Ovid Therapeutics Announces OV101 Shows Comparable PK Profile in Phase 1 Study of Adolescent Patients and Amends Phase 2 STARS Clinical Trial Protocol to Include Adolescents

-- Results from the Phase 1 Clinical Trial of Adolescents with Angelman and Fragile X Syndromes Demonstrated OV101 was Well Tolerated with a Pharmacokinetic Profile Similar to Adults --

-- With Core Expertise in Neurology and Pediatrics, Ovid's Focus is to Develop Therapies for Younger Patient Populations That May Benefit from Early Interventions --

NEW YORK, Nov. 28, 2017 (GLOBE NEWSWIRE) -- Ovid Therapeutics Inc. (NASDAQ:OVID), a biopharmaceutical company committed to developing medicines that transform the lives of people with rare neurological diseases, today announced that upon successful completion of the Phase 1 clinical trial to evaluate the pharmacokinetics (PK), safety and tolerability of OV101 in adolescents diagnosed with Angelman syndrome or Fragile X syndrome, the Company will now include adolescent patients aged 13 years and older in the Phase 2 STARS trial.

The Phase 1 PK study was strategically designed to assess the PK profile of OV101, an agonist of the extrasynaptic GABAA receptor, in adolescent patients. While OV101 has an extensive safety database of over 4,000 adults, the Phase 1 clinical trial represented the first time adolescents were given OV101. In the trial, OV101 was found to be generally safe and well tolerated and its PK profile in adolescents was similar to previous data generated in young adults, supporting the inclusion of adolescent patients in the STARS clinical trial.

"This first clinical data of OV101 in adolescents in genetically-defined disorders with GABA hypofunction provide important information to our overall development strategy and are an important step to enable development of OV101 in younger ages," said Amit Rakhit, M.D., MBA, chief medical and portfolio officer of Ovid Therapeutics. "Angelman syndrome and Fragile X syndrome are disorders that impact patients from birth and our goal is to develop a therapy for a broad range of patient age groups. At Ovid, we strive to provide access to effective therapies as early as possible and we are now able to offer enrollment in our STARS trial to adolescent Angelman syndrome participants. With the inclusion of adolescents, we now anticipate data from the STARS trial to be available in the second half of 2018."

“We have assembled a team at Ovid that not only has deep expertise in neurology but also significant experience developing therapies for children. Part of our core strategy is to rapidly develop our medicines for younger patients, which is a patient population usually addressed late in the drug development process,” said Jeremy Levin, D.Phil., MB Chir, chairman of the board of directors and chief executive officer of Ovid Therapeutics. “Data from our PK study combined with beginning enrollment of adolescent Angelman syndrome patients is an example of how we are executing that strategy. We will continue to execute in a disciplined manner against our goals to develop impactful therapies, particularly for children.”

About the Phase 1 Trial

The Phase 1 single dose, single-arm, open-label clinical trial of OV101 enrolled seven male and five female adolescent patients, aged 13 to 17, who had been diagnosed with either Angelman syndrome or Fragile X syndrome. Participants received a single 5mg oral dose of OV101. Overall results of the PK study met the objectives and showed that PK parameters in adolescents with Angelman and Fragile X syndrome were not significantly different from previous data generated in young adults. Additional details on the Phase 1 clinical trial can be found at www.clinicaltrials.gov.

Ovid recently presented strong preclinical data on the impact of OV101 on animal models of Fragile X syndrome and has also received FDA orphan drug designation for OV101 for both Fragile X syndrome and Angelman syndrome. Coupled with the favorable Phase 1 data, this forms a solid foundation for the overall strategy of developing OV101 for younger age groups in both populations.

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 patients in the United States and Israel aged 13 to 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 and www.angelmanstudy.com.

About Angelman Syndrome

Angelman syndrome is a 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 U.S. 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 GABAA 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 believed to be the only delta (δ)-selective GABAA receptor agonist in development and the first investigational drug to specifically target the disruption of tonic inhibition, a central physiological process of the brain that is thought to be the underlying cause of certain neurodevelopmental disorders. OV101 has been demonstrated in laboratory studies and animal models to selectively activate the δ-subunit of GABAA receptors, which are found in the extrasynaptic space (outside of the synapse), and thereby impact neuronal activity through tonic inhibition.

Ovid is developing OV101 for the treatment of 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. Gaboxadol has previously been tested in over 4,000 patients (approximately 950 patient-years of exposure) and was observed to have favorable safety and bioavailability profiles.

The FDA has granted orphan drug designation for OV101 for the treatment of both Angelman syndrome and Fragile X syndrome. The United States Patent and Trademark Office has granted Ovid patents directed to methods of treating Angelman syndrome using OV101. The issued patents expire in 2035 for Angelman syndrome.

About Ovid Therapeutics

Ovid Therapeutics (NASDAQ:OVID) is a 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 Angelman syndrome and Fragile X syndrome. Ovid has initiated the Phase 2 STARS trial of OV101 in people with Angelman syndrome in 2017 and completed 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 epileptic encephalopathies and in August 2017 initiated a Phase 1b/2a trial of OV935.

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

Forward-Looking Statements

This press release includes certain disclosures that contain “forward-looking statements,” including, without limitation, statements regarding progress, timing, scope and results of clinical trials for Ovid’s product candidates, the timing of clinical data, the development of therapies for younger patients, the provision of access to effective therapies, and the execution of Ovid goals for not only OV101 but also OV935. You can identify forward-looking statements because they contain words such as “will,” “believes” and “expects.” Forward-looking statements are based on Ovid’s current expectations and assumptions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that may differ materially from those contemplated by the forward-looking statements, which are neither statements of historical fact nor guarantees or assurances of future performance. Important factors that could cause actual results to differ materially from those in the forward-looking statements are set forth in Ovid’s filings with the Securities and Exchange Commission, including its Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, under the caption “Risk Factors.” Ovid assumes no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.

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