and translate this newly generated knowledge into tangible strategies to treat this underestimated disease.
- The IHI JU provides a framework for bringing together the various public and private stakeholders as well as facilitating a structured dialogue including patients, caregivers, physiotherapists, nursing home specialists, primary care physicians and regulatory authorities. The action generated by this topic can provide a safe space in which patient stratification, endpoint development and the implementation of digital assessments can be discussed at a pre-competitive level breaking down existing silos and establishing a common ground and framework for guiding future trials.

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Osteoarthritis – Scope of the topic

The overall aim of this topic is to build a public-private partnership that is able to integrate and leverage the plethora of existing and currently-collected data on OA*, as well as the increasing insights and expertise gathered over decades of research. Further, the goal is to use a data driven approach to significantly progress the field by leveraging the novel opportunities that have emerged thanks to increased computing power and innovative methodologies in big data analysis, in order to:

1) integrate different perspectives to improve the understanding of osteoarthritis as a complex disease;

2) foster progress towards regulatory validation of patient-relevant endpoints to measure and predict OA disease progression as well as alternative endpoints to measure response to treatment;

3) allow predictive modelling while actively seeking feedback to incorporate the perception of patients, care givers, primary care physicians and regulators.

*Note that the implementation of prospective clinical studies is not supported by this topic.

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Osteoarthritis – Expected impact

- The federatad **integration of big data from disparate data sources** including the use of digital twin and similar methodological approaches will lay the foundation for advanced clinical trial designs that allow for more efficient and smaller trials, as well as the reduction of patients' burden and exposure to placebo;
- The **development of predictive models for disease progression and joint replacement** which are crucial to efficiently discuss treatment strategies, support assessments of quality in health care and equitably plan and allocate health care resources. In addition, such predictive models can revolutionise outcome trial designs, shortening the trial duration and patient burden as well as reducing development costs.
- **Stratification of different patient groups and targeting treatments to patients' needs and preferences** which enables the development of successful therapies, informs development strategies, improves patient and caregiver engagement and optimises trial designs. This stratification also supports data-based shared decision making for health care solutions in clinical practice;
- **Availability of tools that enable specific functional measurements** and reflect the real-life treatment benefit for patients. These tools have been positively evaluated for practicality and scientific validity and could be used for systematic assessments complementing clinical and patient reported information.
- All of the above will allow for better trial designs that can demonstrate the treatment benefits of medicines and health care solutions in early development programmes with limited numbers of patients.

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Expected outcomes

- **Algorithms and models, including Artificial Intelligence (AI)-based models**, that are adaptable to differences in data availability have been developed and validated in different datasets to allow for the identification of osteoarthritis (OA) patient subpopulations (phenotypes/endotypes) that will benefit from specific, targeted treatment approaches.
- **A validation strategy is provided** for a select set of novel endpoints to measure and predict OA disease progression that enables planning of regulatory implementation pathways.
- **A decision tool is developed** – based on the predictive models – that supports shared decision-making for patients, their caregivers and healthcare providers according to the predicted disease progression, the most likely associated OA disease drivers and the current disease burden.
- A robust, trustworthy, and interpretable **AI framework is established**, that enables the development of guidelines or determines any boundaries for predictive modelling at various stages of value generation e.g. biological discovery, patient subgrouping, and clinical trials enrichment
- **Data platform(s) are designed and implemented** to allow a workable and efficient collaboration across the participating organisations in their respective geographies, respecting each data contributor's access, privacy and consent approaches, which can be facilitated by federated data sharing.

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Expected contributions of the industry consortium

Industry consortium

1. Large **Pharma and Biotech** companies with ongoing OA drug development programs (Novartis, Sanofi, GSK, Novo Nordisk, Rottapharm Biotech)
2. **Medical technology companies** with focus on diagnostic devices and biomarkers (Siemens Healthineers, imorphics/stryker, Nordic Biosciences)
3. **Contributing partners**: Capgemini, Nordic Biosciences Clinical Development, Pacira Pharmaceuticals

Contributions

- **Data**: data from clinical trials (such as patient profiles, soluble or imaging biomarkers, genetics at baseline and follow up information especially from placebo arms or observational cohorts), biobank data, real world data, biomarker data
- **Expertise**: medical expertise, bioinformatics, data science, public health, patient input, clinical and regulatory expertise, data & AI experts, technology architects, data privacy experts
- **Technology**: data science and imaging platforms and tools, including pre-developed imaging algorithms.

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Expected contributions of the applicants

Expertise and resources expected to be brought into the project

- OA disease-specific expertise including all of the following domains: clinical and patho-mechanistic expertise, imaging (software) analyses of whole joints and peri-articular tissues, evaluation of performance assessments using novel technologies, evaluation of patient reported outcome and experience measures, outcome quality;
- AI-driven big data analyses, data science, bioanalytics, bio-statistics/risk modelling, drug development; epidemiology, genetic analyses (GWAS), (epi)genetics; and demonstrated experience in generating and analysing data from new digital tools that enable specific functional measurements and reflect the real-life treatment benefit for patients including expertise in movement science;
- proven experience with prior patient engagement: patient and caregiver networks including institutions such as nursing homes or assisted living facilities as well as networks with primary care physicians and physiotherapists are specifically valuable in this context to meet the needs and preferences of these primary target groups and support the development of sustainable, patient-centred and –accepted solutions;
- experience with regulatory aspects especially with respect to endpoint validation, and previous experience with interaction with regulators; and data privacy and ethics; health economics and outcome research, evidence-based medicine, quality, and efficiency in health care.

Resources:

- Timely access to data from registries, cohorts and any other relevant data collection is critical for the success of the action generated by this topic and has to be clearly documented in the proposal.
- Technology: data lake infrastructure, tools to curate, enrich and augment the data for AI models readiness

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Key Facts

- Budget
  - The maximum financial contribution from IHI is up to EUR 14 000 000.
  - The indicative in-kind contribution from industry partners is EUR 11 416 000.
  - The indicative in-kind contribution from IHI JU contributing partners is EUR 4 260 000.

- Duration
  - The indicative duration of the action is 60 months*.

*This duration is indicative only. At the second stage, the consortium selected at the first stage 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

Vaccines Europe

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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 outline 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/future-opportunities
- 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

See our **guide for applicants and project consortia on regulatory considerations for IMI and IHI projects** for useful advice on regulatory issues to consider when preparing your proposal

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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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#IHICallDays

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Call 8

20 June **14:30-15:30** Modelling regulatory sandbox mechanisms and enabling their deployment

21 June **15:30-16:30** Patient-centred clinical-study endpoints derived using digital health technologies

Online event

COCIR efpia EuropaBio MedTech Europe from diagnosis to cure Vaccines Europe Co-funded by the European Union

Register now

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/!fnJFFM

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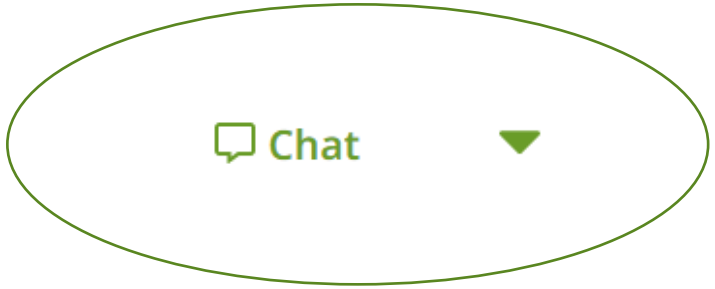
Questions time

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



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