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Oak Hill Bio

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Dear members of the Angelman syndrome community,

It's my pleasure to introduce Oak Hill Bio. We are a small biotech company focused on developing drugs for rare diseases. We recently entered into an exclusive license agreement with Roche for the rights to develop rugonersen, an antisense oligonucleotide (referred to as an ASO) for the treatment of individuals with Angelman syndrome that was studied in the Phase 1 TANGELO trial. We are excited about the encouraging data generated to date with rugonersen and are working hard to advance its development.

We issued a press release today making this announcement, but we also wanted to directly address this community and try to answer some questions that we anticipate you might have.

We are new to this community but have been getting to know you over the last few months as we have been developing our plans. Even over this short period, we have been moved by the dedication and collaborative spirit of those we have met so far. We are inspired by the remarkable impact the Angelman syndrome community has achieved in advancing scientific understanding, facilitating drug development, and creating a powerful voice for those living with the condition. We were also moved by the experiences some of you shared at the recent externally-led Patient-Focused Drug Development meeting. Finally, we look forward to working closely with you and feel a profound responsibility to make a real difference for the families impacted by Angelman syndrome.

If you have any questions or concerns, please do not hesitate to contact us at PatientAdvocacy@oakhillbio.com.

On behalf of the team at Oak Hill Bio,

Josh Distler
CEO

When will timelines and details on the Phase 3 study design be shared with the community?

While we are currently unable to communicate exact timelines, we are engaging with regulatory agencies like the FDA and are committed to sharing plans with the community as soon as they are ready, including details on the design of the trial.

Will TANGELO patients be able to continue to receive rugonersen?

We are committed to trying to make rugonersen available to patients who are still participating in the TANGELO trial who wish to continue receiving the investigational treatment. We are working with investigators to make this possible. We believe there is likely to be a gap between when the TANGELO trial closes and when we will be able to make rugonersen available through an expanded access program*. We understand how hard this is on patients and their families and are working diligently to minimize this interruption of dosing of rugonersen.

* or similar program depending on geography and the local regulatory pathway

Were there any safety concerns with rugonersen?

Roche has stated that their decision not to progress rugonersen into the next stage of clinical development was not related to safety. We believe that the TANGELO study provided evidence that rugonersen has an adequate risk profile that, when combined with encouraging effects on exploratory measures of brain function, cognition, and communication warrants further development. Through the informed consent process, investigators (study doctors) and interested families will consider the information about both the drug's known and potential risks along with information about the drug's efficacy to make informed decisions about participation in future studies.

When will the results of the TANGELO study be released?

Roche provided interim updates on the study at the ASF Research Symposium in July 2024 and at FAST's Annual Global Science Summit in November 2024. A recording of the FAST presentation is available at https://www.youtube.com/watch?v=fFwlcHYxK-w. A manuscript of the results has been submitted to a peer-reviewed journal and we look forward to sharing it with you upon publication.

How will you communicate updates to families, especially those already in the Tangelo trial?

The families who are currently participating in the TANGELO study will continue to receive updates through their Principal Investigator (study doctor) and the site study team. We plan to work through both patient advocacy groups and clinicians to make sure that families are getting information about continued access and new clinical trials, as well as clinical trial results. We intend to hold one or more webinars in collaboration with patient groups to discuss the details of our Phase 3 trial plan and answer questions from the AS community.

Do you plan to expand trials to include younger/older age groups or other genotypes?

We're still working on our development plan, but our expectation today is that the Phase 3 trial will include mutation and deletion genotypes. We recognize the need for treatment options for patients with other genotypes and will work hard to incorporate them in future trial plans. We haven't finalized our plans with respect to the age of patients eligible for the Phase 3 trial. We expect to announce this when we hold a webinar on the trial later this year.