



ΠΑΝΕΠΙΣΤΗΜΙΟ ΚΥΠΡΟΥ
ΤΜΗΜΑ ΧΗΜΕΙΑΣ



Sixth Framework Programme LIFE SCIENCES, GENOMICS AND BIOTECHNOLOGY FOR HEALTH

To
Workshop Delegates and
DG Research, EU Commission

Summary Outcomes Report

Workshop on “How to Establish a European Technology Platform for Innovative Medicines”, April 21-22, 2005, Barceló Hotel Sants, Barcelona, Spain.

The preliminary summary report has two objectives:

- 1) To provide workshop delegates with an overview of the Innovative Medicines Initiative, its purpose and potential functions. It could also provide a basis for future work on national levels and in professional settings.*
- 2) To present recommendations and conclusions from the workshop.*

A full report on the outcome of the workshop will be issued in July 2005.

Background

A European Technology Platform is a concept introduced by the European Commission to:

- Bring together all interested parties in a particular sector. The sector should be chosen for its strategic importance to contribute towards the EU's goals of knowledge-based growth, competitiveness and employment.
- Foster effective public-private partnerships and bring together key stakeholders, under the leadership of industry, around a shared vision for the development of the technologies concerned.
- Define the necessary research and technical priorities in the medium-long term for the sector.

A number of technology platforms are envisaged to receive funding via the Commission's Seventh Framework Programme (FP7).

To drive this forward for the biopharmaceutical sector, the European Commission asked the European Federation of Pharmaceutical Industries and Associations' (EFPIA) to identify the main barriers to innovation in biomedical research with the objective of establishing a European Technology Platform for Innovative Medicines, the Innovative Medicines Initiative, to tackle these.

The overall objective of the Technology Platform is the accelerated development of safe and efficacious medicines, aiming to bring tangible benefits to patients and revitalize the European biopharmaceutical research environment by strengthening the European science base.

The goals of the European Technology Platform for Innovative Medicines are:

- To better use the collective strength of the stakeholders of drug development through co-ordination and co-operation
- To better exploit existing European assets through collaboration
- To improve the drug development process via improved prediction of Efficacy and Safety, underpinned by Knowledge Management and Education and Training
- To create a new spirit and enthusiasm for European Drug research

The objectives of the Barcelona workshop were:

- To provide further input to the work on the four parts of the Strategic Research Agenda, Safety, Efficacy, Knowledge Management and Education and Training.
- To discuss ideas on how the implementation of the SRA could be effectively organised to attract stakeholders and achieve sustainability.
- To activate relevant European stakeholders buy in to the Innovative Medicines Initiative

Day 1

The first day was dedicated to understanding the process and content of the ongoing work on the Strategic Research Agenda. It was kicked off by presentations of the Technology Platform concept by Octavi Quintana-Trias, Director of DG Research and the Strategic Research Agenda by Jonathan Knowles, Chair of EFPIA's Research Directors Group. The work stream leaders for the four parts of the Strategic Research Agenda, presented the recommendations for Knowledge Management, Efficacy, Safety, and Education and Training, This was followed by breakout sessions, summaries are given below.

A1: Knowledge Management, chaired by Nicolas Grand Jean, Novartis, Basel CH

This session was devoted to the innovative approaches on knowledge management supporting the progress of the European pharmaceutical R&D. The main objectives are the development of an integrated environment for collaboration, the definition of a common and flexible platform for federating data, resources and computing services, and the provision of knowledge management support to the Safety and Efficacy work packages. Efforts are done on the definition of relevant logical data layers, development of powerful data resources, applications and services. Discussions during this workshop were focussed on the particularities and potentialities of the data resources. Current datasets should be annotated and curreted for their useful pooling. An appropriate design of the future datasets would overcome current limitations. An inventory of current initiatives on biomedical datasets, representation models and specialised applications should de carried out. An integrated biomedical informatics perspective (from molecules and pathways to phenotypic data, incorporating interdisciplinary expertise) was considered crucial for the progress in the understanding of disease and drug mechanisms.

A2: Efficacy, chaired by Ian Ragan, Eli Lilly, Surrey UK

The session provided an opportunity to discuss the Innomed strategy on efficacy, which has the goal of improving clinical performance and early access to innovative medicine. This will initially be addressed in four areas; cancer, brain, diabetes and inflammation, each of these priorities illustrating specific bottlenecks in drug development

The discussion pointed to the need to merge national disease-oriented research programmes and networks in the corresponding fields. It emphasized the need to build on the European added value in drug development; quality of case records, quality of databases and biobanks, allowing further investigation of the clinical features and biomarkers predicting the response to treatment. Active participation of patients and adaptation of regulatory procedures was discussed. The participants agreed that the planned research and development is important, and debated the scope of the non-competitive areas, and the relevance of the four priorities selected. An important point was the definition of competitive vs. pre-competitive fields as this may differ for big pharma, SMEs and academia.

A3: Safety, chaired by Friedlieb Pfannkuch, F. Hoffmann La Roche, Basel CH

The chair presented the three priority topics for the Safety part of the Strategic Research Agenda for the Innovative Medicines Initiative

1. Framework for biomarker development & *in silico* methods
2. Relevance of non-genotoxic carcinogens and other intractable toxicities
3. 'Virtual' European Office of Toxicology

The participants debated all three items intensely. They felt that it would be necessary to create a governance structure (The “Virtual” EU Office would be ideal), which will then have to prioritize and manage individual projects such as under priority topics 1 & 2 above. It was agreed that the relevance of non-genotoxic carcinogens and other intractable toxicities, is both important and relevant and would indeed impact positively the European scientific environment, the participants made specific recommendations on how to proceed with implementation.

A4: Education and Training, chaired by Jørgen Dirach, Novo Nordisk, Bagsværd, DK

The discussion group on Education and Training initially discussed issues leading to key statements pertaining to the content and objectives of Education versus Training, as well as to the identification of gaps and weaknesses in E&T. Specific issues were identified related to barriers, coordination, flexibility, quality and mobility. Consequently, objectives, key success factors, performance measures, quality management criteria as well as major criteria and characteristics for stakeholders to be on an E&T Council were defined. Universities and Higher Education Institutions should remain the centers for InnoMed related E&T but have to increase flexibility within their curricula and open their courses to participants from industry and utilize industry competence in the faculty. Private vendors may also be considered provided adequate quality. Since Universities are currently changing their curricula to fit to the Bologna architecture there was a consensus that Academia should be providing the bases for productive training by offering advanced courses on emerging disciplines and technologies. It was proposed that Marie-Curie type chairs should be provisioned for industrial people returning to Academia and agreed that a central co-ordinating body with expert sub-groups to assess the availability and quality of training in preclinical, clinical and holistic/integrated drug development should be considered.

Day 2

The second day of the workshop was devoted to presentations and discussions on future stakeholder involvement in the Technology Platform, to bring forward strengths and weaknesses in a bottom up process, along with opportunities and threats that different groups are facing. This functioned as input on how to contribute to improving European competitiveness and

strengthening the EU science base. A summary of these presentations can be found in Appendix 1. The presentations were followed by breakout sessions, which are summarised below.

B1+B2: Issues on the establishment, attractiveness, organisation, functionalities and sustainability of the Platform, chaired by Ole J. Bjerrum, Danish University of Pharmaceutical Sciences, Copenhagen DK, and Jordi Cami, Institut Municipal d'Investigació Mèdica, Barcelona ES.

An organisational structure for the implementation of the Strategic Research Agenda is one way of securing the roll out of the proposed R&D. Such a structure must serve the objectives of the Strategic Research Agenda and its four parts. For these objectives to be successfully achieved the right stakeholders must be interested and participate actively, thus the structure should be organised to be attractive and accessible for potential relevant participants. In this context it is crucial that participation in projects is based on quality criteria. The complexity of the drug development process involves numerous skill sets across various stakeholder groups. For these to collaborate at European level any implementation structure must be transparent and clearly organised. Involving organisations representing stakeholders at the European level will facilitate the establishment of such an implementation structure. The session proposed the following major European clusters of stakeholders involved in the drug development process. Universities and Research centres, Pharmaceutical Industry, Small and Medium size Enterprises SME, Regulatory and Quality authorities, Investors and, last, but not least, Patients.. For some of these stakeholders the representatives are already structured at European level (e.g. EFPIA, EMEA, EDQM, EIB, EIF, EPF). The participants also proposed for EU Networks of Excellence to be present.

Should a separate structure be used to implement the SRA, it should have clear and transparent rules for decision-making. An Executive Platform Committee could make decision with consultation of a European stakeholder forum. A scientific board should be involved with the implementation of efficacy, safety, knowledge management and training and education. Such a structure may have a stable core with a programme plan that envisages funding for the duration of FP7 and potentially beyond. The participants were in favour of the concept of a public-private partnership support as a means for financing the platform.

B3: How to build on existing strength of the stakeholders to better exploit existing assets and resources and create values for industry and SME's, chaired by Jacques Demotes, ECRIN, CIC INSERM, Bordeaux FR.

Existing assets and resources in Europe include a high number of scientific publications from academia, a high level of technology in health systems and clinical research, and a growing innovative SME sector. Furthermore the regulatory agencies are open to discussion, and patients' organisations are willing to be an active partner. However, when discussing how to better exploit these European strengths, several levels of fragmentation appeared as bottlenecks to an efficient partnership: Translational gap between preclinical and clinical steps, insufficient networking at the EU level for basic research, and even more for clinical research and for patient's associations and cohorts; insufficient co-operation between industry, academia and patients. The participants' main conclusion was that networking and European integration is critical in building on European strengths and to improve the European science base. The industry needs academic networks to improve efficiency in preclinical and clinical development. Various pilot models for such networking were discussed, both for biotechnology development and for clinical evaluation.

B4: Post graduate training, chaired by Heidi Foth, University of Halle, Halle DE

An important limitation in academic training that had been identified is that practical skills are vanishing from the curricula of universities as a result of general structural changes, which had been started in almost all countries of Europe. On the other hand, the boundaries between disciplines are vague in term of basic research at molecular levels. The urgent need to strengthen applied sciences is emphasised, because academic training is focussing too early in academic life and the skills of general awareness are vanishing (if not already been lost) in broad areas of research.

Concerning safety science issues, the need for specific postgraduate training activities in toxicology was taken up years ago and postgraduate training programmes were established in several countries in Europe. The existing experience shows that a multidisciplinary or interdisciplinary context can be established only on the basis of core scientific disciplines of individuals. The contents of courses must be thoroughly planned on basic, intermediate and specialised levels. The outcome of postgraduate training must be acceptable by industry, regulatory bodies and academia as well and for this certifications are needed. Participants should be trained to develop a balanced opinion. They will have to identify and handle conflicts between data or conflicts between hypotheses and practical experience.

Excellence in postgraduate training is a matter of contents and of a sound balance between new matters and established knowledge, which can rarely rely on the skills of individuals or individual institutions. Excellence in postgraduate training is also a matter of long-term activity and it cannot develop a reliable strength within normal time lines of a scientific projects. A critical success factor for such activities is that they be established in a fashion, which is independent from a turn over within professional staff. This can only be guaranteed if the driving force is taken up by a strong liaison between academia, professional bodies, industry and regulatory bodies.

B5: University research and education, incl. the forming of collaboration between basic and clinical research, chaired by Daan J A Crommelin, University of Utrecht NL

The session reached consensus that it would be valuable for a Pan-European initiative to set up training programmes in Drug Development at Universities and European Diplomas should be installed if training is expected to increase the overall EU competitiveness in drug development]. General issues debated included:

- Interdisciplinary training activities;
- The need to identify regional initiatives and European ‘best practices’;
- Student levels at which training should be provided as well as multiple entrance possibilities;
- Flexibility of training programmes and performance evaluation.

Training requirements for animal experimentation was emphasised. Further considerations included the predictability of the demand for training, for the needs of trainees and the existence of courses in the drug development area to serve as starting point. The participants agreed to propose an evolutionary approach that could start quickly by adjusting existing MSc and post-graduate training programmes and gradually broaden the offerings with new modules. High quality courses in Safety Sciences, as well as related PhD studies could be offer by centres of

excellence. Nevertheless, too early specialisation should be avoided. A task force to determine the programme content was highly recommended.

Conclusions

There was general agreement throughout the workshop that implementing the European Technology Platform for innovative Medicines is an important component in re-establishing Europe as the primary location for biopharmaceutical research and development. The stakeholders acknowledged the value of industry leadership, the importance of the four topics of the Strategic Research Agenda, and the pre-competitive approach. The disease areas selected are appropriate for urgent research and do not reflect priority diseases of the FP7 life science programme.

Education and Training is a critical component of the Strategic Research Agenda. The strengths to support pharmaceutical industry needs do exist, however, fragmentation on a European level, including lack of collaboration between universities and higher education institutions hinders a coherent and coordinated approach. This highlighted the need for a pan-European organisation of academic institutions engaging in drug development. This, along with the lack of sufficient dialogue between the stakeholders of the drug development process, represents an important hurdle for efficient collaboration. If properly organised the Platform may contribute to remedy this gap.

All stakeholders play an important role in drug development, but as their respective SWOT analyses demonstrated, there are weaknesses to be addressed. Supportive functions of the Platform may optimise the stakeholder contributions, thereby securing more efficient use of the stakeholders' existing European strengths.

For academia the weakness concerns lack of critical mass of research groups, scientist and student mobility, new technologies, trans-disciplinary issues and positive public perception. A pan-European drug development organisation is highly needed to exploit the existing European strengths of the sector.

The clinical sector's contribution to European competitiveness can build on the quality of clinical research infrastructures, capacity of investigation, databases and biobanks, allowing to best exploit biomarkers and clinical data as predictors for safety or efficacy. In addition, the industry needs Europe-wide networks to make clinical research more efficient – infrastructure networks that provide harmonised tools and practice in Europe, research networks encompassing preclinical and clinical research, investigators' networks and patients' registries that facilitate enrolment.

For SMEs weaknesses include inefficient technology transfer from basic research, lack of management expertise, and holistically educated developers, as well as lack of existing accessible biology facilities, GMP units and toxicology databases.

For regulatory more research, conducted at the agencies, was recommended, e.g. by compilation of relevant generic data from old application files, openness to modern methodologies and technologies and reorientation of the regulatory assessment demands.

Learned societies which already organise scientists from academia, industry and the regulatory field, have a long tradition, based on a discipline-oriented European structure. They will, however, need to create a European organisation, which covers the complete drug development

process. They could contribute by participating in a number of the coordination functions needed for and on the forthcoming platform.

Active involvement of patients and patients association's will promote drug development in line with patient's needs, foster their enrolment in studies, and the implementation of new treatment strategies.

Suggestions for better exploitation of existing assets and resources included more harmonisation (e.g. in clinical trials), EU-wide networking of biology facilities, GMP units, toxicology databases, and infrastructures and mobility.

There seemed to be general agreement that an "Executive Platform Committee" could be an appropriate leadership forum, but it was emphasised that the stakeholders should have a voice and consultative role through their European organisations. Various Technical offices, virtual and real, will have to be established.

Sustainability of the platform activities represents a critical issue, which was not addressed in depth. This should be further investigated.

Finally it should be noted that besides the described Technology Platform activities, the FP7 will provide its "normal" collaborative funding of life sciences research according to specific calls.

Participation

A total of 134 delegates from 21 countries accepted the invitation to and joined the 1½ days Workshop, on April 21-22, 2005, in Barcelona, 1/3 representing industry (19 from big pharma and 31 from SMEs), 1/3 academia and the remaining 1/3 the European Commission (8), regulatory agencies (5) and additional organisations (22), respectively. The female ratio was 1 to 5.

Postscript

The successful implementation of the European Technology Platform on Innovative Medicines, and thereby the strengthening of European competitiveness, will depend on the engagement of the stakeholders. Even though the Technology Platform has not yet been officially adopted for the 7th Framework Programme for Research and Technological Development, the concept is progressing with high Commission priority. Why not prepare yourself and your organisation for the final outcome.

Copenhagen, May 27, 2005

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Appendix 1: SWOT Summary