

Second Interim Evaluation

IMI - Innovative Medicines Initiative

Joint Undertaking

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Executive Summary

The overall result of the evaluation of the Innovative Medicines Initiative (IMI) Joint Undertaking (JU) performed by a panel of independent experts (the Panel) is positive.

IMI's main objectives are to address the bottlenecks currently limiting the efficiency, effectiveness and quality of the drug development activities needed to bring innovative medicines to the market. Over the past two review periods, IMI has successfully demonstrated the feasibility of large, multi-stakeholder PPPs for research and development in biomedicine. The new business model created by IMI is well established and has leveraged the research strengths across the European pharmaceutical industry, academia and small to medium enterprises (SMEs). It has established over 40 public-private consortia which are delivering projects of high relevance to healthcare challenges. In addition the open innovation framework provided by IMI has facilitated the formation of consortia comprising a wide range of participants, including patient groups and regulators, and built trust between them. It is now perceived globally as the leading public-private partnership (PPP) in healthcare.

The methodology followed by the Panel was based on the Commission Terms of Reference, which provided a set of predefined questions under the evaluation criteria, effectiveness, efficiency and quality. The Panel built its assessment on (i) documents and other published information and (ii) interviews with a wide range of IMI stakeholders, including representatives of founding members, IMI bodies, participants of on-going IMI-supported research projects, representatives of regulatory bodies, patients' organizations, research and SME associations (listed in Annex 4).

The first interim review identified many strengths of IMI but also some areas for improvement. The current panel agreed with the Governing Board (GB) and the IMI Executive Office that the IMI had been responsive to the recommendations of the first interim review and had made much progress in implementing them, for example, in demonstrating scientific excellence and improved stakeholder engagement. Significant progress has been made in engaging with a diverse group of stakeholders beyond industry and academia, stimulating greater participation of regulators, SMEs and patient groups. Regulators have engaged at many levels with IMI consortia - from observer to direct project participant. Improvements in other areas, such as the development and implementation of a broader communication strategy and the formulation of a range of key performance indicators (KPIs), are on-going.

Since its inception, IMI has brought together over 350 EFPIA research teams, approximately 600 academic scientific teams and more than 100 SMEs to work together across Europe to accelerate medicines development. Achievements have already been seen in many phases of R&D:

- New screening methodologies both pre-clinically and clinically e.g., in diabetes and Alzheimer's disease;
- More rapid identification of new therapeutic targets in areas of high unmet need such autism and schizophrenia;

- Biomarker identification, qualification and validation in a range of different medical conditions;
- Predictive toxicology and safety;
- Enhanced clinical trial design;
- Building effective platforms for data storage, integration and interrogation;
- Biomedical education and training.

Through these activities IMI has been able to reinforce Europe's attractiveness for pharmaceutical R&D, stemming the flow of investment away from Europe to the USA and Asia. Although it is too early to evaluate the success of IMI in terms of new medicines, the impact on the efficiency of the R&D process is already being felt, for instance, in the cost reduction and better design of clinical trials. Most of the projects funded under IMI could not have been attempted by a single company, SME or academic group. Many of the projects have generated meaningful data in a much shorter time frame than would otherwise have been the case. The IMI JU has generated twice as many direct jobs per euro spent compared to FP7 projects, creating approx. 1,500 new jobs thus far, with an average cost per job of 200 000 euro, compared to 400 000 euro per job in FP7 projects. Therefore the IMI has been effective in delivering on its main objectives.

The quality of the research undertaken within IMI projects was judged very high and according to most interviewees had exceeded expectations in terms of scientific excellence. IMI projects have so far produced over 320 publications in more than 150 peer reviewed journals including high impact factor journals such as Nature and Science. The average citation impact of IMI funded research is well above world and European averages and more than 10% of IMI papers are 'highly cited'. It is noteworthy that approximately 2/5 of the publications by IMI researchers are cross-sector - i.e. between academia, big pharma and SMEs.

The efficiency of the IMI was assessed through a review of the KPIs, the governance structure and processes, communication strategies and the use of funding including the dissemination and uptake of research outcomes. In general IMI has demonstrated an improvement in efficiency over the review period, especially through implementation of some of the 1st interim review recommendations. The main recommendations below reflect areas where, in the Panel's view, improved effectiveness and increased efficiency could help the IMI meet its overarching objectives and further increase its impact.

The Panel concluded that the intellectual property terms were clear to most potential participants and that the two stage call process appeared to be working well. There was widespread agreement that the call process had improved significantly and the timelines had been considerably reduced. Although the call topics were reasonably clear, some interviewees felt that they were too prescriptive. It was also unclear to those interviewees how non-EFPIA stakeholders could influence the call topics. The contributions of both the Scientific Committee (SC) and the States Representative Group (SRG) were recognized, but there was a general consensus that the contributions of these bodies to the IMI could be further improved.

Overall the Panel was of the opinion that alongside considerable strengths and achievements of the IMI, there were areas that needed some further attention and other areas where the opportunities provided by the IMI could be better leveraged.

Recommendation 1: The IMI needs to finalize and implement an articulated communication strategy with clear and measurable goals and objectives, addressing both the key stakeholders and a wider audience.

Whilst the IMI has been effective at communicating call topics to potential participants, now is the time, as success stories are increasing, for a concerted, broader communication effort. The IMI needs to implement the recently revised communication strategy and have clear annual objectives and outcomes against which progress on the implementation plan can be measured.

Recommendation 2: Alongside the existing KPIs, aggregated KPIs need to be developed and measured in order to quantitatively demonstrate the IMI impacts and socio-economic benefits.

Whilst KPIs have been developed, they have focused primarily on very concrete scientific output. The socio-economic potential has not been sufficiently well captured. The fact that IMI has helped to create over 1500 direct new jobs, for example, is commendable and more metrics of that kind need to be in place.

Recommendation 3: The IMI should make an additional effort to increase engagement from a wider range of industry stakeholders.

Although previous efforts by the IMI to involve SMEs in IMI projects have attained a level of involvement which is already above other EU-funded health research programmes, there is still room for a greater SME engagement. The Panel received suggestions that IMI could benefit from the participation of smaller pharmaceutical companies that do not have SME status and are not EFPIA members. In addition there is scope for greater EFPIA engagement, especially where expertise resides outside the EU.

Recommendation 4: The IMI Executive Office should seek further ways of reducing bureaucracy and ensure that it has the optimal organizational structure for the tasks ahead.

Whilst the IMI Executive Office has made significant progress in speeding up processes and reaching operational efficiency, the Panel felt that some further adjustments might be needed to improve efficiency. Now that the IMI is well established, the balance of skills between general administration and project management in the Executive Office needs some readjustment to ensure that projects deliver and the benefits are fully realized.

Recommendation 5: The IMI should seek to maximize the potential of its advisory bodies to gain support for the remaining calls and other activities at all levels.

The SC and SRG mandates are clear within the governance structure but their current configuration may not be optimal for these mandates. Moreover there is room for a greater and more pro-active involvement of the SC and SRG with other external experts, for example the SC in the review process and the SRG in dissemination activities in order to leverage their full potential.

Recommendation 6: The IMI needs to plan for and design new and more flexible funding mechanisms to ensure the sustainability of current and future projects, where appropriate.

The IMI has recognised this need and has already put in place one funding mechanism (ENSO) to aid project sustainability, but more mechanisms need to be explored.

As a result of the progress of the current IMI JU and its productivity it has been proposed to be continued and expanded under Horizon 2020. The Panel supports this proposal and in light of the review, has some additional recommendations for the future IMI2 JU.

Recommendation 7.1: Baseline data should be obtained in parallel with the launch of IMI2 in order to allow for better benchmarking and assessment of IMI2 performance.

The Panel feels that a study of the Pharmaceutical Industry in Europe should be undertaken by the Commission and EFPIA in parallel with the launch of IMI2 and compared to the 2007 study that was used as a baseline for the current IMI. This study would provide an overview of the overall impacts and any new developments in healthcare and serve as an updated / interim baseline for the IMI2 initiative under the Horizon 2020.

Recommendation 7.2: Industrial participants from other healthcare related sectors should be involved in IMI2.

It is clear that an integrated approach to healthcare will be required including prevention and diagnosis. The Panel therefore recommends that every effort should be made to involve other industrial participants from these sectors in IMI2.

Recommendation 7.3: The Commission should ensure that IMI2 is transparent and has increased flexibility in terms of governance.

In IMI2 it should be ensured that the roles and mandates of the governance and advisory bodies (in particular the SC and the SRG) are clearly defined and the membership configured with the appropriate expertise to execute their mandates. The lessons learned from both the IMI and other JUs should be incorporated into the revised governance structure of IMI2.

List of Acronyms used in the document

AIP	Annual Implementation Plan
EC	European Commission
EIB	European Investment Bank
EFPIA	European Federation of Pharmaceutical Industries and Associations
ENSO	Exploitation of New Scientific Opportunities financing mechanism
EoI	Expression of Interest
ERR	Economic Rate of Return
EU	European Union
FCH	Fuel Cell Hydrogen
HTA	Health Technology Assessment
IER	Interim Evaluation Report
IMI	Innovative Medicines Initiative
IMI2	The proposed new IMI under the Horizon 2020
IP	Intellectual Property
IPR	Intellectual Property Rights
JTI	Joint Technology Initiative
JU	Joint Undertaking
KPI	Key Performance Indicator
PPP	Public Private Partnership
RDG	EFPIA Research Directors Group
SME	Small and Medium Sized Enterprise
SC	Scientific Committee
SRA	Strategic Research Agenda
SRG	State Representatives Group

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1. Introduction

1.1. Objectives of the Second Interim Evaluation of the IMI JU

The present report is the result of the work of the Independent Expert Group (hereinafter referred to as the “Panel”), appointed to assist the Commission in carrying out the second interim evaluation of the Innovative Medicines Initiative Joint Undertaking (IMI JU). The evaluation performed by the Panel is based on the Terms of Reference¹, defined by the European Commission after consultation with the IMI JU. Its objective was to assess the IMI JU against three criteria: *the effectiveness, the efficiency and the quality of research*. The Panel also evaluated the *progress of the IMI JU towards the objectives set* and the level of *implementation of recommendations from the first interim evaluation*.

1.2. Methodology of the Second Interim Evaluation of the IMI JU

The Panel was composed of five individuals whose areas of expertise encompass various aspects of the pharmaceutical drug discovery and development process, research funding, technology transfer and commercialisation, IP and marketing, finance as well as policy assessment and evaluation issues. Short biographical sketches of the experts are presented in Annex 1².

The methodology followed by the Panel was based on the Terms of Reference, which provided a set of predefined questions under the evaluation criteria. These questions were subsequently revised during the course of the interviews and supplemented by an additional set of “horizontal” questions (both sets are presented in Annex 2). The evaluation was performed by the Panel from March 1 until July 31, 2013 with a combination of remote work, conference calls and four Panel meetings in Brussels. The Panel built its assessment on (i) documents and other published information (see Annex 3 for the list of documents, most of them available on the IMI website³) and (ii) interviews with a wide range of IMI stakeholders, including representatives of both founding members, IMI bodies, participants of on-going IMI-supported research projects, representatives of regulatory bodies, patients organisations, research and SME associations (see the list in Annex 4).

After evaluation of the IMI performance to-date, a SWOT analysis (strengths, weaknesses, opportunities and threats) was carried out to place the assessment in the broader strategic framework, to review findings, and to develop sound recommendations.

¹ Innovative Medicines Initiative Joint Undertaking 2nd Interim Evaluation Terms of Reference, European Commission DG for Research and Innovation document, 2013.

² The views expressed by the independent experts do not necessarily represent the view of their respective institutions.

³ Innovative Medicines Initiative (IMI): www.imi.europa.eu

2. IMI JU– Background and Implementation

2.1. IMI JU Legal Basis

The IMI JU is a Public Private Partnership between the European Union, represented by the Commission (public partner), and the European Federation of Pharmaceutical Industries and Associations (EFPIA) (private partner). The IMI JU was set up by the *Council Regulation for the implementation of the Joint Technology Initiative (JTI) on Innovative Medicines*⁴ on the basis of Article 187 of the TFEU⁵. The IMI JU is established under European Law until 31 December 2017. It is a Union Body, which became autonomous on 16 November 2009, meaning that it has now the operational capacity to implement its own budget. Before the autonomy, the Commission was responsible for the management of the IMI JU⁶.

2.2. IMI JU Objectives

The IMI JU objective is to remove bottlenecks and significantly improve the efficiency, effectiveness and quality of the drug development process, with the long-term aim that the European pharmaceutical sector produces safe, effective, innovative medicines more rapidly. It also aims at stimulating investment in the biopharmaceutical sector in Europe in order to leverage research capabilities in a sector where the EU traditionally enjoys a comparatively strong position. For the past two centuries pharmaceuticals have been a stronghold of the European industry and they still provide by far the largest contribution to the European trade balance in high-technology, R&D intensive sectors⁷.

2.3. IMI JU Governance

The IMI JU is composed of three bodies (Governing Board, Scientific Committee, Executive Director with the support of the IMI Executive Office) and is supported by two external advisory bodies (States Representatives Group and Stakeholder Forum) – see Fig 2.1. The Scientific Committee is a part of the Governance Structure but its role is primarily advisory and not in decision making. A more detailed tabular description of the governance structure and functions has been presented the first Interim Evaluation Report (IER)⁸ and the situation has not changed since then.

The IMI JU periodically produces or updates the IMI Internal Control Standards, the IMI Staff Policy Plans; the IMI Annual Implementation Plans (AIPs); the IMI Annual Activity Reports and Annual Accounts. The IMI JU is housed in Brussels on the same premises as all other JTI JUs (Clean Sky, Fuel Cells and Hydrogen, ARTEMIS and ENIAC).

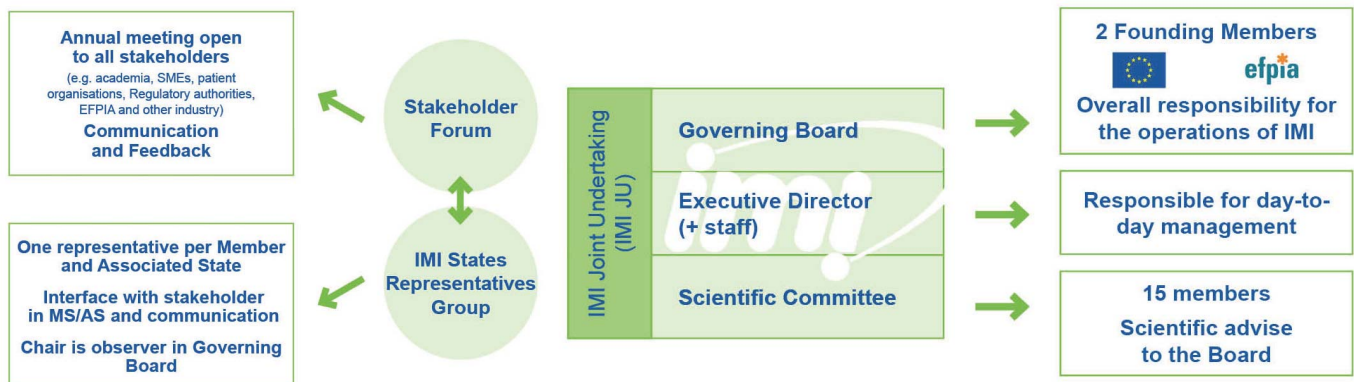
⁴ Council Regulation No 73/2008 (OJ L30 of 04.02.2008, p.38-51)

⁵ TFEU: Treaty on the Functioning of the European Union; Article 187 (ex-Article 171 of the EC Treaty): The Union may set up joint undertakings or any other structure necessary for the efficient execution of Union research, technological development and demonstration programmes.

⁶ Article 16 of the Council Regulation setting up the IMI JU

⁷ Gambardella, A., Orsenigo, L. and F. Pammolli (2000): *Global Competitiveness in Pharmaceuticals: A European Perspective*. Published in: DG Enterprise, European Commission (2001)

⁸<http://www.imi.europa.eu/sites/default/files/uploads/documents/Governance/FirstInterimEvaluationOfIMI2011.pdf>

Fig. 2.1 IMI Joint Undertaking Governance Structure

Source:

http://www.imi.europa.eu/sites/default/files/uploads/documents/Governance/IMI_Governance_March2012.pdf

2.4. Formulation and implementation of IMI JU Research Activities

Since the last interim review the Strategic Research Agenda (SRA) has been revised by the SC in consultation with the SRG and approved by the GB. The Scientific Priorities as defined in the SRA are implemented via the Annual Implementation Plans, which encompass research area descriptions. Topics are defined by EFPIA companies, which are committed to participate in a specific research area. The call topics to be published in the calls for proposals are consulted with the SC and experts in the field through workshops and are also consulted with the SRG and the European Commission. AIPs are prepared by the IMI Executive Office and need approval of the GB. Three of the AIPs produced to date are available on the IMI website:

<http://www.imi.europa.eu/content/documents#aip>.

Research Activities are realised through projects selected in open and competitive calls for proposals which are peer reviewed by independent experts. IMI currently operates a two stage call for proposals which has remained largely unchanged since the first IER.

Between 2008 and 2013, IMI has published nine calls for proposals (for the call 4-9 topics, see Annex 6).

The IMI 8th Call for Proposals was launched on December 17, 2012, with the 9th Call just announced in July 2013. Two more calls for proposals are scheduled to be launched before the end of 2013.

2.5. IMI JU Communication

Communication and Dissemination of the IMI JU is based on the Communication Plan, frequently referred to as an implementation tool for the “Communication Strategy”. The Innovative Medicines Initiative Communication Strategy is only under development.⁹ This document, not yet publicly available at the time of the 2nd Interim Evaluation Review, defines general and specific objectives and sets a comprehensive framework for IMI communication and dissemination.

Until now, this process has been realised through several communication channels and has targeted a wide range of IMI stakeholder groups. Various channels are used to address internal (inside the IMI consortia) and external (IMI stakeholders from research organisations, SMEs, patient organisations, regulators as well as the general public, politicians, Member State representatives) target groups. These channels include: events (webinars, meetings, info sessions, workshops, etc.), publications (scientific peer-reviewed publications, electronic newsletters, other on-line materials, printed articles and information brochures, etc.), website and social media as well as traditional media channels (news, newspaper and periodical articles, movie clips, etc.).

Communication is an important area that is discussed in more detail in subsequent sections of the report. The new IMI Communication Strategy aims to develop clear communication objectives and to increase the level of awareness of IMI amongst all target groups, also identifying critical success factors. Both documents (the IMI Communication Strategy and the Communication Plan) are at this stage internal documents that have been made available to the Panel by the IMI Executive Office.

⁹ Innovative Medicines Initiative Communication Strategy 2013-2014 prepared by
© MEDIA CONSULTA International Holding, version 1, June 28 2013 – internal communication.

2.6. IMI JU Knowledge Management and IP Policy

The IMI JU Knowledge Management and IP Policy is based on the following five documents: IMI IP Policy (2007)¹⁰, explanatory note of IPR Helpdesk (2008)¹¹, Clarification Note (2009)¹², IMI IP Guidance Notes (2010)¹³, all available at: http://www.imi.europa.eu/content/documents#ip_policy, and the Grant Agreement (2013)¹⁴ available at: http://www.imi.europa.eu/content/documents#grant_agreement.

The guiding principles for the IMI IP Policy¹⁵ ensure that it is:

- aligned with IMI objectives as a public-private partnership;
- adapted to specific research needs and challenges;
- enabling broad participation of:
 - private and public entities in IMI projects (academic institutions; small biopharmaceutical companies; large biopharmaceutical companies)
 - patients' organisations and regulatory agencies
- promoting knowledge creation, together with its disclosure and exploitation;
- achieving fair allocation of rights;
- rewarding innovation;
- providing flexibility for participants to establish the most appropriate agreements serving the project objectives.

The guiding principles for Dissemination are:

- obligation to disseminate the foreground;
- disseminate as soon as reasonably practicable, but no later than one year after project expiry or termination.

The material subject to dissemination is described in detail in the Contract Agreement.

IMI IP and Dissemination Policy was designed as one flexible policy to serve multiple interests. It is aimed to enhance bringing medicines to market by providing incentives for participation, freedom to access, compensation for background, dissemination of information thereby supporting European biopharmaceutical industry.

IMI has made an effort to discuss, clarify and guide its project applicants and beneficiaries as well as other stakeholder groups through the IPR issues. It has set up an IP Working Group with representatives from the EC, EFPIA and Member / Associated States. The group considers feedback and the experiences of parties engaged in IMI projects and reports this feedback and the group's recommendations to the Governing Board.

¹⁰ IMI Intellectual Property Policy, 2007

¹¹ IP Policy in IMI Actions, IPR Helpdesk, July 2008

¹² Clarification Note – IMI IP Policy, 2009

¹³ IMI IP Policy Guidance Notes for IMI Applicants and Participants, November 2010

¹⁴ IMI JU Model Grant Agreement Annex II – General Conditions, Part C, IMI-GB-DEC-2013-3

¹⁵ Poinot, Magali: New ways of sharing knowledge, Annual Stakeholder Forum presentation, June 2010, available at: http://www.imi.europa.eu/sites/default/files/uploads/documents/event05/magali-poinot_en.pdf

3. IMI governing bodies' response to the recommendations of the first IER

In this section the recommendations of the first IER and the degree to which they have been addressed are summarised. The material in this section presents the views and responses of the IMI governing bodies, namely the IMI Executive Director as well as representatives of EFPIA and the Governing Board:

An official response to the first IER recommendation has been published by the European Commission in September 2011 and is available on the IMI Website¹⁶.

The current 2nd IER Panel's assessment of each response is summarised at the end of each recommendation.

Recommendation 1: Continuously improve stakeholder involvement in IMI-supported research projects

Engagement across stakeholders in IMI should be further developed. Project participation would be broadened if perceptions of imbalance in the incentives available for SMEs, universities and research organisations were addressed. This must be achieved without losing the engagement of EFPIA organisations. In this regard, issues related to negotiation of intellectual property, reimbursement of indirect costs, and industry in kind contribution must be quickly and adequately addressed. The IMI JU should envision cooperation with non-EU stakeholders.

Several actions have been taken by IMI to implement this recommendation, as described in detail in a comprehensive summary from its Executive Director (See Annex 5)¹⁷ and in the Internal Communication to the Discharge Authority from 2011¹⁸. These actions included, among others: increased communication around each call; improvement in processes e.g. simplification of the financial rules and introduction in 2012 of non-EFPIA in-kind contribution in areas of high public health need as well as targeted efforts at particular stakeholders such as SMEs and patient organisation groups.

The Panel noted that IMI has made progress in this regard, however it is important to keep the momentum in this area as outlined in the Panel's recommendations.

¹⁶<http://www.imi.europa.eu/sites/default/files/uploads/documents/Governance/CommissionResponseInterimEvalIMI2011.pdf>

¹⁷ Note of the IMI Executive Office Director from June 11, 2013, IMI/OUT/2013-2861, a summary of which is provided in Annex 5 to this report.

¹⁸ Internal document C7 0300/2011- 2011/2241 (DEC) – Response and Measures taken by the IMI JU

Recommendation 2: Continuously ensure EFPIA and Commission commitment to IMI's success and sustainability

Continuous, adequate commitment of both the Commission and EFPIA to IMI is necessary to ensure IMI's success and sustainability. The consensus strategy driven by industry in the interest of public health is a unique strength of IMI. It requires industry to better develop its leadership responsibilities and to consolidate its commitment towards IMI. On the Commission side, lessons learnt for the "ideal house" of public private partnerships (see "Sherpa report") should serve the future of IMI and be crucial for other similar initiatives in the future.

This recommendation was targeted to EFPIA and the European Commission and the summary of actions taken was gathered via interviews and direct responses from EFPIA and the EC. The IMI Executive Office has summarised the main actions taken in this area.

Representatives of the European Commission and EFPIA as the funding members stated that they have invested significant time and resources in measuring performance and on this basis have evolved and adapted the IMI framework. According to EFPIA, as a result of setting up the "Simplification task force" in 2011, many of the IMI framework processes have been simplified and improved, allowing for the launch of more ambitious and larger scale projects. Concentrated efforts have been directed towards securing uptake of results – in particular in the clinical and regulatory practice.

The global R&D heads of large pharmaceutical companies (via the so-called "Hever group") have worked with the JU to identify and address key gaps in IMI strategy. Both EFPIA and the Commission have been very active in the on-going JU assessments, consultations and legislative debate on Horizon 2020 and IMI2.

The Panel welcomed the evidence that there is continued support for the IMI from both members of the GB.

Recommendation 3. Ensure excellence and exploit new ways to support IMI scientific objectives.

With the focus on good science to address drug development bottlenecks being the main priority of the IMI JU, the review of the IMI Research Agenda must have high priority and requires industry leadership in collaboration with other stakeholders. The IMI JU needs to consider new ways to better sustain the aims of the IMI Research Agenda.

The IMI Executive Office considered that many actions have been taken to ensure excellence. Research excellence is reflected in participant profiles, scientific output quality (IMI Bibliometric Analysis, 2013)¹⁹ and uptake of results by the regulators (guidance and biomarkers qualification).

According to the IMI Executive Director, the revision of its SRA in 2011 increased the ability of IMI to better explore and exploit IMI scientific objectives, with projects addressing the entire value chain. Focus has also shifted towards the higher end of the

¹⁹ Bibliometric analysis of ongoing projects: Innovative Medicines Initiative Joint Undertaking, prepared by Thomson Reuters on behalf of IMI JU Executive Office, Copyright IMI Executive Office, March 2013.

value chain – health technology assessment (HTA) and healthcare and business models to address patient access, including appointing a staff member of the IMI Executive Office accountable for liaising with regulatory authorities about projects and their outcomes; closer links with the European Medicines Agency (EMA) and its representative, who is now a permanent observer of the IMI SC; contacts were initiated by EFPIA and joint meetings have been held with the FDA (US) and PDMA (Japan) as well as several teleconferences with the relevant global regulators.²⁰

The Panel agreed that publication output was a useful metric of scientific excellence and that IMI has sought to expand the focus of activity. Nonetheless, further development of KPIs to measure and capture excellence is needed, as is discussed later in this IER.

Recommendation 4. Improve IMI communication

The understanding of IMI's purpose is still scattered and diffuse among various stakeholders, three years following the IMI JU legal set up. The underlying concepts of "pre-competitive research" or "open innovation" have also shown a lack of clarity among stakeholders. These issues need to be addressed urgently.

Although, according to the IMI Executive Office and the GB a "communication strategy" was developed in 2011 and updated in 2012, this is an area where they both see (much) room for improvement.

They outlined a number of actions undertaken by IMI in this area including: development of the IMI brand to emphasise the principles of "non-competitive research" and "open innovation" as well as the successes of IMI projects; increased communication activities since 2011 using various channels (including social media) to reach different stakeholder groups including academic teams, SMEs, regulatory agencies, patient organisations and policy makers; preparing communication on sensitive issues (i.e. late stage clinical trials) via webinars and surveys; preparation of key messages for different stakeholders for use by both by IMI staff and IMI ambassadors/multipliers (members of the SC, SRG and national opinion leaders).

It was acknowledged by the GB that the communication strategy needed to be significantly strengthened.

The Panel noted that in May 2013 the previous "communication strategy" (as detailed in the Communications Plan²¹) had been revised and the new Innovative Medicines Initiative Communication Strategy (still under development)²² builds on the success stories of IMI and identifies ways to more effectively reach a broader group of stakeholders in the Member States. This document would seem to form a solid basis for the development of a new detailed Communications Plan.

²⁰ Note of the IMI Executive Office Director from June 11, 2013, IMI/OUT/2013-2861, a summary of which is provided in Annex 5 to this report.

²¹ Note of the IMI Executive Office Director from June 11, 2013, IMI/OUT/2013-2861, a summary of which is provided in Annex 5 to this report.

²² Innovative Medicines Initiative Communication Strategy 2013-2014 prepared by
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Recommendation 5. Reinforce and streamline decision making and well-functioning processes

There is a need for clarification of the remits of all parties in the IMI structure, defining responsibilities and room for action and decision making. This is exemplified by the disparate views and opinions heard regarding which party is responsible for specific tasks relating to the first update of the IMI Research Agenda.

IMI believes that improved clarification has been achieved but this will be an on-going process. They have taken the following actions in this regard: reorganisation of the IMI Executive Office following its relocation in 2011; consolidation of operations followed by engagement and re-deployment of additional staff to better address organisational and stakeholder demands; addition of new competences through recruitment (MBAs, regulatory experience); improved information technology (IT) environments and tools for better coordination with the GB, EFPIA, EC, SRG and SC.

The Panel found in its interviews that there is still room for clarification thereby supporting the need for on-going activities in this area.

Recommendation 6. Ensure best use of IMI results and IMI sustainability

IMI should develop a sound long-term strategy towards knowledge management and learning processes in order to ensure best use of results and sustainability of the IMI concept.

According to EFPIA representatives, IMI lately has paid more attention to sustainability of results and their implementation in research, uptake in companies' research processes, setting up common standards, translating them into regulatory pathways and uptake in healthcare.

The following specific actions were undertaken to address the above recommendation²³: a knowledge management working group was set up in 2011; data standards unification in IMI projects have been ensured so that they adhere to international data standards for clinical subscription; training workshops and seminars were conducted and transatlantic collaboration was increased; engagement of IMI stakeholders in discussions on IMI activities occurred through organisation of Stakeholder Forums (2011, 2012, 2013).

The Panel agreed that much has been achieved. However, still more coordination and cross-project collaboration may be desirable to assure best use of IMI project results and IMI overall sustainability.

²³ Internal document C7 0300/2011- 2011/2241 (DEC) – Response and Measures taken by the IMI JU

Recommendation 7. Develop monitoring and evaluation processes

There is a need to develop sound monitoring and evaluation processes, to generate the indicators and evidence needed to strengthen IMI's capabilities for monitoring of projects and taking strategic decisions. The results should be measured regularly and accountability for results should be ensured.

According to EFPIA, two sets of KPIs – strategic and operational – have been developed and they are subject to review at each board meeting and influence all IMI activities. The GB leadership had some reservations about the robustness of the current KPIs to implement the monitoring and evaluation strategy.

According to the IMI Executive Director a series of actions have been taken since the first IER: identifying a KPI framework that was endorsed by the GB in 2011 with KPI targets for 2012 & 2013 approved by the GB; KPI measurements were presented in 2011 and 2012 IMI Annual Activity Reports; IMI has worked with external organisations to assess of the value of public-private partnerships, i.e. TI Pharma with its conclusions published in *Nature Reviews Drug Discovery* (2012)²⁴ and IMI Bibliometric Analysis Report (2013)²⁵; IMI implemented an IT tool to facilitate tracking of projects' data and the generation of "classical" metrics has been developed and is now used for continuous monitoring.

Whilst the Panel agrees that progress has been made in this area, the Panel felt that more needs to be done to define and measure more rigorous KPIs.

²⁴ Denee, T. R. et al. (2012) *Nature Reviews Drug Discovery* 11:419

²⁵ Bibliometric analysis of ongoing projects: Innovative Medicines Initiative Joint Undertaking, prepared by Thomson Reuters on behalf of IMI JU Executive Office, Copyright IMI Executive Office, March 2013

4. Performance Assessment

Section 4 focuses on what IMI has achieved in over three years of autonomous operations, five years after its legal setup. It relates directly to the 2nd IER Terms of Reference as specified by the European Commission. The Panel's assessment of effectiveness, efficiency and quality has utilised qualitative input in combination with quantitative information available pertaining the executed calls, on-going projects and their outcomes.

The two main sources of information used to evaluate the IMI progress and current position include: interviews with IMI stakeholders and documentation made available by the European Commission and the IMI Executive Office as well as other publicly available data related to IMI.

Twenty four stakeholders were interviewed over a one month period including EFPIA and the EC, members of the IMI governing bodies, the IMI Executive Office, academic and research institutions, SMEs and their associations, patient groups, and regulators. Several of the interviewees were project coordinators from institutions representing both academia and industry. A complete list of interviewees is presented in Annex 4 to this report.

The second source of data included IMI-related documents and information consulted by the Panel, a list of which is provided in Annex 3 as well as in the References section.

From the interview responses to the set of questions referenced in Section 1.2 several important issues have emerged. These have been analysed by the Panel and are described in the following sub-sections. Three areas of strongest emphasis in the subsequent evaluation will be the Key Performance Indicators (KPIs), implementation of the communication strategy and involvement of various stakeholders beyond EFPIA. Other important issues identified by the panel included: progress in achieving the original objectives of IMI, efficiency of communication and dissemination actions, actions taken to ensure sustainability, operational efficiency of the IMI Executive Office, coherence with other initiatives, efficiency in governance, use of the resources and the call process.

In terms of research excellence, the quality of the participants (e.g. involvement of the best research groups in Europe) as well as the quality of programme design, research and scientific output have been analysed. In this section the Panel's observations originating from both sources of information are summarized. However, the information is presented as perceived by the Panel and therefore by nature reflects the Panel's views and opinions. Recommendations based on these findings are presented in Section 6.

In the following sub-sections the consolidated findings from the stakeholder interview process are presented, followed by supporting or contradicting evidence from the available documents as well as other consulted data and information.

4.1. General Comments

In the view of most interviewees and the Panel PPPs are an effective means of stimulating biomedical research and development in Europe. Overall there was a very positive perception IMI amongst the stakeholders interviewed. It emerged clearly that this PPP is seen both in Europe and globally, especially in the USA, as a model for the future.

*“It is early days but the long list of high quality scientific publications from IMI consortia demonstrates a significant contribution to health related research”.
“IMI is becoming more relevant and important”. “The USA is envious of IMI”
(key stakeholders’ comments).*

The potential for the IMI to act as a unifying voice and “one stop shop” for biomedical research and development in Europe was highlighted by several interviewed stakeholders. It has a unique role in consolidating the European pharmaceutical research base. IMI therefore can be seen as one of the key means to underpin future European competitiveness through support of the essential European competence of collaborative medical research. This will, in turn, support highly skilled jobs within research, innovation as well as downstream manufacturing jobs.

“Tools and methodologies created by IMI are starting to help to remove the bottlenecks” (key stakeholder).

There are many examples already from the various IMI projects that can support the above statements. It has already brought together over 350 EFPIA research teams, approximately 600 academic research teams and more than 100 SMEs to work together across Europe to accelerate medicines development. Patient groups and regulators are also involved in many projects. IMI is having an impact at all stages of the drug discovery and development process.

At the *in vitro* level, new screening methodologies have been developed for example in diabetes the first human pancreatic β -cell line has been created. This has resulted in a patent application and commercialization by an SME involved in the project. Preclinical models have also been developed, standardized and validated in many projects. For example, rodent touchscreen technology for cognition measurement has been validated and applied by the IMI consortia: NEWMEDS (rats) and PharmaCOG (mice). This test battery was validated across a number of industrial and academic laboratories using proven and putative pro-cognitive agents. It will allow more robust drug development for cognitive enhancers in schizophrenia and Alzheimer’s disease with easier interpretation of results from different laboratories.

In the EU-AIMS project new mutations of relevance to autism have been identified. In addition a new animal model that replicates a non-syndromic autism was developed and potential for its use in screening for new autism therapies demonstrated.

One particular area where substantial progress has been made is in the validation and development of new preclinical and clinical biomarkers for disorders as diverse as asthma, chronic pain, schizophrenia and diabetes. In diabetes, putative lipidomic and metabolomics biomarkers have been identified together with new genetic markers. In asthma a preliminary phenotype ‘handprint’ which combines molecular, histological, clinical and patient-reported data is undergoing validation and refinement.

In many projects new targets have been uncovered much more rapidly than would otherwise have been the case. In inflammation, the BT-CURE project aims to develop new diagnostic methods to discover the early forms of rheumatoid arthritis (RA) and RA-like diseases and new tools to differentiate the different forms of RA and RA-like diseases. Recently this project has identified the linkage of RA with autotaxin polymorphisms, changes in two microRNAs (microRNA-221/222 and microRNA-323-3p) and other epigenetic changes.

Major advances have also been made in the drug safety arena. SAFE-T has evaluated 153 potential biomarker candidates for drug-induced injury of the kidney, liver and vascular systems and established generic qualification strategy for new translational biomarkers; E-TOX has begun to build a toxicology information database utilising toxicology legacy reports from pharma partners to develop better *in silico* tools for toxicology prediction of new compounds (2087 reports extracted, 2904 cleared, 3643 planned in total); assembled ChOX database using public data covering 175,000 compounds annotated to > 400 targets with > 700,000 activities extracted from 10,000 publications; developed multiple *in silico* models for predictive toxicology and is building ontology for preclinical pathology, 3917 terms and 2535 synonyms have already been mapped. In terms of pharmacovigilance, the PROTECT project has already established the Drug Consumption Database in Europe using data from European National sources and IMS-Health (a health information company) data as well as the database of adverse drug reactions of centrally authorised medicinal products.

Importantly there have already been practical outcomes in terms of clinical trial design and cost effectiveness. Pooling data from 23,401 schizophrenia patients in clinical trials from multiple companies has resulted in a proposal for the reduction in the length of schizophrenia clinical trials as well as a reduction in the number of patients required to be enrolled in such trials. This is an example of both reduced time to key clinical data as well as reduced cost – the average saving per trial being 2.8M euro. Other efforts also include the piloting of new clinical trials designs and the creation of new clinical trial networks and patient registries across a number of different disease areas which will increase the attractiveness of Europe as the clinical trials research centre.

The IMI is also supporting some big data projects to enhance access to new knowledge management tools and build platforms for storage, integration and mining of information e.g. eTRIKS, openPHACTS, EMF and DDMORE. Finally it is already playing a key role in the education and training of patients, researchers and company members in pharmacovigilance, safety and clinical pharmacology.

These positive views are endorsed by the Panel and supported by several publications and presentations by the IMI Executive Office and key stakeholders.²⁶

²⁷ ²⁸ ²⁹ ³⁰

²⁶ Goldman, M. (2012) Clin Pharm Therap 91:3: 418-425.

²⁷ Gunn M et al. (2013) ATLA 40, 307–312.

²⁸ Kirby (2012) Lancet 379: 2229-2230.

²⁹ Laverty et al (2012) Expert Rev. Pharmacoecon. Outcomes Res. 12(5), 545–548

³⁰ IMI Annual Activity Report 2012 available at:

http://www.imi.europa.eu/sites/default/files/uploads/documents/IMI_AAR_2012.pdf

4.2. Is IMI Effective? – Progress towards objectives set

IMI's overall objective is to address bottlenecks in the development of safe and more effective innovative medicines by supporting research that is applicable to the majority of the pharmaceutical development organisations, in a manner that lifts overall European research performance. IMI builds on European strengths and long-term experience in collaborative research, the key factors to the competitive advantage of Europe. IMI is a public private partnership (PPP) that is unique in scale especially for its capability: i) to pool expertise to tackle most complex problems of societal and economic needs in healthcare across the development cycle of new medicines, ii) to address non-competitive unmet medical needs and market failures, iii) to attract a broad stakeholders' participation including regulators and a strong industry involvement in biomedical research. These factors make the IMI PPP the driving force for public health relevant outcomes and a front runner on the global stage in Life Sciences.

4.2.1 Progress towards the initial IMI objectives

Significant progress has been made towards the initial IMI objectives set when the JU was first established, as evidenced by:

- Making the EU the best place for pharmaceutical R&D: There was general agreement that progress has been made in this area and that the IMI contributed to halting the decline of investment in Europe from pharmaceutical companies or has even led to its increase. Its role in times of economic difficulties is seen as well timed and crucial. Figures for the period 2005-2011 show that the % of investigator sites in MAA submissions remained at a steady 35% in 2005 and 2011 – whereas the percentages for the USA were 54% in 2005 and 37% in 2011 (source EMA 2013). Clinical trial numbers in Europe also remained constant with 30B euros invested in clinical trial activities in Europe in 2012 by the pharmaceutical industry (Source Pharma etrack database).

“Europe is becoming the preferred place for pharmaceutical research (RDG member)”

- Contributing to health and socio-economic benefits for EU citizens: Due to the time scales of the pharmaceutical R&D process it is still too early to evaluate this metric. However, as quoted in the Independent Expert Panel Report³¹ accompanying the Impact Assessment of IMI (July 2013)³² and voiced during the interviews by senior level industry and EFPIA representatives, IMI has thus far generated twice as many jobs per euro spent compared to FP7 projects. IMI and its projects contributed to creating approx. 1500 new direct jobs thus far, with an

³¹ Report of the Independent Expert Panel accompanying the Commission Impact Assessment Report of IMI2 a Joint Technology Initiative under Horizon 2020, available at: ftp://ftp.cordis.europa.eu/pub/fp7/health/docs/expert-panel-report-2012_en.pdf

³² Commission Staff Working Document – Impact Assessment – accompanying the document: Proposal for a Council Regulation on the Innovative Medicines 2 Joint Undertaking, Brussels 10.7.2013 SWD(2013) 245; <http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=SWD:2013:0245:FIN:en:PDF>

average cost per job of 200 000 euro, compared to 400 000 euro per job in FP7 projects.³³

- Addressing R&D bottlenecks and stimulating new technologies and methodologies via a clearly defined strategic research agenda: Significant progress has been made in this area, with some tangible outputs, such as methodologies to reduce time and cost of schizophrenia clinical trials or the first human beta pancreatic cell line (which is now being commercialised by one of the SMEs in the consortium). IMI is seen as supporting non-competitive research that would otherwise not have been undertaken. An example is the development of tools to improve patient reported outcomes (PROs). The implementation of PROs requires collaboration of regulators, patient groups and all pharma companies. It would therefore be impossible for any individual or just a few EFPIA companies working together to have accomplished this, but it is being done efficiently within IMI. The value of synergies created by IMI participants can only be fully captured by a wide group of its stakeholders.
- Reinforcing Europe as an attractive place for pharmaceutical R&D: Both industry and academic interviewees thought that IMI has attracted as well as retained pharmaceutical industry operations in Europe. In terms of public health as well as direct and indirect socio-economic benefits, IMI impact will take much longer to be felt. However, measurable progress has already been made in terms of addressing bottlenecks in R&D.

“Call 1 & Call 2 projects seem to be delivering beyond expectation” “Europe is becoming the preferred place for pharmaceutical research” (industry member)

“New connections and added value in terms of in-kind contributions” (academic participant)

The Panel agreed that IMI has made considerable progress towards its initial objectives. Analysis of the project reviews, IMI activity reports and the positive feedback from the interviewees supported this view.

4.2.2 Involvement of other stakeholders

Progress has been made in increasing the involvement of a diverse group of stakeholders beyond the pharmaceutical industry and academic research organizations.

- Regulators: Although some stakeholders thought the role of the EMA and other regulators could be further increased, the regulators are pleased with the progress to date, as it allows them to work at arm’s length with the regulated entities (i.e. pharma companies). EMA, for example, has developed a very structured three-tier approach to its engagement in projects:

Level 1 – participating as an observer, the lightest form of engagement.

Level 2 – special method qualification, to evaluate new technologies at arm’s-length relationship. This pathway has been underutilized by the new technology

³³ See report about results of IMI project participant survey, available at: ftp://ftp.cordis.europa.eu/pub/fp7/health/docs/outcome-imi-participants_en.pdf

developers e.g. in terms of PROs they have not used the quicker pathway for tools to evaluate the outcomes.

Level 3 – direct project participation. This can only be done in purely methodological projects (i.e. post-marketing licensing project, which cannot be done without the regulator).

- SMEs: There is a satisfactory SMEs' participation in IMI. In Calls 1-8 121 SMEs participated in IMI projects and over 20% (or 157.6 million euro) of funding distributed via these IMI calls has been allocated to SMEs.

However, there are still three main areas perceived as barriers preventing a more active SME involvement in IMI projects: Intellectual Property (IP) issues, financial terms of participation and EFPIA – driven constraints. IMI output and its broader impact would likely be amplified through increasing SME participation.

As far as IP is concerned there still appear to be misconceptions about the IP policy, although the degree to which this is seen as a problem varies significantly among different national stakeholders:

“IMI IP policy is not the problem - the proposal process is the main barrier” (SME stakeholder); “SMEs see IP policy as a barrier to IMI participation” (academic).

SME representatives found the financial conditions for SMEs to be less favourable in IMI compared to other Framework Programme 7 funding schemes (i.e. FP7 Health). Currently it is proposed that this will change under the Horizon 2020 framework for IMI2. It was also felt important that IMI seeks to involve more R&D based SMEs than service providers (project management, etc.) in current (as was accomplished in the Lead Factory Project - <http://www.imi.europa.eu/content/european-lead-factory>) and future calls. There is already a positive trend in this respect, as in the first 6 calls most (77, 71%) of these SME teams are from biotech companies; of the rest, 17 (15%) are IT / data management companies, and 15 (14%) work in project management.

There has been a concern among the SME community that participation in IMI might undermine their chance of commercialization. However, it appears that IMI provides a framework that remains non-competitive for pharmaceutical companies whilst allowing SMEs to retain their background IP rights and actually enhance their competitive position. This is supported by recent examples of successful product commercialization out of an EFPIA initiated project by an SME (human pancreatic beta cell line) and a commitment by EFPIA companies to use this product and support its development beyond the IMI projects' context are positive examples that should help to increase SME engagement.

There are examples of SMEs that have been established in Europe rather than elsewhere due to the IMI incentives (e.g. in the area of safety). These cases could be captured and publicized more effectively to a broader audience. This would represent a very effective promotion of the IMI value proposition for Europe.

Several interviewees mentioned that IMI2 should consider how best to engage biotech companies that are not currently involved. Links with investment banks

and venture capital funds were suggested as the possible means to enhance SME engagement. As some valuable IP and assets may result from SME-driven large scale collaborative projects, such as The Lead Factory, engagement of private capital to stimulate its further development seems very important.

- Patient involvement: IMI has already stimulated involvement of patient groups. The need for a continued increase of patient involvement was mentioned by several interviewed stakeholders.

There still is *“a lot of room for improvement [in order] to have a broader range of patient organisations to be involved in the programme” (project participant)*.

Recently IMI commissioned a questionnaire study to assess the potential for patient involvement in the work of IMI. The first work package of the project covered the piloting and revision of a patient organisation questionnaire designed by the London School of Economics. The preliminary results derived from a limited source of interviewees were that:

- Information on R&D relevant to patient organisations is generally sourced from conferences and from within patient organisation networks;
- EUPATI appears to be the most well-known entity within IMI;
- Respondents generally feel the work of IMI is important in driving research, and is relevant to patients and families;
- Patient organisations are interested in becoming involved in the work of IMI, in particular in relation to patient training and giving input to the research agenda;
- Patient organisations would like patients to be more strongly involved in helping researchers understand clinical benefit, in the design of trials and in deciding which medical technologies should be prioritised;
- The majority of respondents had not previously participated in any pharmaceutical related research; and the perception of patient input from clinicians and scientists was perceived as a significant barrier;
- Respondents feel PPPs are a worthwhile way to spend public money;
- Respondents feel more positive about the pharmaceutical industry knowing that they engage in PPPs such as IMI.

Several interviewees stressed the need for engagement of additional stakeholders, e.g., national health funds, reimbursement advisory bodies (HTAs) and end payers of healthcare, although there were differing views on how this should be achieved.

In general the analysis of the project documents and annual IMI reviews supports the need for further increased SME engagement (Bibliometric Analysis (2013),³⁴ Participants Questionnaire.³⁵ Although patients were engaged in many projects there appeared to be room for enhancing their participation.

³⁴ Bibliometric analysis of ongoing projects: Innovative Medicines Initiative Joint Undertaking, prepared by Thomson Reuters on behalf of IMI JU Executive Office, Copyright IMI Executive Office, March 2013

³⁵ Report on the outcome of the Innovative Medicines Initiative project Participants' Questionnaire, 2012, available at: http://www.imi.europa.eu/content/documents#activity_reports

The panel concludes that IMI has proven to be an effective means for stimulating pharmaceutical R&D in Europe and has already delivered tangible scientific and socio-economic benefits.

4.3. Is IMI Efficient?

In considering the efficient use of resources, several aspects were analysed, including: robustness of the monitoring system - the KPIs, the governance structure and processes, communication strategies and the use of funding (including dissemination and uptake of research outcomes).

4.3.1 Key Performance Indicators (KPIs)

The Governing Board (EC and EFPIA alike) recognized the KPI definition needed sharpening. The GB has requested the IMI Executive Office to make the KPIs more tangible, measureable and reflective of the IMI overall objectives. KPIs at both the project level and the overall IMI level need to be measured. Selected KPIs should be common among all projects and facilitate aggregate analysis for the entire IMI. There is a need for continuous monitoring as well as forecasting and foresight of global technological, economic, and societal developments in order to be able to adapt the implementation plans and also to support pro-active measures. Furthermore, there is a need for identifying robust socio-economic indicators.

Current KPIs focus on the IMI performance as a newly built organisation (visibility, citations/publications etc.) but are insufficient to quantify the added-value and the impact of individual measures undertaken and research activities driven by the IMI programmes as well as its broader socio-economic impact. KPIs within the individual consortia projects are not sufficiently communicated outside.

In addition, it appeared that there are thus far un-quantified positive impacts of participating in an IMI consortium. Quantifiable impacts could include increased access to research networks in academia and industry; access by academics to industry tools, know-how and facilities and the initiation of new collaborations outside of IMI between project participants. It would be beneficial to implement their identification and monitoring. As IMI has now matured and reached a consolidation phase it seems appropriate to devote more resources to quantify its socio-economic impact.

The evidence and opinions of the stakeholders support the conclusion that the definition and way of presenting the KPIs does not sufficiently encompass the “value proposition of IMI”. A new set of KPIs has been recently developed by the IMI Executive Office, which broadens the performance metrics beyond the scientific outputs. These have been assessed but not yet approved by the GB.

The scientific metrics (publications, networks of academics, etc.) have been collected³⁶ and are quite convincing. These indicators, however, do not address the downstream macro-economic impacts or the general IMI objectives. The Panel is

³⁶ Bibliometric analysis of ongoing projects: Innovative Medicines Initiative Joint Undertaking, prepared by Thomson Reuters on behalf of IMI JU Executive Office, Copyright IMI Executive Office, March 2013

aware that it is difficult to calculate a return on investment (ROI) from R&D in simple terms. Nonetheless, a convincing long-term strategy and system are needed to better evaluate the overall IMI impact on the biopharma industry in Europe, on the healthcare system and on the European economy. The newly proposed set of KPIs does not yet appear to address this issue.

During the next period, the development and monitoring of a set of KPIs to provide greater impact assessment will need the GB's sustained attention.

4.3.2 Communication and Dissemination

Communication has been seen by the GB as a key issue needing more attention. They recognized, and this was confirmed by interviewees, that communication needs to be more tailored e.g., better targeted communication channels for SMEs and other stakeholder groups are required.

There has been an attempt to address these issues through the development of a recent communication strategy for the period 2013-2014 which was prepared in the second quarter of 2013. The document, however, was made available to the Panel at a later stage of the interim evaluation and most of the interviewed stakeholders were also not aware of it. This document represents a solid basis for the future Communication Plan. It has three overarching goals:

- **Goal 1:** To increase proposals from the very best candidates and consortia
- **Goal 2:** To awareness levels and perception of IMI amongst all target groups
- **Goal 3:** To reach patients in order to involve the 'authentic' patient voice in the drug development process.

Most interviewees thought that IMI lacked visibility within the scientific community while it was also reported that perceptions of IMI in the USA were considered to be more favourable than in the EU.

“There is not enough mainstream publicity.” (academic)

Communication has focused mainly on communicating research results at conferences and in scientific publications – other aspects of IMI need to be communicated more broadly. The Panel heard evidence that more structured outreach activities and that more promotion is needed in non-scientific media such as economic and financial news and periodicals. Also, apparently some interviewees felt that more could be done to enhance communication at the national level in non-English speaking countries. Moreover, it was suggested that the dissemination activities also need to address decision makers in individual Member States.

“We don't see enough about a project funding or outcomes on the news.” (SME representative)

“Even small breakthroughs or successes, when they happen in the US, find their way into mainstream media and are highly promoted. In Europe often a major break-through or achievement can only be found in scientific literature.” (GB member)

The Panel noted that there had been a real effort on the part of the IMI Executive Office to communicate call topics and disseminate the IMI initiatives via publications in scientific journals. However, the Panel felt that, as there have been more successes stories coming out of individual projects, these could form the basis for intensified dissemination targeted to a broader range of stakeholders, including policymakers within the Member States.

Having reached maturity, the IMI Communication Strategy needs to move beyond disseminating research results. The most recent communication strategy document provides a good framework for informing stakeholders about the broader socio-economic impacts. This includes not only patients but also policy and decision makers at the European and national levels, making these target groups aware of “what’s in it for them”. This would aid the development of a clear long-term policy framework, creating a favourable and stable environment to attract more investment in research and clinical trials in Europe. It will be important to ensure that there a detailed implementation plan with clear objectives is implemented based on this strategy.

4.3.3 Sustainability beyond the lifetime of the projects

Sustainability issues had not been considered early enough during the first few calls, especially with regard to infrastructure as well as data storage and management. In recent calls it has become an important issue. A new mechanism has been put in place – the exploitation of new scientific opportunities (ENSO) for projects delivering beyond expectations, which allows project consortia to receive additional funding to assure continuity e.g., developing an option to continue as a non-profit company.

An effort to sustain IMI at the programme level is reflected in a Commission Proposal for a Council Regulation on the Innovative Medicines Initiative 2 (IMI2) Joint Undertaking (JU)³⁷. This will leverage the past investments by the Commission and industry under the current IMI JU and capitalize the knowledge platforms and infrastructures it has established.

The Panel supports the Commission effort to assure IMI sustainability through a Council Regulation for Horizon 2020. At the same time sustainability issues related to data archiving and access as well as sample storage, curation, and mining beyond the duration of individual projects remains to be solved in a systematic manner.

4.3.4 The IMI Executive Office

The interviewed stakeholders provided views on the efficiency of the IMI Executive Office ranging from “highly efficient” to “certain areas highlighted for improvement”. Undoubtedly significant progress has been made since establishing the office and administration of the programme. In general, it was felt that the balance of skills might not yet be optimal in the office in light of current and future tasks, and certain

³⁷ Proposal for a COUNCIL REGULATION on the Innovative Medicines Initiative 2 Joint Undertaking <http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=COM:2013:0495:FIN:EN:PDF>

key skills and capabilities will need to be added or developed. Another criticism voiced by some of the interviewees (*i.e.* project coordinators) was that there was too little cross-project exchange of information.

The Panel understands that there is an authorised maximum ceiling of 36 staff members reached in July 2012, but the direct management of the research programme comprising 60 projects is carried out by 9 scientific managers, which may represent a too heavy burden for them, given the complexity of the process and the level of the work. The Panel noted that the administrative personnel outnumbered the scientific personnel, which may leave some room for optimising the human resources (HR) structure (without increasing the number of IMI Executive Office staff). Also some interviewees regarded the allocation of staff between project management and general administration as unbalanced. The high overheads are partly explained by the small size of the organisation and there may be a need for autonomous and common services in administration, legal affairs, human resources, accountancy, information technology, auditing and procurement. Considering that three JUs are sharing the same premises one should expect that significant savings can be realised by sharing a number of horizontal services.

“Partner satisfaction was probed by the Board among consortium partners and their response was positive”.

Some shortcomings were identified in the HR strategy (or lack of such). It was noted that the personnel selection process of assigning officers to projects was on first-come / first served basis rather than using a thematic approach, where one person covered several thematically related projects.

The Panel thought that the IMI Executive Office had made significant progress in the above areas but agreed with the experience drawn from comments of the interviewees that the organization of the IMI office and the Human Resources balance could be further improved.

4.3.5 Coherence with other initiatives

It was apparent that there was little coordination with initiatives at the national and/or regional level, as voiced by some interviewees, and a lot could be improved in this area. Additional economic benefits could be derived at the regional level if opportunities to build on the IMI platform were identified and supported. The collaboration with the Dutch PPP TI Pharma was noted as a good example.

Overlap with other FP7 Thematic Priorities was not identified as an issue.

4.3.6 Resource allocation by EFPIA

Resources within the companies appear to be adequate for the most part, although reorganisation within companies had led to changes of personnel on specific projects. There had been a couple of instances, where changing priorities had meant that companies had to withdraw from a project, but solutions had been found to enable the industry contributions to be maintained. Some companies are not involved at all in IMI although others are engaged in several projects. The operational resources

committed vary tremendously among EFPIA companies in terms of both: amount and quality.

There was also a perception voiced by some of the stakeholders interviewed that there was a lack of transparency concerning the in kind contribution from companies. This may be an issue of common misperception. The Panel was satisfied with the evidence from the industry and Commission interviews and was convinced that there were no unclear issues around the in-kind resources. This was supported by the fact that the in kind had been audited at several companies and no discrepancies were found. This information should be communicated to prevent the perception of possibly inflated in-kind contributions by the pharma companies, which may have a detrimental effect on the overall IMI image.

In general, the budgetary and financial model restrictions seem to be too narrow for the breadth of the objectives. Different possibilities of involving (national) funds, venture capital and stronger financial engagement of non-EFPIA resources need to be explored. Mechanisms such as cash contribution of EFPIA companies, ability to involve in-kind contributions from outside Europe in justified cases and potential contributions (both cash and in-kind) from non-EFPIA companies have already been proposed and are being evaluated. The Panel supports increasing the flexibility of the co-financing model in all the above aspects.

4.3.7 Call process

There is a wide agreement that the efficiency of the call process has improved dramatically in terms of administrative efficiency and time-to-grant which experienced a reduction from 400 days in call 3 to 185 days in call 6.

Most interviewees were happy or had no issue with the two-stage call process but some recognized that the two stage procedure could lead to spreading the best groups in specific areas across several consortia – especially true for key bodies like EMA, which many consortia might need to include to be successful.

Various stakeholders suggested alternative and more flexible solutions that would allow:

- testing different procedures (i.e., single stage call);
- merging parts of several consortia in second stage;
- considering different models, if and where appropriate.

There was also a general agreement that implementing some of the above flexibilities in handling the calls could be a better solution.

Many interviewees evaluated call topic descriptions as too detailed and prescriptive but at the same time not always completely describing what companies want “[calls] often read more like a call for tender rather than 75% funded collaborative projects” (SME representative).

It was not clear to many interviewees how non-EFPIA stakeholders can influence or have input to call topics.

“An IMI project is more complex but offers practical advantages – when it works well the collaboration with companies is really leveraged.”(key stakeholder)

“two-call procedure started as a very cumbersome mode. This process has improved – time has been reduced and flexibility is greater.” (key stakeholder)

4.3.8 Governance

The Governing Board (GB): It was clear from the responses that the Governing Board is working well and that the 50:50 representation of the Commission and EFPIA is effective.

With reference to Recommendation 5 of the first IER (*reinforce and streamline decision making and well-functioning processes*), the GB still feels that the actions taken have not fully addressed this issue. As well as that clarification of the roles of the IMI governing and advisory bodies will need further elaboration.

The Scientific Committee (SC): Currently, the SC would like to be more involved in the procedure of drafting and updating the SRA, as it seems its role has been reduced since the revision of the IMI SRA in 2010-2011, which was driven by the SC and then approved by the RDG.

The composition and skill set / experience balance of the SC in the future was addressed by many interviewees, with pros and cons mentioned for greater involvement of other stakeholders such as EMA (who are currently an observer on the SC). Getting the “right” members to proactively participate and attend the SC meetings is felt important and there were views claiming that this may need to be incentivized.

Based on a number of interviewee responses, there seems to be ambiguity and a lack of agreement about the future role of SC in IMI2. This role needs to be carefully considered so as to ensure high quality individuals are willing to be involved and its contribution valued.

Members of the SC should be pro-actively involved, especially in the review process. The Panel is aware that equivalent bodies to the SC in other JUs appear to be better integrated. For example in the Clean Sky JU, the Members of the STAB are active as reviewers at the annual and various other reviews throughout the year and at the request of the Executive Director have produced since 2012 a synthesis of the annual reviews outcomes. The members of the Clean Sky STAB have also working groups on socio-economic implications, review the deliverables and have just developed a matrix with various criteria addressing innovation, environment, competitiveness, etc.

The States Representative Group (SRG): The SRG acts as advisory group and represents the interface with the relevant stakeholders in their respective countries. It is envisioned to have an important role in liaising with the national programmes and helping in dissemination and outreach activities. In such capacity, the SRG could act as an ambassador and a communication conduit for the IMI. Efforts in this area, up to now, appear to have been rather modest although there is lack of agreement among the interviewees as to the underlying reasons. It was also remarked that the membership might not be appropriately configured for the mandate of the group.

“SRG would like to have more impact” (GB member)

There appears to be a need to strengthen the synergies between IMI and national initiatives – SRG has the potential to be more instrumental in that process, providing presence of the right participants. This is important as 95% of biotech and pharma research comes from national or regional sources.

“It should be the responsibility of SRG as well as CEOs of large companies with headquarters located in a given European country to try to align national priorities with EFPIA / IMI priorities to solve the burning healthcare problems. IMI should be an integral part of a broader multi-national ecosystem.” (EFPIA, GB members)

The Panel reviewed the governance documents regarding IMI and in general found the roles of the committees adequately described. However, it was evident from interviewees’ comments that there was a lack of clarity and agreement over the current and future roles of the SC and SRG. Reaching clarity and a common view in this area is important, especially considering that additional constructive buy-in from MS is likely to benefit EU-wide research efforts through more efficient resources allocation.

Overall the panel believes that the IMI governance is efficient in the management of the programme and delivery of projects and calls. However, steps for enhancing communication and dissemination as well as developing better performance metrics are still required.

The mandates and roles of the SC and SRG members should be leveraged to their full potential in order to optimise their contribution to IMI.

4.4. Is IMI Research of a High Quality?

4.4.1 Quality of research and scientific output

Generally, most of the respondents were impressed about the quality of the IMI's scientific and research output, which has exceeded expectations. In the view of one GB member "*Significant progress has been achieved to bringing new medicines and diagnostics to the patient.*"

"A long list of high quality scientific publications from IMI consortia demonstrates a significant contribution to health related research" (SRG member).

Despite some criticism from interviewees of the quality of topics for calls for proposals, and the process of their generation, the scientific output of IMI to-date leaves little doubt of its scientific excellence.

To date, IMI projects have produced 320 publications – over one-third of which have been published in the last six months – appearing in more than 150 journals including *Nature, JAMA and Science*.

The volume of IMI research output has also increased at the level of individual projects. EUROPAIN and NEWMEDS have been the most prolific projects funded in Call 1 while output from BTCure has increased rapidly in the last six months and is now in line with EUROPAIN and NEWMEDS. Of the 30 projects funded in Calls 1, 2 and 3, all but three (ABIRISK, EUPATI and PreDiCT-TB, which were Call 3 projects and were only initiated in 2012) have published once to date.

Examples of where this has already impacted the drug discovery and development process have already been given in section 4.1 and the impact across the R&D spectrum of projects is shown in Figure 4.1.

Furthermore, more recently IMI projects have been launched that are likely to yield high quality research results but due to the early stage they have not yet had time to deliver on this promise. This is firstly due to the time it takes to practically get a new project going and secondly to the delay from starting a project to delivering research results and measureable outcomes that is inherent in biomedical research.

IMI project research is wide-ranging – the research portfolio from IMI projects covers a wide range of research fields and this diversity has increased in the last six months.

IMI project research is well perceived – the quality of IMI project research (as indexed by citation impact) has not only been maintained, but has increased while output has grown. The average citation impact of IMI research is well above world and European averages and over twice the world average for specific research fields. Furthermore, over one-tenth of papers from IMI projects are ‘highly-cited’, that is, they belong to the world’s top ten per cent of papers in that journal category and year of publication, when ranked by number of citations received.

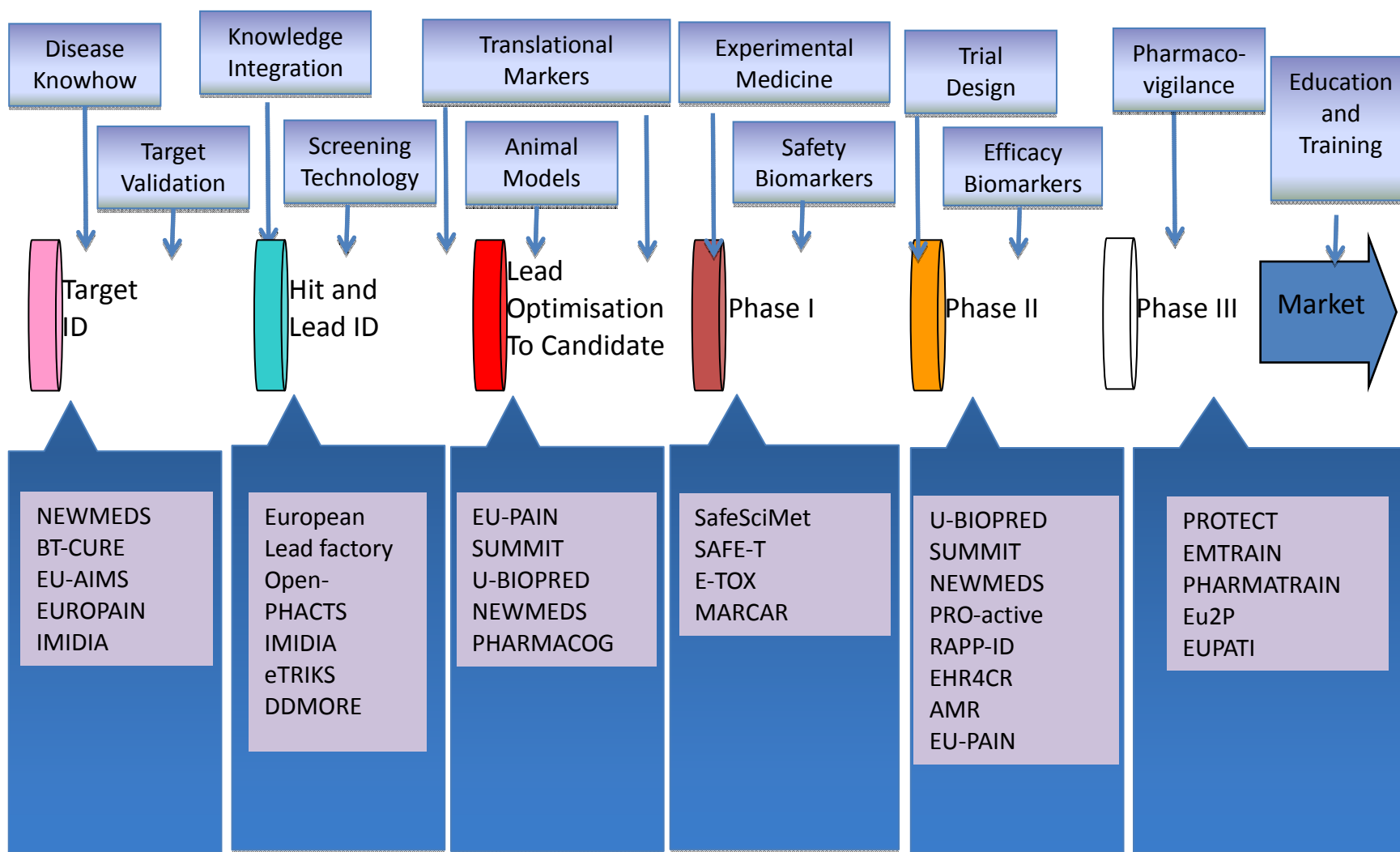
Researchers funded by IMI are well-regarded by their peers and also highly collaborative. About two-fifths of all publications by IMI researchers were cross-sector, for example, between academic institutions and small medium enterprises (SMEs)³⁸.

“Scientific state-of-the-art level is quite cutting edge. Some projects stand out. The Autism project is really transformative and delivering the highest level of science. Validating animal models. Identifying new targets & biomarkers. Making real, measureable progress towards better interventions.” (senior level industry representative)

“Process for formulating new proposals, especially early communication of ideas of calls to professional organisations from EFPIA & IMI could be very beneficial and result in their in higher quality” (Academic project coordinator)

³⁸ Bibliometric analysis of ongoing projects: Innovative Medicines Initiative Joint Undertaking, prepared by Thomson Reuters on behalf of IMI JU Executive Office, Copyright IMI Executive Office, March 2013

Figure 4.1 Breakdown of all IMI projects based on their point of impact in the R&D process



4.4.2 Possibilities for improving the involvement of leading research organisations

On the academic side:

“The very productive publication rate (in the highest ranked scientific journals) of the early consortia speaks to the high quality of the researchers. Researchers and research organisations are attracted, which fit best/provide the most suitable technology/means as partner to achieve the proposed project goals. Whether these are the best in their field cannot be judged.” (academic)

Call participation

A few interviewees raised concerns about the strategy of ambitious leaders to engage all potential competitors and everybody else in “their consortium”. This activity reduces the number of plausible competing bids. In the end this results in not necessarily the best consortia.

The size of the consortia was raised by a couple of participants as an issue although others found the consortia size was appropriate and manageable. In future calls, flexibility of participation might be considered *e.g.* for SMEs to join at a later date or the addition of key public bodies such as EMA, even if they were not originally part of the winning consortium.

On the Industry side:

Some representatives of EFPIA companies in agreement with the GB and the EC raised concerns that in some specific research areas, involvement of industrial researchers and laboratories is limited due to restrictions on the source of in-kind contribution – thus reducing the quality of contribution to specific projects or research areas within IMI. This most commonly occurred where the company’s research was located mostly or exclusively outside the EU, as may be the case for global companies).

There are a number of mid-size pharma companies within Europe that are currently not EFPIA members. It seems important to find a mechanism that would also allow them to participate in IMI.

The Panel was convinced that IMI overall scientific output to date had been of excellent quality.

The Panel agreed that as long as the investments are undertaken within the EU and correspond to the objectives of IMI, there should be no restriction as to the contribution source. This would allow IMI to access researchers from companies with optimal expertise.

5. SWOT Analysis

The SWOT analysis was used as an exercise to place the evaluation in a broader context and to help the Panel build its conclusions and formulate recommendations.

STRENGTHS	WEAKNESSES
<ul style="list-style-type: none"> ● Recognized as a world-leading PPP in healthcare, particularly in the US ● Unique collaboration model to address non-competitive unmet medical needs (addressing market failure) ● A catalyst for private sector investment in European biopharmaceutical R&D ● High quality of scientific output and vibrant networks of academia, SMEs and industry ● Increased the level of trust among many relevant stakeholder groups including regulators ● A critical mass of expertise to tackle the most complex problems of healthcare needs along the entire R&D cycle ● Mobilised resources reinforced by synergies across a broad range of stakeholders ● Industry led initiative with strong support from the CEOs of EFPIA companies and a focus on tangible outcomes 	<ul style="list-style-type: none"> ● Lack of clear, targeted communication strategy; low visibility ● KPIs not mature enough to demonstrate broader socio-economic impact ● Insufficient incentives for SME and non-EFPIA member participation ● Processes and regulations still too bureaucratic; ● Advisory bodies not functioning to their full potential; ● Lack of buy-in by MS leading to lack of alignment with MS policies and strategies; ● Inadequate balance between scientific and administrative tasks of the IMI Executive Office, suggesting a need for new skills ● Not all EFPIA companies involved ● Lack of planning for project sustainability
OPPORTUNITIES	THREATS
<ul style="list-style-type: none"> ● Increasing focus on meeting health challenges of the ageing population with high socio-economic impact ● Building on, and learning from IMI as a proven model to catalyse stakeholder engagement e.g., patients, regulators ● Maximising potential for IMI as a platform for building a common vision e.g. for health policy ● Increasing scope and attracting non-EU investment for biomedical R&D ● Leveraging other potential funding options e.g. via venture capital and/or EIB loans ● Further improvement of the biopharmaceutical R&D environment via removing bottlenecks or improving processes e.g. for clinical trials ● Exploring potential to involve other sectors and stakeholders e.g., payers, HTAs ● Developing new funding models to explore results and increase sustainability 	<ul style="list-style-type: none"> ● Decrease of political support for IMI ● Disrupted balance between pre-competitive and competitive boundaries ● Lack of coordination with national initiatives leading to inefficient use of resources ● Competition from other PPPs worldwide leading to decrease of interest by companies ● Growing regulatory burden and tightening of pricing and reimbursement schemes ● Loss of key personnel from IMI ● Economic slowdown leading to lack of funding ● A negative perception among key stakeholder groups (patients, payers, regulators) ● Losing the competitive advantage to new emerging economies (i.e. China, Brazil) ● Deteriorating reputation and diminished support in the EU as a result of non-performance

6. Achievements, recommendations and plans for the future

Section 6 summarizes i) the progress, improvements made as well as positive changes and outcomes; ii) areas that still need improvement in the short and long term; iii) forward looking statements (working towards IMI2) and puts forward recommendations.

6.1. IMI achievements

IMI has demonstrated the feasibility of large, multi-stakeholder PPPs for research and development in biomedicine. It has become recognized as a world-leading PPP in healthcare especially in the US where regulators in particular see it as an important new model (J. Woodcock, 2010)³⁹. This unique model of funding and interaction between the pharmaceutical industry and key stakeholders has proven effective and efficient in delivering projects relevant to healthcare challenges and in building trust between participants. Specifically it has:

- brought together key stakeholders thus achieving a critical mass to tackle really big and complex issues in healthcare needs across the whole R&D cycle. It has created an effective dialogue between industry and research around a common strategic agenda and has successfully executed it;
- halted the decline in private sector investment in European biopharmaceutical R&D and in fact there has been an increase in development investment in the EU over the past two years (EFPIA data reference). Thus the IMI is already showing an impact on the competitiveness of the industry within Europe;
- implemented an adequate governance structure. Bringing research and industry closer together has enhanced the impact of EU funding at the same time responding to societal challenges;
- continued to provide call topics, and subsequently projects, that address major bottlenecks in pharmaceutical R&D thereby helping to further the health of European citizens;
- provided high quality of scientific output as measured by bibliometrics and also created valuable networks of academia, SMEs and industry experts in particular disease areas. These networks will provide a valuable resource going forward for attracting further development resources and investment;
- facilitated significant involvement of regulators, such as EMA, and of patients and patient associations in projects, which has contributed to a better understanding among various participants of the problems and constraints in drug discovery and development;
- made demonstrable progress towards more rapid identification and validation of new outcome measures such as biomarkers and PROs. This will speed the development of new medicines within Europe as well as open routes to commercialisation for SMEs involved in providing tools for assessing such outcome measures;
- secured large pharma industry's commitment to create a common European biopharmaceutical research and development base through open innovation to deliver future value for both industry and healthcare systems.

In light of the above, the Panel reinforces the recommendations of the Expert Panel for the Impact Assessment Report⁴⁰ that the IMI JU should be continued under Horizon 2020 with enlarged scope.

³⁹ Woodcock, J. (2010), *Clinical Pharmacology & Therapeutics*, 87(5): 521.

⁴⁰ Report of the Independent Expert Panel accompanying the Commission Impact Assessment Report of IMI2 a Joint Technology Initiative under Horizon 2020, available at: ftp://ftp.cordis.europa.eu/pub/fp7/health/docs/expert-panel-report-2012_en.pdf

6.2. Recommendations for further improvement of IMI

The Panel formulated a series of recommendations for action from its observations. These should help IMI to build on its strengths, address weaknesses, minimise the impact of potential threats and turn them into opportunities. Some of the recommendations may guide IMI towards resource consolidation supporting its sustainable future. Others may serve as a stimulus for turning the arising opportunities into concrete achievements.

The bodies who should take responsibility for implementing each recommendation are highlighted in **bold**.

Recommendation 1: IMI needs to finalize and implement an articulated communication strategy with clear and measurable goals and objectives, addressing both the key stakeholders and a wider audience.

Whilst the IMI has been effective at communicating call topics to potential participants, now is the time, as success stories are increasing, for a concerted, broader communication effort. This effort should include:

- 1.1 Finalizing and implementing the newly developed IMI Communication Strategy, linked to the vision of IMI, in order to demonstrate that IMI is delivering value to its stakeholders;
- 1.2 Aligning the Communication and Dissemination Plan with the newly developed Communication Strategy, focusing on clear specific objectives enhancing IMI wider visibility and increasing the dialogue with stakeholders and patient organisations;
- 1.3 Developing clear, measurable targets in the awareness levels and support for IMI among various stakeholder groups;
- 1.4 Formulating and delivering the value proposition (“what’s in it for them”) to the Member State decision and policy makers to assure buy-in and achieve synergy of IMI and IMI2 with the national and other Horizon 2020 programmes in biopharmaceutical research;
- 1.5 Promoting the outstanding scientific achievements, best practice and benefits of IMI to improve the overall awareness and support for IMI among the broad scientific community, especially in Member States with low participation levels;
- 1.6 Improving the communication channels to reach the general public and other important stakeholder groups (i.e. patient organizations, payers and regulators) in order to increase IMI visibility and positive image generating public support.

The above activities should lead to stronger support for IMI and recognition of its achievements and positive socio-economic impact for Europe.

**The Governing Board (1.1-1.2) and
The IMI Executive Office (1.3-1.6)**

Recommendation 2: Alongside the existing KPIs, aggregated KPIs need to be developed and measured in order to quantitatively demonstrate the IMI impacts and socio-economic benefits.

Whilst KPIs have been developed, they have focussed primarily on very concrete scientific output. The socio-economic potential which by definition is less concrete and more abstract has not been sufficiently well captured. The fact that IMI has helped to create over 1500 direct new jobs, for example, is commendable and more metrics of that kind need to be in place.

In order to achieve this, the following actions are required:

- 2.1 Aggregated KPIs need to be defined, compared to the baseline scenario (with IMI vis-à-vis without IMI). Examples could be:
 - 2.1.1 Industry cost savings following IMI, implied by e.g. development of biomarkers leading to a shortening of related clinical trials and on a higher level (new medicines faster);
 - 2.1.2 The extent to which RDI expenditures (in reality they are “investments” in EU's future competitiveness and growth) have increased/or been sustained within the EU during the existence of IMI (additionality);
 - 2.1.3 The extent to which highly skilled jobs / SME jobs have been sustained/created due to IMI;
 - 2.1.4 Perceived attractiveness of the EU as a location for FDI and location of research facilities for pharma and other high-tech sectors.

In sum, calculation of an economic rate of return (ERR or ROI as a proxy) should be performed to attempt capturing the value for money and opportunity cost of IMI. As such these measures should provide an indication of IMI's contribution to the EU objectives of sustaining/increasing EU R&D-intensity, in turn supporting future EU jobs, growth and competitiveness.

- 2.2 Where baseline data on these does not exist, it needs to be captured for future benchmarking of progress.
- 2.3 Aggregate KPIs should be tracked regularly and communicated broadly beyond the scientific & research communities.

These impact indicators will allow longer term benefits of IMI to be better captured justifying further public investments in public private partnerships in biomedical research.

IMI Executive Office – implementation
EFPIA and EU– providing data & information
as well as baseline figures

Recommendation 3: IMI should make an additional effort to increase engagement from a wider range of industry stakeholders.

Building on the success of previous efforts to involve SMEs in IMI projects, which as indicated are already above other EU-funded health research programmes, there is still room for greater SME engagement. The Panel also received numerous suggestions that IMI could benefit from participation of smaller pharmaceutical companies that do not have an SME status and are not EFPIA members. In addition there is scope for greater EFPIA engagement, especially where expertise resides outside the EU.

- 3.1 In order to incentivise even greater engagement of SMEs the following actions should be taken: a) clarification and targeted explicit messages with regard to the IP policy; b) success stories, where SMEs have really gained from their participation in IMI projects, need to be more effectively promoted; c) consideration should be given to more flexible ways (i.e. shorter or task - based involvement) of involving of R&D conducting SMEs.
- 3.2 If feasible, in the upcoming calls IMI should find a way to enable involvement of smaller pharmaceutical companies in IMI projects.
- 3.3 IMI should use the possibility to include expertise outside the EU as in-kind contribution to increase the involvement of non-participating or low participating EFPIA companies.

Through these means, stimulating broader engagement of industry stakeholders in IMI activities, IMI would build a stronger base for future PPPs in biomedical research.

**IMI Executive Office
EFPIA (3.3)**

Recommendation 4: The IMI Executive Office should seek further ways of reducing bureaucracy and ensure that it has the optimal organizational structure for the tasks ahead.

Whilst the IMI Executive Office has made significant progress in speeding up processes and reaching operational efficiency, the Panel felt that some further adjustments might be needed to improve efficiency. Now, that IMI is well established, the balance of skills (between administration and S&T tasks) in the Executive Office may need some adjustments to ensure that projects deliver and the benefits are fully realised.

- 4.1 The Panel recommends that IMI examines which horizontal administrative functions could be shared with other JUs located on the same premises.
- 4.2 IMI should identify the main skill or competence gaps in the IMI Executive Office and investigate possible actions to fill these gaps or develop / add the missing skills.

These actions should lead to a better balance between administrative and scientific staff in the IMI Executive Office and allow it to provide better service to IMI projects.

IMI Executive Office

Recommendation 5: IMI should seek to maximize the potential of its advisory bodies to gain support for the remaining calls and other activities at all levels.

The SC and SRG mandates are clear within the governance structure but their current configuration may not have been the most appropriate for these mandates.

- 5.1 The Governing Board should stimulate greater and more pro-active involvement of the Scientific Committee (SC) members, jointly with other external experts, in project monitoring & review process in order to leverage their full potential (as practiced in other JUs).
- 5.2 In light of the above, the GB should examine whether any adjustments to the membership of the SC should be made to have the required expertise.
- 5.3 The EC is encouraged to utilize opportunities from cross-comparison of JUs with respect to identifying areas of best practice with respect to SRG and SC operational functions.
- 5.4 The SRG should be actively engaged to act as ambassadors for IMI in their respective MS in order to maximize the reach and participation in the upcoming IMI calls.

The above actions could provide vehicles for more active participation of the SC and the SRG in promoting and advocating the IMI success.

**Governing Board (5.1 and 5.2)
EC (5.3) SRG (5.4)**

Recommendation 6: IMI needs to plan for and design new and more flexible funding mechanisms to ensure the sustainability of current and future projects, where appropriate.

IMI has recognised this need and has already put in place one funding mechanism (ENSO) to aid project sustainability but more mechanisms need to be explored.

IMI Executive Office

6.3. Recommendations for the future IMI2

It is clear from the current review and the Commission Impact Assessment Report⁴¹, the accompanying Report of the Independent Expert Panel⁴² as well as the EFPIA's outlook towards IMI2⁴³ that the IMI is working well and should be continued and expanded under Horizon 2020.

There is an envisioned change in the financial rules under Horizon 2020 that will allow alignment of IMI funding with other research programmes under Horizon 2020 in terms of indirect cost and general participation rules. This simplification should also help to further encourage SME participation.

The Panel supports both of these initiatives and in light of these has some further recommendations for the future IMI2:

Recommendation 7.1: Baseline data should be obtained in parallel with the launch of IMI2 in order to allow for better benchmarking and assessment of IMI2 performance.

The Panel feels that a study of the Pharmaceutical Industry in Europe should be undertaken by the Commission and EFPIA in parallel with the launch of IMI2 and compared to the 2007 study that was used as a baseline for the current IMI JU. This study would provide an overview of the high-level impacts / developments and serve as an updated / interim baseline for the IMI2 initiative under the Horizon 2020.

European Commission

Recommendation 7.2 Industrial participants from other healthcare related sectors should be involved in IMI2.

It is clear that an integrated approach to healthcare will be required including prevention and diagnosis. The Panel therefore recommends that every effort should be made to involve other industrial participants from these sectors in IMI2.

Governing Board

Recommendation 7.3: The Commission should ensure that IMI2 is transparent and has increased flexibility in terms of governance.

In IMI2 it should be ensured that the roles and mandates of the governance and advisory bodies (in particular the SC and the SRG) are clearly defined and the membership configured with the appropriate expertise to execute their mandates. The lessons learned from both IMI and other JUs should be incorporated into the revised governance structure of IMI2.

European Commission and the Governing Board

⁴¹ Commission Staff Working Document – Impact Assessment – accompanying the document: Proposal for a Council Regulation on the Innovative Medicines 2 Joint Undertaking, Brussels 10.7.2013 SWD(2013) 245; <http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=SWD:2013:0245:FIN:en:PDF>

⁴² Report of the Independent Expert Panel accompanying the Commission Impact Assessment Report of IMI2 a Joint Technology Initiative under Horizon 2020, available at:

ftp://ftp.cordis.europa.eu/pub/fp7/health/docs/expert-panel-report-2012_en.pdf

⁴³ <http://efpia.eu/topics/innovation/innovative-medicines-initiative/innovative-medicines-initiative-2>

7. Summary and Conclusions

IMI has demonstrated the feasibility of large, multi-stakeholder PPPs for research and development in biomedicine. It has become recognized as a world-leading PPP in healthcare. This unique model of funding and interaction between the pharmaceutical industry and other key stakeholders has proven to be effective and efficient in delivering projects of relevance to healthcare challenges and building trust between participants. Specifically:

- on-going IMI projects have already demonstrated scientific excellence,
- IMI-funded projects are effectively addressing key challenges and barriers in the field of biomedical research and development,
- IMI's operational implementation and efficiency has significantly improved over the past years.

Although IMI has clearly become a success, several areas of its activities could be further improved. Based on information and evidence from available data and a series of key stakeholder interviews, the Panel formulated the following set of recommendations that can help IMI maintain its scientific excellence, further increase its impact and meet its overarching objectives.

Recommendation 1: IMI needs to finalize and implement an articulated communication strategy with clear and measurable goals and objectives, addressing both the key stakeholders and a wider audience.

Recommendation 2: Alongside the existing KPIs, aggregated KPIs need to be developed and measured in order to quantitatively demonstrate the IMI impacts and socio-economic benefits.

Recommendation 3: IMI should make an additional effort to increase engagement from a wider range of industry stakeholders.

Recommendation 4: The IMI Executive Office should seek further ways of reducing bureaucracy and ensure that it has the optimal organizational structure for the tasks ahead.

Recommendation 5: IMI should seek to maximize the potential of its advisory bodies to gain support for the remaining calls and other activities at all levels.

Recommendation 6: IMI needs to plan for and design new and more flexible funding mechanisms to ensure the sustainability of current and future projects, where appropriate.

The Panel also formulated recommendations for the future IMI2:

Recommendation 7.1: Baseline data should be obtained in parallel with the launch of IMI2 in order to allow for better benchmarking and assessment of IMI2 performance.

Recommendation 7.2: Industrial participants from other healthcare related sectors should be involved in IMI2.

Recommendation 7.3: The Commission should ensure that IMI2 is transparent and has increased flexibility in terms of governance.

The IMI JU provides a sound basis for IMI2 to build upon the many lessons learned, further improve efficiency and streamline governance as well as increase its scope in order to even more effectively address the societal challenges of health, demographic change and wellbeing of Europe.

Annexes

Annex 1 Composition of the Expert Evaluation Panel

Jackie Hunter	(Chair)	OI Pharma Partners Ltd.
Marcin Szumowski	(Rapporteur)	BTM Mazovia, OncoArendi Therapeutics
Tom Andersen		European Investment Bank
Maria Rosaria Di Nucci	(Common Expert)	Freie Universität Berlin
Bart Wijnberg		formerly Ministry of Health, Welfare and Sport, Netherlands

Jackie Hunter (Chair) (UK) CEO of OI Pharma Partners Ltd. Her company has helped companies and organisations develop open innovation strategies and support their implementation, especially in life sciences R&D. Previously Jackie was a Senior Vice President at GlaxoSmithKline and chair of the Research Directors Group at EFPIA. At GSK her business unit delivered 17 clinical proof of concept. She has been part of international committees and policy groups on pharmaceutical R&D. As a non-executive director of a public company and a trustee/governor for academic and other organisations she has gained a broad perspective across many stakeholder groups.

Marcin Szumowski (Rapporteur) (PL), President & CEO, OncoArendi Therapeutics, founder, BTM Mazovia. Following a successful research career in the United States, Marcin Szumowski has been involved in technology transfer and start-up companies since 2000 and has co-founded and managed three start-ups, including now publicly traded Medicalgorithmics S.A. (www.medicalalgorithmics.com), where he was President and CEO 2005-2010. Since 2001 he has been head of international relations and project management office at the Nencki Institute of Experimental Biology. He has been a member of the Independent Expert Panel assisting the European Commission with the Impact Assessment of European of the IMI2.

Tom Andersen (DK) is Head of the European Investment Bank's Regional Office for the Near East in Cairo and independent consultant. Until a year ago, he was Deputy Economic Advisor at the European Investment Bank specialised in assessing economic viability of R&D projects and project finance operations in the pharmaceutical and chemical sectors. Previously, he worked on acquisition and divestitures within an industrial conglomerate and for Novo Nordisk, an EU-based pharmaceutical company, evaluating and reporting on developments of its drug discovery and corporate development arm. He has been a member of the First IMI JU Evaluation Independent Expert Panel.

Maria Rosaria Di Nucci (IT) is Senior Researcher at the Environmental Policy Research Centre of the Freie Universität Berlin and independent consultant. She has been working in environmental and energy policy and policy assessment for over 25 years and participated in various EU Initiatives. A further focus of her activities is impact assessment. Dr. Di Nucci is an expert evaluator for European RTD funding organisations and the EC. She participates also in the evaluation of the Clean Sky JU and Fuel Cell and Hydrogen Joint Undertaking, acting as the common expert.

Bart Wijnberg (NL) - before his retirement Bart Wijnberg worked for the Dutch Ministry of Health, Welfare and Sport where he held responsibilities for the commissioning of the seminal WHO Report Priority Medicines for Europe and the World in view of FP7, and for the launching of the Dutch Public Private Partnership Top Institute Pharma (TI Pharma). He was a member of the "Member States, Candidate and Associated Countries Contact Group for IMI" and of the First IMI JU Evaluation Independent Expert Panel.

Annex 2 Predefined Evaluation Questions

General Criteria / questions for 2nd interim evaluation of IMI JU

General	
Q1	In general how are the IMI projects and associated technologies viewed and are they still seen competitive in the short, medium and long term?
Q2	What changes in the global economic/financial context of the pharmaceutical and healthcare sectors have occurred since the initiation of the IMI JU programme and what will their effect on the programme be currently and in the longer term?
Q3	To what extent were the recommendations from the first interim evaluation taken into account/implemented?
Effectiveness: Progress towards meeting the objectives set.	
Q4	<p>What progress has been achieved towards the objectives set in Article 2 of the Council Regulation setting up the JU? In particular:</p> <ul style="list-style-type: none"> • Making Europe the best place for pharmaceutical R&D • Contribution to health and socioeconomic benefits for EU citizens • Addressing the bottlenecks in the R&D process & stimulate new technologies/methodologies via a clearly defined Research Agenda (RA) <p>For example - addressing unmet medical needs in the RA; alignment of call topics with RA items.</p>
Q5	How has the IMI JU ensured complementarity with other activities of FP7?
Q6	<p>To what extent has the IMI JU succeeded in networking/pooling various stakeholders between the public and private sectors and in combining private-sector investment and European public funding?</p> <p>Is the IMI JU considered by stakeholders an appropriate tool for increasing long term research investment in the European biopharmaceutical sector? What is the participation pattern in terms of stakeholders (academic, industrial, including SMEs, and research organisation sectors)?</p>
Q7	Has the IMI JU contributed to/promoted the participation/involvement of Small and Medium-sized Enterprises (SMEs) in its supported RTD activities?
Efficiency: The extent to which the JU has been operated efficiently, whether there has been good communication of objectives and progress, and the ability to address problems as they arose.	
Q8	<p>Have the governing structures evolved adequately to address and improve efficiency of the key processes (i.e. call for proposals, mobilising the public and private sector resources needed, facilitating coordination with national and international activities in this area, reviewing and making any necessary adjustments to the Research Agenda etc.)?</p> <p>Are the roles of the scientific committee and the states representative group clear?</p>
Q9	Are the activities of the IMI JU carried out efficiently and transparently? Do patient and other stakeholders have a clear mechanism by which they can input into call topic selection?
Q10	Are the resources applied to the programmes adequate and is the in kind contribution from industry appropriate?

Q11	<p>Are the project deliverables and objectives set realistically and how are these monitored at a project level and an IMI JU level?</p> <p>How do they align with the overall Key Performance Indicators (KPIs) of the IMI JU?</p> <p>How is quality assessed at the project level?</p>
Q12	<p>Are identified IMI JU KPIs sufficiently robust and quantifiable? What progress has been made in achieving these? When does the IMI JU anticipate addressing some key KPIs?</p>
Q13	<p>Are the IMI JU's objectives and achievements adequately communicated to and understood by external (within EU 27 and outside) stakeholders (IP Policy, pre and non-competitive research, IMI as impartial facilitator / collaboration platform etc.)?</p> <p>Are the IMI JU's activities sufficiently visible to the public?</p>
Q14	<p>Is the IMI JU effective/efficient in terms of knowledge dissemination & exploitation? Is the access to project outcome broad / sufficient enough for the participants from outside the IMI consortia?</p> <p>To what extent has sustainability of the output of the IMI JU been considered in current projects?</p>
Q15	<p>Is the IMI JU perceived as flagship for Public-Private partnership-supported RTD in the world and what more could be done in this respect?</p>
Quality: The extent to which the JU supports top-class RTD in the area.	
Q16	Does the IMI JU attract the best researchers and research organisations active in the field?
Q17	Are the measures described in the SRA and are the topic descriptions in the Call for Proposal texts appropriate to ensure innovation
Q18	Does the IMI JU have access to the best organisations (organisation that are too big for SME too small for EFPIA, imaging companies etc.)? What is the impact of lack of the participation of those organisations?
The Future: The extent to which the JU can inform Horizon 2020.	
Q19	What lessons can be learned from the IMI JU for Horizon 2020 in PPPs? Are there particular changes in governance or financial regulations that might be appropriate?

Supplementary Questions to the SRG-Chair and other SRG members

Following the Sherpa report, Member States can be valuable partners in a JTI since they facilitate synergies with national programmes.

- How does involvement of the MS benefit IMI-JU? How could the IMI-JU benefit the MS?
- Are there any synergies with national programmes and the IMI-JU programmes?
- Has the IMI-JU had an impact on the main related national policies and on national research programmes and spending?
- How can the SRG help achieving a better coordination for alignment and effectiveness?
- Are you satisfied with the interaction with the governing board? Where do you see margins for improvement? Are there particular areas for potential conflict of interest? If so, where?

Questions to interviewees belonging to Industry (I)/Academia (A) and project coordinators (PC)

Q. I/A/PC) Is the participation in the IMI-JU perceived as a competitive advantage?

Q. I/A/PC) Do you consider the present projects' funding rate satisfactory?

Q. I/A/PC) What are your expectations concerning the role and scope of the scientific committee? Do its members cover all key areas in a satisfactory manner?

Q. I/A/PC) On the targets set in the IMI research agenda

- How ambitious are the targets set? Do you consider the targets set for 2015 still adequate? Are they sufficiently ambitious?

- Where do you see the major challenges for Horizon 2020?

Q. A/PC) Are research priorities and needs well covered in the IMI focus research areas? Are the major challenges adequately considered?

Q. A/I/PC) Has the IMI-JU played a role in mobilising additional R&D efforts (leverage effect) at national programme level?

- Does the IMI-JU research programme encompass adequately key areas (such as personalised medicine, regenerative medicine, predictive toxicology and safety, health, education and training of patients and professionals) in order to ensure European competitiveness?

- How supportive do you consider the role and work of the scientific committee? Where do you see margins for improvement?

Questions to the IMI-JU-management

- Which progress has been made towards a better coordination with IMI calls and FP7 calls? Has the IMI-JU ensured complementarity with other activities of FP7?

- Which mechanisms are in place to ensure that the deliverables have enough level of maturity and quality? Who sets quality standards for the projects' deliverables?

- How is the monitoring of progress towards objectives organised? How often and which tools and indicators are applied? How is progress (as reported by Members and Partners) verified by the Director?

- Are programme/AIP KPIs defined and translated into project KPIs?

- How have the Calls for Proposal been assessed and improved from the 1st call to the most recent in relation to the applicant's competence level and innovation?

- Are annual implementation plan targets translated in KPIs that can be monitored along the duration of the plan? Are the AIP defined taking such KPIs into account?
- Do you see margins for improvement in the co-operation with and within the JU governance bodies? If yes, where?
- Is there a process to align R&D performed within IMI-JU to national research programmes?
- Are there commonalities with other JTIs and JUs?
- Are there “institutionalised” or structured regular exchanges with other JTIs and JUs? Where do you see possible synergies?

Do patient groups and other stakeholders have the possibility of commenting/contributing/influencing the call topics proposed by EFPIA and if so what is the mechanism?

Questions to the interviewee of the Scientific Committee

- Is the work/advice of the Scientific Committee properly acknowledged by the Board and by stakeholders? Which modifications would you recommend?
- Does the IMI-JU research agenda adequately encompass key areas such as personalised medicine, regenerative medicine, predictive toxicology and safety, ehealth, education and training of patients and professionals) in order to ensure Europe’s competitiveness?
- How well does the interaction, coordination and cooperation between the governance bodies and the SC as well as the SRG work? How is it ensured that the different bodies work toward consistent and coherent objectives? (same Q also to JU-Management/SRG)

Questions to the interviewee of the Governing Board

- How does the Board ensure alignment with other EU programmes and national programmes in order to obtain maximal synergies and impact?
- Which mechanisms are in place to coordinate and evaluate the IMI-JU leveraging of R&D investment at national programme level?
- How well does the interaction, coordination and cooperation between the governance bodies and the Advisory Boards as well as the SRG function? How is it ensured that the different bodies work toward consistent and coherent objectives? (same Q also to JU-Management/SRG/SC)
- Is there a process to align R&D performed within IMI-JU to national research programmes?

Does the 50:50 representation on the IMI Board of EFPIA and the Commission help or hinder the decision making?

Annex 3 IMI-related Documents and Information Consulted

- **Council Decision** – Council Regulation (EC) No 73/2008 of 20 December 2007 setting up the Joint Undertaking for the implementation of the Joint Technology Initiative on Innovative Medicines (OJ L30 of 04.02.2008, p. 38-51) [Council of the European Union 2008].
- **TFEU** - Treaty on the Functioning of the European Union; Article 187 (ex-Article 171 of the EC Treaty): The Union may set up joint undertakings or any other structure necessary for the efficient execution of Union research, technological development and demonstration programmes
- **Commission Proposal** for a Council Regulation on the Innovative Medicines Initiative 2 Joint Undertaking available at: <http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=COM:2013:0495:FIN:EN:PDF>
- **Impact Assessment** - Commission Staff Working Document accompanying the document: Proposal for a Council Regulation on the Innovative Medicines 2 Joint Undertaking, Brussels 10.7.2013 SWD(2013) 245
<http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=SWD:2013:0245:FIN:en:PDF>
- Recent IMI JU documents available at: (www.imi.europa.eu)
 - IMI Financial Rules
 - IMI IP Rules (all documents)
 - IMI Staff Regulation Implementing Rules
 - IMI Revised Scientific Research Agenda (2011)
 - IMI Annual Implementation Plans (2012, 2013)
 - IMI Annual Activity Reports (2012, 2013)
 - IMI Model Grant Agreement
 - IMI Rules for Participation
 - IMI Rules for Submission, evaluation and selection of proposals
 - IMI Guide for Applicants
 - IMI Call Statistics
 - IMI Project description and webpages.
- **The IMI Assessment of Societal and Economic Effects** (2007) – A report of the Independent Expert Panel available at: http://www.imi.europa.eu/sites/default/files/uploads/documents/imi-ia-report-032007_en.pdf
- **JTI Sherpa Report**, January 2010 – Designing together the ‘ideal house’ house for public-private partnership in European Research [JTI Sherpa Group 2010]
- **First Interim Evaluation of the IMI JU** (2011) available at: <http://www.imi.europa.eu/sites/default/files/uploads/documents/Governance/FirstInterimEvaluationOfIMI2011.pdf>
- **Commission Response to the first IER** (2011) available at: <http://www.imi.europa.eu/sites/default/files/uploads/documents/Governance/CommissionResponseInterimEvalIMI2011.pdf>
- **Note of the IMI Executive Director** from June 11, 2013, IMI/OUT/2013-2861 on implementation of first IER recommendations
- **Response and Measures taken by the IMI JU** Internal document C7 0300/2011-2011/2241 (DEC)
- **Report of the Independent Expert Panel** accompanying the Commission Impact Assessment Report of IMI2 a Joint Technology Initiative under Horizon 2020, available at: ftp://ftp.cordis.europa.eu/pub/fp7/health/docs/expert-panel-report-2012_en.pdf
- Gambardella, A., Orsenigo, L. and F. Pammolli (2000): **Global Competitiveness in Pharmaceuticals: A European Perspective**. Published in: DG Enterprise, European Commission (2001)

- ***Innovative Medicines Initiative Communication Strategy*** 2013-2014 prepared by © MEDIA CONSULTA International Holding, version 1, June 28 2013 – internal communication
- ***Bibliometric analysis of ongoing projects:*** Innovative Medicines Initiative Joint Undertaking, prepared by Thomson Reuters on behalf of IMI JU Executive Office, Copyright IMI Executive Office, March 2013.
- ***Report on the outcome of the Innovative Medicines Initiative*** project Participants' Questionnaire, 2012, available at:
ftp://ftp.cordis.europa.eu/pub/fp7/health/docs/outcome-imi-participants_en.pdf

Published Articles related to IMI

- Denee, T. R. et al. (2012) Measuring the value of public-private partnerships in the pharmaceutical sciences, *Nature Reviews Drug Discovery* 11:419.
- De Rijck and M. Goldman (2010) Correspondence: Clarifying knowledge ownership in Europe's Medicines Initiative, *Nature*, Vol. 466, pp. 1040-1041
- Goldman, M. (2012) The Innovative Medicines Initiative: A European Response to the Innovation Challenge, *Clin Pharm Therap* 91:3: 418-425
- Gunn M et al. (2013) The Rational Use of Animals in Drug Development: Contribution of the Innovative Medicines Initiative, *ATLA* 40, 307–312
- Kirby T. (2012) Europe to boost development of new antimicrobial drugs. *Lancet*, 379:2229-30.
- Laverty et al. (2012) Improving R&D productivity of pharmaceutical companies through public–private partnership: experiences from the Innovative Medicines Initiative, *Expert Rev. Pharmacoecon. Outcomes Res.* 12(5), 545–548
- Strohmeier, R. et al. (2011) IMI Moves forward, *Nature Biotechnology*, Vol. 29, pp. 689–690
- Woodcock, J. (2010) Precompetitive research: A new prescription for drug development? *Clinical Pharmacology & Therapeutics*, Vol. 87 (5): pp. 521-523.

Annex 4 List of People Interviewed

- **Richard Bergström**, Executive Director of EFPIA and EFPIA Representative at the IMI JU Governing Board
- **Catherine Brett**, Communication & Events Manager of IMI JU
- **Salah-Dine Chibout**, Novartis, Global Head Discovery & Investigative Safety
- **Magda Chlebus**, Director of Science Policy at the EFPIA
- **Daan Crommelin**, Executive Director TIPharma, president of the European Federation of Pharmaceutical Sciences (EUFEPS) and member of the scientific advisory board of IMI
- **Antoine Cuvillier**, Head of Administration & Finance IMI JU
- **Daphne Derouane**, IP/Knowledge Management from Industry
- **Roch Doliveux**, Chief Executive Officer of UCB, Member of EFPIA Board, Vice Chair of the IMI JU Governing Board
- **Ruxandra Draghia-Akli**, Director “Health”, European Commission DG Research, Commission Representative & Deputy-Chair of the IMI JU Governing Board)
- **Hans-Georg Eichler**, Senior Medical Officer, EMA
- **Michel Goldman**, Executive Director IMI JU
- **Mike Hardmann**, Pharma coordinator (AstraZeneca), EMTRAIN (Education & Training, call 1)
- **Peter Hongaard Andersen**, Chair of EFPIA Research Directors Group, EFPIA Representative at the IMI JU Governing Board
- **Francois Houyez**, Treatment Information and Access Director, Health Policy Advisor EURORDIS
- **Ulf Johann**, legal counsel in the department for Public and EU Projects at Fraunhofer central administration in Munich
- **Lars Klareskog**, Academia coordinator (Karolinska Institutet), BT-CURE (Rheumatoid arthritis, Call 2)
- **Christian Noe**, Chair IMI JU Scientific Committee
- **Gunnar Sandberg**, Chair, IMI States Representative Group
- **Ferran Sanz**, Academia coordinator (Institut Hospital del Mar, Barcelona), eTOX (Toxicology, call 1)
- **Judith Schallnau**, IP/Knowledge Management WIPO
- **Claire Skentelbery**, Secretary General European biotech network
- **Janet Thornton**, Director of the European Bioinformatics Institute
- **Anne-Fabienne Weitsch**, SME Participant IMIDIA (Diabetes, call 1)
- **Bryn Williams-Jones**, Pharma coordinator OPEN PHACTS (Knowledge management, Call 2)

Annex 5 Summary of actions taken to address the recommendations of the first IMI JU Interim Review Evaluation

Michel Goldman, Executive Director

This note was prepared following the interview with the panel of experts in charge of the second IMI interim review.

1. Involvement of stakeholders in IMI projects

In order to ensure the mobilisation of potential consortium partners eligible for IMI JU funding, IMI regularly organises info days and webinars for potential applicants around the launch of each Call. These events feature presentations on both the Call topics and IMI's procedures and intellectual property (IP) policy. IMI also promotes Calls via its website, newsletter, the press, and social media channels, and sends information on Calls to organisations whose members could be interested in applying. In the same vein, a primer on IMI IP Policy was published in Nature Reviews Drug Discovery (vol. 10, p.322, 2011). In addition, IMI Executive Office staff gives presentations at information meetings in the Member States and associated countries; these are often organised in conjunction with IMI States Representatives (SRG) or National Contact Points (NCP). These meetings were held in the following countries: Austria, Cyprus, Czech Republic, France, Germany, Hungary, Italy, Ireland, Israel, Lithuania, Malta, the Netherlands, Poland, Portugal, Romania, Spain, Sweden, and Switzerland.

In parallel, a series of measures were taken to facilitate the submission of proposals and accelerate the launch of the projects. Indeed, the average time to grant from initial submission of Expressions of Interest to signature of Grant Agreements has been decreased to 185 days for the 6th Call for proposals, which is in line with the target proposed by the European Commission for the forthcoming Horizon 2020 Framework programme.

Special attention was paid to attracting small and medium-sized enterprises (SMEs). A Senior Scientific Officer has been tasked with focusing on SMEs. He has developed links with several SME organisations (EBE, EuropaBio, European Biotechnology Network, Enterprise Europe Network, SMC pharma), developed a webpage dedicated to SMEs, and attended several meetings focusing on SME interests. These efforts resulted in an increased mobilisation of SMEs, especially for Call 5 (European Lead Factory), and Call 8 (Discovery of new antibiotics) that attracted 80 SME applicants. As of 1 May 2013, 18.9 % of IMI JU funding is allocated to SMEs (112 SMEs currently receive IMI JU funding). Additional actions foreseen for SMEs in 2013 include match-making events with venture capital funds.

In order to mobilise and sensitise patient organisations, contacts have been established with several European and national organisations in different disease areas and a first event dedicated to patients and caregivers has been planned for 12 June 2013 with the support of Mary Baker, President of the European Brain Council and a member of the IMI Scientific Committee. Furthermore, a collaboration agreement was signed in 2013 with the research centre in Health and Social Care at the London School of Economics (Professor Panos Kanavos) to assess and enhance the patient's perspective on IMI.

2. Strengthening and deepening consultation with the regulators, in particular the European Medicines Agency

Close links were established with the EMA, primarily through its Senior Medical Officer, Hans-Georg Eichler. He has been invited to be a permanent observer of the IMI Scientific Committee in order to streamline the EMA's input into IMI projects. Two joint EMA-IMI meetings were held in London in order to organise the consultation of the EMA on the regulatory relevance of results obtained by IMI consortia, discuss the EMA's proposals for

IMI topics, and investigate the possibility of launching an IMI Call on adaptive licensing. At the initiative of EFPIA, a joint meeting gathering the FDA, EMA and IMI was organised the FDA's premises and teleconferences with the FDA are also organised. Contacts were also established with the Japan's PMDA regulatory agency and a visit of the Executive Director to PMDA is planned in June 2013.

Furthermore, a Senior Scientific Officer with specific experience and expertise in the area of drug regulation has been tasked with liaising with regulatory authorities and facilitating consultation on the regulatory relevance of the results generated by IMI consortia.

3. Improving IMI communication

Multiple channels including social media were used to improve the visibility of IMI among the different stakeholders. The messages were focused on the achievements of IMI projects which demonstrate:

- how IMI enhances EU competitiveness in the pharmaceutical sector by fostering a new ecosystem based on open innovation;
- how IMI offers new business opportunities to SMEs;
- how IMI contributes to addressing major public health needs (e.g. antimicrobial resistance);
- how IMI fosters European leadership in medical and pharmaceutical sciences by building collaborative intelligence networks.

In addition to the IMI staff, several members of the IMI Scientific Committee contributed to IMI communication and efforts will be continued together with the SRG members acting as IMI ambassadors/multipliers.

The improved IMI communication is reflected in the results of the recent reports on media coverage, in the IMI quotes by industry leaders, including CEOs of large pharmaceutical companies, key scientific opinion leaders and EU policy makers.

In the future, additional communication activities will target policy makers especially members of the European Parliament (an IMI event is scheduled on 3 July in the European Parliament in Strasbourg). For this purpose, new messages will be prepared from the IMI performance metrics (see below).

Furthermore, as suggested by members of the interim review panel, attention will be paid to the use of video clips as additional communication tools (a video about the IMI contributions in autism is currently being finalised).

4. Developing evaluation and monitoring processes

We initiated this endeavour by co-organising two workshops with TI-Pharma on the assessment of the value of public-private partnerships. The conclusion of this exercise, published in *Nature Biotechnology* (11:419, 2012), was that the most meaningful indicators of output and outcomes cannot be measured in the early phases of the projects and that several relevant metrics can be derived in the short term from the analysis of scientific publications. We therefore established a collaboration agreement with Thomson Reuters to perform this analysis; the first results were recently reported and this effort will be continued.

In parallel, we extracted the most meaningful achievements of the projects from annual scientific reports, during interim reviews by external experts, and through direct communication with project coordinators. These achievements were classified in six categories corresponding to the main objectives of the IMI partnership. The translation of these achievements into metrics (e.g. cost savings) is challenging but this effort will be pursued in collaboration with EFPIA.

We developed an IT tool to facilitate tracking of project data and the generation of "classical" metrics. This tool is now operational and will allow reporting of the much-awaited figures by mid-June 2013. In the future, data generated by the IT-tool will be used to generate a Balanced Scorecard that should facilitate the governance of the partnership.

5. Accelerating the recruitment process

The recruitment process has been streamlined so that the authorised maximum ceiling of 36 staff members was reached on 1 July 2012. Fifteen different nationalities are represented in the IMI staff, with a female to male ratio of 1,7.

Among the additional competencies recruited, 2 staff members are holding a MBA degree (Goethe University, Frankfurt and London School of Economics, London) and 1 staff member has a 15-year experience at the European Medicines Agency.

For the future, the IMI office would benefit from additional staff with business development experience in the pharmaceutical industry.

Annex 6 Topics of Calls 4 through 9

Topics 4th IMI Call launched on 18 July 2011 with 7 topics.

1. A European Medical Information Framework (EMIF) of Patient level Data to support a wide range of medical research
 - 1.1 Subtopic 1: Information Framework / Knowledge Management Service Layer
 - 1.2 Subtopic 2: Metabolic complications of obesity
 - 1.3 Protective and precipitating markers for the development of Alzheimer's disease (AD)-other dementias
2. eTRIKS: European Translational Information & Knowledge Management Services
3. Delivery and targeting mechanisms for biological macromolecules
4. In vivo predictive biopharmaceutics tools for oral drug delivery
5. Sustainable Chemistry - delivering medicines for the 21st century
6. Human Induced Pluripotent Stem (hiPS) Cells for drug discovery and safety assessment
7. Understanding and optimising binding kinetics in drug discovery

Topics 5th IMI Call launched on 06 March 2012 with 2 topics.

1. European Screening Centre
2. Joint European Compound Collection

Topics 6th IMI Call launched on 24 May 2012 with 2 topics.

1. Innovative trial design & clinical drug development, with 2 subtopics
2. Learning from success and failure & getting drugs into bad bugs

Topics 7th IMI Call launched on 17 July 2012 with 2 topics.

1. Developing a framework for rapid assessment of vaccination benefit/risk in Europe
2. Incorporating real-life clinical data into drug development

Topics 8th Call launched on 17 December 2012 with 3 themes.

1. Combating Antibiotic Resistance – New Drugs for Bad Bugs (ND4BB)
2. Developing an aetiology-based taxonomy of human disease with 2 topics
3. "European induced pluripotent stem cell bank"

Topics 9th IMI Call launched on with 4 topics launched on 9 July 2013.

1. WEBAE – Leveraging Emerging Technologies for Pharmacovigilance
2. Developing Innovative Therapeutic Interventions Against Physical Frailty and Sarcopenia (ITI-PF&S) as a Prototype Geriatric Indication
3. ND4BB TOPIC 4: Driving re-investment in R&D and Responsible Use of Antibiotics
4. ND4BB TOPIC 5: Clinical development of antibacterial agents for Gram-negative antibiotic resistant pathogens

Annex 7 Information on 40 on-going IMI Projects launched in Calls 1-6

1st Call
2008

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries Total Participants	Start Length	Project Total costs	Website
SAFESCIMET Education / Training	European Modular Education & Training Programme in Safety Sciences For Medicines	Hoffmann-La Roche / VU Univ Amsterdam 15 Pharma 18 Univ / RO	1 Jan 10 60 months	6.451.486 Euros	www.safescimet.eu
EMTRAIN Education / Training	European Medicines Research Training Network	AstraZeneca / Med Univ Wien 16 Pharma 11 Univ / RO	1 Oct 10 84 months	7.528.060 Euros	www.emtrain.eu
PHARMATRRAIN Education / Training	Pharmaceutical Medicine Training Programme	EFCPM⁴⁴ 15 Pharma 35 Univ / RO	1 May 09 60 months	6.653.588 Euros	www.pharmatrain.eu
EU2P Education / Training	European Programme in Pharmacovigilance and Pharmacoepidemiology	Hoffmann-La Roche / Univ Bordeaux 15 Pharma 9 Univ / RO	1 Sept 09 60 months	7.270.886 Euros	www.eu2p.org
IMIDIA Efficacy	Improving beta-cell function and identification of diagnostic biomarkers for treatment monitoring in diabetes	Sanofi / Univ Lausanne 8 Pharma 12 Univ/RO 1 SME	1 Feb 10 60 months	23.638.480 Euros	www.imidia.org
SUMMIT Efficacy	SURrogate markers for Micro- and Macro-vascular hard endpoints for Innovative diabetes Tools	Boehringer Ingelheim /Lund Univ 6 Pharma 20 Univ / RO 1 SME	1 Nov 09 60 months	32.385.366 Euros	www.imi-summit.eu
EUROPAIN Efficacy	Understanding Chronic pain and improving its treatment	AstraZeneca / Kings College London 7 Pharma 12 Univ /RO 1 SME	1 Oct 09 60 months	18.751.899 Euros	www.imieuropain.org/
NEWMEDS Efficacy	Novel Methods leading to New Medications in Depression and Schizophrenia	Lundbeck / Kings College London 10 Pharma 7 Univ / RO 3 SME	1 Sept 09 60 months	25.065.375 Euros	www.newmeds-europe.com
PHARMA-COG Efficacy	Prediction of cognitive properties of new drug candidates for neurodegenerative diseases in early clinical development	GSK /Univ Marseille 12 Pharma 24 Univ / RO 5 SME	1 Jan 10 60 months	29.820.087 Euros	www.alzheimer-europe.org
U-BIOPRED Efficacy	Unbiased Biomarkers for the Prediction of Respiratory Disease Outcomes	AMC Amsterdam⁴⁵ 10 Pharma 31 Univ / RO 3 SME 1 other industry	1 Oct 09 60 months	22.289.902 Euros	www.ubiopred.eu

⁴⁴ EFCPM – European Federation of Course Providers in Pharmaceutical Medicines, University of Basel, acting as coordinator and managing entity of IMI beneficiaries

⁴⁵ AMC Amsterdam - acting as coordinator and managing entity of IMI beneficiaries

1st Call
2008

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries Total Participants	Start Length	Project Total costs	Website
PROACTIVE Efficacy	Physical Activity as a Crucial Patient Reported Outcome in COPD	Chiesi Farmaceutici / Univ Leuven 8 Pharma 10 Univ / RO 1 SME	1 Sept 09 60 months	16.736.468 Euros	www.proactivecopd.com
MARCAR Safety	BioMARKers and molecular tumour classification for non- genotoxic CARcinogenesis	Novartis / Univ Dundee 5 Pharma 6 Univ /RO 1 SME	1 Jan 10 60 months	13.072.736 Euros	www.imi-marcar.eu
ETOX Safety	Integrating bioinformatics and chemo informatics approaches for the development of expert systems allowing the <i>in silico</i> prediction of toxicities	Novartis / IMIM⁴⁶ 13 Pharma 10 Univ / RO 5 SME	1 Jan 10 60 months	13.885.471 Euros	www.etoxproject.eu
SAFE-T Safety	Safer and Faster Evidence Based Translation	Novartis / Univ Tuebingen 11 Pharma 10 Univ / RO 4 SME	15 Jun 09 60 months	35.803.798 Euros	www.imi-safe-t.eu
PROTECT Safety	Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium	European Medicines Agency (EMA) / Danish Medicines Agency 14 Pharma 21 Univ / RO 2 SME	1 Jan 09 60 months	25.900.581 Euros	www.imi-protect.eu

⁴⁶ Fundació IMIM, Barcelona, Spain

2nd Call
2009

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries	Start Length	Project Total costs	Website
		Total Participants			
PREDECT Efficacy	New models for preclinical evaluation of drug efficacy in common solid tumours	<u>Servier / Univ Helsinki</u> 9 Pharma 10 Univ / RO 2 SME	1 Feb 11 60 months	19.116.197 Euros	www.predect.eu
ONCOTRACK Efficacy	Methods for systematic next generation oncology biomarker development	<u>Bayer / Max-Planck Institute</u> 8 Pharma 9 Univ / RO 4 SME	1 Jan 11 60 months	29.855.078 Euros	www.emtrain.eu
QUIC-CONCEPT Efficacy	Quantitative Imaging in Cancer: Connecting Cellular Processes with Therapy	<u>AstraZeneca / EORTC⁴⁷</u> 8 Pharma 14 Univ / RO 1 SME	1 Sept 11 60 months	16.872.662 Euros	www.quic-concept.eu
RAPP-ID Efficacy	Development of RAPid Point-of-Care test Platforms for Infectious Diseases	<u>Johnson & Johnson / Univ Antwerp</u> 5 Pharma 10 Univ / RO 4 SME	1 Apr 11 60 months	14.559.595 Euros	www.rapp-id.eu
BTCURE Efficacy	Be The Cure	<u>UCB / Karolinska</u> 8 Pharma 24 Univ/RO 1 SME	1 Apr 11 60 months	37.327.023 Euros	www.btcure.eu
DDMORE Knowledge Management	Drug Disease Model Resources	<u>Pfizer / Univ Uppsala</u> 10 Pharma 12 Univ/RO 6 SME	1 Mar 11 60 months	21.646.231 Euros	www.ddmore.eu
OPENPHACTS Knowledge Management	The Open Pharmacological Concepts Triple Store	<u>Pfizer / Univ Vienna</u> 9 Pharma 18 Univ / RO 4 SME	1 Mar 11 36 months	17.096.299 Euros	www.imi-summit.eu
EHR4CR Knowledge Management	Electronic Health Record Systems for Clinical Research	<u>AstraZeneca / Eurorec</u> 10 Pharma 22 Univ / RO 3 SME	1 Mar 11 48 months	16.204.132 Euros	www.ubiopred.eu

3rd Call
2010

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries	Start Length	Project Total costs	Website
		Total Participants			
MIP DILI Safety	Mechanism-Based Integrated Systems for the Prediction of Drug-Induced Liver Injury	<u>Astra Zeneca / Univ Liverpool</u> 11 Pharma 9 Univ / RO 6 SME	1 Feb 12 60 months	32.303.046 Euros	www.mip-dili.eu
ABIRISK Safety	Anti-Biopharmaceutical Immunization: Prediction and analysis of clinical relevance to minimize the risk	<u>GSK / Inserm</u> 10 Pharma 27 Univ / RO 2 SME	1 Mar 12 60 months	31.842.998 Euros	www.abirisk.eu
BIOVACSAFE Safety	Biomarkers For Enhanced Vaccine Safety	<u>Novartis / Univ Surrey</u>	1 Mar 12 60 months	30.222.083 Euros	www.biovacsafe.eu

⁴⁷ European Organisation for Research and Treatment of Cancer, Belgium

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries Total Participants	Start Length	Project Total costs	Website
		3 Pharma 14 Univ / RO 2 SME			
PREDICT TB Efficacy	Model-based preclinical development of anti-tuberculosis drug combinations	GSK / Univ Liverpool 3 Pharma 15 Univ / RO 2 SME	1 May 12 60 months	28.383.392 Euros	-
EU AIMS Efficacy	European Autism Interventions - A Multicentre Study for Developing New Medications	F. Hoffman-La Roche / Kings' College London 6 Pharma 16 Univ/RO 4 SME	1 Apr 12 60 months	34.132.099 Euros	www.eu-aims.eu
DIRECT Efficacy	Diabetes REsearch on patient sTratification	Sanofi / Univ Dundee 4 Pharma 21 Univ / RO	1 Feb 12 60 months	41.369.959 Euros	www.direct-diabetes.org
EUPATI Education & Training	European Patients' Academy on Therapeutic Innovation	VFA⁴⁸ / European Patient's Forum 17 Pharma 12 Univ / RO	1 Feb 12 60 months	10.006.115 Euros	www.patientsacademy.eu

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries Total Participants	Start Length	Project Total costs	Website
EMIF Knowledge Management	European Medical Information Framework	Janssen Pharmaceutica / Erasmus Univ 9 Pharma 40 Univ / RO 9 SME	1 Jan 13 60 months	56.317.001 Euros	-
eTRIKS Knowledge Management	Delivering European Translational Information & Knowledge Management Services	AstraZeneca / Imperial College London 10 Pharma 4 Univ / RO 1 SME	1 Oct 12 60 months	23.785.741 Euros	www.etriks.org
COMPACT Safety	Collaboration on the Optimisation of Macromolecular Pharmaceutical Access to Cellular Targets	Sanofi / Univ Utrecht 7 Pharma 14 Univ / RO 1 SME	1 Nov 12 60 months	29.984.840 Euros	www.compact-research.org
ORBITO Safety	Oral biopharmaceutics tools	AstraZeneca / Univ Uppsala 12 Pharma 11 Univ / RO 3 SME	1 Oct 12 60 months	24.394.881 Euros	-
CHEM21 Safety	Chemical Manufacturing Methods for the 21st Century Pharmaceutical Industries	GSK / Univ Manchester 6 Pharma 11 Univ/RO 4 SME	1 Oct 12 48 months	26.423.191 Euros	www.chem21.eu
STEMBANCC	Stem cells for Biological	F. Hoffman-La Roche /	1 Oct 12	55.602.793	www.stembancc.org

4th Call
2011

⁴⁸ Verband forschender Arzneimittelhersteller eV, Berlin, Germany

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries Total Participants	Start Length	Project Total costs	Website
Safety	Assays of Novel drugs and prediCtive toxicology	Univ Oxford 10 Pharma 23 Univ / RO 3 SME	60 months	Euros	
K4DD Safety	Kinetics for Drug Discovery	Bayer / Univ Leiden 7 Pharma 10 Univ / RO 3 SME	1 Nov. 12 60 months	20.987.016 Euros	www.k4dd.eu

5th Call
2012

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries Total Participants	Start Length	Project Total costs	Website
EUC²CLID Beyond High Throughput screening	European Lead factory	Bayer / Univ Leiden 7 Pharma 13 Univ / RO 10 SME	1 Jan 13 60 months	196.539.059 Euros	-

6th Call
2012

IMI Project Acronym Pillar addressed	Name	Coordinator / Managing entity IMI beneficiaries Total Participants	Start Length	Project Total costs	Website
COMBACTE Infectious Diseases	Combating bacterial Resistance in Europe	GSK / UMC Utrecht 3 Pharma 18 Univ / RO 1 SME	1 Jan 13 84 months	135.960.964 Euros	-
TRANSLOCATION Infectious Diseases	Molecular Basis of The outer membrane permeability	GSK / Jacobs Univ 5 Pharma 15 Univ / RO 5 SME 1 other industry	1 Jan 13 60 months	24.348.006 Euros	-

Abbreviations used:

Pharma – Pharmaceutical Companies, Members of EFPIA

Univ / RO – Universities, Research Organisations, Public Bodies & Non-Profit

SME – Small and Medium Enterprises

Annex 8 Call 4-7 Statistics

Overview of IMI Calls for Proposals IV to VII

	4 th call for proposals	5 th call for proposals	6 th call for proposals	7 th call for proposals
Publication Date	18 July 2011	06 March 2012	24 May 2012	17 July 2012
Number of topics	7	2	2	2
Stage 1 Deadline	15 October 2011	16 May 2012	09 July 2012	9 October 2012
Expressions of Interest received	86	14	14	9
Participants	939	162	198	46
Stage 2 Deadline	13 March 2012	13 September 2012	10 October 2012	07 March 2013
Full Project Proposals received	7	1	2	2
Grant Agreements signed	7	1	2	0
Maximum IMI JU financial contribution (mil €)	97,9 (based on signed Grant Agreements)	80 (based on signed Grant agreements)	99 (based on signed Grant agreements)	13 (based on call published)
Indicative in-kind contribution (mil €)	112 (based on signed Grant Agreements)	91 (based on signed Grant agreements)	112 (based on signed Grant agreements)	14 (based on call published)

Annex 9 Executive summary of the impact assessment for IMI2 JU



Brussels, 10.7.2013
SWD(2013) 246 final

COMMISSION STAFF WORKING DOCUMENT

**Executive summary of the impact assessment
accompanying the document
Proposal for a
COUNCIL REGULATION
on the Innovative Medicines Initiative 2 Joint Undertaking**

{COM(2013) 495 final}
{SWD(2013) 245 final}

COMMISSION STAFF WORKING DOCUMENT

EXECUTIVE SUMMARY OF THE IMPACT ASSESSMENT
accompanying the document
Proposal for a
COUNCIL REGULATION
on the Innovative Medicines Initiative 2 Joint Undertaking

This document summarises the impact assessment for the Joint Technology Initiative (JTI) on innovative medicines (IMI), established as a joint undertaking under the 7th Research Framework Programme. The proposal has been produced in the context of the Union's Multiannual Financial Framework (2014-2020), and will contribute to the implementation of the next EU Framework Programme for Research and Innovation, Horizon 2020.

1. Problem Definition

1.1. *The problem that requires action*

An ageing population increases the burden of chronic and degenerative diseases, putting additional pressure on health and care systems at a time of stretched public finances. Effective measures represent a significant part of the solution. However, the role of research and development (R&D) in developing therapies is declining, incentives for some classes of therapy (e.g. antibiotics) are all but absent and structural issues stand in the way of multidisciplinary cooperation, required to solve complex scientific problems that are characteristic of this field. Failure to act is neither in the interest of European public health nor of European competitiveness.

The process of developing therapies is costly, involving many tests before marketing approval can be given. These tests often demonstrate that the therapy in question is unsuitable and thus the investment is lost. This creates an incentive for manufacturers to invest in developing therapies which have a greater chance of success, either because they resemble existing therapies or because the potential return is very high. While this is a sensible business decision, it is not necessarily in the general interest of the EU citizen.

1.2. *Key problem drivers*

The relatively low investment in the biotechnology sector (*vis-à-vis* competitor regions) combined with the fragmented, closed innovation model of drug development in Europe and the complexity of the process act as a disincentive to risk taking by industry. The nature of the scientific challenges is such that data must be shared among various stakeholders. Without a framework enabling this to happen in a controlled environment, cooperation will not take place.

1.3. *Need for public intervention*

A controlled environment will not evolve naturally in the commercial environment, nor can it be achieved by the public sector alone. It can only come about through public cooperation, where the various players (academia, industry, SMEs, clinicians, regulators and patients) share resources, data and expertise, while ensuring that the fruits of their collaboration are shared, risks and costs reduced and productivity increased. The creation of such a risk-sharing environment will reduce the failure rate and those carrying out tests will have a greater incentive to test a wider variety of therapies to the benefit of all concerned, both in terms of promoting public health and legitimately protecting commercial interests.

1.4. *The EU's right to act and the application of the subsidiarity principle*

The right of the EU to act in this field is provided under Article 187 of the Treaty on the Functioning of the EU, authorising the setting up of 'joint undertakings or any other structure necessary for the efficient execution of Union research, technological development and demonstration programmes'.

1.4.1. *The required public intervention can only be provided at European level*

Measures at EU level to support trans-national and cross-sector cooperation between firms on strategic research agendas will help to establish 'critical mass', in particular through joint agenda setting, mobilisation of additional funding and increased leverage on industrial R&D investment.

1.4.2. *Investing at EU level can produce savings in healthcare costs and services*

The research programme will lead to a better classification of diseases, significantly improving diagnoses and treatments. This will prevent patients' unnecessary exposure to the adverse effects of ineffective treatments during clinical development or medical practice. In the latter case, savings have been achieved by discontinuing an ineffective or inappropriate treatment. For example, an analysis carried out in France has demonstrated the monetary benefit of molecular diagnosis of cancer patients. Investing €1.7 million in molecular diagnosis has resulted in savings of €34 million by not administering the cancer drug Iressa® to patients for whom it is ineffective. Even bigger savings can be expected from the classification of chronic diseases.

1.5. *The achievements of the current IMI*

The IMI Joint Undertaking has produced a number of important results:

- considerable leverage effect on industrial R&D investment by virtue of a €1bn contribution from the European Commission and a contribution in kind of €1bn from the European Federation of Pharmaceutical Industries and Associations (EFPIA);
- enhanced cooperation — the IMI Joint Undertaking brings together large-scale industry, SMEs and research organisations from across the European Union;
- joint production of comprehensive strategic research agendas and coordination of other policies due to the involvement of patient organisations and regulatory bodies;

- an open innovation model — the IMI Joint Undertaking has contributed to the transition from a closed to an open model of innovation in biomedical and pharmaceutical research.

1.6. *The lessons learnt from IMI*

Despite these achievements, the implementation of IMI and the 2011 interim evaluation have revealed a number of shortcomings:

- the legal instruments used for setting up the JTIs, and in particular their status as Union bodies, need to be made more flexible;
- the participation rules applied to/by JTI joint undertakings, in reflecting the needs of the various partners, add to the complexity of the initiative;
- monitoring and evaluating achievement of the targets included in the strategic research agenda and technical work plans need to be improved;
- horizontal policy coordination needs to be strengthened (e.g. the advisory potential of the European Medicines Agency (EMA) should be fully exploited);
- internal and external communication needs to be strengthened.

The shortcomings identified stem from the initial design and constitute a starting point for improving the design of the IMI Joint Undertaking, under Horizon 2020.

2. Objectives

The general and specific objectives that have been identified are based on the outcomes of the public consultation, the problems and drivers and the achievements and lessons learnt from IMI.

2.1. Overall objectives

The overall objective is to improve European citizens' health and wellbeing by providing new and more effective diagnostics and treatments while helping safeguard the future international competitiveness of the European biopharmaceutical and life science industries such as diagnostics, vaccines, biomedical imaging and medical information technologies. The IMi2 Joint Undertaking will implement Horizon 2020 objectives, in particular as defined in the Health, demographic change and wellbeing societal challenge, and will address the public health challenges identified in the World Health Organisation report on priority medicines for Europe and the World.

2.2. Operational objectives

The operational objectives of this initiative are to:

- provide structures that facilitate partnerships along the entire life science research and innovation cycle, such as from early discovery to product development, to pharmacovigilance research and surveillance, in an effective innovation-driven collaborative setting that is focused on optimising life sciences research and

- innovation for diagnostics, prevention and therapeutic agents and approaches, and support for the development of evidence-based regulation;
- establish networks for open innovation along the whole innovation cycle of novel medical research and technologies, bringing public research institutions, academia, life science industries, SMEs, patient organisations, regulators, payers, public health authorities and the animal health sector;
 - reduce the fragmentation of research and innovation and increase the level of private-sector spending in Europe;
 - develop and implement strategic agenda setting in a pan-European structure with the necessary critical mass and budget, ensuring continuity and allowing life science industries to make long term investment plans;
 - facilitate research that provides evidence earlier in the drug and vaccine development process through risk-sharing mechanisms.

2.3. Specific objectives

The specific objectives are to:

- improve by 2020 the success rate in clinical trials by 30% in diseases identified in the ‘Priority Medicines for Europe and the World WHO Report’;
- reduce to five years the time taken to reach clinical proof of concept in immunological, respiratory, neurological and neurodegenerative diseases;
- develop at least two new therapies for diseases for which there is a high, unmet need and limited market incentives: antimicrobial resistance (two new classes in the past 30 years) or Alzheimer’s disease (only two treatments of limited efficacy have ever been developed);
- develop diagnostic markers for four diseases (among those mentioned above), clearly linked to clinical relevance and approved by regulators;
- develop a transparent and comprehensive infrastructure model to gather data on disease incidence and the medico- and socio-economic burden of major infectious diseases;
- develop tested novel biomarkers to predict vaccine efficacy and safety (two markers each) early on in the process, to improve multiple-candidate screening to achieve a 50% reduction in the failure rate in phase III clinical trials;
- develop two novel adjuvants for human use to increase the body’s immune response to vaccines, boosting in particular reaction in specific target groups such as the elderly and non-responders;
- identify, for two major infectious diseases and two types of cancer or chronic disorder (e.g. autoimmune diseases), at least: two novel predictive models for efficacy and two novel predictive models for safety;
- strengthen the link between human and veterinary vaccine research.

3. Policy Options

The impact assessment considered four main policy options:

1. Business-as-usual: continuation of the current IMI JTI under Horizon 2020, managed by the Joint Undertaking. Under this option, IMI remains focused on building a collaborative system for biomedical R&D in Europe and speeding up the development of effective and safer medicines for patients.
2. No public-private partnership (PPP) or ‘zero option’: use of Horizon 2020 collaborative projects only. This option facilitates the formulation of common objectives at project level but does not accommodate the formulation of cross-project execution of strategic agendas. Industry participation takes place on a project-by-project basis.
3. Contractual PPP to implement Horizon 2020 actions falling under the ‘Health, demographic change and wellbeing’ societal challenge. Under this option, an industry partnership agreement is concluded and industry proposes a strategy and advises on work programmes. Whilst EU commitment and contribution is set at the launch of PPP, financing amounts and topics are subject to approval under an annual work programme.
4. Modernised JTI: expands the objectives and activities of the IMI Joint Undertaking in line with Horizon 2020 objectives; broadens the scope of the current programme and improves its governance.

4. Assessment of Impacts and Comparison of Options

The four policy options were compared on a range of key parameters, assessing public involvement in life sciences research and innovation.

The outcome of this comparison is that the ‘Modernised JTI’ option is the preferred option. It achieves critical mass at programme and project level; fosters scientific excellence in biopharmaceutical and life science research, which impacts on innovation and is enhanced through financial support from scientific ideas to the market, a stronger output orientation and better dissemination of research results; greater scientific and innovation impact translates into larger downstream economic, competitiveness, social and public health impacts; and allows for more flexibility and reduced administrative costs for applicants and participants, and publically funded entities, such as academic and SMEs, benefit from administrative simplification. This option also maximises cost-effectiveness.

The case of the ‘zero option’ makes the formulation of cross-project execution of strategic agendas difficult. Critical mass is compromised and the level of flexibility, accessibility and broader horizontal policy coordination is lower than with the ‘Modernised JTI’ option. This would translate into smaller economic, competitiveness, social and public health impacts.

The ‘Contractual PPP’ option accommodates the formulation of cross-project execution of strategic agendas, but it constitutes a ‘light’ approach to a public-private partnership, with an indicative budget only and a rather limited commitment from industry.

Summary comparison of options (impact compared with the BAU scenario)

	No PPP	PPP	Modernised JTI
Public health impacts	--	-	+++
Social impacts	--	-	++
Economic and competitiveness impacts	-	-	++
Innovation impacts	--	-	++
Critical mass of resources	--	-	+
Leverage effect (overall R&I resource mobilisation)	--	-	=
Participation of industry and SMEs	--	-	++
Strategic agenda	--	-	+
Addressing fragmentation	-	-	++
Administrative cost and efficiency of governance	-	--	=
Coherence	=	=	++
Efficiency	--	=	++
Effectiveness	--	=	++

5. Monitoring And Evaluation

An appropriate monitoring and evaluation system set up at programme and project level, including a set of approved key performance indicators, will enable an assessment to be made of whether the IMI2 Joint Undertaking is achieving its objectives, with the Governing Board overseeing the work of the Executive Director and the Programme Office.

The external evaluation for the entire programme will be organised by the Commission. An interim evaluation will be carried out before the end of 2017 and a final evaluation after the conclusion of the programme in 2024.

Annex 10 Procedure comparison among the three JUs: FCH, IMI and Clean Sky

	ACTIVITIES	FCH	IMI	CS
JUs Organisation	Legal Form	Legally established in May 2008 as community body involving a PPP based on the principles of the EU Financial Regulations. Full autonomy in November 2010	Legally established in December 2007 as community body involving a PPP under the EU Financial Regulations. Full autonomy in November 2009	Legally established in December 2007 as a community body involving a PPP under the EU Financial Regulations. Full autonomy in November 2009
	Veto Right EC	YES (by failing consensus a majority of 9/12 is required and EC has an indivisible vote of 5/12)	<i>de jure</i> NO, <i>de facto</i> YES (decisions taken by a three-quarters majority and requiring the positive vote by the Founding Members)	YES
	Founding members	EU and 'Industry Grouping'. The Research Grouping became a member late in 2008.	EU and the European Federation of Pharmaceutical Industries and Associations (EFPIA)	EU and Industry consisting of 12 ITD leaders, 72 Associates (and 450 Partners).
	STAFFING	Authorised ceiling of 20 staff of which 18 posts assigned as of June 2013. 5 Project Managers responsible for approx. 150 projects and overloaded with a wide range of administrative functions and other functions dealing (directly or indirectly) with operational activities (financial, legal, audit and communication officers) Additional efficiencies resulting from internal reallocation of resources and sharing of horizontal services with other JUs are already exploited	Authorised maximum ceiling of 36 staff members reached in July 2012. 9 scientific managers for scientific activities +3 communications/external relations. In total 30 + 6 admin. assistants. 80% of staff resources are assigned to directly work or support operational activities.	Authorised ceiling of 24 8 Project officers; 75% of staff dealing with operational activity (technical and financial); 6 staff on horizontal support, e.g. Executive Director, Head of Admin, secretary, Internal Auditor; etc.
	Nr of calls for Proposals	6 in total, one yearly	11 in total (9- 11 to be launched in second half of 2013)	14 in total (one planned in July 2013)

	Nr of projects	estimated total of 150 for 2008-2013	40 signed (estimated 60 in total)	342 GAP (Grant Agreement for Partners) + 7 GAM (Grant Agreement for Members) = 349 projects signed (further 63 under negotiation and further 30 to be published) – Estimated final total -= 442 projects
	Nr of projects per PO	Approx. 25-30 projects/PO The Head of Programme does also manage 13 projects.	Each on-going project managed by a scientific & a finance officer. Projects' portfolio distributed among 9 scientific and 4 finance officers (7 projects per scientific officer on average).	1 GAM and 60 GAPs per PO on average
BUDGET	Funding for RTD	2008-2013: 940 M€ (including max 40 M € for running costs) Contribution on a 50/50 basis by the EC in cash and IG/RG(in-kind for operations and cash for running costs)	2 billion € (1 billion from EC in cash/ EFPIA companies contribute €1 billion in kind), including maximum €40 million contribution per Founding Member. Funding is distributed through open and competitive CfP following a peer reviewed two-stage process.	2008-2013: 780.26 M €. Contribution on a 50/50 basis by the EC (in cash) and the aeronautical industry (in-kind). ITD Leaders commit up to 400m €, Associates members up to 200 m € and Partners receive (through Call for Proposals) a minimum of 200m €.
AUDIT	Internal Audit	Commission's Internal Audit Service (IAS) Internal Audit Capability within the JU		
	External Audit	European Court of Auditors		
COORDINATION amongst JUs	Shared services & facilities	Logistics (building); Common IT infrastructure Shared approach on continuation of JU in H2020 legal basis and financial rules Regular coordination between Internal Audit Functions of the 3 JUs in place for issues of horizontal nature (e.g. audit methodology, approach towards the Court of Auditors). Audit services are also shared between JUs when it is the most cost-efficient solution (e.g. common framework contract on Ex-Post audits, joint engagements...)		
	Synergies/ commonalities	Informal general coordination at executive directors' level (quarterly) and Heads of Administration and Finance IT Governance Committee (quarterly meetings) Common framework contracts (e.g. ex-post audits, interim staffing, IT support) Coordination on case-by-case basis for communication / HR / legal matters /IT/ audits...		
	Planned common activities	2nd October 2013: JTIs joint conference and exhibit at European Parliament		

Potential services to be shared	IT	No objection within JU. There is already a shared IT service (outsourced to an external firm). IT officer of the JU chairs the IT Governance Committee & ensures coordination between JUs on common IT issues.	No objection within JU. Joint management of common infrastructure and services already in place.	No objection within JU Joint management of common infrastructure and services already in place.
	Internal audit	The JU has an Internal Audit Manager covering assurance (i.e. audits) and consulting services on risk management, governance aspects, reporting and ex-post audit . This internal solution with a multi-task approach is considered by FCH JU the most efficient solution to address the necessary 'assurance' and 'advisory' needs of the JU.	The JU has an Internal Audit Manager providing internal assurance and consulting services on governance, internal control, ex-post and risk management processes. This current arrangement, embedded within the JU's internal governance and internal control system is considered by the JU as essential and necessary as to ensure timely and efficient response to the 'assurance' and 'advisory' needs of the JU.	JU has Internal Audit Officer focusing on advisory services, risk assessment, ex-post audit process. "Internal" advisory function, partially management role. This internal solution is considered by CS as more effective. CS claims that the quality function within the JU is essential. Even if the internal audit could be shared, this internal knowledge and advisory role should be kept.
	Other administrative services	JU claims that a combination of "multi-task" of staff in a JU (HR & general affairs; legal & procurement; accounting & finance...) with coordination & cooperation on a case by case basis with other JUs is more efficient as it has the advantage of knowledge of the JU transactions, flexibility and business continuity	Enhanced cooperation and synergies in areas of support services (e.g. IT, HR, Finance) are desirable but remains to be further investigated based on impact analysis of centralisation of common support services by DG RTD for the Research family under Horizon 2020, workload and budget (including staffing level) for IMI2.	Some staff are performing 'multi-task' functions, e.g. the Assistant to the Director is the only person dealing with all HR matters for the JU; the Legal officer is combining the role of legal officer with procurement officer and Data Protection Officer and is also in charge with European Parliament relations; The internal audit function and quality management role are performed by the same person.
GOVERNANCE	Governing bodies	Same structure based on Governing Board and advisory bodies (SC, SRG/STAB, Stakeholder Assembly/Stakeholder Forum/General Forum)		
	Governing Board	The GB consists of the EC (5 members), the Industry Grouping (6 Members) and 1 member of the Research Grouping.	The GB consists of the EC (5 members) and EFPIA (5 Members)	The GB consists of the EC, 12 ITD leaders and 6 Associates (rotating representatives for associates)

	<p>Scope and functions of the SRG</p>	<p>SRG acts as advisory group and should interface with the relevant stakeholders in their respective countries.</p> <p>Around 10 members attend regularly. The Group meets at least bi-annually</p> <p>The chair attends as observer the GB meetings.</p> <p>Up to now there have been limited joint activities. There appear to be a strong interest in reviewing AIP and MAIP and in advising on the strategic orientation of the programme.</p>	<p>SRG acts as advisory group and as an interface with the relevant stakeholders in their respective countries. It is supposed to have an important role in liaising with the national programmes and helping in dissemination and outreach activities. SRG members shall act as IMI ambassadors/ multipliers.</p> <p>The SRG has been invited to propose experts and to contribute to the workshops related to the calls consultations prior to launch.</p> <p>SRG consulted on annual scientific priorities set out in AIPs.</p> <p>SRG proposes candidatures for the SC to be approved by the GB.</p>	<p>SRG acts as advisory group and as an interface with the relevant stakeholders in their respective countries. 14 members attend regularly. Some members are at the same time NCPs or/and members of the programme committee. The CS ED and the GB Chair attend the NSRG meetings and the Chair of the NSRG attends as an observer at the CS GB.</p> <p>During 2012, the NSRG met four times and was represented at the GB meetings.</p> <p>Members take a supportive role particularly in relating with the European Council and take part in information dissemination and Info days. They analyse the results of the calls.</p>
	<p>Scope and functions of the Scientific Committee SC/STAB</p>	<p>The SC (9 members from academia, industry and regulatory bodies) provides scientific advice on the R&D agenda (MAIP & AIP) and participates in the monitoring of the FCH JU programme by acting as experts in the annual Programme Review Days</p>	<p>The SC (15 members, including the EU regulatory agency EMA as observer) provides scientific advice to the Governing Board</p>	<p>The STAB (established in 2010) is involved in monitoring the progress of the 7 ITDs that comprise the technical content of the programme, largely through participation as Reviewers at the Annual Reviews and in the mid-year progress reviews and other reviews throughout the year. Each Board member is associated with the reviews of at least 2 ITDs and also serves to check the quality of the reports delivered by these ITDs. The STAB oversees all the reviews and produces (since 2012, at the ED's request) a synthesis of the annual reviews outcomes.</p>
	<p>Role and authority of exec. director</p>	<p>Chief executive responsible for the management and implementation of the JU programme in accordance with the decisions of the Governing Board. No</p>	<p>Chief executive responsible for the management and implementation of the JU programme in accordance with the</p>	<p>Chief executive responsible for the management and implementation of the JU in accordance with the decisions of the</p>

		system of delegation from the GB to the Exec. Director in place.	decisions of the Governing Board. Few discretionary decisions. A system of delegation from the GB to the Exec. Director for routine operations is envisioned.	Governing Board. CS is a programme, with a common set of objectives, cross-links between platforms, interfaces, priorities and management. The exec Director is in charge with it. The director has delegation for contracts signatures up to a predefined level.
SMEs	Support/ Involvement of SMEs/ CfP statistics	No dedicated PO focusing on SMEs. All POs do their best to involve as many SMEs in the projects. One seat in the JU-GB is reserved for SMEs (in practice, there are 2 SMEs seating in the GB) SME participation > than in FP (in 2008-2012 : 25% of the funding compared to 18% in FP7) >50% of the more than 60 members of the IG are SMEs	A Scientific officer has been tasked with focusing on SMEs and developed links with many SMEs associations. IMI Executive Office supports SMEs through info on IPR, a web based tool kit, advice on negotiating grants & project agreements, rules on financial reporting. SME are selected based on the needs of EFPIA consortium coordinators. In total, there are 141 SMEs participating in IMI projects (15.9% of total participants). 21.4% of IMI Calls funding is allocated to SMEs (Calls 1-8). Perceived benefits for SMEs: to work with large companies, who are potential clients. There has been a steady increase in SME participation in IMI consortia and in Eol.	No dedicated officer focusing on SMEs CfP participants: 38% SMEs winning in CfPs SMEs' share of funding earmarked for CfP (25% of EC contribution) amounts to 35%
	Financial restrictions/ red tape	As the FCH is not part of the guarantee fund it carries out a Financial Viability Check (eligibility for grant pre-financing) which may lead to requiring a guarantee or limiting the amount of pre-financing and this may appear difficult for some SMEs. Possibility to organise a workshop on the topic between FCH JU PO/ EC and SMEs who have been facing these issues.	No need for financial guarantee for SMEs, but a financial viability check is performed. The current IP-policy of the IMI is alleged to discourage the participation of SMEs.	No need for financial guarantee for SMEs, but a financial viability check is performed. SMEs can be mono-beneficiaries which contributes to the high percentage participation in CFPs

Participation in JU CfP procedures and regulations	FUNDING rate	<p>The funding rules are very close to the FP7 ones .The upper funding rates for direct costs are basically the FP7 ones, with the additional requirement of matching between EU funds and in-kind contribution from participants. This might lead to decreased funding rates with a 'correction factor' to be applied across the whole call. Funding rates may differ for each Call depending on 'correction factor' applied. This assessment is done after evaluation of each call and before starting negotiations.</p> <p>Indirect costs (overhead) can be declared based either on 'actual' or on a 'flat rate' model (EC validation system), but are reimbursed at a maximum fixed rate of 20% of the direct costs.</p>	<p>Eligibility for funding limited to academia, research institutes, patient organisations, regulators, SMEs</p> <p>75% RTD contribution to SMEs/academia and other IMI beneficiaries; 20% flat overhead rate or actual indirect costs</p> <p>For other activities , management and training, the IMI JU financial contribution may reach a maximum of 100% of the total eligible costs</p> <p>EFPIA companies contribute with in-kind or cash contribution and are not reimbursed.</p>	<p>The single entity applying is eligible for either 50% or 75% depending on the legal status (for example industry or SME); in case of a consortium, both funding criteria will apply and the resulting funding will be an average of the two percentages, weighted by the actual contributions of each partner.</p> <p>The existing members are only eligible for 50% funding if they are winners of CFPs</p> <p>Budget distribution:</p> <ul style="list-style-type: none"> ▪ Up to : 400 M €: leaders ▪ Up to 200M € : associates ▪ At least 200 M€ CfP <p>Average funding rate in Calls: 65.6%</p> <p>Applicants success rate: 35%</p>
	Rules for participation/ Requirements for consortia	<p>Similar as in FP7 (3 legal entities from at least 3 MS or associated countries etc.) with one addition: at least one member of the consortium must be member either of the IG or of the RG</p>	<p>Two-stage process. In Stage 1 applicants (at least 2 legal entities eligible for IMI funding) submit EoI for joining a consortium of EFPIA member companies. In Stage 2 the successful applicants and EFPIA consortium (at least 2 EFPIA companies) are invited to submit a full proposal. With the 4th revision of the IMI model Grant Agreement, IMI projects have been provided with additional flexibility:</p> <ul style="list-style-type: none"> - to launch competitive calls for the addition of new beneficiaries to on-going projects - for setting up synergies with other on-going IMI collaborative research projects. 	<p>Most of RDD&TD are performed by the Members of CS whose activities are covered by Grant Agreements for Members (GAM). There is one amendment to the GAM per year and per ITD which specifies work plan, resources and budget. Subcontractors are selected by Members through Calls for Tender. Part of the CS programme using 25% of the EC contribution is performed by Partners selected through CfP. Successful CfPs lead to the signature of Grant Agreement for Partners (GAP). Average GAP duration is 20 months.</p> <p>There are also mono-beneficiaries. CS does not require a consortium as a constraint; even a single entity can apply.</p>

	<p>Financial regulations</p>	<p>In-kind contributions ('matching rule')</p> <p>Procedure in use (based on GB approved methodology) to verify that the in-kind contributions provided by the JU participants' match the cash contribution from the EU.</p> <p>The 'correction factor' is the main tool 'to steer' the matching between EU funds and in-kind contribution from the participants, in order to comply with the requirement of the Regulation by the end of the Programme.</p> <p>The verification of the in-kind contributions reported by the participants in the cost claims (CCs) is done at three levels: (1) ex-ante review of 100% of CCs by the JU, (2) audit certificates carried out by beneficiaries' auditors for CCs above pre-defined thresholds and (3) Ex-Post audits by the JU on a sample basis.</p> <p>Assessment and reporting. In addition, FCH JU Council Regulation (art 12.7) requests an independent auditor to assess the level of in-kind contributions on an annual basis and report the results by April of N+1. Since the autonomy of the JU, two annual assessments have been carried out.</p>		<p>In Kind contribution</p> <p>Procedure in use to verify that Members' in-kind contributions to IMI match the cash contribution from the EC. EFPIA in kind contribution is monitored through different levels, Call, Grant agreements, ex-ante and ex-post audits.</p> <p>A limited amount of in kind contribution from outside the EU and associated countries can now account for industry matching contribution. This relates to up to 10% of the global contribution for standard projects within a global cap of 5% of the total industry contribution.</p> <p>For projects of special interest to the EU and society, such as antimicrobial resistance, there is no maximum limit by project but a maximum limit of 30% of the total in kind contribution.</p>		<p>In Kind contribution</p> <p>There is a procedure in use to verify that Members' in-kind contributions to CS match the cash contribution from the EC. The verification is carried out at 3 levels, by audits inside the Members' organizations when preparing their Form C (annual cost claim), by a CS ex-ante check before payment on the basis of the documents provided (which includes a document of audit procedures to be carried out above 200k threshold per claim) and by an ex-post audit of Members' expenses against the specified GAM activities.</p> <p>CS Financial Regulations only allow for either 20% flat rate without justification or real overheads, there is nothing in between.</p>	
	<p>Time to grant Time to pay</p>	<p>Target in H2020</p> <p>< 180 days from evaluation < 90 days</p>	<p>Present</p> <p>Between 341 and 411 days 365 days in 2011 < 90 days</p>	<p>Target in H2020</p> <p>< 180 days from evaluation < 90 days</p>	<p>Present</p> <p>Evaluation process was streamlined. Time to grant (reduction from 400 days in call 3 to 185 days in call 6).</p>	<p>Target in H2020</p> <p>< 180 days from evaluation</p>	<p>Present</p> <p>Latest calls: < 240 days from call publication to GAP 360 days on average for grant signed in 2012</p>

BUDGET	Flexibility	"N+3" rule gives the possibility to re-enter in the budget cancelled appropriations from previous years and this is effectively used	Certain flexibility available. Possibility to shift budget according to the " 3-years rule"	Certain flexibility available. Possibility to shift budget according to the " 3-years rule. Transfer from SAGE ITD to GRA ITD. Internal changes in all ITDs as % share. A ITD (SGO) made available 2.5 M€ to JU which was redistributed to other ITDs; budget flexibility works for CS projects in a timely way.
Coordination with National Programmes & Collaborative Research		Cooperation with national programmes (NOW in Germany and Danish FCH programme); involvement in UK H2 Mobility, possibly in future French H2 Mobility	Enhanced cooperation with SRG, which is consulted on annual scientific priorities and proposal Cfp text prior to launch Interactions with JPI through CfP pre-launch consultation.	Cooperation with NSRG, providing visibility on the CS programme and especially CFPs. Limited or partial interaction about synergies with national programmes.
KPIs/ Metrics		KPIs on: (1) operational aspects linked to Calls/ projects; (2) control aspects encompassing the grant management cycle (e.g. % of complaints on the evaluation process, financial impact of the negotiation process, % of payments made on time, Ex-Post audits: coverage and error rates). The JU reports annually the resulting KPIs in the Annual Activity Report) Concerning project and technology metrics, the on-going project TEMONAS should provide a TEchnology MONitoring and ASsessment tool combining S-O-A methodology and IT-implementation. The tool is tailored for the needs of programme progress evaluation and should enable a targeted comparison and evaluation of project results and achievements in an objective way. The tool has still to be provided to the JU (project finished in	KPIs were initially developed in 2011 and reported to the Governing Board from 2012. In 2013, a dedicated IT tool has been developed to facilitate tracking of project data and the generation of "classical" metrics. This tool is operational and allows reporting a series of metrics from June 2013. In the future, data generated by the IT-tool will be used to generate a Balanced Scorecard that should facilitate the governance of the partnership. Regular release of bibliometric data. Agreement with Thompson Reuters to devise metrics for the analysis of scientific publications. Metrics are derived from scientific reports and interim reviews.	Internal Quality management encompassing internal control standards, KPIs and a system of various TRL. For ITDs indicators include: - Budget vs. planned, - Deliverables/TRL gates/ other milestones/ on time vs. delayed - Risk status per technology/sub system - TRL passed during the quarter - % of review recommendation fulfilled at next Annual Review KPIs related to CfP process include: - topic failure rate, time to contract, SME rate KPIs related to GAPs include: - topic success, eligible proposals, contracts signed on time, delay of final reports - Actual resources consumption of ITDs - SME participation and funding Specific case of TE, providing monitoring and assessment of the improvement in

		May 2013) and has to be filled in with project results data, plus literature data for benchmarking. It is expected to start providing reports in 2014.		environmental impact of aviation (CO ₂ ; NO _x ; noise) due to the maturity of the technologies being developed and demonstrated in CS: this in terms of sectors (by conceptual aircraft types integrating the suitable technologies) at mission, airport and ATS levels.
IPR		<p>The IPR rules are identical to FP7 (foreground and background). The IPR details are agreed between beneficiaries in the mandatory Consortium Agreement.</p> <p>They have to accommodate the interests of a wide range of stakeholders from large companies to SMEs and researchers in different application areas.</p>	<p>Although based on FP7, IPR rules have been adapted to the objectives of IMI and provide flexibility to IMI consortia to reach the most appropriate agreements (e.g. definition of background; scope of research use of results, access rights to third parties after project's end, etc.) .</p> <p>Agreement on IP management shall be reached upfront before the start date of each concerned IMI project.</p> <p>IPR rules are sometimes perceived to act as a barrier for SME participation</p>	<p>IPR rules – same as in FP7 are implemented in both GAMs and GAPs</p> <p>The Foreground, (results generated by the project), is the property of the beneficiary carrying out the work generating that Foreground. Indeed, beneficiaries are not subcontractors of the CS-JU, so IPRs are not the property of the Topic manager or of the CS-JU.</p> <p>Where several beneficiaries have jointly carried out work generating foreground and where their respective share of the work cannot be ascertained, they shall have joint ownership of such foreground.</p> <ul style="list-style-type: none"> • Transfer of ownership can be defined. • A plan for the use and dissemination of foreground needs to be prepared, including patent applications and use of the results.
Quality control	Technical/ scientific reviews	<p>Projects are monitored by the POs (after each reporting period) and (with assistance from external experts) during mid-term review meetings and final meetings when needed Feed-back is provided to beneficiaries for better steering the project in the next period.</p> <p>In parallel an assessment of the program is performed annually via the Programme Review Days.</p>	Scientific officers and external experts, including members from the Scientific Committee.	<p>Review performed by JU with external experts and STAB.</p> <p>Technology evaluator providing monitoring and assessment of the improvement in environmental impact (CO₂; NO_x; noise)</p>

	<p>Ex post audit</p> <p>Error rate threshold allowed by the European Court of Auditors: 2%</p>	<p>Ex-Post audits of beneficiaries are regularly launched in line with the Audit Strategy adopted by the Governing Board.</p> <p>To date, 48 audits have been launched of which 20 are concluded. 97.6% of the errors in favour of the FCH JU detected in the concluded audits have been corrected by the JU. This leads to a residual error rate (i.e. error rate after corrections) of 1.67%, below the Court's threshold (i.e. 2%).</p> <p>In addition to the corrective measures above, two main preventive measures have been established by the JU to reduce the probability of errors occurring and/or being undetected, i.e. (1) communication campaigns to provide guidance to beneficiaries and (2) reinforcement of JU's ex-ante controls.</p>	<p>Ex-post audits of beneficiaries are regularly launched in accordance with the Ex-Post Audit Strategy adopted by the Governing Board. To date, 90 audits of beneficiaries have been launched of which 56 have been concluded, indicating to date an error rate above threshold of 2%. Recovery and corrective actions are now being taken (where possible with offsetting against next payments). In addition, as preventive measures IMI has continue to reinforce its ex-ante controls and has provided training and guidance to beneficiaries, which has appeared very important to reduce errors especially with the many participants that are SMEs or unfamiliar with FP7 rules.</p>	<p>In 2011 and 2012 ex-post audits of financial statements of CSJU beneficiaries have been implemented in line with the Ex-post Audit Strategy adopted by the Governing Board. To date, 65 audits have been launched, out of which 52 have been finalised. Audit results have been implemented (i.e. overpayments were recovered) with more than 96%. The residual error rate, reflecting the remaining errors in favour of the JU - after corrective measures have been taken place- passed from 4.22% in 2011 to 1.29% in 2012, resulting in an accumulated rate of 2.77.</p> <p>In order to reduce the error rates, the JU has put efforts in improving its ex-ante validation process and has provided extensive guidance to its beneficiaries concerning the eligibility of costs for the CS projects.</p>
<p>Continuation in H 2020</p>		<p>Proposed by the Commission</p>	<p>Proposed by the Commission</p>	<p>Proposed by the Commission</p>

