Ovid Therapeutics Announces First Patient Randomized in the Phase 2 STARS Clinical Trial in Adults with Angelman Syndrome

STARS is the first industry-sponsored clinical trial for Angelman syndrome

NEW YORK, Feb. 09, 2017 (GLOBE NEWSWIRE) -- Ovid Therapeutics, a privately held biopharmaceutical company committed to developing medicines that transform the lives of people with rare neurological disorders, today announced it has randomized the first patient in its STARS trial – a Phase 2 clinical trial of OV101 in adults with Angelman syndrome. OV101 (gaboxadol), a delta (δ)-selective $GABA_A$ receptor agonist, is believed to be the first investigational drug to target the disruption of tonic inhibition, a key mechanism that allows a healthy human brain to decipher excitatory and inhibitory neurological signals correctly without being overloaded. Tonic inhibition is believed to play a significant role in the developmental and neurological symptoms characteristic of disorders such as Angelman syndrome and Fragile X syndrome.

“Angelman syndrome is a rare genetic disorder associated with a broad spectrum of symptoms such as development delay, seizures, balance problems and sleep disturbance, resulting in the need for life-long care,” said Lynne Bird, M.D., principal investigator and professor of clinical pediatrics at the University of California San Diego and Rady Children's Hospital-San Diego. “There are currently no FDA approved therapies for the treatment of Angelman Syndrome. The standard of care provides limited treatment options for Angelman syndrome, most of which are general supportive therapies that are used for many other disorders and don’t address the specific pathophysiology of Angelman syndrome. I believe the STARS trial will be the first trial to study an investigative drug that may have the opportunity to address several symptoms that characterize the disorder and that OV101 has the potential to offer a meaningful clinical benefit for people with Angelman syndrome.”

“We are proud to have partnered with the Angelman syndrome community in the design of the STARS trial and thrilled that we have taken an important step in advancing the development of a potential treatment for Angelman syndrome that may benefit the families and individuals living with this disorder,” said Amit Rakhit, M.D., MBA, chief medical and portfolio management officer of Ovid Therapeutics. “OV101 highlights our commitment to developing transformative therapies for people and their families living with rare disorders of the brain.”

About the Phase 2 STARS Trial

The STARS trial is a randomized, double-blind, placebo-controlled Phase 2 clinical trial investigating the safety and efficacy of OV101 that was designed in consultation with the Angelman syndrome community. The trial is expected to enroll approximately 75 adults in the United States aged 18-49 years with a confirmed diagnosis of Angelman syndrome. The primary endpoint of the trial is to assess the safety and tolerability of OV101. Additionally, the trial has several exploratory endpoints to evaluate measures of gross and fine motor skills, maladaptive behavior, sleep, clinical global impression and health-related quality of life questionnaires.

Learn more about the STARS trial at www.clinicaltrials.gov.

About Angelman Syndrome

Angelman syndrome is a rare genetic disorder that is characterized by a variety of signs and symptoms. Characteristic features of this disorder include delayed development, intellectual disability, severe speech impairment, problems with movement and balance, seizures, sleep disorders and anxiety. The most common cause of Angelman syndrome is the disruption of a gene that codes for ubiquitin protein ligase E3A (*UBE3A*). Angelman syndrome affects approximately 1 in 12,000 to 20,000 people in the United States. There are currently no FDA approved therapies for the treatment of Angelman syndrome.

Angelman syndrome is associated with a reduction in tonic inhibition, a function of the delta (δ)-selective $GABA_A$ receptor that allows a human brain to decipher excitatory and inhibitory neurological signals correctly without being overloaded. If tonic inhibition is reduced, the brain becomes inundated with signals and loses the ability to separate background noise from critical information.

About OV101

OV101 (gaboxadol) is a delta (δ)-selective $GABA_A$ receptor agonist and is believed to be the first investigational drug to target the disruption of tonic inhibition, a key mechanism that allows a healthy human brain to decipher excitatory and inhibitory neurological signals correctly without being overloaded. Loss of tonic inhibition is implicated in a host of rare neurological disorders and is established in genetic models. In preclinical models, OV101 has been able to selectively activate the δ-subunit of $GABA_A$ receptors, which are found in the extrasynaptic space (outside of the synapse), and helped regulate neuronal activity through tonic inhibition.

Ovid is developing OV101 for use in both Angelman syndrome and Fragile X syndrome to potentially restore tonic inhibition and relieve several of the symptoms of these disorders. In preclinical studies, it was observed that OV101 improved symptoms of Angelman syndrome and Fragile X syndrome.

In September 2016, the United States Food and Drug Administration granted orphan drug designation for OV101 for the treatment of Angelman syndrome. The United States Patent and Trademark Office has granted Ovid two patents directed to methods of treating Angelman syndrome using THIP (OV101). The issued patents expire in 2035, without regulatory extensions.

About Ovid Therapeutics

Ovid Therapeutics is a privately held, New York-based, biopharmaceutical company using its BoldMedicine™ approach to develop therapies that transform the lives of patients with rare neurological disorders. Ovid's drug candidate, OV101, is currently in development for the treatment of symptoms of Angelman syndrome and Fragile X syndrome. Ovid is also developing OV935 in collaboration with Takeda Pharmaceutical Company Limited for the treatment of rare epileptic encephalopathies. Ovid has initiated a Phase 2 STARS trial of OV101 in adults with Angelman syndrome and Ovid intends to commence a Phase 1 trial in adolescents with Angelman syndrome or Fragile X syndrome. OV935 is expected to commence a Phase 1b/2a trial in rare epileptic encephalopathies in 2017.

For more information, visit http://www.ovidrx.com.

Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements. Forward-looking statements contained in this press release include, without limitation, statements regarding Ovid's expectations regarding the clinical development of OV101, the clinical benefits of OV101 for people with Angelman syndrome and the expected enrollment in the STARS trial. Words such as "may," "believe," "will," "expect," "plan," "anticipate," "estimate," "intend" and similar expressions (as well as other words or expressions referencing future events, conditions or

circumstances) are intended to identify forward-looking statements. These forward-looking statements are not guarantees of future performance and involve a number of unknown risks, assumptions, uncertainties and factors that are beyond Ovid's control. All forward-looking statements are based on Ovid's expectations and assumptions as of the date of this press release. Actual results may differ materially from these forward-looking statements. Except as required by law, Ovid expressly disclaims any responsibility to update any forward-looking statement contained herein, whether as a result of new information, future events or otherwise.

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