ERDERA
European Rare Diseases
Research Alliance

Making Europe a world-leader in rare diseases research and innovation

Connecting the dots in the rare diseases research ecosystem

Rima NABBOUT, MD, PhD
Reference centre for rare epilepsies, EPICARE membre,
Necker Enfants Malades, APHP, Univesity Paris cité, Institut Imagine, Paris France

Co-funded by the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
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ERDERA

EUROPEAN **RARE DISEASES** RESEARCH ALLIANCE

2024 – 2034

Co-funded by the European Union

ERDERA proposal n°101156595 is under grant preparation and will be funded under call HORIZON-HLTH-2023-DISEASE-07 as a COFUND Action.

EJP RD

ASSISTANCE PUBLIQUE HÔPITAUX DE PARIS

Necker ENFANTS MALADES

HÔPITAL UNIVERSITAIRE

Imagine's unique ecosystem for R&I in genetic diseases

Proximity, transversalities, innovation

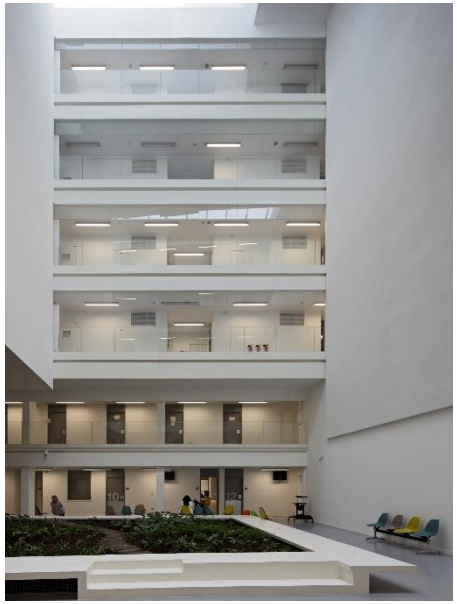
Conference center

Researchers & Physicians
24 research labs
4 associated labs

Students
PhD/MD-PhD
Master/MD-M2

Engineers
18 core facilities

Biobank



Patients
30,000 consultations/year in situ
31 reference centers for rare diseases
8 clinical departments 2 clinical investigation centers

Support services
Personalized management
Fundraising
Communication

Health innovation experts
Technology transfer
Business Development
Entrepreuneurship
Grant office
Clinical research
Regulatory&Legal

Companies & Bioentrepreneurs
7 startups co-founded by *Imagine*'s PIs

codoc · THERANOVIR · medetia PHARMACEUTICALS · smart immune · step pharma · sonio · inatherys

Located inside the Necker-Enfants Malades university hospital campus

Pediatric clinical facility
Adult clinical facility
Medicine and pathology laboratory

Closely interfaced with:

Universities
Research organisms
Hospitals
Companies
Entrepreuners
Investors and donors
Facilitators

imagine
INSTITUT DES MALADIES GÉNÉTIQUES

INSTITUT CARNOT Imagine

ERDERA

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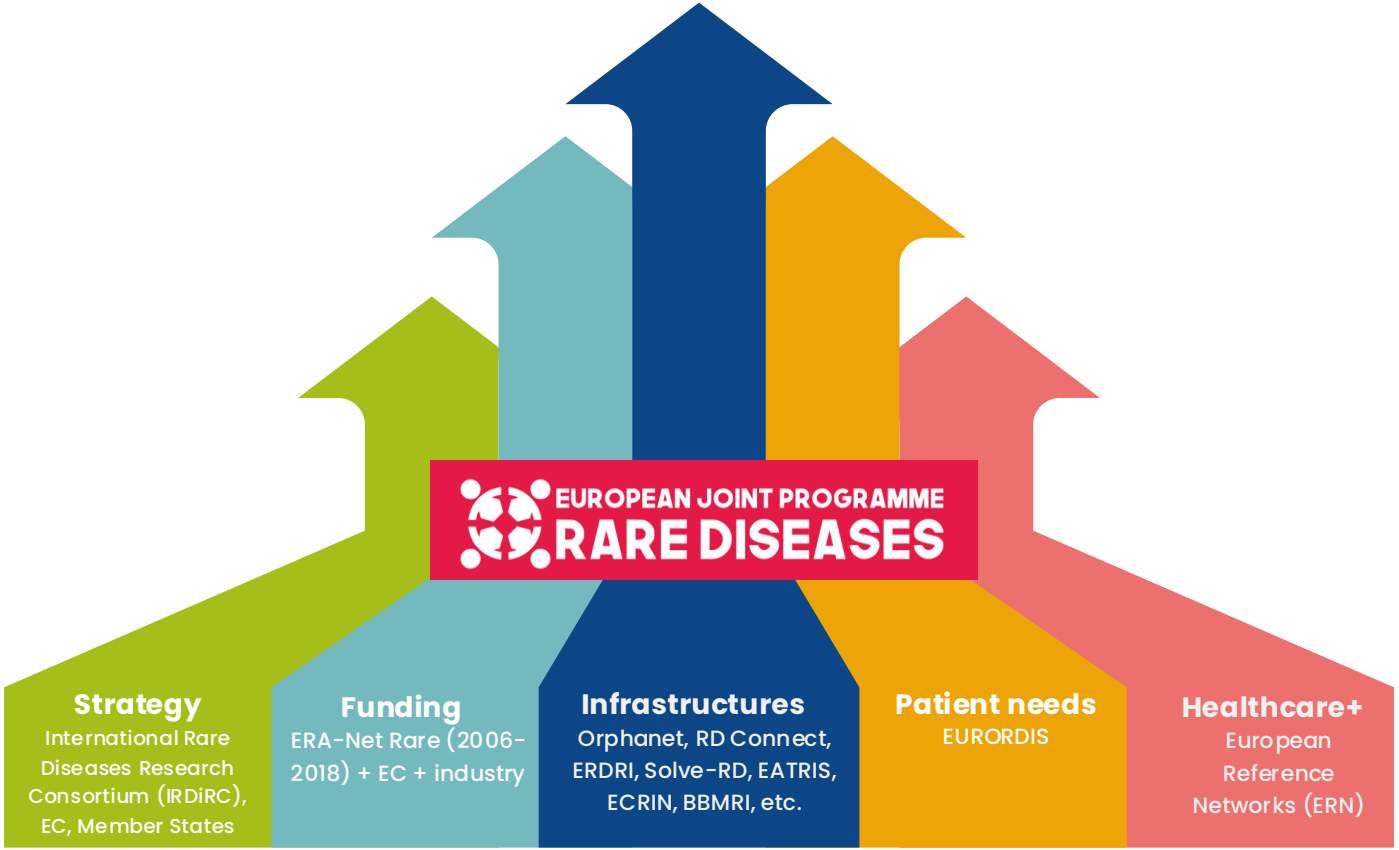
WHEN?


WHERE?


EUROPEAN JOINT PROGRAMME
RARE DISEASES
Strategy
International Rare Diseases Research Consortium (IRDiRC), EC, Member States
Funding
ERA-Net Rare (2006-2018) + EC + industry
Infrastructures
Orphanet, RD Connect, ERDRI, Solve-RD, EATRIS, ECRIN, BBMRI, etc.
Patient needs
EURORDIS
Healthcare+
European Reference Networks (ERN)

Why is ERDERA a major achievement for rare diseases?

ERDERA
European Rare Diseases Research Alliance

Remaining unmet needs

95%

of RDs are disregarded in terms of research & effective treatment options

50%

of RD patients still do not have confirmed molecular diagnosis

4

years - is an average time to be diagnosed when RD is known

52%

of RD patients and carers, RD translates into severe impact on their daily life

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37 Countries

25 EU member states

9 associated countries

3 non-EU

178 Organisations

40 funders

81 research performing organisations

9 patients' organisations

3 research infrastructures

22 private for-profit partners (industry & SME)

23 other (univ, hospital, non-profit, public administration)

Involvement of ERNs

BOND
CRANIO
ENDO
EpiCare
ERKNet
ERNICA
EURO-NMD / DDF
RND
EuroBloodNet
eUROGEN
EYE
ITHACA
MetabERN

EUROPEAN REFERENCE NETWORKS
FOR RARE, LOW-PREVALENCE AND COMPLEX DISEASES
Share. Care. Cure.

European Reference Networks

European Commission | Health

Coordination & Strategy

WP1
Coordination and management

WP2
Communication & dissemination

RD Funding

WP3
Joint Transnational Calls for collaborative research projects

WP4
Clinical trial call management

WP5
Networking to share knowledge on research

Clinical Research Network

WP6
Diagnostic data availability

WP7
Genome re-analysis research pipeline

WP8
Innovation to shorten time to RD diagnosis

WP9
Real world data

WP10
Clinical Outcome Assessment

WP11
Advanced Therapeutic Medicinal Products

WP12
N-of-few approach

Data Services Hub | Expertise Services Hub

WP13 WP14 WP15 WP16 WP17 WP18 WP19

Education & Training Hub

WP20

Acceleration Hub

WP21 WP22

Inter(national) Capacity Alignment

WP23
NMGs promotion and national alignment

WP24
Fostering engagement of underrepresented countries in ERDERA

WP25
ERDERA Global Collaboration

WP13
Rare Diseases-Virtual Platform (RD-VP): Finding and accessing the data ecosystem

WP14
Data readiness services

WP15
Data sharing and analysis services

WP16
Knowledge bases and ontologies for RD research

WP17
Mentoring and consultancy

WP18
Regulatory support service

WP19
Methodological Support

WP20
Education and training in rare diseases research

WP21
Technology accelerator

WP22
Public-Private Collaboration Accelerator

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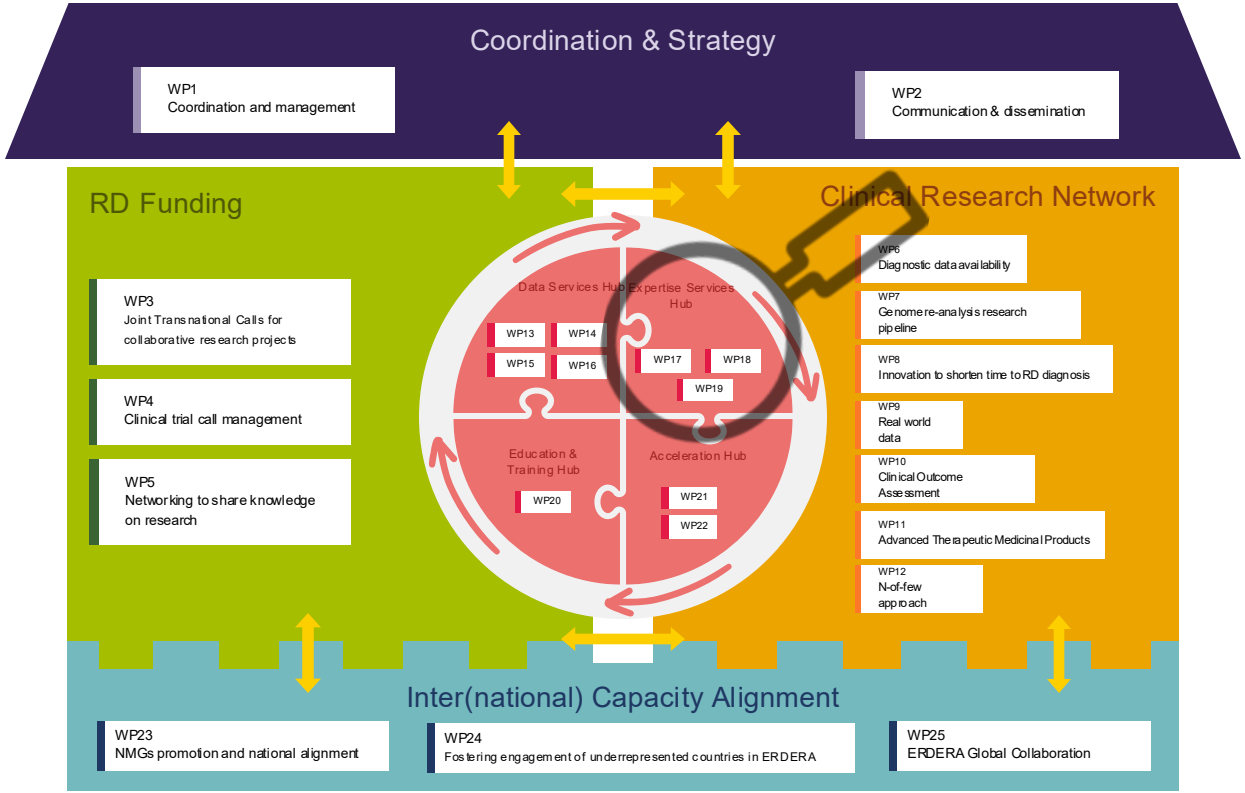

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ERDERA European Rare Diseases Research

Support and expertise Workstream welcomes you!

Mentoring/Consultancy service
WP17

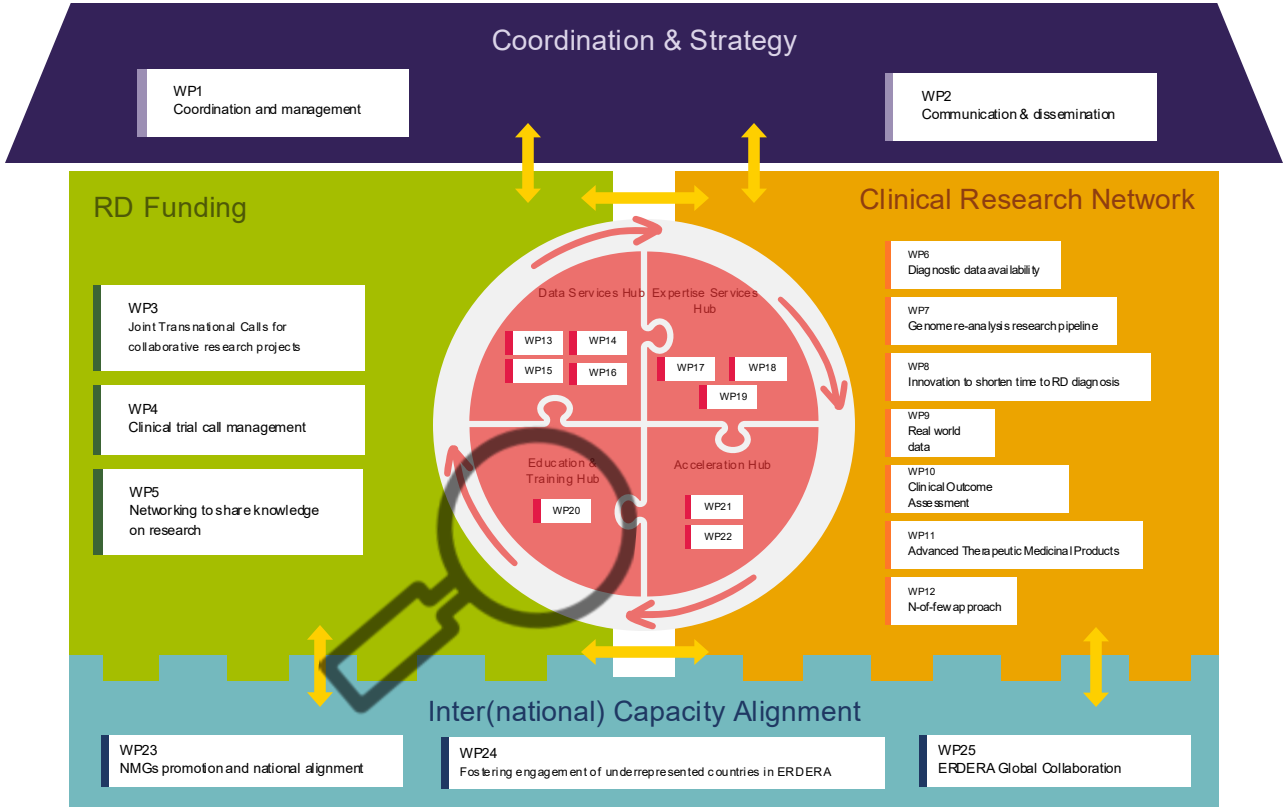
EATRIS
Toni Andreu
toniandreu@eatris.eu

Regulatory Support Service
WP18

Benzi Foundation
Viviana Giannuzzi
vg@benzifoundation.org

Methodological Support
WP19

APHP
Rima Nabbout
rima.nabbout@aphp.fr



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ERDERA European Rare Diseases Research

WP20: Education and Training in RD research

- **Task 20.1 –** Patients and young researchers' trainings
- **Task 20.2-** Identification and fulfilment of training needs
- **Task 20.3 -** RD research training for multistakeholder communities
- **Task 20.4-** European Curriculum on RD research

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Thank you!

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