Ovid Therapeutics Announces Initiation of Phase 2 ROCKET Clinical Trial to Evaluate OV101 in Fragile X Syndrome

July 11, 2018

Phase 2 trial will evaluate OV101 for 12 weeks in up to 30 males aged 13 to 22

NEW YORK, July 11, 2018 (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 it has initiated the Phase 2 ROCKET clinical trial evaluating OV101 for the treatment of adolescents and young adult males with Fragile X syndrome.

OV101 is a novel 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 Fragile X syndrome and other neurodevelopmental disorders. It is believed that OV101 is the first investigational medicine to target the disruption of tonic inhibition.

"Fragile X syndrome is a rare neurogenetic condition associated with distinctive behavior characteristics and challenges, including anxiety and hyperactivity, and compounded with learning disabilities," said Randi Hagerman, M.D., principal investigator, developmental and behavioral pediatrician and medical director of the MIND Institute at the University of California Davis. "Despite being the most common inherited form of intellectual disability, there are currently no approved therapies for the disorder. Current standard of care for individuals with Fragile X syndrome is limited, relying primarily on symptomatic treatments. Through this study, we hope to confirm the safety of OV101 and observe effects on behavioral endpoints in a Phase 2 trial in Fragile X syndrome."

"We are pleased to advance our Fragile X program into a Phase 2 trial and to have taken the next important step in potentially developing a much needed treatment option for people living with this condition," said Amit Rakhit, M.D., MBA, chief medical and portfolio management officer of Ovid Therapeutics. "At Ovid, we understand the critical role of tonic inhibition in neurological disorders and believe that our deep insights provide us the opportunity to develop OV101 as a potentially transformative medicine for the Fragile X community."

About the ROCKET Trial

The ROCKET trial is a Phase 2 randomized, double-blind, parallel-group, trial to evaluate the safety, tolerability and efficacy of OV101 in up to 30 males age 13 to 22 with a confirmed diagnosis of Fragile X syndrome. The primary endpoint of the study is safety and tolerability of OV101 over 12 weeks of treatment in three different cohorts. The secondary efficacy endpoint will evaluate changes in behavior during 12 weeks of treatment.

In addition to Fragile X syndrome, Ovid is also developing OV101 for the treatment of Angelman syndrome. Topline data from the company's Phase 2 STARS clinical trial investigating OV101 for the treatment of adults and adolescents with Angelman syndrome are expected in the third quarter of 2018.

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 medicine 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 (with more than 1000 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 both the treatment of Angelman syndrome and Fragile X 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.

About Ovid Therapeutics

Ovid Therapeutics (NASDAQ:OVID) is a New York-based biopharmaceutical company using its BoldMedicine™ approach to develop medicines that transform the lives of people with rare neurological disorders. Ovid has a broad pipeline of first-in-class medicines. The company's lead investigational
medicine, OV101, is currently in development for the treatment of Angelman syndrome and Fragile X syndrome. Ovid is also developing TAK-935/OV935 in collaboration with Takeda Pharmaceutical Company Limited for the treatment of rare developmental and epileptic encephalopathies (DEE).

For more information on Ovid, please visit [http://www.ovidrx.com/](http://www.ovidrx.com/).

**Forward-Looking Statements**

This press release includes certain disclosures that contain “forward-looking statements,” including, without limitation, statements regarding (i) progress, timing, scope and results of clinical trials for Ovid’s product candidates, and (ii) the potential clinical benefit of OV101 to treat patients with Fragile X. 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 period ended March 31, 2018 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**

<table>
<thead>
<tr>
<th>Investors:</th>
<th>Media:</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lora Pike</td>
<td>W2O Group</td>
</tr>
<tr>
<td>Ovid Therapeutics, Inc.</td>
<td>Kelly Boothe, Ph.D.</td>
</tr>
<tr>
<td>Senior Director, Investor Relations &amp; Public Relations</td>
<td>+1-415-946-1076</td>
</tr>
<tr>
<td><a href="mailto:lpike@ovidrx.com">lpike@ovidrx.com</a></td>
<td><a href="mailto:kboothe@w2ogroup.com">kboothe@w2ogroup.com</a></td>
</tr>
</tbody>
</table>

Ovid Therapeutics Inc.