Ovid Therapeutics Receives Orphan Drug Designation from the U.S. FDA for OV101 for Treatment of Fragile X Syndrome

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NEW YORK, Oct. 10, 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 the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for OV101 for the treatment of Fragile X syndrome. OV101, a delta (d)-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. OV101 has already received orphan drug designation for the treatment of Angelman syndrome.

“We are delighted to receive orphan drug designation for OV101 for the treatment of Fragile X syndrome. The rapid action from the FDA on this decision mirrors our own urgency for developing OV101 as a potential new therapy for people living with Fragile X syndrome, a patient population in desperate need of new treatment options,” said Matthew During M.D., DSc, FACP, FRACP, president and chief scientific officer of Ovid Therapeutics. “This is an important step in the continued advancement of our Fragile X syndrome program and furthers our mission to develop impactful medicines for people with rare neurological disorders.”

Orphan drug designation, which is intended to facilitate drug development for rare diseases, provides substantial benefits to the sponsor, including the potential for tax credits for clinical development costs, study-design assistance, and several years of market exclusivity for the product upon regulatory approval.

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 $GABA_A$ 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.

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 OV101. The issued patents expire in 2035, without regulatory extensions.

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 the continued advancement of the Fragile X syndrome program and the development of impactful medicines for people with rare neurological diseases. 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.

Contacts

Investors:
Burns McClellan
Steve Klass, 212-213-0006
Sklass@burnsmc.com

Media:
Pure Communications, Inc.
Katie Engleman, 910-509-3977
katie@purecommunicationsinc.com

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