rse
rett syndrome europe



Annual report 2025–26

11 October 2025 – 8 November 2026

WHO WE ARE

Rett Syndrome Europe (RSE) is the European federation of national Rett syndrome associations. Founded in 1994 and registered in Luxembourg as a non-profit association (asbl), it brings together associations, families, professionals and partners around one purpose: improving the lives of people with Rett syndrome and strengthening the voice of our community across Europe.

Rett syndrome is a rare neurodevelopmental disorder that mostly affects girls and is usually caused by a change in the *MECP2* gene. It affects movement, communication and many other parts of daily life, and families need coordinated care throughout life.

Our five aims

Article 5 of our statutes sets five aims. They also give this report its structure.

1. **Make Rett syndrome better known**
2. **Improve communication** within the European Rett community
3. **Represent** people with Rett syndrome and their families in Europe
4. **Expand RSE** to all European countries
5. **Promote research**



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Pedro Rocha
President, Rett Syndrome Europe

FOREWORD

From Istanbul to Madrid

Dear friends,

It has been a busy year for Rett Syndrome Europe, with encouraging developments, new initiatives and many opportunities to work together.

One of the most significant developments was the European authorisation of Daybue, the first medicine approved in the EU specifically for Rett syndrome. It followed a difficult regulatory process, closely followed by families and associations across Europe, together with RSE and IRSF. This is an important step, although there is still work ahead to ensure that families across Europe can actually access the treatment.

Our European registry, rettX, has also continued to grow. From 351 registered patients when we met in Istanbul last October, we have reached 572 patients from 49 countries. Behind these numbers are families who have placed their trust in this initiative, contributing to a better understanding of Rett syndrome and, hopefully, to better research in the future.

Throughout the year, we have worked with our member associations and partners on several initiatives. Together, we contributed to a white paper on coordinated care pathways, presented in Brussels, shared our work at ECRD in Prague, and contributed to the charter of the Rett Syndrome World Leaders Forum. We were also pleased to welcome Romania as a new member.

In November, Rett Forum 2026 in Madrid brought people together once again. With over 100 participants from 24 countries, the Forum reflected something we strongly believe in: the importance of connecting families, clinicians, researchers and industry, sharing knowledge and finding ways to improve care for people living with Rett syndrome.

Much of this work happens thanks to volunteers. Our member associations, board members, advisors and many others contribute their time, experience and energy, often alongside their professional and family responsibilities. I want to thank everyone who has helped make this year possible.

Looking ahead, we have plenty to work on: continuing to develop rettX and preparing its first analysis, shaping our Strategic Plan 2027+, and strengthening the connections between our member associations and the wider Rett community.

We may not always move as quickly as we would like, but we are moving forward, and we are doing it together.

Thank you for being part of it.

Pedro Rocha

GOVERNANCE

The Executive Board

RSE is run by an Executive Board of seven volunteers, elected by the General Assembly.



Pedro Rocha
President
Spain



Burak Temiz
Treasurer
Türkiye



Gvantsa Kvantaliani
Board member
Georgia



Bojana Milanov
Board member
Serbia



Mariëlle van den Berg
Board member
Netherlands



Rob Adamek
Board member
United Kingdom



Sergey Dmitriev
Board member
Greece

Scientific Advisory Board

The Scientific Advisory Board (SAB) was relaunched in 2026. Around fifteen clinicians and researchers from across Europe advise the Executive Board, and they led the six working groups of Rett Forum 2026. The SAB has met regularly since its relaunch.

Legal details

Rett Syndrome Europe asbl, a non-profit association under Luxembourg law, registered with the Luxembourg Business Registers (RCS) under F1819. Founded in 1994. Its statutes are published on the Transparency page of rettsyndrome.eu.

OUR MEMBERS

National associations across Europe

RSE's members are national Rett syndrome associations. Together they speak for families in every corner of Europe.

32	37	1	6
countries	member associations	new member: Romania	more countries with individual contributors

AUSTRIA
Österreichische Rett-Syndrom Gesellschaft

BELGIUM
Belgische Rett Syndroom Vereniging

CROATIA
Tihi Anđeli

CYPRUS
Rett syndrome Κύπρος/Kıbrıs

CZECHIA
Spolek rodičů a přátel dívek s Rettovým syndromem

DENMARK
Landsforeningen Rett-syndrom

FINLAND
Rett Finland

FRANCE
AFSR

GEORGIA
Georgian Rett syndrome and other rare diseases Association

GERMANY
RETT Deutschland e.V.
Rett-Syndrom e.V. Deutsche Rett-Syndrom Gemeinschaft

GREECE
Reverse Rett Greece
ΑΓΓΕΛΟΙ ΓΗΣ

HUNGARY
Magyar Rett Szindróma Alapítvány

ICELAND
Rett Syndrome Rannsóknarsjóður Guðrúnar

IRELAND
Rett Syndrome Association of Ireland

ITALY
A.I.Rett/AIRETT

LITHUANIA
Agotos labdaros ir paramos fondas

LUXEMBOURG
ALSR

MALTA
Rett Syndrome Malta

MONTENEGRO
Ret sindrom Crna Gora

NETHERLANDS
Nederlandse Rett Syndroom Vereniging

NORTH MACEDONIA
Rett Sindrom Makedonija

NORWAY
Norsk Forening for Rett Syndrom

POLAND
Ogólnopolskie Stowarzyszenie Pomocy Osobom z Zespołem Retta

PORTUGAL
ANPAR

ROMANIA
New member in 2025–26

SERBIA
Ret udruzenje Srbije

SLOVAKIA
Rettovým Syndrómom Slovensko

SPAIN
Asociación Española de Síndrome de Rett
Asociació Catalana de la Síndrome de Rett
Asociación Princesa Rett

SWEDEN
RSIS

SWITZERLAND
Rett Verein Schweiz

TÜRKIYE
Rett Türkiye

UNITED KINGDOM
Rett UK
Reverse Rett

RSE also has individual contributors in Armenia, Bosnia and Herzegovina, Latvia, Russia, Slovenia and Ukraine. The list reflects the member directory on rettsyndrome.eu on 9 November 2026.

2025–26

The year in numbers

572
patients in rettX, the European registry, on 9 November 2026

+63%
growth of rettX during the year, from 351 patients

49
countries where rettX patients live

1st
medicine for Rett syndrome authorised in the EU (Daybue, 24 August 2026)

2
posters at ECRD in Prague, Europe's main rare disease conference

104
Rett Forum 2026 participants, from 24 countries

56
experts in the Rett Forum, in six working groups led by the SAB

3
websites rebuilt: rettsyndrome.eu, rettx.eu and rettforum.eu

And one new member association: Romania.

INTRODUCTION

A year of firsts

Over the past year, Rett Syndrome Europe has continued to bring together national Rett syndrome associations, families, professionals and partners around a shared purpose: improving the lives of people with Rett syndrome and strengthening the voice of our community across Europe.

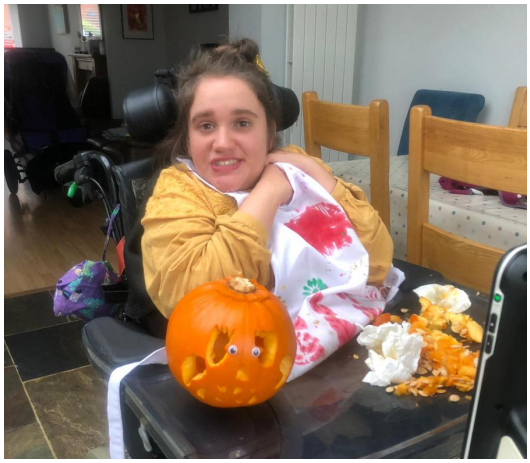
Our work is guided by the five aims set out in Article 5 of our statutes, which also give this report its structure. Connecting our member associations and growing our network go hand in hand, so we report on aims 2 and 4 together. The report closes with RSE's governance, its finances and our priorities for 2027.

The report covers the year from the General Assembly in Istanbul in October 2025 to Rett Forum 2026 in Madrid in November 2026.

The year at a glance

Oct 2025	General Assembly in Istanbul; the reporting year begins
Dec 2025	White paper on coordinated care pathways; Rett Syndrome Policy Forum in Brussels; Medscape recording
Apr 2026	ATMP talk at EURORDIS; rettX poster at the EuroNDD workshop, Warsaw; Forum sponsorship tiers
Jun 2026	Two posters at ECRD, Prague; positive CHMP opinion on Daybue; Forum venue and dates agreed
Aug 2026	European Commission authorises Daybue
Oct 2026	Rett Awareness Month: pRETTy awesome comes to Europe
Nov 2025	rettX presented to the IQSEC2 community
Feb 2026	Negative first CHMP opinion on Daybue; *Rare Revolution* Rare Disease Day edition
May 2026	Family perspective at SENEP, the Spanish Paediatric Neurology Society
Jul 2026	Member Network launched for member associations
Sep 2026	WoLF charter adopted; caregiver study accepted for publication; Forum registration opens
Nov 2026	Rett Forum 2026 and the General Assembly in Madrid








Raising awareness

The first aim of RSE is to make Rett syndrome better known: among clinicians, decision makers, the rare disease community and the public.

European Conference on Rare Diseases

RSE presented two posters at EURORDIS's European Conference on Rare Diseases (ECRD) in Prague on 3–4 June 2026, the most important rare disease conference in Europe. One presented the rettX registry (poster 81, with a registry snapshot at 15 May 2026); the other presented the European caregiver study of the burden of Rett syndrome (poster 240). Both are shown on the next page.

Spanish Paediatric Neurology Society

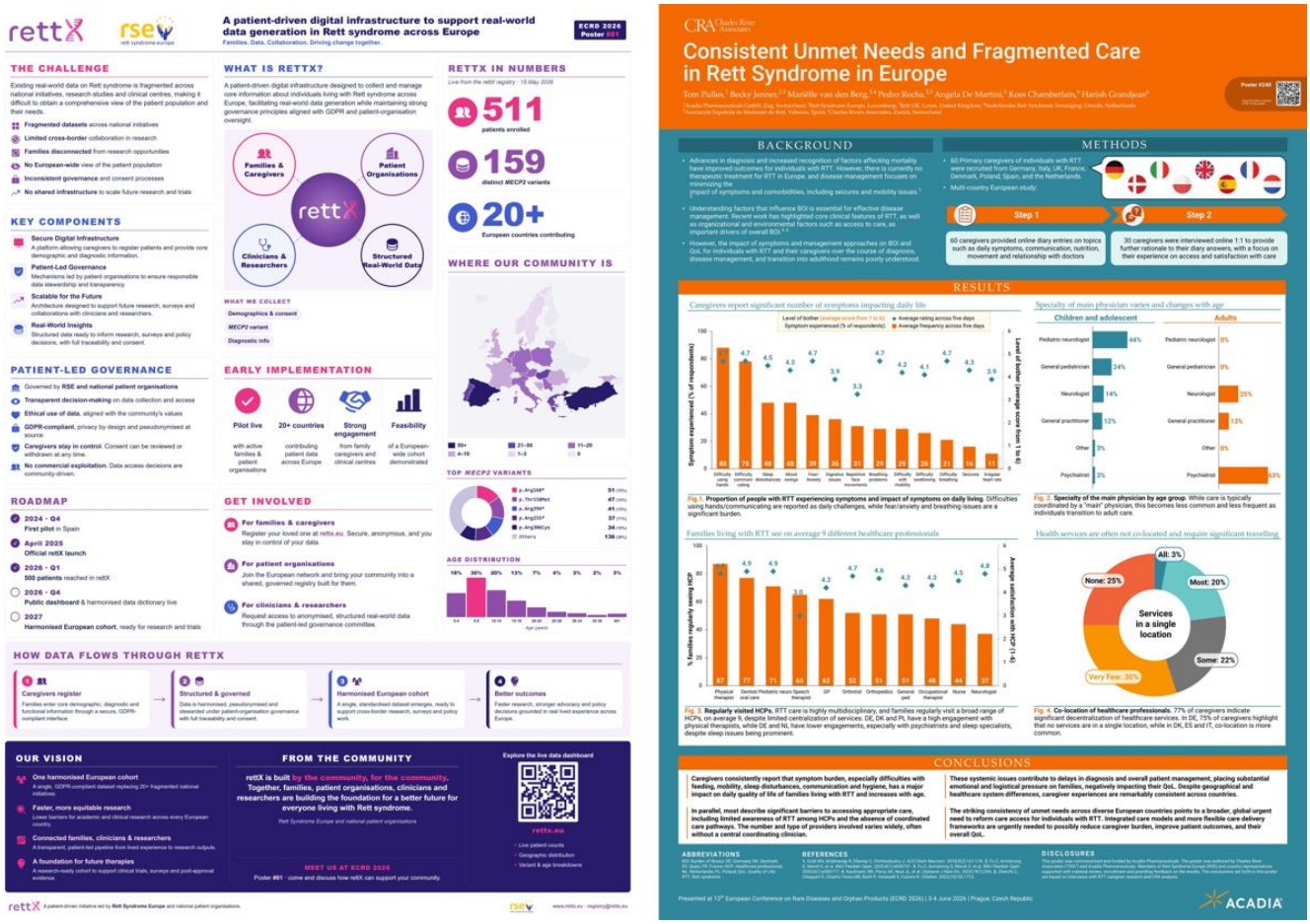
In May 2026 RSE took part in a Rett syndrome awareness round table of the Spanish Paediatric Neurology Society (SENEP). We presented the patient and family perspective, the resources RSE offers, the rettX registry and the Rett Forum to Spanish paediatric neurologists.

Media

Rett syndrome featured in three media pieces during the year: a Medscape education recording with clinicians (December 2025), *Rare Revolution* magazine for Rare Disease Day (February 2026) and a RARE recording (June 2026).

Websites

RSE rebuilt its three websites: rettsyndrome.eu, the home of the federation; rettx.eu, the registry; and rettforum.eu, the Forum.



Posters presented at ECRD, Prague, 3–4 June 2026: rettX (left, poster 81; registry snapshot at 15 May 2026) and the European caregiver study (right, poster 240).

Rett Awareness Month: pRETTy awesome comes to Europe

For Rett Awareness Month in October 2026, RSE ran the first European edition of **pRETTy awesome**, with Acadia's support. The campaign began with parents in Canada. RSE offered member associations pins, stickers, small cards and postcards in six languages (English, Spanish, French, Italian, German and Greek). The postcards' QR codes lead to RSE's member page and to RettLife.eu.

Six associations in four countries took part: two in Germany, two in Italy, one in Greece and one in Spain. The materials were also offered to participants of Rett Forum 2026.



Connecting and growing the European Rett community

Aims 2 and 4 go hand in hand: improving communication within the European Rett community, and expanding RSE to every European country.

A new member

Romania joined RSE during the year.

Member Network

In July 2026 we launched the RSE Member Network: a WhatsApp channel for member associations, with a sign-up form, to improve communication and to connect associations directly with each other and with the board.

Our channels are open

The board held direct calls with member associations during the year, and is happy to hold a dedicated call with any member association that asks for one.

9th European Rett Syndrome Summit

RSE supports the 9th European Rett Syndrome Summit, organised by one of its member associations in Berlin in 2027. RSE has proposed a members' meeting during the Summit.

Rett Forum 2026

The largest single piece of work this year was Rett Forum 2026 in Madrid. It is reported in its own section on the next two pages.



Rett Forum 2026

Rett Forum 2026 (Madrid, 6–8 November, by invitation) brought experts and national associations together to work on European standards of care for Rett syndrome.

The Forum was an in-person meeting. The Scientific Advisory Board was invited first, followed by experts within the budget. RSE held several online "ask me anything" sessions to share information about the Forum with members.

RETT FORUM
EUROPEAN EDITION 2026

Official Rett Forum 2026 artwork.

How we prepared

When (2026)	Step
Early 2026	Online workspace for the Forum set up
March	Introductory calls
April	Expert nominations and sponsorship outreach; sponsorship tiers agreed, so that no single company is the majority funder
June	Venue and dates agreed: Madrid, 6–8 November; "ask me anything" sessions for members begin
July	A dedicated space for the Scientific Advisory Board opened
September	Registration opened

RETT FORUM 2026

Who came to Madrid

104
participants

24
countries: most from Europe, plus the USA, Canada, Israel and Australia

44
participants whose travel RSE supported

Group	Participants	Who
Experts	**56**	Clinicians and researchers, including 7 members of the Scientific Advisory Board
Patient representatives	**30**	Caregivers (23), RSE board members (6) and a translator
Industry	**16**	From 7 companies
Organisers	**2**	Project team

Six working groups

The Scientific Advisory Board leads the Forum's six working groups, with 56 experts taking part. The groups are developing European standards of care for Rett syndrome.

Made possible together

The Forum was co-funded by RSE's partners and by member associations, some of which also covered the travel of experts from their country. They are thanked on page 19.

Three days in Madrid

Rett Forum 2026 took place in Madrid from 6 to 8 November. Participants included the Vice-Chair of the EMA's Committee for Advanced Therapies and representatives of the ITHACA European Reference Network.









Representing people with Rett syndrome and their families



White paper and the Rett Syndrome Policy Forum

RSE representatives and member-association leaders co-authored *Building coordinated care pathways for Rett syndrome: a call to action in Europe*, published in December 2025. It was launched at the Rett Syndrome Policy Forum in Brussels on 3–4 December 2025, where RSE's President spoke, and was then presented to the European Parliament. Members were asked to share it in their countries.

Daybue (trofinetide): the first medicine for Rett syndrome in the EU

After a negative first opinion, the medicine was re-examined, and the European Commission authorised it on 24 August 2026. RSE informed members at each step and coordinated with the International Rett Syndrome Foundation (IRSF).

Date	What happened	What RSE did
February 2026	Negative first opinion of the EMA's medicines committee (CHMP); re-examination requested	Informed members (statement of 4 March)
May 2026	Re-examination under way	Coordinated with IRSF on its letter to the CHMP and the EMA
26 June 2026	Positive CHMP opinion after re-examination	Joined the EU update call and informed members
24 August 2026	European Commission authorisation	Published a statement

European Medicines Agency (EMA)

RSE built a working relationship with the EMA's patient-relations team in February 2026, and sought advice on gene therapy from the EMA's Committee for Advanced Therapies (CAT). The Vice-Chair of the CAT took part in Rett Forum 2026.

RSE aims to become an EMA eligible organisation. This status would allow RSE to take part in the EMA's work on behalf of people with Rett syndrome and their families, and RSE is preparing its application.

Gene and cell therapies

In April 2026 RSE presented *ATMP development through the patient lens* to EURORDIS. ATMPs (advanced therapy medicinal products) are gene, cell and tissue-based medicines. The talk showed how the Rett community has prepared for them, with the rettX registry, natural history data and scientific advisers, and noted that more than 25 companies now have active Rett programmes. It called on Europe to connect science, registries and trial readiness so that these therapies reach patients sooner.

Rett syndrome World Leaders Forum (WoLF)

RSE's President sits on the six-person committee that drafted the charter of WoLF. Associations worldwide adopted the charter unanimously in September 2026, and WoLF was announced on 29 September as a global alliance from 2027. RSE will sit on its Executive Committee.

24 August 2026: the first medicine for Rett syndrome is authorised in the EU.





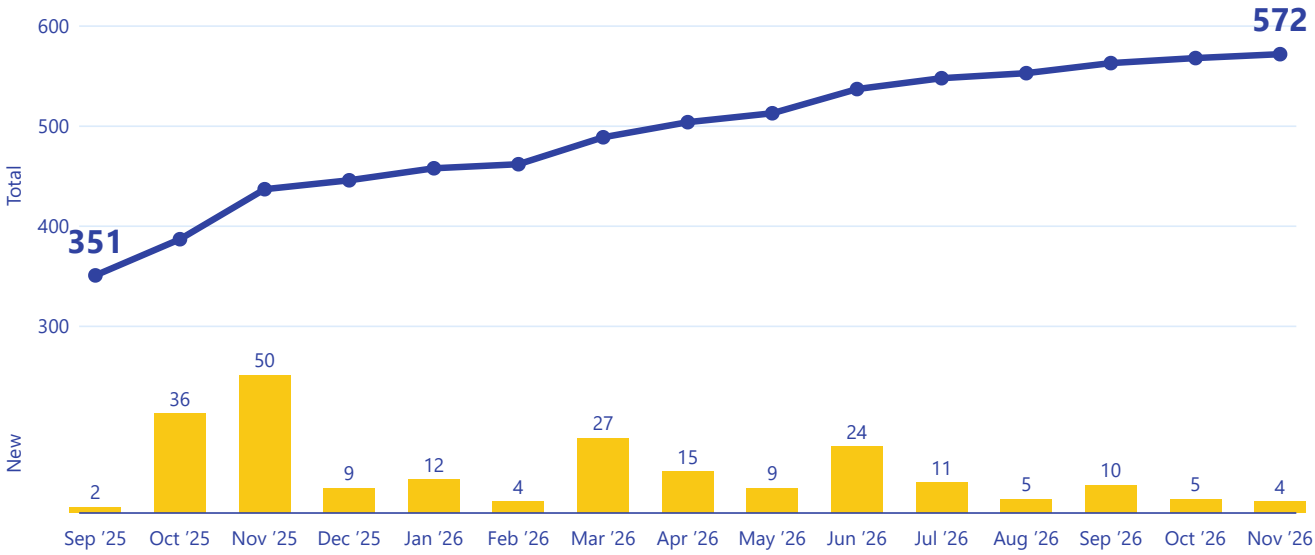


Supporting research

rettX, the European Rett syndrome registry, is at the centre of RSE's work for research. Every registration adds to the evidence that researchers, clinicians and companies need.

rettX grew by 63%

rettX grew from 351 registered patients at the end of September 2025 to 563 at the end of September 2026, and to 572 on 9 November 2026: 221 more patients, or +63%. October and November 2025 alone brought 41% of the growth in the twelve months to September 2026.



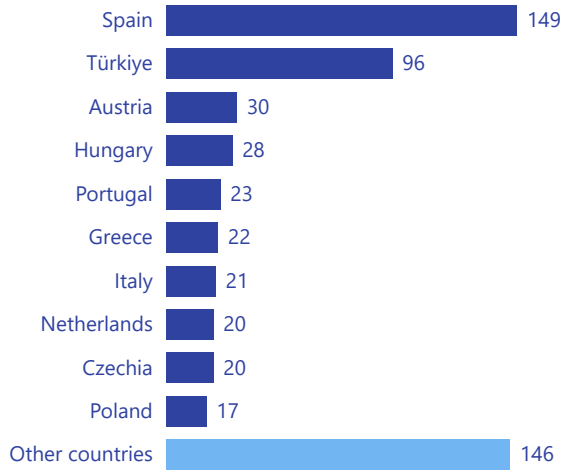
Top: registered patients at month end, November to 9 November (axis starts at 300). Bottom: new registrations per month; November 2026 to date. Source: rettX dashboard, 9 November 2026.

SUPPORTING RESEARCH

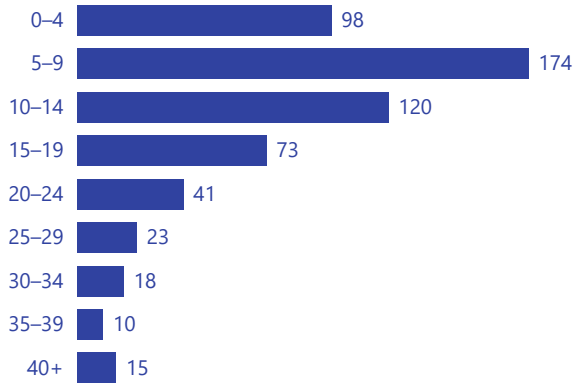
Who is in rettX

On 9 November 2026, rettX held 572 patients, most of them living in Europe.

Patients by country



Patients by age (years)



- **Countries.** Spain (149) and Türkiye (96) together account for 42,8% of patients, followed by Austria, Hungary, Portugal and Greece.
- **Ages.** 47,6% of patients are under 10. 107 (18,7%) are aged 20 or over, including 15 aged 40 or over: almost one in five is an adult.
- **Genetics.** Mutation data are recorded for 491 patients (86%). The most frequent *MECP2* variants are p.(T158M), p.(R168*), p.(R294*) and p.(R255*).

Profile at 9 November 2026. Source: rettx.eu/data, 9 November 2026.

rettX development

The registry gained passwordless login and privacy-preserving identifiers. A "pulse" tracker for medication, seizures and sleep is in trial, and a mobile app is in testing. Our ambition is a federated model, with rettX as the European hub; it is part of the Strategic Plan from 2027.

SUPPORTING RESEARCH

Sharing what we learn

Sharing registry experience

RSE presented rettX to other rare disease communities: at the international meeting of the IQSEC2 community (a related rare neurodevelopmental disorder) in November 2025, at the EuroNDD workshop in Warsaw on 9–10 April 2026, where a poster on building a patient registry was presented with an academic partner, and at the European Conference on Rare Diseases in Prague in June 2026.

Caregiver study

RSE representatives co-authored a multi-country European caregiver study, *Consistent unmet needs and fragmented care in Rett syndrome: insights from a multi-country European caregiver study*. The Orphanet Journal of Rare Diseases accepted it for publication on 30 September 2026. Its findings support the case for coordinated care made in the white paper.

Scientific Advisory Board and the Forum working groups

The Scientific Advisory Board, relaunched in 2026, has met regularly and was central to organising Rett Forum 2026. It leads the Forum's six working groups, with 56 experts taking part, which are drafting European standards of care.







GOVERNANCE AND ORGANISATION

How RSE worked this year

- **Boards.** The Executive Board met regularly, usually monthly. The Scientific Advisory Board was relaunched in 2026 and has met regularly since.
- **Statutes.** After Istanbul, the updated statutes and the new registered address were filed. The statutes are published on the Transparency page of rettsyndrome.eu.
- **Strategic Plan 2027+.** Started at members' request and presented to members in November 2026.
- **Fees and accounts.** The 2026 membership fees have been requested. The 2025 accounts are summarised on page 20.

WHO WE WORK WITH

EURORDIS – Rare Diseases Europe

RSE is a member. Two posters at ECRD in Prague and the ATMP talk.

International Rett Syndrome Foundation (IRSF)

Coordination on Daybue and on the global Rett agenda.

Rett syndrome World Leaders Forum (WoLF)

RSE helped draft the charter and will sit on the Executive Committee from 2027.

European Medicines Agency (EMA)

Patient relations and advice from the CAT; RSE aims to become an EMA eligible organisation.

ERDERA

RSE's application to this EU-funded rare disease research alliance's networking scheme, for Rett Forum 2027, was declared eligible but was not successful.



Thank you

Our partners

RSE thanks its main partners **Acadia, Anavex, Lundbeck, Neurogene, Taysha and UCB Pharma**, the sponsors of Rett Forum 2026. We also thank **Reverta**, a partner of the Forum.

Our member associations

We thank the member associations co-funding the Rett Forum: Rett Syndrom Deutschland (which has also pledged for 2027), Rett Spain, ConRett, the Danish association, Mi Princesa Rett, the Catalan association and AIRETT (Italy), and Rett UK and the Netherlands association, which covered experts' travel.

Volunteers and advisers

We thank the members of the Scientific Advisory Board, the experts of the Forum working groups and every volunteer who gave their time this year.

Above all, RSE thanks the families who share their data through rettX.

FINANCIAL REPORT

Our finances in 2025

RSE's accounts for the 2025 calendar year, as signed by the Treasurer and checked by the internal auditor. All amounts in euro (EUR).

Income 2025	EUR
Donations: Acadia	20.000,00
Donations: Microsoft (employee giving)	14.781,66
Donations: Taysha	1.200,00
Donations: private	50,00
Membership fees	5.435,00
Wise cashback	1,14
Total income	**41.467,80**

+6.797,05
result for the year

Expenses 2025	EUR
Events	25.460,06
Reimbursements	5.634,41
Lawyer, Luxembourg	1.755,00
IT	1.494,03
Association costs, Luxembourg	174,19
EURORDIS membership	100,00
Bank fees	53,06
Total expenses	**34.670,75**

Bank balance	EUR
1 January 2025	33.022,85
31 December 2025	**39.819,90**

Where our funding came from

Source	Type	EUR	Share of income
Acadia	Industry	20.000,00	48,2%
Taysha	Industry	1.200,00	2,9%
Microsoft (employee giving)	Independent	14.781,66	35,6%
Membership fees	Independent	5.435,00	13,1%
Private donations	Independent	50,00	0,1%
Wise cashback	Independent	1,14	0,0%
Industry		**21.200,00**	**51,1%**
Independent		**20.267,80**	**48,9%**

Industry means pharmaceutical and biotechnology companies. In 2025 industry funding came from two companies; the largest single funder provided 48,2% of income. The 2025 accounts were signed by the Treasurer, Burak Temiz, and checked by the internal auditor (Treasurer of Rett Türkiye).

RECENTLY

Autumn 2026

- **Rett Forum 2026** took place in Madrid from 6 to 8 November, with 104 participants from 24 countries.
- **General Assembly.** Member associations met in Madrid and online on 7 November.
- **EMA.** RSE is preparing its application to become an EMA eligible organisation.
- **ERDERA.** RSE's application for Rett Forum 2027 was not successful.
- **Daybue.** After the EU authorisation, access is now decided country by country.

2027

Looking ahead

In 2027, we plan to:

- grow rettX registrations and publish a first analysis of registry data;
- deliver the Strategic Plan 2027+ and hold an extraordinary General Assembly on the statutes;
- hold Rett Forum 2027 at the same scale as Madrid;
- apply for EMA eligible-organisation status;
- support the 9th European Rett Syndrome Summit in Berlin (4–6 March 2027) and take RSE's seat on the WoLF Executive Committee.









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rettsyndrome.eu · rettx.eu · rettforum.eu

Rett Syndrome Europe asbl · Luxembourg · RCS F1819

Photos: families of the Rett community, published on rettsyndrome.eu.