$9 Million Awarded to Research
9 百万奖励基金提供给研究

by **Monica Coenraads** | March 8, 2016

Today RSRT announced $9 million of research awarded in 2015. What sets these awards apart from previous years is not just the significantly increased dollar amount but also the emphasis on clinical research. This focus reflects the critical need to conduct efficient and effective clinical trials with specific measures that are objective and sensitive to changes over short periods of time (months not years). We don't have those measures yet and they must be urgently developed in parallel to the basic science we are funding. When a medication (like a drug that activates MECP2) or a biological (e.g. gene therapy) shows promising results in animal models we must be prepared to test its safety and efficacy in the best possibly designed trial. To address the current knowledge gap RSRT has awarded $4.5 million to finance a unique collaboration among four clinicians (Alan Percy, Aleksandra Djukic, Daniel Tarquinio, Tim Benke) who will explore a number of novel outcome measures.

今天 RSRT 宣布在 2015 年度共提供了 9 百万美元的研究奖励基金。和往年相比，2015 年的基金除了奖励数额增加之外还更侧重于临床研究。这一点集中体现在针对易变指标进行短时间监测的高效临床试验。我们急需将其和我们正在资助的基础科学研究进行平行开发。因为当一种药物（如激活 MECP2 的药物）或一种生物疗法（如基因疗法）在动物试验模型上得到了令人满意的效果，下一步我们必须准备测试其在人体安全性和有效性。为填补当前的知识空白，RSRT 已授予 Alan Percy, Aleksandra Djukic, Daniel Tarquinio 和 Tim Benke 四位临床医生组成的临床科研团队 450 万美元的研究资助。他们将探索许多创新性的研究成果。

This consortium is designed to be nimble, allowing the pursuit of outcomes that look encouraging while quickly discarding those that don't. RSRT will manage the infrastructure to properly curate this ambitious project including a cutting edge data management system that will make anonymized data available to clinicians, scientists, industry and investors, thereby maximizing its potential use. This project is critical for assuring that lab science translates to having an impact on our loved ones struggling with Rett Syndrome and related disorders. It is very exciting that we have reached the stage with Rett research where we must develop clear measures for the impact we expect to have.

我们秉承追赶效果的同时也迅速放弃那些不令人振奋的成果。RSRT 将改善基础设施，以妥善实施这个雄心勃勃的项目，包括建立全球领先的数据管理系统，这一系统的建立将使匿名的数据可用于临床医生、科学家、行业和投资者，从而最大化它的潜在用途。这个项目将实验室成果进行转化，从而使与雷特综合征做抗争的人和受相关疾病困扰的人从中受益。非常令人振奋的是我们已经进入了对雷特综合症疗效进行准确评估的阶段。

As always at RSRT, we are tremendously grateful to all the families that hold events and raise funds, to our generous donors, and to Rett organizations in the U.S. and around the world. None of what RSRT does would be possible without this support.

RSRT 组织将一如既往，我们非常感谢所有帮助我们举办活动和筹集资金的家庭，向我们慷慨解囊的捐助者，以及在美国和世界各地的雷特综合症相关组织。RSRT 的发展离不开你们的支持。

Thank You!
谢谢！

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