



Ovid Therapeutics and University of Connecticut Enter into Strategic Research Collaboration to Accelerate the Development of Next-Generation Genetic Therapy for Angelman Syndrome

July 23, 2020

- Ovid to collaborate with renowned molecular geneticist and Angelman syndrome expert Stormy J. Chamberlain, Ph.D., to advance a short hairpin RNA (shRNA)-based therapeutic with the goal of addressing the underlying genetic cause of Angelman syndrome
- Ovid and UConn to collaborate on genetic therapy for potential future use alone or in combination with OV101, Ovid's small molecule therapy for Angelman syndrome in Phase 3 development

NEW YORK and FARMINGTON, Conn., July 23, 2020 (GLOBE NEWSWIRE) -- Ovid Therapeutics Inc. (NASDAQ: OVID), a biopharmaceutical company committed to developing medicines that transform the lives of people with rare neurological diseases, and the University of Connecticut School of Medicine ("UConn"), today announced a research collaboration and license agreement to accelerate the development of a next-generation short hairpin RNA (shRNA)-based therapeutic for Angelman syndrome and potentially other indications. The most common cause of Angelman syndrome is the loss of function of the gene that codes for ubiquitin protein ligase E3A (UBE3A), which plays a critical role in nerve cell communication, resulting in impaired tonic inhibition. An shRNA-based therapeutic may address this underlying genetic cause of Angelman syndrome by reducing the expression of UBE3A-antisense, potentially restoring the function of UBE3A. This genetic approach may be used in combination with OV101 (gaboxadol), Ovid's novel, small-molecule delta (δ)-selective GABA_A receptor agonist, to restore tonic inhibition and address the underlying symptomatology of individuals with Angelman syndrome. OV101 is currently being evaluated in the pivotal Phase 3 NEPTUNE trial in Angelman syndrome, with topline results expected in the fourth quarter of 2020.

Under the terms of the research collaboration, Ovid will work closely with UConn's Stormy J. Chamberlain, Ph.D., and gain exclusive access to identified genetic sequences for a potential shRNA-based therapeutic. Ovid plans to validate select sequences and leverage its translational medicine capabilities and drug development expertise in Angelman syndrome to advance an shRNA-based therapeutic into clinical studies. Dr. Chamberlain is a recognized leader in the field of Angelman syndrome and UBE3A research and currently serves as the John and Donna Krenicki Associate Professor of Genomics and Personalized Healthcare in UConn's Genetics and Genome Sciences Department. In addition, Dr. Chamberlain chairs the Angelman Syndrome Foundation (ASF) Scientific Advisory Committee and is a member of the Dup15q Alliance Scientific Advisory Board. Ovid will also work closely with UConn's Noelle Germain, Ph.D., Assistant Professor of Genetics and Genome Sciences on these efforts.

"Ovid is deeply committed to the Angelman syndrome community. We have made great progress and are excited to see the topline data from our Phase 3 NEPTUNE trial with OV101 expected in Q4 2020," said Amit Rakhit, M.D., MBA, President and Chief Medical Officer of Ovid Therapeutics. "We believe OV101 has the potential to serve as a core therapy for this disorder and are now focused on building a comprehensive and strategic Angelman syndrome longer term pipeline. If successful, OV101 may be used in combination with genetic approaches in the future to address the needs of Angelman syndrome. This collaboration with Drs. Chamberlain and Germain, both accomplished scientific leaders in the field of Angelman syndrome, will enable us to accelerate and share in their mission to identify and develop next-generation genetic therapies. Together with our early-stage microRNA approach, this research collaboration now provides us with additional targets against this disorder, greater strategic optionality, and underpins our broad capability to bring new therapies to individuals living with Angelman syndrome both near-term and into the future."

"Our lab shares in Ovid's demonstrated commitment to advance innovative therapeutic options for Angelman syndrome," stated Dr. Chamberlain. "An shRNA therapeutic can target the genetic cause of Angelman syndrome at its source and may offer potential advantages to other next-generation approaches, including antisense oligonucleotide therapy, via a lower rate of degradation and turnover and plasmid delivery allowing for a less-frequent dosing profile. Ovid is uniquely positioned to accelerate an shRNA therapeutic through late preclinical and clinical development, and our lab looks forward to working with the team at Ovid towards our common objective of impacting the lives of individuals living with Angelman syndrome and their families."

About Angelman Syndrome

Angelman syndrome is a rare genetic condition that is characterized by a variety of signs and symptoms. Characteristic features of this condition 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 loss of function of the gene that codes for ubiquitin protein ligase E3A (UBE3A), which plays a critical role in nerve cell communication, resulting in impaired tonic inhibition. Individuals with Angelman syndrome typically have normal lifespans but are unable to live independently. Therefore, they require constant support from a network of specialists and caregivers. Angelman syndrome affects approximately 1 in 12,000 to 1 in 20,000 people globally.

There are no approved therapies by the U.S. Food and Drug Administration (FDA), European Medicines Agency or rest-of-world for Angelman syndrome, and treatment primarily consists of behavioral interventions and pharmacologic management of symptoms.

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.

Ovid is developing OV101 for the treatment of Angelman syndrome to potentially restore tonic inhibition and thereby address several core symptoms of Angelman syndrome. Ovid is conducting a pivotal Phase 3 clinical trial with OV101 in Angelman syndrome (NEPTUNE), with topline results

expected in the fourth quarter of 2020. In addition, Ovid is also exploring OV881, a microRNA approach that may reduce the expression of UBE3A-antisense and potentially restore UBE3A expression.

About Ovid Therapeutics

Ovid Therapeutics Inc. 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 www.ovidrx.com.

About UConn

UConn Health is Connecticut's only public academic medical center. Based on a 206-acre campus in Farmington, UConn Health has a three-part mission: research, teaching and patient care. Home to the UConn School of Medicine, School of Dental Medicine and UConn John Dempsey Hospital with over 5,000 employees supporting nearly 1,000 students, over 800,000 annual patient visits, and innovative scientific research contributing to the advancement of medicine. For more information, visit health.uconn.edu.

Ovid Forward-Looking Statements

This press release includes certain disclosures that contain "forward-looking statements," including, without limitation, statements regarding: development of short hairpin RNA based therapeutics, development of combination therapies, potential benefits of OV101, anticipated reporting schedule of clinical data for OV101 and the potential benefits and value of this collaboration. You can identify forward-looking statements because they contain words such as "will," "appears," "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 include uncertainties in the development and regulatory approval processes, and the fact that initial data from clinical trials may not be indicative, and are not guarantees, of the final results of the clinical trials and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. Additional risks 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". Such risks may be amplified by the COVID-19 pandemic and its potential impact on Ovid's business and the global economy. Ovid assumes no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.

Ovid Therapeutics Contacts

Investors and Media:

Ovid Therapeutics Inc.
Investor Relations & Public Relations
irpr@ovidrx.com

Or

Investors:

Steve Klass
Burns McClellan, Inc.
sklass@burnsmc.com
(212) 213-0006

Media:

Katie Engleman
1AB
katie@1abmedia.com

UConn Contact

Jessica McBride, PhD
jessica.mcbride@uconn.edu
860-878-5058



Source: Ovid Therapeutics Inc.