

The importance of multi-national trials
for rare diseases – the Euramos model

3- Funding pan-European Trials: Role of ESF

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EMRC (European Medical Research Councils) Head of Unit

Membership in ESF

- 30 Countries
- 78 Member Organisations



■ 29 Countries

■ 38 Member Organisations

| | | | |
|-----------------|------------------------------------|--------------|------------------------------|
| •Austria | FWF | •Greece | NHRF |
| •Belgium | ÖAW | •Hungary | OKTA |
| •Bulgaria | FNRS | •Iceland | Hungarian Academy of Scien. |
| •Croatia | FWO | •Ireland | RANNIS, |
| •Cyprus | Bulgarian Academy of Sciences | •Italy | Health Research Board |
| •Czech Republic | Croatian Academy of Sciences & Art | •Lithuania | CNR |
| •Denmark | CRPF | •Luxembourg | Lithuanian State Science and |
| •Estonia | Acad. Of Science of Czech Rep | •Netherlands | Studies Foundation |
| •Finland | Danish Medical Research Council | •Norway | Fonds National de la Rech. |
| •France | Estonian Academy of Sciences | •Poland | NWO |
| •Germany | Estonian Science Foundation | •Portugal | KNAW |
| | Academy of Finland | •Romania | Norwegian Research Council |
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| | Inserm | •Spain | FCT |
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| | DFG | •Switzerland | National University Research |
| | | •Turkey | Council |
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| | | | Sciences and Arts |
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| | | | Medical Research Council |

Mission of EMRC

- The mission of the European Medical Research Councils (EMRC) is to promote innovative medical research and its clinical application towards improved human health.
- EMRC offers authoritative strategic advice for policy making, research management, ethics, and better health services.
- In its activities, EMRC serves as a voice of its Member Organisations and the European scientific community.
- EMRC disseminates knowledge and promotes the socio-economic value of medical research to the general public and the decision makers.

EMRC EUROCORES

ECT Pan European Clinical Trials:

Profidys Coordinator:

Prof. Philippe Orcel
 Inserm - France

Euramos Coordinator:

Prof. Stefan Bielack
 Olgahospital - France

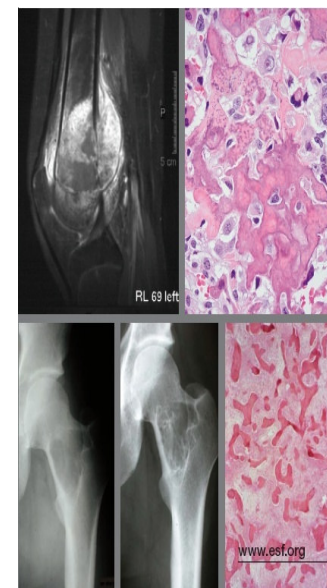
Abstract:

2 Pan-European Clinical Trials on Rare Diseases and Paediatrics funded for 6 years from February 2005:

EURAMOS: osteosarcoma

PROFIDYS: fibrous dysplasia of the bone

12 European countries (AT, BE, CH, DE, DK, FI, FR, HU, NL, NO, SE, UK) + USA + Canada



Activities in 2008:

- PROFIDYS Steering Committee Meeting, 22 January, Lyon, FR
- Global PROFIDYS Conference, 22 January, Lyon, FR
- Pan-European Clinical Trial Training Course 24/25 January, London, UK

Budget:

2,3 M€

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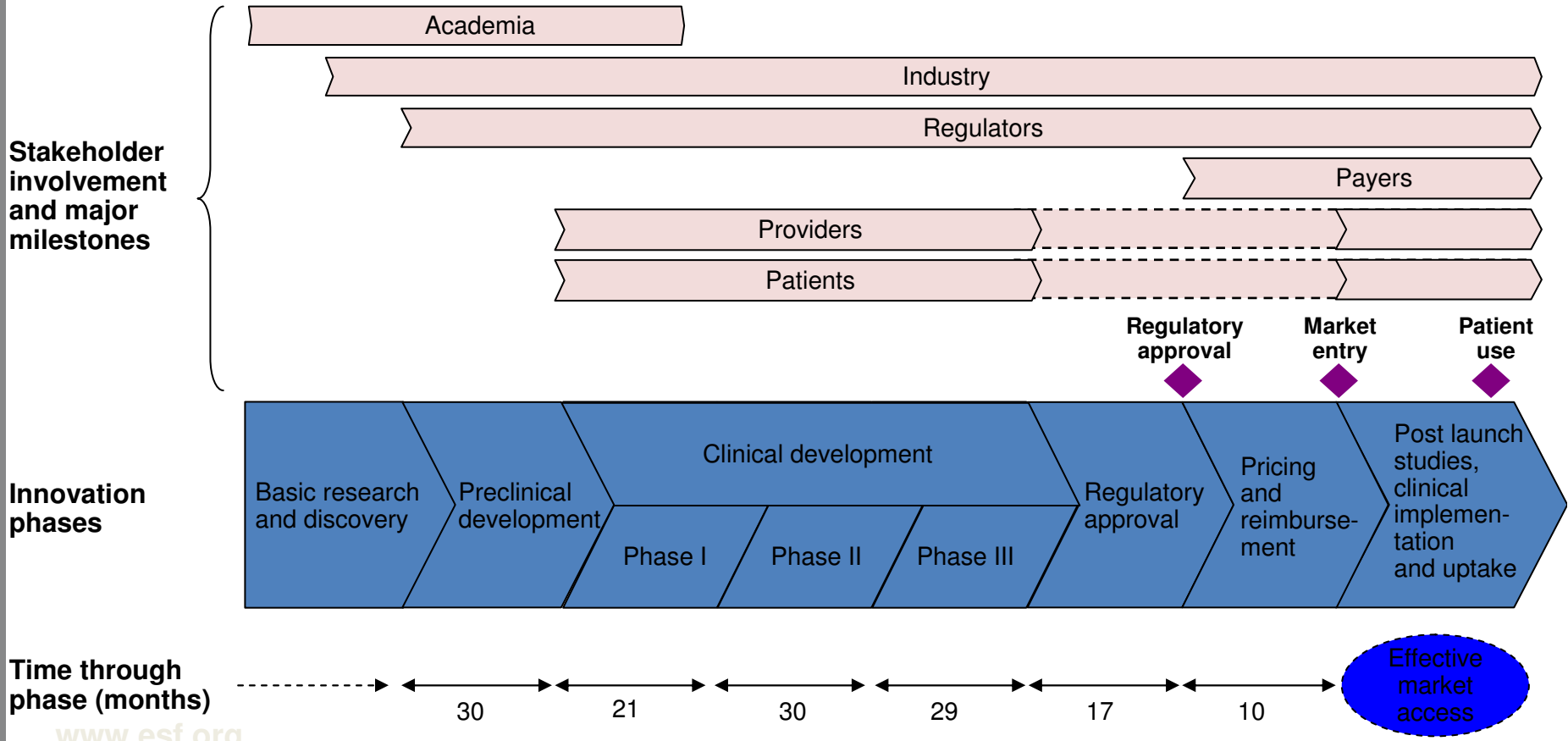
Johanne Martinez-Schmitt
 e-mail: jmartinez@esf.org

Medical Innovation: Paradigm Shift

- Paradigm shift from large markets to personalized/individualized medicine and need for knowledge transfer across disciplines and research sectors (e.g., academia & industry)
- Patient centered process
- Patients unmet needs (WHO, 2004)

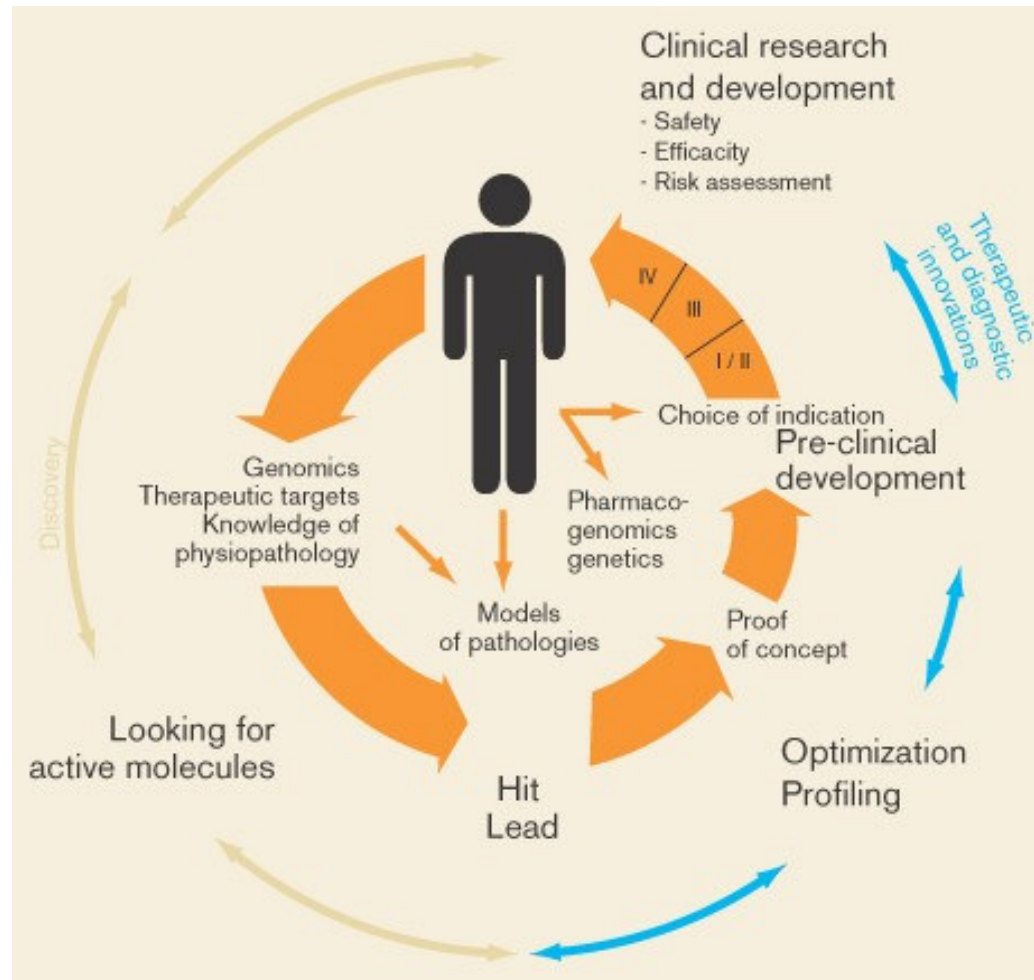
Medical Innovation: Paradigm Shift

Current process for the development and commercialization of drugs in Europe (EBC EFNA)



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Patient Centered Process

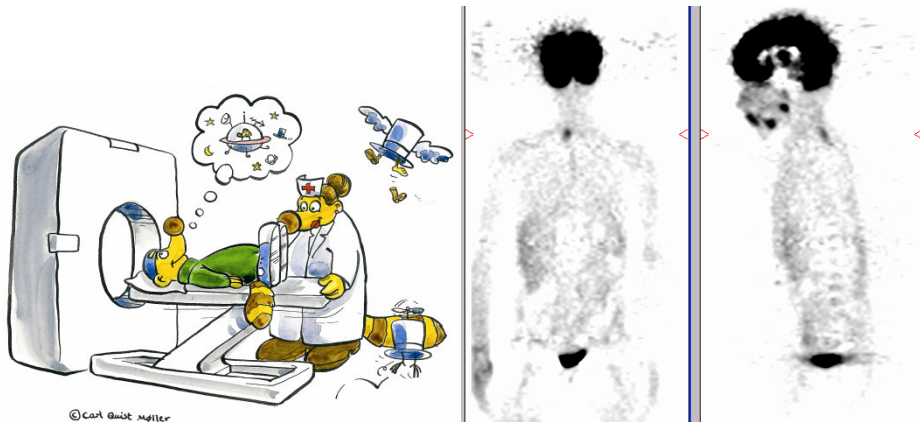


Priority diseases for Europe

(WHO, 2004)

17 priorities

- Diseases for which **basic and applied research is required**: cancer; acute stroke
- Diseases for which **biomarkers are absent**: Alzheimer disease; osteoarthritis
- Diseases for which **better formulations** are required: cardiovascular disease (secondary prevention); diabetes; postpartum haemorrhage; paediatric HIV/AIDS; depression in the elderly and adolescents
- **Neglected diseases** or areas: tuberculosis; malaria and other tropical infectious diseases such as trypanosomiasis, leishmaniasis and Buruli ulcer, HIV vaccine
- Diseases for which **prevention** is particularly effective: chronic obstructive pulmonary disease including smoking cessation; alcohol use disorders: alcoholic liver diseases and alcohol dependency
- **Future public health threats**: infections due to antibacterial resistance; pandemic influenza



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On the European level there is a need to develop, coordinate and adopt better systems for **evidence-based medicine** and **health technology** assessment for recommendations of standard practice in patient care.

In conclusion



EMRC: Clinical and Translational research should be strengthened in Europe

NIH in USA: Clinical and Translational research very important focus areas

Launch in 2007 of the ESF-EMRC Forward Look on

“Investigator-Driven Clinical Trials”

Chair: Prof. Jurgen Schölmerich (Vice-President DFG) in collaboration with NIH and FDA

Forward Look (FL)

FL07-01: Investigator Driven Clinical Trials (IDCT)

Abstract :

This Forward Look will develop over the coming year and lead to a consensus conference (tentative date 16-17 September 2008, Strasbourg) followed by the publishing of a report highlighting the key recommendations on how to better coordinate the various national and European initiatives in this domain and overall strengthen investigator-driven clinical trials in Europe in an international perspective.

Status: Launched in July 2007

Budget :

160 000 €

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Julien Weber
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- Categories and Design of Clinical Trials
- Regulatory and Legal issues, IPR and Data sharing
- Funding and Models of Partnerships
- Management and Logistics of Clinical Trials
- Education and Training, Career and Authorship

ESF Regulatory and Legal issues

- **Regulatory and legal status:** was mainly targeted to address large Pharma R&D, need to support Academia and Biotech and to establish early dialog with Competent Authorities
 - 1/ Actual references in Europe for medicinal drug on human use: First in place at the international level (US, EU, JP) ICH E6 for GCP (1996)
 - EU Directives: 2001/20/EC (and guidelines) enforced by 1 May 2004 and completed by 2005/28/EC
 - 2003/94/EC for GMP
 - EU Directive for medical devices 93/42/EC
 - EU Directive for personal data 95/46/EC
 - EU Regulation for paediatrics and rare diseases and thoughts are given in US to develop specific regulation for women and ethnic groups

SW Regulatory and Legal issues

2/ Other guidelines incl. draft for specific cases and new situations:

- Draft guidance on 'specific modalities' for Non-Commercial Clinical Trials referred to in Commission Directive 2005/28/EC laying down the principles detailed guidelines for good clinical practices
- Draft guideline on requirement for first-in-man clinical trials for potential high risk medicinal products (EMA/CHMP/SWP/28367/2007): including chemical and biological medicinal products. It covers the first administration of a single dose of high-risk medicinal product and the initial single ascending dose phase of clinical development

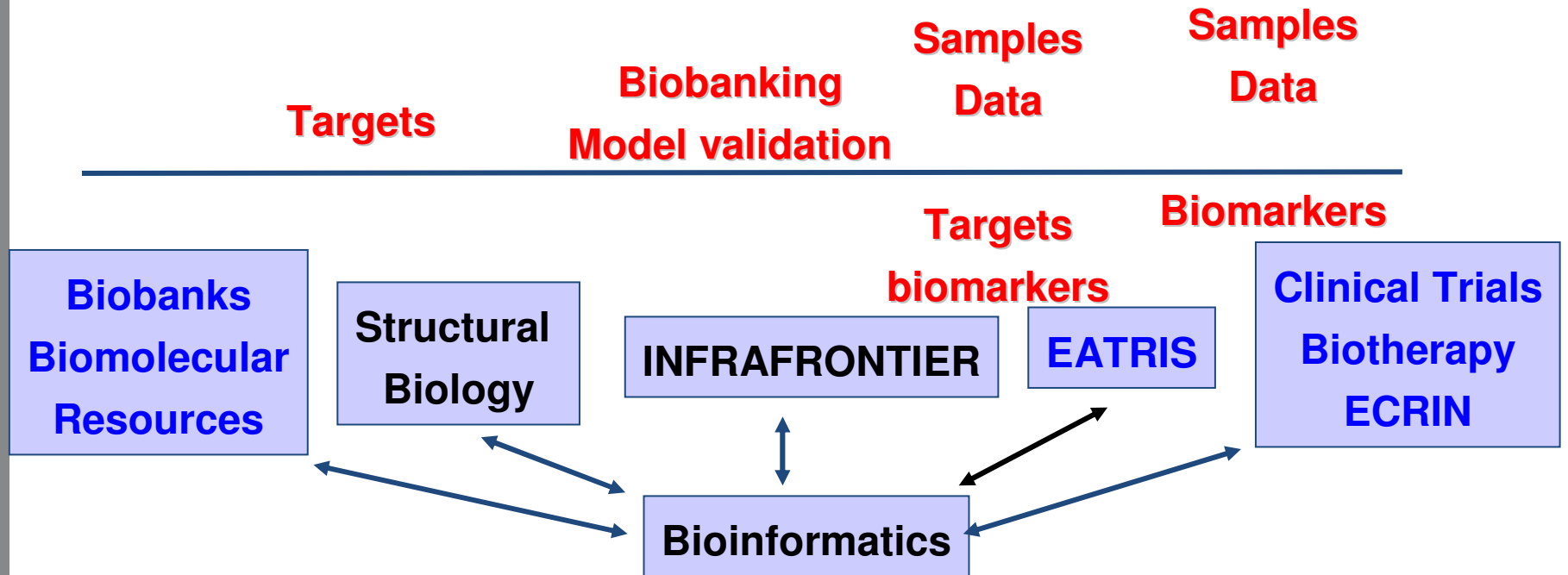
To be developed?

- Diagnosis
- Theranostics
- Population survey and biobanking
- Physiology and physiopathology
- Surgery
- Socio-Economic Studies

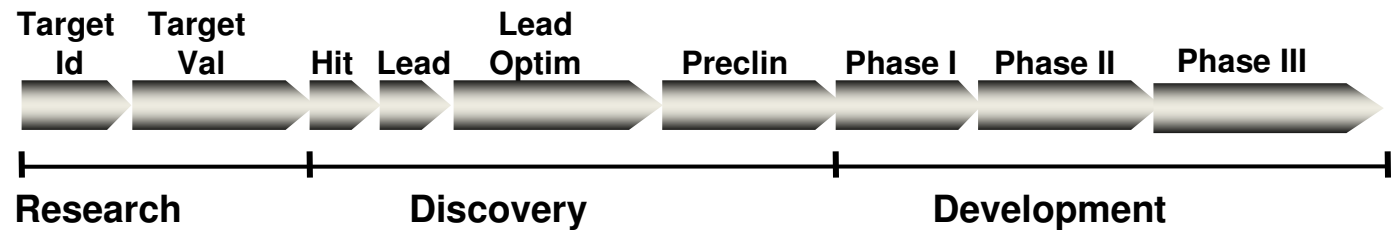
SW Management Logistics

ESFRI Initiatives in BMS

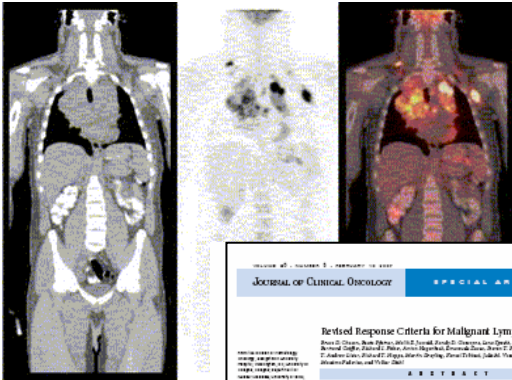
Synergies between BMS ESFRI Infrastructures : *Concerted approach*



Prof. C. Bréchet (former Inserm CEO)
ECRI 6 June 2007
Hamburg (DE)



The special case of medicine

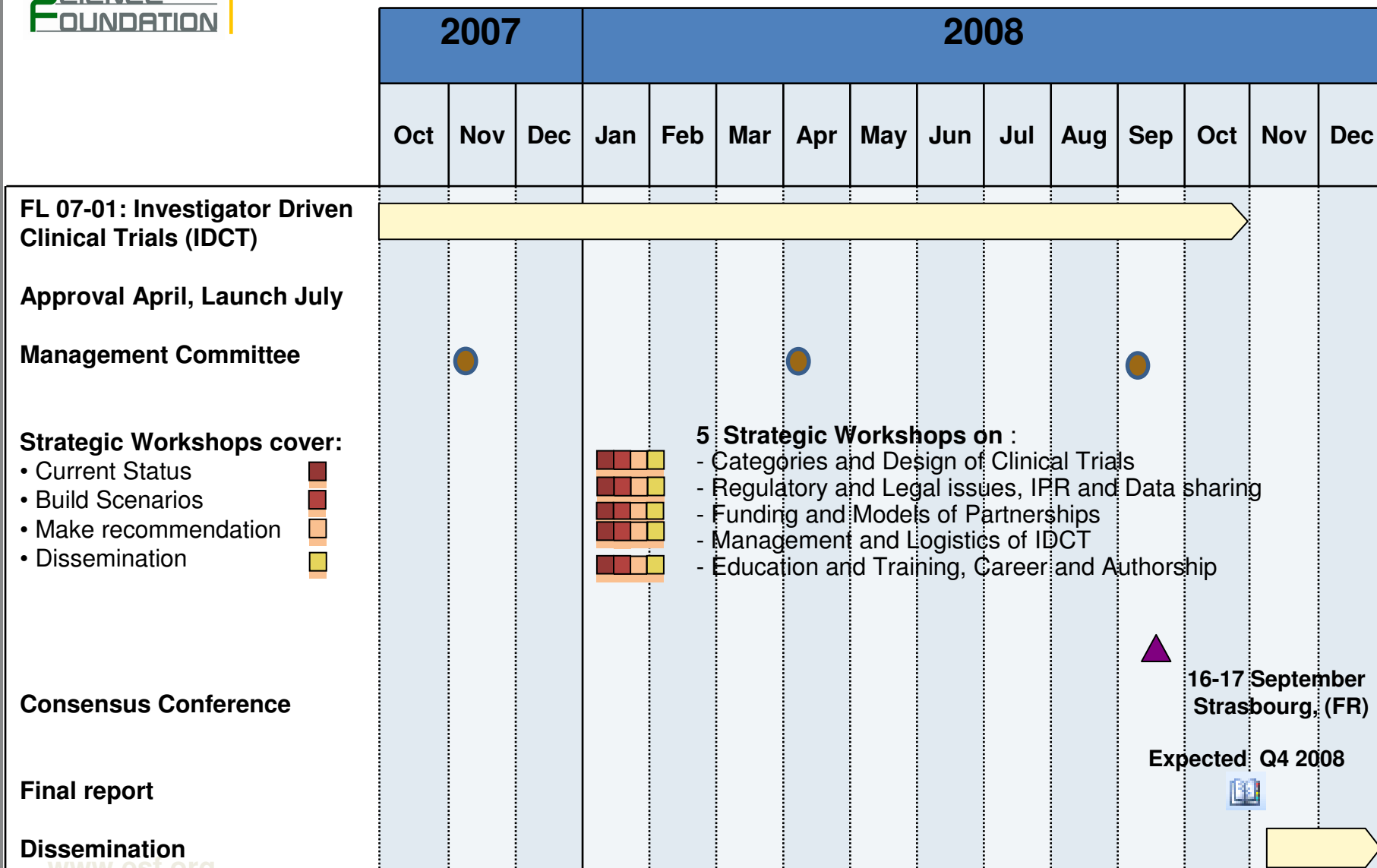


The **University Hospitals** produce the majority of research in health & lifesciences in Europe.

Need for **GMP facilities**

ESF - EMRC FL 07-01

'Investigator Driven Clinical Trials' Time Line



Thank you for your attention