

## THE CHALLENGE

Existing real-world data on Rett syndrome is fragmented across national initiatives, research studies and clinical centres, making it difficult to obtain a comprehensive view of the patient population and their needs.

- Fragmented datasets** across national initiatives
- Limited cross-border** collaboration in research
- Families disconnected** from research opportunities
- No European-wide** view of the patient population
- Inconsistent governance** and consent processes
- No shared infrastructure** to scale future research and trials

## KEY COMPONENTS

- Secure Digital Infrastructure**  
A platform allowing caregivers to register patients and provide core demographic and diagnostic information.
- Patient-Led Governance**  
Mechanisms led by patient organisations to ensure responsible data stewardship and transparency.
- Scalable for the Future**  
Architecture designed to support future research, surveys and collaborations with clinicians and researchers.
- Real-World Insights**  
Structured data ready to inform research, surveys and policy decisions, with full traceability and consent.

## WHAT IS RETTX?

A patient-driven digital infrastructure designed to collect and manage core information about individuals living with Rett syndrome across Europe, facilitating real-world data generation while maintaining strong governance principles aligned with GDPR and patient-organisation oversight.



### WHAT WE COLLECT

- Demographics & consent
- MECP2 variant
- Diagnostic info

## RETTX IN NUMBERS

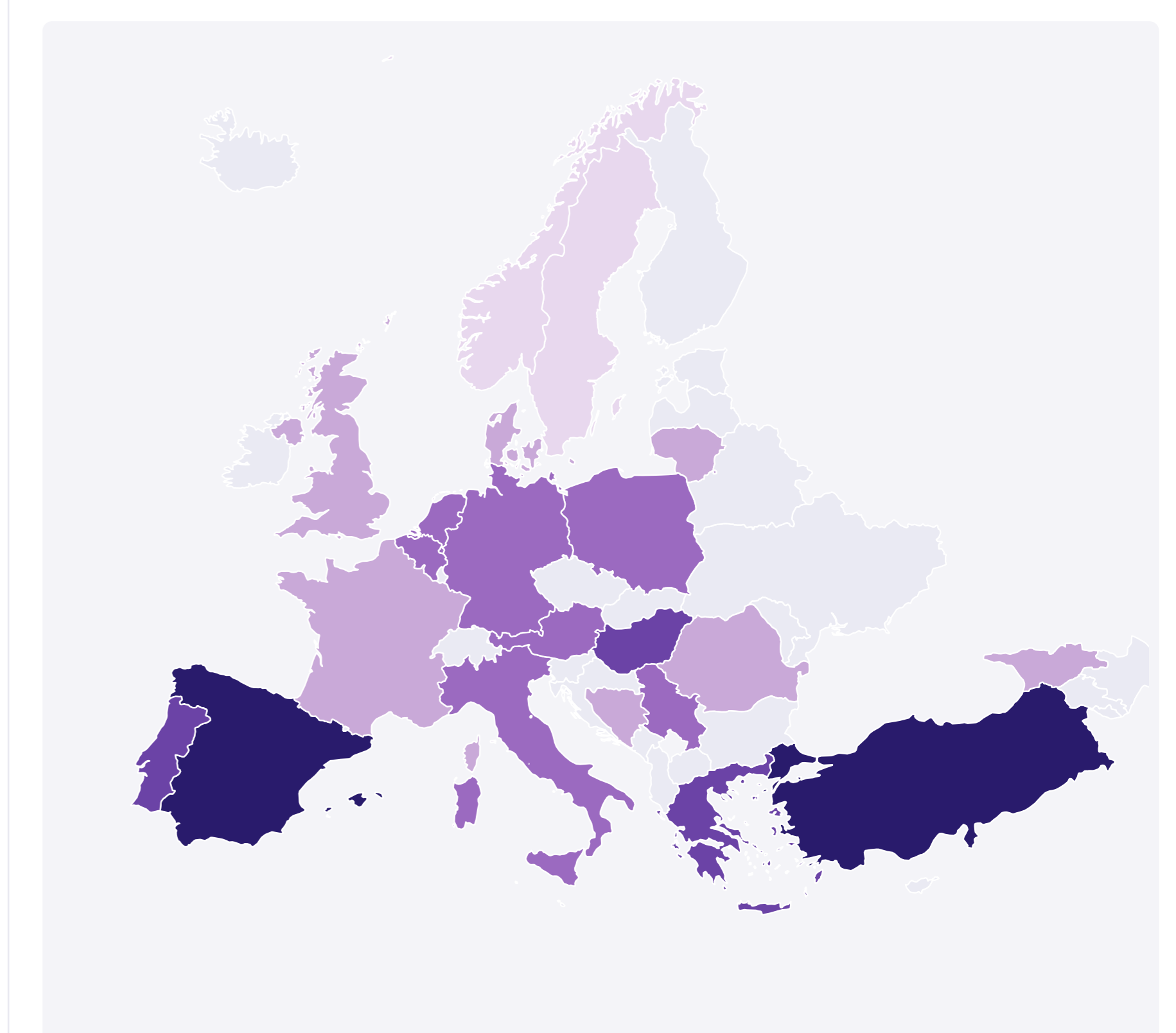
Live from the rettX registry · 15 May 2026

**511**  
patients enrolled

**159**  
distinct MECP2 variants

**20+**  
European countries contributing

## WHERE OUR COMMUNITY IS



50+ 21-50 11-20  
4-10 1-3 0

## PATIENT-LED GOVERNANCE

- Governed by **RSE and national patient organisations**
- Transparent decision-making** on data collection and access
- Ethical use of data**, aligned with the community's values
- GDPR-compliant**, privacy by design and pseudonymised at source
- Caregivers stay in control**. Consent can be reviewed or withdrawn at any time.
- No commercial exploitation**. Data access decisions are community-driven.

## EARLY IMPLEMENTATION

- Pilot live**  
with active families & patient organisations
- 20+ countries**  
contributing patient data across Europe
- Strong engagement**  
from family caregivers and clinical centres
- Feasibility**  
of a European-wide cohort demonstrated

## ROADMAP

- 2024 · Q4**  
First pilot in Spain
- April 2025**  
Official rettX launch
- 2026 · Q1**  
500 patients reached in rettX
- 2026 · Q4**  
Public dashboard & harmonised data dictionary live
- 2027**  
Harmonised European cohort, ready for research and trials

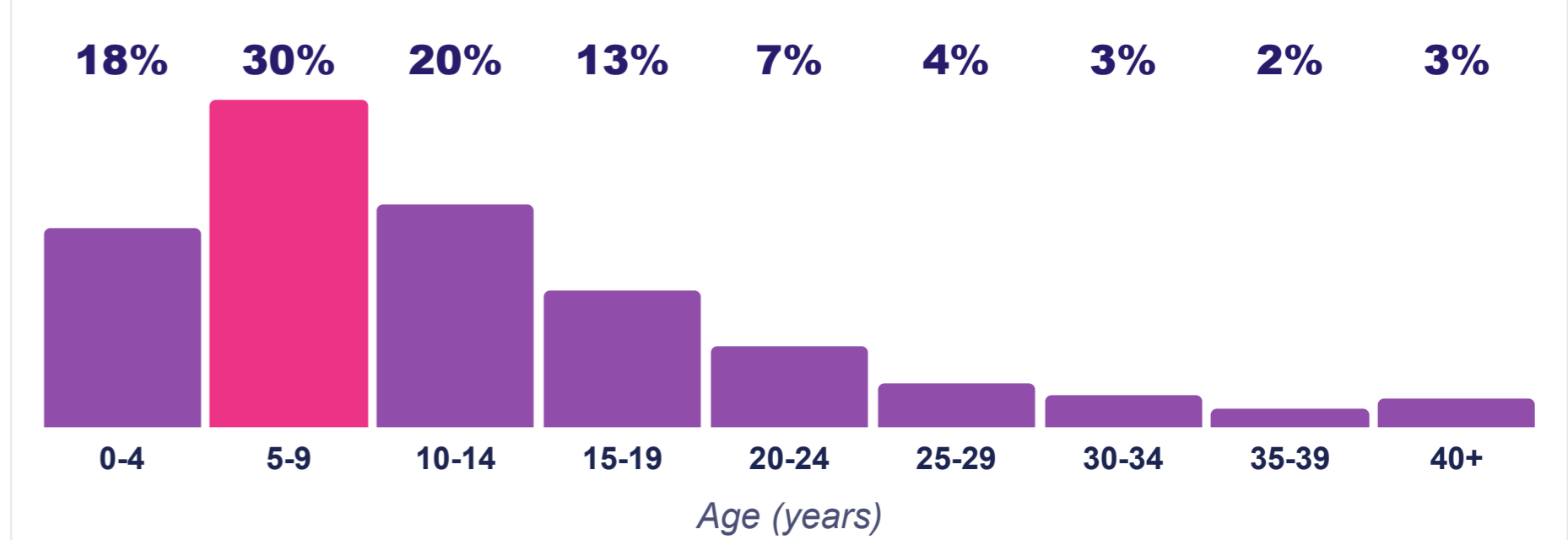
## GET INVOLVED

- For families & caregivers**  
Register your loved one at [rettX.eu](https://rettX.eu). Secure, anonymous, and you stay in control of your data.
- For patient organisations**  
Join the European network and bring your community into a shared, governed registry built for them.
- For clinicians & researchers**  
Request access to anonymised, structured real-world data through the patient-led governance committee.

### TOP MECP2 VARIANTS



### AGE DISTRIBUTION



## HOW DATA FLOWS THROUGH RETTX



## OUR VISION

- One harmonised European cohort**  
A single, GDPR-compliant dataset replacing 20+ fragmented national initiatives.
- Faster, more equitable research**  
Lower barriers for academic and clinical research across every European country.
- Connected families, clinicians & researchers**  
A transparent, patient-led pipeline from lived experience to research outputs.
- A foundation for future therapies**  
A research-ready cohort to support clinical trials, surveys and post-approval evidence.

## FROM THE COMMUNITY

rettX is built **by the community, for the community**. Together, families, patient organisations, clinicians and researchers are building the foundation for a better future for everyone living with Rett syndrome.

Rett Syndrome Europe and national patient organisations

MEET US AT ECRD 2026

Poster #81 · come and discuss how rettX can support your community.

Explore the live data dashboard



rettX.eu

- Live patient counts
- Geographic distribution
- Variant & age breakdowns