Ovid Therapeutics Announces TAK-935/OV935 Granted Orphan Drug Designation by U.S. FDA for Treatment of Lennox-Gastaut Syndrome

Represents the second orphan drug designation for this compound being co-developed by Ovid and Takeda

NEW YORK, Dec. 20, 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 United States Food and Drug Administration (FDA) has granted orphan drug designation to TAK-935/OV935 for the treatment of Lennox-Gastaut syndrome, a type of developmental and epileptic encephalopathy. Takeda Pharmaceutical Company Limited and Ovid formed a global collaboration in January 2017 to develop and commercialize TAK-935/OV935 for the treatment of developmental and epileptic encephalopathies, a group of rare epilepsies that cause significant morbidities and can worsen over time.

Lennox-Gastaut syndrome is a rare disorder that is often diagnosed between three and five years of age. Patients diagnosed with Lennox-Gastaut syndrome experience multiple seizure types that are difficult to manage and have many of the same symptoms as other rare pediatric epilepsies.

“The FDA’s decision to grant orphan drug designation to TAK-935/OV935 for the treatment of Lennox-Gastaut syndrome reflects the Agency’s recognition of the urgent need for additional treatments for this severe type of epilepsy,” said Matthew During, M.D., DSc, FACP, FRACP, president and chief scientific officer of Ovid Therapeutics. “This orphan drug designation is the fourth one we have received in two years for our two lead investigational products, underscoring our commitment to develop impactful medicines for people living with rare neurological disorders and our ability to rapidly advance our programs.”

TAK-935/OV935 is a potent, highly-selective, first-in-class inhibitor of the enzyme cholesterol 24-hydroxylase (CH24H). It is believed that CH24H is involved in over-activation of the glutamatergic pathway, which has been shown to play a role in the initiation and spread of seizure activity. To Ovid and Takeda's knowledge, TAK-935/OV935 is the only molecule with this mechanism of action in clinical development.

"TAK-935/OV935 has a novel mechanism of action and could be a potentially transformative therapy for individuals diagnosed with rare epilepsies, such as Lennox-Gastaut syndrome," said Dr. Emiliangelo Ratti, head of Takeda's Neuroscience Therapeutic Area Unit. "The granting of orphan drug designation is an important milestone for the clinical development program for TAK-935/OV935, which we are currently evaluating in a Phase 1b/2a clinical trial with data anticipated in 2018."

Orphan drug designation is intended to facilitate and expedite drug development for rare diseases for which there are no current treatments available. It also 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 Lennox-Gastaut Syndrome
Lennox-Gastaut syndrome is one of several disorders that together are designated as developmental and epileptic encephalopathies. Studies estimate that Lennox–Gastaut syndrome affects approximately 14,500 to 18,500 children under the age of 18 and over 30,000 children and adults in the United States. It is also estimated that between 1 percent and 4 percent of childhood epilepsies are a result of Lennox–Gastaut syndrome. Only 10 percent of these patients have seizures that are fully controlled by existing therapies.

Developmental and epileptic encephalopathies include epilepsy syndromes associated with severe cognitive and behavioral disturbances. The International League Against Epilepsy (ILAE) defines an epileptic encephalopathy as a condition in which "the epileptiform EEG abnormalities themselves are believed to contribute to a progressive disturbance in cerebral function."

These epilepsies cause significant morbidities for patients beyond what might be expected from the known underlying pathology alone and can worsen over time. Developmental and epileptic encephalopathies typically present early in life and are often associated with severe cognitive and developmental impairment in addition to frequent treatment-resistant seizures throughout the person's lifetime. These disorders vary in age of onset, developmental outcomes, etiologies, neuropsychological deficits, electroencephalographic (EEG) patterns, seizure types and prognosis.

About TAK-935/OV935
TAK-935/OV935, which is being studied in developmental and epileptic encephalopathies, is a potent, highly-selective, first-in-class inhibitor of the enzyme cholesterol 24-hydroxylase (CH24H). CH24H is predominantly expressed in the brain, where it plays a central role in cholesterol homeostasis. CH24H converts cholesterol to 24*S*-hydroxycholesterol (24HC), which then exits the brain into the blood plasma circulation. Glutamate is one of the main neurotransmitters in the brain and has been shown to play a role in the initiation and spread of

seizure activity. Recent literature indicates 24HC is involved in over-activation of the glutamatergic pathway through modulation of the NMDA channel, implying its potential role in central nervous system diseases such as epilepsy. To Ovid and Takeda's knowledge, TAK-935/OV935 is the only molecule with this mechanism of action in clinical development.

TAK-935/OV935 has been tested in preclinical models to provide data to support the advancement of the drug into human clinical studies in patients suffering from rare epilepsy syndromes. A novel proprietary PET ligand, developed by Takeda and Molecular Neuroimaging, LLC (MNI), has been used to determine target enzyme occupancy of TAK-935/OV935 in the brain. In addition, the effect of TAK-935/OV935 on CH24H enzyme activity in the brain has been assessed by following measurable reductions in the plasma concentration of 24HC.

TAK-935/OV935 has completed four Phase 1 clinical studies, which have assessed tolerability and target engagement at doses believed to be therapeutically relevant. The FDA has granted orphan drug designation for TAK-935/OV935 for the treatment of both Dravet syndrome and Lennox-Gastaut syndrome. TAK-935/OV935 is being co-developed by Ovid and Takeda Pharmaceutical Company Limited.

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 the progress, timing, scope and results of clinical trials for Ovid's product candidates, the reporting of clinical data regarding Ovid's product candidates, 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 September 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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