OptiNose Announces Positive Early Phase Clinical Trial with OPN-300, an Investigational Product in Development for Treatment of Autism Spectrum Disorders

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Study May be First Human Oxytocin Study to Find Evidence Suggesting Direct "Nose to Brain" Activity

Primary Results Published in Translational Psychiatry[1]

YARDLEY, PA, July 15, 2015— OptiNose today announced positive results from an early phase trial of OPN-300, an investigational product in development for the treatment of Autism Spectrum Disorders (ASD or Autism) have been published in the peer-reviewed journal Translational Psychiatry[1]. The publication reports the primary results from a randomized, placebo-controlled, double-blind, double-dummy, 4-arm cross-over study comparing intravenous (IV) administration of the hormone oxytocin with two doses of OPN-300 (8IU or 24IU) delivered intranasally with a device incorporating the patented OptiNose Bi-Directional™ Breath Powered™ Drug Delivery System technology. OPN-300 is an investigational drug-device combination product that uses a unique new technology to deliver the hormone oxytocin deeply into the nose to target sites in the upper part of the nose. Target sites high in the nose are believed to have potential to enable or enhance direct-to-brain activity of drugs, particularly drugs which do not otherwise cross easily into the brain. The study evaluated social cognition and other effects of OPN-300 in 16 healthy adult volunteers. The results provided encouraging early phase evidence supporting direct nose-to-brain effects of oxytocin when delivered using the OptiNose technology, and have solidified OptiNose enthusiasm for further development of OPN-300. Currently there are no drugs approved anywhere in the world for the treatment of the core social deficiency of Autism.

In this study low-dose OPN-300, despite producing similar blood levels as the control administration of IV oxytocin, resulted in statistically significantly greater brain (social-cognitive) effect, as measured using emotional ratings of facial images (the primary outcome). The findings of this study support the possibility of direct nose-to-brain effect, independent of blood absorption, with low dose OPN-300 using OptiNose Bi-Directional Breath Powered delivery technology. There is tremendous need for better therapies in many neurological, neurodegenerative, and psychiatric disorders.

"Researchers have been searching for a way to get improved and more reliable brain activity with many medications. These include oxytocin to treat diseases like ASD and schizophrenia, and a variety of other drugs for treatment of Alzheimer's and other brain diseases", said Ole A. Andreassen, M.D., Ph.D., Professor, NORMENT – KG Jebsen Centre for Psychosis Research, Institute of Clinical Medicine, University of Oslo, and Division of Mental Health and Addiction, Oslo University Hospital, Oslo, Norway. "The OptiNose technology significantly changes the way drug is delivered high up in the nose, and may be the drug delivery solution we've been looking for. If we can improve social cognition in healthy people with OPN-300 low-dose oxytocin, then we may be able to address a core symptom suffered by millions of patients worldwide with Autism."

"Nose-to-brain activity in humans has been sought after for years. It's important because getting medicine from a pill, or even an injection, into the brain to treat brain diseases is difficult due to a natural barrier that blocks most drugs in the blood from entering the brain. Nose-to-brain drug delivery could be an important way of solving the problem of getting drugs into the brain so they can do their job," said Per Djupesland, M.D., Ph.D., CSO, inventor of the technology, and one of the Founders of OptiNose. "Although animal data has been encouraging, many would argue that medication transport from the nasal cavity directly to the brain has not been previously proven in humans. Today's results are quite promising and bolster our belief that we can enable and enhance the treatment of common brain disorders with OptiNose delivery technology." Work to initiate a phase II trial of OPN-300 in patients with Autism has begun in Norway.

"Our pipeline is producing extremely encouraging results," said Peter Miller, Chief Executive Officer of OptiNose. "AVP-825, the investigational product for migraine that we developed and out-licensed, is under review by the U.S. Food & Drug Administration and we look forward to a positive decision in the second half of this year. Recently, we were

pleased to report that our investigational product OPN-375, a potentially important new treatment for Chronic Nasal Inflammatory Diseases, met both of its primary endpoints in its first double-blind phase III trial: reduction in congestion/obstruction and in nasal polyp size. And now in this trial, OPN-300 produced promising results that suggest nose-to-brain activity that supports a development program on the path to a treatment for social-cognitive symptoms of Autism. We plan to continue to develop these and other important products for people with needs that are not being met by today's therapies."

To access the online publication, please click the link; http://www.nature.com/tp/journal/v5/n7/full/tp201593a.html

Background Information

About OptiNose Technology: Bi-Directional™ Breath Powered™ Drug Delivery Systems

OptiNose's patented technology for closed-palate Bi-Directional Breath Powered drug delivery systems is unique in that its exhalation devices exploit the natural functions of a patient's breath to deliver medications beyond the nasal valve into deep, targeted areas of the nasal cavity. A user exhales into the device, naturally closing the soft palate and sealing off the nasal cavity from the throat. The exhaled breath carries medication from the device into one side of the nose through a specially shaped sealing nosepiece, balancing the pressure across the soft palate. Narrow nasal passages are gently expanded and medication is transported well beyond the nasal valve to targeted sites. After delivering medication to the targeted sites, air flows around to the opposite side of the nasal cavity and exits through the other side of the nose rather than into the throat or lungs.

About OPN-300

OPN-300 uses OptiNose's patented technology to deliver a peptide called oxytocin high and deep in the nasal cavity in a manner intended to enhance direct-to-brain effects. In this early phase study, OPN-300 used an oxytocin formulation provided by Sigma-Tau Industrie Farmaceutiche Riunite SpA.

About OptiNose

OptiNose is a Specialty Biopharmaceutical Company developing a promising pipeline of late stage new products. The Company's patented closed-palate Bi-Directional™ Breath Powered™ drug delivery systems enable differentiated drug-device combination products using exhaler devices that deposit drugs high and deep in the nose. OptiNose successfully out-licensed a first product at the end of phase 3 (AVP-825 for Migraine, licensed to Avanir in North America, since acquired by Otsuka Pharmaceutical Co., Ltd.), and has reported clinical success with other products, including OPN-375, a product in development for Chronic Nasal Inflammatory Diseases (CNID). OPN-375 has potential to address important unmet needs in the treatment of serious CNID, such as Chronic Rhinosinusitis. Other OptiNose pipeline products also target large markets with significant unmet need, including nose-to-brain applications of the technology such as OPN-300 for Autism. OptiNose has corporate offices in the US, Norway and the UK. For more information, please visit www.optinose.com.

Investors in OptiNose include Avista Capital Partners in New York, WFD Ventures LLC located in New York and Entrepreneurs Fund LP based in Jersey, Channel Islands.

1. Quintana, D.S., Westlye, L.T., Rustan, Ø.G., Tesli, N., Poppy, C, Smevik, H., Tesli, M., Røine, M., Mahmoud, R., Smerud, K., Djupesland, P.G., Andreassen, O.A. (2015). Low dose oxytocin delivered intranasally with Breath Powered device affects social-cognitive behavior: a randomized 4-way crossover trial with nasal cavity dimension assessment. *Translational Psychiatry*, 5, doi:10.1038/tp.2015.93

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