Ovid Therapeutics Reports Second Quarter 2018 Financial Results and Highlights Recent Clinical Progress

August 9, 2018

-- **OV101 in Angelman syndrome:** plan to initiate an open-label extension study (ELARA) in the fourth quarter of 2018 and discuss with regulatory authorities next steps for a registrational pathway based on positive Phase 2 STARS data --

-- **OV101 in Fragile X syndrome:** topline data from Phase 2 ROCKET trial in males with Fragile X syndrome expected in 2019 --

-- **OV935/TAK-935 in rare developmental and epileptic encephalopathies:** data from Phase 1b/2a study expected in fourth quarter of 2018; ENDYMION, open-label extension study, now enrolling patients --

NEW YORK, Aug. 09, 2018 (GLOBE NEWSWIRE) -- Ovid Therapeutics Inc. (NASDAQ: OVID), a biopharmaceutical company committed to developing medicines that transform the lives of people with rare neurological disorders, today reported financial results for the second quarter ended June 30, 2018 and provided an overview of the company's recent clinical progress.

“Earlier this week we announced positive topline data from the Phase 2 STARS trial in Angelman syndrome, the first industry-sponsored, randomized, double-blind, placebo-controlled study with a hierarchy of prespecified endpoints,” said Jeremy Levin, DPhil, MB, BCHir, chairman and chief executive officer of Ovid Therapeutics. “OV101 is the first investigational medicine to demonstrate a significant clinical effect in overall symptomatology (using CGI-I) since the condition was identified more than 50 years ago. Importantly, CGI-I was the first measure on the prespecified endpoint analysis in the STARS trial. This resulted in a robust design and helped ensure that investigators captured the impact of OV101 on the totality of global neurological deficits and symptomatology. Angelman syndrome is a complex disorder that impacts all areas of the brain and we believe CGI-I is an appropriate assessment tool for overall Angelman syndrome symptomatology. Having also identified what we believe is the optimal dose of once-daily OV101 to bring forward, preparations are underway to discuss with regulatory authorities the next steps for a registrational pathway.”

Dr. Levin continued, “Beyond the STARS trial, the Phase 2 ROCKET trial with OV101 in adolescents and young adult males with Fragile X syndrome is initiated, and we plan to start the accompanying SKY ROCKET non-drug study in the third quarter of 2018. Our collaboration with Takeda for the development of OV935/TAK-935 in developmental and epileptic encephalopathies (DEE) also continues to advance in the clinic. We look forward to a busy rest of the year, as we anticipate topline data from our Phase 1b/2a clinical trial of OV935 in adults with DEE, as well as the initiation of ELEKTRA and ARCADE, two Phase 2 studies in pediatric DEE patients.”

Recent Progress and Upcoming Milestones

**OV101 for Angelman Syndrome**

- On August 6, 2018, Ovid Therapeutics announced positive topline data from the Phase 2 STARS study, a 12-week, randomized, double-blind, clinical trial which evaluated OV101 for the treatment of adults and adolescents with Angelman syndrome. Angelman syndrome is a rare, lifelong, genetic disorder that affects approximately 1 in 15,000 people in the general population. The study met its primary endpoint of safety and tolerability. The first prespecified efficacy measure showed a robust and statistically significant improvement (p=0.0006) in physician-reported CGI-I at 12 weeks of treatment in the OV101 once-daily dose arm compared to placebo. Based on these data, Ovid believes CGI-I is an appropriate measure for Angelman syndrome to capture the totality of global neurological deficits, and it plans to incorporate CGI-I as an endpoint in potential future trials. The company now plans to expand the development of OV101 for Angelman syndrome and discuss next steps for a registrational path with regulatory authorities.
  - CGI-I is a commonly used measure in clinical studies. It allows the clinician to capture a constellation of improvement in symptoms from subdomains, including the parent/caregiver history observations, into a single rating of improvement following start of treatment. With this measure, the investigator considers all aspects of the patient's function in a 7-point scale where a 1-3 rating represents improvement, 4 represents no change, and 5-7 represents worsening of symptoms.
- The topline STARS data were presented by Matthew During, M.D., DSc, FACP, founder and chief scientific officer of Ovid Therapeutics, at the 2018 Angelman Syndrome Foundation/Duplication15q Research Symposium on August 6, 2018. Ovid plans to present the full clinical data from the STARS study at an upcoming medical meeting.
- Ovid Therapeutics created a website specifically to provide disease education on Angelman syndrome. Learn more at www.angelmansyndrome.com.
- In the fourth quarter of 2018, Ovid plans to initiate the ELARA study using the once-daily dose of OV01. ELARA is an open-label extension study for Angelman syndrome patients who have previously participated in any OV101 clinical trial.
In July 2018, Ovid initiated the Phase 2 ROCKET clinical trial, which is a randomized, double-blind, parallel-group trial to evaluate the safety, tolerability and efficacy of OV101 over 12 weeks of treatment in three treatment arms: 5mg once-daily, 5mg twice-daily, and 5mg three times a day. The secondary efficacy endpoint will evaluate changes in behavior during 12 weeks of treatment. Topline data from this trial is expected in 2019.

Ovid plans to initiate in the third quarter of 2018 the SKY ROCKET study, a 12-week, non-drug study to assess the suitability of several behavioral scales in individuals with Fragile X syndrome. The trial is an observational study designed to provide additional data on the key endpoints that are being explored in the ROCKET trial as well as to provide comparative data on the benefit offered by the standard of care. The SKY ROCKET study will enroll up to 30 males ages 5 to 30 with Fragile X syndrome.

OV935 for Rare Developmental and Epileptic Encephalopathies

Ovid and its collaboration partner, Takeda Pharmaceutical Company Limited, have completed enrollment of 18 patients in a Phase 1b/2a clinical trial of OV935 in adults with DEE. The primary endpoint of this study is safety and tolerability. Secondary endpoints include evaluation of pharmacokinetic (PK) parameters. The trial also includes exploratory endpoints evaluating the change from baseline in seizure frequency and 24-hydroxycholesterol (24HC) levels. Plasma 24HC is being further assessed as a potential biomarker for OV935, which may inform future clinical trial designs and help clinicians individualize the use of this investigational medicine. Topline data from the Phase 1b/2a trial are expected in the fourth quarter of 2018.

On July 18, 2018, Ovid and Takeda announced plans to initiate three clinical trials in the third quarter of 2018: the ELEKTRA study in pediatric patients with Dravet syndrome and Lennox-Gastaut syndrome, the ARCADE study in pediatric patients with CDKL5 deficiency disorder and Duplication 15q syndrome, and the ENDYMION study, an open-label extension trial for patients with DEE who participated in a previous OV935 clinical study. The ENDYMION study started to enroll patients in July 2018.

In May, at the 14th EILAT Conference on New Antiepileptic Drugs and Devices (EILAT XIV), Ovid presented new preclinical findings for OV935 providing further evidence of OV935’s novel mechanism of action as a potential treatment for DEE.

Investigators plan to present new preclinical data of OV935 at the 13th European Congress on Epileptology in Vienna, Austria, taking place August 26-30, 2018.

OV329 for Treatment-Resistant Epilepsy

Also at EILAT XIV, Ovid presented preclinical data highlighting OV329, a next-generation GABA aminotransferase (GABA-AT) inhibitor, as a potential therapeutic approach to treatment-resistant epilepsy.

Second Quarter 2018 Financial Results

Research and development expenses were $8.1 million for the second quarter of 2018, as compared to $6.1 million for the same period in 2017. The increase of $2.0 million was primarily due to an increase in preclinical and development expenses.

General and administrative expenses were $5.1 million for the second quarter of 2018, as compared to $4.2 million for the same period in 2017. The increase was primarily due to higher payroll and payroll-related expenses due to growth in headcount as the company expanded its operations, and an increase in professional fees associated with operating as a public company.

Net loss was $12.9 million, or net loss per share of $0.53, for the second quarter of 2018, as compared to a net loss of $10.2 million, or net loss per share of $0.57, for the same period in 2017.

As of June 30, 2018, cash, cash equivalents and short-term investments totaled $62.6 million.

About OV101

OV101 (gaboxadol) is believed to be the only delta (δ)-selective GABA<sub>A</sub> 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 demonstrated in laboratory studies and animal models to selectively activate the δ-subunit of GABA<sub>A</sub> 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. In the STARS Phase 2 trial, OV101 showed a favorable safety profile, was well tolerated, and showed a significant improvement on the Clinical Global Impressions-Improvement (CGI-I) scale in individuals with Angelman syndrome. Gaboxadol has previously been tested in over 4,000 patients (1,000+ patient-years of exposure) and was observed to have favorable safety and bioavailability profiles. The U.S. Food and Drug Administration (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 without regulatory extensions.

About 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. This mechanism of action may be especially important in CDD and Dup15q since the NMDA receptor-mediated synaptic transmission underlies the pathological mechanisms of these syndromes. 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.

About OV329
OV329 is a preclinical compound being developed by Ovid Therapeutics for epilepsy and other neurologic disorders, as part of the company’s epilepsy portfolio. OV329 functions by inactivating GABA aminotransferase (GABA-AT), a key metabolic enzyme of the brain’s major inhibitory neurotransmitter, GABA. By inhibiting the metabolism of GABA, OV329 leads to increased intracellular concentrations of GABA. Given that epilepsy is characterized by excessive neuronal excitation, the enhanced release of GABA may suppress this excitatory signaling and may reduce seizures. GABA-AT is a well-validated target for treatment-resistant epilepsy and has applications in multiple seizure types such as refractory complex partial seizures and infantile spasms.

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 in development for the treatment of Angelman syndrome and Fragile X syndrome. Ovid is also developing 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.ovidx.com/](http://www.ovidx.com/).

Forward-Looking Statements
This press release includes certain disclosures that contain “forward-looking statements,” including, without limitation, statements regarding (i) the initiation, progress, timing, scope and results of clinical trials for Ovid’s product candidates, (ii) the company’s preclinical and clinical development plans, (iii) the development of new therapies for previously unidentified disorders, (iv) the number of patients to be enrolled, (v) the timing of reporting of clinical data regarding Ovid’s product candidates, (vi) the presentation of scientific data at scientific meetings; and (vii) the approval or registration of Ovid’s product candidates by regulatory authorities. 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.

### Condensed Statements of Operations (Unaudited)

<table>
<thead>
<tr>
<th></th>
<th>For the Three Months Ended</th>
<th>For the Three Months Ended</th>
<th>For the Six Months Ended</th>
<th>For the Six Months Ended</th>
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<tr>
<td></td>
<td>June 30, 2018</td>
<td>June 30, 2017</td>
<td>June 30, 2018</td>
<td>June 30, 2017</td>
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<tr>
<td>Operating expenses:</td>
<td></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Research and development</td>
<td>$ 8,116,385</td>
<td>$ 6,074,927</td>
<td>$ 16,590,942</td>
<td>$ 37,359,355</td>
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<td>General and administrative</td>
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<td>$ 4,213,173</td>
<td>$ 10,048,615</td>
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<td>Total operating expenses</td>
<td>$13,209,696</td>
<td>$10,288,100</td>
<td>$26,639,557</td>
<td>$44,550,394</td>
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<tr>
<td>Loss from operations</td>
<td>$(13,209,696)</td>
<td>$(10,288,100)</td>
<td>$(26,639,557)</td>
<td>$(44,550,394)</td>
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<tr>
<td>Interest income</td>
<td>$ 274,556</td>
<td>$ 39,721</td>
<td>$ 521,662</td>
<td>$ 63,205</td>
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<td>Net loss</td>
<td>$(12,935,140)</td>
<td>$(10,248,379)</td>
<td>$(26,117,895)</td>
<td>$(44,487,189)</td>
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<tr>
<td>Net loss attributable to common stockholders</td>
<td>$(12,935,140)</td>
<td>$(10,248,379)</td>
<td>$(26,117,895)</td>
<td>$(44,487,189)</td>
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<tr>
<td>Net loss per share attributable to common stockholders, basic and diluted</td>
<td>$(0.53)</td>
<td>$(0.57)</td>
<td>$(1.06)</td>
<td>$(3.18)</td>
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<td>Weighted-average common shares outstanding basic and diluted</td>
<td>24,625,966</td>
<td>18,112,554</td>
<td>24,617,555</td>
<td>13,998,428</td>
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### Selected Condensed Balance Sheet Data
(Unaudited)

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<tr>
<th></th>
<th>June 30, 2018</th>
<th>December 31, 2017</th>
</tr>
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<tbody>
<tr>
<td>Cash, cash equivalents and short-term investments</td>
<td>$62,594,596</td>
<td>$87,125,600</td>
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<td>Working capital</td>
<td>$57,693,048</td>
<td>$82,566,948</td>
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<tr>
<td>Total assets</td>
<td>$68,612,088</td>
<td>$89,457,603</td>
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<tr>
<td>Total stockholders’ equity</td>
<td>$61,115,860</td>
<td>$83,436,503</td>
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</table>

1 Working capital defined as current assets less current liabilities

### Contacts

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