



E-RARE efforts towards patients' involvement in rare diseases research and funding activities

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Conflicts of Interest

I DECLARE NO CONFLICT OF INTEREST

Introduction

- About ERA-NET E-Rare
- Joint transnational calls
- **Partnerships with Patients' Organizations**
- RE(ACT) Congress
- European Medicines Agency workshop

What is an ERA-Net?

ERA-Net
=
European Research Area Network

- To accelerate the Cooperation and Coordination of Research Activities carried out at National/Regional level
- To contribute to the development of European Research Area by improving coherence and coordination across Europe of such research programmes
- To enable national systems to take on tasks collectively that they would not have been able to tackle independently
- **ERA-Net consortium is composed of funding bodies**

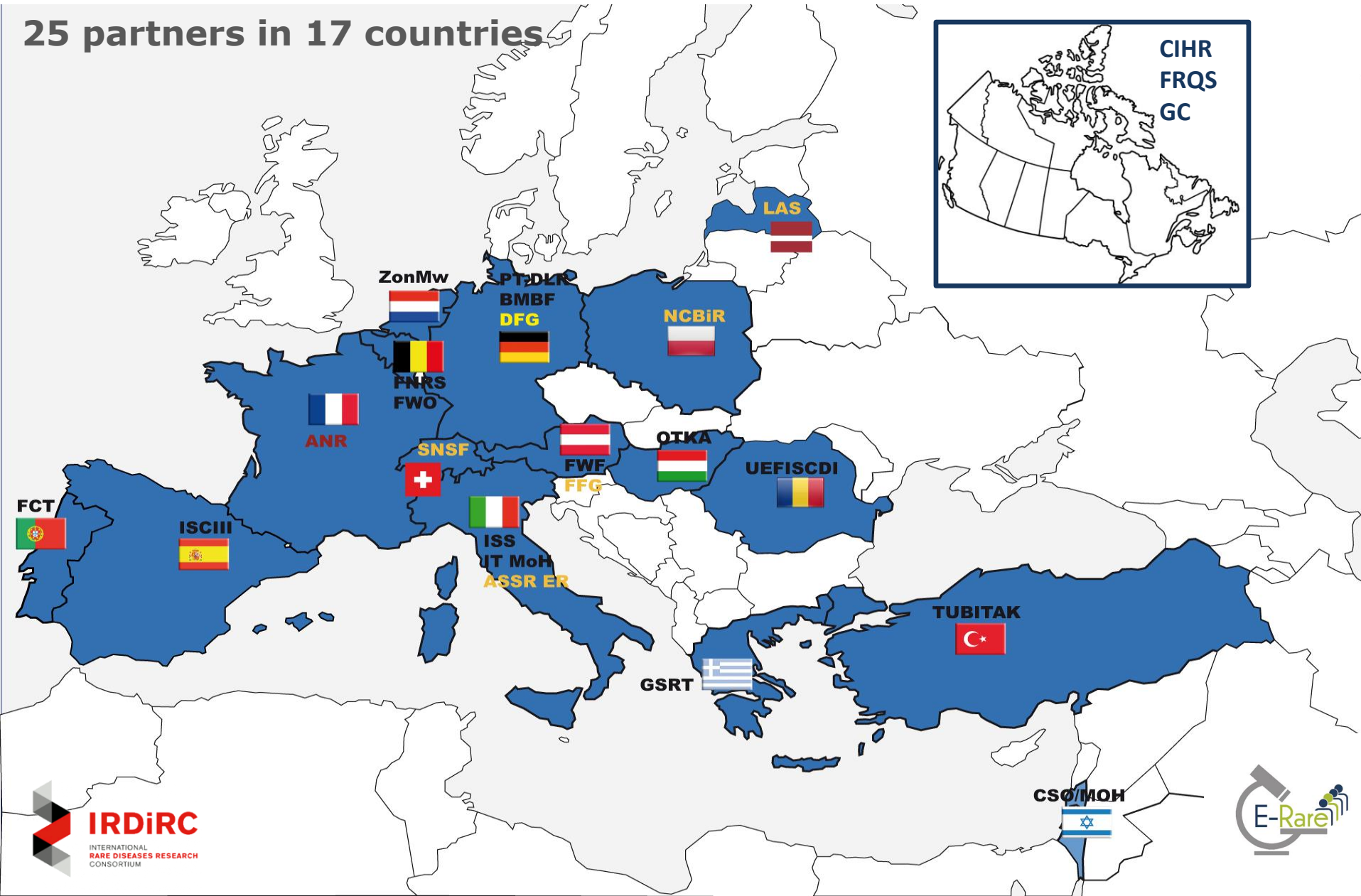
Major facts about E-Rare

- ERA-NET for research programmes on rare diseases
- Created in 2006 (E-Rare-1) renewed in 2010 (E-Rare-2) at present funded under Horizon 2020 ERA-Net Co-fund E-Rare-3 (2014 - 2019)
- 25 partners from 17 European, Associated and non-European countries (AT, BE, CA, CH, ES, FR, GR, HU, IT, IL, LV, NL, PO, PL, RO, TR)
- Objective: coordinate and promote **European and international efforts** for funding research on rare diseases

E-Rare-3: beyond Europe



25 partners in 17 countries



RD community in different countries

TWO MAJOR GROUPS:

- Well defined, strong community with academic and clinical coverage; supported at national level either through national plans or specific funding programmes; existence of spin offs or start ups and clinical trials funding available; access to tools like genetic identification, bioinformatics, omics, etc. (AT, BE, CA, DE, FR, ES, IT, IL, NL, CH)
- Fragmented; no specific approach to RD at national level or national plan in development; lack of national registries or longitudinal studies; lack or low access to specific tools (GR, HU, RO, LT, TR)

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This short analysis underlines the importance of E-Rare as a funding programme but also as an international initiative, gathering partners with different “background”.



E-Rare activities

Coordination & support activities

- Harmonization of national research programmes
- Development of strategic agenda
- Development of common funding mechanisms
- Monitoring and assessment of funded research projects
- Collaboration with relevant stakeholders
- Communication & dissemination

Joint Transnational Calls

- No cross-border funding
- Virtual common pot = each agency funds its own national research teams (except co-fund)
- Launched with regular periodicity (yearly)
- Topic to be defined: open vs focus

E-Rare Joint Transnational Calls

Joint Transnational Calls

- Preparation starts every year in May - June
- Any institution that has funding capacity can join
- New partners have the same rights as E-Rare partners regarding the whole joint call process: decision on the call topic, preparation of the documents, evaluation, funding decision
- Each institution establishes its own national rules that are communicated to applicants
- There is no obligation to participate in subsequent joint calls

E-Rare Joint Transnational Calls: SCOPE

Clinical studies

Natural history of diseases, Registries, Databases, Genotype/Phenotype Correlation studies, Diagnosis/Prognosis

Pre-clinical Therapeutic Research

Development of new therapeutic tools, Proof-of-concept, Innovative biotechnology research, Drug toxico/pharmacol.

Physiopathology


Gene identification, Epigenetics, Molecular mechanisms, New animal models, Omics, Biomarkers

Focused calls:

Young Investigator

Novel therapeutic approaches

E-Rare Joint Transnational Calls: FUNDING

																		
E-Rare-1	JTC 2007		X	X	X	X	X		X									
	JTC 2009		X	X	X	X	X	X	X	X	X	X						
E-Rare-2	JTC 2011	X/X	X	X	X	X	X		X	X	X							
	JTC 2012 (YI)	X	X	X	X		X	X	X	X		X	X					
	JTC 2013	X/X	X	X	X	X	X		X	X		X	X	X/X	X	X/X	X	
	JTC 2014	X	X	X	X	X	X	X	X	X		X	X	X/X	X	X	X	X
E-Rare-3	JTC 2015	X/X	X/X	X	X	X/X	X	X	X	X/X	X	X	X	X/X/X	X	X	X	X

- JTC 2007: 6 call partners from 6 countries → JTC 2015: 23 call partners from 17 countries
- 150 – 180 applications per call (2015 call → 230 applications)
- The global success rate of E-RARE proposals is 10% (PP vs FF) or 25% (FP vs FF)
- Average budget per call: 12 Mio€ Average budget per project: 700 K€
- Since January 2008 we implemented 7 joint calls:
78 Mio. € to support 449 research groups in 98 research consortia
- JTC 2015 with co-funding from European Commission of about 4.6 M€



E-Rare-3

Partnerships with Patients' Organizations



E-Rare-3 collaborations: Patients' Organizations

PATIENTS' ORGANIZATIONS INVOLVEMENT

- EURORDIS present in E-Rare Advisory Board, mutual exchange and support
- BUT
- Presence and involvement of patients (and society) in E-Rare activities judged not sufficient
 - Since 2013 Canadian Institutes of Health became E-Rare partner → shared their experience with Patients' Organizations
 - In 2014 Muscular Dystrophy Canada financed French research team (established collaboration with French National Research Agency)
 - Only 2 out of 25 funding agencies had experience in partnering with POs



E-Rare-3 collaborations: Patients' Organizations



PATIENTS' ORGANIZATIONS INVOLVEMENT

- Aims at development of a *funding model for collaboration with POs for E-Rare JTCs* building upon the Canadian experience/model to further expand E-Rare's patient engagement activities
- EURORDIS: key role in brokering conversation with patients' organizations and bringing their input
- Connecting patients & researchers
- In alignment with IRDiRC's policies & guidelines concerning participation by patients and/or their representatives in research



INVOLVEMENT OF PATIENTS' ORGANIZATIONS

YES BUT WHY FUNDING?

E-Rare-3 collaborations: Patients' Organizations

PATIENTS' ORGANIZATIONS INVOLVEMENT

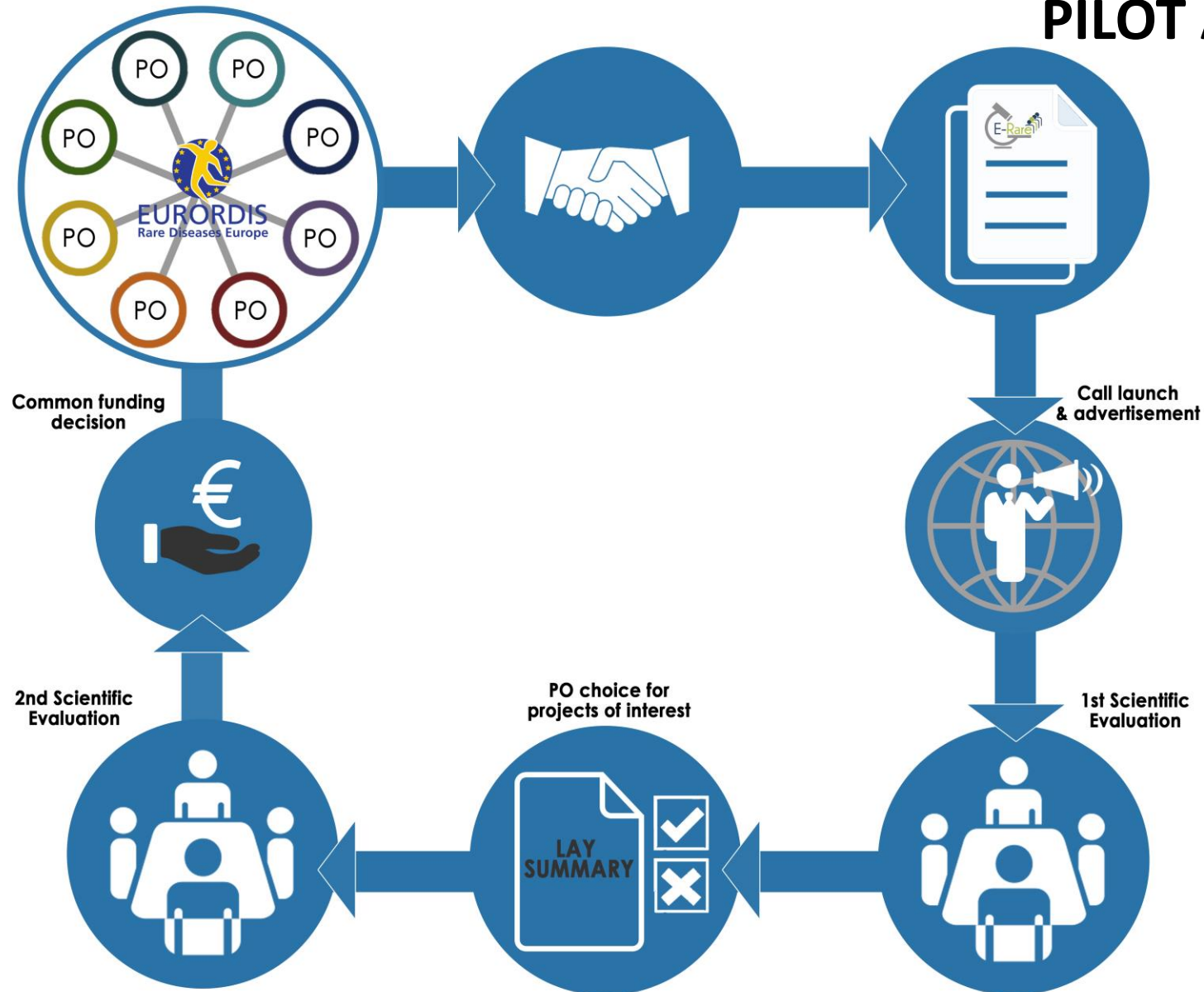


- EURORDIS: launched a survey in 2014 identified 60 POs interested in participating in the activities of E-Rare (44 had funding capabilities)
- Funding of research is what we know and what we do the best → good starting point
- Need for change in the perception of the place of patients in the activities of public institutions and researchers → must be done progressively
- It is a learning process for both funders and patients' organizations → needs time



E-Rare-3 collaborations: Patients' Organizations

PILOT ACTION



E-Rare-3 collaborations: RE(ACT) Congress

BLACKSWAN
FOUNDATION



**RE(ACT) CONGRESS 9 – 12 MARCH 2016,
CROWN PLAZA FIRA, BARCELONA**



- 500 participants expected
 - 6 scientific sessions
 - Special session dedicated to Patients & Research
 - Workshop with EMA
 - Preceded by E-Rare Monitoring meeting & strategic works
- CIBERER annual congress



www.react-congress.org



E-Rare-3 collaborations: EMA

EUROPEAN MEDICINES AGENCY



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

- Assistance for rare diseases researchers
- Promotion of scientific advice for orphan medicines
- Communication & learning (dedicated pages on E-Rare website)



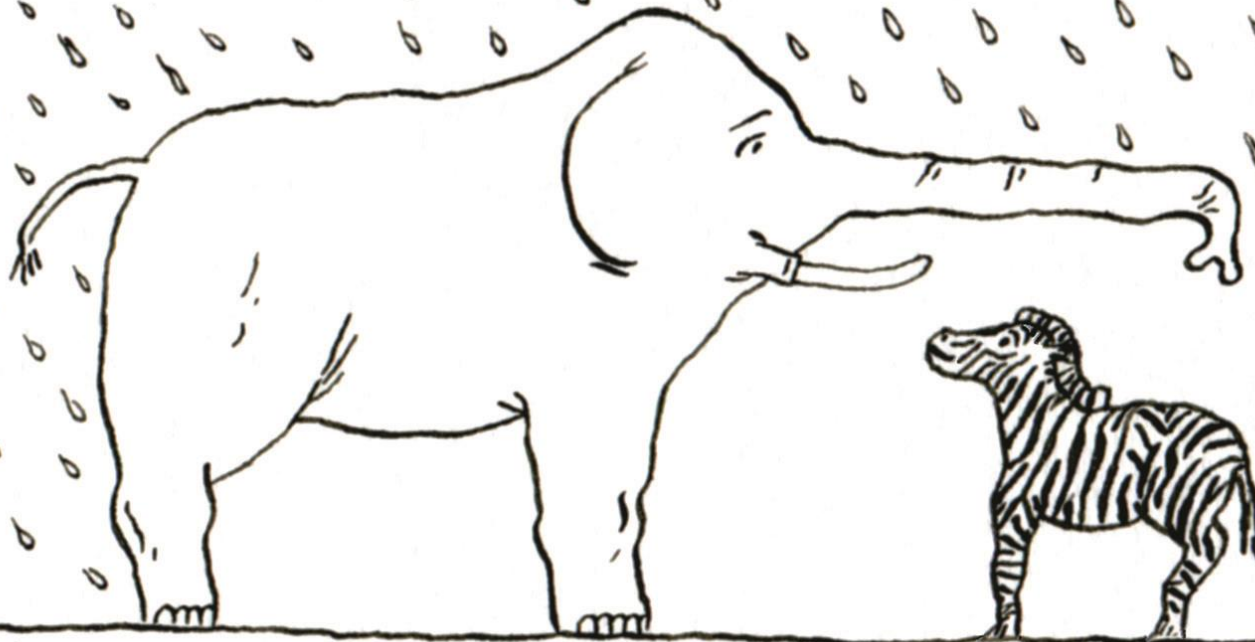
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

WORKSHOP on “Interactions of EMA and RD researchers on prelicencing activities”, 9 of March 2016, RE(ACT) Congress

- Prelicencing activities of EMA
- Presentation of EMA structures (COMP, SAWP, PDCO, CAT)
- Orphan designation and incentives for researchers
- Protocol assistance – how does it work?
- Lessons learnt: Horizon 2020 success stories on obtaining OD
- Face-to-face meetings with EMA officers - Session dedicated to researchers that already prepared a draft submission package for OD or protocol assistance



Thank you



Have you done something rare today?

www.e-rare.eu

