

Dear Rett syndrome community,

We are writing to provide you an update on our AVXS-201 program for Rett syndrome. [As we shared previously](#), last year we withdrew our Investigational New Drug (IND) application so that we could initiate additional preclinical (animal) studies. These studies, which included additional pivotal studies and new quality controls, were needed to ensure that we have a robust data package for the U.S. Food and Drug Administration (FDA) when we submit the revised IND application.

We recognize the significant unmet need among patients with Rett syndrome, and understand that these delays were a cause for concern. However, we want to reassure the community that we are fully committed to pursuing a gene therapy for Rett syndrome, and that we are continuing our work with a sense of urgency and purpose.

Today, we are pleased to let you know that most of these preclinical studies are now complete.

Looking ahead to the coming months, we will be working closely with the FDA to progress AVXS-201 forward toward an IND submission. As a reminder, an accepted IND is required before we can proceed to clinical trials for Rett syndrome patients.

We look forward to continuing to collaborate closely with the Rett community, and commit to providing you with updates on a regular basis as we have more information available to share.

Sincerely,

The AveXis 201 Development Team