

IRDiRC as a multilateral research cooperation framework with US NIH, ...

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Fund for Health Research	
 MINISTERIO DE ECONOMÍA Y COMPETITIVIDAD	 Instituto de Salud Carlos III
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SPAIN	

Ways of successful science, technology and innovation cooperation between Europe and the USA

BILAT USA & SFIC

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ISCIII.

MISSION



- is a Public Body of **Spain** with legal personnality whose main mission on Reearch is:
 - *the generation of scientific knowledge in Biomedical and Health Sciences and to promote innovation in health care and disease prevention*
 - reports to the Ministry of Economy and Competitiveness and the Ministry of Health, Social Services and Equality.
- It manages the Strategic Action for Health **Research** (AES) within the National Plan for R+D+I.

Budget = 333 M € (2010)



SPAIN. PLANING AND FUNDING FOR R+D+I

PLANING

- NATIONAL PLAN (NP) R+D+I → Ministry of Economy & Competitiveness (MINECO)
- STRATEGIC ACTION FOR HEALTH *RESEARCH* (AES) → *National* Institute of Health “Carlos III”
- NATIONAL STRATEGY FOR INNOVATION (NSI) → Ministry of Economy & Competitiveness (MINECO)

FUNDING

- GENERAL DIRECTORATE OF SCIENTIFIC & TECHNICAL RESEARCH, MINECO → NP R+D+I (no prioritised Research)
- ISCIII → AES

RESEARCH

EU:

- ERA-Nets
- TFEU art. 185/ex 169, JPIs, ESFRI-ERICs, ...

Intramural

ISCIII's Reference Centers (7)

Extramural

•250 M € (2010)

Competitive

Strategic Action for Health Research (AES)

- Projects & infrastructure grants
- Research & Technical Manpower
 - Fellowships
 - Contracts
- Mobility & Sabattical
- **Loans**

Structural

- Participated Centers
- Foundations (3)
- Consortia (10)
- Omics Platforms (4)
- Health Research Institutes (16)
- Alliances
 - Joint Centers
 - National-Regions-EU/international.



ISCIII. POSITIONING IN RARE DISEASES (RD) RESEARCH [i]



ISCIII's RESEARCH FUNDING (2010)

• **47 COMPETITIVE GRANTS**
5.03 M €

PERFORMANCE CENTERS

• **RARE DISEASES RESEARCH INSTITUTE (IIER) 1.3 M €**

• **CENTER FOR BIOMEDICAL NETWORK RESEARCH ON RARE DISEASES (CIBER-ER) 5.45 M €**

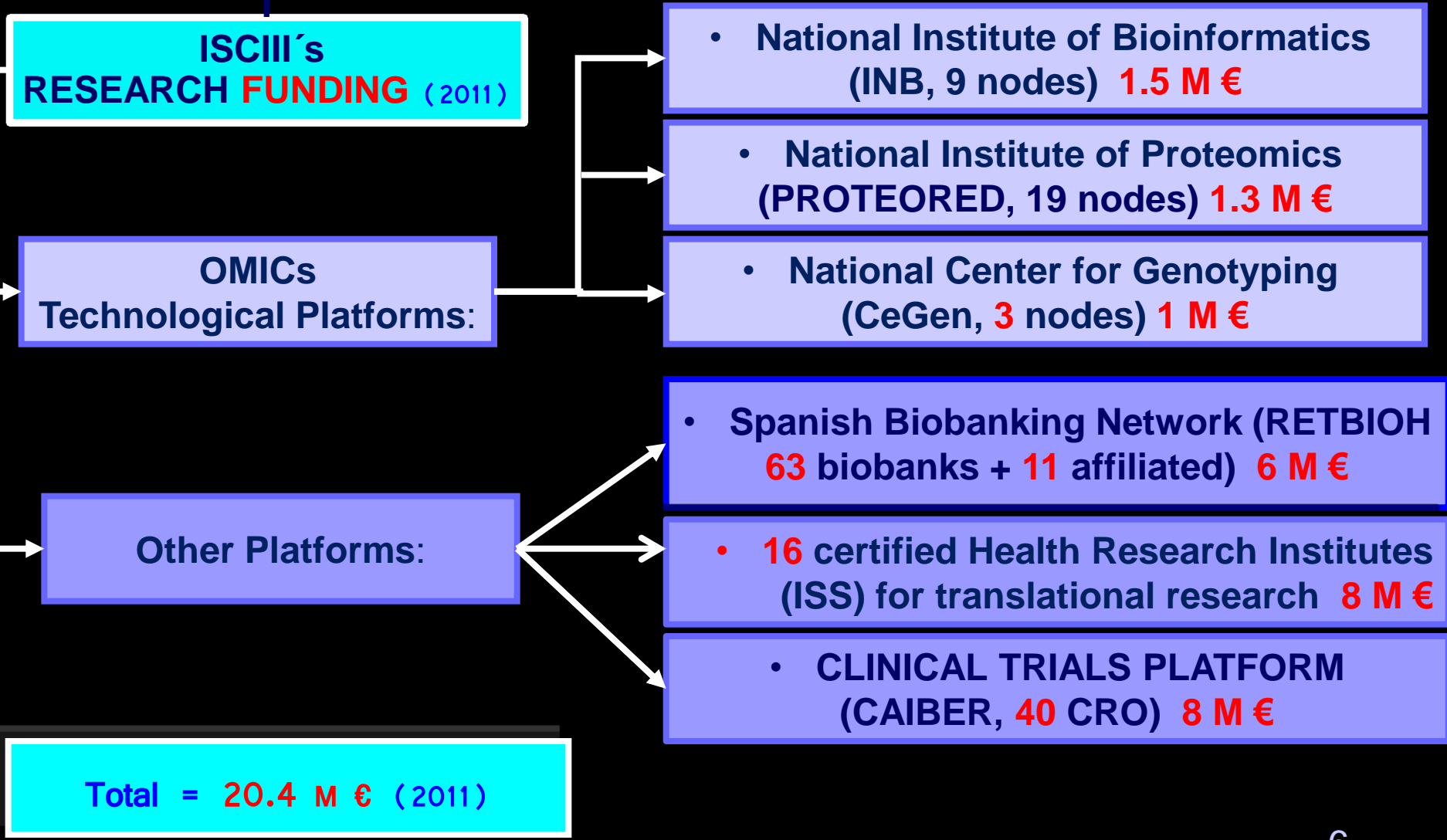
Total = 12.28 M € (2010)

• **OTHER CIBERs**
0.50 M €

+ 42 COMPETITIVE GRANTS
4.8 M € (2011)



ISCIII. POSITIONING IN RARE DISEASES (RD) RESEARCH [ii]





IRDiRC. Aims

IRDiRC will team up researchers and organisations investing in **Rare Diseases (RD) research** in order to achieve two main objectives by the year **2020**, namely:

- **to deliver 200 new therapies for RD and**
- **means to diagnose most RD.**

An **IRDiRC** funding member should invest **≥ US \$10 M** **over 5 years** in **RD research projects/programmes** contributing towards **IRDiRC** objectives.

- **Single Funding Body.**
- **An Alliance of Funders contributing together too.**

Eligible commitments: **RD research Projects/programmes**
since **01/01/2010**



IRDiRC Executive Committee. Funding Bodies



- Western Australian Department of Health, Australia
- Canadian Institutes for Health Research, Canada
- Genome Canada, Canada
- European Commission (DG Research and Innovation), **European Union**
- Agence Nationale de la Recherche, **France**
- Association Française contre les Myopathies, **France**
- Bundesministerium für Bildung und Forschung, **Germany**
- Istituto Superiore di Sanita, **Italy**
- Telethon Foundation, **Italy**
- The Netherlands Organisation for Health Research and Development (ZonMw), **the Netherlands**
- Prosensa, **the Netherlands**
- National Institute of Health Carlos III (ISCIII), **Spain**
- National Institute for Health Research, **UK**
- National Cancer Institute, **US**
- National Eye Institute, **US**
- National Human Genome Research Institute **US** - Mendelian Disorders Genome Centres, **US**
- National Human Genome Research Institute–Nat. Cen. Translat. Therapeutics, Library programme, **US**
- National Institute of Arthritis and Musculoskeletal and Skin Diseases, **US**
- National Institute of Child Health and Human Development, **US**
- National Institute of Neurological Disorders and Stroke, **US**
- Office of Rare Diseases Research, **US**
- Sanford Research, **US**
- Food and Drug Administration' s Orphan Products Grant Program, **US**
- Shire Human Genetics Therapies, **US**



Executive Committee



Scientific Committees

Diagnostics

Interdisciplinary

Therapies



Working Groups

Sequencing

Ontologies

Model systems

Clinical

Registries

Natural history

Biomarkers

Etc.

1 representative per funding body
1 representative per group of funders (accumulative funding)
Representatives of umbrella organisations of patient advocacy groups
Chairs of the Scientific Committees

Approx. 15 members with balanced representation of scientists, patients, industry, etc.

Representatives of funded projects



Spain. Call 2011 for IRDiR Collaborative Research Joint Proposals (US \$ 10 M)



a) RD Patients' Registries, serving IIER as core and in cooperation with the relevant Units of the Autonomous Communities of Spain.

- Up to one **5 year** project only funding the launching with up to **2.4 M € for 3 years**.

- **1 project funded: RDR**

b) Translational collaborative research project proposals.

- Up to **4** proposals (overall funding up to **7.6 M € for up to 5 years**).
- Up to one may be a high risk science proposal (up to **4 M € for 5 years**)..

b-1) diagnostic strategies: Identification, characterisation and validation of (bio)-markers for diagnosis and prognosis, and therapy monitoring, development of innovative screening systems and diagnostic tools. Proof of Concept

- **1 project funded: TREAT-CMT**

b-2) therapeutic strategies: Advanced phases in the pipeline of drug development from target validation to clinical trial. Cellular and/or animal models, and pre-clinical studies using pharmacological, gene or cell therapies. Proof of Concept.

- **1 project funded: Drugs4Rare**



On Our solutions [i]

How and why did you make your choices related to ...

- ... the preparation of the cooperation
 - Reserchers´ lobbying blended with Policy will.
 - legal (framework) agreements / letters of intent?
 - Letter of intent of Spain (ISCIII on behalf of the Ministry of Science and Innovation), April 19 th, 2011.
 - place of jurisdiction? N/A.
 - IP rules? National or according project consortium ´s agreement.
- ...the implementation during the cooperation
 - ISCIII launched in 2011 a call for collaborative joint proposal within IRDiRC aims to honour our funding commitment: fresh US \$ 10 M over 10 years.
 - Other Funding Bodies: checking national funded projects already on IRDiRC
 - management / administrative procedures before, during (and after, for a completed project] the collaboration?
 - Soft loose cooperation.
 - Researcher exchange / guest researchers?
 - Driven by the Principal Investigators of the funded projects..
- ...financial agreements, e.g. managing a common budget?
 - No, but not excluded. Financial issues are a home matter for the time being.
- ...the end of cooperation, e.g. evaluation or peer review systems?
 - This cooperation has no date line. Each Funding Body´s finance commitment should be revised and renewed every 5 years



Spain.

On Our solutions [ii]



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What about mutual access, reciprocity and balanced participation within the collaboration scheme?

- A soft loose cooperation mode allowing parties' freedom of implementation within a common **Strategic Research Agenda (SRA)** and potential cross feeding.

Why collaboration in this research field? (political will, budget, national priorities, former analysis, follow-up of something, ...)

From the perspective of **Spain**:

- Political window of opportunity.
- RD National Strategy: Disease burden, interpersonal solidarity, scientific relevance,
- Will for our capacities ´ Europeization and Internationalization.
- No scientific and financial way to afford large scale projects by a single country.



Our Recommendations

- What was the most „striking issue“ (could mean biggest challenge, but also positive experiences)
 - when preparing your cooperation agreement?
 - Complacency at interim: between starting and regular performance.
 - when implementing your cooperation activity
 - Change from local to international research performing.
- Outlook for the future:
 - Will you continue, widen, stop this activity/programme?
 - Yes.
 - Could it be „opened“ for other EU MS/ AC?
 - Yes.
- Other remarks / encouragement / criticism
 - Still in procedural evolution: learning by doing.

BILAT USA
Link 2 US



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Many thanks for your attention !!