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ERNS: THE CHALLENGE OF EQUAL CARE FOR ALL

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European
Reference
Networks

24 networks of excellence in RD

➤ Kick-off: 2017-03-09, Vilnius

24 networks, ~1 000 000 patients/year

- Triangle of highly-specialized healthcare, research and education
- **The largest EU platform for virtual and physical cross-border healthcare, clinical and translational research in rare and complex diseases**

- **Economies of speed, scale and scope for multiple tasks**

develop and implement RD clinical guidelines, collect cohorts and data of rare patients, create a curriculum for RD education, perform an ultra-rare disease clinical trial, make RD monitoring for policy decisions, etc.

- **Multidisciplinarity, accessibility, quality assurance, TACKLING INEQUITIES**

“POST CODE LOTTERY” IN RARE CANCERS: GEOGRAFIC INEQUALITIES

G. Gatta, A. Trama, R. Capocaccia, RARECARENet Working Group (2019). Epidemiology of rare cancers and inequalities in oncologic outcomes:

5-year survival, 41 rare cancers:

- IT, DE, BE: **55%;**
- BG, LT, SK: **40%.**

Laura Botta, Gemma Gatta, Annalisa Trama, ... RARECAREnet working group (2020). Incidence and survival of rare cancers in the US and Europe:

5-year survival, 196 rare cancers:

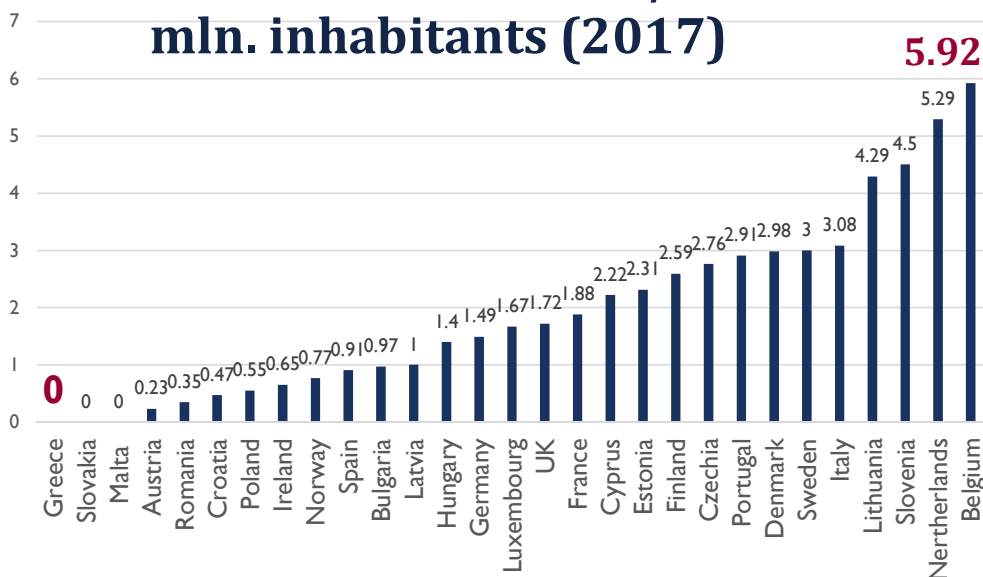
- US: **54%;**
- Europe: **48%.**

Fewer diagnoses – worse outcomes.

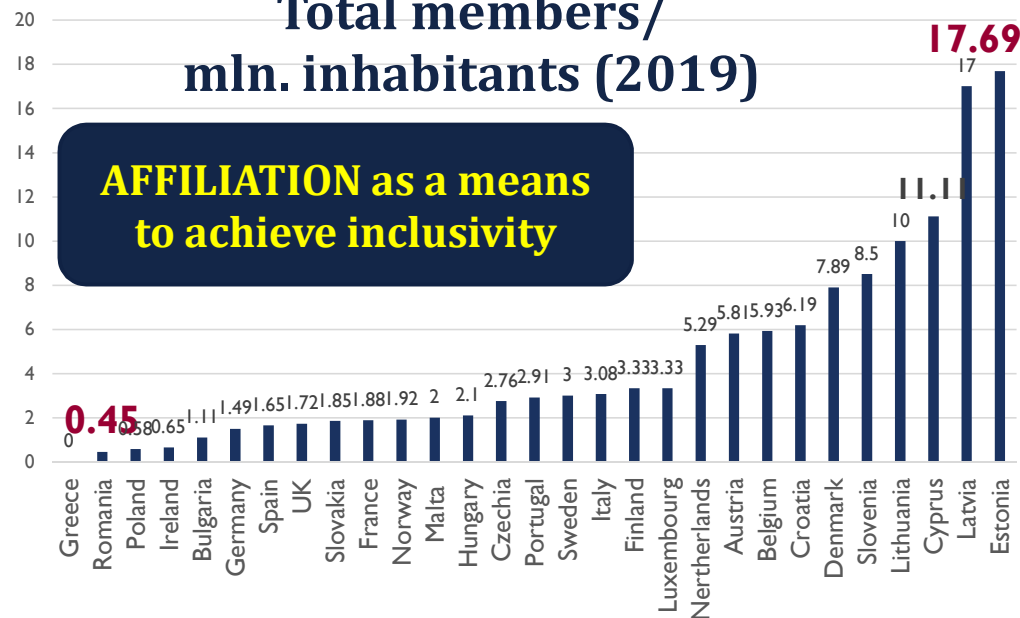


INCLUSIVITY of ERNs: geographical

ERN Full members/ mln. inhabitants (2017)



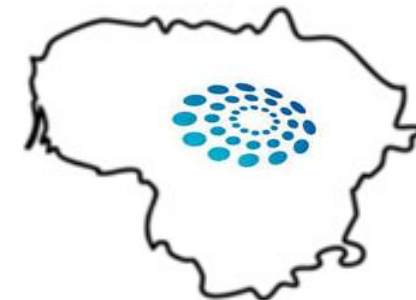
Total members/ mln. inhabitants (2019)



**AFFILIATION as a means
to achieve inclusivity**

- **ERN Full Members: rigorous quality control and full responsibilities;**
 - **ERN Affiliated Partners and Coordinating Hubs: national designation according to the needs of MS.**
 - ❑ EU-13 countries comprise **20%** of the EU inhabitants;
 - ❑ 2016 Call for ERN FM: number of ERN FM in EU-13 MS - **11.3%**;
 - ❑ 2019 Call for ERN AP: number of ERN FM + ERN AP in EU-13 MS - **20%**.
- Unfortunately and despite of that, some inequities remain among MS and **inside MS** (regionally).

LT: PARTICIPATION IN ERNs (2017)



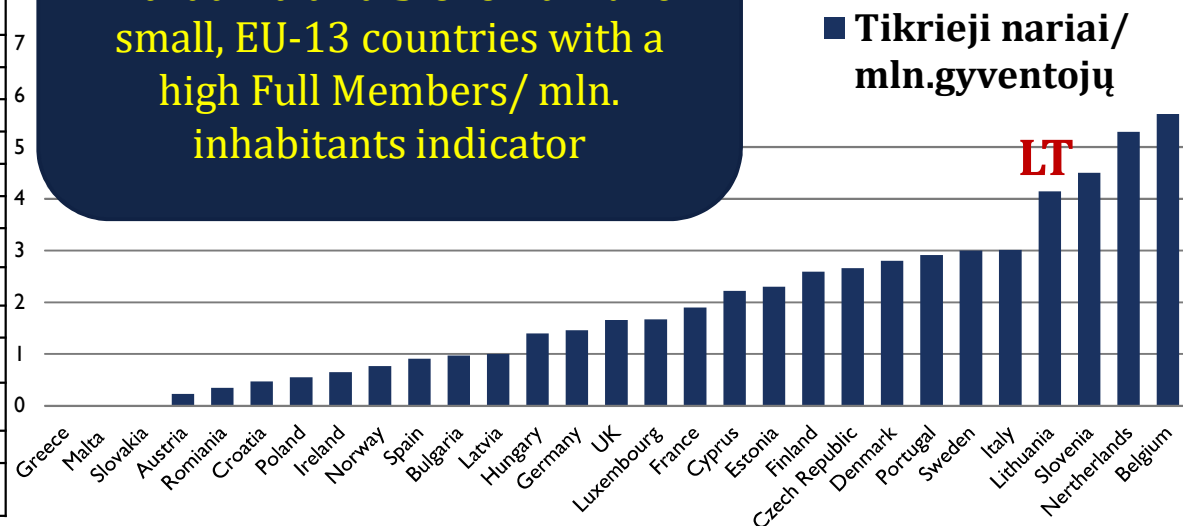
12 ERN Full Members

16 ERN Afiliated partners

5 applications for Full Members

University Hospital Leuven	19	BE
AO di Padova	18	IT
Karolinska University Hospital	18	SE
Erasmus MC: University Medical Center Rotterdam	18	NL
Assistance Publique-Hôpitaux de Paris, Hôpital Necker-Enfants Malades	15	FR
Pediatric hospital Bambino Gesù, Rome	15	IT
Radboud University Medical Center Nijmegen	14	NL
Great Ormond Street Hospital for Children NHS Foundation Trust	13	UK
University Hospital Ghent	12	BE
Motol University Hospital	12	CZ
Academic Medical Center Amsterdam	12	NL
University Medical Center Utrecht	12	NL
Charité Universitätsmedizin Berlin	11	DE
Universitätsklinikum Freiburg	10	DE
Centro Hospitalar e Universitário de Coimbra, EPE	10	PT
Hospital Universitari Vall d'Hebron	10	ES
University Medical Center Groningen	10	NL
University Hospitals Saint-Luc	9	BE
Copenhagen University Hospital Rigshospitalet	9	DK
Hospices Civils de Lyon	9	FR
AOU Siena	9	IT
Central Manchester University Hospitals NHS Foundation Trust	9	UK
Assistance Publique-Hôpitaux de Paris, Hôpital Bicêtre	8	FR
Klinikum der Universität München	8	DE
Foundation IRCCS CA'Granda Ospedale Maggiore polyclinic - Milan	8	IT
Vilnius University Hospital Santaros Klinikos	8	LT
Leiden University Medical Center	8	NL
Birmingham Children's Hospital NHS Foundation Trust	8	UK

Success story?
Lithuania and Slovenia – two small, EU-13 countries with a high Full Members/ mln. inhabitants indicator



CENTRALIZATION



Competition and ambitions

- **Quality assurance**
- **Cost-efficiency and effectiveness**
- **Better clinical outcomes**

R. Kalaiselvan et al. (2018). Impact of centralization of services on outcomes in a rare tumour: Retroperitoneal sarcomas.

J.-Y. Blay et al. (2017) Improved survival using specialized multidisciplinary board in sarcoma patients.

J.-Y. Blay (2018) Management of sarcoma patients: centralization in reference centers to fragmentation of systemic treatment.

E. Styring et al. (2012) Simple Guidelines for Efficient Referral of Soft-Tissue Sarcomas.

C. Collignon (2020) et al. Soft tissue sarcoma in children, adolescents and young adults: Outcomes according to compliance with international initial care Guidelines.

J.-Y. Blay et al. Surgery in reference centers improves survival of sarcoma patients: a nationwide study.

O. Derbel et al. (2017) Survival impact of centralization and clinical guidelines for soft tissue sarcoma.

L. Perrier et al. The cost-saving effect of centralized histological reviews with soft tissue and visceral sarcomas, GIST, and desmoid tumors: The experiences of the pathologists of the French Sarcoma Group.

Etc.....

S. Stordeur et al. (2016) Reference centres for adults with rare and complex cancers – Policy recommendations to improve the organisation of care in Belgium.

RARE DISEASE CARE PATHWAYS

HUGE DIVERSITY OF RD PATHWAYS

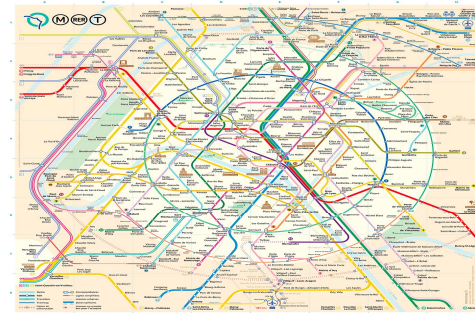
Constituents of any given RD pathway depend on:

- Disease
- Patient
- National care systems

Diagnostics	Patient empowerment I, II level
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Diagnostics	Complex	Patient empowerment
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ORIGINAL ARTICLE



Navigation of patients, data and funds:

legal, organizational, informational measures.

Optimal balance between:

- (a) **highly-specialized, expensive** services and
- (b) those, that may be provided at a **less-specialized** healthcare level, **local**.



Rare disease care pathways in the EU: from odysseys and labyrinths towards highways

Rirute Tumiene^{1,2}  Holm Graessner^{3,4,5}

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

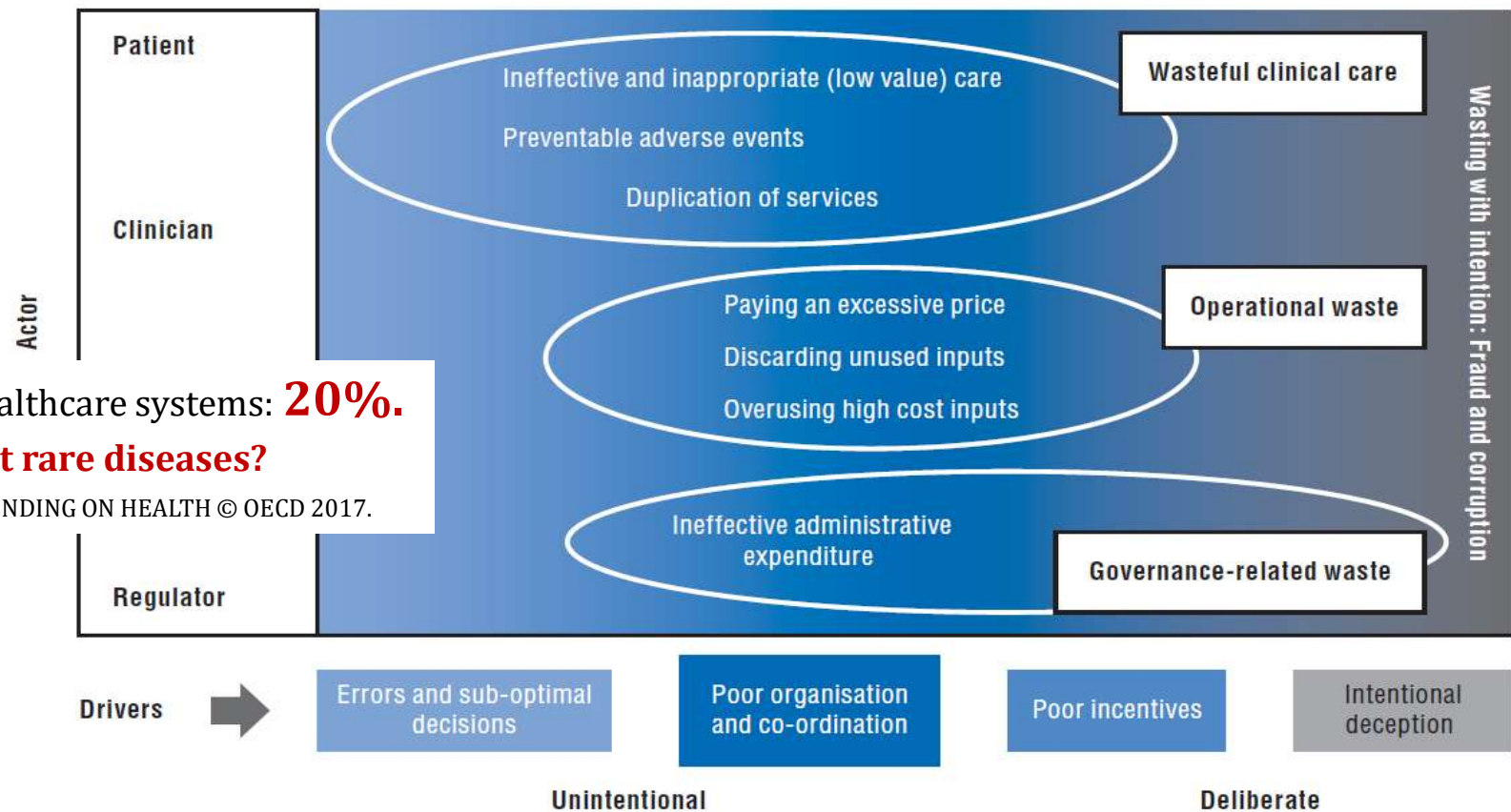
Member States are invited to set up a Board of Member States which shall decide whether or not to approve the proposals for Networks, their membership and the termination of a Network.

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.

EXTENSIVE WASTEFUL SPENDING IN RD

Figure 1.1. Three categories of waste mapped to actors involved and drivers



Average wasting in healthcare systems: **20%.**

What about rare diseases?

From: TACKLING WASTEFUL SPENDING ON HEALTH © OECD 2017.

ERN sustainability
ERN abilities
to reveal the full potential



Our diversity
geographic, economic, health systems
Autonomy, subsidiarity

Our decisions, based on **common European values**:

- **Solidarity,**
- **Respect for human dignity and human rights,**
- **Equality,**
- **The rule of law.**

Thanks for your attention

EU Healthcare Expenditure Relative To Population Size

