EURORDIS Membership Meeting 2015 Madrid

From patients empowerment to an open science model:

the experience of Determinazione rara

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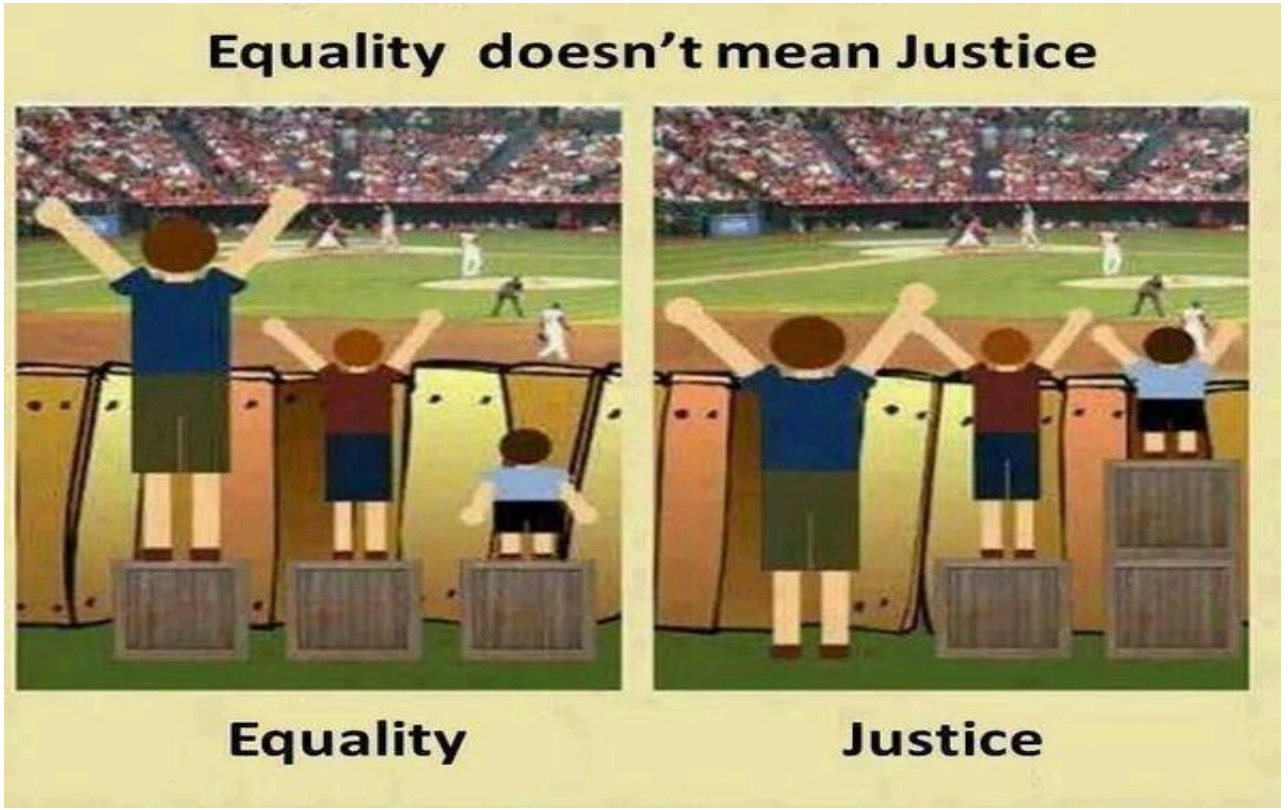
“the power of things”
a matter a facts

- ✓ From rights to capabilities:

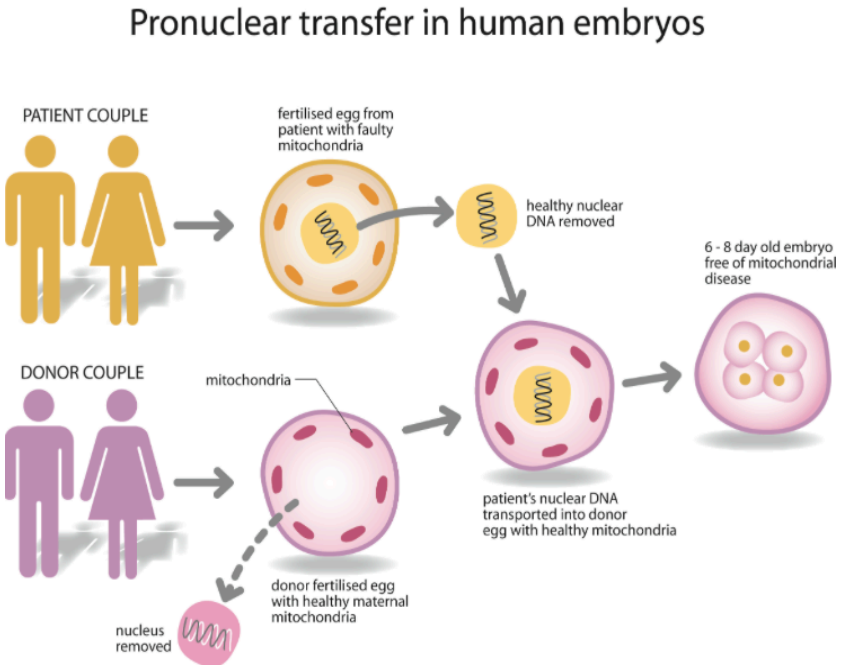
  differently equal, everyone as actor of her own life



“the power of things”
a matter a facts

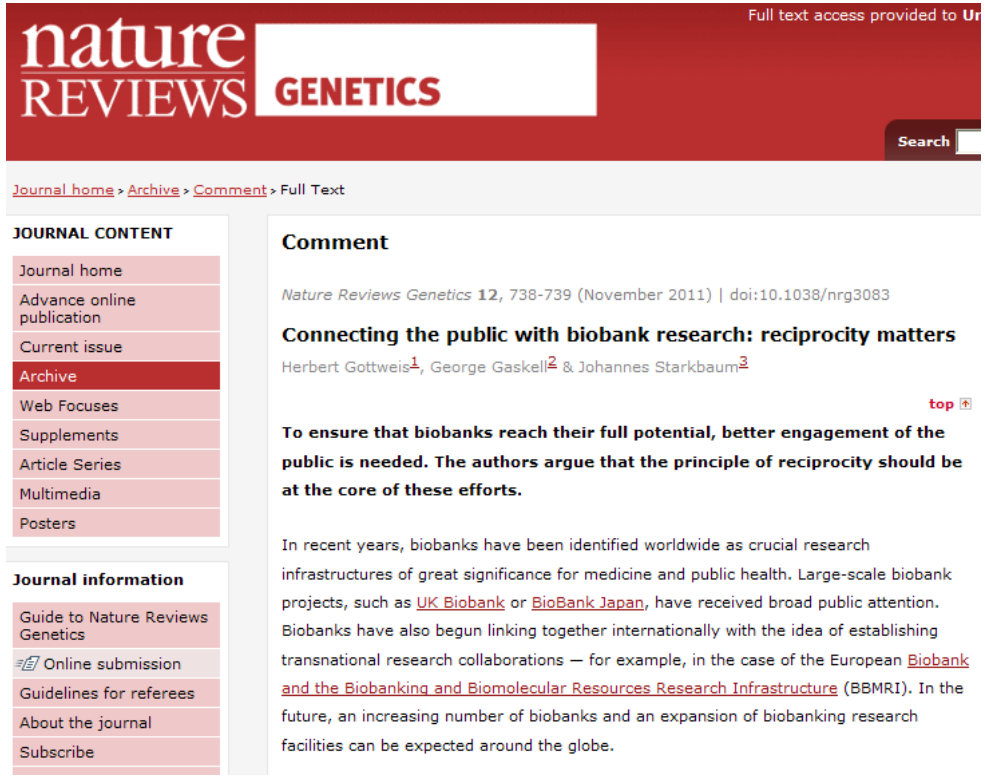
Gen-ethics & technological turn of science and medicine:

we can regenerate life. The era of regenerative & precision medicine.



“the power of things”
a matter a facts

Among global (biological, clinical, genomic) data, big data and profiling: engagement, knowledge & co-operation



“the power of things”
a matter a facts

- ✓ Transformation of Democratic models and the European welfare state
- ✓ “Compelling” awareness of limited resources

Process on Corporate Social Responsibility
in the Field of Pharmaceuticals
Platform on Access to Medicines in Europe
Working Group on Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA-OMP)

FINAL REPORT – 17th April 2013[1]

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Committee for Orphan Medicinal Products (COMP)

Email Print Help Share

The Committee for Orphan Medicinal Products (COMP) is the committee at the European Medicines Agency that is responsible for reviewing applications from people or companies seeking 'orphan-medicinal-product designation'.

This designation is for medicines to be developed for the diagnosis, prevention or treatment of **rare diseases** that are life-threatening or very serious. In the European Union (EU), a disease is defined as rare if it affects fewer than 5 in 10,000 people across the EU.

See the full overview of the COMP's role.

Composition

The members of the COMP are **nominated by the EU Member States**, in consultation with the Agency's Management Board. Six members are nominated by the European Commission. They are chosen on the strength of their qualifications and expertise with regard to the evaluation of medicinal products.

To represent patient organisations, the European Commission appoints three members. The European Commission also appoints three independent members on the basis of a recommendation from the Agency.

A Green Paper on Democratising the NHS

People Power and Health

‘Democratising expertise’ is not about ‘majority voting in science’, but rather about guaranteeing ‘due process’ in the way expertise is developed, used and communicated.

This implies principles such as **accessibility, accountability, and pluralism.**

WHITE PAPER ON EUROPEAN **GOVERNANCE**

Group 1b.

Democratising expertise and establishing Europen scientific references. May 2001

"…there are different levels of citizens' empowerment, from the ability to influence the overall administration of the health care system and to participate in the decision - making process, through the ability to further particular interests through organizations of patients or citizens, through representation on boards or executive bodies governing health care establishments, and through direct influence over the provision of health care through the freedom of choice."

Recommendation NO. R(2000) 5 of the Committee of Ministers to Member States on the development of structures for citizen and patient participation in the decision-making process affecting health care

The Expert Patient

Department of Health

Oviedo Convention– Convention for the Protection of Human Rights and Dignity of the Human Being with regard to the Application of Biology and Medicine: Convention on Human Rights and Biomedicine, 1997

Chapter X: Public Debate

Art. 28 Public debate

"…the fundamental questions raised by the developments of biology and medicine are the subject of appropriate public discussion in the light, in particular, of relevant medical, social, economic, ethical and legal implications, and that their possible application is made the subject of appropriate consultation"

“the force of things”
a matter a facts

- ✓ Gen-ethics & technological turn of science and medicine:
  We can regenerate life

- ✓ The impact of IT- Information Technology on daily life:
- ✓ among global data, big data and profiling

- ✓ Transformation of Democratic models and the European welfare state

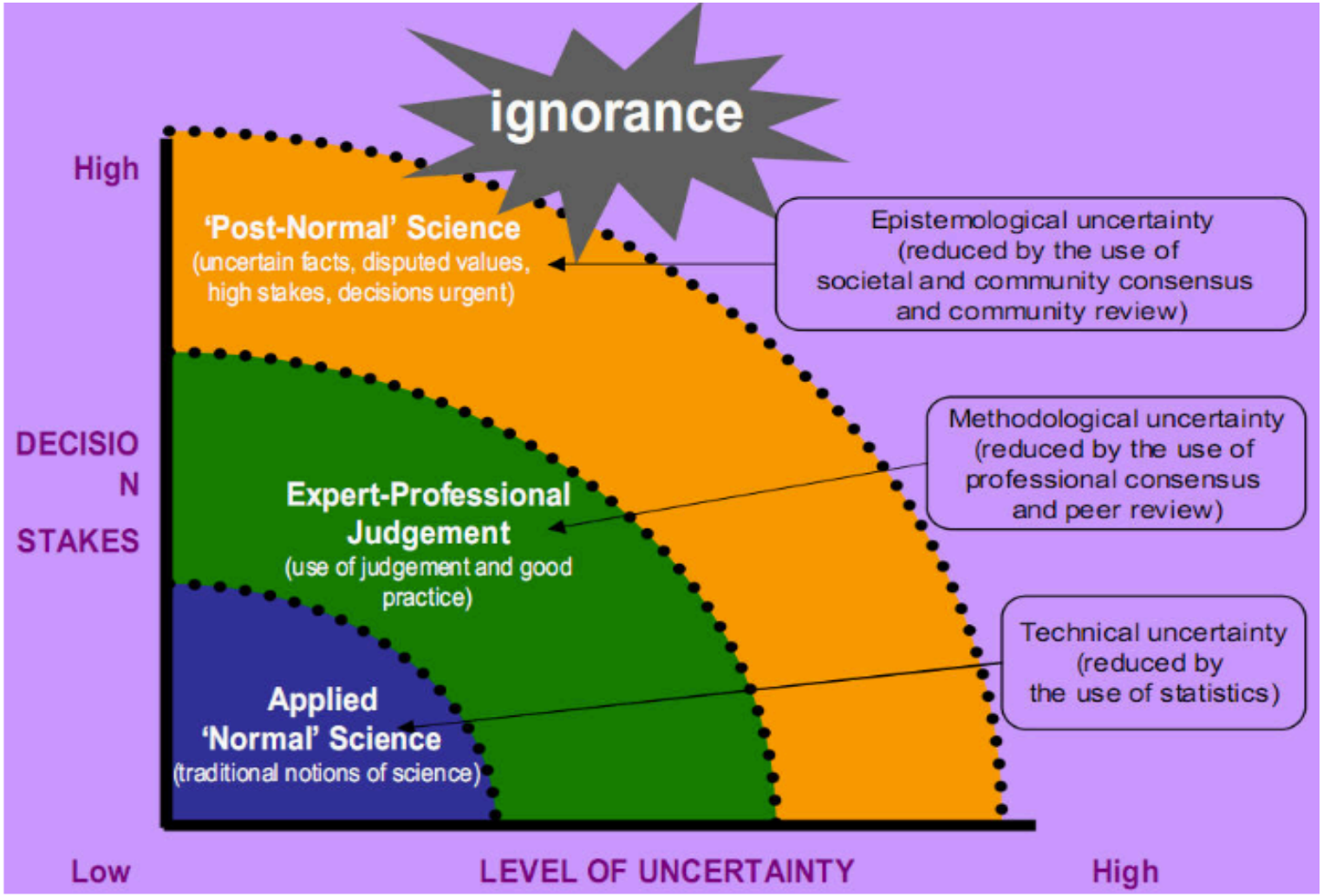
- ✓ “Compelling” awareness of limited resources

Choices of high social and individual impact at stake

inclusion & engagement = necessary step

Post- normal science

"Facts are uncertains, values in dispute,
Stakes high and decisions urgent"
(Silvio Funtowicz)



Space under construction , the complexity paradigm

- Plurality of actors
- Plurality of knowledge, experience
- Plurality of values and interests
- Variability of pathways
- Uncertainty of results

=

Dynamic knowledge & decision processes

Deliberative agorà

Participatory research

Participatory pathways, responsible research

Recognition of the other as part involved, as a partner

Systematic inclusion of

- **different experiences,**
- **Diverse knowledge and expertise,**
- **Values, interests of those involved**

Transparency in governance

Traceability and accessibility of the outcomes and results

- **Co-production of knowledge**

  Scientific knowledge is

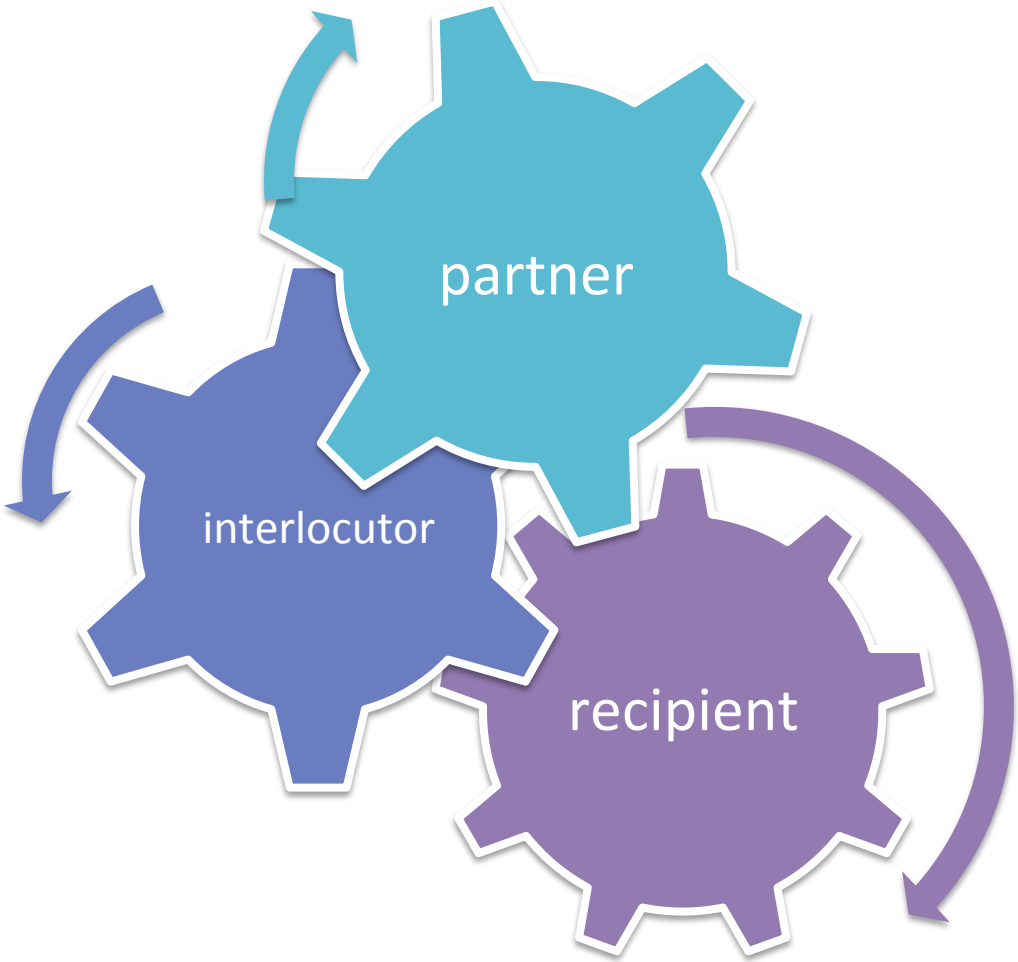
  - under construction,

  - a composition of knowledge, inclusive of knowledge of experience

- **Co-operation**

- **Co- responsibility**

Discovering partners



Planning the meal together

- Beyond an helicopter science towards a responsible science/research
- Getting out of the medical mind-set
- Empowering the system: inclusion, accountability, transparency
- Growing investigators
- Promoting a Scientific koyné and a science as Agorà

Empowering together

Modelling an innovative empowerment programme to answer the challenge of complexity

Discovering partners on the frontiers of advanced research infrastructures

Funded by the Ministry of Labour and
Social Policy. Law 383/2000 annuality 2012

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ONLUS

determinazione RARA

DETERMINAZIONE RARA: rehearsals of enlarged scientific community

A training advanced national programme

A laboratory of modelling good practice of research

Inside the research places

28 selected Patients Organizations together with

the principal National Research Institutions

AIFA – Italian medicine Agency

ISS-CNMR, Rare Disease national centre

TELETHON FOUNDATION onlus

TIGET – San Raffaele Telethon Institute for Gene Therapy

Bambino Gesù Hospital

& Infrastructures

Ethical Committees National Network

TNGB – Telethon Network of Genetic Biobanks

BBMRI ERIC IT – Biobanking & Bimolecular Research Infrastructure

some Regional Registries

Abruzzi

Lazio

Sicilia

Puglia

some Patients Registries

Parent Project Registry

RAM-NET

EPN National Registry

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DETERMINAZIONE RARA: a participatory framework

WHERE:

Crossing the threshold, inside the research places

WHEN:

from November 2013 to June 2014,

6 stages & a great public final event

HOW:

- **Information 2.0**
- **Collaborative platform**
- **cross-disciplinary, experience-based-training, peer & interactive laboratories**

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DETERMINAZIONE RARA: representation as engagement

A two-way hourglass

Enzymatic role as a promoter of education & empowerment to enable Association members in high complexity scenarios

Enzymatic role as expertise "carrier" to be active actor & coauthor in Institutional committees & contexts

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DETERMINAZIONE RARA: step by step towards a systemic empowerment

Session A POs & Bambino Gesù Hospital & TNGB & BBMRI IT

Proactively together on the frontiers of research

1. Subjects of rights, research subjects
2. Biobanking, advanced research and scientific citizenship

Session B POs & AIFA & ISS - CNMR

To care: not only orphan drug

3. From research to an early access
4. Therapies under construction

Session C POs & ISS-CNMR & Region Registries & Patients Registries

RD Registries: a virtuous circle between data and scientific knowledge

5.Good practice, data collection & POs role

6. Collecting data in order to develop scientific knoeldge: the POs in the front line

… 2014, 20 June… TOWARDS A PARTICIPATORY PACT OF RESEARCH: *EARLY DIALOGUE* WITH POS

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Participatory & open Information, indicator of good practice

Additional Protocol, 2005

Article 14 – Consent

No research on a person may be carried out, subject to the provisions of both Chapter V and Article 19, without the **informed, free, express, specific and documented consent of the person**. Such consent may be freely withdrawn by the person at any phase of the research.

Article 28 – Availability of results

1. On completion of the research, a report or summary shall be submitted to the ethics committee or the competent body.
2. The conclusions of the research shall be made available to participants in reasonable time, on request.
3. The researcher shall take appropriate measures to make public the results of research in reasonable time

Time, 13 March 2009

Biobanks

Active involvement & good practice laboratory

Recommendation 2: There should be better coordination and collaboration between national oversight bodies (e.g. data protection authorities and ethics committees) as well as mutual recognition of decision-making to eliminate unnecessary duplication of oversight and compliance requirements, with training to support this.

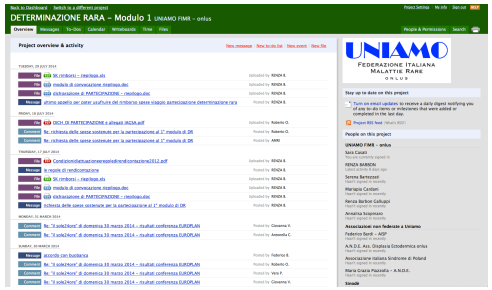
Recommendation 3: For European biobanks to operate successfully there need to be sustainable governance mechanisms to involve and engage the public, and in doing so ensure their continual participation, trust and support.

Biobanking 2.0 toward 3.0

Recommendation 8: The potential to use web 2.0 technologies to involve patients, research participants and the wider public, in the governance of biobanks should be supported to ensure that Europeans can have trust in biobank research and those organizations that establish and maintain biobanks.

Outcomes: collaborative space, tools & good practice

A collaborative digital platform



Patients Glossary

Web Application to manage patients data: towards a Patients Registry

FROM RECIPIENTS TO PARTNERS!

A co-producted toolkit

- Handbook for a good practice of informed consent in biomedical research
- Handbook for Patients Associations for a good practice of biobanking
- Beyond the off-label use of medicinal products
- Rights and access pathways to the medicinal product : an information journey

Building site of good practice

- New model of TNGB informed consent
- Updating of TNGB Information leaflet
- 5 new agreements between POs and TNGB
- Advisory board participation
- Setting R&I Agenda: Contribution to the new configuration of Telethon Foundation call on orphan neglected diseases
- Promoting early access: Open AIFA

Science WITH & FOR Society.

Horizon 2020

Responsible Research & Innovation

Choose together – ENGAGEMENT
Unlock the full potential – GENDER EQUALITY
Creative learning fresh ideas – SCIENCE EDUCATION
Share results to advance – FROM OPEN ACCESS TO OPEN SCIENCE
Do the right think and do it right – ETHICS AS A WAY OF ENSURING HIGH QUALITY RESULTS

Responsible Research & Innovation

Setting R&I agenda
Supervising and assessing R&I
Actively initiating and funding research
Shaping R&I process
Gathering data
Dissemination of R&I outcomes

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Thank you!

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