‘Snapshot’ of Transversal activities regarding ERNs and Clinical Research

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European Commission

RD ACTION
DATA AND POLICIES FOR RARE DISEASES

WG Research

European Reference Networks

COORDINATION	TRANSPLANTCHILD ,EURACAN, ERKNET, LUNGERN, METABERN, ERNPEDCAN, BONDERN, ENDOERN, RECONNET, ERNSKIN, ERNRITA, VASCERN, EUROBLOODNET, GENTURIS, GUARD-HEART, ERNEYE
• Luca Sangiorgi (BOND) • Eduardo López Granados (Transpantchild)* • Alberto Pereira (ENDO) • Franz Schäffer (ERK NET)	

* Current spokeperson

SCOPE

European Reference Networks

WG Research

European Reference Networks (ERNs) are virtual networks involving healthcare providers across Europe. They aim to tackle complex or rare diseases and conditions that require highly specialised treatment and concentrated knowledge and resources.

The currently existing 24 ERNs provide also an unprecedented platform to identify pre-clinical, translational and clinical research gaps within their areas of expertise, and to promote, support, or even lead research initiatives in collaboration with other stakeholders such as patients associations, industry, research institutions, pan-European infrastructures and policy makers.

The Working Group on Research of the ERN Board of coordinators aims to potentiate the research capabilities of individual networks by means of cooperation and mutual reinforcement in common strategic areas and activities, building up on existing resources and capabilities, and promoting new developments in a harmonized and integrative way.

WORKING PROCEDURES	Governance and process for decision making within the groups: Co-chairs with an annual rotating spokesperson. Voting if any decision has to be taken. Inventory of needs, strengths and capabilities. Communications: Within the group: regular webex teleconferences. Face to face meeting on occasions coinciding with the ERNs CG meetings. DG Sante (contact person Hélène Le Borgne), Documents and information uploaded on CIRCA BC

European Reference Networks

WG Research

GOALS	**- Building up research capabilities among ERNs: survey among all ERNs regarding mapping research interests, priorities, capacities and resources** **-Next H2020 call: how ERNs could interact with this process** **-Interaction with IMI initiatives, EMA, and in general the aspects related to clinical trials** **-Big Data and ERNs: how we could approach to this aspect**

ACTIVITIES SO FAR European Reference Networks WG Research	- **Organization of the first research group meeting, Brussels 12th October 2017** - **INTENSE PARTICIPATION OF ERNs in the drafting of the EJP on rare diseases** - **Preparation RD-ACTION, European Medicines Agency, and DG SANTE Workshop: How can ERNs add value to clinical research in rare diseases and highly specialised domains? (L. Sangiorgi)** - **Regular teleleconferences**

Ethics WG & Companies' Position

- WG on Ethics & Legal Issues: Chaired by Nicoline Hoogebrugge (ERN GENTURIS) with additional ERN members ERN-ITHACA, MetabERN, ERN-EYE, VASCERN
- Developing a Statement on 'MANAGING CONFLICTS OF INTEREST'

- Sept 2017 – Workshop of EURORDIS RoundTable of Companies

RD-Action Workshop, EMA London May 29th 2018
Transversal activities regarding ERNs and Clinical Research

Prof. Eileen Treacy (on behalf of ERN Board of Member States, Working Group for ERNs and Industry - Chair: Prof. Helena Kaariainen)

Commission Delegated Decision (2014/286/EU)

ERNs 'should be governed by a board of the Network composed of representatives from each Member in the Network. The Board should be in charge of producing and adopting the rules of procedure, work plans and progress reports and any other documents related to the activities of the Network'.

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ERNs 'should improve access to diagnosis, treatment and provision of high-quality healthcare to patients who have conditions requiring a particular concentration of resources or expertise and could also be focal points for medical training and research, information dissemination and evaluation, especially for rare diseas
(Delegated Decision, 2014/286/EU)

'Patient-centred'. Priorities: Best practice guidelines for diagnosis and care, clinical research, registries, clinical trials and social care.

Operational criterion 6.3.3 (for networks): The network adheres to ethical criteria, is transparent and avoids any conflict of interest when developing and implementing clinical guidelines, patient pathways and other clinical decision making tools.

Conflict of interest: (USA Institute of Medicine 2009): 'set of circumstances that create a professional judgement or actions regarding a primary interest (i.e. promoting and protecting the integrity of research, the welfare of patients and the quality of medical education) that will be unduly influenced by a secondary interest (e.g. financial or academic interest)'.

https://ec.europa.eu/health/sites/health/files/ern/docs/statement_industry_conflictofinterest_en.pdf

European Reference Networks

Board of Member States

Statement on European Reference Networks (ERNs) and industry

November 2016

In recognition of the importance of industry in improving our knowledge of rare conditions and developing clinical tools and therapies, the Board of Member States agrees with engagement between ERN members and industry where appropriate, for example in clinical trials and research projects.

However, there is no legal provision for the involvement of external stakeholders, including industry, in the operation and governance of ERN. To address this issue and to steer ERN in their thinking on engagement

- *A complete transparency policy should apply to the relationship between ERNs and Industry*
- *Each designated ERN should define its own Conflict of Interest Policy and ensure disclosure of all financial and non financial conflicts of interest before any engagement commences*
- *Conflict of Interest policies for Networks and HCPs must respect national and European legislation*

ROLE OF THE ERNs IN THE EUROPEAN JOINT PROGRAMME ON RARE DISEASES (EJP RD)

EUROPEAN JOINT PROGRAMME ON RARE DISEASES

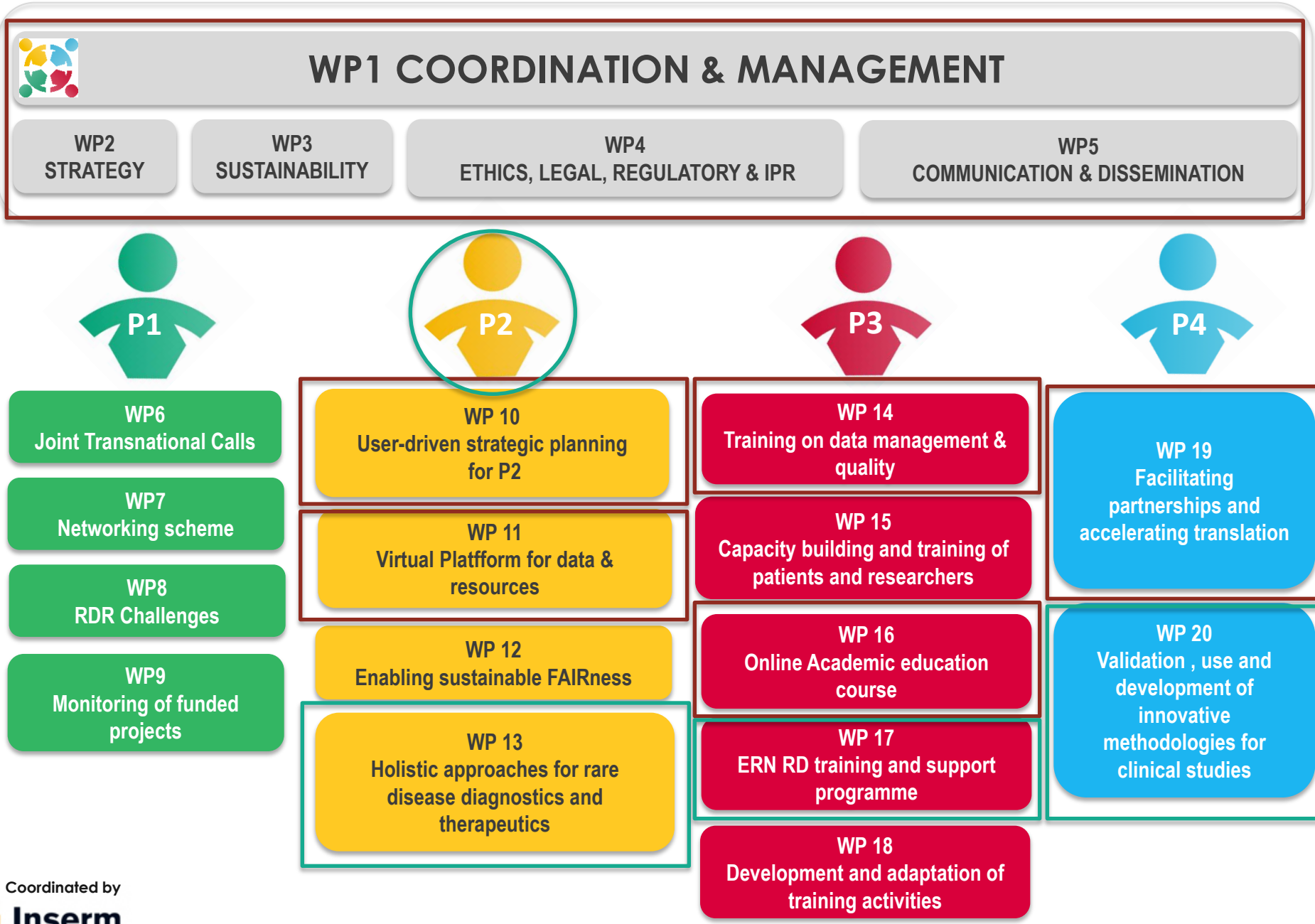
European Joint Programme on Rare Diseases

- **Union contribution:** 55 M€ (70% reimbursement rate)
- **Total budget (min. submitted):** 93,53 M€ (→ expected > 110 M€)
- **Number of partners:** 85
- **Number of participating countries(beneficiaries and LTPs):** 33 including 25 EU MS (AT, BE, BG, CZ, DE, ES, EE, FI, FR, 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

- **Types of partners:**
  - 29 research funding bodies/ministries
  - 12 research institutes
  - 22 universities/hospital universities
  - 11 hospitals

  } **24 ERNs**

  - 5 EU infrastructures (BBMRI, EATRIS, ECRIN, ELIXIR, INFRAFRONTIER) + EORTC
  - EURORDIS & ePAGs
  - 5 charities/foundations (FTELE, AFM, FFRD, FGB, BSF)


WP1 COORDINATION & MANAGEMENT
WP2 STRATEGY
WP3 SUSTAINABILITY
WP4 ETHICS, LEGAL, REGULATORY & IPR
WP5 COMMUNICATION & DISSEMINATION
P1
P2
P3
P4
WP6 Joint Transnational Calls
WP7 Networking scheme
WP8 RDR Challenges
WP9 Monitoring of funded projects
WP 10 User-driven strategic planning for P2
WP 11 Virtual Platfform for data & resources
WP 12 Enabling sustainable FAIRness
WP 13 Holistic approaches for rare disease diagnostics and therapeutics
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
WP 19 Facilitating partnerships and accelerating translation
WP 20 Validation , use and development of innovative methodologies for clinical studies
Coordinated by
Inserm
La science pour la santé
From science to health

EUROPEAN JOINT PROGRAMME ON RARE DISEASES

WP20: Accelerating the validation, use and development of innovative methodologies tailored for clinical trials in RDs

Disseminate → Demonstrate → Design → Develop

- 7 ERNs involved from T0 (Metab-ERN, ERN-BOND, EPICARE, Transplantchild, Paedcan, ERKNet, ERN-SKIN)
- 1 TASK FORCE GROUP responsible for coordination & monitoring of WP20 activities through "extended" expertise (inclusion of ERN experts and other experts dependent on the topic(s)/task(s) to treat)
- **3 main objectives:**
  - Support in design and planning of RD clinical studies:

    Creation of support office within ECRIN, dedicated and adapted to the needs of ERNs
  - Demonstration projects on existing statistical methodologies to improve RD clinical trials:

    Dedicated "internal" call for demonstration projects to validate existing innovative methodologies to provide necessary proofs of their efficacy and thereby raise awareness and usage of these methodologies → ERNs as key partners in these projects and dissemination channel
  - Projects on innovative methodologies to improve RD clinical trials in limited populations:

    Based on recommendations of IRDiRC TF for "Small population CTs" and TFG identification of gaps and most promising areas of research on methodologies. Involvement of clinicians, patients and methodologists considered as most relevant