



## Recap of Session 1: Pharma and Biotech Industry Updates – 7/27/2020

Ovid reminded us about how their drug, OV101, works. They described their ongoing clinical trials, NEPTUNE and ELARA. NEPTUNE is their Phase 3 clinical trial of OV101 and ELARA is the extension study of OV101 to test long-term safety, tolerability, and efficacy.



Roche/Genentech discussed their Freesias and Tangelo studies. Freesias is a study to understand what AS looks like in children prior to receiving study medications. Tangelo is their clinical trial involving their ASO. They are screening potential AS participants and will begin dosing in the next few months.



Biogen discussed their study to measure UBE3A in the CSF (cerebrospinal fluid) in order to determine how well ASOs are turning on UBE3A in the brain.



PTC continues to work hard and make progress on their AAV gene therapy approach. They encourage all families to continue to participate in data collection.



Ionis described their process for identifying promising ASOs and announced that they have found a better candidate ASO. This will delay their trial but they are excited about the new compound and will be in Phase I trials in 2021.



Stridebio introduced us to their AAV gene therapy technology and introduced us to their approach, which will be distinct from other gene therapies currently under investigation. They discussed their next steps with the Angelman community.



Neuren described their development of a compound that mimics the effects of IGF-1, a growth factor critical for normal brain development.



Genetx filled us in about the clinical trial process, including IRBs and FDA's Fast Track designation, as well as the impact that COVID-19 has had on their Phase I/II trial, KIK-AS.

