MEDICAMENTOS INNOVADORES
Plataforma Tecnológica Española

Plataforma de Mercados Biotecnológicos
(Spanish Biotech Platform)

NANOMED SPAIN

PLATAFORMA ESPAÑOLA INNOVACIÓN TECNOLOGÍA SANITARIA

V Conferencia Anual de las Plataformas Tecnológicas de Investigación Biomédica: Medicamentos Innovadores, Nanomedicina Tecnología Sanitaria y Mercados Biotecnológicos Fomentando la *Open Innovation*

Proyectos colaborativos en Drug Discovery

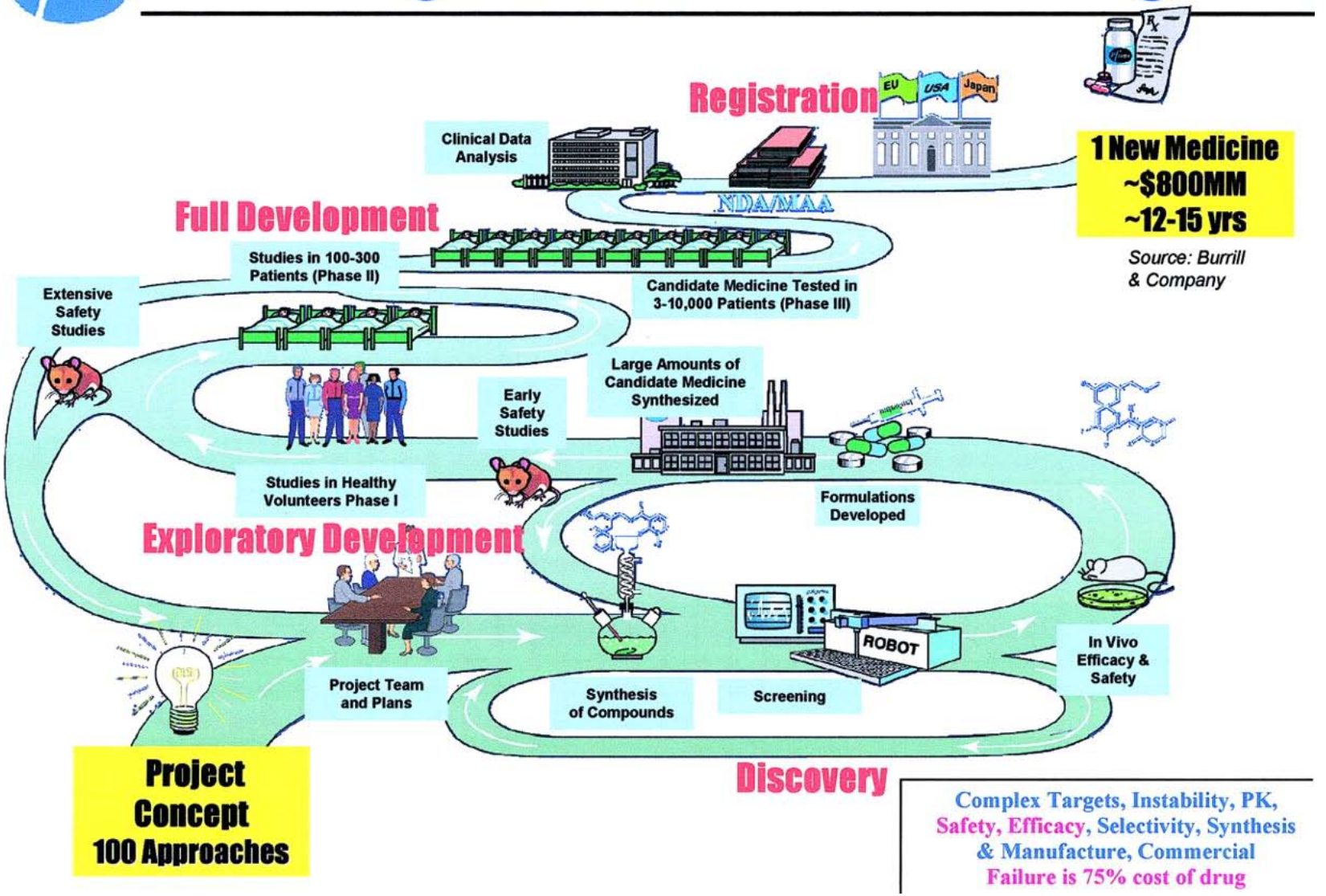
European Lead Factory: Joint European Compound Library and Screening Centre

Innovative Medicines Initiative (IMI) 5th Call for proposals – February 2012

Jordi Quintana
Parc Científic Barcelona (PCB)

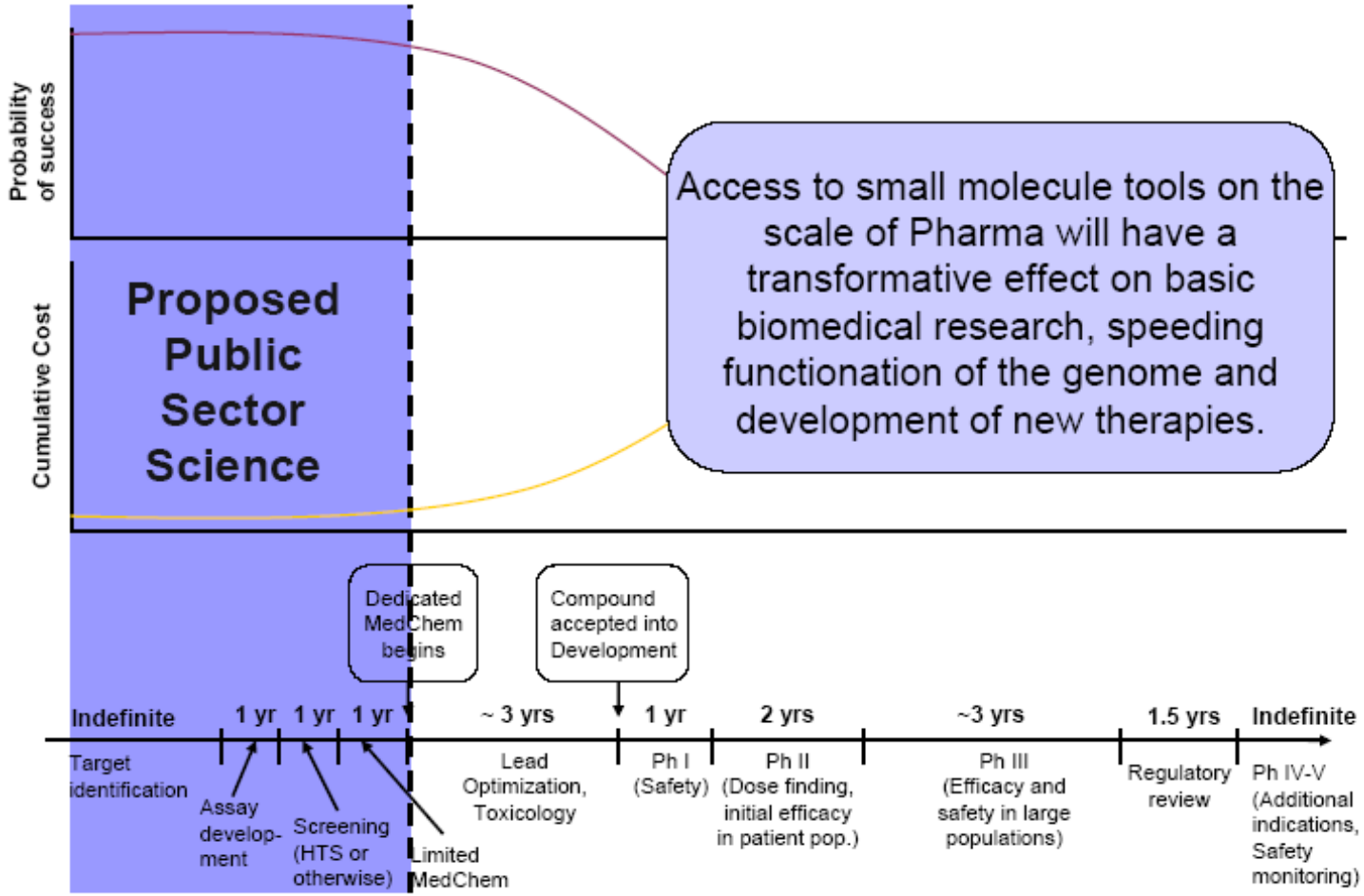
Barcelona, 14 febrero 2012

The Long Path from Idea to Drug



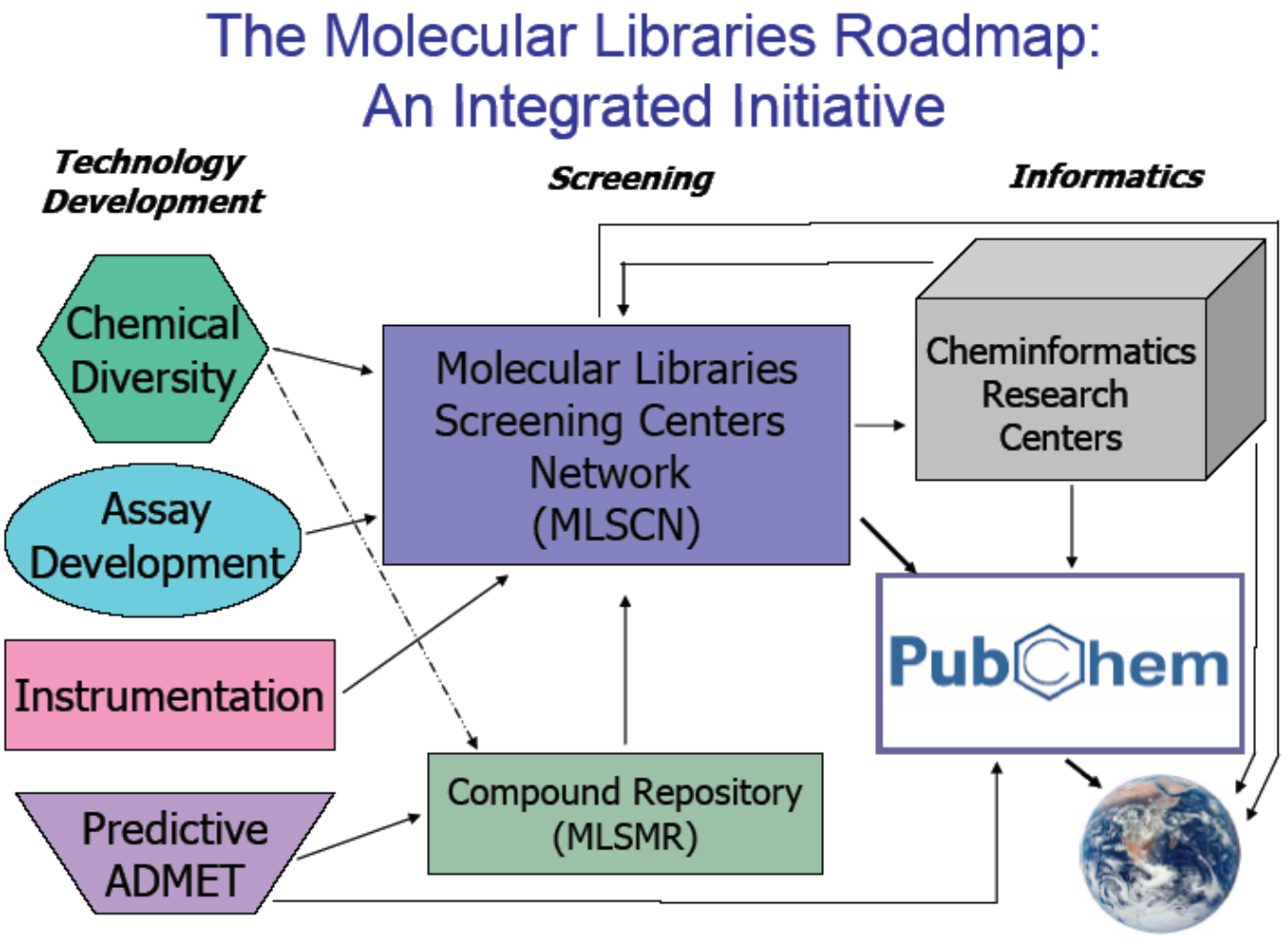
From: Scott P. Kennedy and B. J. Bormann, *Experimental Biology and Medicine* 231:1690-1694 (2006)

The Drug Development Pipeline



From: http://www.mli.nih.gov/

Projects in the USA: NIH Roadmap / Molecular Libraries Initiative



http://www.mli.nih.gov/, https://www.mli.nih.gov/mlscn/index.php, http://mlsmr.glpg.com/MLSMR_HomePage/project.html, http://pubchem.ncbi.nlm.nih.gov/

ESFRI ROADMAP – OCT.2008 / EU-OPENSCREEN

EU-OPENSCREEN
ESFRI ROADMAP 2008

European Infrastructure of Open Screening Platforms for Chemical Biology

About EU-OPENSCREEN
- Concept
- ESFRI
- Contact

Partnership
- European network
- Platforms
- Shared libraries
- Job opportunities

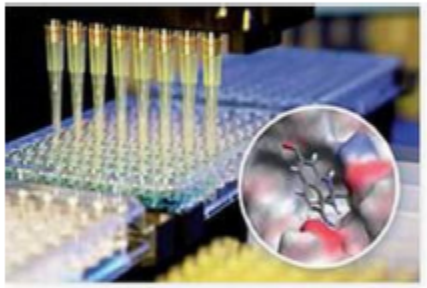
Preparatory Phase
- Current call

Further information
- News and press releases
- Events
- Articles
- Links

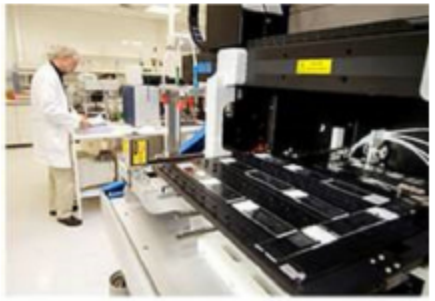
WELCOME TO EU-OPENSCREEN

A European Infrastructure of Screening Platforms

EU-OPENSCREEN, the European Infrastructure of Open Screening Platforms, integrates high-throughput screening platforms, chemical libraries, chemical resources for hit discovery and optimisation, bio- and cheminformatics support, and a database containing screening results, assay protocols, and chemical information.



These platforms – offering the most advanced technologies – will be used by European researchers from academia and SMEs in order to identify compounds affecting new targets. Open access to an integrated infrastructure for Chemical Biology will thus satisfy the needs for new bioactive compounds in many fields of the Life Sciences (e.g. human and veterinary medicine, systems biology, biotechnology, agriculture and nutrition).



http: www.eu-openscreen.eu

CHEMBIOBANK PROJECT

A joint initiative to build an annotated molecular library in Spain

Chemistry, logistics, project coordination

Pharmacological screening

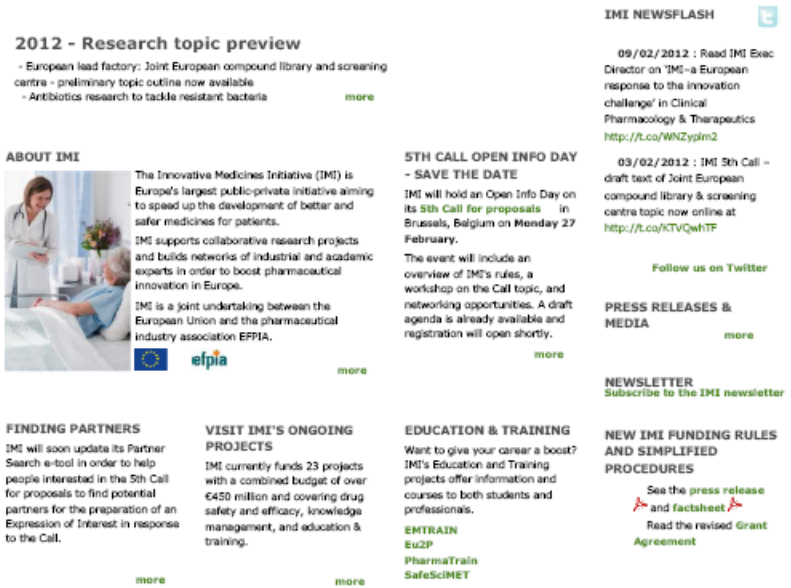
Virtual screening

European Lead Factory: Joint European Compound Library and Screening Centre

Innovative Medicines Initiative (IMI) 5th Call for proposals – February 2012

http://www.imi.europa.eu/

The **Innovative Medicines Initiative (IMI)** is Europe's largest public-private partnership aiming to improve the drug development process by supporting a more efficient discovery and development of better and safer medicines for patients.



IMI supports research projects in the areas of safety and efficacy, knowledge management and education and training. Projects are selected through open **Calls for proposals**

European Lead Factory: Joint European Compound Library and Screening Centre

Innovative Medicines Initiative

5th Call Open Info Day
Brussels, 27 February 2012

Preliminary Agenda

9:30-10:30 Registration & networking breakfast

10:30–11:30 Plenary Session

Chair: Michel Goldman, Executive Director, IMI

- Welcome and introduction
  Michel Goldman
- The 5th Call in the context of IMI's revised Scientific Research Agenda
  ***Daan Crommelin**, Utrecht University and Vice-Chair of the IMI Scientific Committee*
- Overview of IMI rules and procedures
  ***Magali Poinot**, Legal Manager, IMI*
- Communicating IMI's Calls for proposals in the Member States
  ***Jan Skriwanek**, German Aerospace Center (PT-DLR) and member of the IMI States Representatives Group (SRG)*

11:30-11:45 Break

11:45-12:30 Practical advice on applying for IMI funding

Roundtable discussion

- Writing a successful proposal – dos and don'ts
  ***Hugh Laverty**, Senior Scientific Project Manager, IMI*
- Applying for IMI funding – an academic's experience
  ***Thierry Troosters**, KU Leuven (PROactive project)*
- IMI and SMEs
  ***Claire Skentelbury**, European Biotechnology Network*
- Q&As

12:30-13:30 Networking lunch

13:30-15:00 Workshop

European lead factory: Joint European compound library and screening centre
*Speaker: **Jörg Hüser**, Bayer HealthCare*
*Moderator: **Hugh Laverty**, IMI*

15:00-16:00 Networking tea / coffee break

16:00 End of meeting

efpia

Innovative Medicines Initiative
Avenue de la Toison d'Or, 1060 Brussels
tel: +32 (0)2-221 81 81 – web: www.imi.europa.eu

5th Call 2012

Save the date! IMI will hold an Open Info Day on the 5th Call on Monday 27 February. Find out more

IMI's 5th Call for proposals currently includes one indicative topic:
European lead factory: Joint European compound library and screening centre

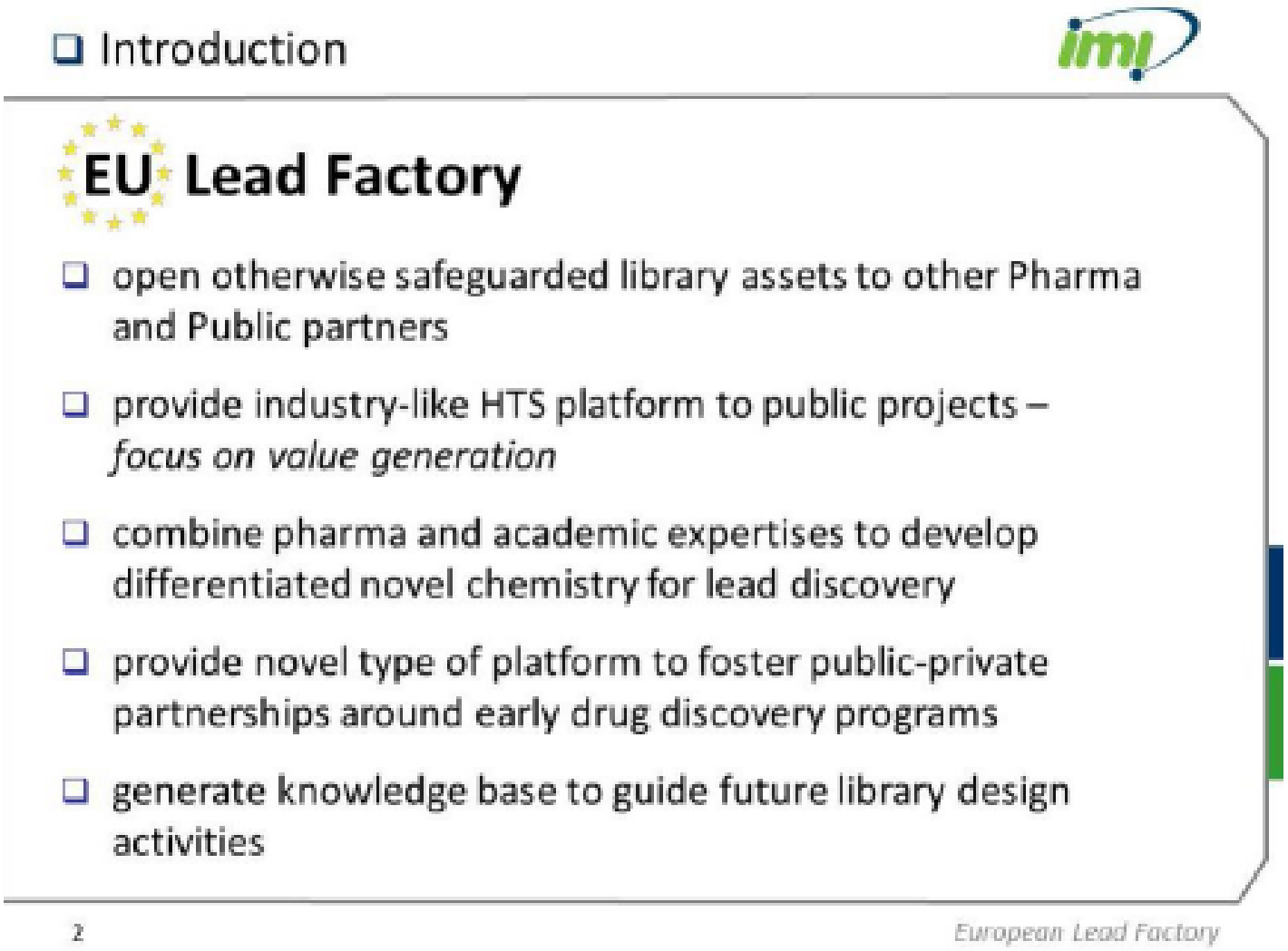
New! Download a draft of the proposed European lead factory topic text

http://www.imi.europa.eu/sites/default/files/uploads/documents/5th_Call/IMI_Call5_EuropeanLeadFactory_Draft20120130.pdf

The 5th Call for proposals is scheduled to be launched in February

Jörg Hüser, Bayer Healthcare (slides)

European Lead Factory: Joint European Compound Library and Screening Centre



Jörg Hüser, Bayer Healthcare (slides)

European Lead Factory: Joint European Compound Library and Screening Centre

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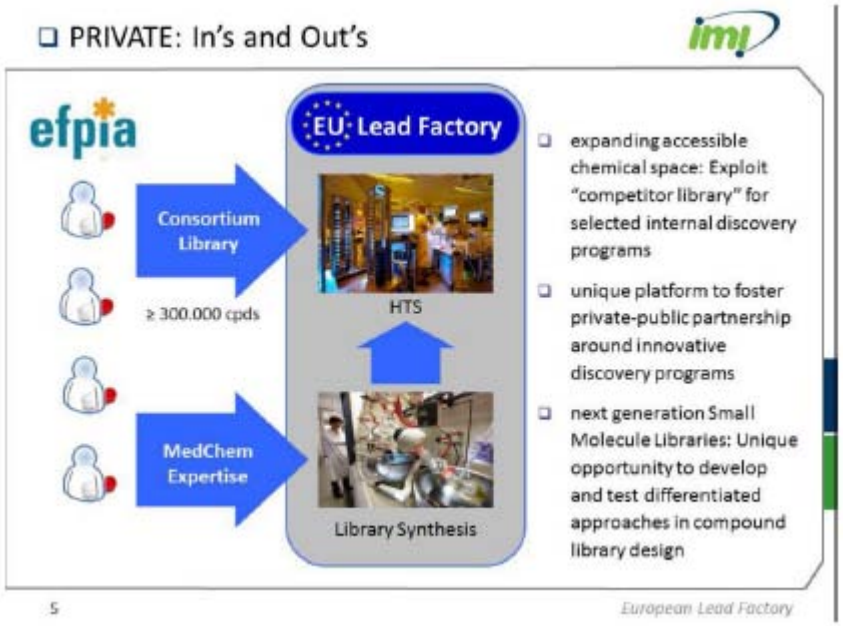
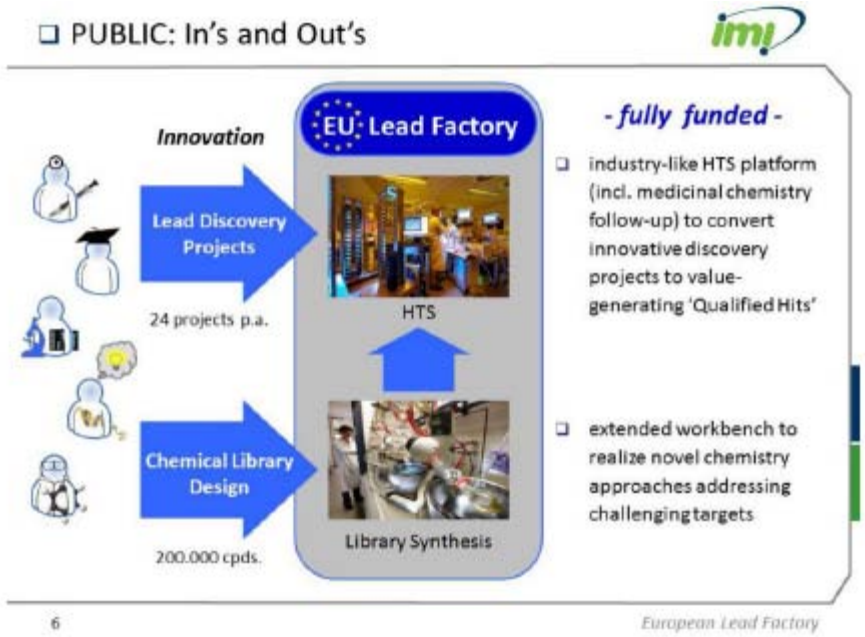
European Lead Factory: Joint European Compound Library and Screening Centre





Jörg Hüser, Bayer Healthcare (slides)

European Lead Factory: Joint European Compound Library and Screening Centre

Project Workflow

- Qualified Hit List will be disclosed to Project Owner:
  - up to (≤) 50 different compounds with graded or no activity in 1° HTS assay
  - compiled by Screening Centre scientist and Delegate
- notification of Compound Provider and Clearence of possible 3rd party rights
- disclosure of Qualified Hit List to Project Owner:
  - Project Owner is free to establish IP on compounds in QHL or derived thereof
  - QHL compounds are blocked for 'exclusivity period' from prosecution in Consortium or Public screening projects

Indicative Budget and Duration of Project

- **75-80 million EURO** EFPIA 'in kind contribution'
  - → 6 x 50.000 compounds
  - → EFPIA HTS project work
  - → support and management
  - → early partnering of public projects
- 1 : 1 split between HTS and Library Topics
- 5 year funding period

Applicant Consortia (1)

2 Sub-Topics: European Screening Centre & Joint European Compound Collection

➡ Applicant Consortia can apply independently for selected sub-topic

EU Screening Centre:

- providing overall project management
- managing the compound library logistics, i.e. storage and plating,
- development and/or adaptation of target or pathway-specific bioassays for HTS,
- performing HTS campaigns for publicly sponsored projects,
- generating a suite of generic tests for follow-up studies ensuring a stringent hit selection process,
- supporting all projects, private and public, in all aspects of data analysis,
- providing directly or through associated partners initial medicinal chemistry support, i.e. analytics, re-synthesis, limited hit expansion.

Applicant Consortia (2)

2 Sub-Topics: European Screening Centre & Joint European Compound Collection

➡ Applicant Consortia can apply independently for selected sub-topic

EU Joint EU Compound Collection:

- management of multi-partner consortium,
- know-how in innovative library design for lead finding,
- extensive expertise in high-throughput chemistry and compound library generation,
- provision of suitable IT infrastructure and expertise in computational chemistry
- technical platform and process for 'crowd-sourcing' of design proposals from broad public audience

Jörg Hüser, Bayer Healthcare (slides)

European Lead Factory: Joint European Compound Library and Screening Centre

draft of the proposed European lead factory topic text from www.imi.europa.eu

European Lead Factory:

Joint European Compound Library and Screening Centre

All information regarding the IMI 5th Call for proposals is indicative and subject to change. Final information about the IMI 5th Call will be communicated after approval by the IMI Governing Board.

This Call theme consists of two Topics:

- European Screening Centre
- Joint European Compound Collection

Submitted EoIs should address one of these two topics. At the second stage the successful applicant consortium for each topic will merge with the EFPIA consortium to prepare the Full Project Proposal for the Call Theme.

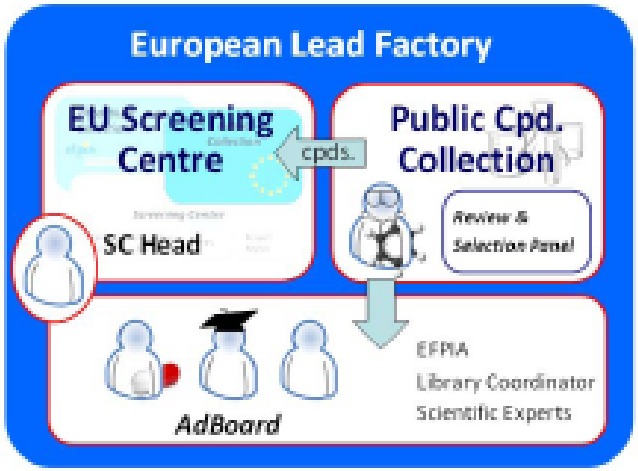
Background

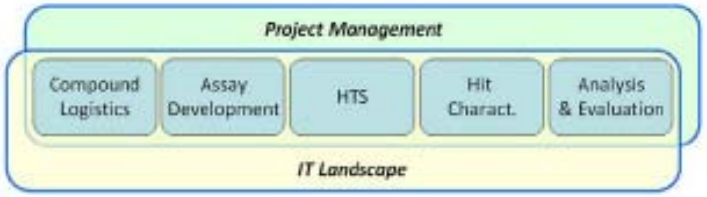
Discovery of novel small molecule lead structures is a major driver of the early drug discovery process. Among a diverse set of discovery strategies, experimental high-throughput screening (HTS) of comprehensive compound collections has provided a major avenue towards lead structure identification. Size, design, and quality of the compound libraries are of utmost importance for the output of HTS. Despite continuous efforts and numerous success stories, a large number of disease relevant drug targets still lack suitable lead structures. Reasons for this intractability are typically manifold, including a low druggability, e.g. protein-protein interactions, and/or difficulties combining target activity with the required pharmacokinetic and metabolic properties in one small molecule. Moreover, Pharma discovery portfolios are increasingly dominated by such challenging projects further jeopardizing the productivity of early drug discovery, and consequently Pharma's output of innovative medicines in general. The still limited understanding of many areas of disease biology is yet another challenge to early drug research reflected in a sparse flow of novel druggable targets with a sufficiently validated disease link. HTS has gained relevance also in this field based on the notion that identified small molecule modulators of a specific molecular target or a cellular pathway might provide suitable tools to unravel target or pathway function in health or disease. Beyond Pharma's activities in this field, the build-up of chemical libraries and HTS has gained increasing relevance also in the academic arena. Triggered by the NIH roadmap in 2004 in the United States[1], this area has recently seen active growth also in Europe, e.g. in the EU OpenScreen Initiative. However, these activities are quite scattered and the libraries and screening efforts do not have the scale and background to address the challenges faced. Publicly funded HTS activities, including the NIH initiative, frequently deliver hits with still borderline activity, questionable target or pathway specificity, and little utility for broader use in experimental pharmacology.[2] The limited expertise in designing bioassays with balanced sensitivity and robustness, the general shortage of medicinal chemistry support for HTS follow-up[3], and a lack of scrutiny in 'hit' characterization constitute major factors preventing a more successful application of HTS in the academic sector.

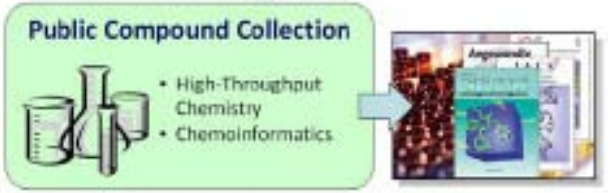
Problem statement

As described above, Pharma's capability to generate a sufficient number of innovative drug candidates is under increasing pressure. Pharma's discovery organizations are increasingly

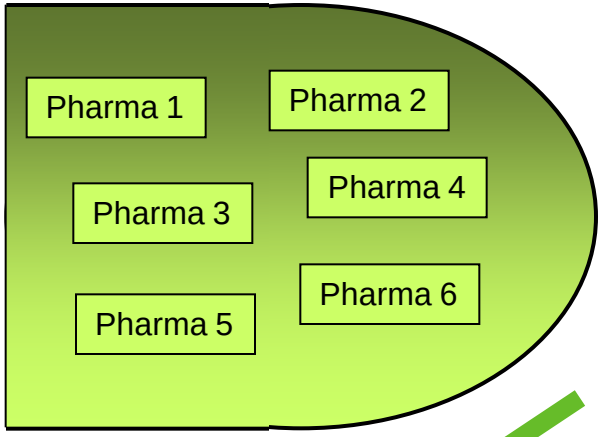
[1] Austin, C.P. *et al.* (2004) *Science* **306**, 1138-1139
2 Oprea, T.I. *et al.* (2009) *Nature Chemical Biology* **5**, 441-447.
[3] Frye (2010) *Nature Chemical Biology* **6**, 159-161; Kodadek (2010), ibid., 162-165





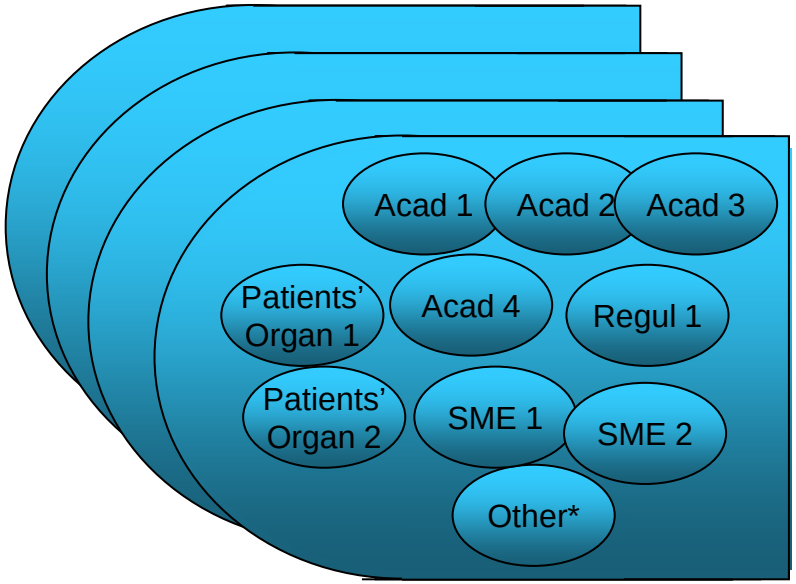


Building an IMI consortium



Step 1:

A set of EFPIA companies define a topic on which they commit to collaborate

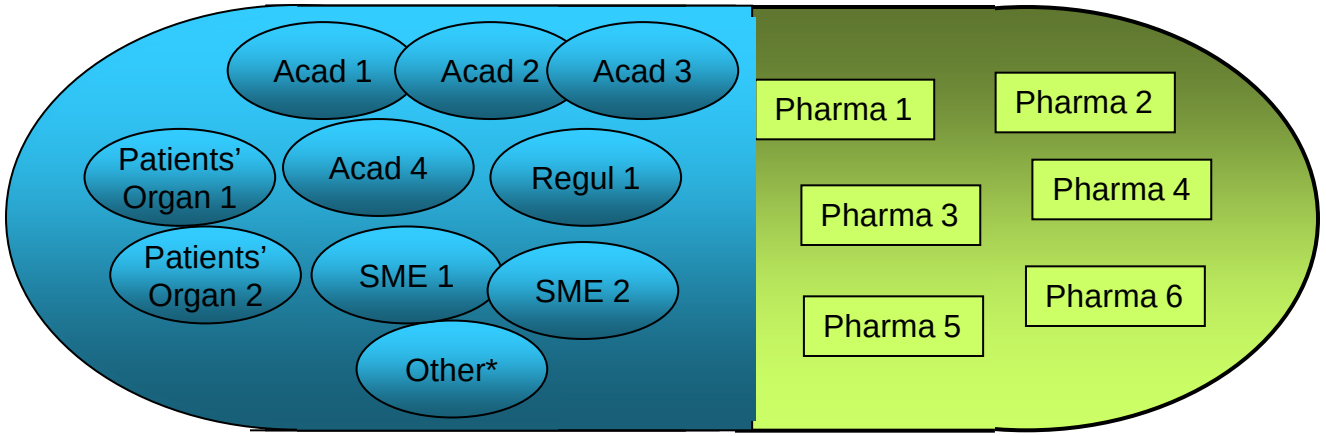


Step 2:

Consortia eligible for EU funding compete through Expressions of Interest which are ranked by independent experts

Step 3:

The top-ranked EU-fundable consortium joins the EFPIA companies to form the final consortium which develops the full proposal, subject to peer-review before final approval



CONCLUSIONS

- **Drug Discovery public-private partnership** projects growing worldwide.
- **NIH Molecular Libraries Program, EU-OPENSCREEN, Chembiobank** are examples of *Chemical Biology* initiatives that may eventually lead to Drug Discovery projects.
- The **European Lead Factory: Joint European Compound Library and Screening Centre** proposal from the *5th Call of the Innovative Medicines Initiative (IMI)* may provide new lead compounds for Drug Discovery projects through a Public-Private Partnership Consortium.