

Webinar | IMI2 - Call 15

Integrated research platforms enabling patient-centric drug development

05.07.2018

Agenda

- How to use GoToWebinar – Catherine Brett, IMI
- Introduction – Nathalie Seigneuret, IMI
- The Call topic – Ann Van Dessel, Janssen & Francesco Patalano, Novartis
- Involvement of SMEs, patient groups, regulators – Nathalie Seigneuret, IMI
- Questions & answers

How to use GoToWebinar

Expand / minimise control panel →

Microphone status →

Full screen →

Raise / lower your hand
e.g. if you want to ask a
question orally

Send a question in writing →

The screenshot shows the GoToWebinar interface with several key elements highlighted by a red border and green arrows:

- Expand / minimise control panel:** A red circle highlights the right-pointing arrow icon at the top of the control panel.
- Microphone status:** A red circle highlights the microphone icon with a slash through it, indicating it is muted.
- Full screen:** A red circle highlights the full-screen icon (a square with a smaller square inside).
- Raise / lower your hand:** A red circle highlights the hand icon with a green dot, indicating the hand is raised.
- Send a question in writing:** A red circle highlights the text input field containing the placeholder "[Enter a question for staff]" and the "Send" button.

The interface also displays the following information:

- File View Help (menu)
- Audio (title bar)
- Sound Check (status)
- Computer audio (selected) / Phone call (unselected)
- MUTED (status)
- Transmit (Plantronics Savi 7xx-M) (dropdown)
- Receive (Plantronics Savi 7xx-M) (dropdown)
- Talking: Liz Davis (status)
- Questions (title bar)
- Webinar Housekeeping (title bar)
- Webinar ID: 608-865-371 (text)
- GoToWebinar (logo)

How to use GoToWebinar - audio

To listen via your computer, select **Computer audio**

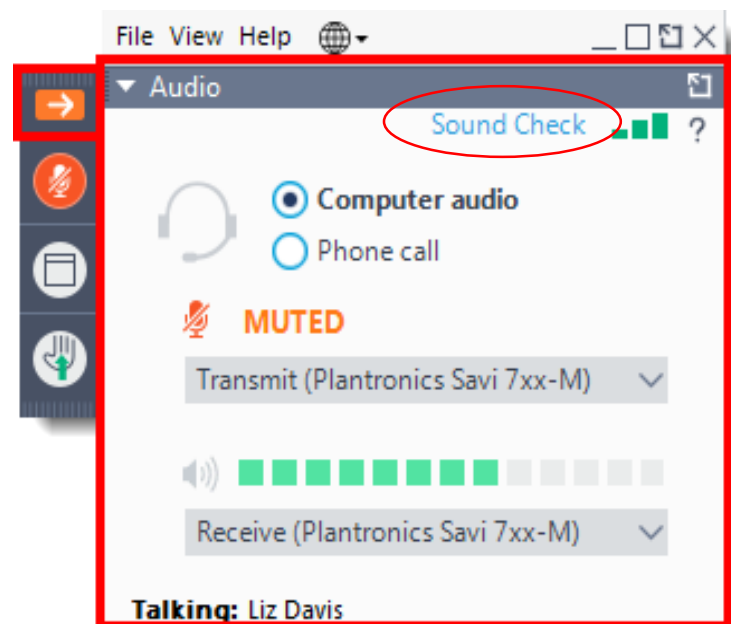
Can't hear us?

- Check your **speakers are switched on and not muted**
- Do a **Sound Check** to make sure GoToWebinar is picking up the right speakers
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Before we start...

- This webinar is being recorded and will be published on the IMI website and / or IMI YouTube channel
- Presentation slides will be published on the webinar web page
- A participant list will be circulated and published on the website
- All information regarding future IMI Call topics is indicative and subject to change. Final information about future IMI Calls will be communicated after approval by the IMI Governing Board.



Webinar | IMI2 - Call 15

Integrated Research Platforms enabling patient-centric drug development

Nathalie Seigneuret

Today's webinar

Will cover all aspects of the Call topic

- Introduction to IMI programme
- Proposed project
 - Objectives, need for public-private collaborative research
 - Key deliverables
 - Structure of the project
 - Expected contribution of the applicants
 - Contribution of industry consortium

Will not cover rules and procedures

- A webinar on rules and procedures will take place on
Tuesday 10 July at 10:30

IMI – Europe’s partnership for health

IMI mission

IMI facilitates open collaboration in research to advance the development of, and accelerate patient access to, personalised medicines for the health and wellbeing of all, especially in areas of unmet medical need.

IMI – Ecosystem for innovative collaborations

- Allow engagement in a cross-sector, multi-disciplinary consortium at the forefront of cutting-edge research
- Provide the necessary scale by combining funding, expertise, knowledge, skills and resources
- Build a collaboration based on trust, creativity and innovative and critical thinking
- Learn from each other - new knowledge, skills, ways of working
- Take part in transformative research that will make a difference in drug development and ultimately patients' lives

IMI is a **neutral platform** where **all involved** in drug development can engage in **open collaboration** on **shared challenges**.

IMI 2 budget (2014 – 2024)

EU funding goes to:

Universities

SMEs

Mid-sized companies

Patient groups

etc...



€1.638 bn



€1.425 bn

Other
€213 m

IMI 2 total budget
€3.276 billion

EFPIA companies

receive no funding

contribute to projects 'in kind'

Associated Partners
e.g.
charities,
non-EFPIA
companies

How a topic is generated

Industrial partners align themselves around a real challenge for industry and agree to work together **and commit resources**

New ideas from public sector, universities, SMEs etc. are needed to address the challenge

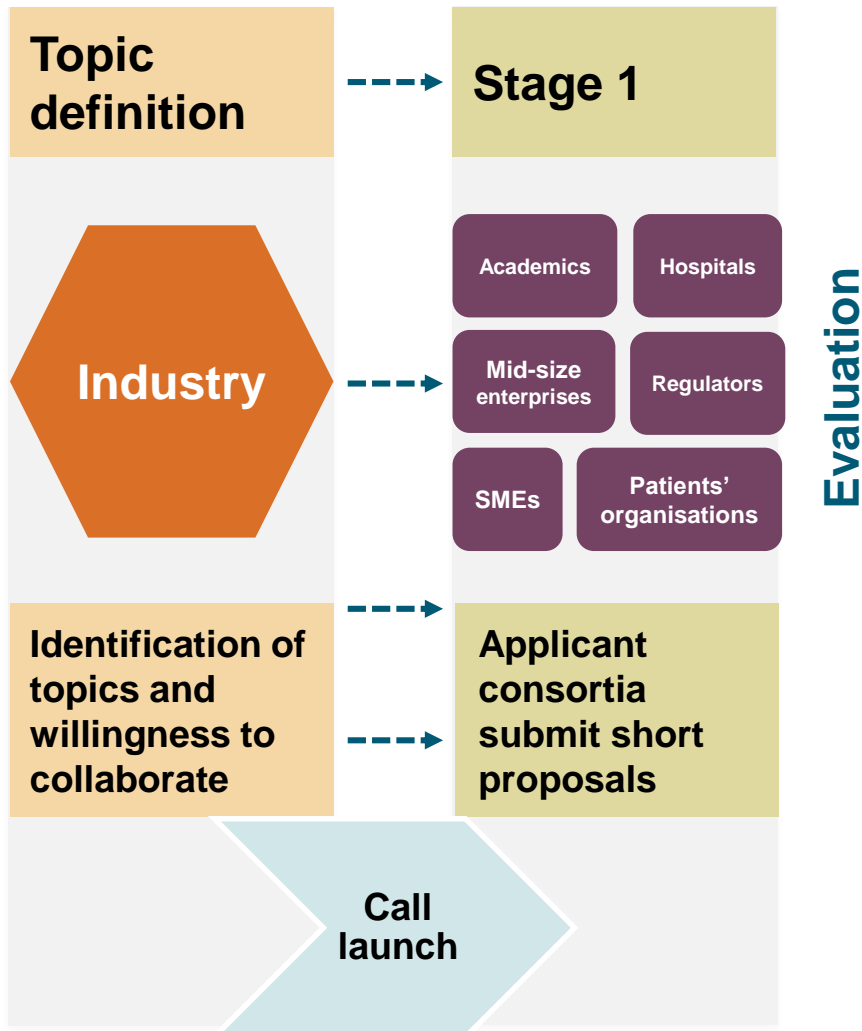
Scale is a key to success and is provided through IMI funding

Outcomes should be transformative for the industry as well as having a clear “public” value

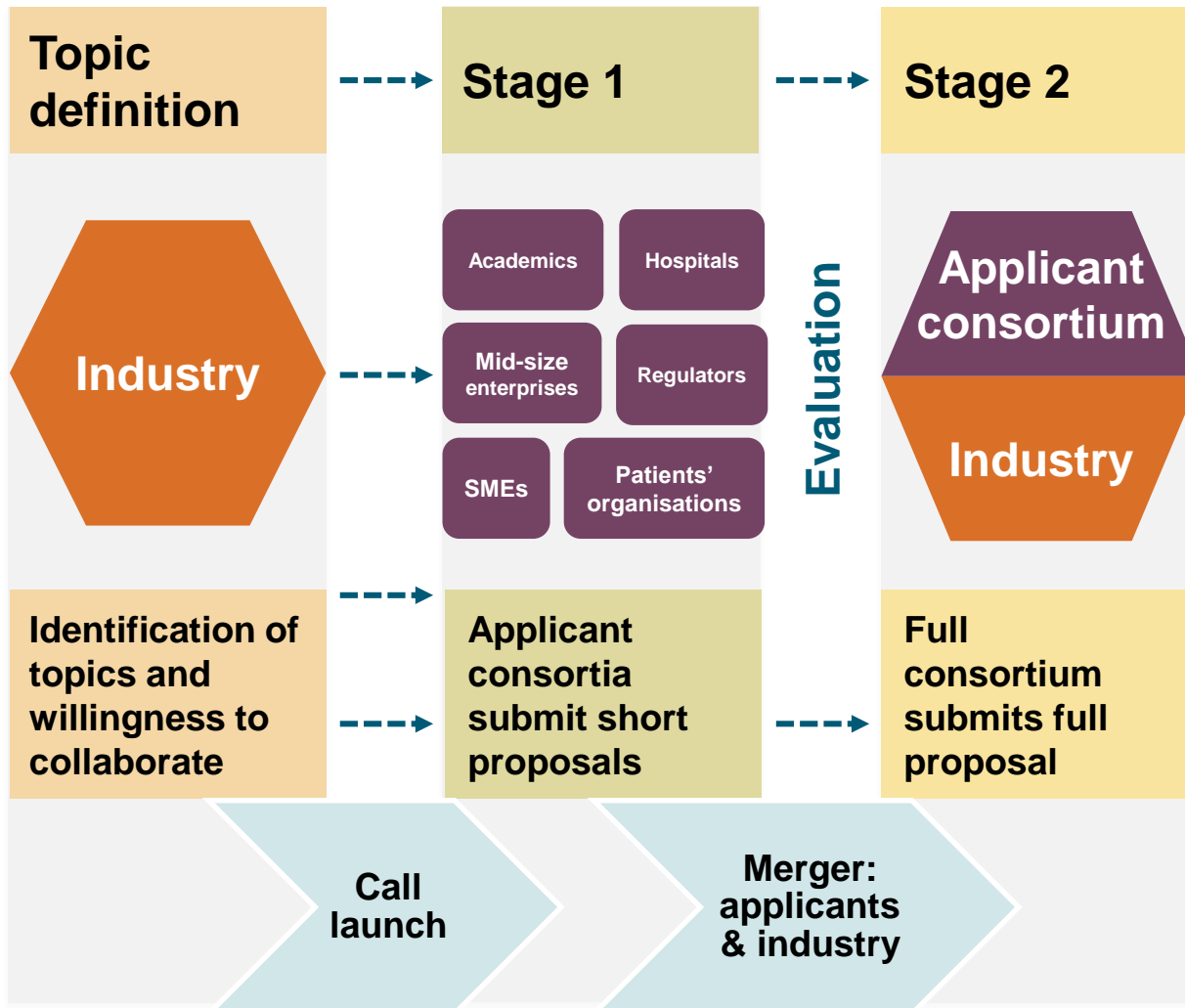
Typical IMI project life cycle



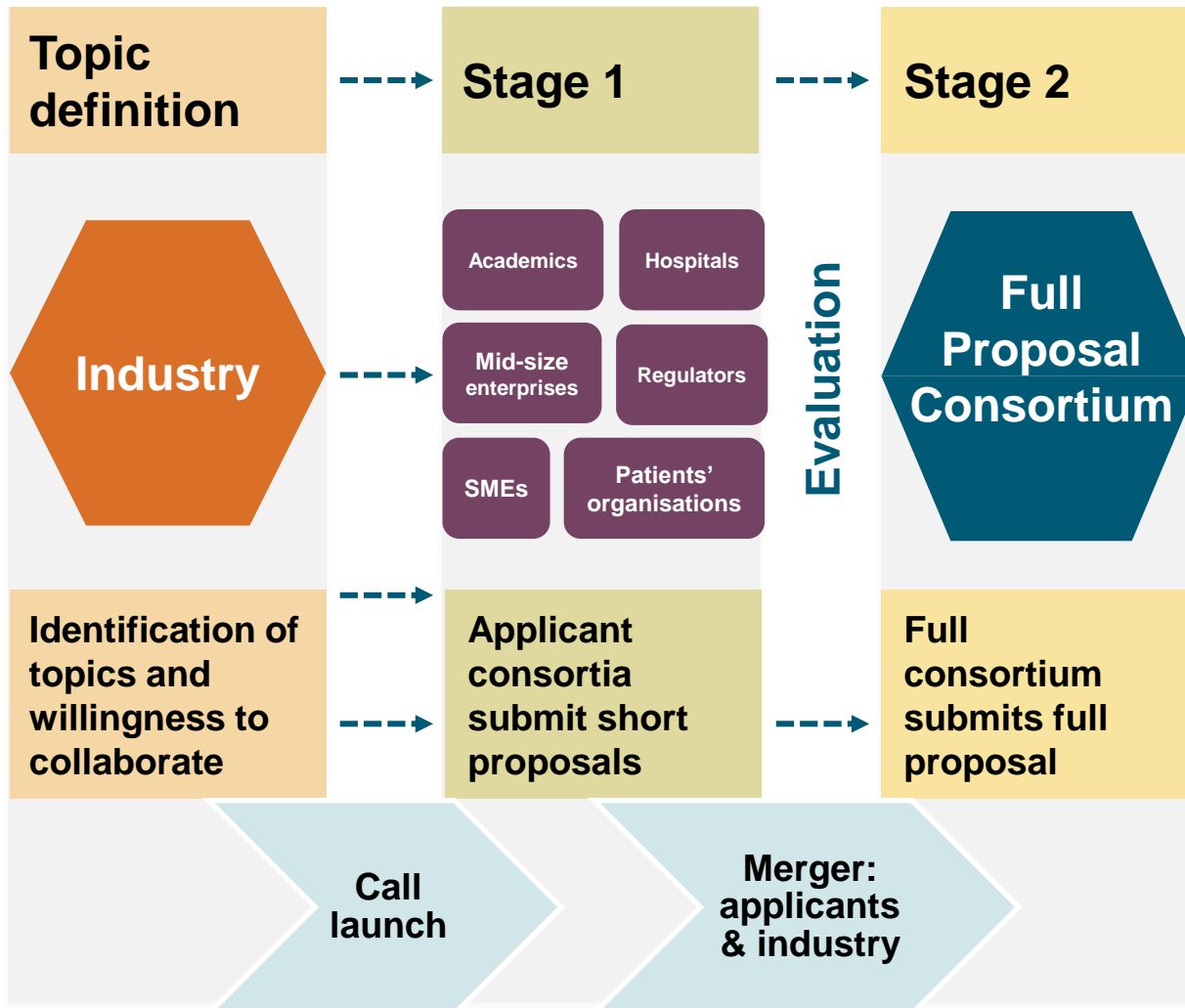
Typical IMI project life cycle



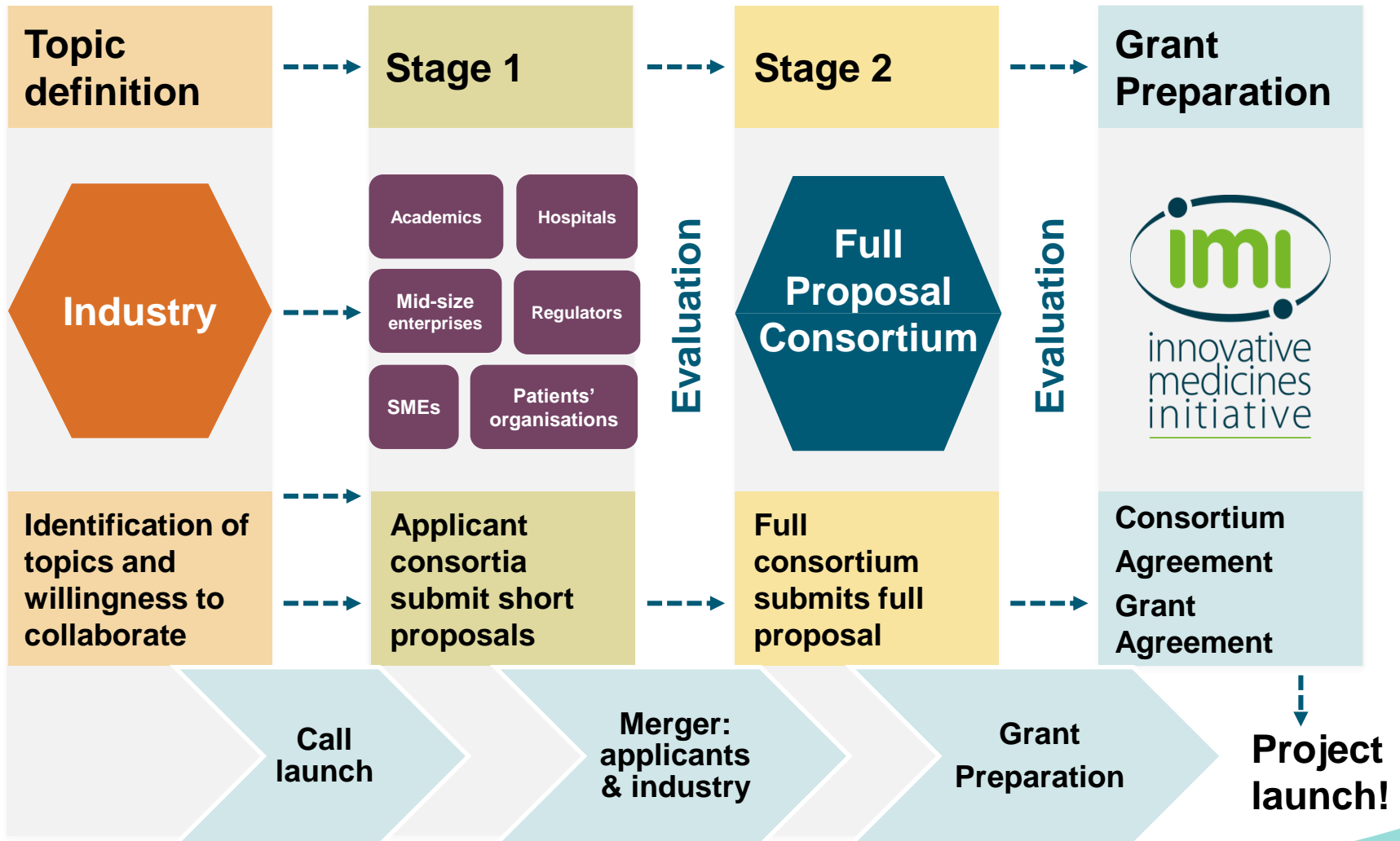
Typical IMI project life cycle



Typical IMI project life cycle



Typical IMI project life cycle



Submitting a proposal

- <https://ec.europa.eu/research/participants/portal/desktop/en/opportunities/h2020/index.html>

The screenshot displays the 'Participant Portal' for 'RESEARCH & INNOVATION' by the European Commission. The main navigation bar includes 'HOME', 'FUNDING OPPORTUNITIES', 'HOW TO PARTICIPATE', 'EXPERTS', and 'SUPPORT'. A search bar and 'LOGIN'/'REGISTER' buttons are also present. The left sidebar lists 'EU Programmes 2014-2020' with categories like 'Search Topics', 'Updates', and 'Calls', where 'H2020' is selected. The main content area is titled 'Calls for Proposals' and features a 'Horizon 2020' section with a globe icon and a link for 'Advanced search for topics Calls for tenders on TED'. Below this, there are filterable categories: 'Excellent Science' (with sub-items: European Research Council (ERC), Future and Emerging Technologies (FET), Marie-Sklodowska-Curie Actions, Research Infrastructures) and 'Industrial Leadership' (with sub-items: Leadership in enabling and industrial technologies (LEIT), Information and Communication Technologies). A 'Status' filter is set to 'Calls with forthcoming topics' and 'Calls with open topics'. The 'Sort by' dropdown is set to 'Publication date', and a search filter 'IMI2' is entered in the search box.

Proposal Template

- Available on IMI website & H2020 submission tool
- For first stage proposals, the page limit is **30 pages**.

Title of Proposal

List of participants

Table of Contents

1. EXCELLENCE	3. IMPLEMENTATION
1.1 Objectives	3.1 Outline of project plan — Work packages, and major deliverables
1.2 Relation to the call topic text.	3.2 Management structure and procedures
1.3 Concept and approach	3.3 Consortium as a whole
1.4 Ambition	3.4 Table 3.1a: List of work packages
2. IMPACT	4. PARTICIPANTS
1 Expected impacts	4.1. Participants (applicants)

Evaluation Criteria (1/2)

■ Excellence

- Clarity and pertinence of the proposal to meet all key objectives of the topic;
- Credibility of the proposed approach;
- Soundness of the concept, including trans-disciplinary considerations, where relevant;
- Extent that proposed work is ambitious, has innovation potential, and is beyond the state of the art;
- Mobilisation of the necessary expertise to achieve the objectives of the topic, ensure engagement of all relevant key stakeholders.

■ Impact

- The expected impacts of the proposed approach as mentioned in the Call for proposals;
- Added value from the public private partnership approach on R&D, regulatory, clinical and healthcare practice as relevant;
- Strengthening the competitiveness and industrial leadership and/or addressing specific societal challenges;
- Improving European citizens' health and wellbeing and contribute to the IMI2 objectives.

Evaluation Criteria (2/2)

- **Quality and efficiency of the implementation**
 - Coherence and effectiveness of the outline of the project work plan, including appropriateness of the roles and allocation of tasks, resources, timelines and approximate budget;
 - Complementarity of the participants within the consortium (where relevant) and strategy to create a successful partnership with the industry consortium as mentioned in the topic description in the Call for proposal;
 - Appropriateness of the proposed management structures and procedures, including manageability of the consortium.

Tips for writing a successful proposal

- Read **all the call-relevant material**:
www.imi.europa.eu
- Begin forming your consortium **early**
Partner search tools & networking events
- Provide **reviewers** with all the information requested to allow them to evaluate your proposal
- **Finalise and submit your proposal early**
- Contact the **IMI Office** (**NOT** industry topic writers):
infodesk@imi.europa.eu

Common mistakes

- Admissibility/Eligibility criteria not met:
 - submission **deadline** missed
 - minimum of **3 legal entities** from **3 member states & H2020 associated countries** not met
- The proposal does **not address all the objectives** of the topic
- A proposal is **scientifically excellent** but will have **limited impact**
- **Complementarity** with Industry consortium not well described.

Find project partners

- Network with **your contacts**
- **Network** with fellow webinar participants
- Use **Partner Search Tools**:
 - EU participant portal:
https://ec.europa.eu/research/participants/portal/desktop/en/organisations/partner_search.html
 - German NCP partner search tool: www.imi-partnering.eu
- Get in touch with your **local IMI contact point**:
www.imi.europa.eu/about-imi/governance/states-representatives-group
- Talk to your **Health National Contact Point** (NCP)
- Network on **social media** (e.g. IMI LinkedIn group)

Participation of SMEs, patient groups, regulators

We encourage the participation of a wide range of health research and drug development stakeholders in our projects.

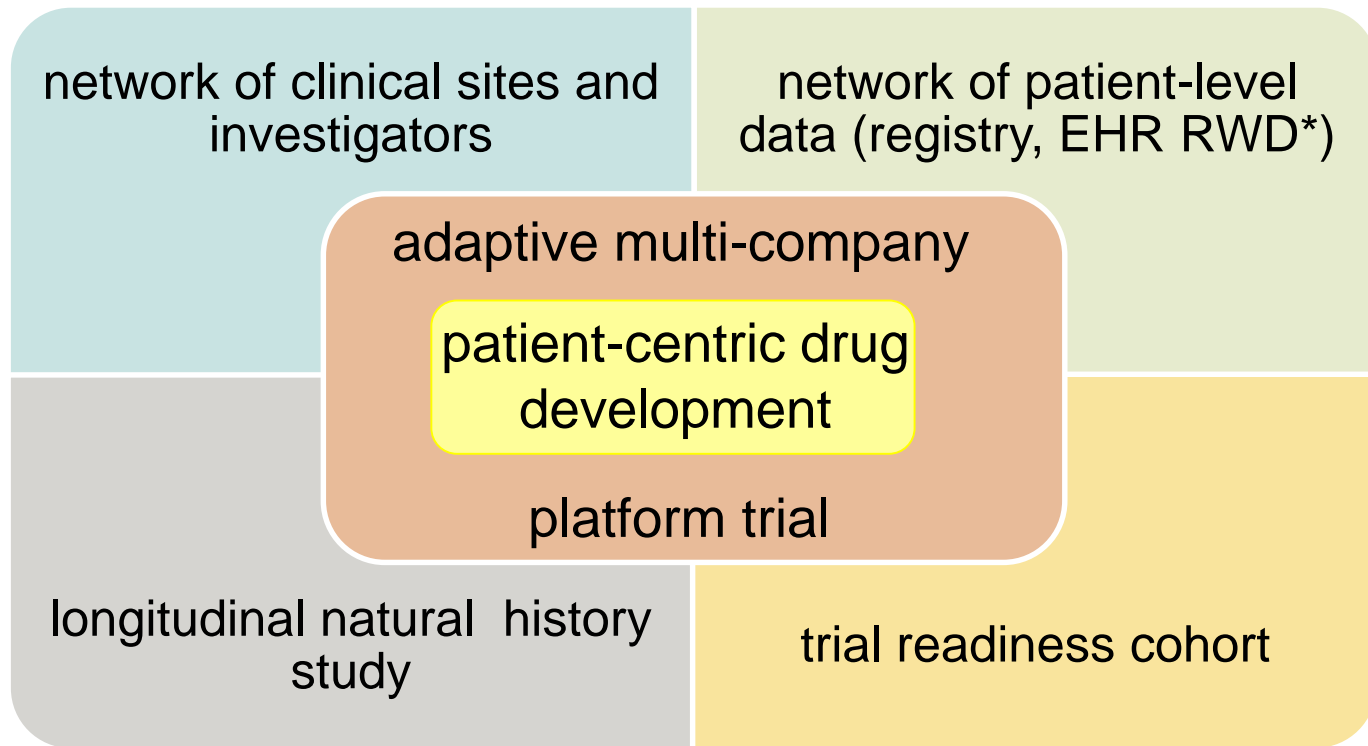
- SMEs and mid-sized companies
- Patient organisations
- Regulatory bodies
- Companies / organisations from related fields (e.g. diagnostics, animal health, IT, imaging etc...)

Integrated Research Platforms enabling patient-centric drug development

Ann Van Dessel, Francesco Patalano
05.07.2018 • IMI webinar

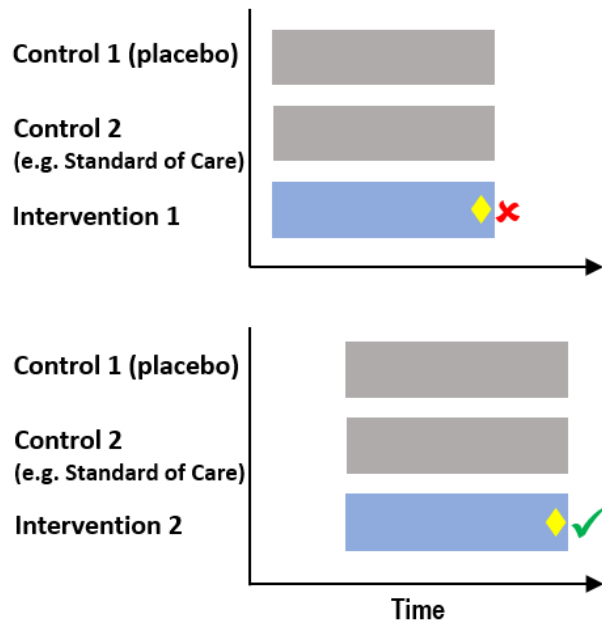
Integrated Research Platform elements

Fit-for-purpose, collaborative, patient-centric, reusable and sustainable setup for efficiently testing multiple interventions

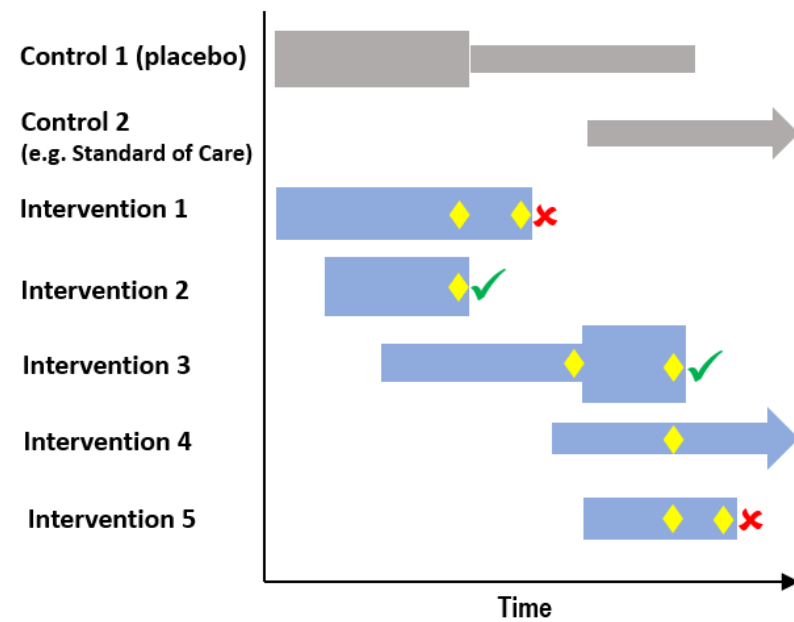



Efficiency of Platform Trial approach


Traditional Clinical Trial paradigm




Perpetual open adaptive Platform Trial



 (interim) analysis

 stop for futility

 graduate to Ph 3

Need for public-private collaboration

- A lasting **culture shift** is required to transform the current **silo approach to clinical trials**
- **All stakeholders** involved in the invention, development and use of medical innovations must **collaborate and contribute** to the design and implementation of IRPs:
 - Patient groups
 - Health care providers and investigators
 - Academic research groups
 - Pharmaceutical product developers and adjacent industries
 - Health authorities, HTA bodies, payer organisations
 - SMEs

Objectives of the full project

- **Develop and disseminate reusable best practices**, tools and guidelines for establishing multi-company platform trials with common foundational elements applicable to all disease areas including a master protocol
- **Develop platform trial designs** and protocols with clinical and patient-level data networks in 4 disease areas:
 - major depressive disorder (MDD)
 - tuberculosis (TB)
 - non-alcoholic steatohepatitis (NASH)
 - neurofibromatosis (NF) (case study for rare diseases)

INTEGRATED RESEARCH PLATFORM

COMMON ELEMENTS & BEST PRACTICES

Regulatory Aspects
Clinical Operations Framework
Quantitative Design and Statistical Methods
Legal and Intellectual Property Framework
Network of Patient Level Data

Design of a Platform Trial

- Identification of patient population and treatment regimens
- Platform Trial design including clinical and statistical methods
- Health Authority and stakeholder endorsement
- Master Protocol for Platform Trial

Clinical Network of Sites and Investigators and Network of Patient-Level Data

- Building Clinical Network of Sites and Investigators
- Identification of Patient Registries and relevant data sources
- Designing longitudinal Natural History Studies and Trial Readiness Cohorts
- Implementing Longitudinal Natural History Studies and/or Trial Readiness Cohorts

Implementation of PoC platform trial

- Develop Platform Trial infrastructure
- Trial governance and selection of interventions
- Develop intervention-specific protocol appendices
- Platform Trial site preparation and initiation

MDD

TB

NASH

NF

*Execution of
platform trials is not
in scope of this
Action.*

Pre-competitive nature

- Consortium anticipated to consist of patient groups, public partners, for-profit and not-for-profit private partners and SMEs
- Consortium will collaborate to develop guidelines, tools and protocols that will be shared and jointly used among consortium members, and made available to the public
- Regular closed (within consortium) and public meetings to obtain input, achieve and discuss progress, and disseminate results
- Call scope does not include platform trial execution, only design

Expected impact

- **Clinical proof of concept to be achieved quicker**, more successful, and at a lower burden to patients, investigators and sponsors
- **Patients to benefit quicker** from medical innovations through:
 - accelerating new medicines development
 - faster enrolment in clinical trials, with fewer patients on placebo and higher likelihood of allocation to most promising treatments
 - increased participation of patients in clinical trials by reducing placebo and comparator arms
- **Developing the reusable IRP platform approach** will deliver:
 - four feasible platform trial protocols, fully ready for execution
 - common foundation elements applicable to other diseases
 - tangible advantage for developing innovative medicines
 - advance collaborative public and private medicines research

Suggested architecture of the project

Work package 1: Governance and project management

Work package 2: Common foundational elements

- regulatory aspects
- clinical operations framework
- quantitative design & statistical methods
- legal and IP framework

Work package 3: Clinical network and network of patient-level data

- network of disease experts, clinical sites and investigators
- network of patient registries, EHRs, RWD and other research data

WP 4: IRP for MDD

WP 5: IRP for TB

WP 6: IRP for NASH

WP 7: IRP for NF (rare disease)

Expected contributions of the applicants

- Expertise and experience in:
 - leading, **programme management**, compliance and communication of PPP
 - **drug development** policy, **regulatory** decision-making and **HT assessment**
 - **legal** aspects related to consortia, clinical trials and IP
 - statistics, **statistical modelling/simulation**, adaptive (platform) trial design
 - **longitudinal natural history studies** and **trial readiness cohorts**
 - lead and access **clinical networks** & networks of **patient-level data** (EHRs, RWD)
 - **data management** and security, **patient privacy and consent**
 - **clinical trial operations**, clinical programme management
 - **patient recruitment**, design/implementation **clinical databases**, trial **regulations**
 - **ethics** and obtaining ethics approval
 - research, clinical and development expertise in **MDD, TB, NASH and NF**
 - **biomarker** identification and qualification, clinical endpoint definition, trial design
 - experience with **GCP responsibilities**; able to be clinical trial sponsor

Expected contributions of industry

- Industry Consortium: Janssen (lead), Novartis (co-lead), Allergan, AstraZeneca, Novo Nordisk, Otsuka, Pfizer, Sanofi, Servier, Teva
- Associated Partners: Children's Tumor Foundation, TB Alliance, SpringWorks Therapeutics
- Contributing expertise in:
 - leading and managing large scale **public-private partnerships**
 - research and **drug development in the disease areas** in the proposal
 - quantitative science, **adaptive trial designs**, statistical method development
 - **regulatory sciences** and strategic collaborations with health authorities
 - **operationalising execution** of platform and adaptive clinical trials
 - **longitudinal natural history studies**, patient **registries** and **readiness cohorts**
 - expertise in building **hospital networks** and technologies to **utilise EHRs**
 - experience and access to research and **disease-specific clinical networks**
 - **legal expertise** in IP and complex partnership co-development structures

What's in it for you?

- Accelerate new medicines for high medical need disease areas
- Be part of shaping the future clinical R&D paradigm
- Be part of a diverse public-private clinical R&D network
- Influence the design of clinical trials, to address patients needs
- Acquire expertise in state-of-the-art innovative clinical trial design
- Acquire reusable learnings and deep expertise of IRP paradigm

Key deliverables of the full project (1/2)

- **Toolbox of common methodology** (standards, best practices, templates and guidance documents) in service for all disease area IRPs and platform trials
 - **General framework for IRP** and platform trial sponsorship, oversight and compliance
 - **Process for governance and compound selection**
 - **Processes and technology to utilize patient-level data**
 - **Statistical clinical operations and regulatory methodologies**
 - **Templates for collaboration and clinical trial agreements**
 - **Framework and templates for data sharing** and for creation, use and protection of IP
 - **Templates for platform trial master protocol** and intervention-specific appendices

Key deliverables of the full project (2/2)

- **Communication strategy and stakeholders engagement** to enhance IRP/platform trial acceptance
- **Governance structure and coordination** across disease IRPs, including longitudinal natural history studies and readiness cohorts
- **Four (4) disease specific IRPs**
 - **master protocols** endorsed by competent authorities
 - **clinical networks of investigators** and **networks of patient-level data**
 - disease-specific **sustainability plans**
- **Sustainability plan** for governance, maintenance and expansion of common elements and framework for clinical networks and networks of patient-level data
- Key performance indicators for:
 - performance and execution of IRPs and platform trials
 - value creation and long-term impact of the action



Thank you

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Involvement of SMEs, patient groups, regulators

Nathalie Seigneuret

SME participation

IMI encourages the participation of SMEs in applicant consortia as they can offer a complementary perspective to other organisations, and help deliver the long-term impact of the project.

Contribution of SMEs would be considered especially beneficial in providing the following expertise and activities:

- statistics and modelling & simulation;
- technology for querying EHRs, registries and RWD;
- legal and IP;
- project management and communication;
- medical & scientific writing supporting regulatory interactions;
- business process design;
- clinical operations;
- patient engagement.

Patient participation

There are many ways you can improve project performance by working with your patient partners e.g:

- patient insight on the common foundational elements and governance aspects for IRPs
- patient centricity for the design of each disease-specific platform trial
- community outreach and dissemination

“The patient, doctor and researcher – each is a different kind of expert.”

Interactions with regulators

- **Have a plan for interaction** with relevant **milestones** and **resources** allocated, as needed
- Consider the **formal regulatory process** to ensure **regulatory acceptance of project results** (e.g. qualification procedure for biomarkers)
- Get familiar with **services offered for dialogue** (e.g. at EMA through qualification advice, Innovation Task Force, briefing meetings)
- Consider involving regulators as project participants or in the **advisory board**
- Have a plan for dialogue with **HTA bodies / payers**, if relevant

To maximise impact of science generated by projects



Engage in dialogue with regulatory authorities

More info:

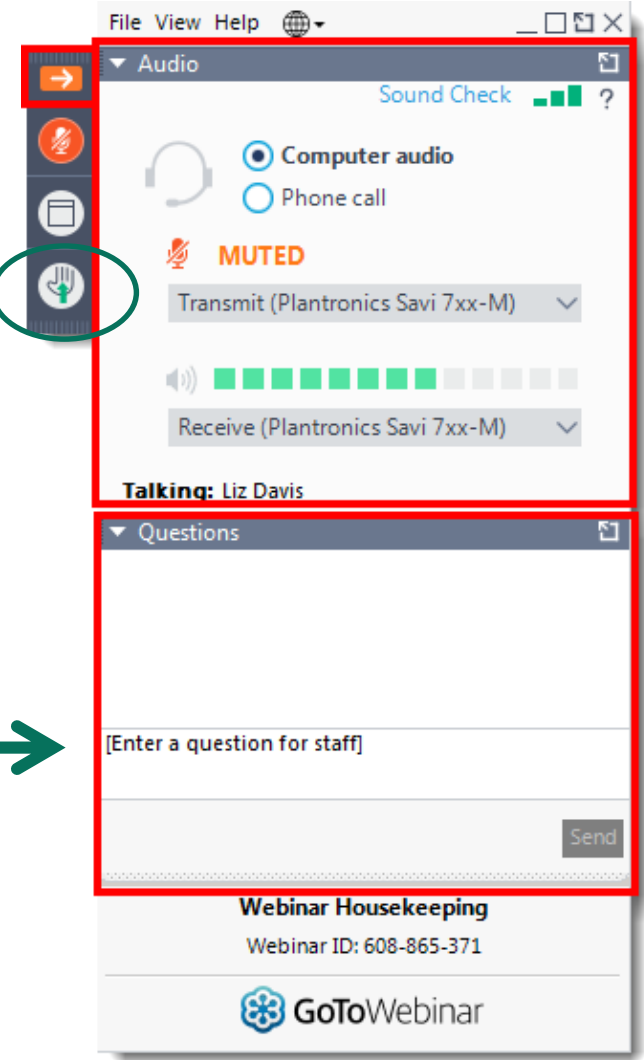
- [Webinar & presentations](#) 'How to engage with regulators EMA / FDA'
- 'Raising awareness of regulatory requirements: [A guidance tool for researchers](#)'



Questions & answers

Questions?

Raise your hand if you want to ask a question orally



Send a question in writing



After the webinar, send any questions to the **IMI Programme Office**

applicants@imi.europa.eu



Thank you!