Ovid Therapeutics and NeuroPointDX Announce Collaboration to Identify Biomarkers for Angelman Syndrome

– Partnership Marks First Step in Ovid Therapeutics' Commitment to Biomarker Development –

NEW YORK and MADISON, Wis., Feb. 09, 2017 (GLOBE NEWSWIRE) -- Ovid Therapeutics, a privately held biopharmaceutical company committed to developing medicines that transform the lives of people with rare neurological disorders, and NeuroPointDX, a privately held biotech company based in Madison, Wisconsin and Cambridge, Massachusetts, today announced that they have entered into a collaboration to identify novel biomarkers of Angelman syndrome by analyzing metabolomic profile data as part of Ovid's ongoing randomized, double-blind, placebo-controlled Phase 2 clinical trial (STARS).

Angelman syndrome is a rare genetic disorder that is characterized by a variety of signs and symptoms, including delayed development, intellectual disability, severe speech impairment, problems with movement and balance, seizures, sleep disorders and anxiety. Like many other neurological disorders, symptoms and response to treatments vary widely from person to person.

"With the initiation of the STARS trial and this collaboration, we are making important progress in better understanding Angelman syndrome and developing much needed treatment options. The metabolic profile of this syndrome is not well understood, and we believe this biomarker study will produce critical data to fill this gap and inform us about the impact of OV101 as a potential treatment option," said Matthew During, M.D., DSc, FACP, FRACP, president and chief scientific officer of Ovid. "This collaboration is the first step in Ovid's broader rare neurological disorder biomarker strategy to identify molecular markers of treatment responders and guide enrollment of participants in our clinical trials."

"We are excited to partner with Ovid Therapeutics on this biomarker collaboration, particularly as it furthers our mission to improve the lives of people impacted by neurological disorders by identifying biomarkers that can improve diagnosis and inform more precise treatment strategies," said Elizabeth Donley, chief executive officer of NeuroPointDX.

NeuroPointDX uses its metabolomics platform technology to identify differences in children with autism spectrum disorders compared to typically-developing children and between subgroups of children on the spectrum for earlier diagnosis and more precise treatment. The collaboration will leverage NeuroPointDX's expertise in metabolomics in an effort to identify biomarkers associated with Angelman syndrome. Metabolomics is a process that allows comprehensive exploration of changes in small molecules present in the metabolism of patients to provide insight into the physiology of a disease and the response to treatment. In the STARS trial, this analysis is designed to provide molecular insights into disease mechanism and assess the

potential response to OV101 to help understand the physiological impact of the compound in people with Angelman syndrome. This study may help identify the individuals that are most likely to respond to treatment.

About Angelman Syndrome

Angelman syndrome is a rare genetic disorder that is characterized by a variety of signs and symptoms. Characteristic features of this disorder include delayed development, intellectual disability, severe speech impairment, problems with movement and balance, seizures, sleep disorders and anxiety. The most common cause of Angelman syndrome is the disruption of a gene that codes for ubiquitin protein ligase E3A (UBE3A). Angelman syndrome affects approximately 1 in 12,000 to 20,000 people in the United States. There are currently no FDA-approved therapies for the treatment of Angelman syndrome.

Angelman syndrome is associated with a reduction in tonic inhibition, a function of the delta (δ)-selective $GABA_A$ receptor that allows a human brain to decipher excitatory and inhibitory neurological signals correctly without being overloaded. If tonic inhibition is reduced, the brain becomes inundated with signals and loses the ability to separate background noise from critical information.

About OV101

OV101 (gaboxadol) is a delta (d)-selective $GABA_A$ receptor agonist and is believed to be the first investigational drug to target the disruption of tonic inhibition, a key mechanism that allows a healthy human brain to decipher excitatory and inhibitory neurological signals correctly without being overloaded. Loss of tonic inhibition is implicated in a host of rare neurological disorders and is established in genetic models. In preclinical models, OV101 has been able to selectively activate the δ-subunit of $GABA_A$ receptors, which are found in the extrasynaptic space (outside of the synapse), and helped regulate neuronal activity through tonic inhibition.

Ovid is developing OV101 for use in both 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 September 2016, the United States Food and Drug Administration granted orphan drug designation for OV101 for the treatment of Angelman syndrome. The United States Patent and Trademark Office has granted Ovid two patents directed to methods of treating Angelman syndrome using THIP (OV101). The issued patents expire in 2035, without regulatory extensions.

About Ovid Therapeutics

Ovid Therapeutics is a privately held, 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 symptoms of Angelman syndrome and Fragile X syndrome. Ovid is also developing OV935 in collaboration with Takeda Pharmaceutical Company Limited for the treatment of rare epileptic encephalopathies. Ovid has initiated a Phase 2 STARS trial of OV101 in adults with Angelman syndrome and Ovid intends to commence a Phase 1 trial in adolescents with Angelman syndrome or Fragile X syndrome. OV935 is expected to commence a Phase 1b/2a trial in rare epileptic encephalopathies in 2017.

For more information, visit http://www.ovidrx.com/.

About NeuroPointDX

NeuroPoinDX is the diagnostics division of Stemina Biomarker Discovery. Stemina is a biotechnology company based in Madison, WI and Cambridge, MA that has developed a robust and reproducible proprietary

platform for identifying changes in metabolism ("biomarkers") utilizing highly sensitive analytical equipment and its proprietary platform technology. The Company's diagnostics division, NeuroPointDX, identifies biomarkers of neurological disorders in cellular models of disease and human samples. These biomarkers provide greater insight into the pathways that are disrupted in patients with neurological disorders. This information can be translated into clinical diagnostics, individualized treatment recommendations, and new potential therapies.

The first area in which the Company is conducting clinical studies is in patients with autism and other neuro-developmental disorders. In September 2015, Stemina launched its 1,500 patient study the Children's Autism Metabolome Project (CAMP). The study is funded by a $2.7 million grant from the National Institutes of Mental Health and an investment by the Nancy Lurie Marks Family Foundation. The CAMP study is the most comprehensive study of the metabolism of children with autism, and other neuro-developmental disorders ever conducted. This study may result in a panel of validated blood tests for autism that will more accurately diagnose and inform individualized treatment decisions based on the child's own metabolism.

For more information about NeuroPointDX and Stemina, visit www.neuropointdx.com and www.stemina.com.

Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements. Forward-looking statements contained in this press release include, without limitation, statements regarding the collaboration with NeuroPointDX to identify novel biomarkers of Angelman syndrome and Ovid's progress and expectations regarding the clinical development of OV101. Words such as "may," "believe," "will," "expect," "plan," "anticipate," "estimate," "intend" and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements. These forward-looking statements are not guarantees of future performance and involve a number of unknown risks, assumptions, uncertainties and factors that are beyond Ovid's control. All forward-looking statements are based on Ovid's expectations and assumptions as of the date of this press release. Actual results may differ materially from these forward-looking statements. Except as required by law, Ovid expressly disclaims any responsibility to update any forward-looking statement contained herein, whether as a result of new information, future events or otherwise.

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