1 **Supplementary Materials**

2 **The European joint programme on rare diseases: building the rare diseases**

3 **research ecosystem**

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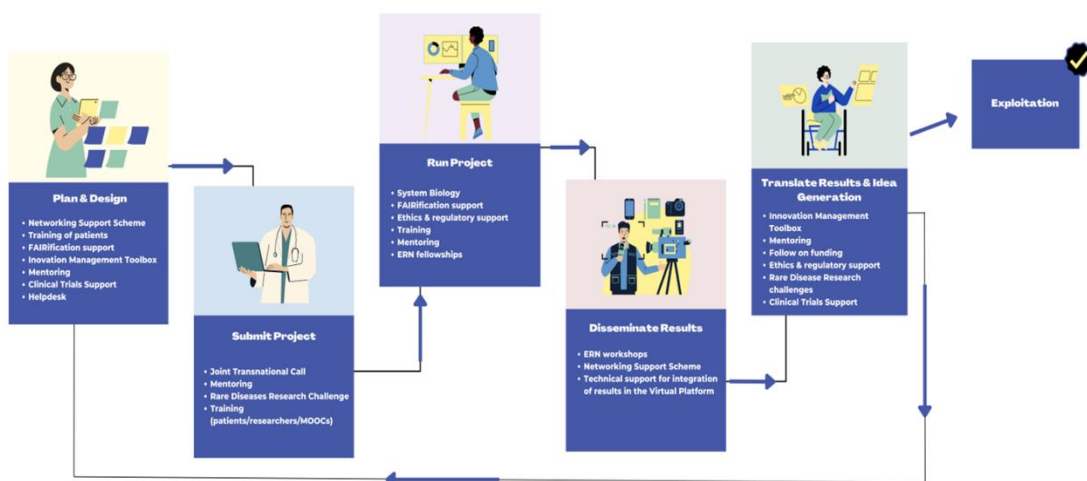
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Supplementary Figure 1. The EJP RD research support cycle shows the EJP RD services offered to the Rare Diseases community at all stages of the research project development.

Supplementary Table 1. EJP RD funded projects fostering demonstration and innovation in clinical trials methodologies.

Call	Project title
Rare Diseases Research Challenge	CHALLENGE #1: Development of a non-invasive tool for measuring rare disease patient mobility in daily living è Digital tools 4 Rare Diseases (DT4RD)
	CHALLENGE #2: Delivery system for intranasal administration of biological drugs to neonates è Intranasal Device for Neonates (INDENEO)
	Challenge #4: Pre-clinical assay to detect instability of microsatellite repeat è Development and validation of a novel pre-clinical assay to detect triplet repeat expansions (TRXassay)
Clinical Trials Methodology Demonstration Projects	EBStatMax -State-of-the-art design and analysis for maximal success with minimal patient burden in Epidermolysis bullosa trials
	Improve PSP - Application of improved statistical methodologies for clinical trials in Progressive Supranuclear Palsy

	EPISTOP-IDEAL - EPISTOP clinical trial data set re-use with statistical methodologies tailored for clinical trials in rare diseases
Innovation project in clinical trials methodology	iSTORE - Innovative Statistical Methodologies to Improve Rare Diseases Clinical Trials in Limited Populations
	EVIDENCE-RND - Creating robust evidence for longitudinal progression changes and treatment effects in ultrarare neurological diseases: the case of multisystemic autosomal-recessive ataxias

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