



Innovative Medicines Initiative

Da IMI a IMI2

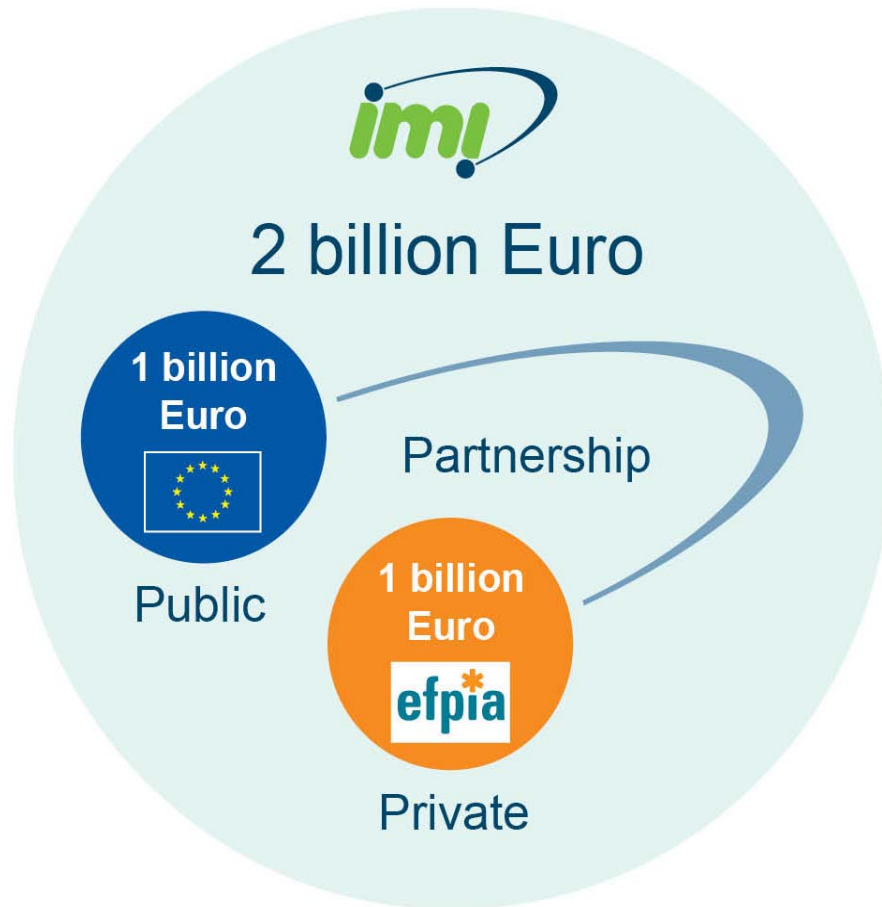
Elisabetta Vaudano

Info Day 2014

Innovative Medicines Initiative: *Joining Forces In The Healthcare Sector*



Innovative Medicines Initiative



The largest European public/private partnership in Life Science :

- Launched in 2009, last (11) Call December 2013
- To make drug R&D processes in Europe more **innovative** and **efficient**
- To enhance Europe's **competitiveness**
- To address key **societal challenges**

Features:

- 1:1 funding, joint decision making
- All EU funds go to SMEs, academia, patient organisations and regulatory agencies
- Large pharmaceutical industry, represented by EFPIA, contributes in-kind

The IMI Portfolio



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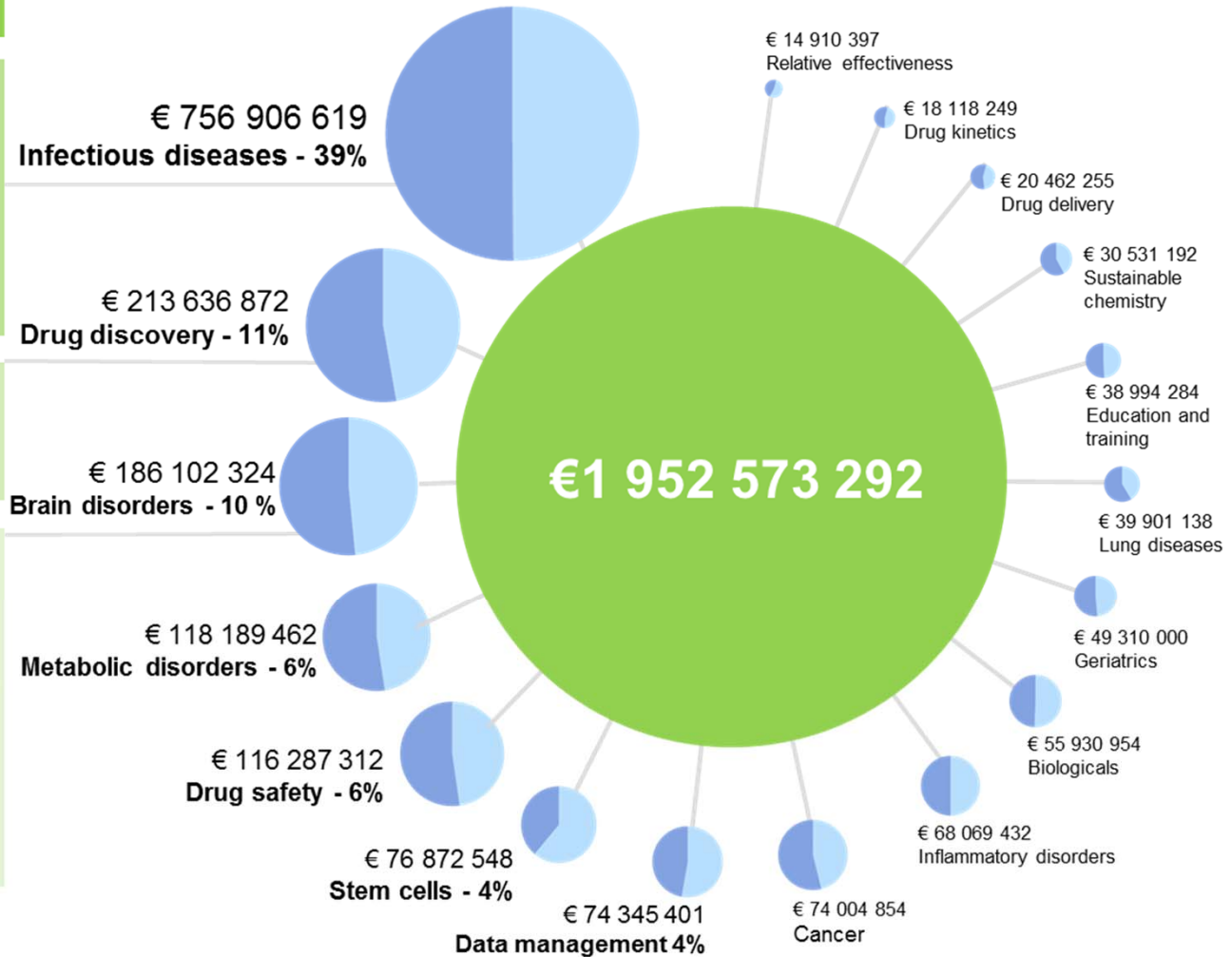
■ Corporate contribution ■ IMI funding

Partners

AiCuris	Johnson&Johnson
Animal Health	Medimmune
Division of Sanofi	Merck
Astellas	Merck Sharp & Dohme Corp
AstraZeneca	Merial
Basilea	Novartis
BoehringerIngelheim	Pfizer
Cubist	Rempex
GSK	Sanofi
Janssen	

AstraZeneca	Novartis
Bayer	Pfizer
Janssen	Sanofi
Lundbeck	UCB
Merck	

Abbott	Janssen
AbbVie	Lundbeck
AC IMMUNE	Merck
Amgen	Novartis
Astellas	NOVO NORDISK
AstraZeneca	Orion Corporation
BIOGEN IDEC	Pfizer
BoehringerIngelheim	Roche
Eisai	Sanofi
Eli Lilly	SERVIER
ESTEVE	UCB
Grunenthal	Vifor
GSK	



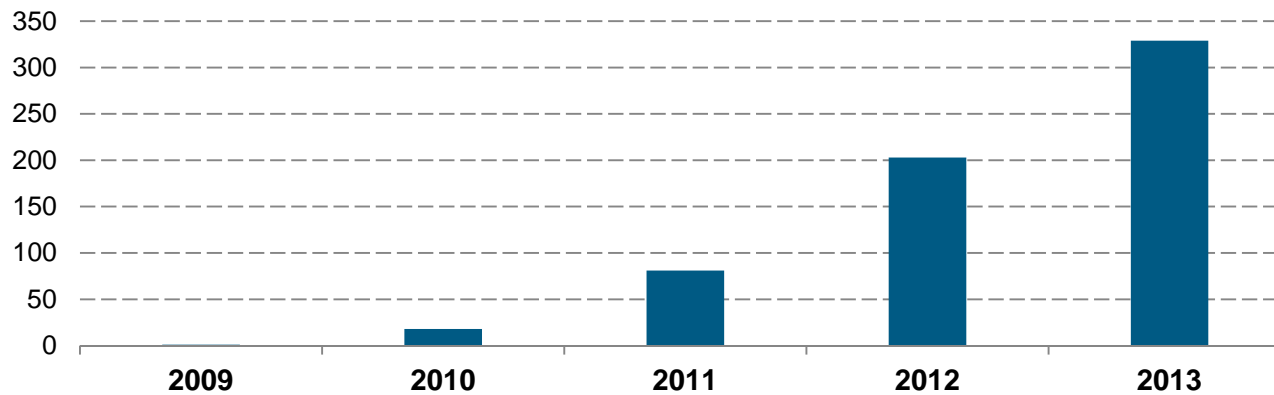
IMI Scientific Output



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708 PUBLICATIONS **3709** CITATIONS **2.04** CITATION IMPACT **19%** HIGHLY CITED

Number of publications per year



Making A Difference



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Implementation of project results inside industry

Project	Area	Results description
IMIDIA	diabetes	The human beta cell line EndoC BetaH1 has been validated by Endocells and 3 pharma partners confirming their initial insulin secretion capacity. These cells have been successfully transferred as a research tool for drug discovery to industrial partners.
DDMORE	knowledge management	Several drug/disease models identified by DDMORE are adopted or further developed inside the industry.
eTRIKS	knowledge management	Adoption of the eTRIKS results, TransMART system deployments in 5 pharmaceutical companies.
EUROPAIN	Chronic pain	Preclinical model of spontaneous pain in rodents has been developed, standardized, validated, and is already used for internal decision making in the drug development process. The ultraviolet B (UVB) pain model has also started to be used for in house R&D.

Impact On Regulatory Framework



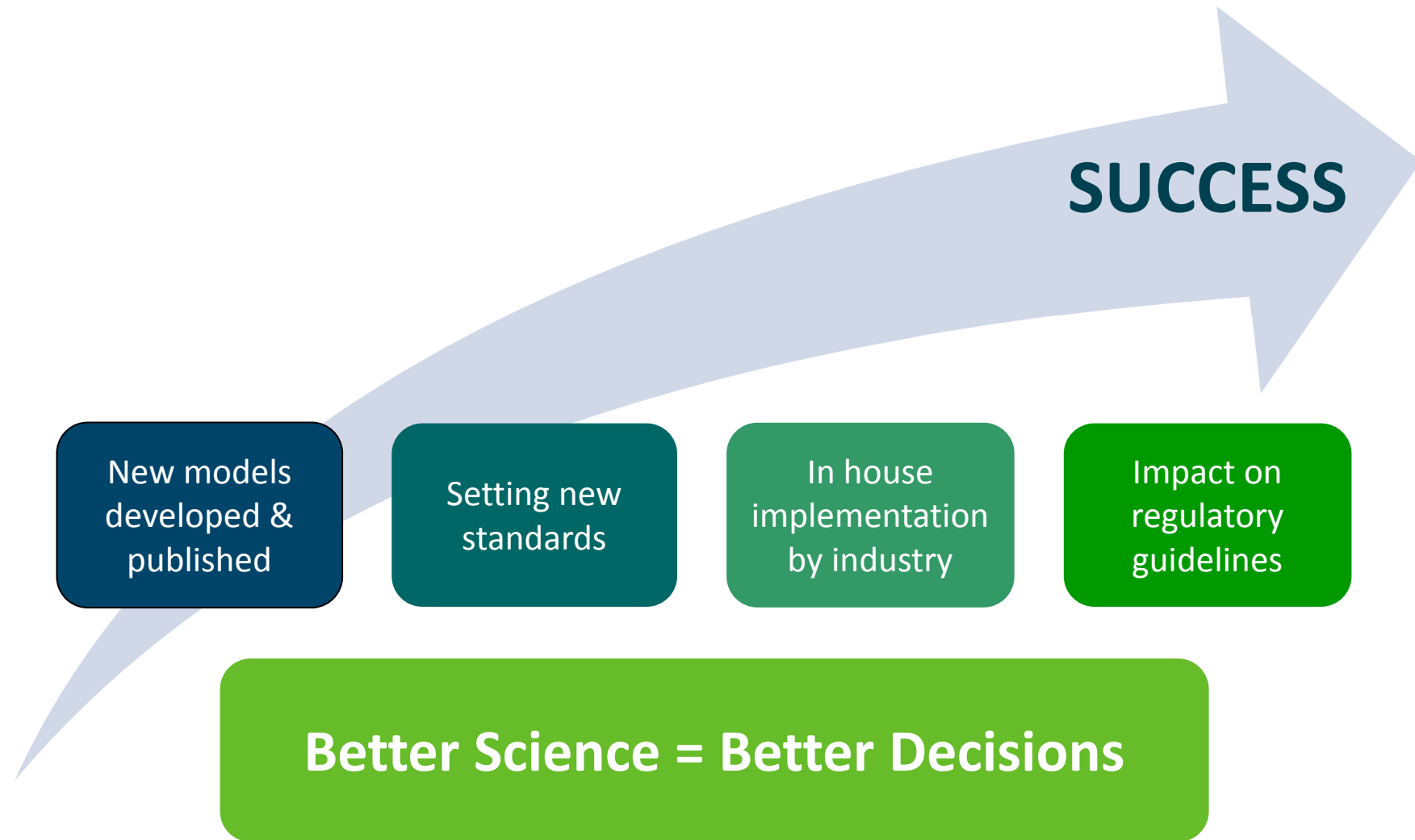
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Project	Area	Results description
PROactive	COPD	Qualification Advice completed at the EMA
EU-AIMS	autism	Started EMA formal scientific advice procedure for qualification of 5 biomarkers in ASD
eTOX	drug safety	Provided an update on the eTOX database and the prediction system to the CHMP Safety Working Party (SWP) at EMA. Scientific Advice Procedure was initiated.
MARCAR	cancer	Has developed new biomarkers, technologies, and alternative test systems that help explain or predict animal and/or human carcinogenic pathways and mechanisms for non-genotoxic carcinogenesis. This will provide enhanced scientific rationale for Carcinogenicity Assessment Document (CAD) submissions, with potential impact for ICH S1 carcinogenicity testing guideline revisions.
Safe-T	drug safety	Developed and now progressed towards an aligned EMA/FDA qualification a set of novel safety biomarkers for drug-induced kidney, liver, and vascular injury.
DDMORE	knowledge management	In May 2012 an advisory meeting with EMA and FDA representatives was held. Through a Modelling Review Group , DDMoRe is in regular contact with both the EMA and FDA regarding the qualification of the content of the project's Model Library.

The Measures Of Success



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Innovative Medicines Initiative: *Key Principles*



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- “Non-competitive” collaborative research for EFPIA pharma companies
- Competitive calls to select partners of EFPIA companies
- Open collaboration in public-private consortia (data sharing, dissemination of results)

The Role Of The Programme Office



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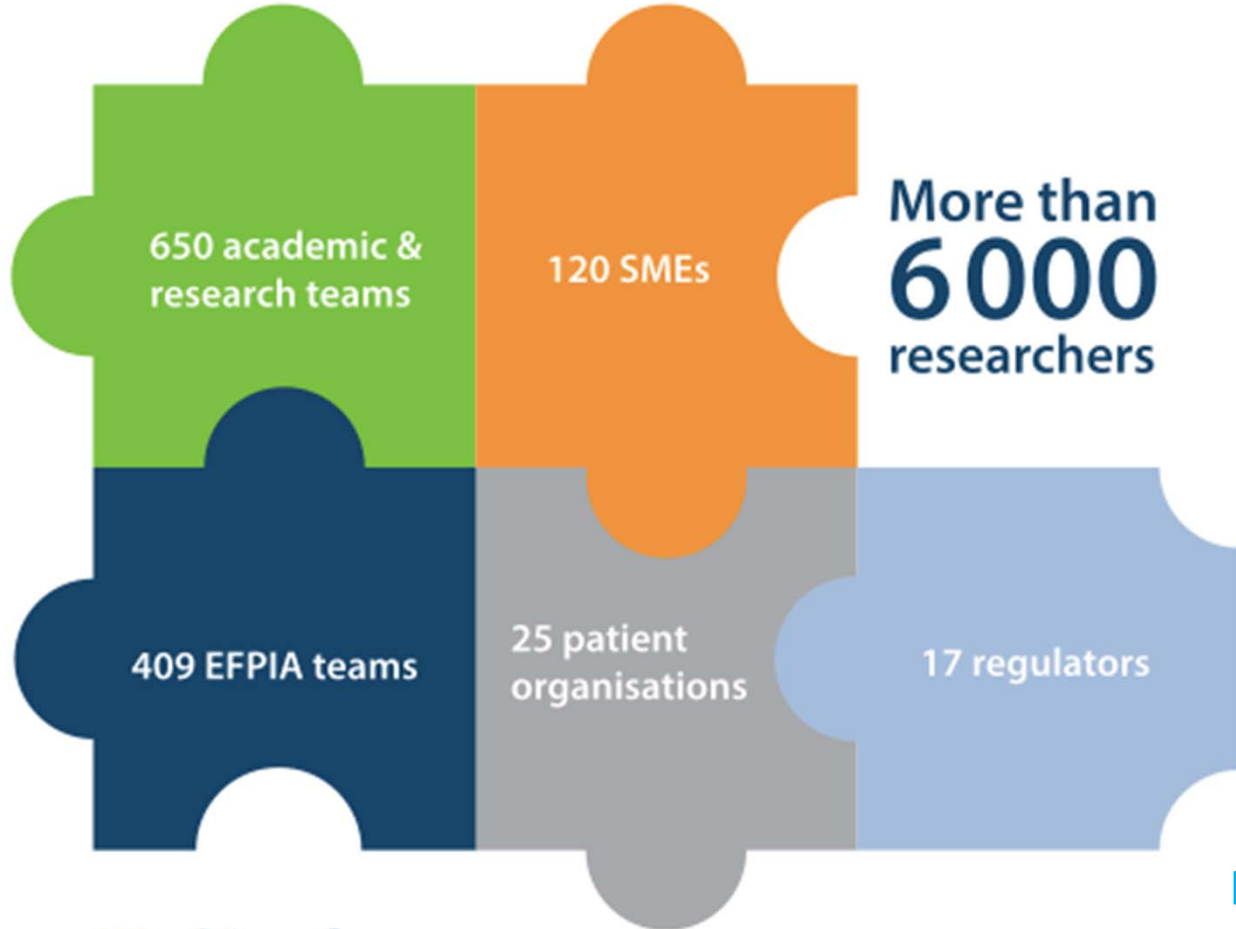
A neutral broker:

- To **implement** programmes and activities in the **common interest** of **all** stakeholders
- To **monitor** the use of public funds and industry investment
- To **guarantee** fair and reasonable conditions for optimal knowledge exploitation and dissemination
- To **facilitate** the interaction between stakeholders, including Intellectual Property agreements
- To actively **communicate** and promote IMI and its activities

Innovative Medicines Initiative: Creating Multi-stakeholder Team-Works



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61% of projects reported some form of **PATIENT INVOLVEMENT**

REGULATORS ON BOARD OF **12** PROJECTS

50% of projects have **REGULATORY AUTHORITIES** representatives in Scientific Advisory Boards

Working for

- Collective intelligence networks
- Improved R&D productivity
- Innovative approaches for unmet medical needs



SME Participation In IMI Projects (Up To 8th Call)



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Total IMI commitment	€ 723 million
Total received by SMEs	€ 133 million
% SME	18.4%
Total IMI participations	886
Total SME participations	135
% SME	15%

SME Success Stories



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SME involved in **SAFE-T** project

“Thanks to IMI our company went from **6 to 50 employees**.
Now we are ready to go to further expand.”



SME involved in **IMIDIA** project –

“1st product released to the market in 2013 – **IMI was instrumental in validation of the first cell line product**, 2nd product release planned this year, 3rd diagnostic product in development.

In preparation: **a new patent filing** to protect technologies for the creation of third generation human beta cell lines.



SME involved in **PharmaCog** project

“We are developing a blood panel for AD for diagnosis, stratification and companion diagnostics in AD. **The Panel was tested on 300 patients in IMI project**”



SME involved in **eTOX** project

“We have developed in silico models for predicting toxicity, which were validated by pharmas in eTOX. Now **we have signed a contract with one of the companies to use our models in house.**”



- IMI makes efforts to enhance **patient centric approach**

Patient dedicated workshops

Involving **patients at all levels**

Providing **forum for discussion**

- IMI best practice examples:

EUPATI

U-BIOPRED

PROactive

Key collaborative activity areas:

Diabetes, CNS disorders, Tuberculosis, Patient Reported Outcomes, Cancer, Preclinical Safety and Education & Training.

IMI projects have signed

14

MEMORANDA of UNDERSTANDING
with other international consortia

IMI signed horizontal agreements with:

Critical Path, Juvenile Diabetes Research Foundation as well as
with **Clinical Data Interchange Standards Consortium**.

Verso IMI2

The Premises

- Alignment with **Horizon 2020** objectives of the Health challenge
- Addressing healthcare priorities identified by the WHO 2013 report on priority medicines for Europe and the world
- Strategic Research Agenda aimed at progressing the vision of personalised medicines, for both prevention and treatment
- Collaboration across sectors to harness all knowledge and technologies which can contribute to IMI2 vision - diagnostics, imaging, IT, medical devices, ...

IMI2 - *Broad Participation To Achieve Ambitious Goals:*



Bigger budget: 3.275 Billion Euro, equally shared by EU and other partners

- **Not limited to EFPIA members:** open for other industries and organisations, which can contribute to the PPP goals (Healthcare IT, medical devices,...) giving them the opportunity to establish their own projects, or join forces. Dedicated budget (213 millions Euro)
- The principle of in kind/cash contribution matched by IMI funding for public beneficiaries **will be retained** and **extended** beyond EFPIA members to all associated partners.

Strategic Research Agenda



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Comprehensive framework
for a 10-year programme

Prepared with input from
80+ organisations

Project ideas from industry
and third parties will be
screened against it

<http://goo.gl/jqMP9g>



The right prevention and treatment
for the right patient at the right time
Strategic Research Agenda for
Innovative Medicines Initiative 2



efpia

efpia
European Federation of Pharmaceutical
Industries and Associations

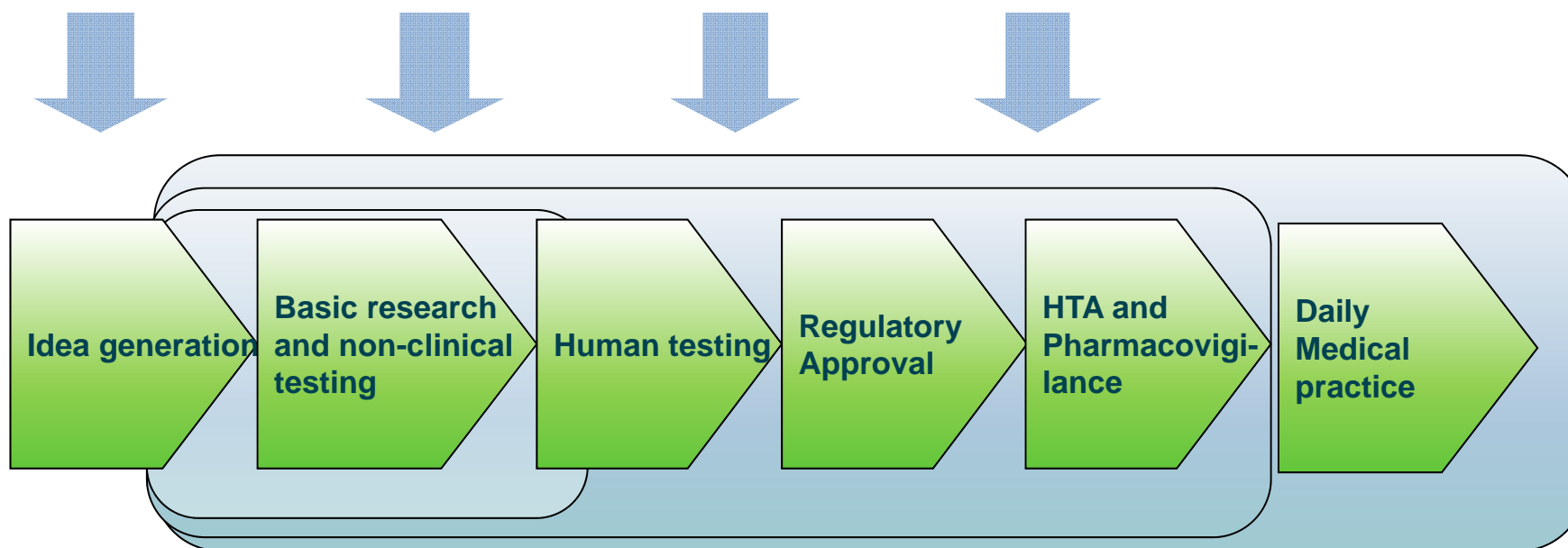
ve Vaccines Europe
An industry for healthy lives

ebe
European
Biopharmaceutical
Enterprise

imi
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The Evolution Of IMI: From Bottlenecks In Industry – To Bottlenecks In Industry And Society

Make Drug R&D processes in Europe more efficient and effective and enhance Europe's competitiveness in the Pharma sector

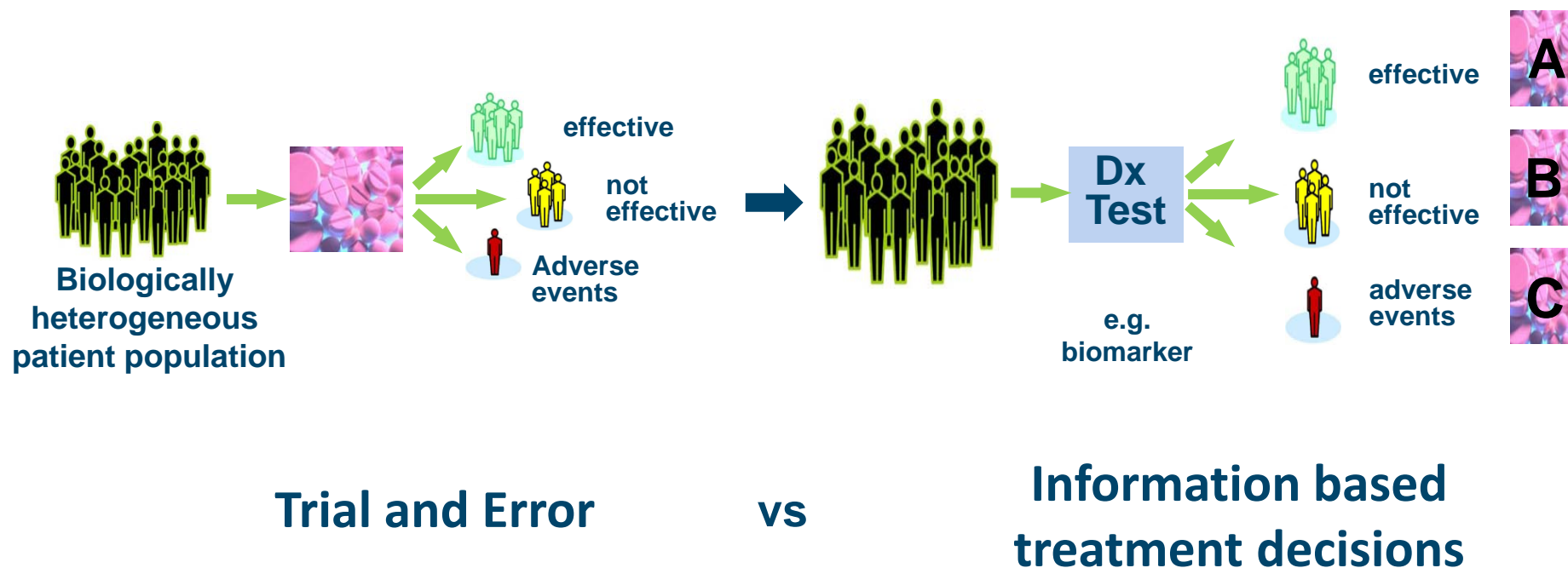


Primary focus of early IMI calls
2007 SRA

Shift to also addressing challenges in society and healthcare
2011 SRA

IMI 2 includes real life medical practice
2013 SRA

The Vision For IMI2 – *The Right Prevention And Treatment For The Right Patient At The Right Time*



IMI2: The First Call



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Two topics:

Translational approaches to disease modifying therapy of type 1 diabetes mellitus (T1DM)

Discovery and validation of novel endpoints in dry age-related macular degeneration and diabetic retinopathy

.

Translational Approaches To T1DM: Background

- A chronic disease affecting worldwide around 17 Million people and with **highest incidence rate in Europe** (~ 22 / 100.000/ year), with **major regional differences**.
- The **incidence** of childhood T1DM is reported to be **rising rapidly** worldwide, especially in the under 5 year old age group.
- T1DM is generally seen today as an **autoimmune disease**, but **its cause is unknown** (genetic susceptibility, diabetogenic trigger(s) and/or exposure to a driving antigen).
- The disease is currently **not preventable and no cure** is available. The only available pharmacotherapy for T1DM patients is the lifelong injection of insulin.

Translational Approaches To T1DM: Aims and Objectives



Better Disease Biology and Translational Medicine (Target & Biomarker Identification)

- Generation of a high quality and comprehensive European network of clinical and translational research centres (providing a prospective clinical trials database for T1DM) including at risk and early T1DM patients.
- Establishment of systematic large-data repository enabling extensive cross functional data mining and integrated data analysis
- Phenotypical characterization (in silico based on medical records as well as active through experimental medical studies)
- Systematic prospective and retrospective launch of broad “-omics” characterization of human biological samples
- Development and characterization of the most appropriate preclinical T1DM model(s) for discovery of novel clinical therapies.

Translational Approaches To T1DM: Aims & Objectives

Innovative clinical trial paradigms for preventative and disease modification trials in T1DM.

- Development of standardized entry criteria and endpoints for T1DM trials (both metabolic and immune profiles) with participation of patient advocacy groups, and regulatory authorities.
- Implementation of the use of electronic data capture devices to collect an array of “real world data”
- Testing and development of novel bio-statistical methodologies applicable to new compositions of relevant end points for T1DM clinical trials.
- Evaluation of novel mono- and combination approaches (i.e. combining multiple immune modulatory approaches, immune cell migration modification, immune tolerance inducers, β -cell enhancing therapeutics) in people with T1DM.

Translational Approaches To T1DM: Key Deliverables

- **An improved understanding** of the immunological and beta cell biology aspects of T1DM to disentangle its heterogeneity both in at risk and early diagnosed patients and for staging participants in future T1DM clinical trials.
- **The development** of novel and relevant endpoints & readouts for T1DM clinical trial based on clinical & standardised molecular “real world data” obtained from T1DM patients, and on the application of novel bio-statistical methodologies.
- **Pre-clinical T1DM models** with improved translational value.
- **Improved understanding** of the human T1DM disease biology and **optimised clinical trial setting** to allow testing novel mono- and combination approaches in T1DM.

Translational Approaches To T1DM: Key Facts



EFPIA PARTICIPANTS AND ASSOCIATED PARTNERS

Sanofi (coordinator), Juvenile Diabetes Research Foundation (JDRF) (co-coordinator), Leona M. and Harry B. Helmsley Charitable Trust, Novo Nordisk, Eli Lilly, GSK.

DURATION OF THE PROJECT

The indicative duration of the project is 84 month (7 years).

BUDGET

Total budget of just over €35 million,

H2020 €17.6 million

€12.6 million EFPIA, €2.8 million JDRF, €2.2 Leona M. and Harry B. Helmsley Charitable Trust



Translational Approaches To T1DM: What we Are Looking For



APPLICANT CONSORTIUM

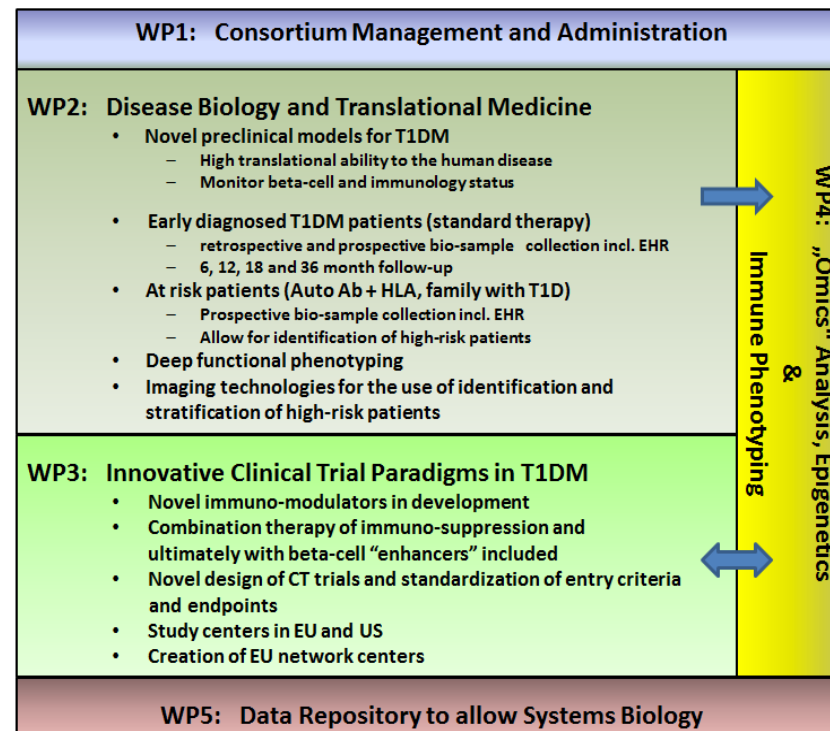
- Academic endocrine clinics and associated supporting departments
- Basic, translational, and clinical researchers from the fields of T1DM autoimmunity and β -cell biology
- Drug discovery and medical staff in Pharmaceutical Industry and Small and Medium size Enterprises
- Hands-on data base specialists and big data managers
- Patient organizations/representatives
- Experts in regulatory science and health technology assessment preferably representing European health authorities.

The project will be expected to establish a T1DM Patient Advisory Committee

Translational Approaches To T1DM: Suggested Work Plan



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To be included:

- A plan for interactions with Regulatory Agencies/Health Technology Assessment bodies with relevant milestones and appropriate resource allocation
- Attention to data standardisation/use of standards (e.g. CDISC)
- Synergies with other EU and global initiatives, including IMI projects

Novel Endpoints For Retinal Diseases



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- Retinal diseases **among leading causes** of blindness worldwide
 - Age-related macular degeneration (AMD): Early form reported to occur in 30% of the population of 75 years and above (**over 50% by age 80**); late form in 4 - 8% of the population over 70 years
 - Approximately **93 million** affected by diabetic retinopathy (DR) in 2010
- **Limited treatment options** for dry form of AMD or DR
- **Major development hurdles:** lack of suitable endpoints for early exploratory and pivotal clinical trials, lack of predictive markers and models

Novel Endpoints For Retinal Diseases: Aims & Objectives

To evaluate novel endpoint candidates for dry AMD and DR:

- technical, medical and health economic appropriateness
- bridging preclinical and clinical studies.

Methods in scope:

- Visual function testing beyond Best Corrected Visual Acuity (BCVA)
- Electrophysiology
- Imaging methods to assess retinal structure
- Soluble and genetics biomarkers
- Patient reported outcome tools and Quality of Life-related endpoints
- A combination of these methods.

Novel Endpoints For Retinal Diseases: Key Deliverables

Generation of robust data resulting from retrospective and/or prospective studies as basis for discussion of regulatory acceptability of the endpoints for future clinical programmes.

It is expected that the proposed research program delivers data on:

- Technical evaluation of methods (validity, repeatability, reliability, interpretability, translatability and acceptability by patients)
- Development of novel methods and tools
- Clinical validation of methods/tools in patient studies for dry AMD & DR
- Collection of biomarkers for selection of high risk populations
- Synergies between dry AMD and DR vs condition-specific aspects

Novel Endpoints For Retinal Diseases: Key Facts



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EFPIA PHARMA PARTICIPANTS AND OTHER PARTNERS

Bayer HealthCare (coordinator), Sanofi, Novo Nordisk, Zeiss

DURATION OF THE PROJECT

The indicative duration of the project is 60 month (5 years).

BUDGET

Total budget €14 million

H2020 €7 million

EFPIA €7 million



Novel Endpoints For Retinal Diseases: What We Are Looking For

Setting-up & running of studies required to meet topic's objectives

Multidisciplinary applicant consortium with a track record of

- Clinical expertise in ophthalmology
- Clinical research experience
- Access to patients and databases
- Public health expertise
- Health economic expertise
- Understanding of pre-clinical models in ophthalmology
- Biomarkers
- Data management
- Regulatory, ethics, patients and project management

•

Novel Endpoints For Retinal Diseases: Suggested Work Plan

- Architecture for the full proposal to be suggested by the Applicant consortium
- Intention to set-up of an Advisory panel to the Consortium comprising payers, regulatory agencies and other relevant expert advisors
- Plan for interactions with Regulatory Agencies/Health Technology Assessment bodies expected
- Attention to data standardisation/use of standards (e.g. CDISC)
- Synergies with other EU and global initiatives, including IMI projects

Towards IMI2

Regole & Processi

A Single Set Of Rules



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➤ Covering all H2020 research and innovation actions

➤ IMI 2 fully aligned with H2020, but where needed flexibility was kept:

- Eligible entities
- IP

IMI2 life cycle



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- **Call definition**
 - Scientific Research Agenda
 - SGGs
 - Consultation SRG/SC
 - GB approval
- **Call Launch**
- **Call Evaluation by external experts**
 - Normally, 2 stages (industry joins 2nd stage)
- **GA signature, Project implementation and reporting**

Participation Rules

Attracting Stakeholders



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- Any entity carrying out work relevant to the IMI2 objectives in a Member State or Associated Country
- Anyone else
 - when foreseen in the annual work plan / Calls documents
 - with the agreement of the IMI JU

BUT

Not all participating entities are eligible for funding

More Entities Eligible For Funding



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- Academic institutions
 - Non-profit organizations
 - Small & medium-size enterprises
 - **Mid-sized enterprises**
 - Non-profit patient organizations
 - Non-profit public bodies and intergovernmental organizations including specialized agencies
- established in a Member State or associated country**

Simplified Conditions For Participation



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- **Minimum conditions for standard collaborative actions**

At least three legal entities each established in a different Member State or an associated country

- **Additional conditions**

In the annual work plan (and Call documents)

Funding rules

One Single Funding Rate Per Project



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One project = One rate

For all beneficiaries and all activities in the grant.

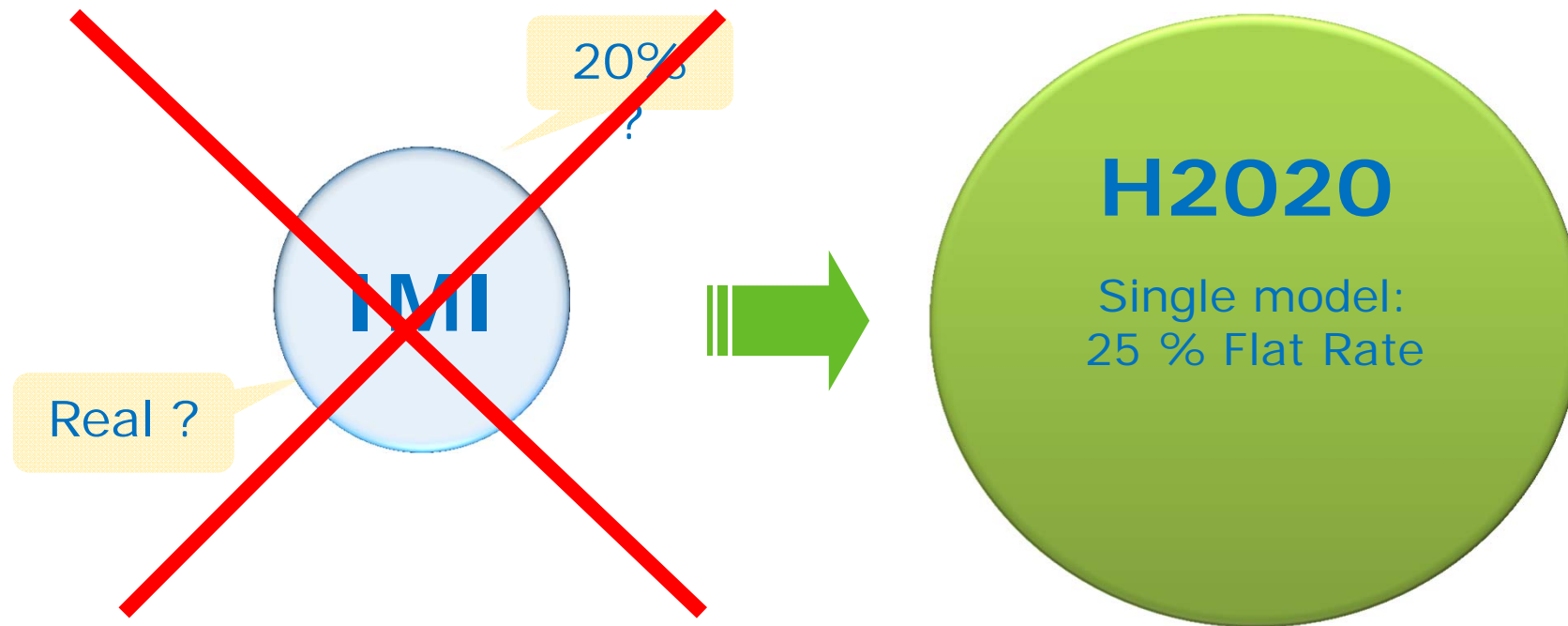
Defined in the annual work plan/Call documents:

- Up to 100 % of the eligible costs;
- but limited to a maximum of 70 % for innovation projects (exception for non-profit organisations - maximum of 100%).

One Single Indirect Cost Model



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A New More Attractive Funding Model:



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An example

 <i>Majority of beneficiaries</i>	Direct costs	Indirect costs	Total costs	% EU contribution	EU contribution
Flat-rate (20%) (or actual)	100	20	120	75%	€ 90

HORIZON 2020	Direct costs	Indirect costs	Total costs	% EU contribution	EU contribution
100/25 Funding	100	25	125	100%	€ 125

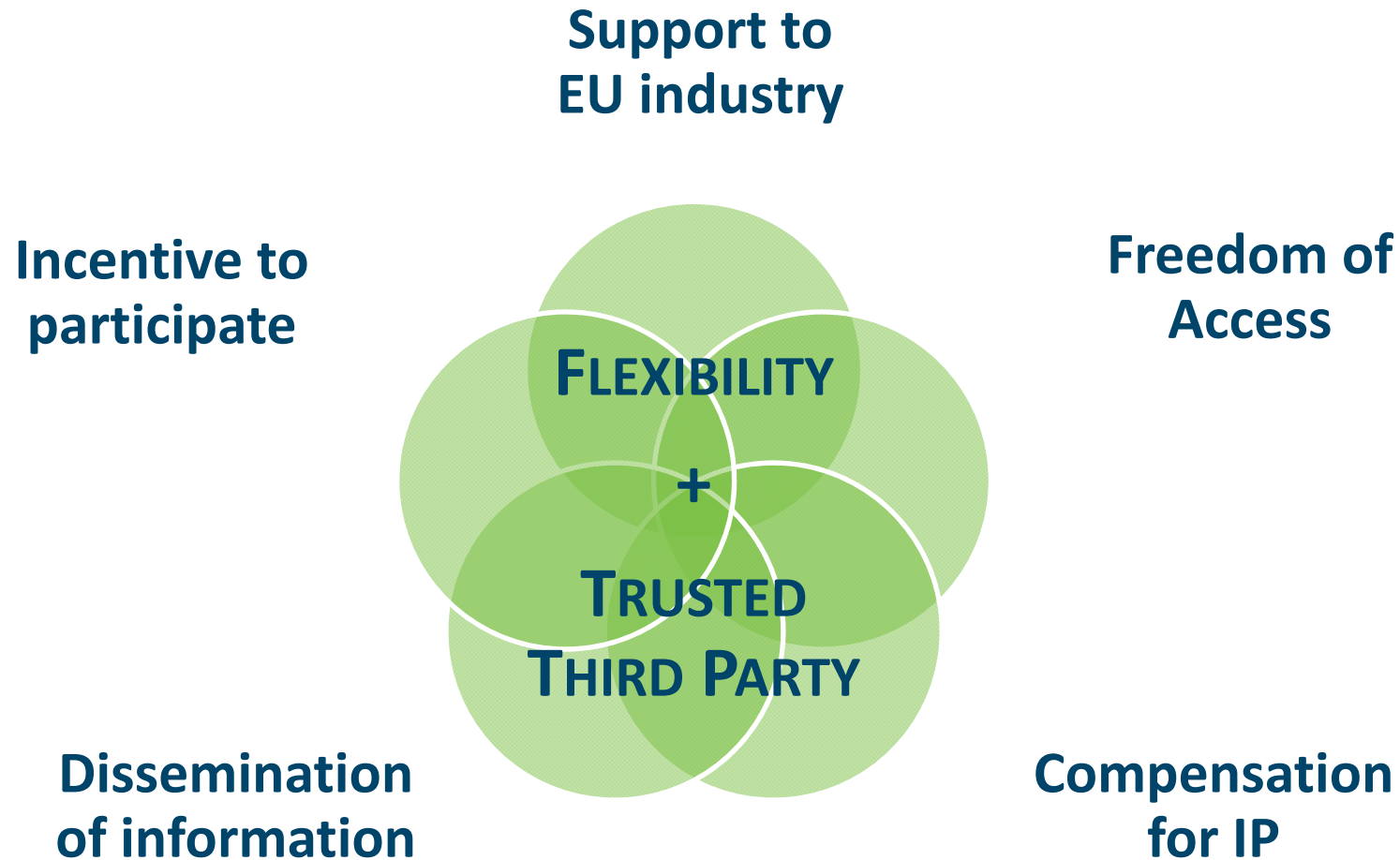
In-kind (And Cash) Contribution

- EFPIA companies
- Other industries and partners (when Associated Partners to IMI2)
 - Actual direct and indirect costs or average FTE + cash contributions
 - Based on the usual management principles and accounting practices
 - Contributions from affiliated entities as part of in-kind

When relevant to IMI2 objectives, up to 30% in-kind contribution from 3rd countries (Not associated to H2020)

Intellectual Property Rules

One Set Of Rules For Multiple Interests



Dissemination Modalities



Each beneficiary has the obligation to disseminate its own results

As soon as reasonably practicable

NEW for publications: Open access is mandatory

Mandatory mention to IMI support & EFPIA in-kind contribution all communications/ patent applications

From Call to Grant award

One Single Set Of Evaluation Criteria



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- Two stages evaluation: only “Excellence” and “Impact” considered at stage 1
- Thresholds and weighting in the Call documents
 - **New** Each proposal evaluated 'as it is', not as 'what could be'

Keeping The Momentum



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A maximum Time To Grant of 8 months

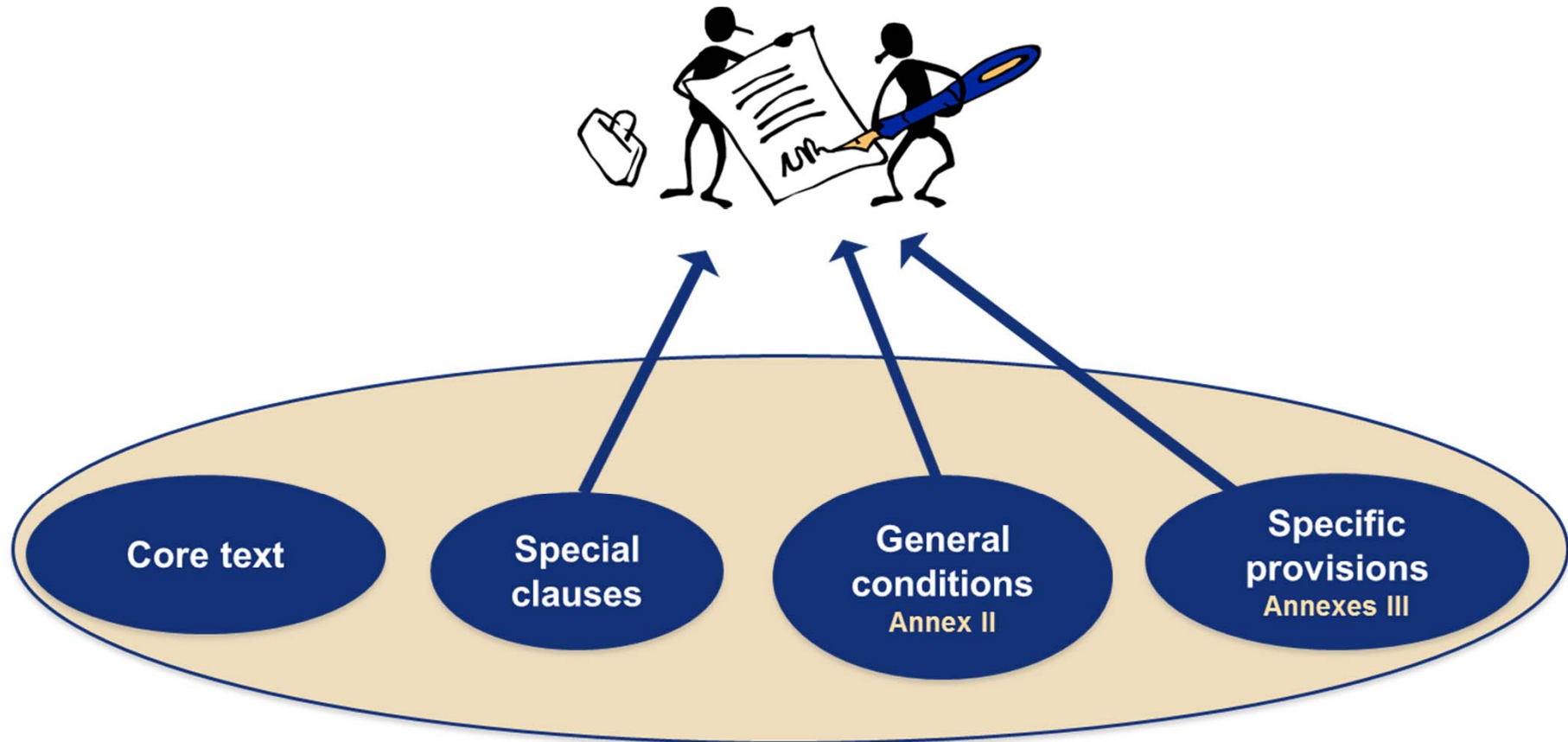


3 months
for signature
of GA

NEW

Legal entity validated in parallel

Simplified Model Grant Agreement



Writing a successful proposal

Common Mistakes

- **Eligibility criteria not met:**
 - submission deadline missed
 - **a single legal entity is not a consortium**
 - parts of the proposal not uploaded (this should not be a problem anymore with SOFIA)
 - submitted text does not respect the proposal template (sometimes received even slides!)
 - **proposal out of scope** (if you have doubts on how to respond to the Call contact us)

Common Mistakes

- Applicants do not have the capabilities to address all of the objectives or there is redundancy between partners
- Submitted text so concise that it **does not clearly state what is proposed** in practice
- The proposal **does not address all the objectives** (in some cases proposals have nothing to do with the topic!)
- **Ethical issues** not addressed

- **Read all the Call-relevant material that is provided on the IMI website – www.imi.europa.eu**
- Understand **IMI's Rules** and respect them
- **If in doubt ask** a member of the Programme Office
- Take advantage of Webinars and other information events: network early!!!
- Your proposal should provide **reviewers** with all the information requested to allow them to evaluate it as it is.
- **Finalise your submission**

Submitting A Proposal



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The screenshot shows a web browser window with the URL <https://sofia.imi.europa.eu/Pages/Login.aspx> highlighted in an orange box. The page features the IMI logo and a banner image of scientists. Navigation links include [Log In], Helpdesk, Request Access, and Forgot your password?. Below the banner is a 'LOG IN' section with a form for email and password, a 'Keep me logged in' checkbox, and a 'Log In' button. A link for 'Forgot your password?' is also present. At the bottom, a footer contains the text 'The Innovative Medicines Initiative (IMI) is Europe's largest public-private initiative aiming to speed up the development of better and safer medicines for patients.' and 'Specific Privacy Statement [SOFIA: Submission OF Information Application](#) Copyright © 2013 IMI.', with the latter highlighted in an orange box.



www.imi.europa.eu

H2020 Participant Portal



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(A-Z) Sitemap About this site Contact Legal Notice English



RESEARCH & INNOVATION

Participant Portal

European Commission > Research & Innovation > Participant Portal > Home

HOME

FUNDING OPPORTUNITIES

HOW TO PARTICIPATE

EXPERTS

SUPPORT



LOGIN



REGISTER

Welcome to the Research and Innovation Participant Portal

About the Participant Portal:

The Participant Portal is your entry point for the electronic administration of EU-funded research and innovation projects. It hosts services for managing proposals and projects throughout their lifecycle.

The Participant Portal supports activities funded mainly by the following EU programmes:

- **7th Framework Programme for Research and Technological Development (FP7)**
- **Competiveness and Innovation Framework Programme (CIP)**

Using the Participant Portal:

As a guest user, browse the public pages to:

- search for funding opportunities
- download guidance and legal documents
- search for the participant identification code (PIC) of an organisation
- contact the FP7 support services and browse the FAQ for guidance on the Participant Portal tools

As a registered user, benefit from personalised services for proposal submission, negotiation, and project management.



WHAT'S NEW



FUNDING OPPORTUNITIES



HOW TO PARTICIPATE?



WORK AS AN EXPERT



ACCESS MY PERSONAL AREA



INFORMATION AND SUPPORT

More information

- **H2020 Rules for Participation**
- **IMI2 Delegated Regulation**
- **IMI2 Call Documents (*soon*)**
- **IMI2 model Grant Agreement (*soon*)**
- **IMI2 annotated Grant Agreement (*soon*)**

www.imi.europa.eu/content/documents

Webinar Schedule

- **Translational approaches to disease modifying therapy of Type 1 Diabetes Mellitus (T1DM)**
Friday, 11 July 2014, 14:00 – 16:00 CEST (Brussels time)
- **New procedures, rules, guidance & IPR for Calls for proposals**
Monday, 14 July 2014, 14:00 – 16:00 CEST (Brussels time)
Closed. Second date: Wednesday 3 September 2014, 14:30 – 16:00 CEST (Brussels time)
- **Discovery and validation of novel endpoints in dry age-related macular degeneration and diabetic retinopathy**
Tuesday, 15 July 2014, 16:00 – 18:00 CEST (Brussels time)

IMI2 Info Day:

Crowne Plaza Hotel, Brussels, Tuesday 30 September 2014.



The day will include an overview of IMI 2's funding and intellectual property (IP) rules, tips on applying for funding under IMI 2, and workshops and presentations of the IMI 2-Call 1 Call topics by the topic writer. In addition, there will be plenty of networking opportunities and IMI staff will be on hand to answer questions relating to the new Call topics and the IMI 2 application procedures.

IMI warmly encourages small and medium-sized enterprises, mid-cap businesses, patient organisations, regulatory authorities, academic teams, industry, hospitals and other organisations with an interest in IMI projects to take part in the Open Info Day.



Your Contact Points

- Contact the Programme Office
infodesk@imi.europa.eu
- Get in touch with your **local IMI contact point**
www.imi.europa.eu/content/states-representatives-groups
- Talk to your **Health National Contact Point (NCP)**

Stay In Touch

- Visit our website
www.imi.europa.eu
- Sign up to our newsletter
bit.ly/IMInewsletter
- Follow us on Twitter
[@IMI_JU](https://twitter.com/IMI_JU)
- Join our LinkedIn group
bit.ly/LinkedInIMI
- E-mail us
infodesk@imi.europa.eu



Thank You

Questions?