AveXis Reports on Rett Gene Therapy Program: AVXS-201
AveXis公司有关雷特基因治疗的报告 – AVXS-201
by RSRT | February 28, 2018
发布于RSRT\2018年2月28日

AveXis Reports on the Rett Syndrome Gene Therapy Program – Clinical Trial Likely to Start Within a Year
AveXis公司有关雷特基因治疗的报告 – 临床试验很可能在一年之内开始
In June of 2017 AveXis announced that based on encouraging data generated by RSRT's *Gene Therapy Consortium* it would advance the lead *MECP2* gene therapy candidate toward clinical trials.
在2017年6月，AveXi公司宣布，根据RSRT基因治疗联盟所产生的令人鼓舞的数据，它将把MECP2基因疗法的候选药物推向临床试验。

Yesterday the company provided an update on its Rett Syndrome program during a conference call to report their 2017 financial results, recent corporate accomplishments and upcoming milestones.
昨天，该公司在一次电话会议中，对其"雷特综合症"项目进行了更新，报告了他们2017年的财务状况、最近的企业成就和即将到来的里程碑。

We encourage you to listen to the ***conference call***, which is interesting and encouraging. Please note that the Q&A at the end of the call addresses a number of relevant questions raised by analysts about the Rett program. Brian Kaspar, Chief Scientific Officer of AveXis and past member of the *Gene Therapy Consortium* ***presented preclinical data with these slides***. A ***press release*** is also available.
我们鼓励你去听电话会议，这很有趣，也很鼓舞人心。请注意，在电话会议结束时的分析师提出的问答部分，一些雷特项目的相关问题。AveXi公司的首席科学主任布莱恩. 卡斯帕（Brian Kaspar）是基因治疗联盟的前任成员，他在这些幻灯片中展示了临床前的数据。还发布了新闻稿。

Below are key highlights from the call.
以下是电话会议中的主要亮点。

· AveXis is on-track to meet with the FDA next quarter to present data from the Spinal Muscular Atrophy Type 1 (SMA 1) gene therapy product, AVXS-101. This data package submission (called Biologics License Application) is the last step before the FDA can approve AVXS-101 and make it commercially available.

· AveXis公司正准备在下个季度与美国食品药物管理局（FDA）会面，介绍脊髓肌肉萎缩型1型（SMA 1）基因治疗产品AVXS-101的数据。这个数据包提交（被称为生物制品许可应用程序）是美国食品药物管理局（FDA）批准AVXS-101并使其商业化的最后一步。

· Initiated a Phase 1 trial of AVXS-101 in SMA Type 2 using an intrathecal route of delivery (injection into the spinal fluid). This is the same delivery route that will be used in the Rett Syndrome trial. The first two patients that have been treated with this delivery method have reported no adverse effects.
在SMA 2型ii型中，使用了一种插入式输送路线（注入脊髓液），对AVXS-101进行了第一阶段试验。这是在雷特综合症试验中使用的同样的传递路线。使用这种方法治疗的前两名患者没有显示任何不良反应。

· Will be initiating a pivotal trial for AVXS-101 in Europe. Pivotal means that the trial is intended to provide sufficient data for approval by the European FDA equivalent, the EMA. It's important for all of our European families to know that AveXis has the infrastructure and desire to market their products in Europe as well as the US.
将在欧洲启动一项关键的AVXS-101试验。Pivotal意思是，该试验旨在提供足够的数据，以获得欧洲食品及药物管理局的批准。对于我们所有的欧洲家庭来说，了解AveXis公司拥有基础设施和在欧洲和美国市场推广其产品的愿望是非常重要的。

· Obtained exclusive rights from REGENXBIO to the AAV9 gene therapy delivery system and are expanding their gene therapy program to pursue Rett Syndrome (AVXS-201) and amyotrophic lateral sclerosis (AVXS-301).
从REGENXBIO获得了AAV9基因治疗输送系统的专有权利，并正在扩大其基因治疗项目，以寻求在雷特综合症（AVXS-201）和肌萎缩性脊髓侧索硬化症（AVXS-301）上的应用。

· Preclinical mouse studies using AVXS-201 led to the longest living Rett mouse to date with sustained symptom reduction.
在使用AVXS-201的临床前试验鼠研究中，迄今为止，使用了持续时间最长的雷特试验鼠，得意持续的症状减轻。

· Primate studies using AVXS-201 show no signs of adverse effects or over-expression of *MECP2*.
在灵长累动物身上使用AVXS-201没有显示不良反应或MECP2的过度表达。

· Production of clinical trial grade AVXS-201 using AveXis' own scalable manufacturing platform is underway.
用AveXis公司自己的可扩展性的制造平台进行的临床试验级AVXS-201的生产正在进行中。

· Plan to submit Investigational New Drug (IND) applications for both Rett and ALS, in late 2018/early 2019. An IND is the proposal to the FDA to begin human trials. If the FDA has no substantial issues with the application, a clinical trial may proceed 30 days after the IND submission.
计划在2018年底/2019年初为雷特和ALS提交试验性新药（IND）的应用申请。一种试验性新药是向美国食品药物管理局（FDA）提出的开始人体试验的建议。如果美国食品药物管理局（FDA）对申请没有实质性的问题，临床试验可能在试验性新药（IND）申请提交后30天内进行。

We are delighted with the progress that AveXis has made in advancing the AVXS-201 program. **Pending FDA approval it's likely that a gene therapy clinical trial will start within a year.** We will inform the community when details on the trial become available.
我们对AveXis公司在推进AVXS-201项目方面取得的进展感到高兴。在美国食品药物管理局(FDA)审批期间，基因治疗临床试验很可能将在一年内启动。我们将向社会公布有关试验成为可能的相关细节。

We profusely thank all of our Rett families and donors, in the US and beyond, who have played such a critical role in getting us to this point.
我们非常感谢我们在美国及其他地区的所有的雷特家庭和捐助者，他们在使我们达到这一目标方面发挥了至关重要的作用。