

About RSRT (The Rett Syndrome Research Trust)

关于 RSRT 组织 (可信赖的雷特综合症研究组织)

The stakes are high. Our efforts aim higher.

风险很高，但我们努力的目标更高。

RSRT was launched in 2008 to drive research toward a cure for Rett Syndrome and related MECP2 disorders. Our mission is urgent: 15,000 girls and women in the United States and 350,000 globally live with the agonizing symptoms of Rett Syndrome.

RSRT 组织创办于 2008 年，主旨在推动雷特综合症及 MECP2 相关疾病的研究。我们的使命相当紧迫：在美国有 1.5 万，在全球有 35 万饱受雷特综合症病痛折磨的女性患者。

RSRT funds more research than any other Rett organization in the U.S. and abroad. Since 2008, RSRT has awarded \$36 million to research. An average of ninety-six percent of every dollar donated to RSRT goes directly to our research program.

Keeping our internal team lean and fiercely motivated allows us to focus funds and energy on what matters most: healing our children as quickly as possible.

RSRT 组织提供比全球任何其他雷特研究组织更多的研究基金。2008 年以来，RSRT 已提供了累计 3600 万美元的研究基金。平均 96% 的经费由 RSRT 直接捐赠给我们的研究项目。我们时刻保持我们内部团队的高效性和积极性，使我们能够集中资金和精力在最重要的事情上：尽快治愈我们的孩子。

As one of the few neurological disorders to show dramatic symptom reversal in mice, Rett Syndrome has attracted unprecedented interest from university and pharmaceutical scientists. Through innovative research and constant engagement with scientists, clinicians, industry, investors and affected families, RSRT is at the epicenter of this activity.

雷特综合症作为成功在试验鼠身上实现症状逆转的少数几个神经系统疾病之一，吸引了来自大学和制药公司的科学家们前所未有的关注。通过科学家、医生、生物制药公司、投资者和受影响的家庭的创新性研究和不断参与，RSRT 组织在这其中起到了核心作用。

We do more than just write checks: we are changing the landscape of Rett research. We set the research agenda by proactively identifying and monitoring promising therapeutic areas, seeking out scientific and industry partnerships and working closely with them to advance programs through the drug development pipeline. We are risk takers who do not shy away from bold and innovative projects unlikely to be funded by the NIH or other more conventional funding agencies.

我们不仅仅只是出资支持项目研究：我们正在改变雷特研究的格局。我们通过主动寻找和监测有希望的治疗领域制定出研究日程表，找出科学和产业方面的合作伙伴并与他们紧密协作，以通过药物发展推动研究项目。我们并不回避那些大胆的并有些冒险的创新项目，往往正是这样的项目不太可能获得由美国国立卫生研究院或其他传统组织提供的资金支持。

We believe Rett Syndrome can be cured, but time matters. A rigorous, coordinated, and agile research agenda will get us there as quickly as possible. Our ultimate goal is to become obsolete – by finding a cure.

我们相信雷特综合症是可以被治愈的，但是时间紧迫。严谨、协调、紧凑的研究日程将使我们尽可能快地实现目标。我们的最终目标是通过寻找一种治愈方法使雷特综合症成为过去。

About Rett

关于雷特综合症

Imagine the symptoms of Autism, Cerebral Palsy, Parkinson's, Epilepsy and Anxiety Disorder... all in one little girl.

想象一下，在一个小女孩身上就患有自闭症、脑瘫、帕金森、癫痫和焦虑症.....。

Rett Syndrome is serious lifelong neurological disorder that is caused by random mutations in a gene called MECP2.

Diagnosed almost exclusively in girls, symptoms typically appear in toddlerhood. Many children with Rett are unable to speak, walk or use their hands. Breathing problems, feeding tubes, seizures, anxiety, gastrointestinal and orthopedic issues are common.

雷特综合症是由一种由叫做 MECP2 基因的随机突变引起的严重的终身神经疾病。雷特综合症几乎只发生在女孩身上，症状通常首先出现在幼儿期。许多雷特患儿还不能说话、走路或使用双手。常见的症状则有呼吸问题、喂养困难、癫痫发作、焦虑、胃肠道和骨科问题。

Despite the debilitating nature of the disorder our girls are so much more than a laundry list of symptoms. Their beauty and their strength inspire and motivate us.

尽管这种疾病带给我们的女孩太多痛苦的症状，她们的美和她们力量始终鼓舞并激励着我们。

Rett Syndrome stands apart from other neurological disorders due to the dramatic reversibility of symptoms in animal models.

Our confidence that Rett Syndrome is curable guides our every decision.

雷特综合症不同于其他神经功能障碍疾病，它的症状在动物模型中有可逆性。我们相信雷特综合症是可以被治愈的，这种信心指引着我们做出每一个决定。

Although very rare, boys can also have Rett Syndrome. There are several specific genetic scenarios that can happen. Please visit our Genetics Primer to learn more. Almost all of the research that RSRT supports is as relevant to boys with Rett Syndrome as it is to girls. RSRT's goal of treatments and a cure applies to girls and boys with the disorder.

虽然非常罕见，但男孩也可能患有雷特综合症。有几种特殊的遗传基因情况可能发生。请访问我们的遗传学读物，以了解更多。如同女孩雷特综合症，几乎所有 RSRT 支持的研究项目均涉及到男孩的雷特综合症。不管是女孩还是男孩，治愈他们是 RSRT 的目标。