Press Release

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Ovid Therapeutics Initiates Pivotal Phase 3 Clinical Trial in Angelman Syndrome; Multiple Data Readouts Across Rare Neurological Disease Pipeline Expected in 2H 2019 and Early 2020

Phase 3 NEPTUNE Trial of OV101 Expected to Enroll the First Patients in the Third Quarter of
2019; CGI-I Angelman Syndrome Scale will be the Sole Primary Endpoint

Ovid to Review NEPTUNE Trial Details and Additional Pipeline Program Highlights at Today's
R&D Day to be held from 12:00 p.m. to 3:00 p.m. in New York City

NEW YORK, June 27, 2019 (GLOBE NEWSWIRE) -- Ovid Therapeutics Inc. (NASDAQ: OVID), today announced that following a Type C Meeting with the U.S. Food and Drug Administration (FDA), it has initiated the pivotal Phase 3 NEPTUNE trial with OV101, a novel delta (δ)-selective $GABA_A$ receptor agonist, in Angelman syndrome. Ovid plans to enroll the first patients in NEPTUNE during the third quarter of 2019 and anticipates topline data from the trial to be available by mid-2020. The results of the trial, if positive, are intended to support a New Drug Application for OV101 for the treatment of Angelman syndrome. Members of Ovid's senior management team, along with external clinician experts, will review the Phase 3 NEPTUNE trial design as well as provide additional pipeline program highlights at today's R&D day to be held from 12:00 p.m. to 3:00 p.m. EDT in New York City.

"Following productive discussions with the FDA, we have initiated our pivotal Phase 3 NEPTUNE clinical trial in patients with Angelman syndrome, which provides a clear path to registration," said Jeremy Levin, DPhil, MB, BChir, Chairman and Chief Executive Officer of Ovid Therapeutics. "We will use the CGI-I scale as used in the Phase 2 STARS trial as the sole primary endpoint in our Phase 3 NEPTUNE trial. We have renamed the scale CGI-I-AS to

reflect the fact that relevant anchors and training materials are specific to Angelman syndrome. The use of CGI-I-AS as a sole primary endpoint is important. As was demonstrated in the Phase 2 STARS trial, the CGI-I scale is designed to detect change from baseline in each individual in a disorder with substantial clinical heterogeneity."

Dr. Levin continued, "Beyond Angelman syndrome, we continue to execute across our pipeline and are looking forward to multiple expected data readouts over the coming quarters, including from the Phase 2 ROCKET trial with OV101 in Fragile X syndrome, the ENDYMION trial with OV935 (soticlestat) in rare developmental and epileptic encephalopathies, as well as the Phase 2 ARCADE trial with OV935 in Dup15q syndrome or CDKL5 Deficiency Disorder."

Key Program Updates and Highlights

OV101 for Angelman Syndrome

• Following an End-of-Phase 2 Meeting with the FDA held in 2018 and a subsequent FDA Type C Meeting held in 2019, Ovid has initiated the pivotal Phase 3 NEPTUNE trial, which includes the following elements:

- 12-week, two-arm, randomized, double-blind, placebo-controlled trial;
- Once-daily dose of OV101 or placebo;
- Approximately 60 pediatric patients ages 4 to 12 years diagnosed with Angelman syndrome;
- The sole primary endpoint for the Phase 3 NEPTUNE trial will be the Angelman syndrome-specific CGI-I-AS scale. The CGI-I scale is the same as that used in the Phase 2 STARS trial. Ovid refined CGI-I anchors and clinician training materials specific to the core symptoms of Angelman syndrome, renaming the endpoint CGI-I-AS;
- The trial includes secondary endpoints for sleep, communication, motor skills, socialization, daily living skills and behavior domains;
- A limited number of children ages 2-3 years will also be enrolled to study pharmacokinetics (PK) and safety/tolerability.

• Ovid plans to enroll the first patients in the Phase 3 NEPTUNE trial in the third quarter of 2019, with topline results from the trial expected by mid-2020.

• The open-label extension ELARA trial for individuals with Angelman syndrome who previously completed a clinical trial with OV101 continues to enroll patients.

• Ovid has also received agreement from German regulatory authorities (BfArM) regarding the design of the Phase 3 NEPTUNE trial and the use of CGI-I-AS as a sole primary outcome measure.

• Ovid expects to conduct scientific advice meetings with the European Medicines Agency (EMA)'s Committee for Medicinal Products for Human Use (CHMP) during the second half of 2019 to discuss the regulatory approval pathway for OV101 in Europe.

OV101 for Fragile X Syndrome

• The Phase 2 ROCKET trial continues to enroll patients and results are expected around year-end 2019 or early 2020.

OV935 (soticlestat) for Rare Developmental and Epileptic Encephalopathies (DEE)

• Ovid continues to enroll in the open-label extension ENDYMION trial for individuals with DEE who previously completed a clinical trial with OV935.

- Interim data from the ENDYMION trial are expected in the third quarter of 2019.

• The open-label Phase 2 ARCADE trial in individuals with Dup15q syndrome or CDKL5 Deficiency Disorder continues to enroll.

- Data from the ARCADE trial are expected in the first quarter of 2020.

• The global Phase 2 ELEKTRA trial in children with Dravet syndrome or Lennox-Gastaut syndrome continues to enroll.

Ovid also plans to present additional details around its early stage research programs during today's R&D day event.

R&D Day Webcast
A live audio webcast of today's R&D Day can be accessed through the Events & Presentations section of the company's website at investors.ovidrx.com. An archived replay of the webcast will be available on the company's website for 90 days following the event.

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

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