Ovid Therapeutics Announces Initiation of Phase 1 Clinical Trial of OV101 for Adolescents with Angelman Syndrome or Fragile X Syndrome

Pediatric Development is Planned Upon Completion of Preclinical Testing

NEW YORK, April 10, 2017 (GLOBE NEWSWIRE) -- Ovid Therapeutics, Inc., a privately held biopharmaceutical company committed to developing medicines that transform the lives of people with rare neurological diseases, today announced that it has initiated a Phase 1 clinical trial to evaluate the pharmacokinetics (PK), safety and tolerability of OV101 in adolescents diagnosed with Angelman syndrome or Fragile X 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 neurodevelopmental symptoms characteristic of disorders such as Angelman syndrome and Fragile X syndrome.

“Those with neurodevelopmental disorders such as Angelman syndrome and Fragile X syndrome are affected from birth. Our goal is to provide medicines to these individuals at all ages. The initiation of this trial represents the first step in our strategy to develop OV101 for an adolescent population. This is an important step because having access to medicines at a younger age has the potential to have a transformative effect on the lives of these patients and their families,” said Amit Rakhit, M.D., MBA, chief medical and portfolio officer of Ovid Therapeutics. “We look forward to continuing to partner with the Angelman and Fragile X syndrome communities as we work to develop therapies that can have the greatest impact on the challenges they face every day.”

The Phase 1 single dose, single-arm, open-label, clinical trial of OV101 will measure PK parameters. The trial is expected to enroll adolescent patients, ages 13 to 17, who have been diagnosed with either Angelman syndrome or Fragile X syndrome. Additional details on the Phase 1 clinical trial can be found at www.clinicaltrials.gov.

In addition to this clinical trial in adolescents, Ovid is planning to initiate clinical development in a pediatric population upon completing the required preclinical testing.

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 United States Food and Drug Administration (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 Fragile X Syndrome

Fragile X syndrome is the most common inherited form of intellectual disability and autism, with a prevalence of 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females in the United States. Individuals with Fragile X syndrome often have a range of behavioral challenges, such as cognitive impairment, anxiety, mood swings, hyperactivity, attention deficit, poor sleep, self-injury and heightened sensitivity to various stimuli, such as sound. Additionally, individuals with Fragile X syndrome are prone to comorbid medical issues including seizures and sleep disturbance. Fragile X syndrome results from mutations in the *FMR1* gene, which blocks expression of the Fragile X Mental Retardation Protein (FMRP), an important protein in GABA synthesis. There are no FDA-approved therapies for Fragile X syndrome, and treatment primarily consists of behavioral interventions and pharmacologic management of symptoms.

In studies of individuals with Fragile X syndrome and in experimental models, extrasynaptic GABA levels are abnormally reduced, and there is also dysregulation of GABA receptors. This ultimately contributes to a decrease in tonic inhibition, causing the brain to become inundated with signals and lose 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. 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 help 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 FDA 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 diseases. Ovid's drug candidate, OV101, is currently in development for the treatment of symptoms of Angelman syndrome and Fragile X syndrome. Ovid has initiated the Phase 2 STARS trial of OV101 in adults with Angelman syndrome and a Phase 1 trial in adolescents with Angelman syndrome or Fragile X syndrome. Ovid is also developing OV935 in collaboration with Takeda Pharmaceutical Company Limited for the treatment of rare epileptic encephalopathies. OV935 is expected to initiate a Phase 1b/2a trial in rare epileptic encephalopathies in 2017.

For more information on Ovid, please 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 for the treatment of Angelman syndrome or Fragile X syndrome, the clinical benefits of OV101 for people with Angelman syndrome or Fragile X syndrome, the expected enrollment in the Phase 1 trial and the timing of clinical trials. 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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