1) **GeneTx Biotherapeutics, in partnership with Ultragenyx Pharmaceutical, recently announced that the Food and Drug Administration (FDA) has cleared GeneTx's Investigational New Drug (IND) application for clinical trial. What does this mean?**

   a. *The United States Food and Drug Administration's **Investigational New Drug (IND)** program is the means by which a Sponsor obtains permission to start human clinical trials and to ship an experimental drug across state lines (usually to clinical investigators) before a marketing application for the drug has been approved.*

2) **What type of drug is GeneTx bringing to clinical trial?**

   a. *GeneTx will be evaluating GTX-102, an investigational antisense oligonucleotide (ASO).*

3) **What is an ASO and how does GTX-102 work?**

   a. *Antisense oligonucleotides or "ASOs" are short pieces of RNA and/or DNA that are often intended to bind to RNA in the body to increase or decrease the production of protein that is associated with a disease.*
   b. *Investigational GTX-102 is intended to target the father's normally silent copy of the UBE3A gene in an attempt to produce the UBE3A protein that is missing or deficient in the neurons of the brain in people with AS.*

4) **Is GTX-102 considered a gene therapy?**

   a. *No, GTX-102 is not a gene therapy and does not alter the gene thought to be responsible for AS. Investigational GTX-102 is intended to target the father's normally silent copy of the UBE3A gene in an attempt to produce the UBE3A protein that is missing or deficient in the neurons of the brain in people with AS*

5) **GeneTx announced that they will be initiating a Phase 1/2 study called KIK-AS in early 2020. What is a Phase 1/2 study and why is GeneTx's study named KIK-AS?**

   a. A Phase 1 study evaluates the safety , side effects, and various doses of an investigational new drug. A Phase 2 study assesses whether the investigational new drug is potentially effective.
   b. KIK-AS stands for ***K**nockdown of UBE3A-antisense **i**n **k**ids with **A**ngelman **S**yndrome.*

6) **Have ASOs been used in patients with other diseases?**

a. *ASOs have been used in other diseases, some of which are neurological disorders. In 2016 an ASO was approved by FDA for the treatment of a neurological disease called Spinal Muscular Atrophy which is administered by lumbar puncture like the investigational drug in the KIK-AS study. Currently there several clinical trials evaluating investigational ASOs administered by lumbar puncture such as for Huntington's disease, Batten Disease, ALS, and others.*

7) When will GeneTx start the KIK-AS study of GTX-102?

a. *GeneTx anticipates initiating the KIK-AS study in the first half of 2020.*

8) Who can participate in this study?

a. *Information about the KIK-AS study, including eligibility criteria, number of patients that will be enrolled, required assessments, and location of study sites is expected to be available to the community on February 15, 2020.*

9) What is GeneTx Biotherapeutics' role in the KIK-AS study?

a. *GeneTx Biotherapeutics is the Sponsor of the KIK-AS study. As the Sponsor, GeneTx's responsibilities include a number of important elements such as selecting the study investigators; monitoring the progress of the study, ensuring all required regulatory approvals are obtained, and ensuring the study is conducted in accordance with the research plan, etc.*

10) What is the Foundation for Angelman Syndrome Therapeutics' (FAST) role in the KIK-AS study?

a. *FAST has no role in the KIK- AS study*. *FAST funded the preclinical studies of an antisense oligonucleotide for the treatment of Angelman syndrome at the laboratory of Dr. Scott Dindot at Texas A&M University. In December 2017, FAST formed GeneTx Biotherapeutics, LLC and licensed the technology, invented by Dr. Dindot from Texas A&M University Systems.*

11) Who is Ultragenyx Pharmaceutical and what is their role for this study?

a. *Ultragenyx (UGX) is a biopharmaceutical company specializing in rare diseases. They have secured an option to acquire GeneTx and are currently partnered with GeneTx for the development program of GTX-102 and are providing staff support, including strategic guidance and clinical expertise.*