



Hinc itur  
ad astra



# EQUITY AND INCLUSIVITY IN EUROPEAN REFERENCE NETWORKS AND EUROPEAN JOINT PROGRAMME ON RARE DISEASES

**BIRUTĖ TUMIENĖ**

VILNIUS UNIVERSITY, FACULTY OF MEDICINE,

VUH SANTAROS KLINIKOS, COORDINATING CENTER FOR RARE DISEASES,

ERN BOARD OF MEMBER STATES, LT REPRESENTATIVE, CHAIR OF WG FOR ERN INTEGRATION INTO NATIONAL SYSTEMS

ORPHANET LITHUANIA NATIONAL COORDINATOR

EUROPEAN JOINT PROGRAM ON RARE DISEASES, PILLAR 3 CO-LEADER

IRDIRC DIAGNOSTIC SCIENTIFIC COMMITTEE VICE-CHAIR



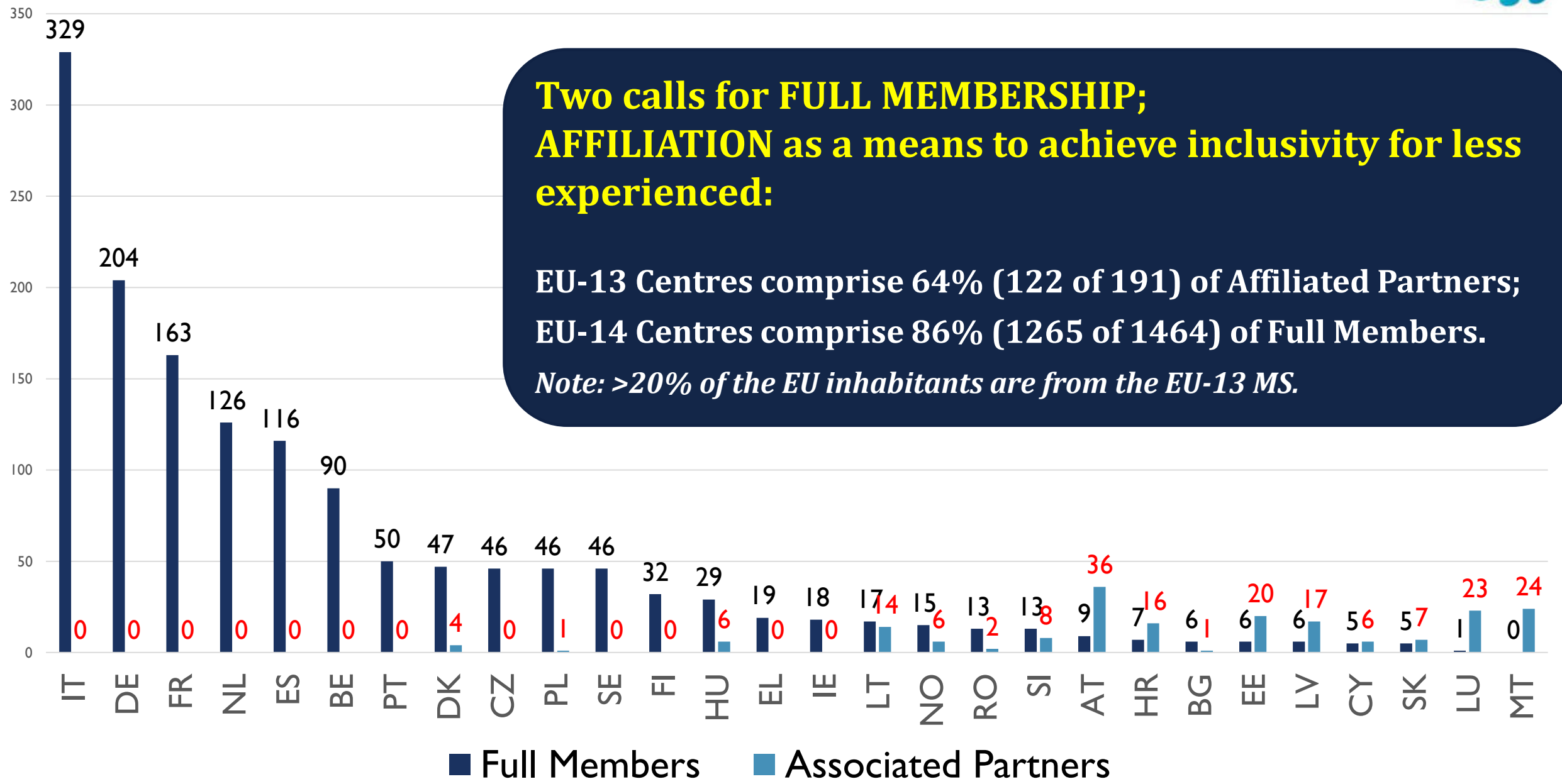
# Integration of ERNs: European and national



*“What is the use of enormous amounts of expertise if they remain confined to the individual centres participating in the network? These centres must be able to reach all patients **in their territories – and in other Member States**, if no national reference centre has been established – in order to really make a difference to the care of these patients. Thus, ERNs are effective only in so far as they are inclusive, proactively reaching out to the populations they serve.”*

– Expert Panel on Effective Ways of Investing in Health (EXPH): Opinion on Application of the ERN model in European cross-border healthcare cooperation outside the rare diseases area, 2018.

# GEOGRAPHICAL INCLUSIVITY of ERNs: All ERN Centres (> 1600)



# GEOGRAPHICAL INCLUSIVITY of ERNs: Full Members/ million of inhabitants



**LT and SI:**  
**Success story? Regional leadership?**

## Hospitals with the largest number of ERN Full Members (> 10 Full Members)

NL: Erasmus MC: University Medical Center Rotterdam – 24;  
 FI: Helsinki University Hospital – 21;  
 IT: AO di Padova – 21;  
 DK: Rigshospitalet - 20;  
 IT: IRCCS Ospedale Pediatrico Bambino Gesù, Roma – 20;  
 BE: UZ Gent – 20;  
 SE: Karolinska University Hospital – 20;  
 BE: UZ Leuven – 19;  
 ES: Hospital de Sant Joan de Déu – 19;  
 DK: Aarhus University Hospital – 18;  
 ES: Hospital Universitari Vall d' Hebron – 18;  
 NL: Radboud University Medical Center Nijmegen – 18;  
 NL: University Medical Center Utrecht – 18;  
 IT: Fondazione Policlinico Universitario A. Gemelli – Roma – 16;  
 BE: Cliniques universitaires Saint-Luc – 15;  
**CZ: University Hospital Motol – 15;**  
 DE: Charité Universitätsmedizin Berlin – 15;  
 FR: APHP, Hôpital Necker Enfants Malades – 15;  
 IT: Fondazione IRCCS CA'Granda Ospedale Maggiore Policlinico – 15;

NL: Academic Medical Centre of Amsterdam – 15;  
 NL: University Medical Centre Groningen – 15;  
 NL: Leiden University Medical Center – 14;  
 SE: Sahlgrenska University Hospital – 14;  
 BE: Antwerp University Hospital – 13;  
 DE: Universitätsklinikum Hamburg-Eppendorf – 13;  
 NO: Oslo University Hospital - 13;  
**LT: Vilnius University Hospital Santaros Klinikos - 12;**  
 DE: Universitätsklinikum Freiburg – 11;  
 FI: Hospital District of Helsinki and Uusimaa – 11;  
 IT: AOU Senese – 11;  
 IT: IRCCS Istituto Giannina Gaslini, Genova – 11;  
**SI: University Medical Center Ljubljana - 11;**  
 ES: Hospital Clinic de Barcelona – 10;  
 ES: Hospital Universitario La Paz – 10;  
 IT: AOU Federico II di Napoli – 10;  
 IT: AOU Meyer di Firenze – 10;  
 PT: Centro Hospitalar Universitario do Porto – 10.

## Hospitals with the largest number of ERN Full Members and Affiliated Partners (> 10 FM and AP)\*

LU: Centre Hospitalier du Luxembourg – 24 (1);  
**MT: Mater Dei Hospital – 24 (0);**  
 DK: Rigshospitalet - 22 (20);  
**EE: Tartu University Hospital - 21 (5);**  
 DK: Aarhus University Hospital – 20 (18);  
**SI: University Medical Center Ljubljana - 19 (11);**  
**LT: Vilnius University Hospital Santaros Klinikos - 18 (12);**

**HU: Semmelweis University - 16 (10);**  
 NO: Oslo University Hospital - 16 (13);  
 AT: Medical University of Vienna/ Vienna General Hospital - 14 (2);  
**HR: University Hospital Center Zagreb - 14 (6);**  
**LV: Children`s Clinical University Hospital, Riga - 14 (5);**  
**LT: Hospital of Lithuanian University of Health Sciences Kauno Klinikos - 13 (5);**  
 AT: Medical University Innsbruck – 12 (2).

# GEOGRAPHICAL INCLUSIVITY of ERNs: All ERN Centres/ million of inhabitants



**We still have some "whitish" spots on the EU map**

# DIRECTIVE 2011/24/EU on the application of patients' rights in cross-border healthcare

## *Article 12*

### *European reference networks*

1. ...The networks shall be based on **voluntary** participation by its members, which shall participate and contribute to the networks' activities **in accordance with the legislation of the Member State** where the members are established and shall at all times be open to new healthcare providers which might wish to join them, provided that such healthcare providers fulfil all the required conditions and criteria referred to in paragraph 4.

6. Measures adopted pursuant to this Article **shall not harmonise any laws or regulations** of the Member States and shall **fully respect the responsibilities of the Member States** for the organisation and delivery of health services and medical care.

# ERN Board of Member States: **MANDATES**

## COMMISSION IMPLEMENTING DECISION 2014/287/EU

### **Article 6: Board of Member States**

Member States are invited to set up a Board of Member States which shall decide whether or not to approve the proposals for Networks, their membership and the termination of a Network.

## COMMISSION IMPLEMENTING DECISION (EU) 2019/1269

(2) To increase the efficiency of the European Reference Networks, the Board of Member States should become the forum for exchanging information and expertise in order to steer the development of the ERNs, provide guidance to the Networks and to the Member States and advise the Commission on matters related to the establishment of the Networks.





# European Joint Programme on Rare Diseases in numbers

35

## participating countries

- 26 EU Member States (AT, BE, BG, CZ, DK, EE, FI, FR, DE, EL, HU, HR, IE, IT, NL, LV, LT, LU, MT, PL, PT, RO, ES, SE, SK, SI)
- 7 associated (AM, GE, IL, NO, RS, CH, TK)
- UK & CA

**Widening countries:**  
**16/35, 46%**

**101 M€**  
**Budget**

Union contribution: 55 M€  
(70% reimbursement rate)



87

## beneficiaries

- 9 hospitals
- 12 research institutes
- 31 research funding bodies/ministries
- 24 universities/hospital universities
- 5 EU infrastructures
- 5 charities/foundations
- EURORDIS

**+ 50 linked third parties**  
**+100% associated networks**



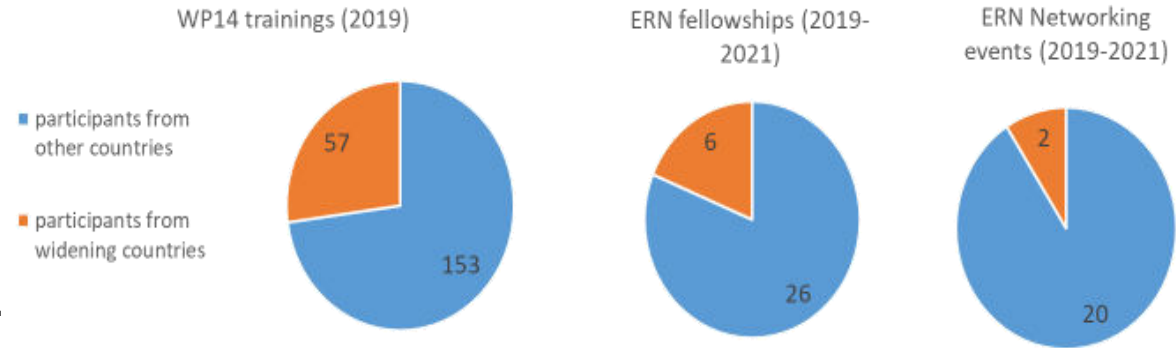


# EJPRD: measures to increase participation of widening countries

## Empowerment and capacity building:

- **Rotation of courses:** **22%** (8 out of 36) of all Pillar 3 courses have been or will be provided in widening countries.
- **Fellowships** to participants coming from widening countries.
- **Adaptation of trainings** to the local needs of participants.

*E.g., participants from widening countries comprised from **12 to 50%** of all course participants in WP14 trainings in 2019, **20%** of ERN research mobility fellowships, **10%** of beneficiaries in ERN Research training workshops.*



## Rare disease research funding:

- **Networking scheme** (“COST-like” activities):

*In the first five rounds, funded applicants from widening countries comprised **21%** (31 of 151) of applicants.*

- **Joint transnational calls (JTCs), WIDENING principles:**

(i) consortia may involve **up to 8 partners** instead of max. 6, if beneficiaries from the Widening countries or Early Stage Investigators are included;

(ii) beneficiaries from widening countries may join the consortia **in the second stage** of applications.



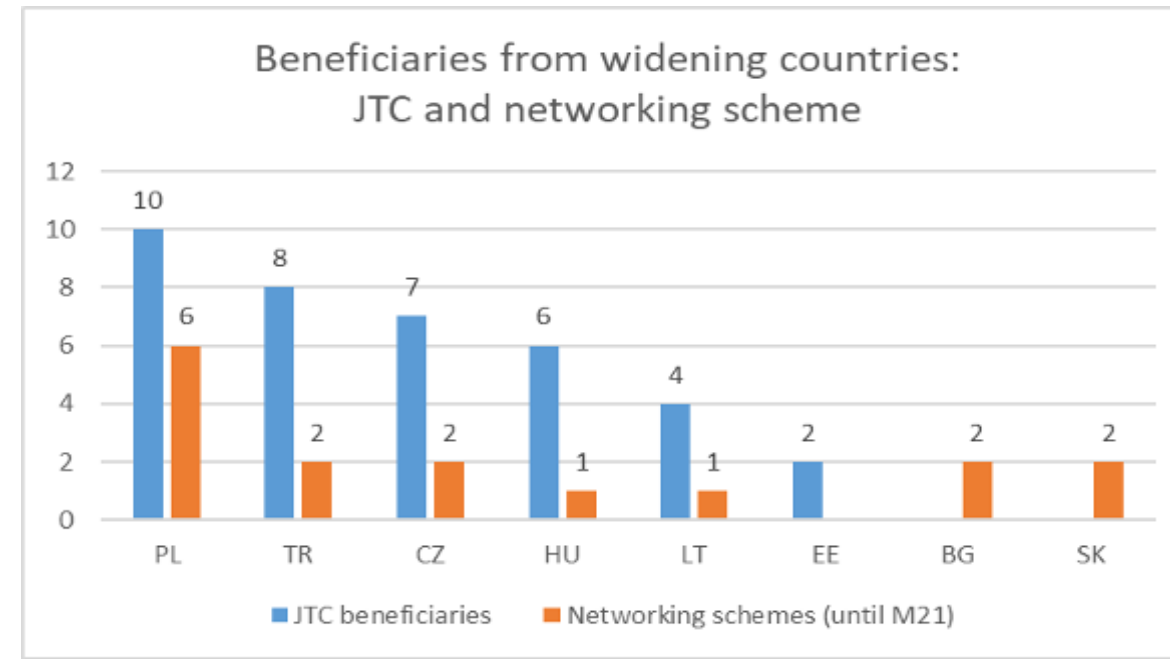
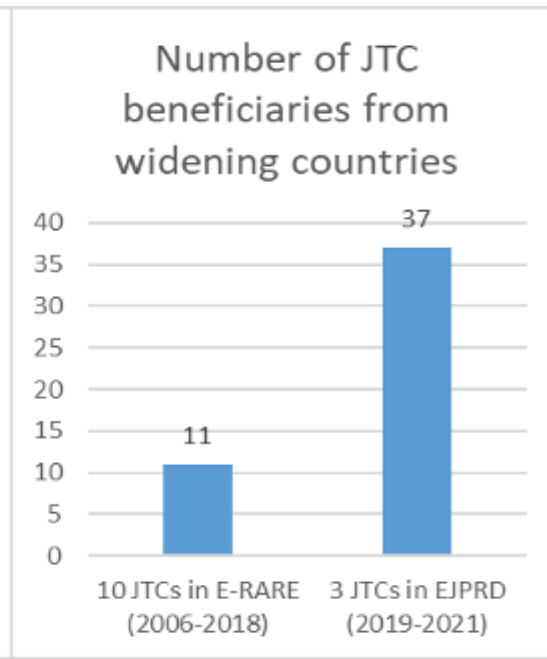
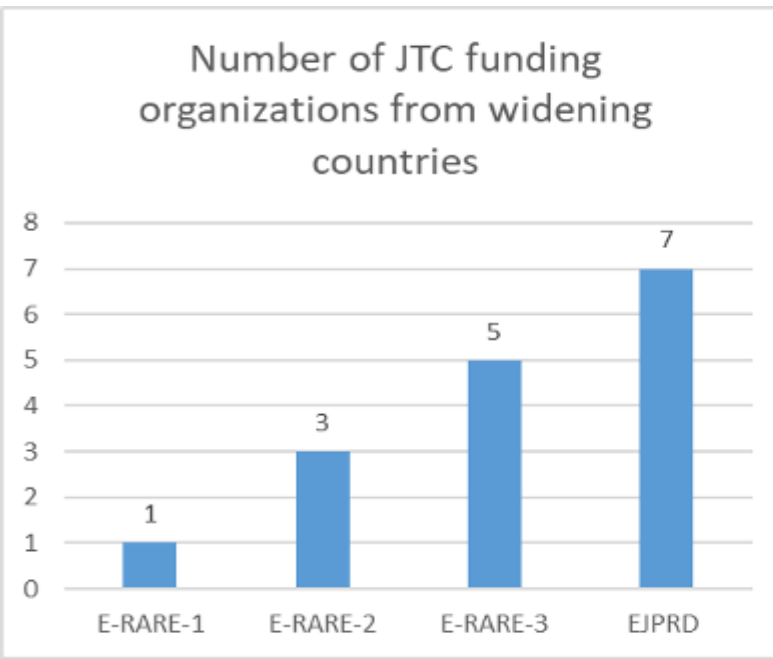
# EJPRD: measures to increase participation of widening countries

## Widening principles were applied in E-RARE since 2015 and in EJPRD.

- Number of JTC **funding organizations** from widening countries increased *from 1 in E-RARE-1 to 7 in EJPRD*.
- **Beneficiaries** from widening countries comprised *from 7 to 12% in EJPRD JTCs 2019 to 2021*.

*On average, 47% of EJPRD JTC consortia involved at least one partner from widening countries.*

- Overall, beneficiaries from widening countries received **4,1% to 8,7%** of funding in JTC2019 and JTC2020.
- The average financial contributions per beneficiary were **30% to 41%** lower for beneficiaries from widening countries.
- However, widening principles may result in a win-win situation for all consortium participants: the average sum of the total requested funding was **8% to 18% higher** in projects that involve participants from widening countries.





European  
Reference  
Networks

Working for patients with  
rare, low-prevalence and  
complex diseases

**Share. Care. Cure.**



**European and global** collaboration  
in rare diseases

*Thanks for your attention*