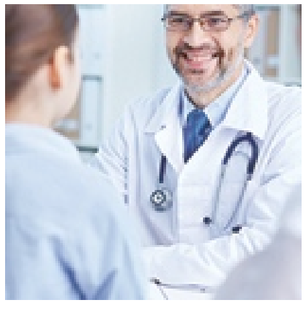
European Federation of Pharmaceutical Industries and Associations

Innovative Medicines Initiative





EMA - EBE Regulatory Conference on ATMPs

Salah-Dine Chibout, Novartis
Global Head Discovery & Investigative Safety/ Global Head Preclinical Safety Therapeutic Areas

IMI accelerates innovation

Multiple companies join force where they would fail alone:
Identify missing or weak links in medicines pathways that hold progress
Combine (often) proprietary knowledge, data and assets
Open them up for challenge by and **collaboration with public partners**
Validate proposed solutions during project lifetime in R&D practice

Solutions for diseases with high burden and cost for patients and society

Solutions that challenge current business models and focus on value for patients and sustainable healthcare

Tracking and addressing science gaps and inefficiencies from discovery to disease management

Essential features for research and policy agendas

- Public private partnership
  - Companies and public partners work <u>together</u>
  - Industry cost is not reimbursed: it is our in kind contribution
  - Public partners (including companies up to € 500 mio turnover) cost is reimbursed by EU: grants for collaborating with industry

- Industry defines the research agenda and projects (but does not chose with whom to work)
  - Beyond the publication: impact on research, regulatory and medical practice

- Managed by a neutral broker that allows participation of authorities and patients

innovative medicines initiative

The Innovative Medicines Initiative: the largest public-private partnership for health research worldwide

€5,276 billion

IMI1 €2 billion from 2008 – 2014

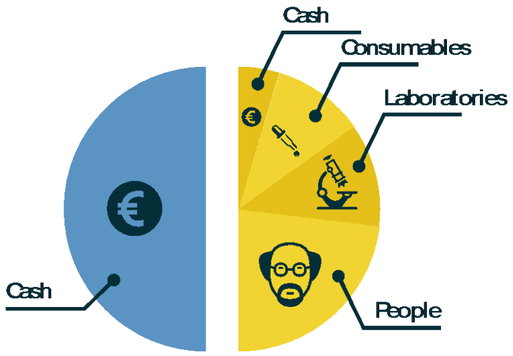
IMI2 €3,276 billion from 2014 - 2024

Part of the EU FP7 and Horizon 2020 R&D funding

Source: http://www.imi.europa.eu/content/mission

The public contribution

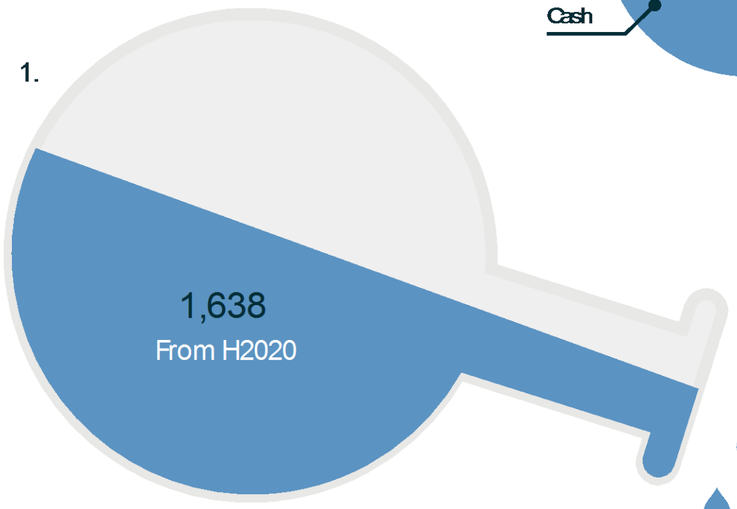
1,638 Billion €



The private Industry in-kind contribution

1,638 Billion €

1.



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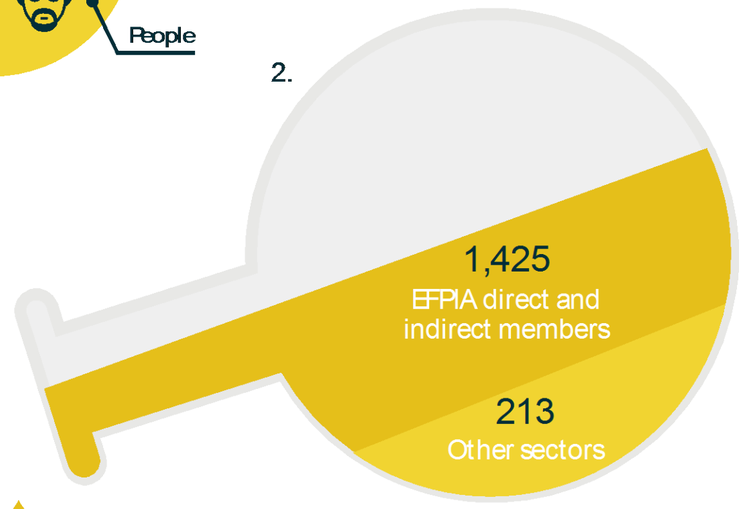
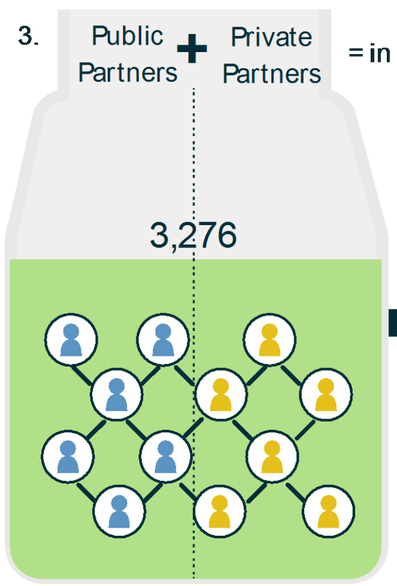


CASH for grants for PUBLIC PARTNERS

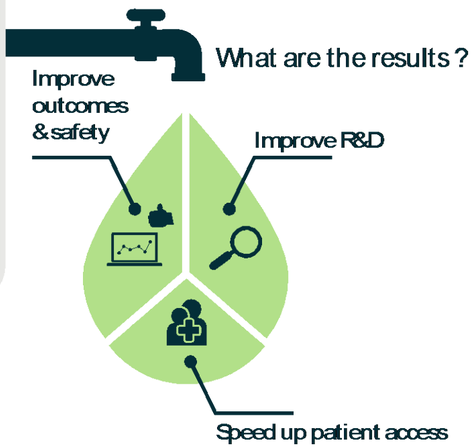
3. Public Partners + Private Partners = in IMI2 consortia



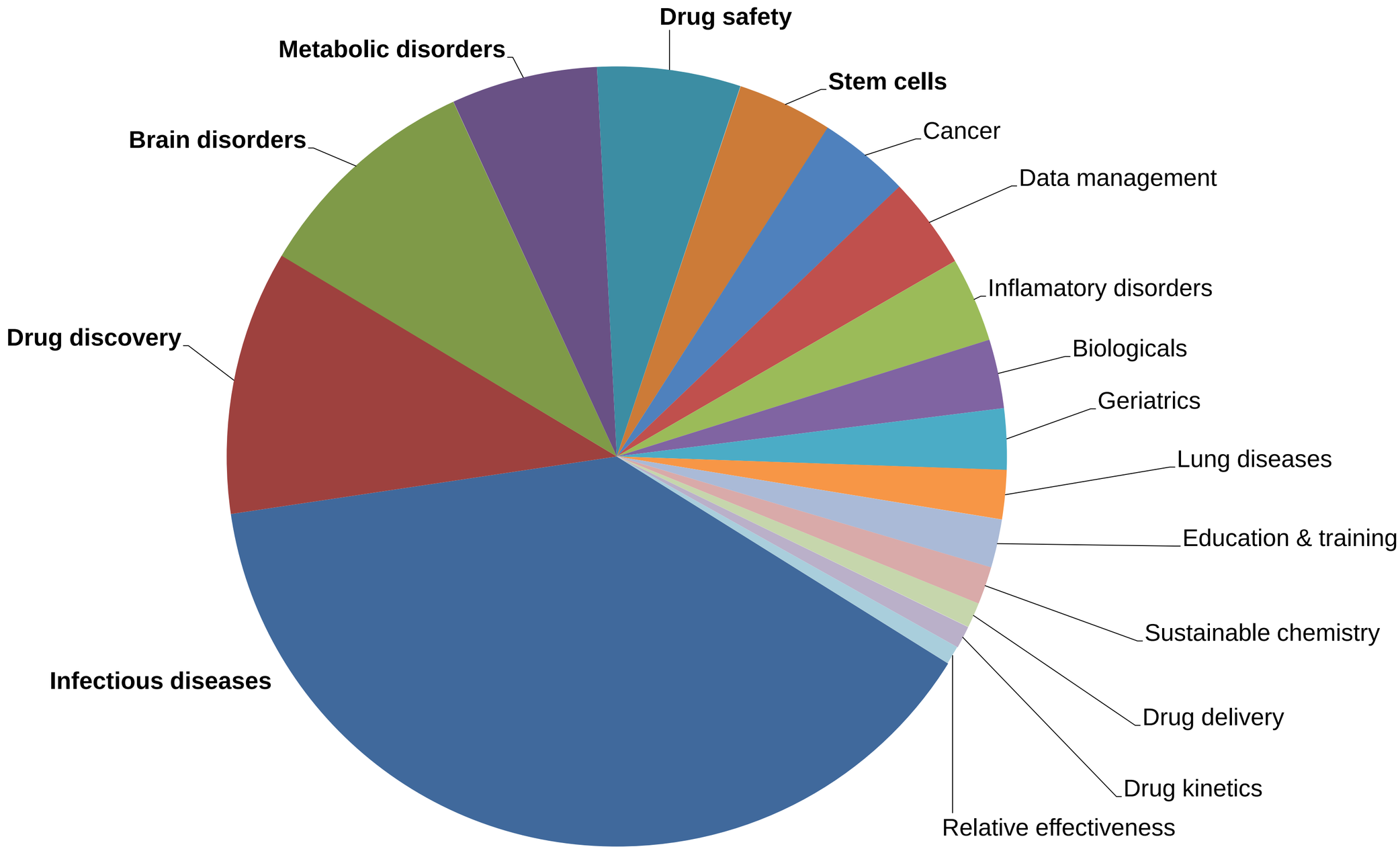
3,276 Billion €

2014 - 2024

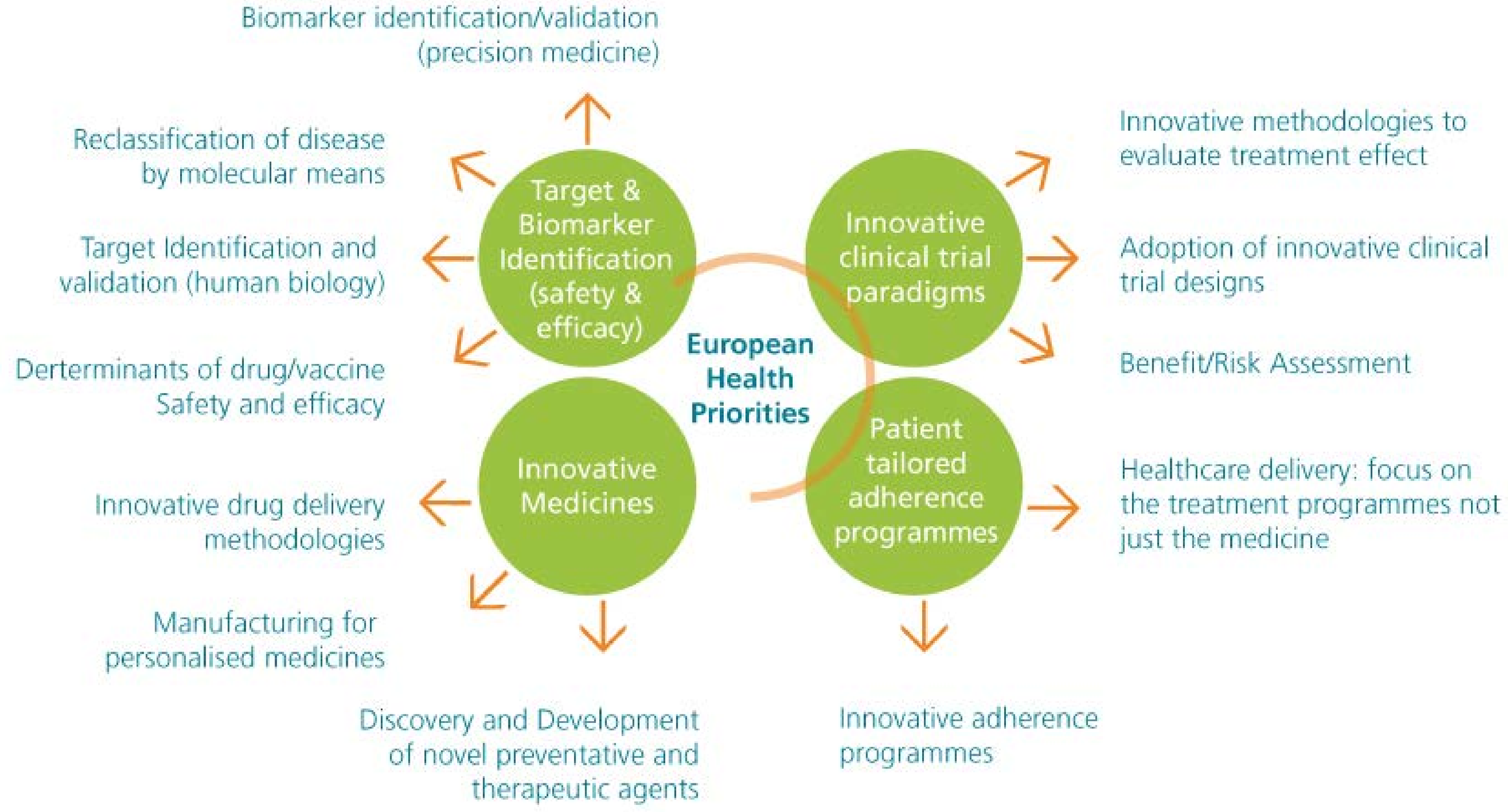
What are the results ?



Distribution of IMI funding per area



Major Axis of Research



DRIVE CHANGE IN DELIVERY OF MEDICAL PRACTICE

End-to-End: Alzheimer's Example

Patient access, healthcare systems

ROADMAP
Outcomes definition and tracking

Aetionomy
Interventions and Modelling

AETIONOMY
Knowlege base, novel hypotheses and biomarkers

EMIF-AD
Characterisation and Modelling

EMIF
Legal and Ethical framework, Parent cohorts, fingerprinting, data browser

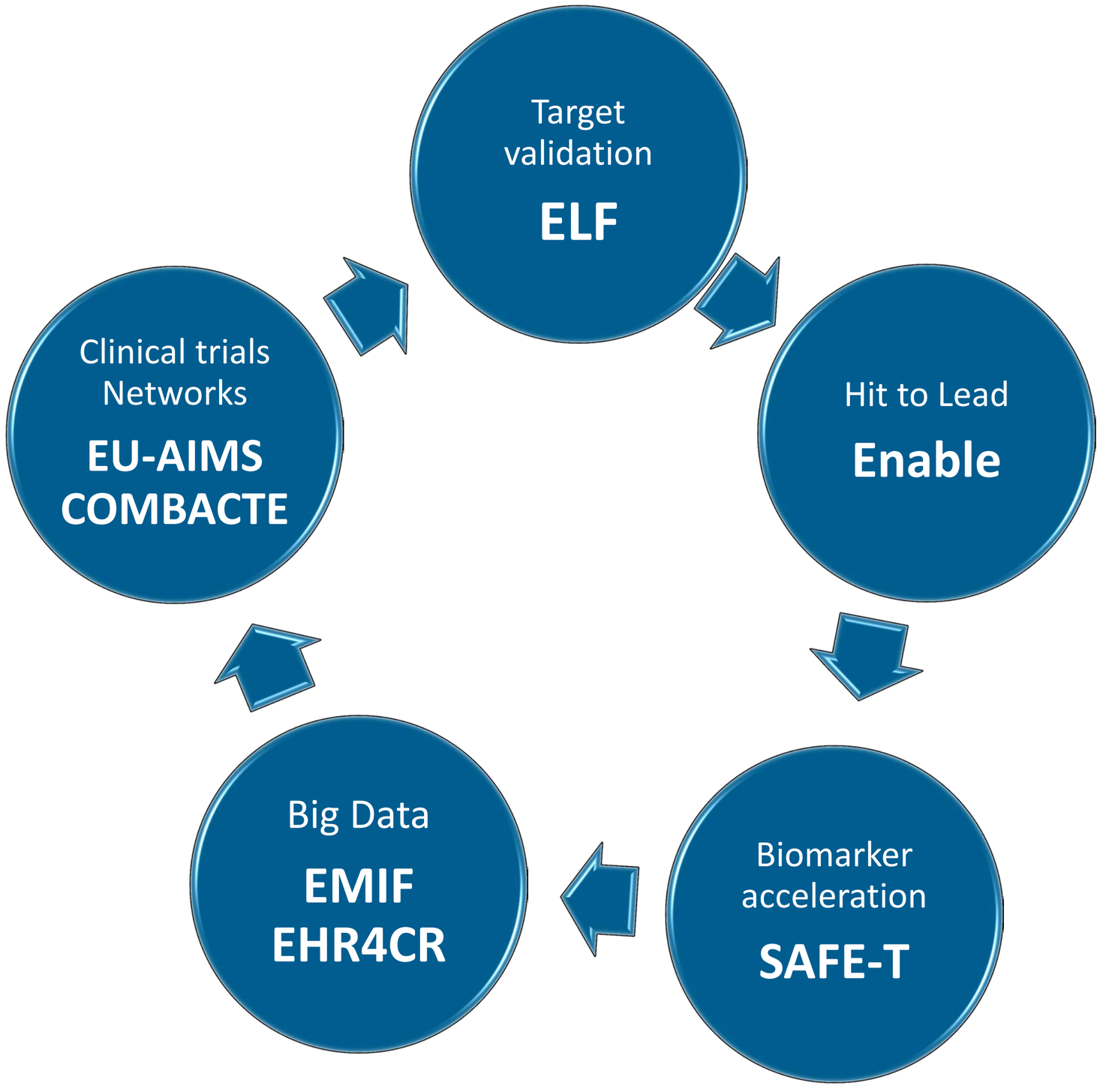
Pharma-Cog Biomarkers

PharmaCog
Advancing science and treatment of Alzheimer's Disease
Combination of tools to accurately measure disease progression

EPAD
Trial and Modelling

EPAD
European Prevention of Alzheimer's Dementia Consortium
Trial, Hypothesis validation

End-to-End: R&D accelerator



European Lead Factory

Rationale

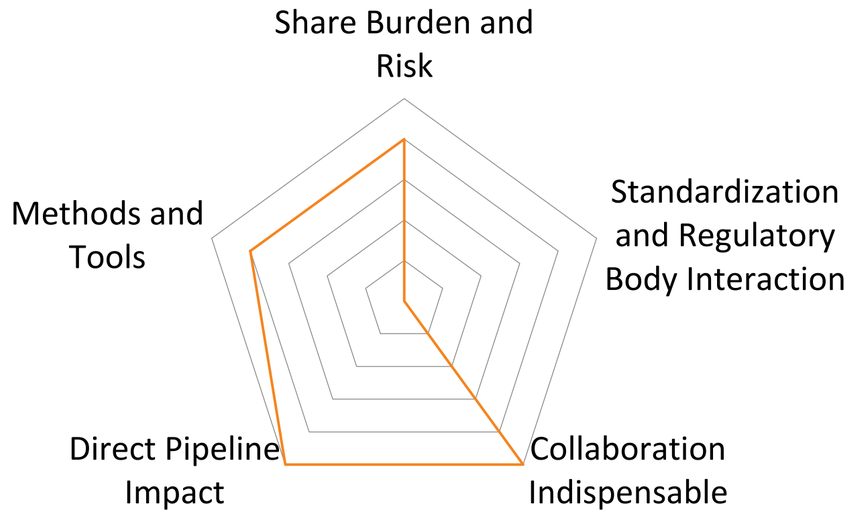
Need

1. Access to **high-quality chemical library** for academics/ SMEs to translate academic biological discoveries/ targets into suitable chemical matter
2. Access to otherwise **unattainable chemical space** for pharma partners through compound sharing and synthesis of novel chemical libraries
3. Access **new biology from academia/ biotech**

Aim

- **Provide starting points for lead discovery or high-quality pharmacological tools** both for academics/ SMEs proposing targets and EFPIA companies.
- **Create partnering opportunities** for public partners and EFPIA companies to progress hits along the pharma value chain

Partnership Project Profile



Funds

IMI funding:	€ 80 Mio
Academia / Biotech cont.	€ 25 Mio
Pharma resources :	€ 91 Mio
TOTAL PROJECT COST:	**€ 196 Mio**

Duration: 01.01.2013 – 31.12.2017

Total budget: € 196 million

Boosting Drug Discovery

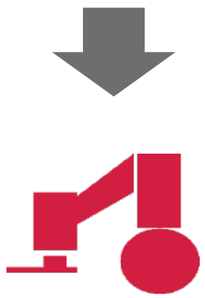
European Lead Factory

Public targets through crowd-sourcing process

DRUG TARGETS

50% EFPIA targets
50% public targets

Joint
European
Compound
Library



European
Screening
Centre

EFPIA contribution
(>300,000 cpds)

Public contribution
(200,000 cpds by 2017)

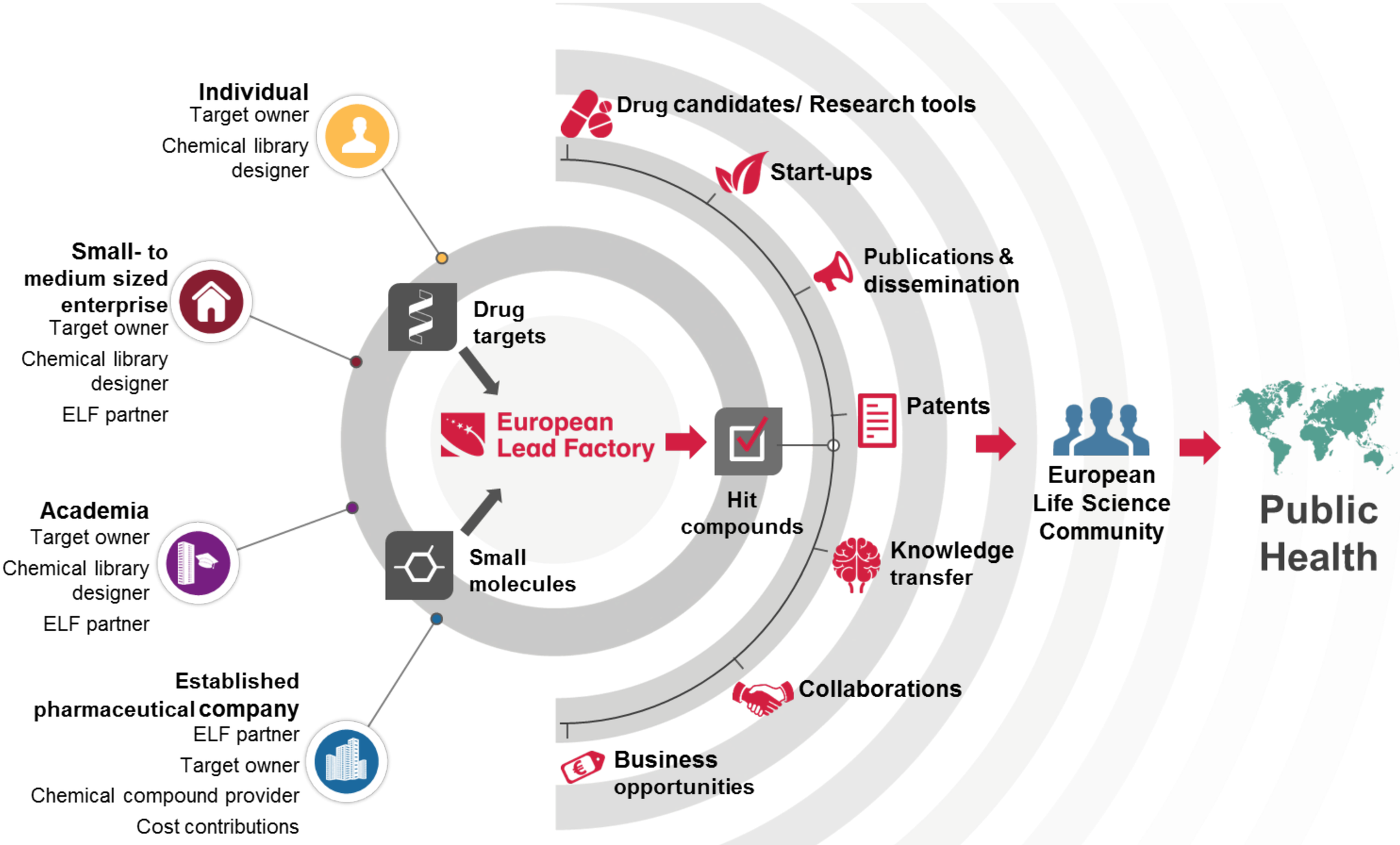
LIBRARY DESIGNS

Seven Pharma companies provided a high-quality cross-section of their in-house libraries

Chemistry consortium designs and synthesized Public Compound Collection (PCC)

European Lead Factory

Short and long-term benefits



European Lead Factory

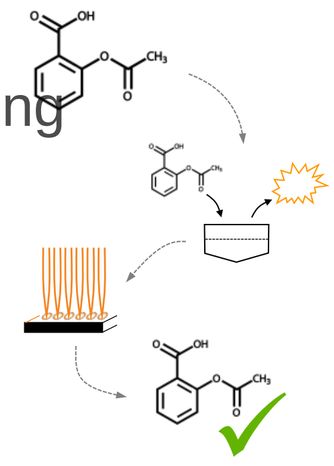
What's in it for the stakeholders?

Output

- *129* Public target proposals
- *73* Accepted targets
  - 2/3 from academia
  - 1/3 from SMEs
- *49* Public screens completed
- *43* Qualified hit lists (QHLs)
- *12* Improved hit lists (IHLs)

Capabilities

Access to a unique, unprecedented screening library,
assay development,
and state-of-the-art screening including hit validation capabilities



Progress

Public programme advanced to be developed within ND4BB ENABLE programme; a further project lead progressed as asset in **ScandiCure** start-up

Knowledge

Training and education activities increased public knowledge on early drug discovery; establishment of a network of scientists working in drug discovery across Europe; more than 40 publication in peer-reviewed journals

The Big Data for Better Outcomes programme

Goal: Support the evolution towards outcomes-focused and sustainable healthcare systems, exploiting the opportunities offered by big and deep data sources

COORDINATION AND SUPPORT ACTION (CSA)

DISTRIBUTED DATA NETWORK

1 **Design sets of standard outcomes and demonstrate value**

2 **Increase access to high quality outcomes data**

3 **Use data to improve value of HC delivery**

4 **Increase patient engagement through digital solutions**

ROADS: ALZHEIMER'S DISEASE

HEMATOLOGIC MALIGNANCIES

CARDIOVASCULAR

PROSTATE CANCER

MULTI-DISEASE / MULTI-MORBID PATIENTS

Coordination and operational topics

Themes / Enablers

Disease-specific topics

HARMONY project

To improve outcomes in Hematologic Malignancies, we are teaming with leading institutions across Europe

Largest funded project within IMI2

51 partners, including 44 public partners from 10 different EU countries

"IMI projects are best practice in the industry" – FDA representative

efpia

www.efpia.eu

ATMPs

Key challenges and future IMI2 topics

- Based on feedback from the IMI Stakeholder Forum on Advanced Therapies, **five potential IMI2 topics** are currently being considered:

  - **Precision Genome Editing (PGE)**
  - **Clinical development and patient access**
  - **Clinical development of cell therapies in cancer**
  - **Manufacturing**
  - **Immunogenicity**

Prioritised for development in 2017

Precision Genome Editing (PGE)

- **Scope**
  - Address gaps in our understanding of precision genome editing (PGE) biology, function and applicability.
  - Increase confidence in the accuracy, safety and efficacy of the technologies for both research and therapeutic applications.
- **Examples of deliverables**
  - Novel characterization assays and tools for the quantification of on-target/ off-target effects, ie. New DNA analytic technologies or advanced 'next generation sequencing' (NGS) platforms.
  - Optimization of existing PGE platforms - ie. Bioinformatic tools and design guidelines to increase target selectivity.
  - Development of new pre-clinical cell/animal testing paradigms, ie:
  - Develop and provide access to qualified reagents, platforms and data.
  - Define the boundaries between the competitive and precompetitive space, through continued dialogue between researchers, manufacturers and platform development, throughout the programme.

Clinical development

- **Scope**
  - Framework for the data-enabled optimization of clinical trials for different types of ATMPs.
  - Infrastructure and methodologies for the efficient utilization of existing and new registries and other data repositories.
  - Enhance interoperability between databases and integration of data
  - Update policies, processes and qualification pathways to assess clinical utility of existing data and new evidence requirements.
- **Examples of deliverables**
  - Technical capabilities around data source standards and interoperability.
  - Quality standards, accuracy and regularity of data entry, reporting and analytics.
  - Develop new data network architectures and links, as well as dataset query protocol designs, to avoid fragmentation.
  - Increase built in flexibility to accommodate emerging knowledge and changing requirements.
  - Address challenges in database sustainability
  - Clarify status of patient level data protection, access controls and surveillance.
  - Clinical trial registries could also expand to provide evidence in further support of HTA evaluations, focused on patient outcomes.

Patient access

Scope

- Capture the challenges across the pathway from the bench to the bedside, and across the different types of ATMPs.
- Clarify evidence requirements for a comprehensive assessment and commercialization framework.
- Allow sufficient flexibility to accommodate the pace of scientific progress.
- Secure the appropriate use of hospital exemption and leverage existing schemes, ie. Orphan/rare disease funds.

Examples of deliverables

- Analysis of pipeline projects and commercial products, investment decisions etc.
- Identify success/failure drivers and key go/no-go decision factors across the product journey from R&D to the health systems (case studies).
- Devise analytical frameworks and performance indicators to compare EU countries, with US and other global competitors.
- Model/propose novel reimbursement and payment schemes.
- Tabulate the key HTA considerations and contrast with evidence for regulatory approvals and surveillance.
- Analyze case examples on hospital exemption across Member States.
- Identify and evaluate existing and propose new modelling methods and data tools (ie. Registries) through specific projects and work streams.

How an overarching project could look like: Clinical Development of Cell Therapies in Cancer

- **Scope**
  - Address gaps in early financing of proof-of-concept studies.
  - Improve comparability of clinical benefit of cell therapies in cancer.
  - Enable combination therapy with checkpoint inhibitors and targeted therapies
- **Examples of deliverables**
  - Public-private partnership in pre-PoC stage to improve number and quality of clinically tested approaches
  - Early focus on demonstrating clinical benefit –alone or in combination with checkpoint inhibitors and targeted therapies- of cellular therapies
  - Search for biomarkers of activity and mode of action of cell therapies.
  - Define and standardize production quality standards & specifications.
  - Analyse feasibility of production on an adequate scale
  - Focus investigators on „affordability and profitability“.
  - Use of historical and real-world evidence to compare outcomes in ATMP clinical trials.
  - Define the boundaries between the competitive and precompetitive space

Conclusion

- IMI **is delivering**
- This is a true **partnership**, where companies, public partners and SMEs work together
- There is an opportunity to **transform the ATMP landscape**