Ovid Therapeutics Receives FDA Rare Pediatric Disease Designation for OV101 for the Treatment of Angelman Syndrome

June 19, 2020

NEW YORK, June 19, 2020 (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 Rare Pediatric Disease Designation to OV101 (gaboxadol) for the treatment of Angelman syndrome. OV101 is believed to be the only delta (δ)-selective $GABA_A$ receptor agonist in development and is currently being evaluated in the Company's pivotal Phase 3 NEPTUNE trial in Angelman syndrome, with topline results expected in the fourth quarter of 2020. The FDA has previously granted Orphan Drug and Fast Track designations for OV101 for the treatment of Angelman syndrome.

Under the Creating Hope Act passed into federal law in 2012, the FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the U.S. If a new drug application (NDA) for OV101 in Angelman syndrome is approved, Ovid may be eligible to receive a priority review voucher from the FDA, which can be redeemed to obtain priority review for any subsequent marketing application or may be transferred and/or sold to other companies for their programs, such as has recently been done by other voucher recipients.

"OV101 has the potential to become the first FDA-approved therapy for individuals living with Angelman syndrome. Receiving Rare Pediatric Disease Designation from the FDA is a significant milestone for this program and underscores the critical value of our work," said Amit Rakhit, M.D., MBA, President and Chief Medical Officer of Ovid Therapeutics. "Importantly, with this designation, we may be eligible to receive a priority review voucher from the FDA, providing significant value as we work diligently towards the completion of our ongoing pivotal Phase 3 NEPTUNE trial. We are grateful to the FDA and Congress for having enacted this law which helps Ovid and all companies developing innovative drugs for rare pediatric conditions."

About Angelman Syndrome
Angelman syndrome is a rare genetic condition that is characterized by a variety of signs and symptoms. Characteristic features of this condition 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 loss of function of the gene that codes for ubiquitin protein ligase E3A (UBE3A), which plays a critical role in nerve cell communication, resulting in impaired tonic inhibition. Individuals with Angelman syndrome typically have normal lifespans but are unable to live independently. Therefore, they require constant support from a network of specialists and caregivers. Angelman syndrome affects approximately 1 in 12,000 to 1 in 20,000 people globally.

There are no approved therapies by the FDA, European Medicines Agency (EMA), or rest of world for Angelman syndrome, and treatment primarily consists of behavioral interventions and pharmacologic management of symptoms.

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 OV101 (gaboxadol)
OV101 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, a central physiological process of the brain that is thought to be the underlying cause of certain neurodevelopmental disorders. OV101 has 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 modulation of tonic inhibition.

Ovid is developing OV101 for the treatment of Angelman syndrome and Fragile X syndrome to potentially restore tonic inhibition and thereby address several core symptoms of these conditions. In both these syndromes, the underlying pathophysiology includes disruption of tonic inhibition modulated through the δ-subunit of $GABA_A$ receptors. In preclinical studies, it was observed that OV101 improved symptoms of Angelman syndrome and Fragile X syndrome. This compound has also previously been tested in more than 4,000 patients (more than 1,000 patient-years of exposure) and was observed to have favorable safety and bioavailability profiles. Ovid is conducting a pivotal Phase 3 clinical trial with OV101 in Angelman syndrome (NEPTUNE) and has completed a Phase 2 signal-finding clinical trial with OV101 in Fragile X syndrome (ROCKET).

OV101 has received Rare Pediatric Disease Designation from the FDA for the treatment of Angelman syndrome. The FDA has also granted Orphan Drug and Fast Track designations for OV101 for both the treatment of Angelman syndrome and Fragile X syndrome. In addition, the European Commission (EC) has granted orphan drug designation to OV101 for the treatment of Angelman syndrome. The U.S. Patent and Trademark Office has granted Ovid patents directed to methods of treating Angelman syndrome and Fragile X syndrome using OV101. The issued patents expire in 2035 without regulatory extensions.

About Ovid Therapeutics
Ovid Therapeutics Inc. is a New York-based biopharmaceutical company using its BoldMedicine® approach to develop medicines that transform the lives of patients with rare neurological disorders. Ovid has a broad pipeline of potential first-in-class medicines. The Company's most advanced investigational medicine, OV101 (gaboxadol), is currently in clinical development for the treatment of Angelman syndrome and Fragile X syndrome. Ovid is also developing OV935 (soticlestat) in collaboration with Takeda Pharmaceutical Company Limited for the potential treatment of rare developmental and epileptic encephalopathies (DEE). 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: advancing and commercializing Ovid’s product candidates, progress, timing, scope and the development and potential benefits of Ovid’s product candidates; and the anticipated reporting schedule of clinical data regarding Ovid’s product candidates. You can identify forward-looking statements because they contain words such as “will,” “appears,” “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 include uncertainties in the development and regulatory approval processes, and the fact that initial data from clinical trials may not be indicative, and are not guarantees, of the final results of the clinical trials and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. Additional risks 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 under the caption “Risk Factors”. Such risks may be amplified by the COVID-19 pandemic and its potential impact on Ovid’s business and the global economy. 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 and Media:
Ovid Therapeutics Inc.
Investor Relations & Public Relations
irpr@ovidrx.com

Or

Investors:
Steve Klass
Burns McClellan, Inc.
sklass@burnsmc.com
(212) 213-0006

Media:
Katie Engleman
1AB
katie@1abmedia.com

Source: Ovid Therapeutics Inc.