Webinar | IMI2 – Call 12
Development of sensitive and validated clinical endpoints in primary Sjögren's Syndrome (pSS)

18.07.2017 • 14:30 CEST

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

  Monday 17 July, 14:30-16:00
  (presentation & recording available online)

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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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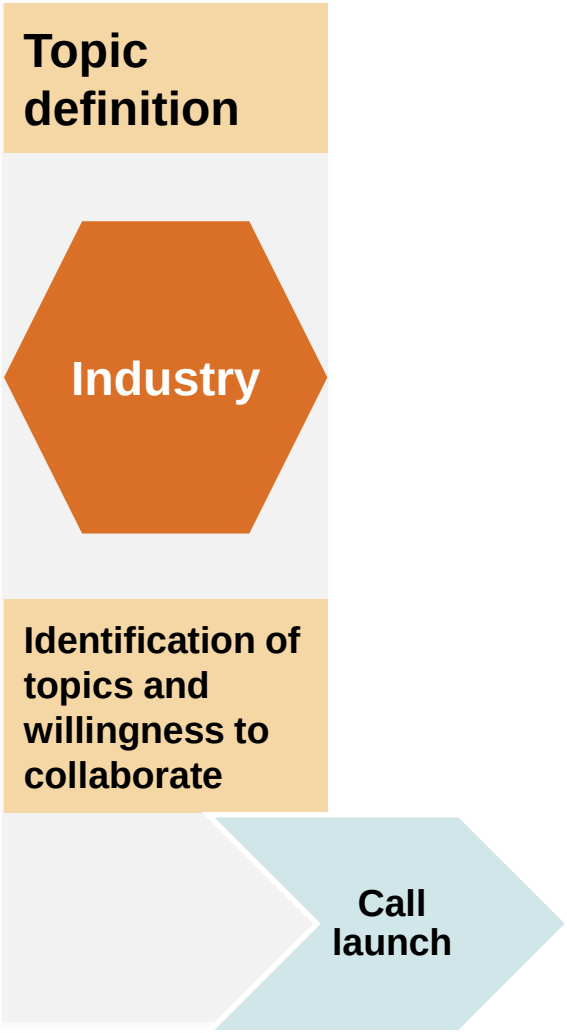
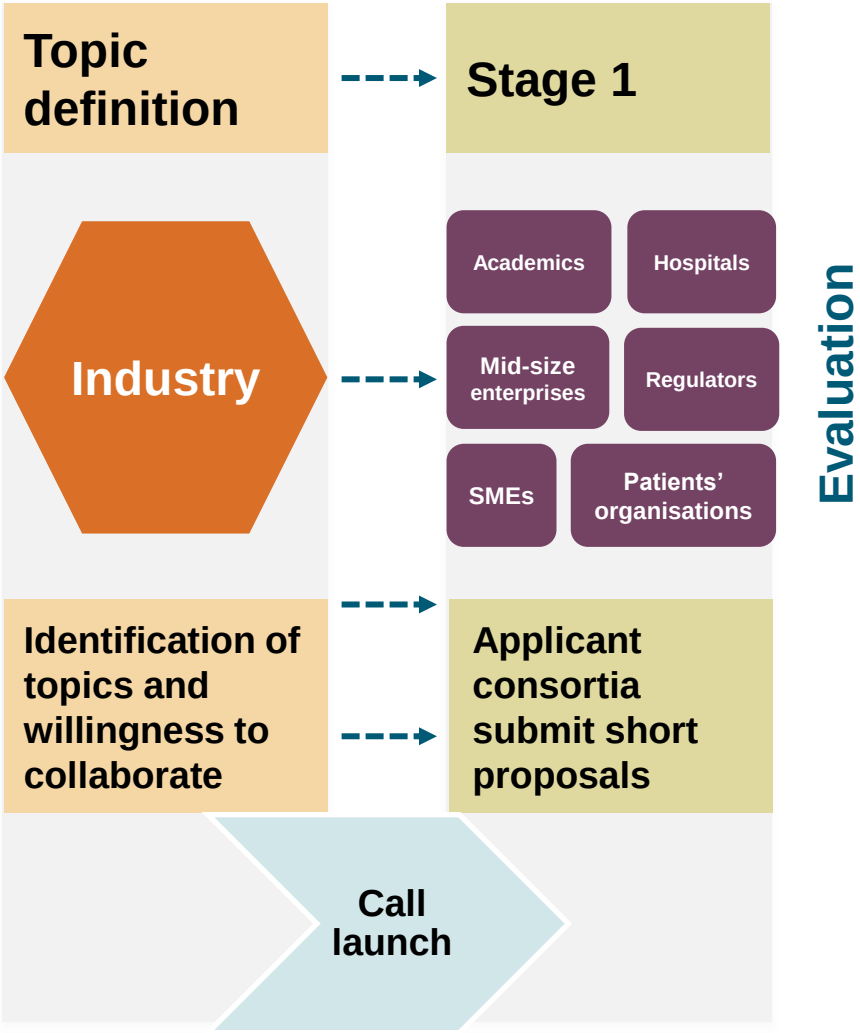
Typical IMI project life cycle



Typical IMI project life cycle



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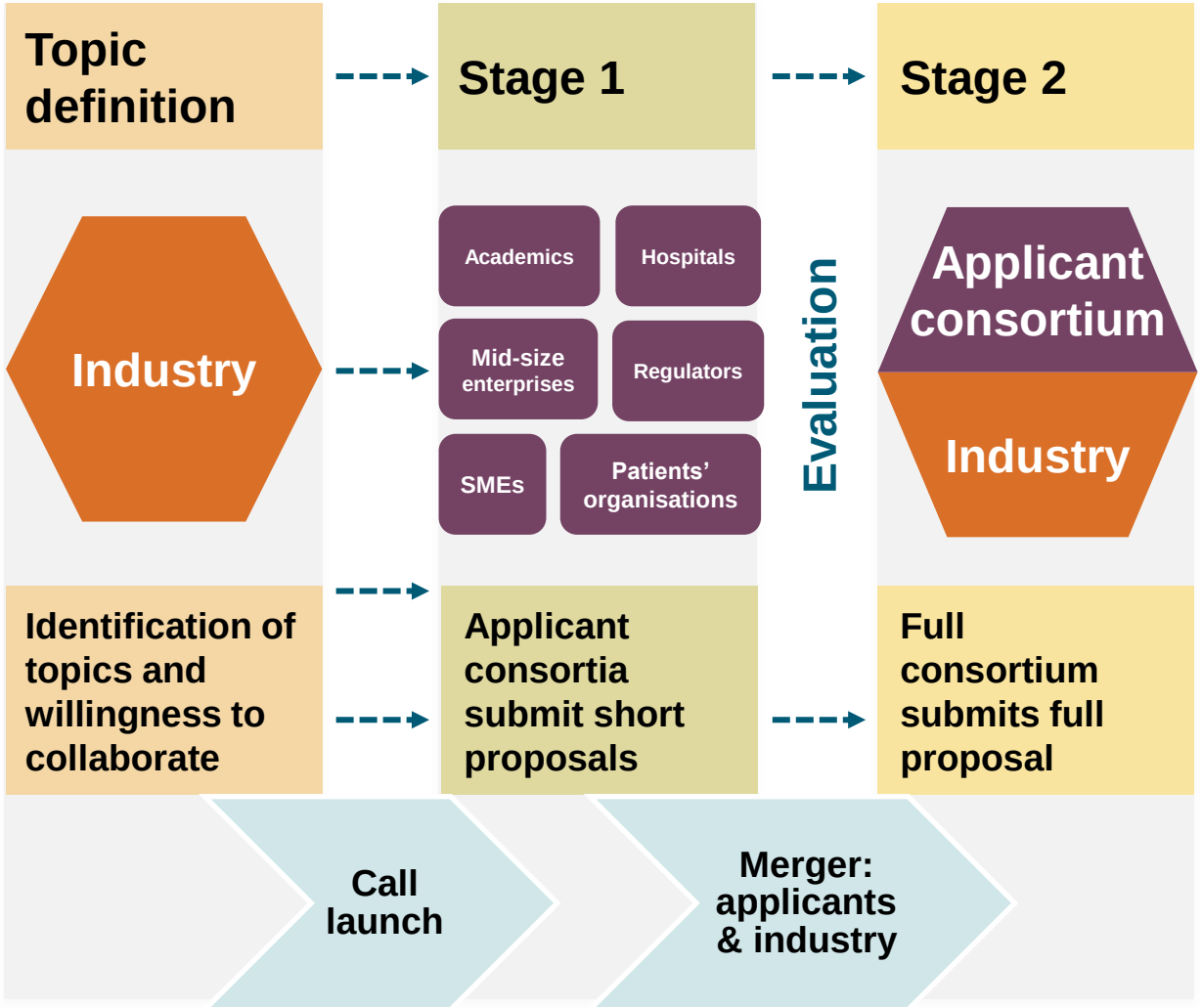
Typical IMI project life cycle



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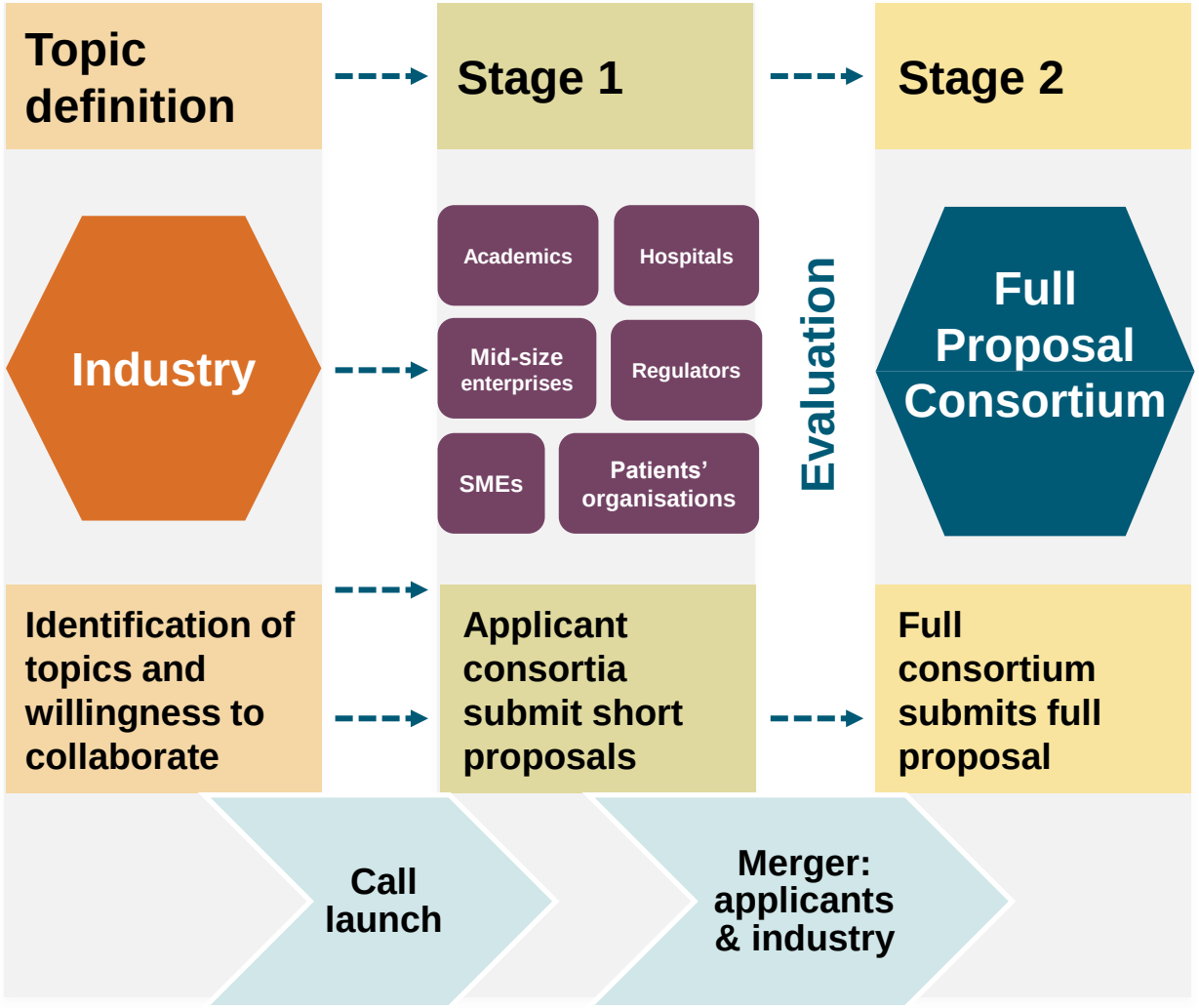
Typical IMI project life cycle



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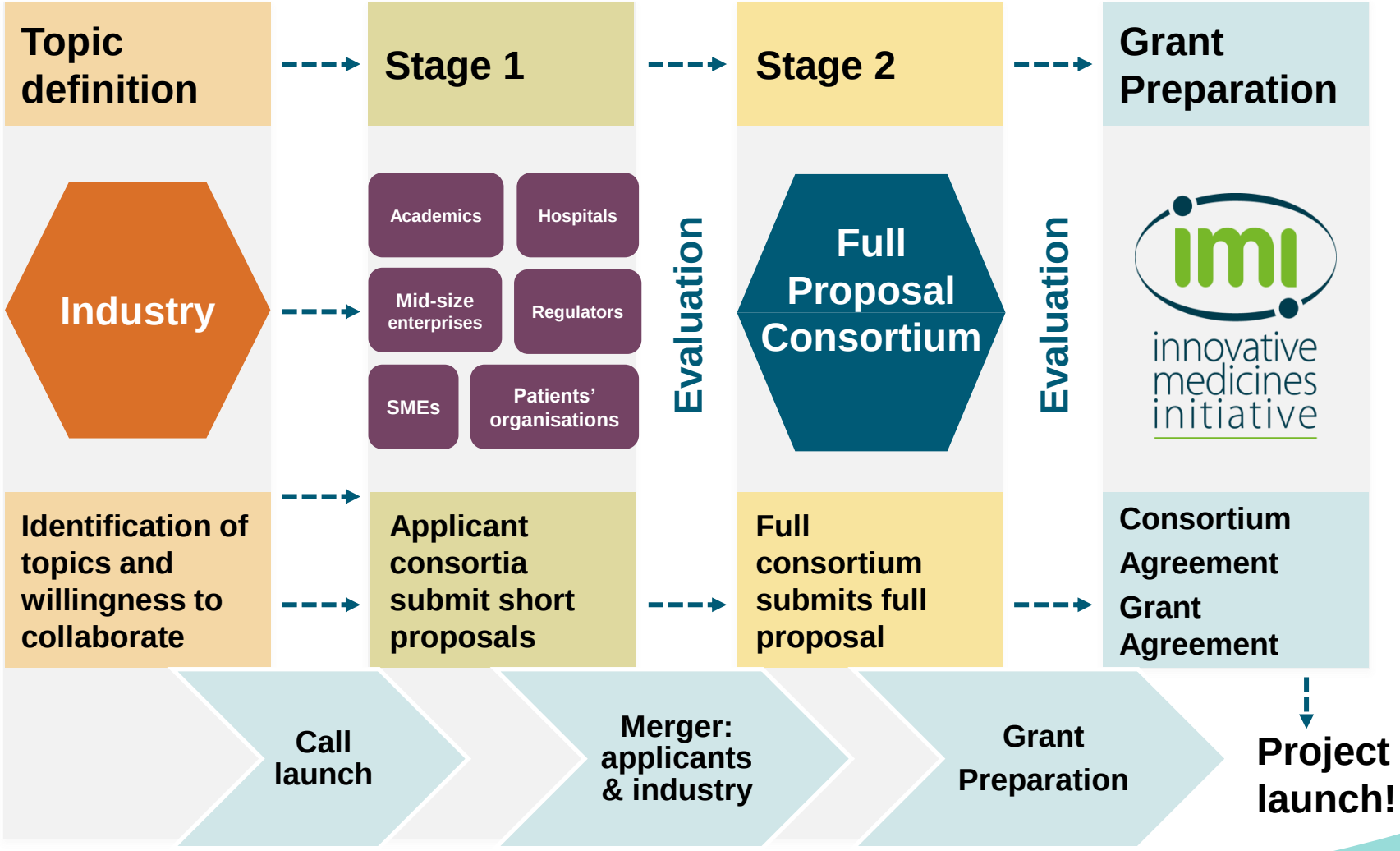
Typical IMI project life cycle



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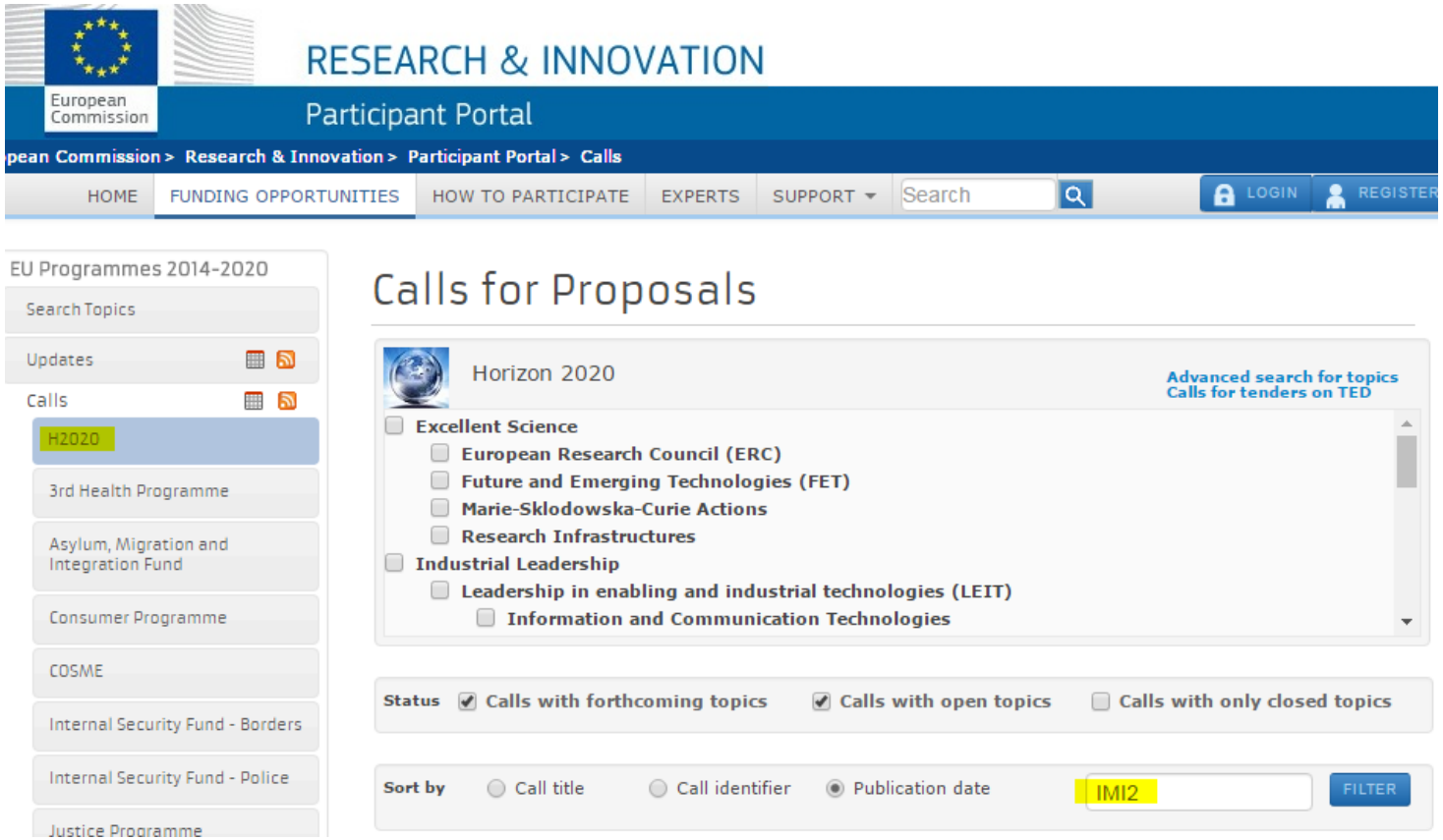
Submitting a proposal

- 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.

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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:**
  - IMI http://www.imi.europa.eu/content/partner-search
  - German NCP version: http://www.imi-partnering.eu
  - Fit for health: http://www.fitforhealth.eu/
- Get in touch with your **local IMI contact point**: www.imi.europa.eu/content/states-representatives-groups
- Talk to your **Health National Contact Point** (NCP)
- Network on **social media** (e.g. IMI LinkedIn group)

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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

Development of sensitive and validated clinical endpoints in primary Sjögren's Syndrome (pSS)

Peter Gergely on behalf of the Industry Consortium

18 July 2017 • IMI webinar

Primary Sjögren‘s syndrome (pSS)

Common disease with high unmet need and no disease modifying drug

- Prevalent systemic autoimmune disease
  - 0.3 to 0.5% of adult population; female:male ratio 9:1
- Clinical features
  - Secretory glandular involvement
    - Oral: difficulty swallowing, increased infections, poor dentition
    - Eyes: foreign body sensation, irritability, abrasion/ulceration
    - Vagina: painful intercourse
  - Extra-glandular involvement variably present in articular, GI, renal, pulm., vascular, nervous systems
  - Severe, disabling fatigue (85% of patients)
- Severity of systemic measures (e.g. SF36 vitality) on par with cancer, depression and other major diseases
- 40x ↑ lifetime lymphoma risk; occurs in ~5% of patients

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Challenges for medicines development in pSS

- Data from controlled and adequately powered clinical trials in pSS are scarce
- Mixed results in clinical trials using recently developed specific treatment outcome measures (e.g. ESSDAI, ESSPRI)
- Important features of pSS (such as swallowing difficulties, mental health challenges, sexual dysfunction, dental problems are not (adequately) captured.
- Overall, the utility of the currently available measures (including sensitivity to change in Patient Reported Outcomes (PROs) and in various ESSDAI domains) in assessing efficacy and disease-modifying potential of an investigational drug still to be determined.
- No objective, validated measure or functional marker of disease activity for assessing therapeutic benefits of improvement
- Sensitive and validated endpoints including objective measures/biomarkers of improvement needed to increase the likelihood of success of drug development in pSS

European League against Rheumatism (EULAR) Sjögren's syndrome disease activity index (ESSDAI)
EULAR Sjögren's syndrome patient reported index (ESSPRI)

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Need for public-private collaboration

- The ability to measure and monitor clinically relevant outcomes in pSS populations is an early need in the field of drug development prior to the existence of proven disease-modifying therapies.
- The development of such measures by generating larger datasets, if handled by the academia or industry alone, is a major challenge.
- Therefore, engagement of all relevant stakeholders via IMI including academia, pharmaceutical industry, patient groups, regulators, payers, HTA and health policy makers is essential

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Pre-competitive nature of IMI

- The IMI design allows pharma companies to collaborate with academia, payer, regulatory, patient organisations and other important stakeholder partners to prepare health outcome data for health/social care and data systems as a base for discussion and decisions for support for health care system transition to value based healthcare and increased health outcomes focus
- Value based health care with increased health outcome focus is expected to allow a better use of scarce resources within the health care budgets of member states

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Overall goal and themes of proposal

Initiative within IMI2

Goal

- The overarching objective of this proposal is to develop sensitive and validated clinical endpoints for use in future clinical trials of pSS

Objectives

1

Data generation and review

- Review and analysis of existing data (e.g. from registries or published data)
- Results from prospective RCTs from the industry partners

2

Development of new outcome measures

- Based on the review and analysis activities
- Involvement of regulators, patient groups and payers
- Novel endpoints (e.g. digital endpoints)

3

Application and validation by prospective testing

- Dedicated, prospective clinical trial to test proposed new pSS outcome measures, as well as existing ones

4

Analysis of the outcome of the validation trial and validation of the new endpoint(s)

- Comparison of the performance of the new outcome measures to that of the existing ones

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Key deliverables of the full project

- New sensitive and validated pSS outcome measures
  - (i) Identification and characterization, (ii) prospective qualification and (iii) potential regulatory and payer acceptance of new disease scoring tools to assess key features of pSS including disease activity, organ specific improvement and reduced damage under therapy.
  - Identification and validation of a biomarker or sets of prognostic markers that could be used as a surrogate endpoint(s) in Phase II trials, and which would be early predictors of long-term organ specific changes or adverse systemic outcomes
- Evidence for the characterization and usefulness of the currently available outcome measures (e.g. ESSDAI or ESSPRI).

Projects and initiatives that may be considered for collaboration

- HarmonicSS (http://cordis.europa.eu/project/rcn/207205_en.html), a Horizon 2020 ongoing project.
  - One of the goals of HarmonicSS is the “Data generation and review”, that is very similar to the scope of this topic. Thus, a collaboration with this project would allow a more rapid progression and a more thorough and extensive data analysis. The synergy of the two initiatives would therefore be of mutual benefit. The prospective validation trial may also be done in collaboration.
- PRECISESADS (www.precisesads.eu), a IMI ongoing project that aims to molecularly reclassify Systemic Autoimmune Diseases.
- EULAR (www.eular.org) task force responsible for classification guidelines and EULAR sponsored EU pSS registries
- In addition, collaborations with transatlantic projects and initiatives:
  - E.g. by the American College of Rheumatology (www.rheumatology.org) and/or by the Sjögren's Syndrome Foundation (https://www.sjogrens.org) may also be considered.

Expected impact

- Enhance the development of new systemic treatments in pSS and result in more efficient clinical trial designs
- Increase the success rate in clinical trials for pSS
- Reduce time to reach clinical proof of concept for medicine development
- Develop new diagnostic and treatment biomarkers for pSS

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Industry Consortium: expected contribution

- Program management
- Clinical trial design
- Clinician, clinical pharmacologist, statistician or clinical scientist from each company
- Biostatistical / data management expertise
- Regulatory expertise in interacting with regulatory health authorities
- Clinical operations
- Business planning and development; financial planning
- Legal conselling
- Industry-sponsored clinical trials and data

Applicant Consortium: expected contribution

- Experience and knowledge in conducting clinical trials in pSS
- Expertise in science of drug development
- Access to large representation of pSS population(s)
- Patient reported outcomes, development and validation
- Physicians and other healthcare providers
- Patient advocacy organisations
- Regulatory expertise, including interacting with regulatory authorities
- Expertise in interacting with national payers
- Information technology / data management
- Expertise in legal and clinical compliance aspects
- Strong project management and communication expertise
- Office administration and website management

Industry partners, proposed budget and project duration

- Industry partners:
  - Novartis (lead)
  - GlaxoSmithKline
  - Bristol-Myers Squibb
  - Servier
  - Eli Lilly
- Proposed budget:
  - 8.2 EUR mio in-kind from industry partners
  - 8.2 mio EUR from IMI
- Proposed duration: 6 years

Suggested architecture

Proposed Work Packages

1. Project management and Oversight of IMI project
2. Understanding of pSS disease mechanisms and outcomes
3. Generation of novel enpoints, design and execution of clinical trial to validate endpoints
4. Evaluation of vadidation trial results
5. Biomarkers
6. Engagement of key stakeholders including health authorities and payers
7. Legal and ethical compliance
8. Communication

What's in it for you?

- Research/Academia: Access to data from industry sponsored trials and ability to drive the development of novel endpoints and recommendations on appropriate outcome measures in pSS
- Regulatory/HTA/Payers/Policy Makers: Contribute and shape the generation of comprehensive outcomes evidence to establish a commonly accepted base for the use of outcome measures in pSS
- Patient Organisations: Contribute to ("have a say" about) the development of patient relevant outcomes and digital patient engagement solutions
- SMEs: Outline data strategies to collect and characterize health outcomes relevant for decision making in value based health care

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Thank you

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@IMI_JU

Involvement of SMEs, patients and regulators

Nathalie Seigneuret
IMI webinar • 18.07.2017

SME Participation

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

In particular, in this topic, SMEs may include (not limited to:

- Biostatistics and pharmacometrics specialty groups.
- Healthcare research and analysis groups.
- CROs

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Patient Participation

In this particular topic, clear opportunities to improve project performance by working with your patient partners:

- to contribute to development and standardisation of study procedures and processes
- assess feasibility
- input into development of clinically meaningful endpoints and benefit-risk discussion
- community outreach and dissemination

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

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Interaction with Regulators

To maximise impact of science generated by projects

Engage dialogue with Regulatory Authorities

- Consider having a plan for interaction with relevant milestones, resources allocated
- When relevant need for formal regulatory processes to ensure regulatory acceptance of projects results (e.g qualification procedure for biomarkers)
- Get familiar with various services offered for dialogue (e.g at EMA through qualification advice, Innovation Task Force, briefing meetings)
- If not participant, consider Regulators in the advisory board
- For more info see ‘’Raising Awareness of Regulatory Requirements: A guidance tool for researchers’’ available at http://www.imi.europa.eu/sites/default/files/uploads/documents/RegulatoryRequirementsGuide.pdf
- Consider also plan for dialogue with HTA bodies/payers as relevant

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Questions & answers