Ionis' statement about the FDA advisory committee meeting on tofersen

On Jan. 23, the Federal Register published a notice that the U.S. Food and Drug Administration will convene a virtual meeting of the Peripheral and Central Nervous System Drugs Advisory Committee to review data supporting the New Drug Application (NDA) for tofersen, an investigational medicine for the treatment of superoxide dismutase 1 amyotrophic lateral sclerosis (SOD1-ALS). The advisory committee meeting is scheduled for March 22, 2023.

SOD1-ALS is an ultra-rare genetic form of ALS that affects approximately 330 people in the U.S.[1] It is progressive, leads to the loss of everyday functions and is uniformly fatal. The NDA for tofersen has a Prescription Drug User Fee Act action date of April 25, 2023.

Additional details about the advisory committee meeting can be found in a separate statement by Biogen which is available on their website.

[1] Brown CA, Lally C, Kupelian V, Flanders WD. Estimated Prevalence and Incidence of Amyotrophic Lateral Sclerosis and SOD1 and C9orf72 Genetic Variants. Neuroepidemiology. 2021