

Webinar | IMI2 – Call 13

Pilot programme on a clinical compound bank for repurposing

- Cardiovascular diseases and diabetes**
- Respiratory diseases**
- Neurodegenerative diseases**
- Rare / orphan diseases**

6 December 2017 • 10:30 CET

Agenda

- How to use GoToWebinar – Catherine Brett, IMI
- Introduction – Salome Koussoroplis, IMI
- The Call topic – Lorraine Webber AstraZeneca
- Questions & answers

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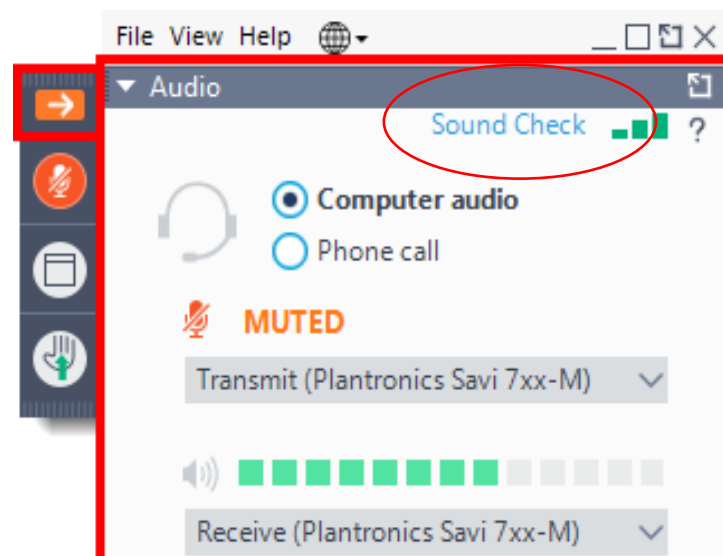
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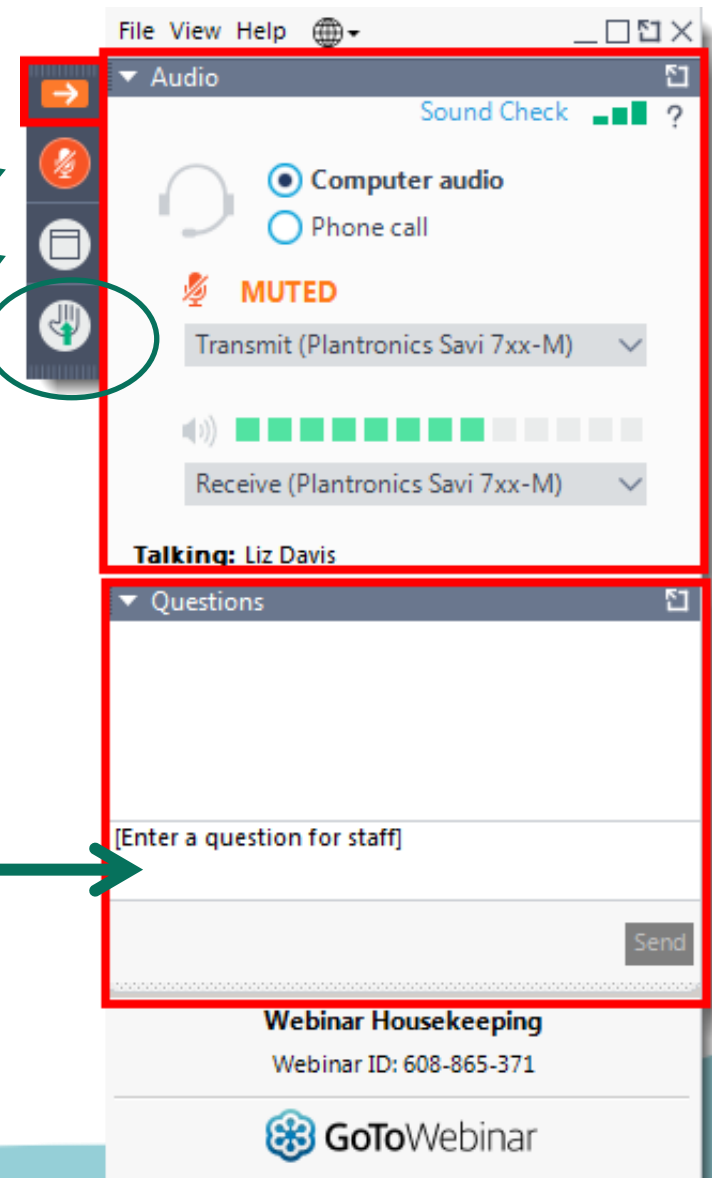
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question orally

Send a question in writing →



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

Webinar | IMI2 - Call 13 - Topics 12-15

Pilot programme on a clinical compound bank for repurposing:

- cardiovascular diseases and diabetes
- respiratory diseases
- neurodegenerative diseases
- rare/orphan diseases

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 Thursday 7 December, 15:00-16:30

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

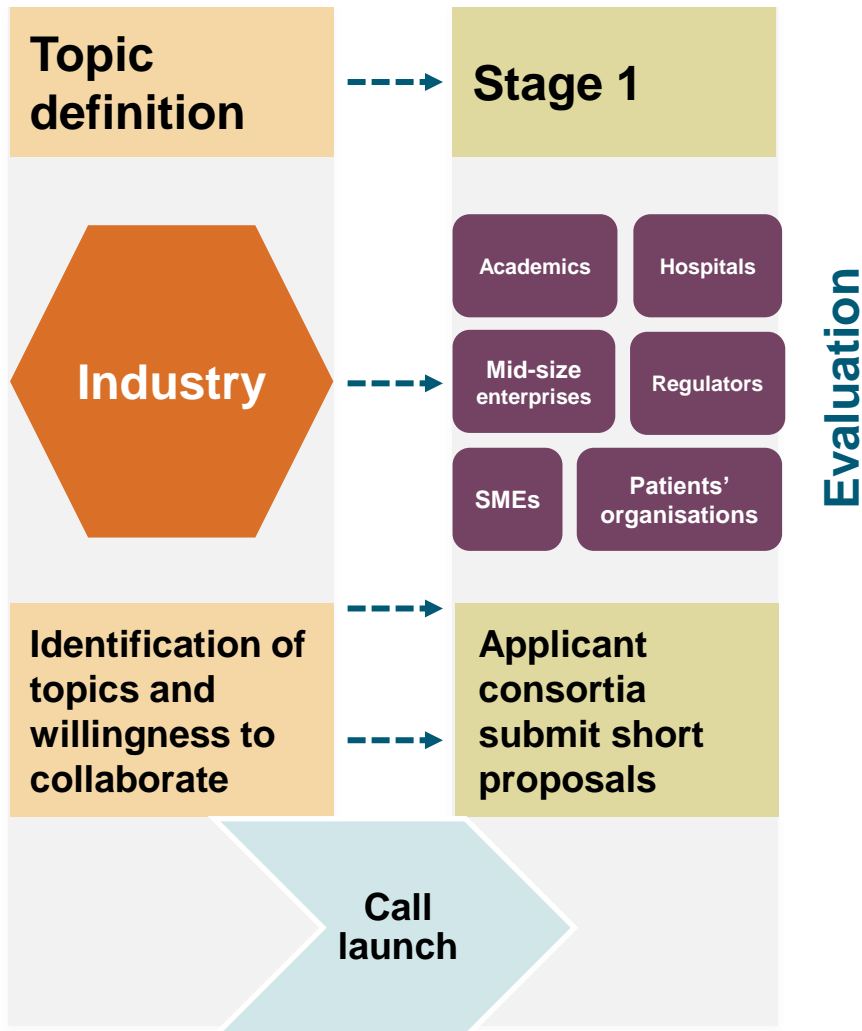
Scale is a key to success and is provided through IMI funding

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

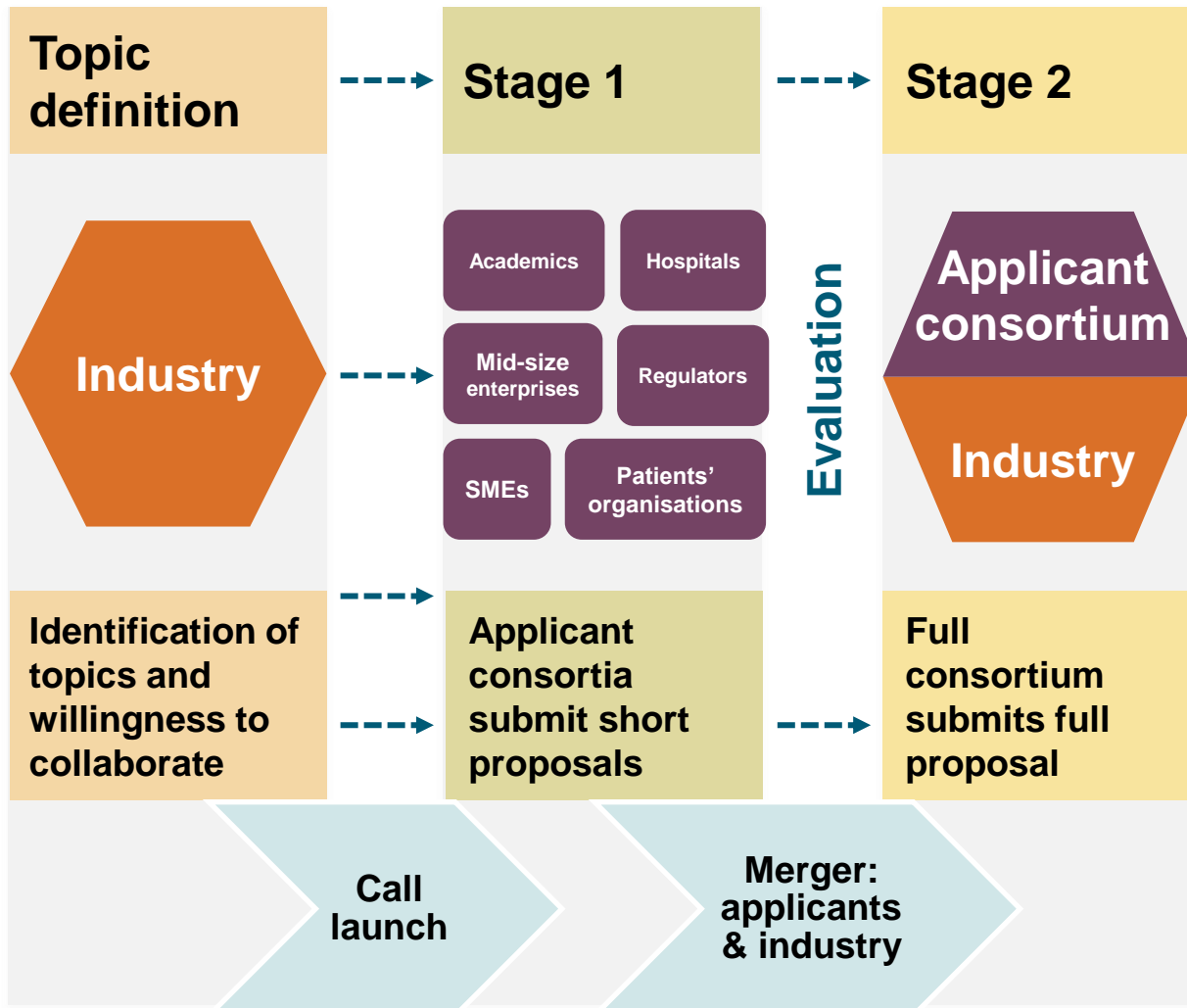
Typical IMI project life cycle



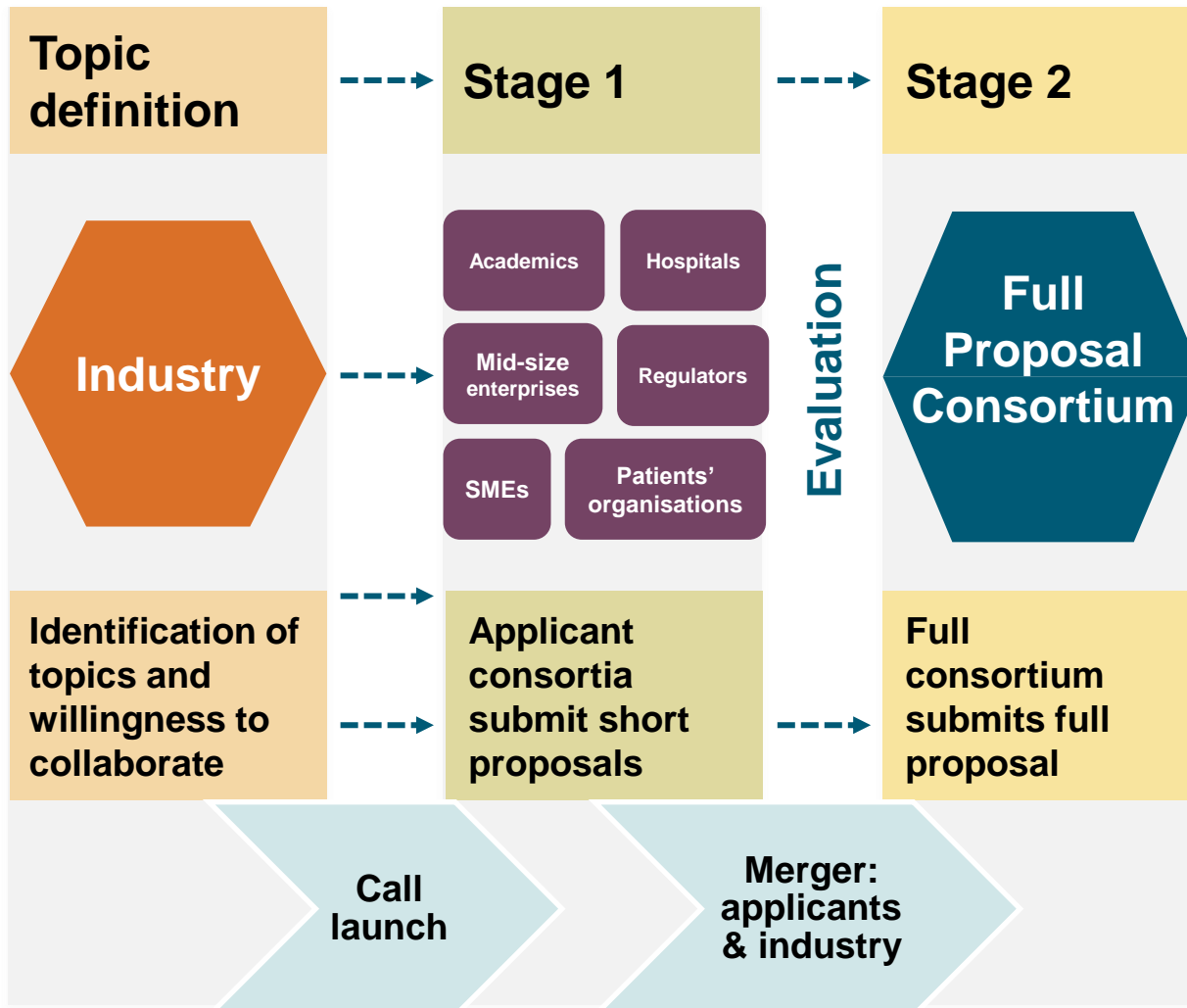
Typical IMI project life cycle



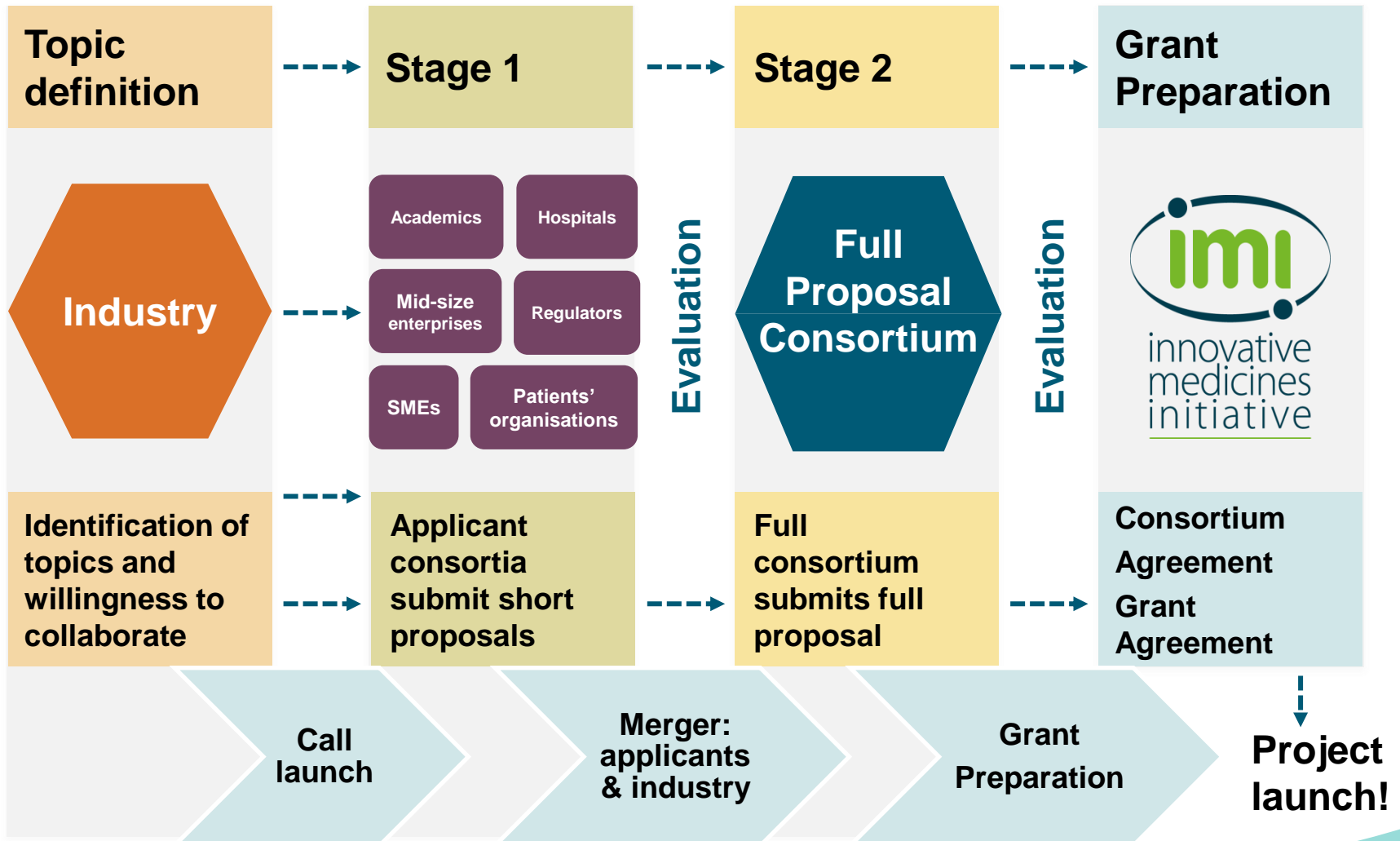
Typical IMI project life cycle



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 'H2020', '3rd Health Programme', and 'Asylum, Migration and Integration Fund'. The main content area is titled 'Calls for Proposals' and features a 'Horizon 2020' section with a list of topics: 'Excellent Science' (including ERC, FET, Marie-Sklodowska-Curie Actions, and Research Infrastructures) and 'Industrial Leadership' (including LEIT and ICT). 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
- Regulatory bodies
- Patient organisations
- Companies / organisations from related fields

SME participation

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

For example, being closer to the market, SMEs can drive the tangible outputs of the project, and help ensure these outputs are sustained beyond the project lifetime and therefore help lead to faster impact on healthcare.

Therefore, where possible, include SMEs in your Short Proposal

A webinar on opportunities for SMEs for Call 13

7 Dec 2017, 15:00 - 16:30

Check the list of interested SMEs on the Call 13 web page

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

Pilot Programme on a Clinical Compound Bank for Repurposing

Lorraine Webber
06.12.2017 • IMI webinar

Introduction

- On average, it takes about 14 years and over €2 billion for a new drug to travel from the research lab to market approval;
- Only 10% of compounds entering pre-clinical testing ever make it to clinical trials, and only 20% of those achieve market approval;
- This call aims to provide researchers with access to high-quality pharma industry compounds that have stalled during development;
- These compounds are valuable tools that researchers can use to test novel hypotheses for alternative therapeutic indications, with the aim of identifying a new use for the compound (“repurposing” or “repositioning”);
- Similar *Open Innovation* schemes already exist, such as the NIH-funded ‘New Therapeutic Uses’ program in the US.

Need for public-private collaboration

- The European research organisations have access to cutting edge science and innovative spirit;
- Pharma companies have expertise in developing their discoveries towards the clinic and obtaining regulatory approval, as well as access to high-quality compounds;
- Together, it is possible to accelerate research in drug repurposing and potentially speed up development of new treatments.

Objectives of the full project

- The overall objective is to take one of the nine previously deprioritised clinical compounds listed, and investigate their therapeutic potential in new clinical indications in areas of high unmet need;
- Proposals should cover Ph2 Proof of Concept studies;
- Pre-clinical work (up to 1 year) may be included if deemed necessary to validate the target before progression to the clinic. Clear go / no go criteria should be given.

Objectives of the full project

- Proposals are invited in the disease areas listed below:
 - Cardiovascular diseases and diabetes
 - Respiratory diseases
 - Neurodegenerative diseases
 - Rare/orphan diseases
- Compound profiles are included in the Appendix
- Only innovative research will be funded

Expected impact

- Achieving early proof-of-concept for new mechanisms with the potential to lead to further development of novel drugs in areas of high unmet need and/or those with greatest disease burden
- Repurposing pharmaceutical assets which have already passed through several stages of the R&D process offers significant time, cost and risk savings
- Supporting EU academic institutions to conduct well-designed research with quality compounds, resulting in high impact publications and patents when possible
- Advancing science and knowledge of disease (patho)physiology through testing of new hypothesis;
- Boosting European competitiveness by contributing to closer links between industry and academia across the EU.

Suggested architecture of the project

- The applicant consortium should include their suggestions for the project architecture in the short proposal

Expected contributions of the applicants

- Presentation of a strong scientific hypothesis for the compound in the new indication, based on existing pre-clinical or clinical data;
- Experience and capability to:
 - submit an application for regulatory and ethics approval for an investigator-sponsored clinical trial (in all member countries where the trial will recruit patients);
 - design and conduct all aspects of an investigator-sponsored clinical trial using an investigational medicinal product (including data analysis and reporting) under GCP in the proposed indication;
 - recruit sufficient number of patients within a few clinical study centres;
- Clinical and pre-clinical expertise as necessary for the project;
- Strong project management and communication skills.

Expected (in kind) contributions of industry consortium

- AstraZeneca and Servier will supply compounds for these pilot topics.
- The EFPIA companies will cover the costs associated with provision of drug product for the clinical study (as well as non-GMP compound for pre-clinical work).
- The EFPIA companies will provide the associated documentation on the compound required for investigator-sponsored applications for Clinical Trial Authorisation to regulatory and ethics committees.
- The EFPIA companies will provide expert support for e.g. study design, protocol writing, study oversight etc. throughout the duration of the funded studies.

What's in it for you?

- Access to high quality, clinic-ready compounds and clinical research funding
- Generation of novel IP and data, which may be licensed from the research institution to support further development of the indication
- Opportunity for high impact publications

Key deliverables of the full project

- Initiation and completion of new Phase 2A clinical proof-of-concept study in the chosen indication which was not previously investigated with the specific compound;
- Preclinical data to support a go/no go decision for initiation of the clinical study in the new indication, if this is deemed necessary for the selected project;
- Dissemination of the results in high impact publications.

Compounds Included (1 of 3)

AZD0328 (Nicotinic acetylcholine receptor alpha 7 agonist)
Original indication: schizophrenia

AZD0530 (Src tyrosine kinase family inhibitor)
Original indication: Oncology

AZD1981 (Chemoattractant receptor-homologous molecule expressed on Th2 cells (CRTh2) antagonist)
Original indication: asthma

AZD4017 (11-beta-hydroxysteroid dehydrogenase type 1 (11 β -HSD1) inhibitor)
Original indication: metabolic disorders

Compounds Included (2 of 3)

AZD7325 (Gamma-aminobutyric acid receptor A alpha 2 & 3 (GABA_{Aα2,3}) positive modulator)

Original indication: generalised anxiety disorder

AZD1656 (Glucokinase (GK; hexokinase 4) activator)

Original indication: diabetes / metabolic disease

AZD5904 (Myeloperoxidase (MPO) inhibitor)

Original indication: inflammation

Compounds Included (3 of 3)

S 38093 (Moderate antagonist/inverse agonist at histaminergic H₃ receptors, moderate antagonist at adrenergic α_{1A} and Sigma 1 receptors)

Original indication: Alzheimer's disease

S 47445 (positive allosteric modulator of alpha-amino-3-hydroxy-5-methyl-4-isoxazole-propionic acid (AMPA) receptors (AMPA-PAM))

Original indication: depression



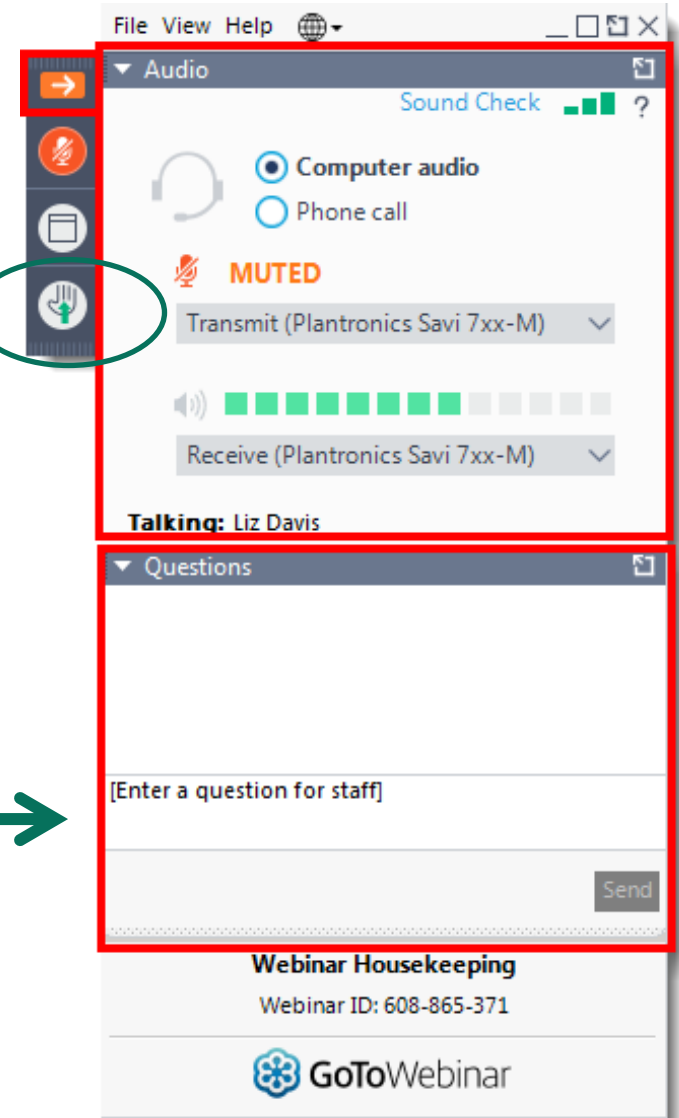
Questions

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