

## Ketamine Clinical Trial for Rett Syndrome Launches

氯胺酮用于治疗雷特综合征的临床试验已启动

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You may have heard about a clinical trial testing ketamine in Rett Syndrome. We're excited to be leading this important study and really asking in an unbiased way if ketamine can improve the symptoms of Rett Syndrome.

你可能已经听说过有一项将氯胺酮用于治疗雷特综合征的临床试验。我们很高兴能领导这项重要的研究，并以公正的态度切实探寻氯胺酮是否能改善雷特综合征的症状。

Ketamine is an old drug and has been studied in all kinds of areas – from anesthesia, where it was initially approved by FDA in 1970 (yes, that's 50 years ago), to anxiety, pain relief, bi-polar disorder, obsessive-compulsive disorder, depression and others. You may have seen just recently that a nasal version of ketamine was approved by the FDA to treat depression. That means there is a lot of experience with ketamine over the years and due to its old age, **we can be less concerned about unknown safety issues popping up.**

氯胺酮是一种老药物了，并且在若干领域都进行过相关研究——最早是1970年（没错，是50年前）FDA批准其用于麻醉，后来拓展到针对焦虑、疼痛、双极性障碍、强迫症、抑郁症和其他疾病的应用上。你可能最近也看到了FDA批准了一种鼻用氯胺酮用于治疗抑郁症的消息。这些都意味着由于氯胺酮是一款老药物，这么多年来已经对它的使用累计了丰富的经验，所以我们可以不用那么担心会出现一些未知安全问题。

Here's why we're excited. Ketamine is primarily considered an NMDA receptor antagonist (meaning it inhibits a receptor) but it also interacts with a lot of other receptors and signaling pathways in the brain that can change brain function. In addition to action in other neurological disorders, ketamine also shows symptom improvement in Rett mice as described in several publications, improving brain signaling, movement, and breathing. These mouse studies show that ketamine boosts key proteins needed for neuronal function in the absence of Mecp2 and that some changes in the brain persist after dosing is over. If the changes ketamine induces are retained beyond dosing, it raises interesting possibilities for effective intermittent or pulse-type dosing regimens.

解释一下我们感到激动的原因：氯胺酮被认为主要是一种 NMDA 受体拮抗剂（拮抗剂的意思是它抑制受体），但它也与大脑中的许多其他受体和信号通路相互作用，因此能够改变大脑功能。除了对其他神经系统疾病的作用研究，在一些文献中也描述了氯胺酮在 Rett 小鼠身上也表现出改善症状的作用，比如改善了大脑信号传导、运动能力和呼吸状况。这些小鼠研究表明，氯胺酮能在缺乏 MeCP2 蛋白质的情况下提高那些神经元功能所需的关键蛋白质水平，而且在给药结束后，小鼠大脑中的变化仍然能持续。如果氯胺酮引起的变化在给药结束后仍然持续，这就意味着有可能存在一种有效的间歇性或脉冲式给药方案。

Our excitement doesn't stop there. We have also seen some interesting results in humans as well. One case report of a Rett patient receiving ketamine for seizure control had remarkable improvements in Rett symptoms that persisted for several weeks after a 5-day dosing regimen of oral ketamine, and a pilot study in 4 Rett patients receiving 4 different IV doses showed consistent improvements in breathing. We also hear the occasional yet repeated story where ketamine was administered as part of a procedure and families report notable changes in their child. However, not everyone has this experience. So what is really going on here? Well, that's what we're hoping to find out.

让我们激动的原因还不止于此。我们在人类身上也看到了一些有意思的结果。有一份雷特综合征患者接受氯胺酮治疗用于控制癫痫发作的病例报告显示，患者在采用 5 天疗程口服量氯胺酮治疗方案后，其雷特综合征症状得到了明显改善，且改善持续了数周。另外，在由 4 名雷特综合征患者接受 4 种不同静脉注射剂量的一项试点研究显示，患者的呼吸状况有了持续改善。我们也有时候也听到相同的故事，就是当某些家庭使用氯胺酮作为日常治疗流程的一部分后，他们发现并报告孩子身上发生了显著的变化。不过，并不是每个人都有这种经历。那么这里到底发生了什么？好吧，这就是我们想知道的。

The goal of the study is to definitively answer the question of **whether or not ketamine treatment leads to notable improvements in symptoms**. We are testing ketamine dosed orally over a range of 4 doses below those that cause anesthesia. The study is designed as a cross-over, meaning that each patient will receive ketamine as well as placebo, so we can directly compare the responses to the two treatments in the same patient, and we can compare how all the patients who receive a particular dose respond, as well as patients across different doses. We decided to test the 5-day dosing regimen that was successful in the most encouraging case reported to date.

这项研究的目的是明确回答**氯胺酮治疗是否能显著改善雷特综合征症状**的问题。我们正在测试的口服氯胺酮研究以四种低于麻醉量的剂量范围给药。此项研究设计为一个交叉试验，也就是意味着每个病人都将接受氯胺酮以及安慰剂，所以我们可以直接比较在同一患者身上两种治疗方式的反应，我们也可以比较所有患者接受某一个特定剂量的药物的反应，以及比较患者在采用不同剂量时的反应。我们决定验证 5 天剂量方案，这来自迄今为止最鼓舞人心的报道中的成功经验。

The study is double-blind and randomized, which means no one will know what treatment is being given during the study (not the participant or the study doctor or anyone involved in managing the study), and that the treatment order is randomly chosen by a computer for each patient. These conditions make the study conclusions stronger because no one rating the patient's symptoms knows what treatment is being tested in that moment. It helps everyone focus on what is truly observed and prevents bias, whether conscious or unconscious.

本项研究是双盲、随机的，这意味着没有人会知道在研究过程中给患者采用了什么治疗方式(参与者、医生、和试验有关的管理人员都不知道)，而且治疗方式的顺序也是由计算机随机为每位患者选择的。这些情况会让研究结论更加有说服力，因为没有人会在对患者症状进行评分时知道患者当时正在接受什么治疗方法。这将帮助每个人专注于真正观察到的东西，防止各种有意识的或无意识的主观偏见。

What do we hope to accomplish? We are working closely with the FDA to ensure we conduct this study at the most rigorous standards for safety and efficacy with the goal to provide meaningful and interpretable data. **At the end of the study we hope the study will confirm that ketamine is safe to administer to Rett patients and we also hope to say that ketamine dosed orally is or is not useful for managing Rett symptoms.** If it is useful, we hope to know which doses are best suited for effective treatment. And if it is a good treatment, ketamine is already on the market so there would be no delays in getting access.

我们希望达到什么目标呢？目前我们正与 FDA 密切合作，以确保我们按照最严格的安全性和有效性的标准进行这项研究，目标是研究能够提供有意义和可解释的数据。当研究结束时，我们希望能证实氯胺酮对雷特综合征患者是安全的，我们也希望能确定口服氯胺酮的方式对控制 Rett 症状到底有没有治疗作用。如果有用，我们希望知道哪种剂量最适合治疗。最后，如果这真的是一种好的治疗方法，因为氯胺酮已经在市场上正常销售，所以患者可以立即获得这种治疗方法。

If you're interested in helping to answer if ketamine is useful for individuals with Rett Syndrome, please check out [the study](#) on [clinicaltrials.gov](https://clinicaltrials.gov) to learn more about patient eligibility requirements and what centers in the US are participating.

如果您有兴趣帮助回答氯胺酮是否对雷特综合征患者有用，请查看 [clinicaltrials.gov](https://clinicaltrials.gov) 上的研究信息，了解更多关于参与临床试验的患者资格要求，以及位于美国参与研究的中心名单。