ERDERA

ERDERA – the European Rare Diseases Research Alliance – is a European partnership uniting over 170 public and private organisations across 37 countries around a single goal: turning cutting-edge science into tangible benefits for the thirty million Europeans living with a rare disease.

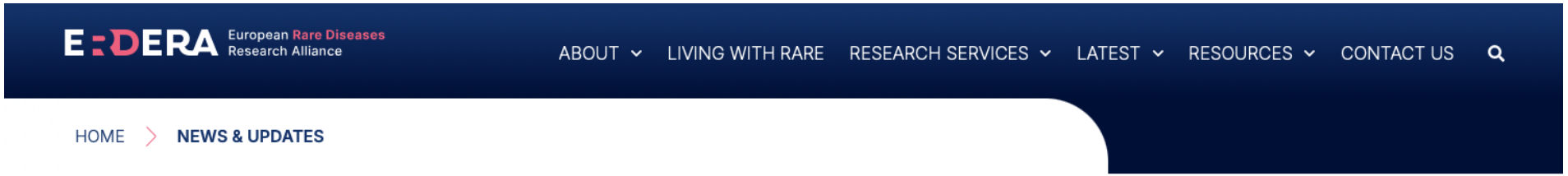
ERDERA
European Rare Diseases
Research Alliance

With an estimated overall budget of 380 million euros until 2031, ERDERA aims to have a major impact on rare diseases by supporting patient driven research to develop new treatments and diagnostic pathways and harnessing the potential of health and research data, Artificial Intelligence (AI) and digital technologies.

The European Union contributes around 150 million euros to this co-funded partnership via Horizon Europe, while the rest of the funding comes from member states, countries associated to Horizon Europe and in cash and in-kind contributions from public and private partners.

Built on the solid foundations laid by the European Joint Programme on Rare Diseases (EJP RD), ERDERA brings the community, the data and the resources together to accelerate prevention, diagnosis and treatment.

ERDERA



Hearing and sensory disorders

TREATDFNA9 advances antisense oligonucleotide therapy for DFNA9, a rare adult-onset autosomal dominant sensorineural hearing loss, by selectively targeting the mutant *COCH* transcript. The consortium will study molecular efficacy and pharmacokinetics in preclinical models, define therapeutic outcome measures and establish DFNA9 prevalence across Europe to support future clinical translation.

Bone, connective tissue and glycosylation disorders

PROOF repurposes RAVICTI (4-phenylbutyrate), an approved ammonia scavenger for urea cycle disorders, as a potential chaperone therapy for osteogenesis imperfecta. Using osteogenesis imperfecta mouse models and advanced human 2D and 3D cell culture systems, it will assess effects on collagen I secretion, cellular homeostasis and bone properties, and work with partners to accelerate translation towards a phase II clinical trial.

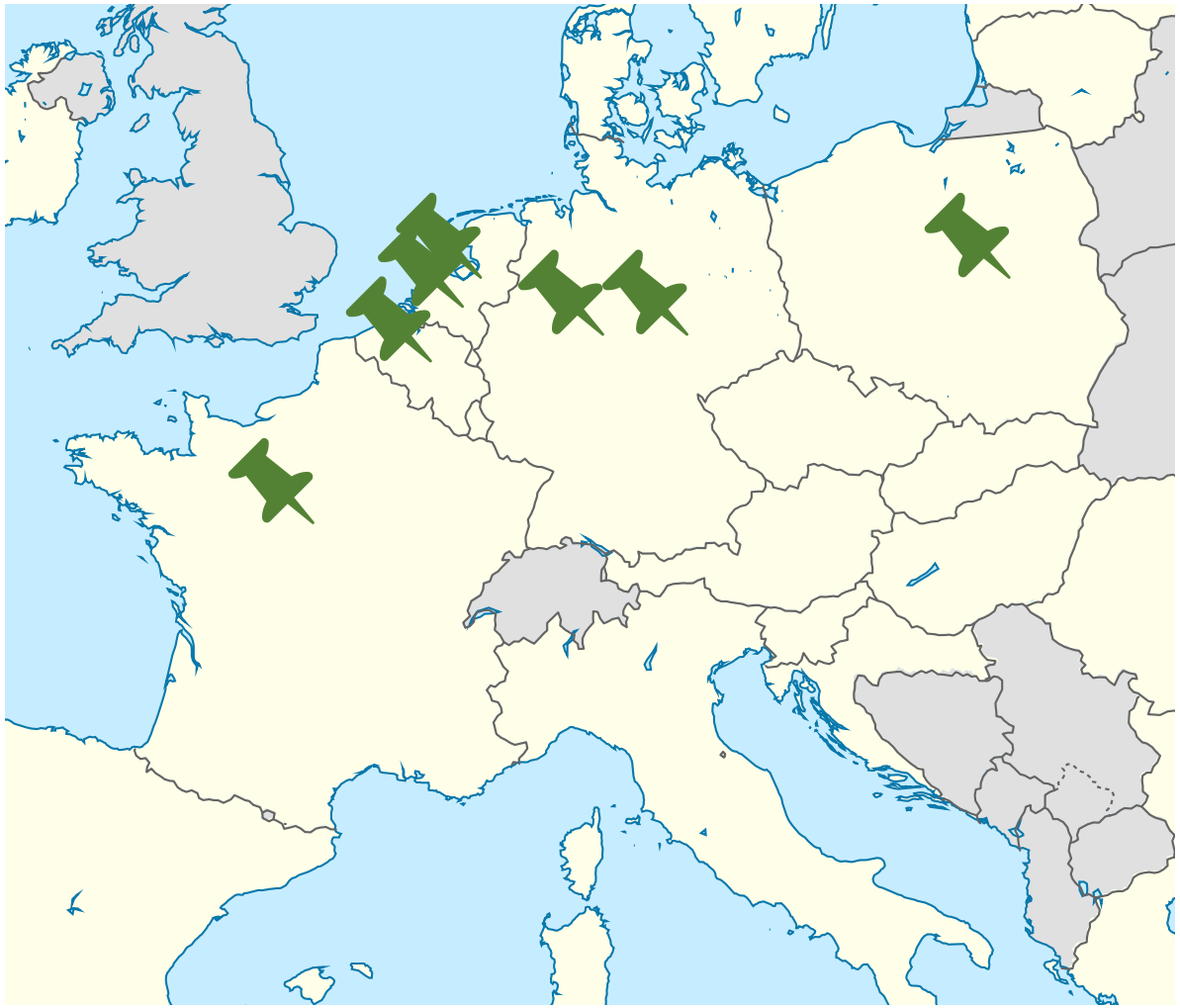
Kidney and liver diseases

ALP-RARE establishes a pre-clinical target validation and therapy development pipeline for Alport spectrum disorders, combining standard-of-care with candidate therapies such as 4-PBA, finerenone and A1M-based peptides. Multicentre pre-clinical randomised controlled trials, patient-derived kidney models and biomarker work will identify effective combinations and surrogate endpoints to support future human studies.

ASCENT-PSC builds an atlas and pre-clinical model platform for Primary Sclerosing Cholangitis, a rare liver disease currently without approved medical therapies other than transplantation. By integrating genomic, transcriptomic and proteomic data with precision-cut liver slices, human liver organoids and microbiome-modified mouse models, the consortium will identify and functionally test promising therapeutic targets.

Subsidie van
~ 1,6 miljoen euro

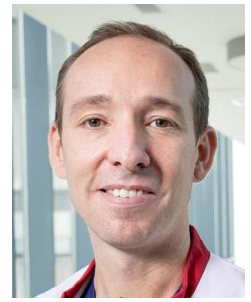
TREAT-DFNA9



Sydney Australia

Partners:

1) Stichting de Negende van…
2) Radboudumc (coordinator)
3) Universiteit Antwerpen (Vincent van Rompaey)
4) Institute Pasteur (Raphael Etournay)
5) Halle Universiteit (Eric Lehner)
6) Hearing Institute Warsaw (Monika Oldak)
7) Cochlear (Freddy Dueck)



ERDERA project doelen

Verder ontwikkelingen van de genetische pleister voor P51S DFNA9

- Activiteit van de genetische pleister gehumaniseerde muis “Elise”
- Lokalisatie en distributie in slakkenhuis van Cavia + afleveren medicijn met implantaten
- Verspreiding van DFNA9 door Europa (alle varianten/mutaties)
- Eerste stappen richting genetische pleisters voor andere varianten met behulp van “organoïden” (gekweekte binnenoorcellen uit menselijke stamcellen)

- ! Onderzoek omtrent wensen/eisen aan nieuwe medicijnen voor DFNA9 !
- ! Kennisdag 2028 (of 2029) gecombineerd met een wetenschappelijk symposium !

Wetenschappelijk programma

Volgend jaar uitgebreide update over therapeutisch onderzoek uit Nijmegen en Antwerpen – maar we zijn er wel voor vragen en snelle updates tussendoor!

Evi
Biomarkers/ perilymfe

Fien
DFNA9 muis, genetische pleister

Lize
DFNA9 muis, gentherapie

Wetenschappelijk programma

Oogproblematiek bij DFNA9

Dr. Gergely Lozonscy (Radboudumc, oogheelkunde)

In aanvulling:

Doel: opzetten onderzoek naar de cornea en oogleden van de DFNA9 muis, kweken van DFNA9 cornea organoïden uit stamcellen