

# EUROPEAN JOINT PROGRAMME ON RARE DISEASES



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# RARE DISEASE RESEARCH LANDSCAPE IN EUROPE

- Rare Diseases (RD) affect 6-8% of European citizens in the course of their lives and represent a substantial burden on patients and their families
- Research on Rare Diseases is strongly supported by the EC and Member states
  - international collaborative research by EC framework programmes, ERA-Net E-Rare, national programmes
  - infrastructures and tools (RD-connect, European Research Infrastructures like ECRIN, BBMRI etc.)
  - coordination efforts (International Rare Diseases Research Consortium (IRDiRC), RD-action)
  - improvement of quality, safety and access to highly specialized healthcare through European Reference Networks (ERNs)
  - information on RD (Orphanet)
- BUT RD landscape is fragmented → need for stronger coordination of future Research and Care activities in Europe

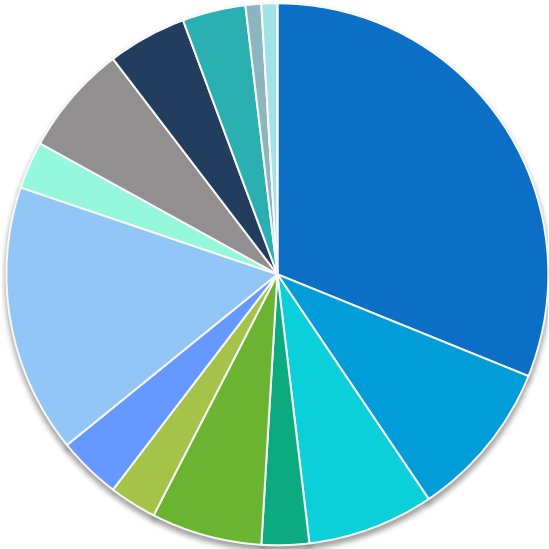
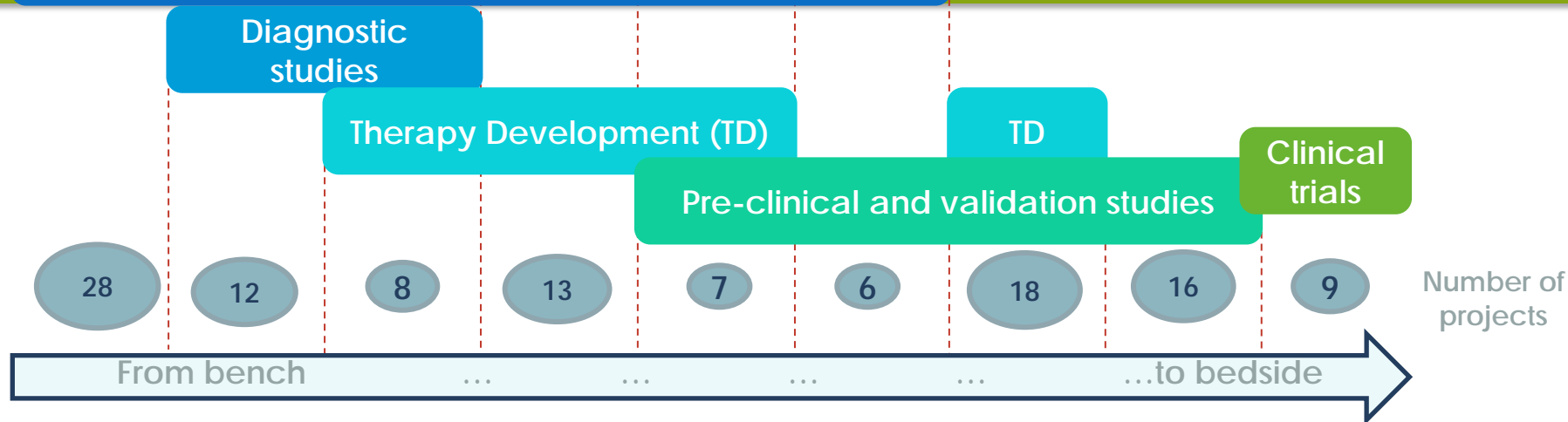
# MAJOR FACTS ABOUT E-RARE

- 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 disease



# E-RARE CALLS

## Molecular and pathophysiological studies



- Neurology
- Metabolic diseases
- Dermatology
- Others
- Musculoskeletal Diseases
- Rheumatology
- Pulmonary/Respiratory Diseases
- Hematology/Immunology
- Nephrology/Urology
- Cardiology/Vascular Diseases
- Ophthalmology
- Dismorphology
- Endocrinology
- Psychiatry/Psychology

**9** Joint Transnational Calls:

**117** funded projects

**104 M€** invested

Including **556** research partners funded

Medical domains represented in the funded projects



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## OBJECTIVES of EJP RD

- **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

## IRDiRC goals 2017 - 2027

- All patients coming to medical attention with a suspected rare disease **will be diagnosed within one year** if their disorder is known in the medical literature; all currently undiagnosable individuals **will enter a globally coordinated diagnostic and research pipeline;**
- **1000 new therapies** for rare diseases will be approved, the majority of which will focus on **diseases without approved options;**



# EUROPEAN JOINT PROGRAMME ON RARE DISEASES

- **Union contribution:** 55 M€ (70% reimbursement rate)
  - **Total budget (min. submitted):** 93,53 M€ (→ expected > 110 M€)
  - **Number of partners:** 87
  - **Number of participating countries(beneficiaries and LTPs):** 33  
including 25 EU MS (AT, BE, BG, CZ, DE, ES, EE, FI, FR, GR, HU, IE, IT, NL, LT, LV, LU, MT, PL, PT, RO, SE, SK, SL, SV, UK), 8 associated (AM, CH, GE, HR, IL, NO, RS TK) and third countries (CA)
  - **Timeline:** Jan 2019 – Dec 2024
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- **Types of partners:**
    - 31 research funding bodies/ministries
    - 12 research institutes
    - 22 universities/hospital universities
    - 11 hospitals
    - 5 EU infrastructures (BBMRI, EATRIS, ECRIN, ELIXIR, INFRAFRONTIER) + EORTC
    - EURORDIS & ePAGs
    - 5 charities/foundations (FTELE, AFM, FFRD, FGB, BSF)
- } **24 ERNs**



# EJP RD STRUCTURE

COORDINATION  
& TRANSVERSAL ACTIVITIES

INTEGRATIVE RESEARCH & INNOVATION STRATEGY

COMMUNICATION

SUSTAINABILITY

HELPDESK OFFICE – FAST TRACK TO SUPPORT & INFORMATION

1

COLLABORATIVE  
RESEARCH  
FUNDING

2

COORDINATED  
ACCESS TO  
DATA &  
SERVICES

3

CAPACITY  
BUILDING &  
EMPOWERMENT

4

ACCELERATING  
TRANSLATION &  
THERAPY  
DEVELOPMENT



# PILLAR 1- COLLABORATIVE RESEARCH FUNDING

## WP6: Joint Transnational Calls for collaborative research projects

- 2 cofunded calls + 2 non-cofunded – open to research teams from countries with funders involved; Germany: BMBF & DFG
- First call 2019: 31 funders from 23 European and associated countries like Israel, Turkey, Canada; about 35 M€ budget
- Topic: Transnational research projects to accelerate diagnosis and/or explore disease progression and mechanisms of rare diseases
- Expected dates: preannouncement this week, call published mid-December 2018, deadline for pre-proposal submission mid February 2019
- Expected future call topics: preclinical therapy development, social and health systems related research, clinical therapy development





# PILLAR 1- COLLABORATIVE RESEARCH FUNDING

## WP7: Networking to share knowledge on rare diseases

- small support schemes for networking and establishment of new collaborations for knowledge sharing
- workshops/events/share of knowledge
- 30 K€ max per grant, 1.5 Mio. overall budget
- open all year long, start in mid 2019
- open to all countries involved in EJP RD

## WP8: Rare disease research challenges

- address needs (challenges) from industry by funding collaborations between industry and academics and/or SMEs to show proof of concept (POC) of solutions for scientific and business challenges
- maximum grant amount for 1<sup>st</sup> phase POCs of 150.000 € and for 2nd phase of 100.000 €
- industry collaborator should complement this with 30% of funding (50 000 €)
- total budget: ~1.5M €, funding for up to 6 projects
- start in 2020



# THANK YOU FOR YOUR ATTENTION!

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# WP1 COORDINATION & MANAGEMENT

WP2  
STRATEGY

WP3  
SUSTAINABILITY

WP4  
ETHICS, LEGAL, REGULATORY & IPR

WP5  
COMMUNICATION & DISSEMINATION



P1

WP6  
Joint Transnational Calls

WP7  
Networking scheme

WP8  
RDR Challenges

WP9  
Monitoring of funded projects



P2

WP 10  
User-driven strategic planning for P2

WP 11  
Virtual Platform for data & resources

WP 12  
Enabling sustainable FAIRness

WP 13  
Holistic approaches for rare disease diagnostics and therapeutics



P3

WP 14  
Training on data management & quality

WP 15  
Capacity building and training of patients and researchers

WP 16  
Online Academic education course

WP 17  
ERN RD training and support programme

WP 18  
Development and adaptation of training activities



P4

WP 19  
Facilitating partnerships and accelerating translation

WP 20  
Validation, use and development of innovative methodologies for clinical studies