Rett Syndrome Clinic in Atlanta Launches Clinical Trial

雷特综合症临床试验在亚特兰大启动

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Dr. Daniel Tarquinio, the director of the Rett Syndrome Clinic in Atlanta will soon be recruiting for a 10 person, open label trial (everyone gets the drug) testing triheptanoin. The drug, also called UX007, is a colorless oil which is broken down in the body to help fuel specific chemical reactions that produce energy. Administration of UX007 has shown improvements in both metabolism and mortality in disorders with impaired energy production (for example, mitochondrial disorders). Treatment with UX007 improved longevity, motor function, and social interaction in the mouse model of Rett Syndrome, and may have improved metabolic dysfunction as well. Additionally, in general acute and chronic mouse models of epilepsy, UX007 acts as an anticonvulsant, although the mechanism for this is unclear.

位于亚特兰大的雷特综合征临床研究主任 Daniel Tarquinio 博士宣布将很快在亚特兰大招募 10 名药物试验志愿患者，进行三庚酸甘油脂的开放式标签试验（测试者和受试者都知道其测/受试的药物）。该药物也被称为 UX007，为无色油脂，其在体内被分解从而为产生能量的化学反应供给燃料。UX007 药物改善了由于能量产生障碍（例如，线粒体功能障碍）而引起的新陈代谢紊乱并降低了相关疾病的死亡率。在雷特综合征试验小鼠模型上，UX007 的治疗提高了受试鼠寿命、 运动和社交功能，并改善了模型小鼠的新陈代谢。此外，虽然机理尚不明确，UX007 对急性和慢性癫痫老鼠模型具有抗惊厥作用，。

The primary objective of the study is to evaluate the safety and tolerability of UX007 in subjects with Rett Syndrome using laboratory values, electrocardiogram, rate of adverse events (AE), and physical exam.

这次试验的主要目的是评估 UX007 在雷特综合症患者上的安全性和耐受性，其中包括监测患者的心电图，不良反应比率（AE）以及常规的身体检查。

The secondary objectives are to evaluate the efficacy of UX007 in improving overall seizure frequency and dystonia.

这次试验的第二个目的是评估 UX007 药物在改善整体惊厥频率和肌张力障碍的功效。

The study will enroll 10 pediatric, adolescent, and adult participants with Rett who have seizures (at least four seizures per month), dystonia (at least four dystonic episodes per month), or both. They must be on a stable medication regimen, defined as having had no medications added, taken away or dose adjustments for 30 days prior to the start of the study. Pediatric participants must be at least 2 years old at the time of consent, and must be in the post-regression period, defined as having had no clear loss of language or hand use in the 6 months prior to the study.

这项研究将招募 10 名儿童、 青少年和成年雷特综合症患者。 受试者要求有癫痫发作 （每月至少四次癫痫发作）或/和肌张力障碍 （每个月的至少四次肌张力障碍发作）的病史。此外受试者还必须有稳定的用药规律，即在试验开始前 30 天没有新增或停用药物，或是药物剂量调整。儿童的参与者必须至少 2 周岁，并且在开始试验前 6 个月内没有明显的语言和手部行为丧失。

Participants will be evaluated for inclusion during a screening/baseline period of 2 months. Eligible participants will begin UX007 after 2 months using a 2-week titration schedule until the subject has reached age-specific target dosing. Once the maximum dose is reached, the participant will continue to receive UX007 for 4 months. Participants will be monitored for an additional 2-month period.

受试者将首先通过两个月的筛选和基本标准评估。合格者将在两个月之后开始服用 UX007 药物，通过逐渐测加药量达到特定年龄群的目标计量，一旦确定最大剂量，参与者将连续服用四个月的 UX007 并接受额外两个月的观察。

The trial is funded by Ultragenyx and RSRT.

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For more information about the trial please contact Dr. Tarquinio.

关于试验的更多信息请联系 Tarquinio 博士