

Beehive Mill  
Jersey Street  
Ancoats  
Manchester M4 6JG

+44 161 552 5001  
[enquiries@reverserett.org.uk](mailto:enquiries@reverserett.org.uk)

[reverserett.org.uk](https://reverserett.org.uk)

## **Research Strategy**

### **Our mission**

Reverse Rett is a UK patient advocacy and research organisation focused on delivering treatments and a cure for Rett syndrome.

### **Our Vision**

Our vision is a world where girls and boys with Rett syndrome are diagnosed much quicker and earlier than they are right now.

A world where we know much more about the progression of the condition and the factors that affect that progression.

A world where at every stage of development, treatments are available to mitigate against the distressing symptoms which can occur.

A world where we ultimately deliver a cure and enable people with Rett syndrome to lead free and healthy lives.

## **Our Principles**

- **EQUALITY**

All patients with Rett syndrome regardless of age or gender should have access to emerging treatments and cures.

- **COMPASSION**

Beyond treatments and a cure, all patients with Rett syndrome are likely to require some level of holistic medical management, the extent of which will be based on whether they have been able to access and/or benefit from the treatments/cure or not and the age they were and condition they were in when this became available.

- **COLLABORATION**

We believe in collaboration. Delivering treatments and a cure for Rett syndrome is a big job and it needs all of us to bring about real change everywhere.

- **DIVERSITY**

We value diversity. We want to ensure that no one is marginalised as we move together towards our common goal.

- **SCIENCE**

We are full members of the Association of Medical Research Charities and abide by all the rules therein associated.

Reverse Rett exists to deliver treatments and a cure for Rett syndrome.

We fund international Rett syndrome research through our longstanding partnership with the US based Rett Syndrome Research Trust (RSRT) who are responsible for peer review and monitoring of those projects.

Our UK work has a translational impact with clear patient benefits. We work to ensure that as treatments and a cure for Rett syndrome become viable, they are accessible to patients in the UK.

## **What is Rett syndrome?**

Rett syndrome is a post-natal neurological condition which strikes at random in early childhood, affecting little girls almost exclusively. Many children live into adulthood, requiring total, 24- hour-a-day care.

There is no treatment beyond supportive, and often ineffective, measures such as feeding tubes, bracing, orthopaedic and GI surgeries, and medications for anxiety and seizures.

First recognized only 25 years ago, the prevalence of Rett syndrome equals that of Cystic Fibrosis, Huntington's and ALS but is vastly underfunded in comparison to those disorders.

## **What causes Rett Syndrome?**

Rett syndrome is most often caused by mutations in a gene called MECP2, on the X chromosome.

This gene makes a protein, also called MeCP2 (but written differently), which is necessary for normal brain function. We know from [the reversal experiments of 2007](#), that when this protein is replaced at adequate levels in mice, the symptoms of Rett syndrome go away.

It wasn't until 2013, that researchers found a tangible way to translate those original reversal experiments into something that could potentially work in a human with Rett Syndrome; [gene therapy](#).

In 2021, we are moving into a new era of treatment for Rett syndrome with several gene therapies about to move into human clinical trials.

## **What we do:**

- We advocate for and fund improved clinical treatment for children and adults with Rett syndrome and related disorders.
- We fund laboratory and clinical research projects focused on improving their lives.
- We are custodians of the Rett Registry UK
- We facilitate the implementation of UK and European clinical trials.
- We collate relevant up to date health and research-based best practice information and make it accessible for anyone supporting someone with Rett syndrome.
- We collaborate with other like-minded Rett syndrome and rare disease organisations around the world. It just makes sense.

## **International laboratory research funding**

Reverse Rett works in collaboration with the US based Rett Syndrome Research Trust (RSRT) and other organisations internationally, to compound a global effort to accelerate treatments and a cure for Rett Syndrome and MECP2 Duplication Syndrome.

Projects which are financially supported by Reverse Rett via the Rett Syndrome Research Trust (RSRT) US, are not peer reviewed by Reverse Rett as they are peer reviewed and monitored through the RSRT peer review process.

Reverse Rett contributions to the Rett program at RSRT are made to the Cobb Lab and the Bird Lab at the University of Edinburgh via a UK based RSRT bank account.

Reverse Rett works with RSRT to ensure that animal welfare and consideration of the 3Rs is included throughout grant application review.

## **UK Clinical research funding**

Within the UK, we aim to use funds deployed nationally in ways that will have the maximum practical and positive effect on the lives of patients with Rett syndrome in the UK, with particular regards to their ability to access emerging treatments.

All new applications include the question, *'do you use animals in your research?'*

New projects will be assessed against our project selection criteria to ensure we focus on activities that make the best use of our funds.

All new projects must fall within the scope of our purpose and must pass a stage 1 assessment before progressing to stage 2.

## **Project selection criteria**

### **Stage 1 Assessment (Gateway questions)**

1. What is the specific unmet clinical need being targeted?
2. How does the project fulfil this need?
3. Is the research underpinned by scientific excellence?

### **Stage 2 Assessment**

1. How will the research help patients?
2. Has the right team been identified?
3. Why is this research needed now?

## **Our Research Review Committee**

Our Research Review Committee (RRC) comprises a mix of industrial, academic and clinical experts as well as lay people.

The RRC is responsible for assessing applications and making recommendations to the Reverse Rett Board of Trustees, ensuring that UK research funds are spent wisely.

The RRC has a dedicated Animal Welfare Officer who will work with external peer reviewers and members of the committee on how to consider specific aspects of the 3Rs when reviewing grant applications.

One member of the Board of Trustees of Reverse Rett sits on the RRC but is a non-voting member.

The RRC is asked to provide feedback and recommendations to the Trustees of the charity regarding:

- Development of the charity's research strategy.
- External referees for applications.
- Assessment of and final recommendations on applications.
- Reporting and dissemination of research result.
- Consideration of the use of animals in research.
- Linking to the national centre for the three Rs where appropriate.

Reverse Rett is committed to impartiality. The RRC comprises a significant number of experts who are not in receipt of charity research funding.

The RRC has a Conflict of Interest Policy and those with a conflict are not in a position to influence funding decisions.

## **Criteria for funding UK research**

Reverse Rett will fund research which supports the basic principles enumerated in this document.

Reverse Rett does not invite applications on a rotational basis.

