Ovid Therapeutics Announces Positive Preclinical OV101 Data Demonstrating Behavioral Improvements in Fragile X Syndrome Model

NEW YORK, Oct. 16, 2017 (GLOBE NEWSWIRE) -- Ovid Therapeutics Inc. (NASDAQ:OVID), a biopharmaceutical company committed to developing medicines for patients with rare neurological diseases, today announced new positive preclinical data on OV101 that shows normalization of behavioral abnormalities that resemble those seen in people with Fragile X syndrome. The improvements seen with OV101, a novel agonist of the extrasynaptic GABA receptor, were consistent across multiple behavioral measures in a model of Fragile X syndrome. The data were presented at the 18th International Fragile X and Related Neurodevelopmental Disorders Workshop.

“Treatment with OV101 results in significant behavioral improvements that are consistent across all behavioral endpoints tested,” said Matthew During, M.D., DSc, FACP, FRACP, president and chief scientific officer of Ovid Therapeutics. “We designed this study to deepen our understanding of the mechanisms of action and potential benefit of OV101 to treat patients with Fragile X syndrome.”

In the study, researchers demonstrated that acute administration of 0.5 mg/kg of OV101 to *Fmr1* knockout mice fully normalized behavioral abnormalities relevant to Fragile X syndrome (hyperactivity, anxiety, irritability and aggression, and restricted and repetitive behaviors). All effects were highly statistically significant ($p < 0.001$). It is believed that symptoms of Fragile X syndrome are a result of disrupted tonic inhibition, the key to the brain's ability to discriminate signal from noise. The results presented indicate that by specifically targeting the delta (δ)-subset of GABA receptors, OV101 may be able to alleviate symptoms of Fragile X syndrome by modulating the GABA pathway and restoring tonic inhibition.

“We have built a strong foundation in scientific analysis, translational medicine, drug development and regulatory capabilities. This study builds upon this foundation and underscores the potential role of OV101 in modulating tonic inhibition, an important underlying mechanism in certain neurodevelopmental disorders,” said Amit Rakhit M.D., MBA, chief medical and portfolio officer of Ovid Therapeutics. “Together with the recent FDA orphan drug designation for OV101 for the treatment of Fragile X syndrome, this data is another important step in our disciplined strategy to develop OV101 as a potential first in class compound for Fragile X syndrome.”

About OV101
OV101 (gaboxadol) is believed to be the only delta (δ)-selective $GABA_A$ receptor agonist in development and the first investigational drug to specifically target the disruption of tonic inhibition 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 $GABA_A$ 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. To date, gaboxadol has 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 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 two patents directed to methods of treating Angelman syndrome using OV101. The issued patents expire in 2035, without regulatory extensions.

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 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 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 and has 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 reporting of clinical data regarding Ovid's product candidates, the development of OV101 as a potential first in class compound for Fragile X syndrome and the potential use of TAK-935/OV935 to treat rare epilepsies. 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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