Die Strategische Forschungsagenda der IMI2 und Ausblick auf Call 1 und 2

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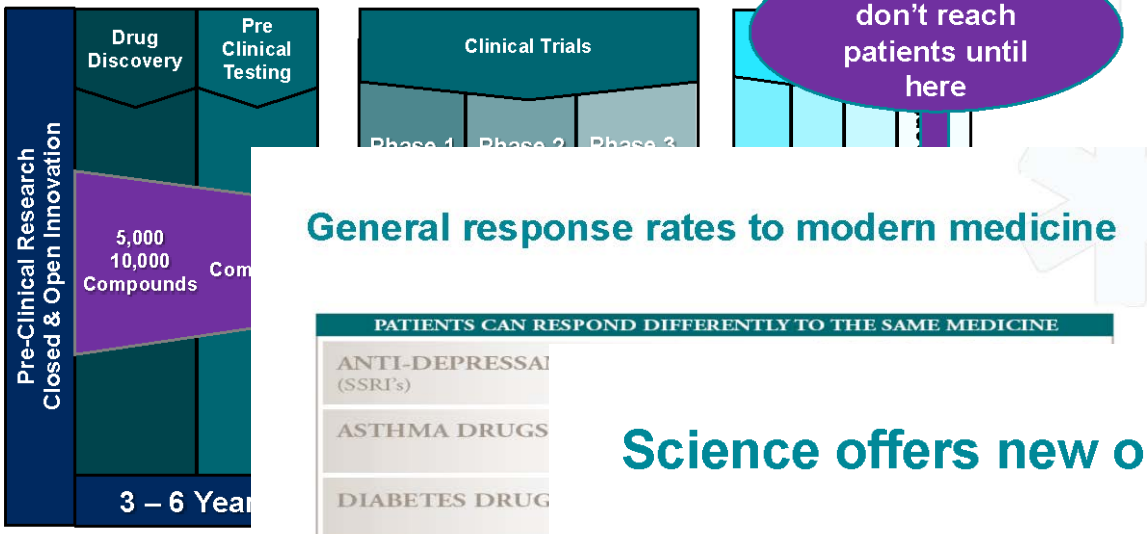
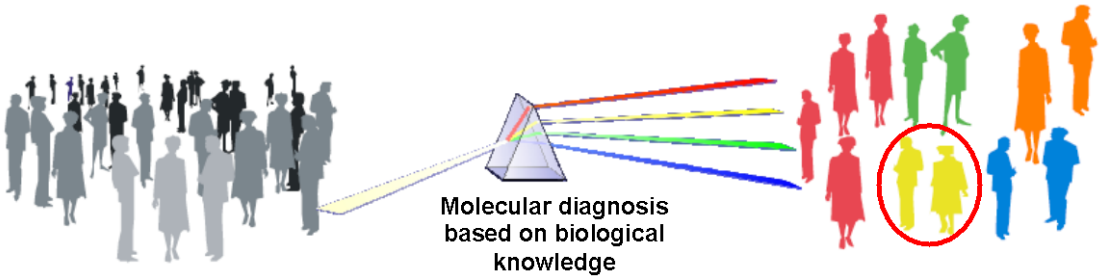
European Federation of Pharmaceutical Industries and Associations
www.efpia.eu

Current EU pathways are expensive and slow in getting new therapies to patients



General response rates to modern medicine

Science offers new opportunities



We treat a population.
Some respond and some don't

We treat a *targeted* population
They all respond

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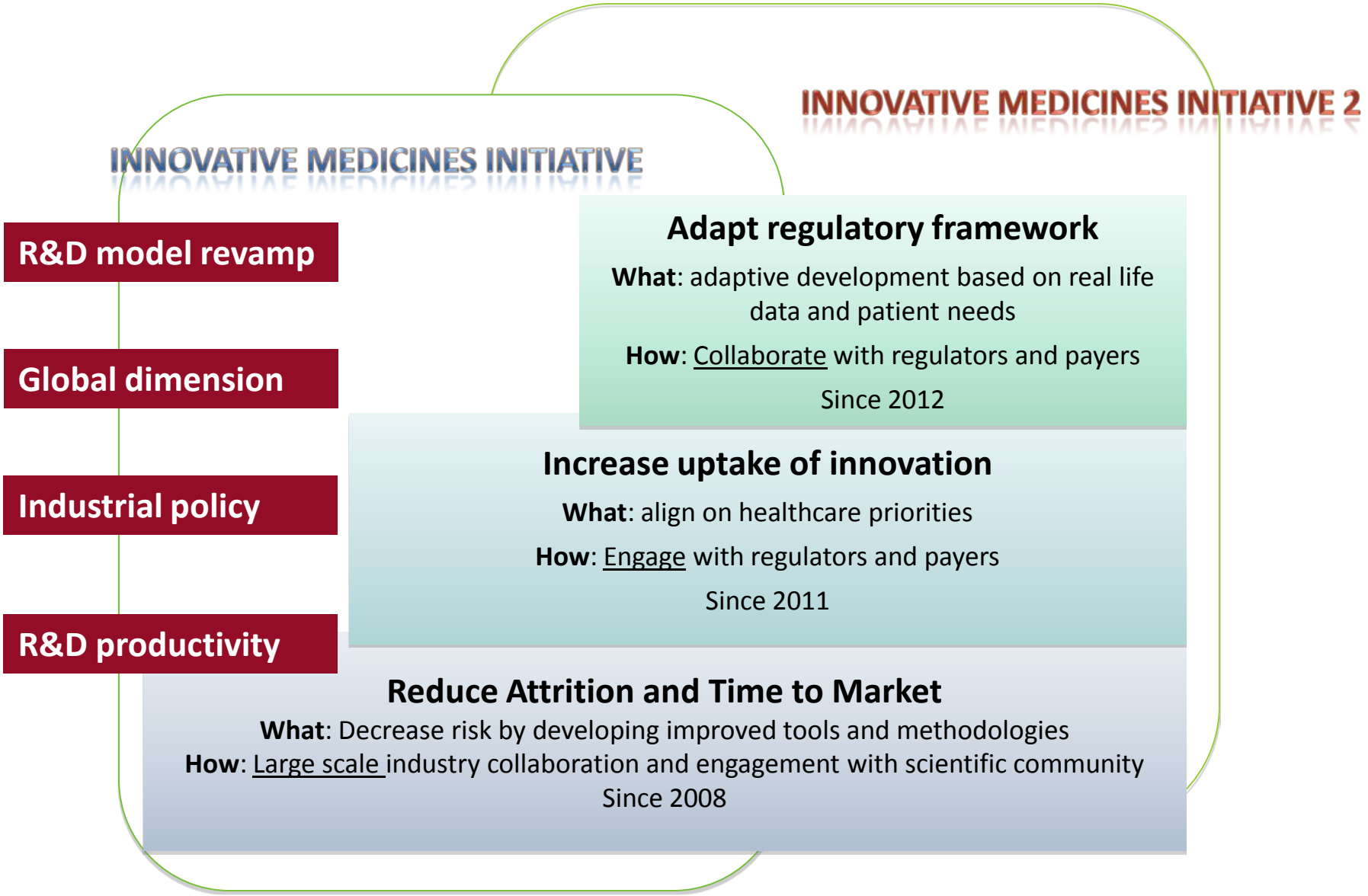
IMI - tool for implementation of research, regulatory and industrial policy agendas



Objectives of IMI2 – what the Regulation says

- increase the success rate in clinical trials
- where possible, reduce the time to reach clinical proof of concept in medicine development
- develop new therapies for diseases for which there is a high unmet need and limited market incentives
- develop diagnostic and treatment biomarkers for diseases clearly linked to clinical relevance and approved by regulators;
- reduce the failure rate of vaccine candidates in phase III clinical trials through new biomarkers for initial efficacy and safety checks;
- provide support for the development of tools, standards and approaches to assess efficacy, safety and quality of regulated health products.

IMI2 Strategic Research Agenda (SRA)

Comprehensive framework for a 10-year programme

Prepared with input from 80+ organisations (internet and targeted)

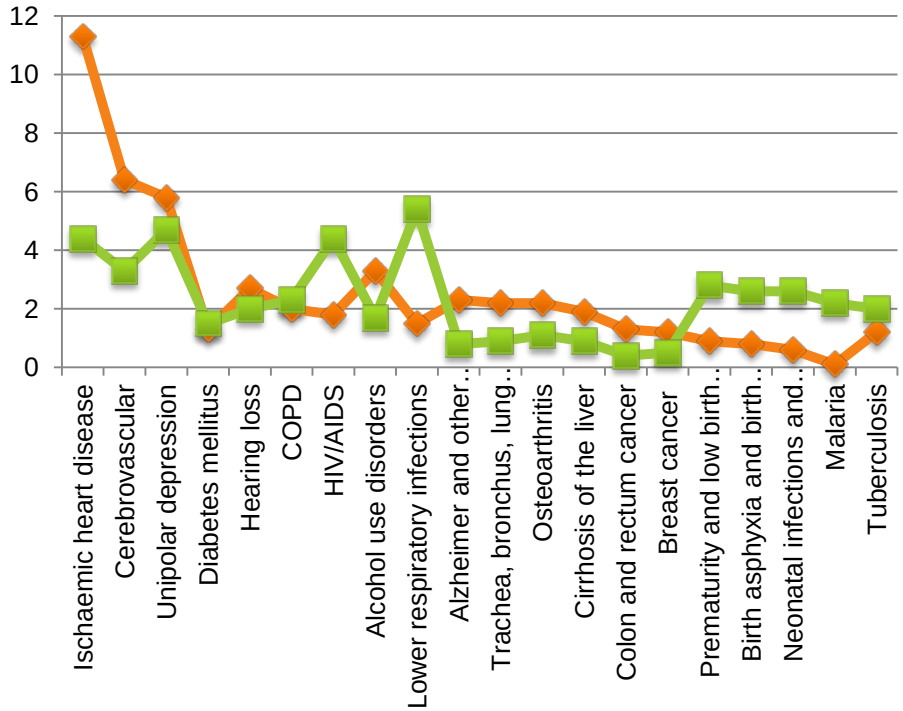
Project ideas from industry and third parties will be screened against it

http://goo.gl/jqMP9g

Therapeutic areas covered by the IMI2 SRA

WHO 2013 report on priority medicines for Europe and the World

Percentage of DALYs for top 20 high burden diseases and conditions



Therapeutic Areas in IMI2 SRA

(no priority order)

6. EUROPEAN HEALTH PRIORITIES

- 6.1. Antimicrobial resistance
- 6.2. Osteoarthritis
- 6.3. Cardiovascular diseases
- 6.4. Diabetes
- 6.5. Neurodegenerative diseases
- 6.6. Psychiatric diseases
- 6.7. Respiratory diseases
- 6.8. Immune-mediated diseases
- 6.9. Ageing-associated diseases
- 6.10. Cancer
- 6.11. Rare/Orphan Diseases
- 6.12. Vaccines

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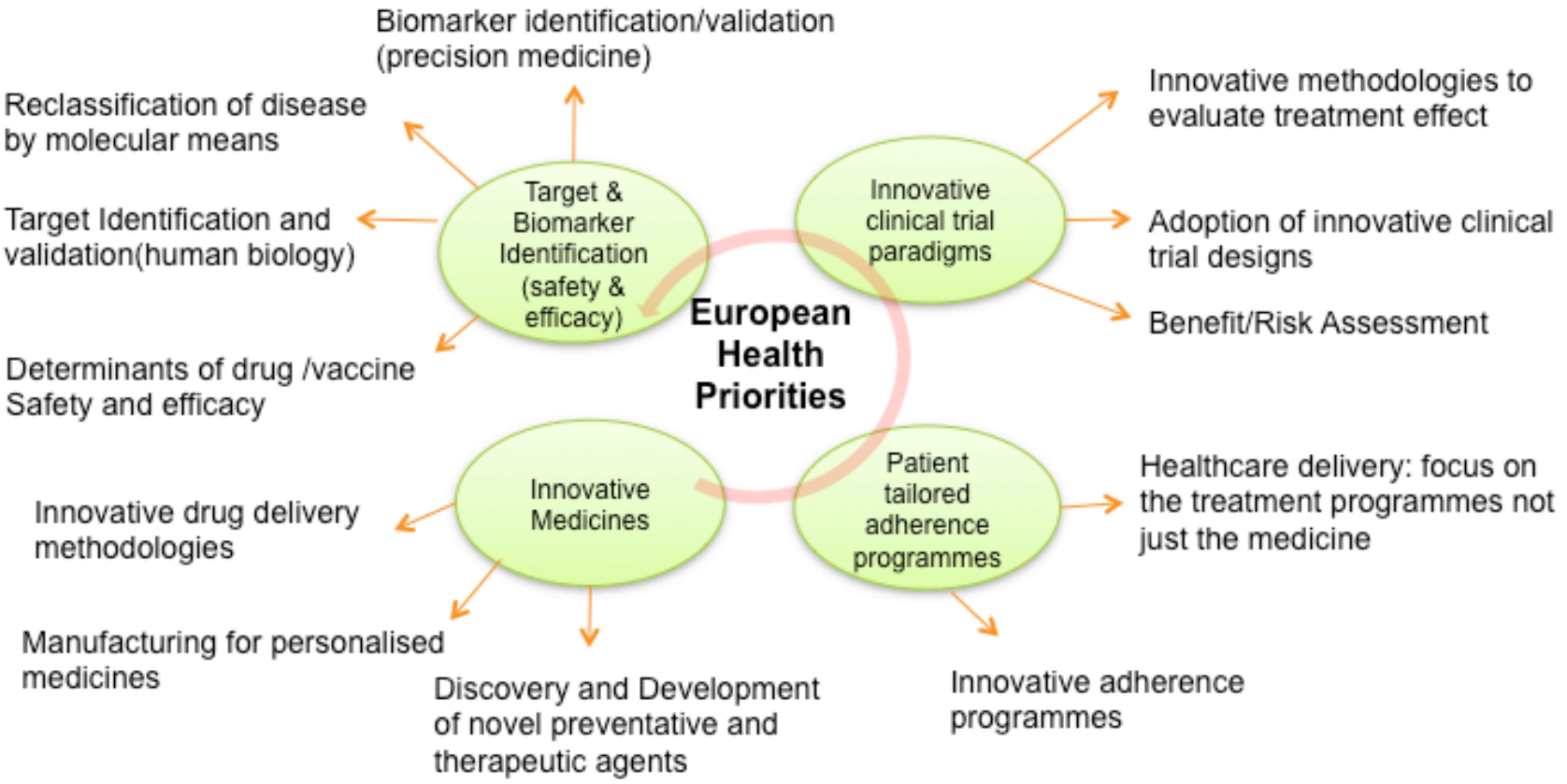
The right prevention and treatment to right patient at the right time



Drive change in delivery of medical practice

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IMI2 scientific programme: the need for focus

Therapeutic Areas and Cross-cutting Themes

1. **Neuro-degeneration**
   - Successfully prevent and treat dementia and other neurodegenerative diseases
2. **Prevention and treatment of immune-mediated disease**
   Advance immunological understanding to deliver new treatments and develop new and better vaccines for non-infectious diseases
3. **Metabolic disorders**
   - Tackle all phases of disease and its complications, including prevention and early interception
4. **Infection control**
   - Address multidrug resistance and create incentives for reinvestment (including antimicrobials, antivirals, vaccines) and develop new and better prophylactic vaccines
5. **Translational Safety**
   - Identification of predictors of safety and development of point of care for safety biomarkers and development of new human biology platform to predict toxicity and safety during early drug development

Differentiating Enablers for all themes

Towards early and effective patient access to innovative prevention and treatment solutions (MAPPs):

- Target validation based on human biology
- Stratified medicine, precision medicine
- Innovation in clinical trials
- Data generation and interpretation (knowledge management)
- Prevention, disease interception, patient adherence (incl. societal acceptance of vaccines)
- Effect on medical practice and outcomes (health/disease management)
- Regulatory framework (including pharmacovigilance)
- Patient access

First projects under IMI2

- Current projects for 1st Call – **launched 9 July**
  - Translational approaches to disease modifying therapy of Type 1 Diabetes Mellitus
  - Discovery and validation of novel endpoints in retinal diseases

- Postponed to 2nd Call
  - RADAR: Remote assessment of disease and relapse (**real world data - in support to all priority areas and new R&D models)**

Call 1 – Topic 1

Translational Approaches to Disease Modifying Therapy of Type 1 Diabetes Mellitus (T1DM)

- Increasing prevalence of T1DM, but no prevention or cure so far
- Deeper insight into the heterogeneous, phenotypic characteristics of people either at risk of developing T1DM or having manifest disease required → needs a multi-stakeholder approach,
- Key deliverables: 1) pan-European clinical trial and translational research network including a T1DM patient registry; 2) Innovative clinical trial paradigms; 3) Stronger patient involvement via a Patient Advisory Committee
- EFPIA partners: Sanofi (coordinator), Juvenile Diabetes Research Foundation (JDRF) (co-coordinator), Novo Nordisk, Eli Lilly, GSK, Helmsley Charitable Trust.
- Indicative budget: 17.63 mio Euro over 7 years

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Call 1 – Topic 2

Discovery and Validation of Novel Endpoints in dry Age-related Macular Degeneration (dryAMD) and Diabetic Retinophathy (DR)

- No satisfying treatments available for dryAMD and DR
- Clinical endpoints beyond BCVA and predictive markers needed
- Multi-stakeholder approach needed including imaging companies
- Key deliverables: Development of novel methods (e.g. imaging, proteomics, metabolomics, genomics, epigenetics; animal models, and tools as e.g. disease/endophenotype specific patient reported outcome tools or novel visual function testing protocols) and their clinical validation.
- EFPIA partners: Bayer HealthCare (coordinator), Sanofi, Novo Nordisk, Zeiss
- Indicative budget: 7 mio Euro over 5 years

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Call 2 – Potential Topic

Remote Assessment of Diseases And Relapse (RADAR)

- A better, more frequent monitoring of changes in disease state would offer great opportunities; in principal possible via novel technologies, but validation needed

Goals of the project:

- Develop and validate the science of using biosignatures to characterise disease and predict changes in disease state through observational studies (various disease areas)
- Encourage innovation and development of novel biosensors and the associated knowledge management technology
- Understand the regulatory pathways for using remote assessment in healthcare
- Develop standards for Information Exchange that enable seamless integration of sensor, data capture, data management, & analysis technologies

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Continuous flow of topics

- It is expected to have ~ 2 calls per year
- Topics will come from the priority areas / Strategic Governing Groups but also address other fields in the scope of the Strategic Research Agenda
- Ideas from third parties are always welcome

Contact points

- Strategic Research Agenda for IMI2: http://www.efpia.eu/uploads/Modules/Documents/def_efpia_brochure_sra_a4_web.pdf

- Network of national contact points http://ec.europa.eu/research/participants/portal/desktop/en/support/national_contact_points.html

- IMI partner search tool for help finding collaboration partners http://www.imi.europa.eu/content/partner-search

- IMI Executive Office infodesk@imi.europa.eu

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IMI and IMI2: from science to patients - together

Thank you for your attention!

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