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Ovid Therapeutics Receives Orphan Drug Designation from the U.S. FDA for OV101 for the Treatment of Patients with Angelman Syndrome

NEW YORK--(BUSINESS WIRE)-- Ovid Therapeutics, a privately held biopharmaceutical company based in New York City, announced today that the U.S. Food and Drug Administration has granted orphan drug designation to OV101 for the treatment of patients with Angelman syndrome. OV101 is the first potential therapy to target the disruption of tonic inhibition, a key mechanism that allows the brain to fine-tune neurological signaling and accurately decipher excitatory from inhibitory signals, seen in this disorder.

"Angelman syndrome is a rare, genetic neurological disorder that causes profound developmental and neurologic disabilities. OV101 is the first potential therapy that may address several of the clinical symptoms of Angelman syndrome, such as gait and movement disturbance, anxiety and disrupted sleep patterns," said Dr. Jeremy Levin, chairman and CEO of Ovid Therapeutics. "The granting of orphan drug designation is a significant milestone for the OV101 clinical development program and highlights the high unmet medical need for new therapies that may transform the lives of patients with Angelman syndrome and their families."

Under the U.S. Orphan Drug Act, the FDA's Office of Orphan Products Development provides special status and incentives to encourage the development of drugs for diseases affecting fewer than 200,000 people in the U.S. Orphan drug designation conveys up to seven years of marketing exclusivity if the compound receives regulatory approval from the FDA and offers various development incentives, including tax credits related to clinical trial expenses, an exemption from the FDA-user fee and FDA assistance in clinical trial design. The granting of orphan designation does not alter the standard regulatory requirements, timing and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies.

About Angelman Syndrome

Angelman syndrome is a rare, genetic disorder that causes developmental disabilities and neurologic problems, such as difficulty speaking, balancing and walking, as well as other symptoms such as anxiety, sleep disturbances and seizures. The most common, genetic cause is a deletion of the ubiquitin protein ligase E3A (UBE3A) gene located on chromosome 15. The first signs of Angelman syndrome are usually developmental delays, such as lack of crawling or walking, seen between the ages of 6-12 months.

Patients with Angelman syndrome lack a key mechanism of the brain known as tonic inhibition, which fine-tunes neurological signals to allow a healthy human brain to clearly conduct messages from one nerve to another and thus maintain a normal balance between excitatory and inhibitory signals without the system being overloaded. Tonic

inhibition, a function of the delta (δ)-selective GABA-A receptor, functions to maintain tight control over the level and amount of signaling. When tonic inhibition is “on high,” the brain can decipher signals correctly without being overloaded, but as the level of tonic inhibition is reduced, the brain becomes inundated with signals and loses its deciphering capability.

About OV101

We believe OV101 is the only clinically tested delta (δ)-selective GABA-A receptor agonist and is being developed to help restore tonic inhibition in patients living with Angelman syndrome.

OV101 has the potential to enhance neuronal function to address the pathophysiology of disease. OV101 has been shown in pre-clinical models to normalize tonic inhibition and influence sleep, motor control and cognition, increasing the possibility of improved outcomes for patients.

The STARS trial, a Phase 2, prospective, randomized, double-blind, placebo-controlled clinical trial of OV101 in adults with Angelman syndrome, is currently being initiated in the U.S. in order to assess safety parameters and exploratory efficacy endpoints.

About Ovid Therapeutics Inc.

Ovid Therapeutics Inc. is a privately held, New York-based, biopharmaceutical company committed to transforming the lives of patients with orphan diseases of the brain. Ovid focuses on patients and their unmet medical needs. Using the significant operational, product development, and business development experience of its management team, Ovid aims to become a leading neurology company, with multiple products and a rich pipeline, coupled with compelling research and development. Ovid has raised $80M in financings led by Fidelity Management and Research Company and including Cowen Private Investments, Sanofi-Genzyme BioVentures, Tekla Capital Management, Sphera Global Healthcare Fund, Jennison Associates, Redmile Group, DoubleLine Equity Healthcare Fund, and Cormorant Asset Management, as well as additional blue chip mutual funds and leading life sciences investors.

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