RealiseD
compRehensive mEthodological and operational Approach to cLinical trIalS in ultra-rarE Diseases

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From challenge to collective change: Reimagining Clinical Trials for Rare and Ultra-Rare Diseases through Co-creation

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BACKGROUND

RealiseD —compRehensive mEthodological and operational Approach to cLinical trIalS in ultra-rarE Diseases— is a public private partnership addressing a critical gap as 95% of rare and ultra-rare diseases (RD and URD) lack effective treatments, and traditional clinical trial approaches often fail.

Bringing together 50 partners from 14 countries, RealiseD advances multistakeholder co-creation, with patient organisations working alongside regulators, HTA bodies, academia, clinicians, and industry to redefine the RD and URD clinical trial landscape. Through real world clinical use cases, RealiseD innovates standards in clinical trial design and execution and statistical innovation, with regulatory and HTA dialogue, multistakeholder collaboration, and patient engagement.

GOALS

- Co-create cutting-edge operational and methodological tools
- Apply methodologic innovations to real-world clinical use cases including rare blood, bone, epilepsy, and eye disorders
- Partner with European Reference Networks (ERNs) to build a clinical trial site network and streamline patient identification and enrolment.
- Accelerate equitable therapeutic development and identify patient-relevant endpoints to improve outcomes.
- Establish new standards for clinical trialsin RDs and URDs, increasing uptake and implementation through early and continued multistakeholder dialogue.

To achieve these goals, patient engagement and multistakeholder collaboration are embedded across RealiseD. A Patient Advisory Group (PAG) has been established including 34 patient representatives from 23 patient organisations in 10 countries, complemented by European Patient Advocacy Groups (ePAG) members from collaborating ERNs and other people living with rare diseases (PLWRD). Engagement takes place through dedicated multistakeholder meetings and activities including webinars, communications, publications, and plain language summary materials. This co-creation approach guides the development of practical playbooks to support sustainable impact on clinical trial design and implementation.

CONCLUSIONS

RealiseD represents genuine patient partnership in methodological and operational clinical trial innovation.

Through this process, RealiseD leads with a commitment to innovations for a patient–centered and regulator-accepted clinical trial ecosystem for RDs and URDs.

26
PUBLIC PARTNERS

11
PRIVATE PARTNERS

13
ASSOCIATED PRIVATE PARTNERS

ACADEMIA	EUROPEAN REFERENCE NETWORKS (ERNs)	PHARMACEUTICAL COMPANIES	REGULATORY AND HTAs	ERICs, RESEARCH ORGANISATIONS	PATIENT ORGANISATIONS, CROs, SMEs
Sigmund Freud Private University Vienna; UHasselt; KU Leuven; Paracelsus Medizinische Universität; Humanitas University; Universitätsmedizin Göttingen; RUB; Medizinische Universität Wien; Radboudumc	ERN EYE; EpiCARE; ERN EBN; ERN BOND; European Reference Network MetabERN; ERKNet	AstraZeneca; Bayer; Boehringer Ingelheim; Ipsen; Janssen; Novartis; novo nordisk; sanofi; Servier; Roche; UCB	Critical Path Institute; European Medicines Agency; IQWiG	conect4children; eatris; Inserm; Vall d'Hebron Research Institute	EURORDIS; IDDI; teamit.

CLINICAL AND HEALTHCARE PARTNERS

Assistance Publique Hôpitaux de Paris Greater Paris University Hospitals; ASU FC Azienda sanitaria universitaria Friuli Centrale; Les Hôpitaux Universitaires de Strasbourg; SJD Sant Joan de Déu Fundació de Recerca; Servizio Sanitario Regionale Emilia-Romagna; Universiteit Gent; Heidelberg University Hospital; Otto von Guericke Universität Magdeburg; Vall d'Hebron Barcelona Campus Hospitalari

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