Ovid Therapeutics Announces OV101 Granted Fast Track Designation by U.S. FDA for Treatment of Angelman Syndrome

NEW YORK, Dec. 19, 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 Fast Track designation to OV101 for the treatment of Angelman syndrome.

“This designation is an important milestone for both Ovid and the Angelman community as it enables increased dialog with the FDA, speeding our ability to bring this potential therapeutic option to people living with Angelman syndrome. We believe that OV101, with its novel mechanism of action, has the potential to be an innovative and impactful therapy,” said Amit Rakhit, M.D., MBA, chief medical and portfolio management officer of Ovid Therapeutics. “In addition to the regulatory milestones of orphan drug and Fast Track designations for Angelman syndrome, we achieved significant clinical progress with our OV101 program. As a result of positive Phase 1 data, we were recently able to expand our ongoing Phase 2 STARS clinical trial to include both adults and adolescents with Angelman syndrome. We look forward to data from the STARS trial in the second half of 2018.”

OV101 is a delta (δ)-selective GABAA receptor agonist that targets the disruption of tonic inhibition, a central physiological process of the brain that is thought to be the underlying cause of Angelman syndrome and other neurodevelopmental disorders. Ovid is currently studying OV101 in its Phase 2 STARS clinical trial, a randomized, double-blind, placebo-controlled study investigating the safety and efficacy of OV101 in patients with Angelman syndrome. Upon successful completion of a Phase 1 pharmacokinetic (PK) and safety study showing that OV101 has a similar PK profile in adolescents as in adults, Ovid recently amended the STARS protocol to include patients aged 13 years and older.

The FDA’s Fast Track process is designed to expedite the development and review of drugs used to treat serious conditions and fill an unmet medical need. Fast Track designation enables the company to have early and frequent communication with the FDA throughout the drug development and review process, often leading to faster drug approval and patient access.

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 and Fast Track designations for OV101 for the treatment of Angelman syndrome and orphan drug designation for the treatment of Fragile X syndrome. The U.S. 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 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 U.S. 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 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 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 FDA Fast Track process leading to faster drug approval and patient access. 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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