4th IMI2 Call for proposals

Tel +32 (0)2 221 81 81 • Fax +32 (0)2 221 81 74
infodesk@imi.europa.eu • www.imi.europa.eu
Postal address: IMI JU • TO56 • 1049 Brussels • Belgium
Visiting address: Ave de la Toison d'Or 56-60 • 1060 Brussels • Belgium

Introduction

The Innovative Medicines Initiative 2 (IMI2) Joint Undertaking has been created[1] following the below principles:

- Research related to the future of medicine should be undertaken in areas where societal, public health and biomedical industry competitiveness goals are aligned and require the pooling of resources and greater collaboration between the public and private sectors, with the involvement of small and medium-sized enterprises (SMEs).
- The scope of the initiative should be expanded to all areas of life science research and innovation.
- The areas should be of public health interest, as identified by the World Health Organisation report on priority medicines for Europe and the World (2013 update: http://www.who.int/medicines/areas/priority_medicines/en/).

The initiative should therefore seek to involve a broader range of partners, including mid-caps[2], from different sectors e.g. biomedical imaging, medical information technology, diagnostic and/or animal health industries (while ensuring gender matters are considered). Involving the wider community in this way should help to advance the development of new approaches and technologies for the prevention, diagnosis and treatment of diseases with high impact on public health.

The IMI2 Strategic Research Agenda (SRA) is the main reference for the implementation of research priorities for IMI2. Based on the SRA the 2014 scientific priorities for IMI2 have been prepared, which include themes on regulatory, health technology assessment and healthcare delivery challenges which are addressed in this call.

Applicant consortia are invited to submit proposals on the topic. These proposals should address all aspects of the topic. The size and composition of the consortium should be adapted to the scientific goals and the expected key deliverables.

While preparing their proposals, applicant consortia should ensure that needs of patients are adequately addressed and, where appropriate, patient involvement is encouraged. Synergies and complementarities with other national and international projects and initiatives should be explored in order to avoid duplication of efforts and to create collaborations on the global level and to maximize European added value in health research. Where appropriate, the involvement of regulators is also strongly encouraged.

Before submitting a proposal, applicant consortia should familiarize themselves with all call documents such as the IMI2 Manual for evaluation, submission and grant award, and the IMI2 evaluation criteria. Applicants should refer to the specific templates and evaluation procedures associated with coordination and support actions (CSAs).

[1] The Council Regulation (EU) No 557/2014 of 6 May 2014 establishing the Innovative Medicines Initiative 2 Joint Undertaking.

[2] Under the IMI2 JU, mid-sized companies having an annual turnover of EUR 500 million or less, established in a EU Member State or an associated country, are eligible for funding.

Topic 1: Enabling platform on medicines adaptive pathways to patients

Topic details

Topic code	IMI2-2015-04-01
Project type	Coordination and support action (CSA)
Submission & evaluation process	2 stages

Specific challenge

The pharmaceutical industry is facing considerable challenges as shown by the attrition rates for new medicine developments that have significantly increased across all Research & Development (R&D) phases since the early nineties of the previous century. The regulatory environment is lagging behind rapidly evolving science; conventional R&D models are no longer financially viable and have become a major hurdle to efficient drug development; general response rates to modern medicines are not satisfactory. A more flexible pathway within the current pharmaceutical legislation and reimbursement framework would not only accelerate access of crucial therapies to patients in need but would also increase the probability of success, as therapies would be oriented towards those deemed most likely to respond. Medicinal therapy is rapidly moving towards a personalised medicine paradigm, targeting smaller groups, better defined and better responding groups of patients with the ultimate goal to offer more efficacious and safer treatments.

The cost of development for industry and for healthcare providers could be significantly reduced with such an approach. There are already initiatives to explore new regulatory pathways, including the New Drug Development Paradigms (NEWDIGS) initiative at Massachusetts Institute of Technology (MIT)[3], the FDA's Breakthrough Program[4], the UK's Early Access to Medicines Scheme (EAMS)[5] and more recently the EMA Adaptive Licensing Pilot project[6]. These initiatives are encompassed under the concept of Medicines Adaptive Pathways to Patients (MAPPs). MAPPs refers to a flexible development and access pathway within the current regulatory framework that maximizes the positive impact of new medicines on public health by balancing timely access for patients with the need to provide evolving information on benefits and risks. It requires the early marketing authorisation of a product focused on a well-defined and targeted population identified by predictive preclinical and clinical evidence as well as various sources of real world evidence. It implies a clear safety and efficacy profile and may integrate a number of elements such as adaptive clinical trial design, patient centric benefit/risk assessments and the continuous evaluation of a therapy as new evidence (including real world evidence) becomes available. MAPPs, therefore, relate to the entire life cycle of a medicine from development, through licensing to patient access (pricing/reimbursement and healthcare delivery).

Although MAPPs is already discussed in many public forums, a pre-requisite for the success of MAPPs implementation lies in full and common understanding of its value, not just for industry but across the entire innovation life cycle: for regulators, Health Technology Assessment (HTAs) bodies, payers, governments, clinicians and, most importantly, patients.

The EMA's pilot project is testing the concept of adaptive licensing with real assets, in order to gather sufficient knowledge and experience, address a range of technical and scientific questions and refine how the

3.http://cbi.mit.edu/research-overview/newdigshomepage/

4.http://www.fda.gov/regulatoryinformation/legislation/federalfooddrugandcosmeticactfdcact/significantamendmentstothefdcact/fdasia/ucm341027.htm

5.http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm

6.http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2014/03/news_detail_002046.jsp&mid=WC0b01ac058004d5c1

adaptive licensing pathway should be designed for different types of products and indications. At the European Commission, DG Health & Food Safety has started a reflection process with the Member States on legislative and general policy aspects.

As compared to adaptive licensing, MAPPs go beyond since encompassing the entire life cycle of a medicine from development, through licensing to patient access (pricing/reimbursement and healthcare delivery). To support MAPPs implementation, new enabling methodologies and tools may need to be developed and tested through research projects. Coordination and pragmatic integration is needed to define a new approach to R&D and patient access, as well as its applicability within the current legislative framework and its viability for all stakeholders.

Support is therefore required to bring together, under a neutral collaborative framework, relevant stakeholders from the public and private sectors to foster discussion and coordinate scientific activities under IMI2 to progress innovative, pragmatic and viable solutions required to implement MAPPs within the current pharmaceutical legislation. Therefore this coordination and support action (CSA) should be operational promptly to coordinate effectively the MAPPs activities within IMI2.

Scope

The overall scope of this CSA is to establish an enabling platform with relevant stakeholders for the coordination of MAPPs related activities within IMI2 and engaging a dialogue with relevant stakeholders.

More specifically, the CSA will provide a forum that will enable:

- **gap analysis**: identify scientific challenges and opportunities for the application of MAPPs, taking account of tools, methodologies and infrastructures developed in IMI projects and other initiatives including any learnings of the EMA Adaptive Licensing Pilot project;
- **informing research activities**: facilitate the inclusion of MAPPs enablers (tools and methodologies) in new IMI2 research and innovation actions based on the gap analysis;
- **knowledge management**: horizon scanning on non IMI activities relevant to MAPPs to create a comprehensive repository of knowledge and opportunities for coordination.

Activities such as experts meetings, engagement with on-going and future IMI projects and relevant groups involved in IMI projects' definition (e.g. IMI Strategic Governing Groups, EFPIA Research Directors Group, IMI2 bodies) and dissemination/communication of conclusions and recommendations, will contribute to align understanding of impact of MAPPs versus current paradigm and to share learnings between all stakeholders.

Expected impact

The expected impact would be a comprehensive scientific research plan for the development and exploitation of tools, methodologies, infrastructures to help informing the whole product life-cycle and provide the science-based evidence to enable early patient access to innovative prevention and treatment options.

Potential synergies

The proposal should build on achievements and learnings from relevant IMI projects and the results from the IMI 'Think Tank' (IMiPACT) initiative. It should aim to create synergies and complementarities with relevant projects, in particular IMI projects such as Get Real and H2020 related projects. The proposal should also consider other initiatives both at EU level (e.g SEED, EMA adaptive licensing pilot) as well as globally (e.g NEWDIGS).

Industry consortium

AstraZeneca (lead), BMS (co-lead), Amgen, Astellas, Bayer, Boehringer-Ingelheim, Eli Lilly, GSK/GSK vaccines, Ipsen, Janssen, Lundbeck, Merck KGaA, MSD, Novartis, Novo Nordisk, Pfizer, Roche, Sanofi R&D/Sanofi Pasteur, UCB, Lysogene and EFPIA office.

Industry will provide expertise in regulatory, HTA/pricing and reimbursement, R&D, clinical development, clinical trials, benefit/risk assessment, legal and IP, medical and health affairs and communication. The industry consortium will contribute to:

- perform the analysis of IMI projects outputs, conceptual work around translation of these outputs into regulatory and medical outcomes;
- establish liaison with non IMI initiatives, coordination with various industry fora and across geographic areas, and liaison with other industry sectors;
- interact with on-going and future IMI projects and relevant groups involved in IMI projects' definition (e.g. IMI Strategic Governing Groups, EFPIA Research Directors Group);
- monitor non IMI activities relevant to MAPPs;
- prepare materials for internal and external communication and dissemination of recommendations and conclusions of the forum.

The industry consortium will also provide their expertise in the conduct and follow up of management tasks to support to the platform (including any IT system to help the work of the platform and the communication between partners) as well as support to the organisation of meetings/workshops/teleconferences.

Applicant consortium

The Applicant Consortium is expected to address the objectives and make key contributions in synergy with the industry consortium. This may require to mobilise: Knowledge and expertise in medicinal products' life cycle; Sound understanding of the R&D pathways and their challenges; Ability to develop outreach and communication strategies on the role and challenges of MAPPs to the stakeholders and public at large; Proven expertise for managing and coordinating major projects of this complexity and scale.

The Applicant Consortium is expected to be multidisciplinary and to enable effective collaboration between key stakeholders (e.g regulatory agencies, HTA, payers, academia, hospitals, SMEs, and patient organizations).

The size of the consortia shall be carefully considered by the applicants and limited to secure the operational efficiency of the CSA.

Indicative duration

The indicative duration of the coordination and support action is 30 months.

Potential applicants must be aware that the Innovative Medicines Initiative 2 (IMI2) Joint Undertaking, if so foreseen in the applicable annual work plan, may publish at a later stage another call for proposals restricted to the action already selected under this call in order to allow continuation of the existing enabling platform and to enhance its results and achievements by extending its duration and funding. Consortia will be entitled to open to other beneficiaries as they see fit. The detailed scope of the call will be described in the relevant annual work plan.

Indicative budget

The indicative EFPIA in-kind contribution will be EUR 1 130 000.

The indicative IMI2 JU contribution will be a maximum of EUR 1 130 000.

Suggested architecture of the full proposal

The Applicants are expected to suggest architecture for the full proposal to set up the platform that addresses the scope and the expected impact of this CSA, as well as incorporating and complementing the industry consortium contribution.

The consortium will be expected to keep informed the European Commission of the activities of the CSA, in particular the responsible unit of DG Health & Food Safety.

The successful applicant consortium will be expected to adhere to the following principles, if inappropriate please provide rationale in the short proposal:

1) Disseminate scientific publications and research data on the basis of open access. Collection, processing and generation of research data is to follow documented data management procedures (see "Guidelines on Open Access to Scientific Publications and Research Data in Horizon 2020" and "Guidelines on Data Management in Horizon 2020 "). In order to ensure adherence to the legislation concerning protection of personal data, controlled access digital repositories and data governance will need to be established.

2) Use well-established data format and content standards in order to ensure interoperability to quality standards. Preferably existing standards should be adopted. Should no such standards exist, consideration should be given to adapt or develop novel standards in collaboration with a data standards organization (e.g. CDISC).

Conditions for this call

Applicants intending to submit a short proposal in response to the IMI2 Call 4 should read the topic text, above, the IMI2 Manual for submission, evaluation and grant award and the IMI2 Evaluation Criteria.

Call Identifier: H2020-JTI-IMI2-2015-04-two-stage

Type of action: Coordination and support action

Publication Date: 17 December 2014

Stage 1 Submission start date: 17 December 2014

Stage 1 Submission deadline: 11 February 2015 – 17:00:00 Brussels time

Indicative Budget: From EFPIA companies: EUR 1 130 000. From the IMI2 JU: EUR 1 130 000.

Call topics

IMI2-2015-04-01	The indicative contribution from EFPIA companies is EUR 1 130 000 The financial contribution from IMI2 JU is a maximum of EUR 1 130 000	Coordination and support action. Two stage submission and evaluation process. Only the applicant consortium whose proposal is ranked first at the first stage is invited for the second stage.

Indicative timetable for evaluation and grant agreement

	Information on the outcome of the evaluation (first stage)	Information on the outcome of the evaluation (second stage)	Indicative date for the signing of grant agreements
IMI2-2015-04-01	Maximum 3 months from the date of submission to the first stage.	Maximum 2 months from the date of submission to the second stage.	Maximum 2 months from the date of informing the applicants following the second stage evaluation.

Consortium agreements

In line with the Rules for Participation and Dissemination applicable to IMI2 actions[7] and the IMI2 model grant agreement, participants in Coordination and Support Actions are not required to conclude a consortium agreement.

[7] Regulation (EU) No 1290/2013 of 11 December 2013 and Commission Delegated Regulation (EU) No 622/2014 of 14 February 2014.