



**E-RARE &
THE EUROPEAN JOINT PROGRAMME
ON RARE DISEASES**

**RE(ACT) 2018 Congress
7-10 of March 2018
Bologna, ITALY**

**E-RARE –
THE ERA-NET FOR RESEARCH PROGRAMMES
ON RARE DISEASES**

MAJOR FACTS ABOUT E-RARE

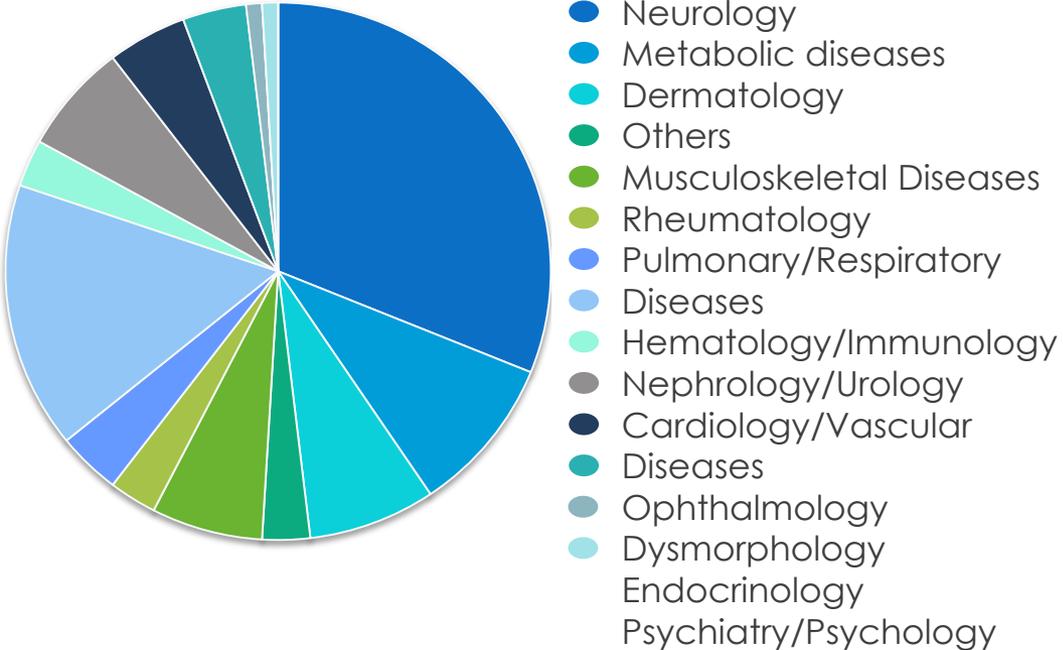
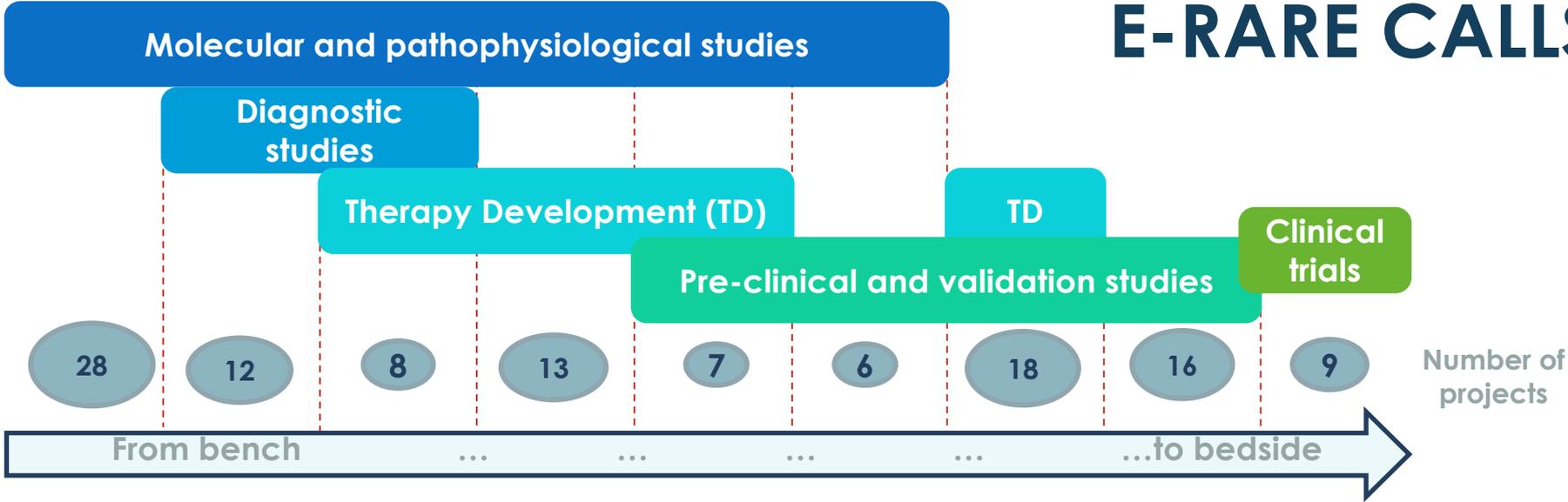
- ERA-NET for research programs on rare diseases
- Created in 2006 (E-Rare-1) renewed in 2010 (E-Rare-2) at present funded under Horizon 2020 ERA-Net Co-fund
- E-Rare-3 (2014 - 2019)



- 28 partners from 20 European, Associated and non-European countries (AT, BE, CA, CZ, CH, ES, FR, GR, HU, IT, IL, JP, LT, LV, NL, PO, PL, RO, TR)
- **Objective: coordinate and promote European and international efforts for funding research on rare diseases**
- E-Rare is mentioned in more than half of National Plans for Rare Diseases



E-RARE CALLS



9 Joint Transnational Calls:

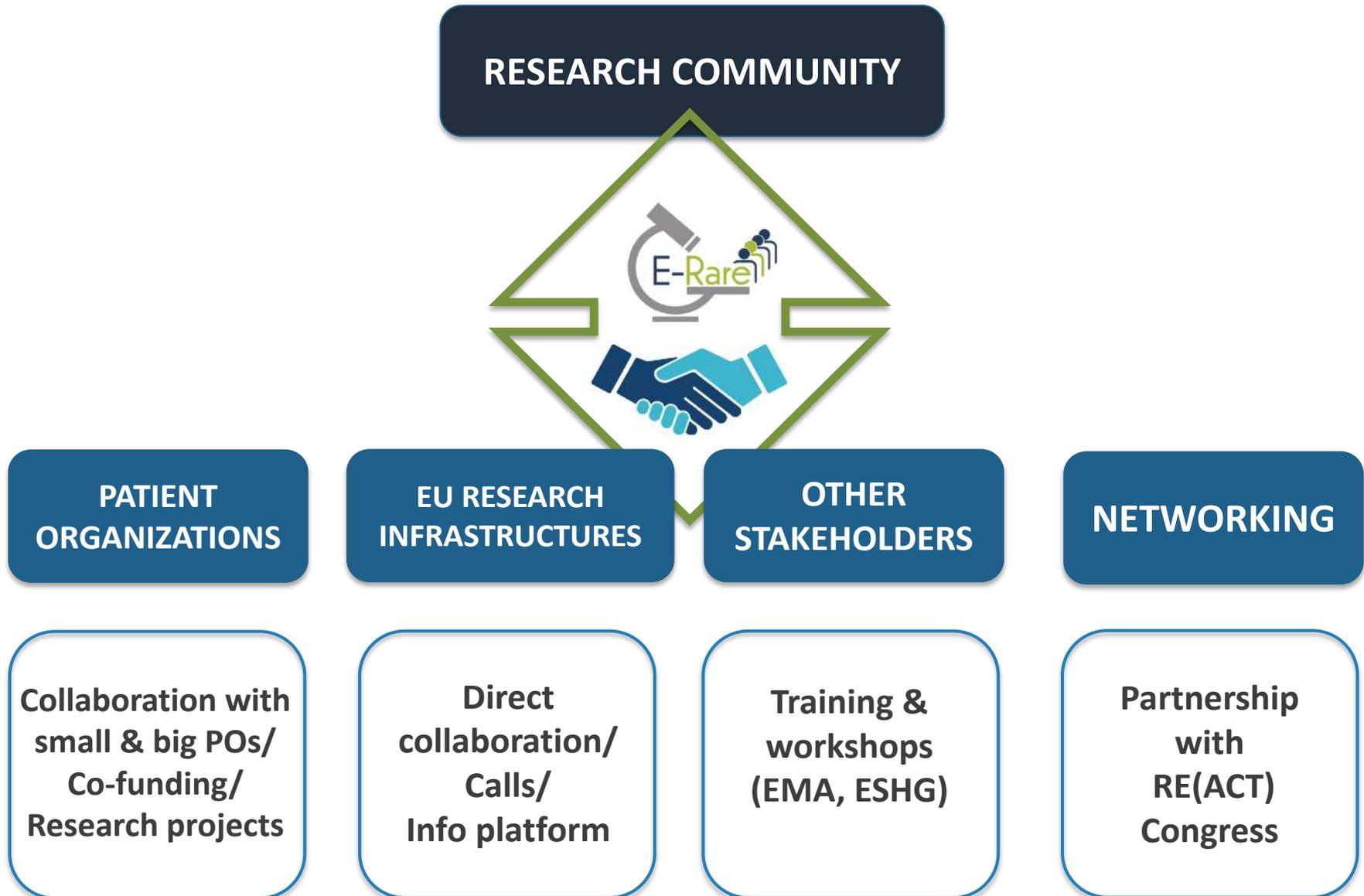
117 funded projects

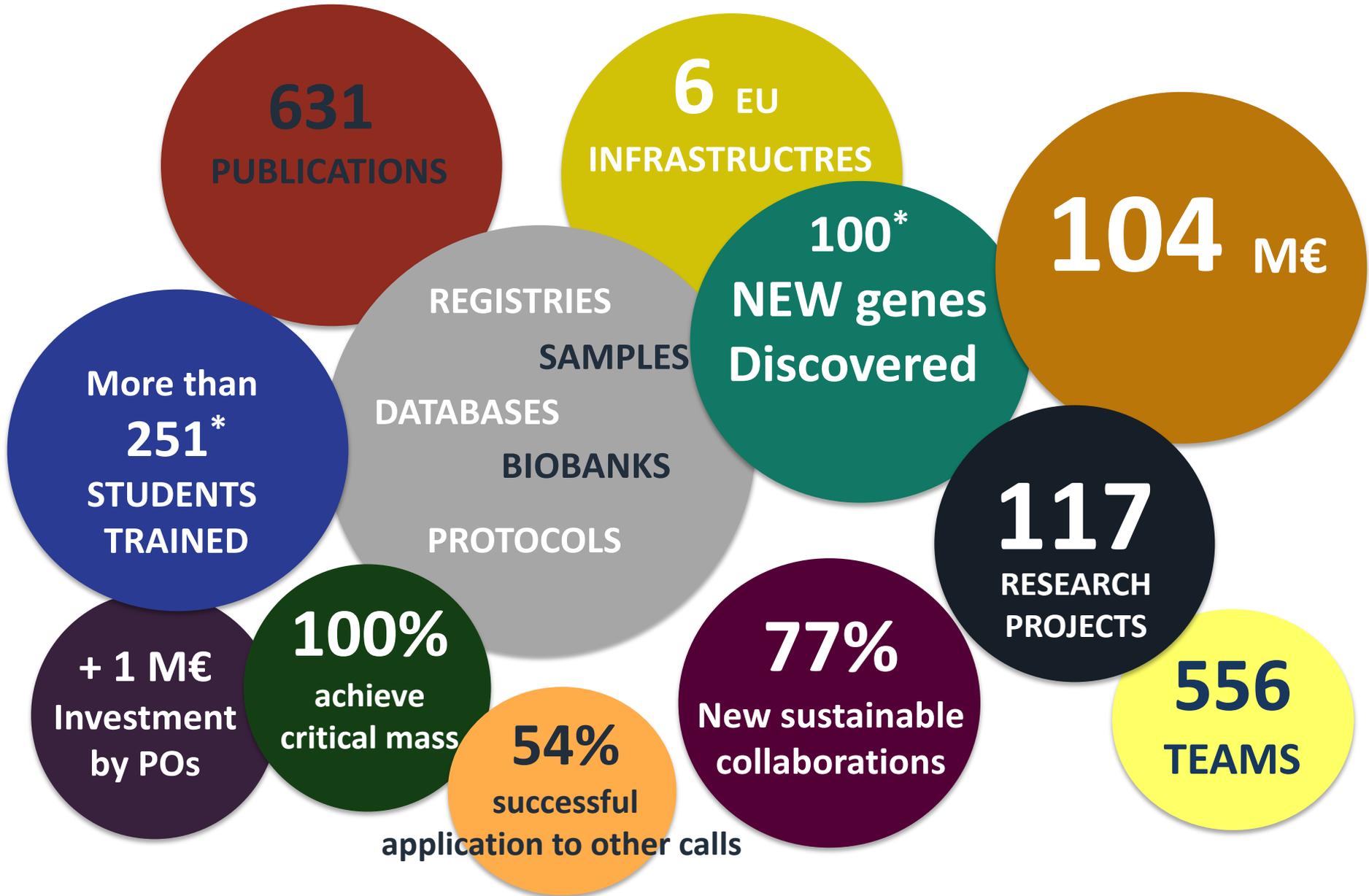
104 M€ invested

Including **556** research partners funded

Medical domains represented in the funded projects

E-Rare facilitates & connects

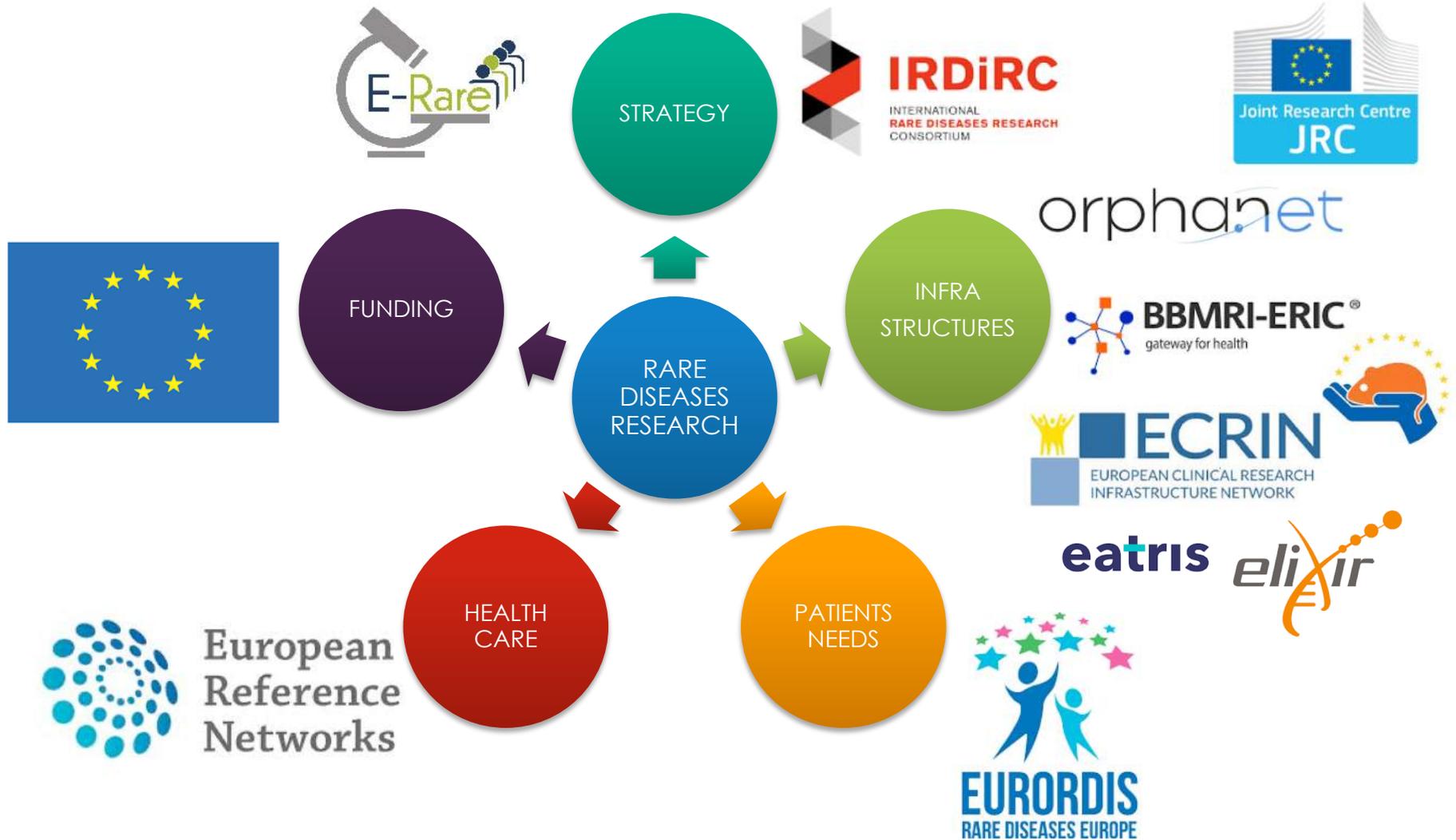




** In 48 finalized projects*

**EJP RD –
THE EUROPEAN JOINT PROGRAMME
ON RARE DISEASES**

RARE DISEASES LANDSCAPE IN EUROPE



EUROPEAN JOINT PROGRAMME ON RARE DISEASES

Background of preparatory work

- **March 2016** - First discussions started at E-Rare strategic workshop in Barcelona
- **August 2016** - The Commission sends official invitation to MS to nominate experts to the EJP RD working groups
- **October 2016** - First meeting of the MS EJP RD expert group
- **Dec 2016 – Feb 2017** - Preparation of the 1st Concept Draft of the EJP RD
- **April 2017** - 2nd meeting of the MS EJP RD experts group
- **Apr – June 2017** – nomination of new experts to future WGs
- **June 2017** – kick off meeting of the extended WGs of the EJP RD
- **June – Oct 2017** – preparation of the 2nd Concept Draft of the EJP RD → more than 70 experts involved + 24 ERN coordinators representing EU, associated and non-EU countries
- **12 Oct 2017** – 2nd EJP RD Concept Draft sent to the EC and WGs + ERN Coos
- **17 Oct 2017** – last meeting of the MS
- **End Oct 2017** – official publication of the WP 2018 – 2020 → kick off of the writing phase
- **Oct – Nov 2017** – call for Letters of Interest
- **11 Dec 2017** - Kick off of preparatory phase of EJP RD
- **31 Dec 2017** – final identification of contributors
- **30 of March 2018** – submission of the project



EUROPEAN JOINT PROGRAMME ON RARE DISEASES

OBJECTIVES

- **Main objective:** Create a research and innovation pipeline "from bench to bedside" ensuring rapid translation of research results into clinical applications and uptake in healthcare for the benefit of patients
 - **Specific objective:** improve integration, efficacy, production and social impact of research on rare diseases through the development, demonstration and promotion of sharing of research and clinical data, materials, processes, knowledge and know-how, and an efficient model of financial support for research on rare diseases
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- **Union contribution:** 55 M€
 - **Total budget (min):** 78,5 M€ (→ expected > 110 M€)
 - **Foreseen number of partners:** 70 – 100
 - **Foreseen number of participating countries:** 29 including 23 EU MS, 4 associated (CH, IL, NO, TK) and third countries (AU, CA, JP, USA)
 - **Timeline:** Jan 2019 – Dec 2024

EJP RD STRUCTURE

COORDINATION
& TRANSVERSAL ACTIVITIES

INTEGRATIVE RESEARCH STRATEGY

SUSTAINABILITY

ETHICAL & REGULATORY

COMMUNICATION

1

FUNDING

2

COORDINATED
ACCESS TO
DATA &
SERVICES

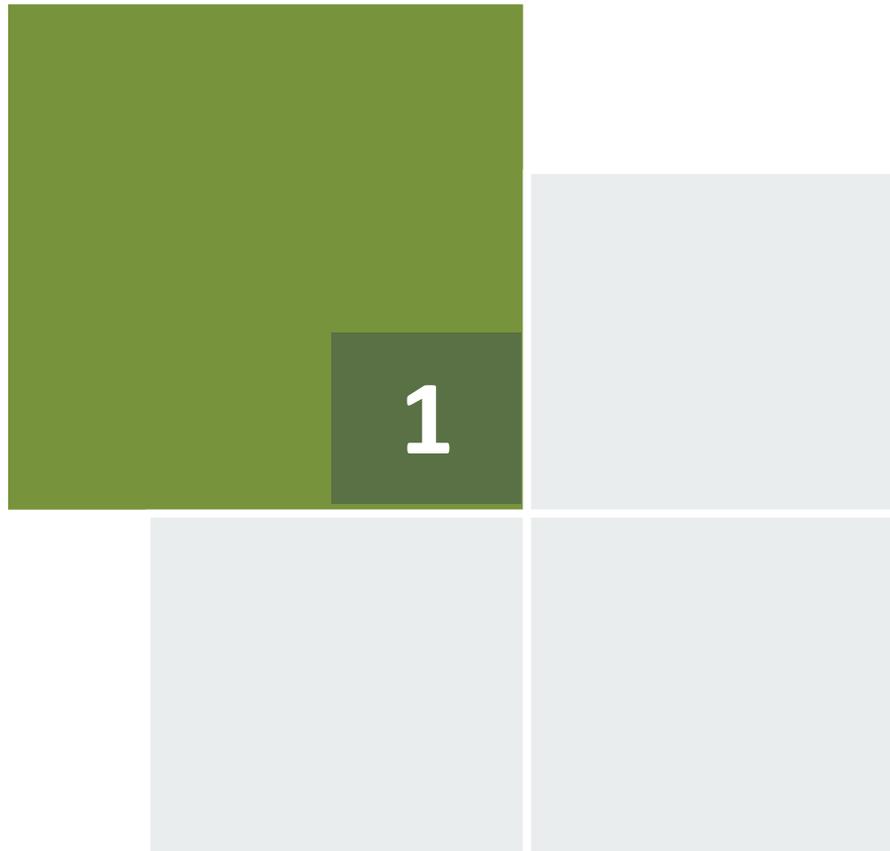
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CAPACITY
BUILDING &
EMPOWERMENT

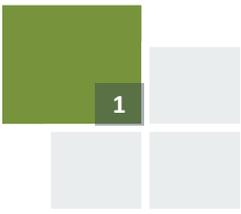
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ACCELERATING
TRANSLATION
OF RESEARCH &
THERAPY
DEVELOPMENT

PILLAR 1: COLLABORATIVE RESEARCH FUNDING



Ralph SCHUSTER (DLR, DE) & Sonja van WEELY (ZonMw, NL)



PILLAR 1-PROPOSED STRUCTURE

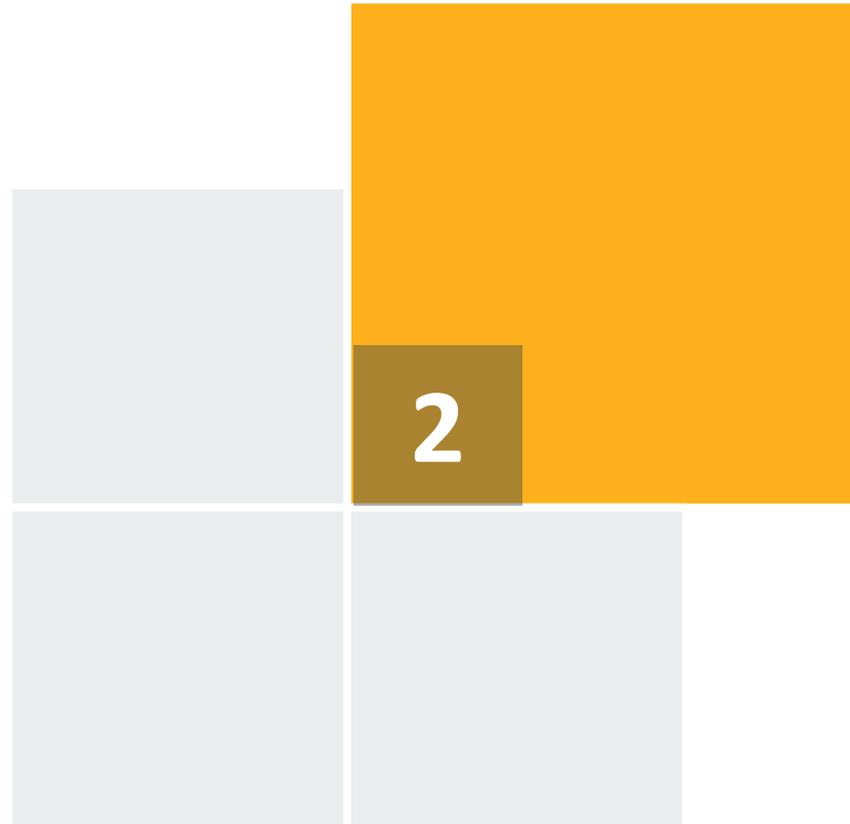
WP1: Joint Transnational Calls for collaborative research projects

WP2: Networking to share knowledge on rare diseases

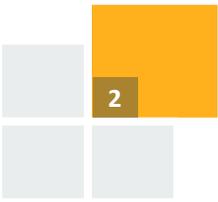
WP3: Rare disease research challenges

WP4: Monitoring of granted projects

PILLAR 2: COORDINATED ACCESS TO DATA AND SERVICES



Ana RATH (INSERM-Orphanet, FR) & Olaf Riess (Univ Tübingen, DE)



PILLAR 2 - PROPOSED STRUCTURE

WP1: Management of the next-generation data ecosystem strategic plan

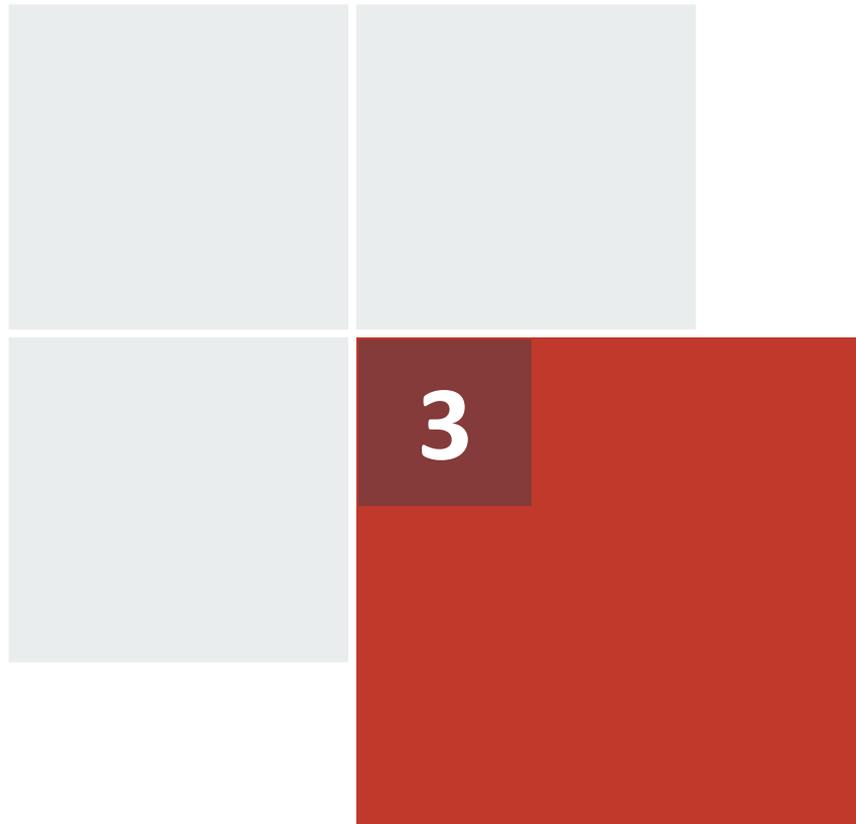
WP2: Common platform for discoverable data and resources for RD research

WP3: Enabling sustainable FAIRness and Federation at the data record, patient and sample level for RD

WP4: Enabling multidisciplinary, holistic approaches for rare disease diagnostics and therapeutics

WP5: Accelerating research capacity of the ERNs to increase diagnostic capacity and to hustle compound validation for therapy development

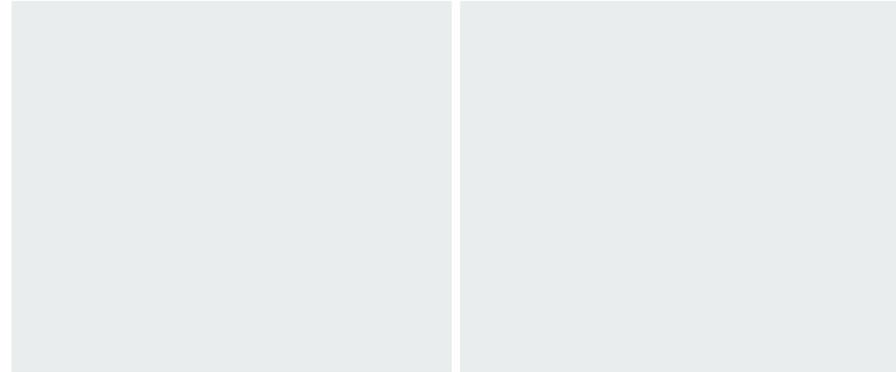
PILLAR 3: CAPACITY BUILDING AND EMPOWERMENT



Virginie BROS-FACER (EURORDIS), Biruté TUMIENE (Univ Vilnius, LT)



PILLAR 3 - PROPOSED STRUCTURE



WP1: Training on data management & quality

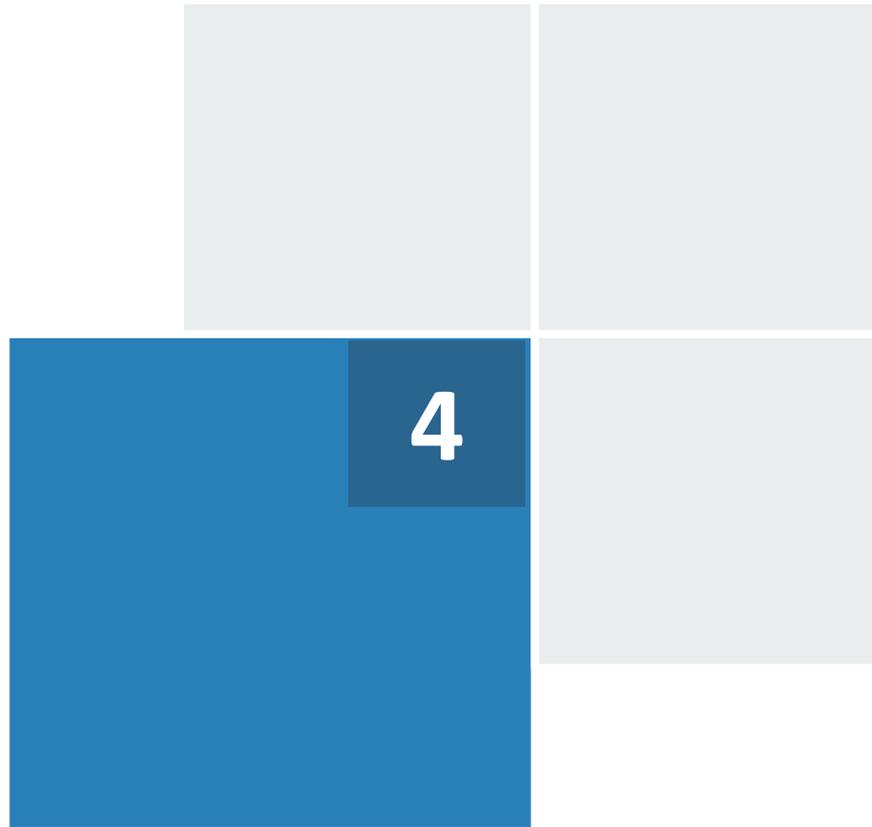
WP2: Capacity building & training of patients and researchers

WP3: Online academic education course

WP4: ERN RD training & support programmes

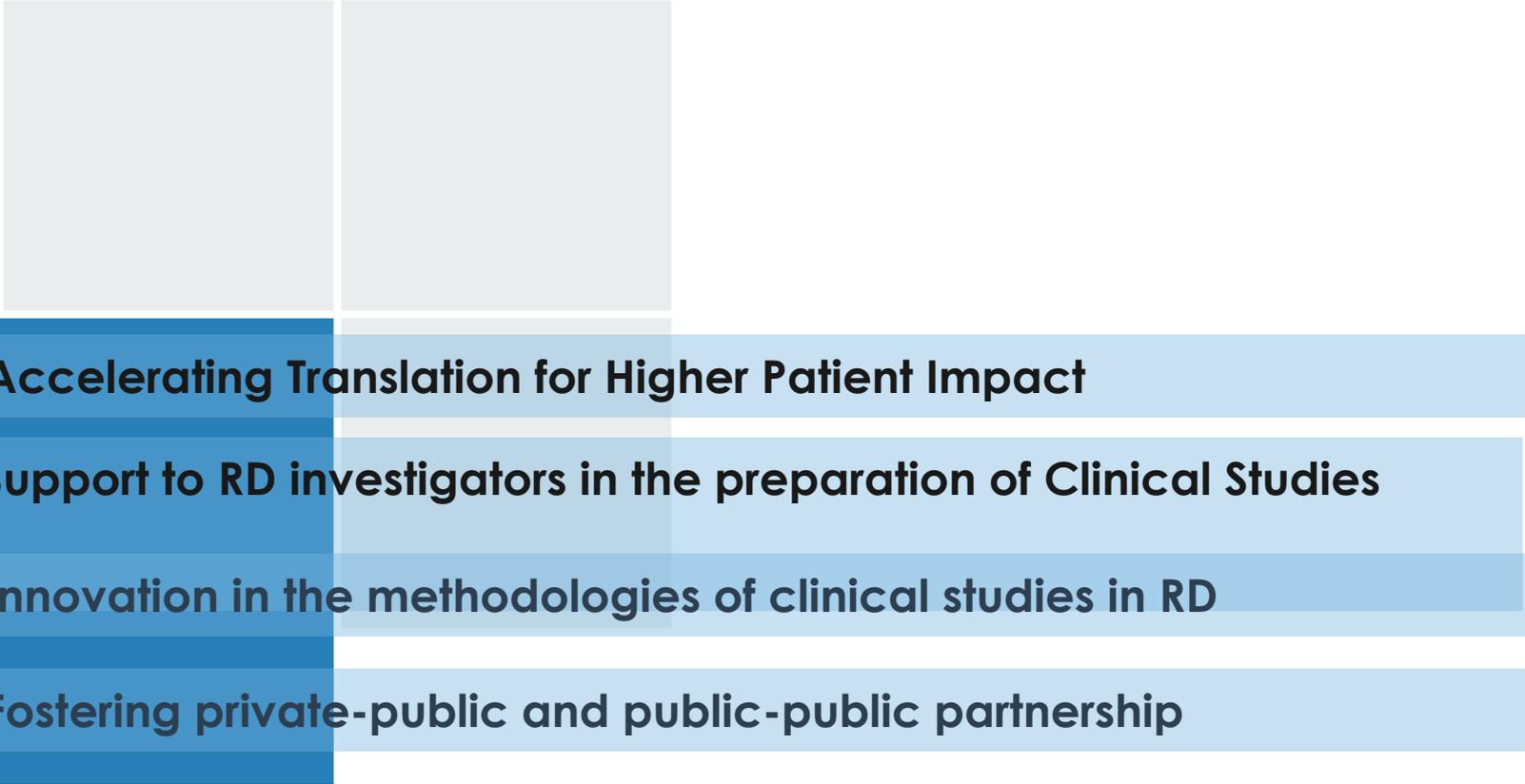
WP5: Development and adaptation of training activities

PILLAR 4: ACCELERATING TRANSLATION & THERAPY DEVELOPMENT



**Rima NABBOUT (Imagine, FR), Anton USSI (EATRIS)
Maurizio SCARPA (ERN chair)**

PILLAR 4 - PROPOSED STRUCTURE



WP1: Accelerating Translation for Higher Patient Impact

WP2: Support to RD investigators in the preparation of Clinical Studies

WP3: Innovation in the methodologies of clinical studies in RD

WP4: Fostering private-public and public-public partnership

GOVERNANCE STRUCTURE

STRATEGY LEVEL

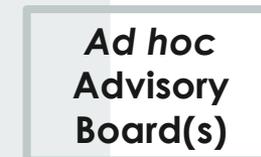
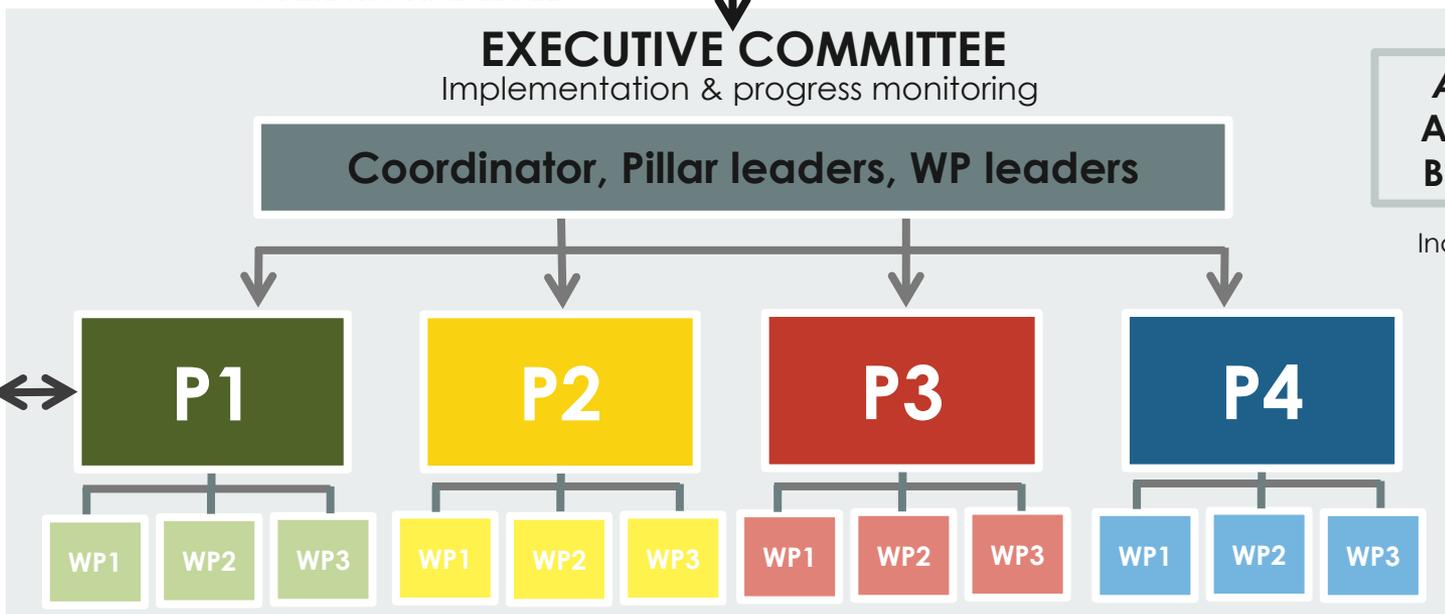


COORDINATION LEVEL



Ensures alignment of strategies

OPERATIONAL LEVEL



Independent advice



P1 call topics & management



THANK YOU

for any questions contact:

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