



Innovative Medicines Initiative

Generation of research tools to translate genomic discoveries into drug discovery projects

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efpia*

Need for public-private collaboration



Common interest

- Genomic information is growing and is revealing many potential targets for therapeutic intervention but translation into drug discovery projects is not keeping pace.
- Disease understanding and target characterization in particular in pioneer areas of (disease) biology and drug discovery are limited by access to high quality research tools for proteins.
- A partnership to generate and exploit research tools provides an opportunity to create knowledge in new areas of disease biology at a scale and breadth that can not be achieved timely within a single sector.

Combine skills, share costs, avoid duplication of effort and establish Europe as global leader

- High quality tools are difficult and expensive to produce, their production and characterization often requires skill sets that span academia and industry, and access to the tools can be severely limited by conditions set by the supplier.
- Tools can reveal disease relevance of target when tested in disease relevant assays using human disease tissue, which is challenging to access and needs involvement of clinical scientists.

Opportunity to establish joint quality standards to ensure reproducibility of research outputs.



Objectives of the full project

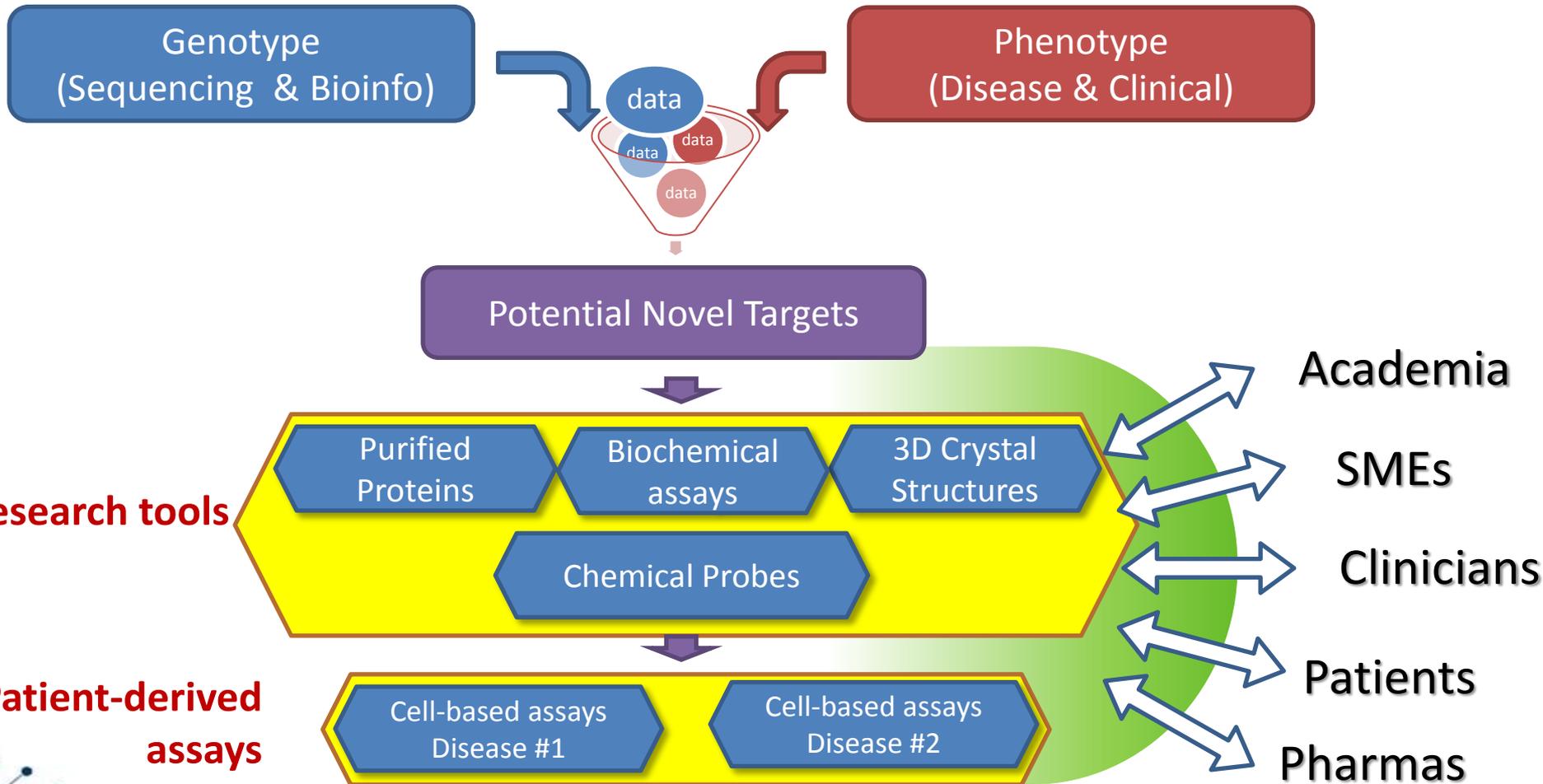


Expand the range of drug targets required to address unmet medical need

- Translate genomic discoveries into enabling research tools for proteins linked to disease.
- Exploit these tools systematically in relevant models of human disease (derived from human tissue) for target identification & validation and lead discovery.
- Initial focus should be on targets related to inflammatory mechanisms/diseases with an emphasis on epigenetic regulators as well as ion channels & solute transporters.



Grafical outline of the objectives of the full project



Pre-competitive nature



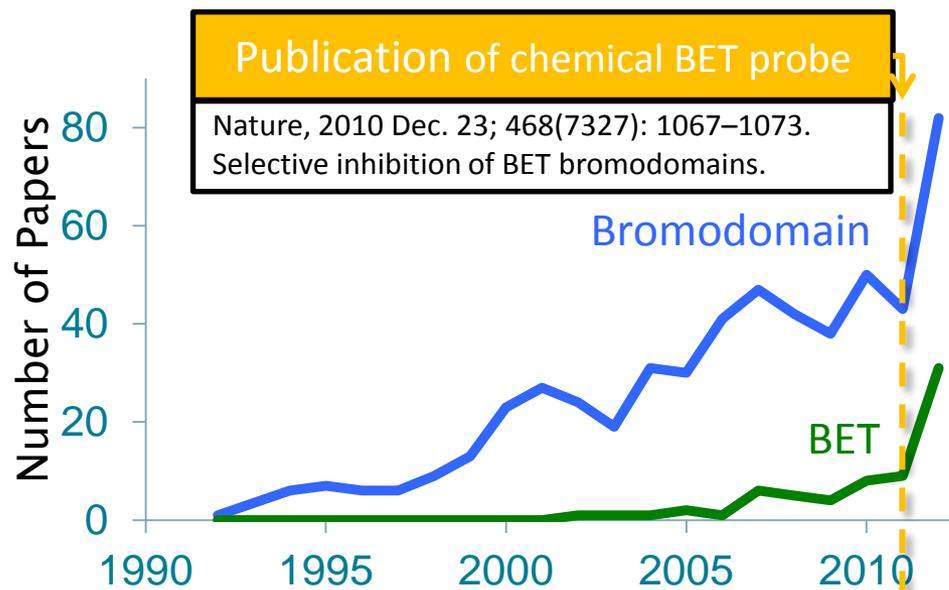
- Protein-focused research tools are used in target discovery and characterization – the earliest stages of drug discovery.
- Risk sharing permits sustainable effort on novel, unexplored targets which would not be explored in a timely manner by individual sectors.
- A pre-competitive framework
 - allows the partnership to share the research output with the wider scientific community which is expected to amplify its impact.
 - accelerates knowledge translation and engagement of leading academics and clinicians.
 - promotes sector integration; actively engage academia, SMEs and industry to generate and exploit novel, readily-accessible research tools for knowledge discovery



Expected impact on the R&D process



- Enables scientists in all sectors to access research tools for “pioneer” proteins
- Accelerates testing of genotype/phenotype associations deriving from GWAS, rare disease initiatives, RNAi screens and mouse K/O efforts
- Catalyses innovative drug discovery projects to address unmet medical need



- Chemical probe and assays with clinical tissues
- New disease links
 - NUTmidline carcinoma
 - Septic Shock/ Inflammation
 - Myeloma
 - Leukemia
 - MYC regulation
 - HIV re-activation



Suggested architecture of the project



Human Gene-Disease Links

1. Selection of targets and protein families

2. Protein expression and purification

3. Assays
(biochem./biophys./cell)

4. Structure Determination

5. Chemical hit ID and chemical probe generation

6. Cell & tissue assays (inflammation & immunology)

7. Bio- and Cheminformatics

9. Project Management & Results Dissemination

8. Collaborative network

Additional phenotypic assays

Additional chemical probes

Launch/Add Value to Drug Discovery Projects



Expected contributions of the applicants



The Applicant Consortium needs to address all objectives from all WPs and is expected to demonstrate excellence and track record in:

1. Expertise and leadership in protein science, chemical probe generation & assays

Human proteins and protein structures, target family based approach, cell-based assays of inflammatory mechanisms, approach the project at large scale

2. Established wide and continuously evolving network of thought leaders in all sectors

Chemistry, biological assays, human biology, genetics and the clinic

3. Track record of successfully collaborating with industry

Impact on internal drug discovery projects, governance and management of large projects



Expected (in kind) contributions of EFPIA members



EFPIA members: Novartis, Bayer, Janssen, Pfizer

1. Indicative budget

- EFPIA: Total of 21 600 000 € (14 800 000 € in-kind and 6 800 000 € in cash)
- IMI JU: Total of 21 200 000 €

2. In-kind contributions

- Target selection/prioritization
- Scientific background on disease biology
- Experimental support for structural biology (cDNAs, synchrotron access)
- Provide focused chemical libraries (fragments, hits, tool compounds)
- Design and synthesis of chemical probes
- Experimental support to characterize chemical probes



What's in it for you?



Patients

- Increase and accelerate research activities in areas of unmet medical needs

Academia

- Rapid access to high quality research tools to advance disease biology
- Seeding research in novel areas of target spaces

Open Access
Research Tools

Freedom-to-
Operate for all
parties to
innovate

SMEs

- Opportunity to create technology-driven service platforms
- Access to user network

EFPIA

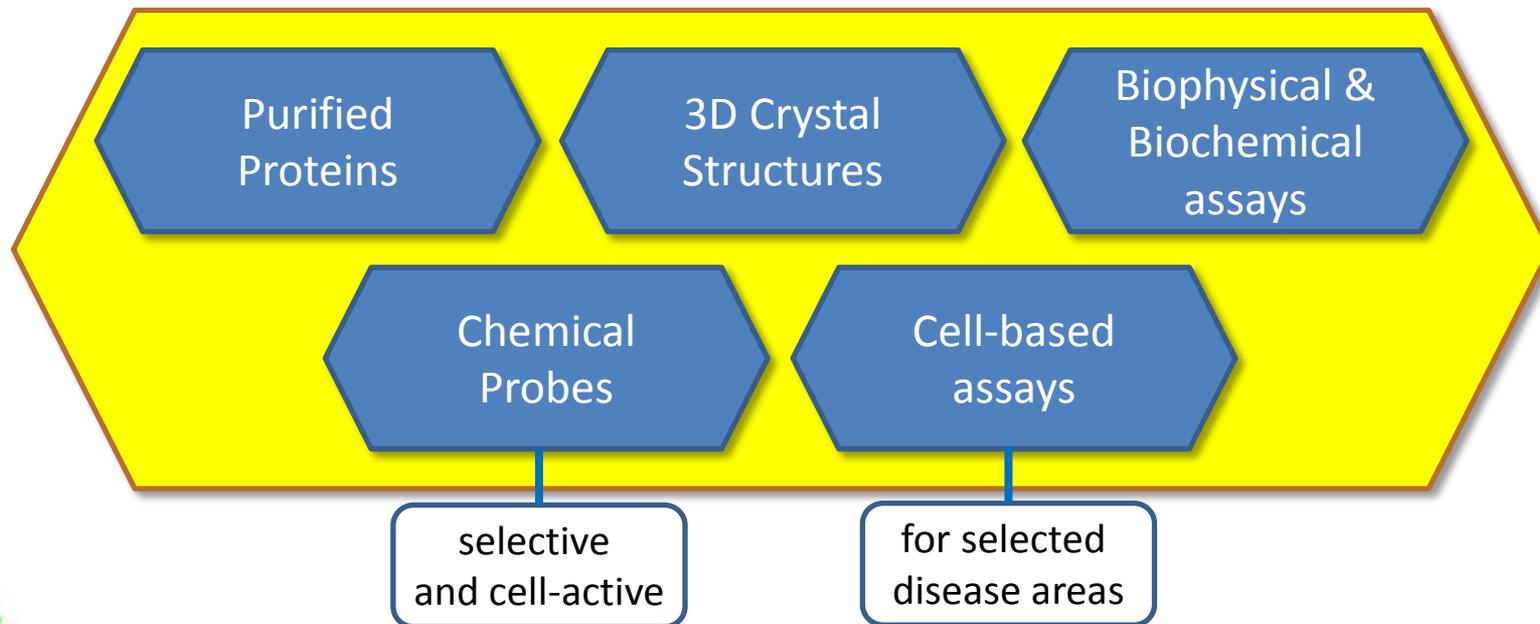
- Identify pioneer areas of biology meeting unmet medical needs
- Jump-start proprietary projects



Key deliverables of the full project



High quality research tools, the associated information and the resulting knowledge for novel and prioritized targets



Questions?



- Contact the **IMI Executive Office**

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