

Sustainability of EJP RD Results

PILLAR 0

Coordination services EJP RD & IRDIRC SciSec

Proactive, adaptable and efficient planning, evaluation, monitoring and repair solving to optimise quality results, fostering links with other initiatives for efficiency and synergistic actions.



Central Helpdesk

Provision of centralised easy access to RD expertise, with request processes and tailored services for users, nurturing through dissemination strategies to increase visibility of EJP RD services.



Sustainability Handbook

The general guidance on sustainability key factors helps the RD Community and other actors to plan and operate in a sustainable manner.



Advisory Regulatory Ethics Board (AREB ELSI Services)

Reference single contact point, easily identified, for ELSI questions, with the provision of quality and time-efficient ethics review of RD-research proposals, accelerating their implementation.



ReACT) Congress and IRDIRC Conference

Promotion of scientific cooperation and research on rare and orphan diseases in a unique Face-to-Face congress that fosters RD Research and Policy independently on medical domain.



PILLAR 1

Monitoring

Tool that permits the centralisation and analysis of monitored/funded projects, identified by the handbook from experts. Annual collection of information from projects and analysis of their progress.



Networking Support Scheme

Organisation of networking events that involve RD research stakeholders, key for RD research to advance. Encouragement of underrepresented stakeholders and tourist involvement.



Research Challenges Scheme

Innovative funding scheme involving consortia of applicants (Academia, SME, and FNDs) Advisory Organizations) and industry sponsors. Public-private partnerships facilitator.



PILLAR 2

Virtual Platform assets

Federated platform of resources for RD research that facilitates the discovery, access and analysis of data and samples scattered across several resources in Europe. It represents the central value of the research ecosystem.



Which updates do you have relating stakeholders interested in funding the element?

Pillar 3

Orphanet training material & module

Empowerment of Orphanet network members at national level, to deliver local training of the nomenclature and ontology. Training for trainer sessions and trainer's toolkit.



Registries and undiagnosed courses

Opportunity to meet and create networking among professionals involved in RD registries, undiagnosed rare conditions and facilitators, promoting further interactions & collaborations.



Training quality assurance Next Generation Sequencing (NGS)

Large scope on NGS diagnostics, with yearly updates, helping in the translation of research tools to diagnostic applications. Up to date training with an international level with expert trainers.



Trainings on biobanks and samples

Revisiting communication and knowledge exchange about RD research biobanking and related sampling procedures to deliver training, increasing visibility and sharing of proprietary material.



Program delivery for the 3 schools (EURORDIS Academy)

Empowerment of rare disease patient advocates with knowledge and skills to take part in patient engagement roles side-by-side with researchers, regulators, and industry and policy makers.



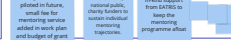
Innovation Management Toolbox

Centralised repository of resources that provides searchability and answers to specific questions. It may contain topics as the Sustainability Handbook.



Mentoring Service

Easy access to full gamut of therapy development expertise that increases the success rate in translational research projects of individual researchers by stimulating their productivity and building confidence.



Workshops/training contents for paediatric patients

Facilitator of the involvement of paediatric patients in ethically sound rare disease research. The workshops aim to provide young rare disease patients with engagement skills in RD research.



Online academic course (MOOC)

Cover the lack of formalised training on RD, by delivering free, easily accessed, and engaging MOOCs on translational rare disease research topics, with scalable contents, to a wide audience.



ERN Workshops (training multipliers)

Sharing of knowledge and methodologies applicable for several different RD fields, highly relevant for ERNs, at the European level, with perspective of accreditation.



ERN Workshops material

ERNs Workshops (training multipliers) material. This training material complement research coverage and topics that are cutting-edge and of cross ERNs benefit.



Secondments (Mobility fellowships)

The fellows have the possibility to obtain a highly specific and tailored training, acquiring new research experiences and learning new methodologies, through exchanges between institutions.



Clinical Trials Methodologies Dissemination



PILLAR 4

Clinical Trials Support Office

Support services for the planning and design of clinical studies for Rare Diseases (RD), with special focus in translational research. Built up on the expertise of partners with years of experience in their respective fields.

