

## My First 9 Months at RSRT

我在 RSRT 最初的 9 个月

by Timothy Riley, PhD | July 3, 2019

Timothy Riley | 2019 年 7 月 3 日

The past 9 months since joining RSRT have been amongst the most exciting of my professional career. Never in the history of life sciences has the development of scientific technologies that can benefit individuals with Rett been so rapid.

自从加入 RSRT，这过去 9 个月已经成为了我职业生涯中最激动人心的时期之一。在生命科学的历史上，从未有过如此之快的科学技术的发展，可以让雷特综合征患者这样的个体能得到受益。

In my role as RSRT's Chief Scientific Officer, ensuring that these technologies are put to work on behalf of your loved ones is a responsibility I take to heart. I have had the privilege of representing individuals with Rett Syndrome in many ways over the past several months. With frequent trips to universities up and down the East Coast of the USA, as well as in Europe, I am in close contact with the numerous professors we fund to better understand their research, their challenges and importantly, gauge their progress.

作为 RSRT 的首席科学官，我谨记在心的责任就是确保这些技术能够为大家所爱的人发挥作用。在过去的几个月里，我有幸以各种方式代表患有雷特综合征的患者工作。我频繁的访问各所大学，从美国东海岸到欧洲，与众多我们资助的教授保持密切联系，以便更好地了解他们的研究、他们面临的挑战，以及更重要的：评估他们的进展。

Our Gene Therapy Consortium is developing new coatings on the outside of the vector viruses called AAV so that they distribute better throughout the brain. The investigators that we have funded to work on reactivating the healthy but silent copy of MECP2 have made a breakthrough in doing this in a selective manner. The scientists working on DNA editing have the single base editors ready to go and are now testing them on patient derived cells collected through our Outcome Measures & Biomarkers Development (OMBD) initiative.

我们的基因治疗联盟正在开发一种在叫 AAV 的病毒载体外部的新表面覆层，这可以让它们能更好地在大脑中分布。我们资助的致力于重新激活那份健康但沉默的 MECP2 基因拷贝的研究人员已经在开发特定基因选择性激活的方法上取得了突破。从事 DNA 编辑工作的科学家们已经准备好了单碱基编辑器，现在正在来自患者身上的细胞里测试，而这些患者也是通过我们的效果测量和生物标志物开发 (OMBD) 计划募集的。

In my dual role as RSRT's Chief Business Officer I have also engaged with dozens of pharmaceutical and biotech companies educating them about our Roadmap to a Cure and assessing their interest in Rett. I have counseled academics on the importance of protecting their discoveries by filing intellectual property (IP) and then brokered introductions to companies in the hopes that the IP will be licensed and developed.

在我同时作为 RSRT 首席商务官的另一种角色中，我还与数十家制药和生物科技公司进行了接触，向他们介绍我们的治愈路线图 (Roadmap to a Cure)，并评估他们对雷特综合征的兴趣。我也给学术界就保护他们的发现和知识产权 (IP) 的重要性提供了建议，然后帮他们和公司牵线搭桥，希望知识产权能够被授权和进一步开发。

These interactions are already proving worthwhile. One of the largest pharmaceutical companies in the world, which has a keen interest in gene therapy, is also very interested in our MECP2 reactivation efforts. Our RNA editing efforts, already a hot area of science, is generating considerable interest amongst established

companies, start ups and investors. Two companies already have option agreements with the universities involved in our Gene Therapy Consortium 2.0 to move concepts forward once progress is made in the academicians' labs.

事实证明，这些沟通交流是值得的。世界上最大的，并且对基因治疗有着浓厚的兴趣的制药公司之一，也对我们的 MECP2 再激活的工作非常感兴趣。我们热门的 RNA 编辑科学领域的工作引起了许多知名公司、初创企业和投资者的极大兴趣。有两家公司已经与参与我们基因治疗联盟 2.0 的大学达成了期权协议，一旦在研究机构的实验室取得进展，就可以推动产品从概念开始研发。

Taking advantage of networking opportunities is core to what my colleagues and I do on behalf of the Rett community and in that spirit I've also attended conferences on a variety of topics as well as a very productive BIO partnering event in Philadelphia.

利用社交网络带来的机会是我的同事们和我代表雷特综合征社区工作的核心思想，本着这种精神我参加了各种主题的会议，以及在费城举办的一个非常有成果的生物产业合伙人的活动。

Despite all this progress and activity, there are hurdles to overcome and big unknowns remain. These unknowns can only be answered through the systematic and rigorous drug development process that is required by the FDA. Questions such as the body's immune reaction to the viral delivery vehicle and/or to the DNA and RNA editors we would need to deliver to the brain. We will proceed cautiously yet aggressively to answer these questions and overcome the challenges.

尽管取得了这些进展和成果，但仍有一些障碍需要克服，而且仍然存在巨大的未知数。这些未知只能通过 FDA 要求的系统且严格的药物开发流程来探明。例如，人体对病毒载体的免疫反应，和/或对我们需要传递给大脑的 DNA 和 RNA 编辑器的免疫反应。我们将谨慎而积极地回答这些问题并克服这些挑战。

My colleagues and I have big plans for the remainder of the year. The ability to safely and effectively deliver biologic therapeutics diffusely to the brain is fundamental and relevant to all of our curative strategies. So a major objective of RSRT is to identify and support delivery technologies that extend beyond AAV9. We are in close contact with a number of professors and companies working in this area and are actively soliciting proposals.

我和我的同事们为今年剩下的时间制定了宏伟的计划。安全有效地将生物医药制剂递送到大脑的能力是基础，也是和我们所有治愈策略都相关联的。因此，RSRT 的主要目标是找到和支持比 AAV9 的更好的递送技术。我们与在这一领域工作的许多教授和公司都保持密切联系，并正在积极寻求各种方案。

Beyond Rett, we have also developed a roadmap for MECP2 Duplication Syndrome and have identified and received commitments from investigators to submit proposals for a number of exciting interventions. With funding from duplication families we hope to launch several pivotal projects.

除了雷特综合征之外，我们也为 MECP2 重复综合征制定了路线图，并已物色到相关研究人员和接收到提交的关于

数项令人兴奋的干预措施的建议。有了来自 MECP2 重复综合征患者家庭的资助,我们希望开始启动几个关键项目。

As I said in my introductory blog, I was hired at RSRT to move the needle and that is exactly what I intend to do.

正如在我的自我介绍的那篇博客中说的,我在 RSRT 的工作是要真正推动一些事情,这也正是我打算做的。

If you have questions or would like to have a phone call to delve more deeply into any of the above topics feel free to contact me. I am always available to schedule some time to chat.

如果您有任何问题或想要通过电话更深入地研究以上任何一个主题,请随时与我联系。对于聊这些问题,我永远有时间。