Ovid Therapeutics Receives “Company Making a Difference Award” from CDKL5 Deficiency Disorder Community

NEW YORK, Oct. 22, 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 it has received the 2018 CDKL5 Forum *Company Making a Difference Award* for initiation of the Phase 2 ARCADE trial with OV935/TAK-935, and its commitment to the CDKL5 deficiency disorder (CDD) patient community. The award was announced today in London, UK at the CDKL5 Forum, the largest annual conference on the advancement of science and therapeutic development for CDD.

CDD is a rare, severe, neurological disorder that causes frequent, treatment-resistent seizures in the first few months of life. CDD results in a constellation of severe challenges, including developmental delay and intellectual disability, movement disorder, difficulty sleeping, scoliosis, visual impairment, microcephaly and various gastrointestinal difficulties. There are currently no FDA-approved therapies for CDD.

“We hand out the *Company Making a Difference Award* annually to recognize industry partners for their commitment and support, and this year we are pleased to recognize the team at Ovid Therapeutics,” said Ana Mingorance, Ph.D., chief development officer of Loulou Foundation. “Since the day Ovid selected CDD as a condition to pursue, they have shown amazing support and care for our community, and we are excited to partner with such a human-centric organization.”

The ARCADE trial is a Phase 2 multi-center, open-label, pilot study designed to evaluate the treatment of OV935 in pediatric patients with epileptic seizures associated with CDD or Duplication 15q (Dup15q) syndrome. The first patients have already been enrolled into ARCADE. This study is part of a global collaboration with Takeda Pharmaceutical Company Limited.

“We are honored to be recognized for our dedication and efforts to bring a novel treatment option to the clinic for those with CDD and other rare epilepsies,” said Amit Rakhit, M.D., MBA, chief medical and portfolio management officer of Ovid Therapeutics. “It was important to everyone at Ovid that I travel to London to accept this award to show our unwavering support and partnership with the CDD community. We thank LouLou Foundation and the community and look forward to providing updates on our progress with the ARCADE study.”

The CDKL5 Forum is a unique community of collaboration and knowledge exchange, made up of leading scientists, clinicians and company representatives from around the world, united in the mission of better understanding the CDKL5 gene and disorder. The objective is to share current research on CDKL5 and to stimulate peer-group discussion and brainstorming around existing and future avenues of research and therapeutic approaches, in order to accelerate treatments and ultimately find cures for this neuro-genetic disorder. Now in its fourth year, the Forum represents the flagship annual event of the CDKL5 Program of Excellence, established by the Loulou Foundation and the Orphan Disease Center of the University of Pennsylvania’s Perelman School of Medicine.

About the ARCADE Trial

ARCADE is a Phase 2, multi-center, open-label, pilot study that will evaluate the treatment of OV935 in pediatric patients, aged 2 to 17 years old, with epileptic seizures associated with CDD or Dup15q syndrome. The primary endpoint is the change in motor seizure frequency in patients treated with OV935 by disorder (CDD and Dup15q). The key secondary endpoints include safety and tolerability, including percentage of participants considered treatment responders, change in CGI-S/C and correlation of OV935 concentration and plasma 24HC levels.

ARCADE is expected to enroll approximately 15 children with each condition at clinical trial sites in the United States. This study consists of a four to six week screening period to establish baseline seizure frequency followed by a 12-week treatment period (2-week dose titration and 10-week maintenance period.) To learn more about ARCADE visit clinicaltrials.gov or www.arcadestudy.com. At the end of treatment, eligible patients can roll over into the ENDYMION study. Additional details on the ENDYMION clinical trial can be found at www.clinicaltrials.gov.

About Cyclin-Dependent Kinase-Like 5 (CDKL5) Deficiency Disorder (CDD)

Cyclin-Dependent Kinase-Like 5 (CDKL5) deficiency disorder, also known as CDD, is a rare, severe, neurological disorder caused by mutations in the CDKL5 gene on the X-chromosome. The CDKL5 gene provides instructions for making a protein that is essential for normal brain and neuron development, and may play a role in regulating the activity of other genes. CDD causes early onset and treatment resistant epilepsy in the first few months of life. Other common features of CDD include severe developmental delay and intellectual disability, poor fine motor skills, difficulty sleeping, scoliosis, visual impairment, microcephaly and various gastrointestinal difficulties.

About Duplication 15q (Dup15q) Syndrome

Duplication 15q syndrome, also known as Dup15q syndrome, is a rare, severe, neurological disorder that results from duplications of chromosome 15q11.2-q13.1. In most cases, the chromosome mutation is not inherited but occurs during formation of reproductive cells or during embryonic development. Those with Dup15q syndrome experience seizures, hypotonia (poor muscle tone), developmental delays and intellectual disability. Difficult to control seizures are the most devastating symptom of Dup15q[ii]. The severity of Dup15q and associated symptoms varies based on the size and location of the duplication and which genes are involved. There is insufficient demographic data to determine the prevalence of Dup15q in the general population.

About Investigational OV935/TAK-935

OV935/TAK-935 is a potent, highly-selective, first-in-class inhibitor of the enzyme cholesterol 24-hydroxylase (CH24H) being investigated as an anti-epileptic drug (AED). CH24H is predominantly expressed in the brain, where it plays a central role in cholesterol homeostasis. CH24H converts cholesterol to 24-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 CH24H 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. Ovid and Takeda believe that OV935's novel mechanism of action may potentially treat rare epilepsies by inhibiting CH24H to decrease 24HC levels, effectively decreasing glutamate hyperactivity. To Ovid and Takeda's knowledge, OV935 is the only molecule with this mechanism of action in clinical development. OV935 is an investigational drug, not approved for commercial use.

OV935 has successfully completed four Phase 1 clinical studies, which have assessed tolerability, PK and target engagement at doses believed to be therapeutically relevant. In preclinical models, a novel proprietary PET ligand was used to determine target occupancy of OV935 in the brain. OV935 is being co-developed by Ovid and Takeda Pharmaceutical Company Limited.

The United States Food and Drug Administration (FDA) has granted orphan drug designation to OV935 for the treatment of both Dravet syndrome and LGS.

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 patients with rare neurological disorders. Ovid has a broad pipeline of potential 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 OV935/TAK-935 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/.

Forward-Looking Statements
This press release includes certain disclosures that contain "forward-looking statements," including, without limitation, statements regarding the potential clinical benefit of OV935 to treat patients with rare epilepsies, number of patients enrolled, the initiation, progress, timing, scope and results of clinical trials, and the effects of OV935 on efficacy, safety and tolerability. 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 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:
Patient advocacy:
Luke Rosen
Ovid Therapeutics Inc
lrosen@ovidrx.com

Media:
Jerica Pitts
W2O Group
jpitts@w2ogroup.com
(312) 858-3469

Ovid Therapeutics Inc.