Webinar | IMI2 – Call 13
A sustainable European induced pluripotent stem cell platform

11 December 2017 • 10:30 CET

Agenda

- How to use GoToWebinar – Catherine Brett, IMI
- Introduction – Isabella Tamagnini, IMI
- The Call topic – Andreas Ebneth, Janssen
- Involvement of SMEs, patients and regulators - Isabella Tamagnini, IMI
- Questions & answers

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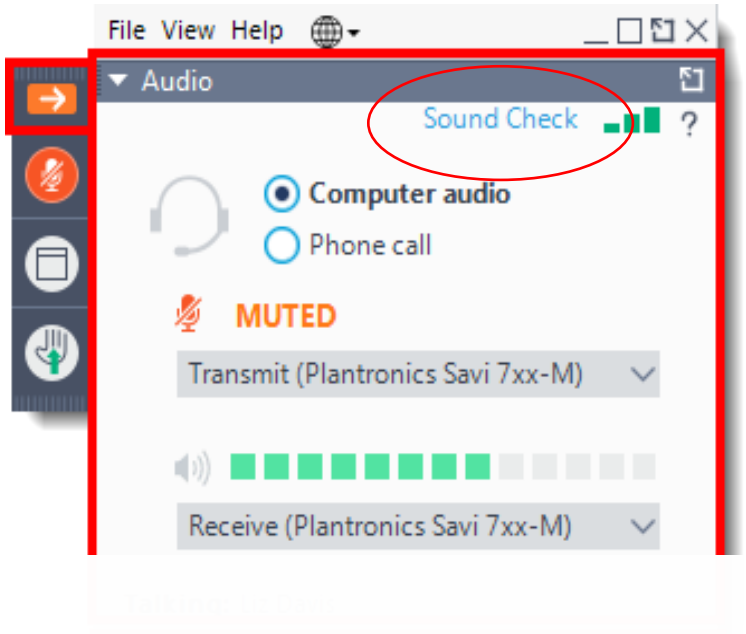

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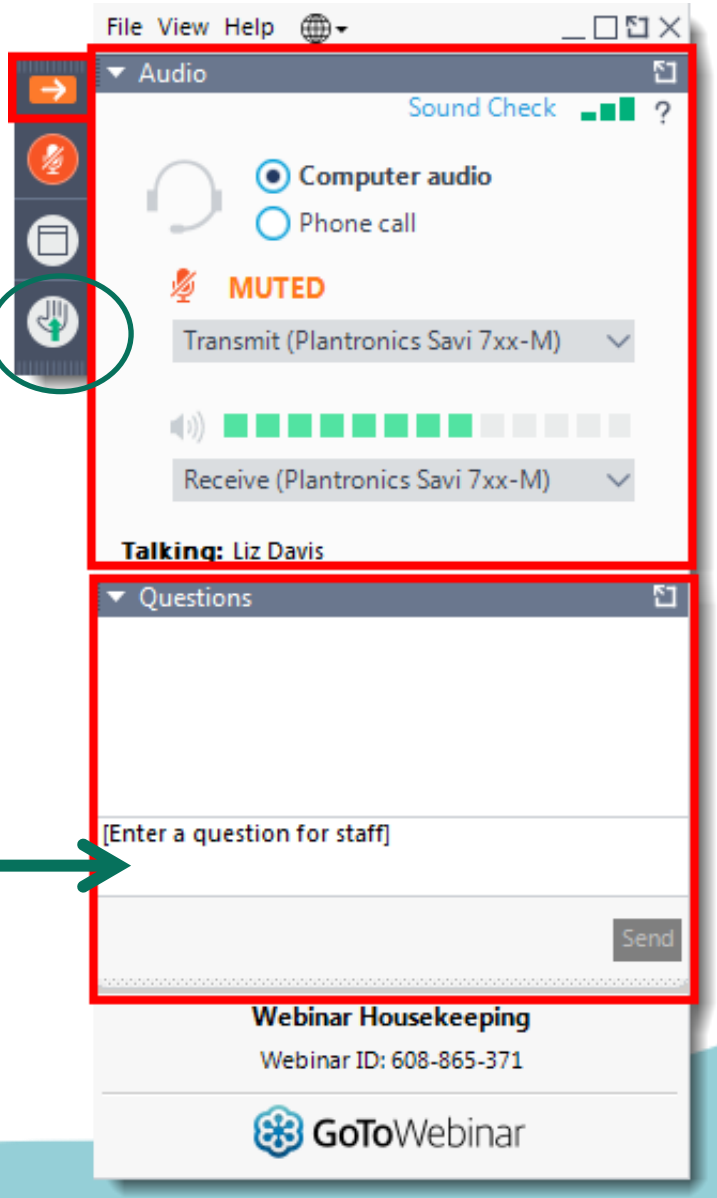

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Before we start…

- 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
- IMI2 – Call 13 has been launched and all Call documents & details of how to apply can be found on the IMI website

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Webinar | IMI2 - Call 13

A sustainable European induced pluripotent stem cell platform

Isabella Tamagnini

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 took place on Thursday 7 December – the recording is online. It will be repeated on Tuesday 16 January 2018 – sign up via the IMI website

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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.

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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**.

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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

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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

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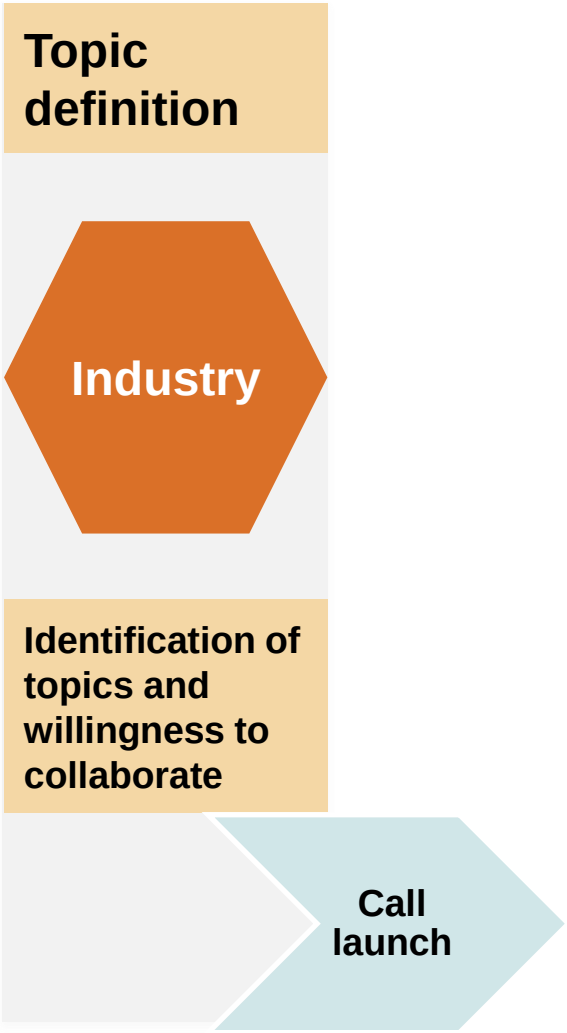
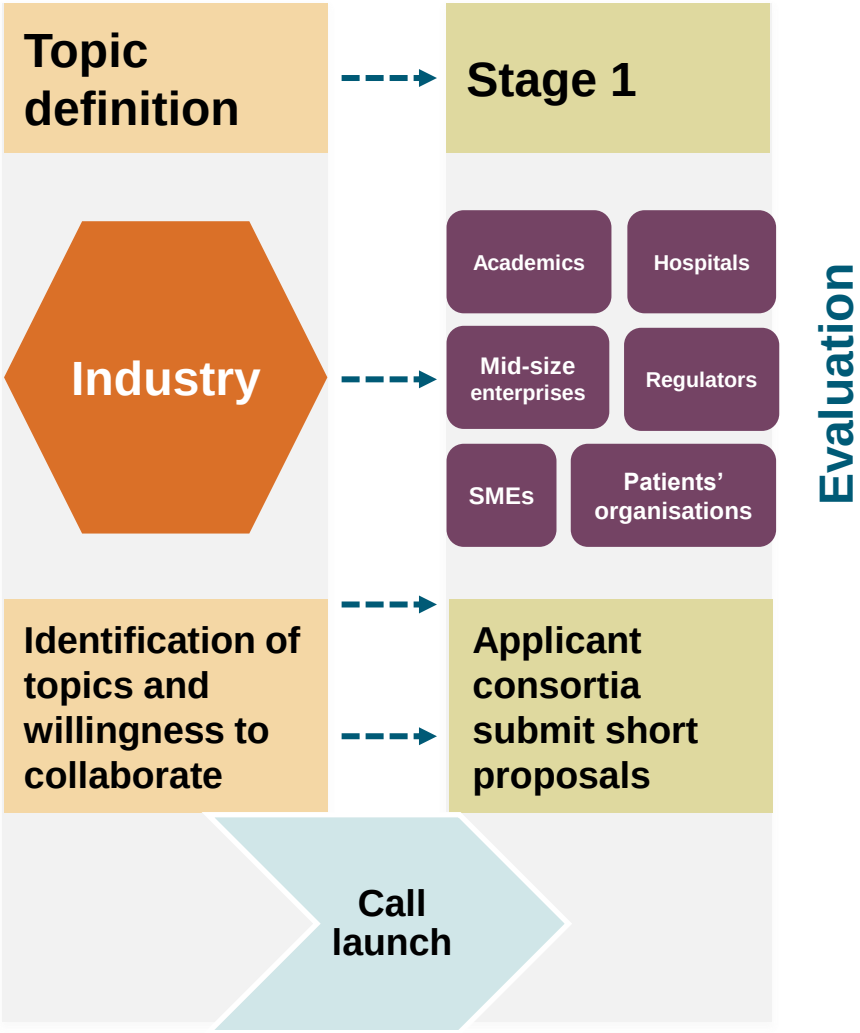
Typical IMI project life cycle



Typical IMI project life cycle



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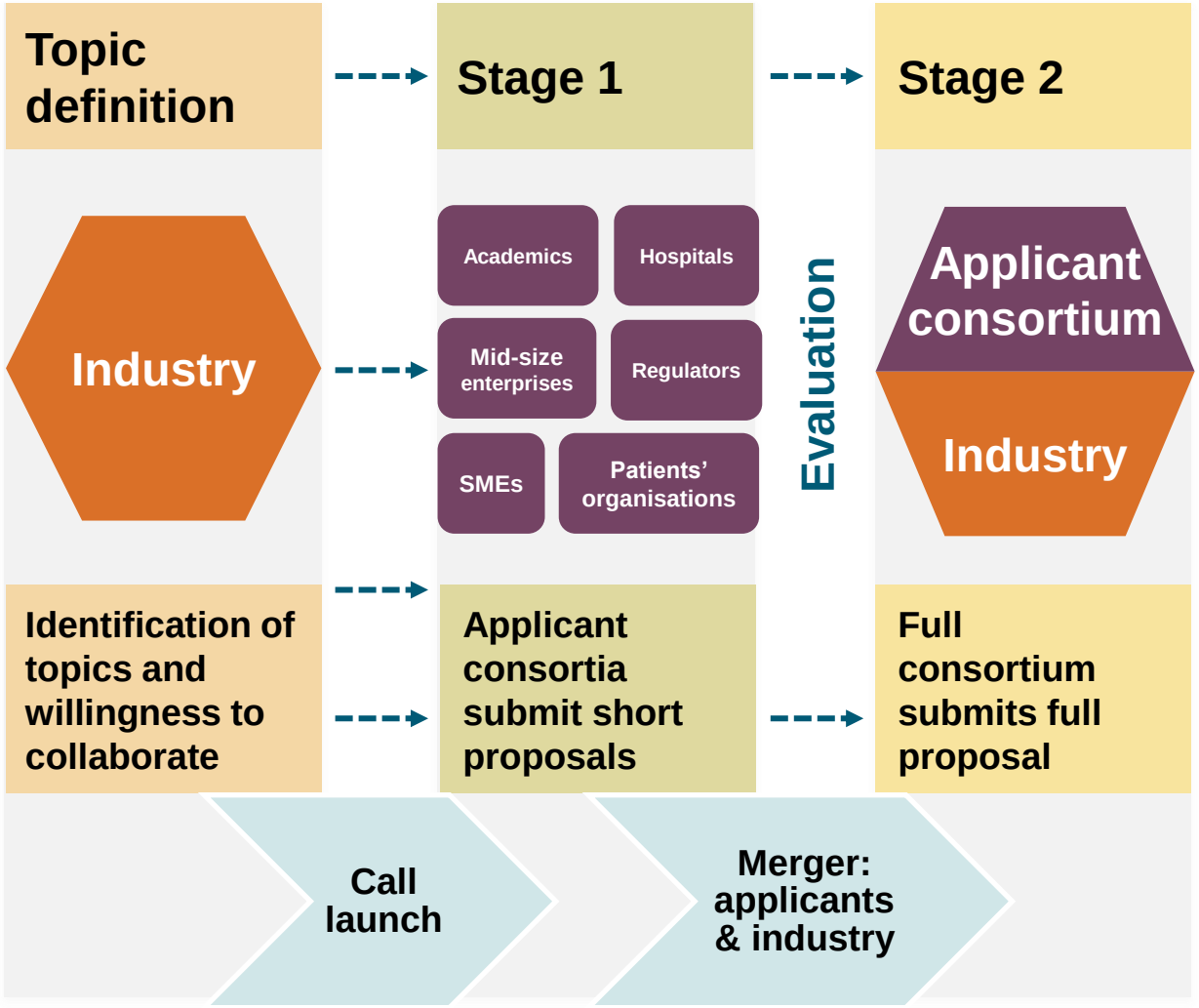
Typical IMI project life cycle



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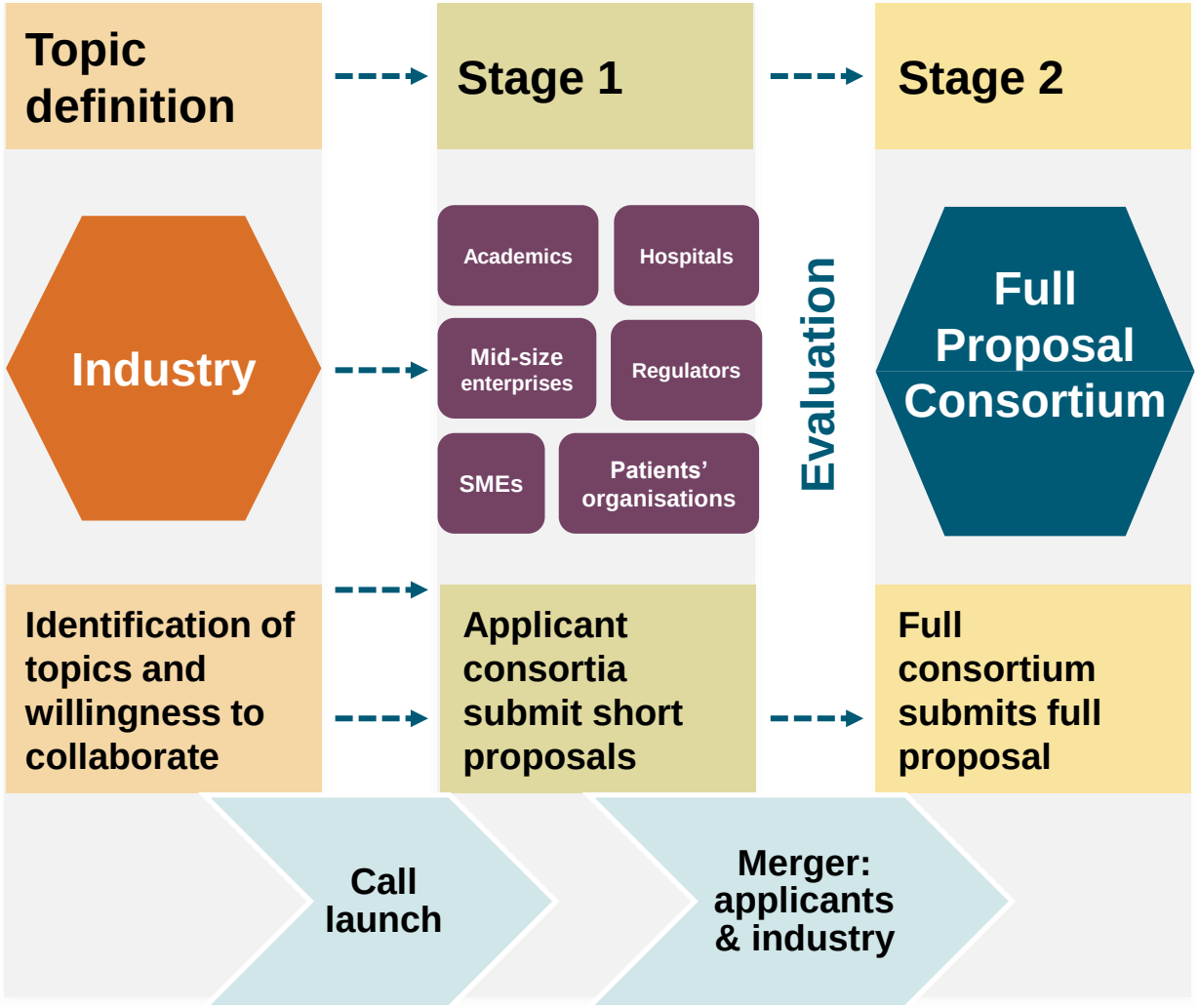
Typical IMI project life cycle



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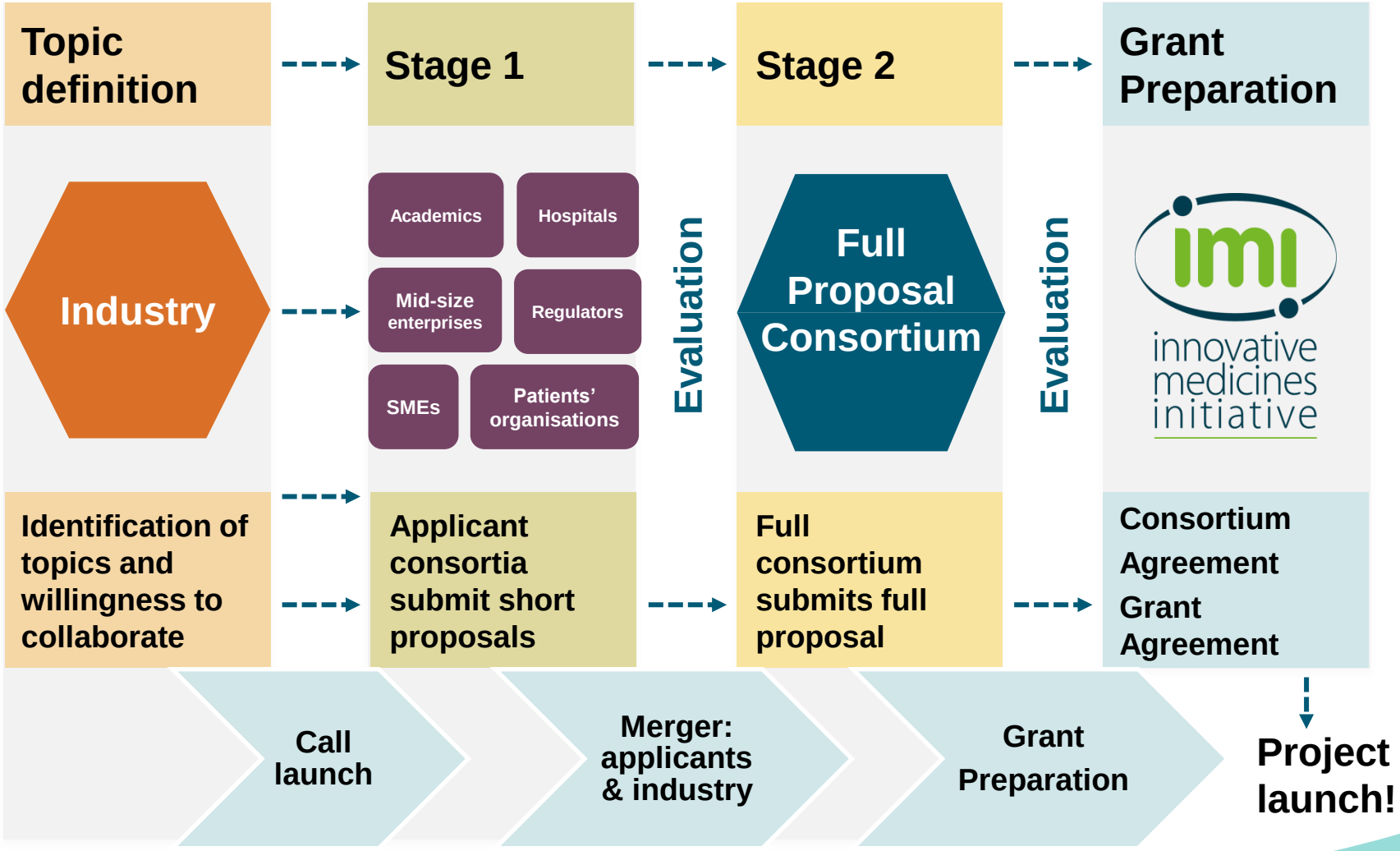
Typical IMI project life cycle



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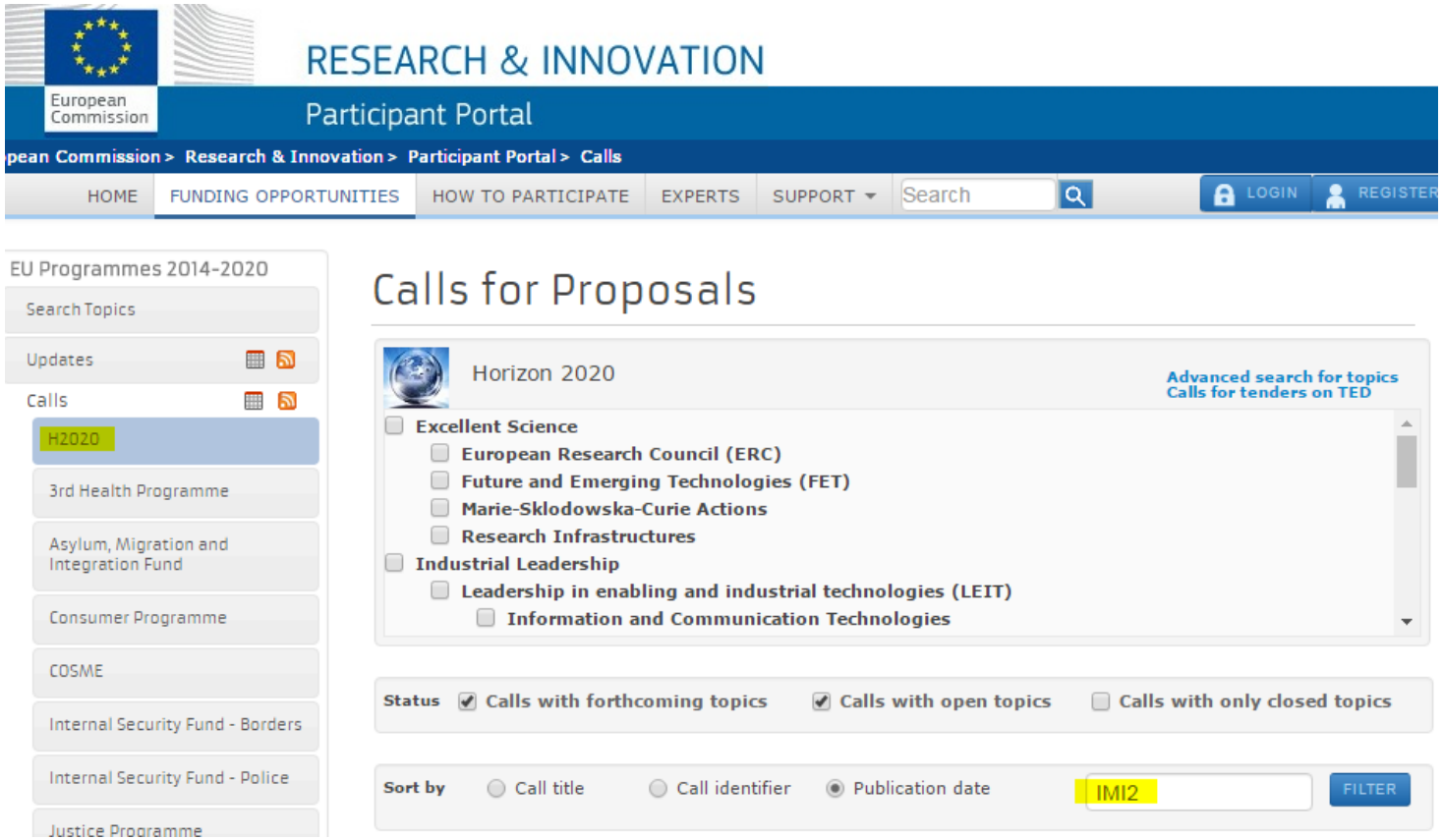
Submitting a proposal

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



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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

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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

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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.

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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)

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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
  – *check the list of interested SMEs on the Call 13 web page*
- Patient organisations
- Regulatory bodies
- Companies / organisations from related fields (e.g. diagnostics, animal health, IT, imaging etc…)

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A sustainable European induced pluripotent stem cell platform

Andreas Ebneth (Janssen)

11.12.2017 • IMI webinar

Need for public-private collaboration

- iPSC research and banking continues to be fragmented across Europe and lacks scale to support current demands of R&D. Strong public-private collaboration needed for:
  - Setting up the logistics and infrastructure of a sustainable European iPSC platform
  - Securing availability of iPSC assets established within PPPs including EBiSC
  - Expanding repository with additional iPSC lines
- Academic laboratories and SME's with access to and ownership of necessary human iPSC lines, technologies and logistics.
- EFPIA partners closely interacting with the iPSC banking entity & private partners, advising & supporting further expansion of the iPSC repository.

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Objectives of the full project

- A European iPSC repository that operates on a non-for-profit basis and allows researchers access to a continuously expanding number of well-characterised and fully quality controlled (QC) iPSC lines with clarified access information.
  - Establishing a fully self-sustainable European human iPSC banking facility
  - Operational within first three months of the action (at scale) by building on and incorporating existing lines, knowledge and infrastructure established within former European-wide initiatives (e.g. EBiSC)
  - Ability to deliver and handle at start research-grade iPSC lines with integrated data:
    - Neurodegeneration, cardiovascular, safety, diabetes, auto-immune and selected monogenic diseases.

Pre-competitive nature

- The Consortium will be working in a pre-competitive collaborative landscape where tools and results will be made available to the public
  - and jointly used / shared between consortium members
- Regular meetings and workshops will be held to openly discuss results and progress
  - to make assets, protocols & further information available to the outside
- Pre-competitive collaboration between Academia, SMEs and EFPIA will enable continuous improvement of iPSC technologies and the delivery of novel methodologies to meet the demands of public and private R&D activities
  - to facilitate & accelerate R&D activities utilizing iPSCs across Europe

Expected impact

- A uniquely positioned European iPSC repository hub accelerating and facilitating European R&D activities
  - QC'ed iPSC repository representing important disease areas such as Alzheimer's Disease, other tauopathies like frontotemporal dementia, Parkinson's disease, diabetes, neuropathic pain, cardiovascular diseases
- Novel methodologies for more precise experimental designs to discover novel pathogenic pathways, drug targets and new medicines
- A self-sustainable resource that beyond the runtime of the project will continue to support and fuel European (and worldwide) basic research as well as drug development campaigns in pharma companies.

Suggested architecture of the project

Work Package 1 – Project Management

Work Package 2 – Banking Operations

- Continuation, integration, expansion, optimisation
- Refinement SOPs
- Access to patient/donor fibroblasts

Work Package 3 – Bulk Production Capabilities / SOPs and QC for expansion and differentiation of iPSCs / NPCs

Work Package 4 – Proof of Concept experiments

- Neuroscience, diabetes, cardiovascular diseases, etc.
- Gene editing (Crispr/Cas) to establish disease-relevant iPSCs
- Reprogramming of patient derived somatic cells

Expected contributions of the applicants

- Project management (administration, communication, dissemination)
- Preparation of business plan for self-sustainable iPSC bank
- Banking operations: continuation, expansion, further optimisation
- Refinement of SOPs for QC of cell lines and differentiated iPSCs
- Integration of iPSC lines from other projects
- Connection with clinical networks, biobanks, etc allowing access to patient/donor fibroblasts
  - Full donor consent, free distribution for R&D, freedom to operate
- Bulk production capabilities/SOPs to fuel screening campaigns
- Knowledge and capability in gene editing and reprogramming somatic cells to derive iPSCs.

Expected (in kind) contributions of industry consortium

- Industry Consortium: Janssen (lead), Bayer, Eli Lilly, Lundbeck, Novo Nordisk, UCB, Pfizer, Takeda, Fujifilm, Servier
- Indicative Budget:

  EFPIA in-kind contribution: EUR 4 000 000

  Maximum financial contribution IMI2 JU: EUR 4 600 000
- Industry Contribution:
  - Co-leadership and overall coordination of project
  - Advise in iPSC line establishment and identification of cell lines to be subjected to bulk production
  - Support in:
    - Analysing iPSC-derived neurons, pancreatic cells and cardiovascular cells
    - Adaptation of iPSC technology to automated screening technology

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What's in it for you?

- Involvement in international Consortium consisting of Academia, SMEs, and Pharma for an indicative period of 3 years
- Privileged access to tools and state-of-the art technology to progress research and close collaboration with leading EFPIA companies
- Getting part of international iPSC banking activities across Europe / World
- Establishing and sharing precompetitive research and development tools for the scientific community to progress research in disease relevant therapeutic areas
- Exposure to industrial demands of iPSC technology to fuel drug development campaigns

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Key deliverables of the full project

- Self-sustainable iPSC banking facility that fully leverages significant pre-existing infrastructures and know-how and is operational within the first three months of the action
- Capacity to handle existing lines and incorporate new ones
- Mirror cell line bank at capacity
- Application and continous improvement of SOPs
- Defining QC criteria for new iPSC lines
- European and world wide distribution infrastructure
- Continued iPSC-line laboratory information management system
- Efficient and reproducible protocols for bulk production
- Expanding the repository by including additional iPSC lines
- Support the iPSC banking entity with ethical and legal aspects

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Involvement of SMEs, patient groups, regulators

Isabella Tamagnini

SME participation

IMI encourages the participation of SMEs in applicant consortia as they can offer a complementary perspective to other organisations.

Under this topic, the contribution of SMEs with relevant expertise and experience in iPSC line derivation and Quality Control would be considered especially beneficial.

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Patient participation

IMI encourages applicants to consult patient organisations or patient advocacy groups, e.g. regarding:

- patient consent forms
- relevant communication about the project and its potential value
- dissemination of the project results
- etc.

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

Interactions with regulators

- Consider having a **plan for interaction** with relevant **milestones**, **resources** allocated
- You may need to go through a **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)
- If regulators are not project participants, consider including them in an **advisory board**
- Consider also a plan for dialogue with **HTA bodies / payers** if relevant

To maximise impact of science generated by projects

Engage in dialogue with regulatory authorities

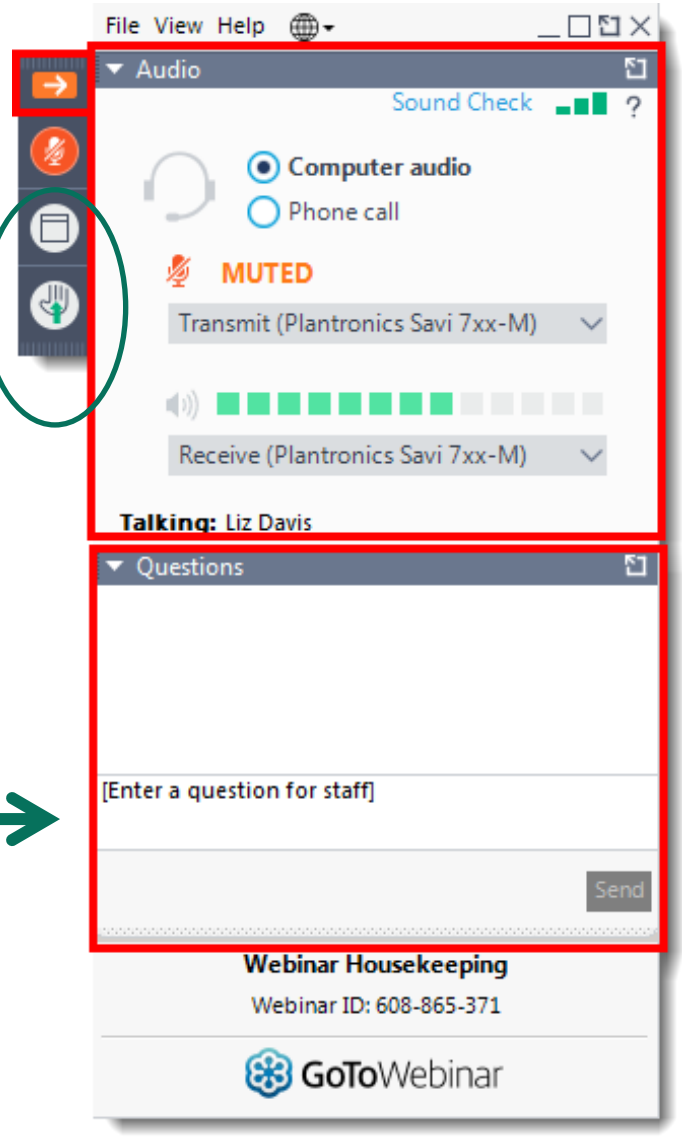
More info: 'Raising awareness of regulatory requirements: A guidance tool for researchers'

www.imi.europa.eu/sites/default/files/uploads/documents/apply-for-funding/call-documents/imi2/RegulatoryRequirementsGuide.pdf

Questions

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**

infodesk@imi.europa.eu

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