

Vision and Guts

远见与胆识

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Our recent announcement that AveXis is planning to initiate a gene therapy clinical trial within a year (pending FDA approval) is exciting and worthy of celebration. Getting us to this point took years of hard work and millions of dollars raised and thoughtfully awarded. It is truly a moment worthy of stepping back and recognizing the effort of so many people – our scientists, our families who fundraise, our generous donors, our relentless trustees and our passionate staff.

We would not be at this point without you.

Nevertheless, this is not the time to think that our work is done – that we can coast from here on out. In actuality this is the time to double down. Here's why:

我们最近宣布，AveXis计划在一年内启动一项基因治疗临床试验（在等待美国食品药品监督管理局-FDA批准之际），这是令人兴奋的，值得庆祝。让我们达到这一目标需要数年的辛勤工作和数百万美元的融资和深思熟虑的奖励。这是一个值得我们回顾并认识到如此多的人的努力的时刻——我们的科学家，我们的家庭筹款，我们慷慨的捐助者，我们无情的受托人和我们充满激情的工作人员。如果没有你，我们就不会达到这一节点。

然而，现在不是认为我们的工作已经完成的时候——我们可以从这里出发。实际上，现在是加倍下注的时候了。原因如下：

1. While getting to the trial is a big step, it's the results that matter. And we don't know what those results will be. Will they be dramatic or not? Will all symptoms be addressed or not? Will older children and adults benefit? Until we have a cure or dramatic improvements in hand, our work must continue.

到达临床试验阶段是迈出了一大步，但结果才是重要的。我们不知道结果会是什么。它们会是激动人心的吗？所有的症状都能得到解决吗？年长的儿童和成人会受益吗？在我们有了治愈或激动人心的改善之前，我们的工作必须继续。

2. We have a solid research plan to follow, *Roadmap to a Cure*, that prioritizes not only gene therapy but also three other approaches that attack Rett Syndrome at its genetic core. Each of these approaches has to be aggressively pursued until we know for certain where we will find a cure.

我们有一个坚实的研究计划要遵循，一个治疗的路线图，它不仅优先考虑基因治疗，而且还有其他三种方法，在它的遗传核心中攻克雷特综合症。这些方法都必须积极地推进，直到我们确定我们能找到治愈方法。

3. We have the staff, scientists and advisors to execute on this bold plan. And importantly we have the vision and the guts to act boldly. Pursuing strategies like gene therapy is not for the faint of heart.

我们有工作人员、科学家和顾问来执行这个大胆的计划。更重要的是，我们有远见和勇气大胆地行动。像基因疗法这样的策略并不适合胆小的人。

4. Our business model is to invest significant dollars in high impact areas such as gene therapy via collaborative Consortia who generate sufficient data to attract an industry partner. We now have proof that this strategy works and we will apply that model to other areas of our research portfolio.

我们的商业模式是在高影响领域投入大量资金，例如通过合作的联盟来产生足够的数据来吸引行业合作伙伴。我们现在有证据证明这个战略有效，我们将把这个模型应用到我们研究的其他领域。

I hope the AveXis news has inspired you. If you are actively fundraising for us, I am beyond grateful. Please continue to do so with renewed optimism and determination.

我希望AveXis的消息已经激发了你们。如果你正在为我们筹款，我除了感激。请继续保持乐观和坚定的信念。

If you are not fundraising for us I ask that you consider channeling that inspiration into action. We are in the midst of a groundswell movement with families in the US and around the world taking charge of their children's future. I invite you to be a part of that groundswell!

如果你没有为我们筹款，我请你考虑将这种激发转化为行动。我们正处于一场潮流运动之中，美国和世界各地的家庭都在为孩子的未来负责。我邀请你成为那股风潮的一部分！

Contact us to get started.

请联系我们，开始吧。