Ovid Therapeutics Announces TAK-935/OV935 Has Received Orphan Drug Designation from U.S. FDA for Treatment of Dravet Syndrome

NEW YORK, Dec. 05, 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 to TAK-935/OV935 for the treatment of Dravet syndrome, a severe and rare form of childhood epilepsy that typically presents during the first year of life. Takeda Pharmaceutical Company Limited and Ovid formed a global collaboration to develop and commercialize TAK-935/OV935 for the treatment of developmental and epileptic encephalopathies in January 2017.

Dravet syndrome is classified as a developmental and epileptic encephalopathy, a group of rare epilepsies that cause significant morbidities and can worsen over time. Children with Dravet syndrome experience frequent seizures, loss of muscle control, cognitive deficits and, in approximately 10 percent of cases, death before the age of 12 years. Moreover, in those who survive into adulthood, their long-term intellectual development and seizure outcomes are typically extremely poor.

“We are pleased by the FDA’s decision to grant orphan drug designation to TAK-935/OV935 for the treatment of Dravet syndrome, a severe and debilitating disease,” said Dr. Emiliangelo Ratti, head of Takeda’s Neuroscience Therapeutic Area Unit. “This designation is a significant step forward in researching a potential treatment option for people living with Dravet syndrome for whom therapeutic options are severely limited, and an important milestone for this investigational molecule.”

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.

“We believe that TAK-935/OV935, with its novel mechanism of action, has the potential to be an innovative treatment for people with rare epilepsies, such as Dravet syndrome,” said Matthew During M.D., DSc, FACP, FRACP, president and chief scientific officer of Ovid Therapeutics. “We have

rapidly advanced this program into a Phase 1b/2a clinical trial and anticipate data in 2018. We look forward to continuing our work with Takeda to bring this potentially transformative therapy to patients."

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 Dravet Syndrome
Dravet syndrome is a severe form of childhood epilepsy that typically presents during the first year of life. It is believed to be largely caused by mutations in the SCN1A gene. Children experience frequent seizures, loss of muscle control, cognitive deficits and, in approximately 10 percent of cases, death before the age of 12 years. While some patients may survive into adulthood, their long-term intellectual development and seizure outcomes are typically extremely poor. The incidence of Dravet syndrome in the United States ranges from 1 in 15,700 to 1 in 20,900 births. Patients are frequently treated with combinations of classic anti-epileptic drugs, none of which are particularly effective. No drugs have been approved specifically for the treatment of Dravet syndrome in the United States and only one drug, the anticonvulsant stiripentol, has been approved in Europe.

Dravet syndrome is one of several disorders which together are designated as developmental and epileptic encephalopathies. This group includes 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 24S-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. 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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