

Rett Superheroes | Rett 超级英雄

by [Tim Freeman](#) | January 4, 2019

Tim Freeman | 2019 年 1 月 4 日

We launched [Roadmap to a Cure](#), RSRT's three-year \$33 million strategic research plan, in March of 2017. We're now 21 months into the 36-month period, 60% through the timeframe. I'm excited to report that as of January 1 we've raised almost \$24 million towards the goal. That's 73% of the way there. We're ahead of schedule!

2017 年 3 月，我们启动了“通向治愈的地图”项目，这是 RSRT 希望用三年时间筹集 3300 万美元用于战略性研究的计划。现在是 36 个月中的第 21 个月，我们已用去了 60% 的时间。我很高兴地宣布，截至 2019 年 1 月 1 日，我们已经为这个目标筹集了近 2400 万美元，这相当于筹款目标的 73%。我们比预想的进度还要快！

Nothing I can say here adequately expresses the gratitude I feel to every one of RSRT's supporters. I know my RSRT colleagues, our trustees, and every family affected by Rett feel the same gratitude that I do. To the families that fundraise for us, my thanks and admiration is deeply felt and personal. Whether you are one of our [Roadmap Trailblazers](#) or you fundraise for us through an [event](#) or [RettGive](#) or Facebook, you should be very proud. We would not be here without you.

在此，任何语言都无法表达我对 RSRT 每一位支持者的感激之情。我知道我的 RSRT 同事、我们信托基金的受托人，以及每一个受 Rett 影响的都怀有和我一样的感激。对于那些为我们筹集资金的家庭，我个人发自内心的感谢和钦佩。无论您是我们“Roadmap Trailblazer”项目的一分子，还是通过任何活动、RettGive 或 Facebook 上为我们筹集资金，您都应该感到非常的自豪。没有您，我们不能做到现在的这一切。

\$24 million is such a big number that it's hard to visualize, so I did a little googling to help me wrap my own mind around it. I learned that if you stacked up a million one-dollar bills it would measure 358 feet, about the height of a 30-story building. If you stacked up 24 million one-dollar bills it would stretch more than a mile-and-a-half, way taller than any building. The \$33 million we need to raise would be over 2.2 miles high if stacked in bills!

2400 万美元是一个大到难以想象的数字，所以我在谷歌上搜索了一下，以帮助自己搞清楚这些概念。我了解到，如果您把一百万张 1 美元的钞票叠起来，它会有 358 英尺高，大约相当于有 30 层楼。如果您把 2400 万张 1 美元的钞票叠起来，它会向空中伸展超过 1.5 英里，比任何建筑都高。而我们目标筹集的 3300 万美元，如果叠起来的话，会有 2.2 英里高！

That gave me a fun visual on what all who are involved with RSRT have built with your generosity and efforts. Far more important is what those dollars have really made possible. The first human clinical trial in gene therapy is on track to begin this year; a second-generation gene therapy is being developed to ensure

maximum impact on lives; potentially curative approaches in MECP2 reactivation, RNA editing and transsplicing, and protein replacement are making promising strides; five leading clinics are members of the Clinical Trial Consortium and are setting the stage for the highest quality clinical trials. Spearheading all this are Monica Coenraads, Randy Carpenter, Tim Riley, and Jana von Hehn, who never cease to amaze me with their knowledge and dedication and their ability to recruit and empower world-class researchers to attack Rett Syndrome with the boldest approaches that are at the vanguard of science. In short, I have never been more optimistic about the future, and I mean the near future, for all with Rett, including my own daughter. The \$24 million raised so far has made all this possible.

这让我看到了一个有趣的画面，所有参与 RSRT 工作的人取得的成果其实都是依赖您的慷慨和努力。更重要的是，这些资金已经在让某些事情变成可能。首个用于人类的基因治疗临床试验将按计划今年启动；以确保对患者能有更大效果的第二代基因疗法产品也已开始研发；在 MECP2 再激活、RNA 编辑和反式剪接、以及蛋白质替换等方面，各种有潜力的治疗方法也取得了有希望的进展；临床试验联盟成员中的 5 个业内领先的机构正在为最高质量的临床试验打牢基础。带领这一切的先锋是 Monica Coenraads, Randy Carpenter, Tim Riley, 以及 Jana von Hehn。我不断地为他们的知识、奉献精神和管理能力而赞叹，这些特质让他们能够聘请和授权世界级研究人员以最大胆的、处于科学前沿的方法攻关 Rett 综合症的治愈。简而言之，对所有患 Rett 综合征的人，包括我自己的女儿，我对未来——我是指不久的将来——从来没有像现在这样乐观过。正是迄今为止筹集的 2400 万美元使这一切成为了可能。

We have \$9 million more to raise to fully carry out the priority approaches of Roadmap to a Cure. That's going to take the continued energy of families that are already fundraising, and it's going to take more families getting involved with us. As far as I'm concerned, all who helped get us this far are superheroes, capable of leaping tall buildings, including ones that are a mile-and-a-half tall! If you're not involved with us yet, join the superheroes who already are and help us take the next leap over the 2.2-mile-high building. We need you more than ever. *Are you in?*

我们还需要筹集 900 万美元，以全面实施“通向治愈的地图”项目中的高优先计划。这依赖于那些已经在筹款的家庭的继续努力，同时也需要更多的家庭参与到我们的工作中来。在我看来，所有帮助我们走到这一步的人士都是超级英雄，他们有能力跳过大楼，包括那个 1.5 英里高的！如果您还没有加入我们，请加入超级英雄行列里来，帮助我们跳过下一座 2.2 英里高的大楼。我们比任何时候都需要您。您加入吗？