

Allievex Completes Series A Financing Led by Pappas Capital and Novo Holdings

Newly Formed Clinical-Stage Biotechnology Company to Develop Treatments for Rare Pediatric Neurodegenerative Diseases

Enters Licensing Agreement with BioMarin Pharmaceutical for Tralesinidase Alfa, Investigational Enzyme Replacement Therapy for Sanfilippo Syndrome Type B

Pappas Capital and Novo Holdings to Join Allievex Board



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Allievex Corporation →

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MARBLEHEAD, Mass., Oct. 23, 2019 /PRNewswire/ -- Allievex Corporation ("Allievex"), a clinical-stage biotechnology company founded by Pappas Capital, today announced the completion of a Series A financing co-led by Novo Holdings and Pappas Capital. Allievex has also appointed Kyle Rasbach, Ph.D., PharmD., Managing Partner at Pappas Ventures and Tiba Aynechi, Ph.D., Partner at Novo Holdings, to its Board of Directors.

Proceeds from the fundraise will be used to support registrational Phase 2 clinical studies of Allievex's lead product candidate, tralesinidase alfa (formerly known as BMN 250), an investigational enzyme replacement therapy to treat Sanfilippo syndrome type B or mucopolysaccharidosis IIIB (MPS IIIB) for which Allievex has obtained an exclusive, worldwide license from BioMarin Pharmaceutical Inc. Under the terms of its agreement with BioMarin, Allievex will assume all financial obligations associated with the development and commercialization of tralesinidase alfa, while BioMarin will provide certain manufacturing services in support of a marketing authorization filing.

"Our mission at Allievex is to develop novel therapies for children with rare pediatric neurodegenerative diseases, and we are pleased to begin executing on this mission with tralesinidase alfa," said Thomas P. Mathers, Founder, President and Chief Executive Officer at Allievex and a Partner at Pappas Ventures. "I am excited to leverage the expertise of the Allievex team and Board as we grow our business and product pipeline of promising treatments for neurodegenerative diseases."

Dr. Rasbach added, "Pappas's strategy is to build innovative life sciences companies and Allievex is an exceptional example of our strategy at play. Allievex has successfully closed its first investment round and Tom has assembled an experienced team of biotech veterans with deep knowledge of the drug development process and a track record of bringing innovative therapies to market. We believe Allievex has tremendous potential to transform the lives of children with rare diseases and look forward to helping drive significant value for the business over the long-term."

"It is with confidence that we transition the development of the tralesinidase alfa program to Allievex. They share BioMarin's dedication to making a big difference for certain pediatric rare disease populations and the MPS IIIB community. We are grateful to the children and families participating in the clinical studies and will support Allievex through a smooth transition," commented Hank Fuchs, M.D., President of Worldwide Research and Development at BioMarin.

Dr. Aynechi concluded, "As a parent, it is heartbreaking to imagine the pain and suffering of the children and families devastated by MPS IIIB. Novo's significant investment in Allievex to enable the continued development of tralesinidase alfa for MPS IIIB is an excellent example of our commitment to improving patients' lives. We have great confidence in the Allievex team and look forward to advancing a pipeline of rare disease programs."

Tralesinidase Alfa Clinical Program and Transition

The tralesinidase alfa development program consists of four independent and complementary multicenter, international studies. BMN 250-901 is an observational study of the progression of Sanfilippo syndrome type B over time and has been completed. BMN 250-201 is an ongoing Phase 2 treatment study consisting primarily of eligible patients rolling over from the BMN 250-901 observational study. BMN 250-202 is a treatment extension study designed to study

longer term treatment effects of tralesenidase alfa. BMN 250-902 is a natural history study of the progression of Sanfilippo syndrome type B over time in children 0-18 years of age with all levels of cognitive function.

Under the terms of its agreement with BioMarin, Allievex will manage all clinical studies in the tralesenidase alfa program, which will continue uninterrupted. All study participants in the treatment studies will continue to receive tralesenidase alfa to evaluate its safety and efficacy as a potential treatment. BioMarin will provide necessary technical support and guidance throughout the transfer process to support a smooth transition by the end of December 2019.

About Tralesenidase Alfa

Tralesenidase alfa is an investigational enzyme replacement therapy using a novel fusion of recombinant human alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2) for the treatment of Sanfilippo syndrome type B or mucopolysaccharidosis IIIB (MPS IIIB). Designed to restore NAGLU activity in the brain, tralesenidase alfa is delivered directly to the fluid surrounding the brain (cerebrospinal fluid) by an intracerebroventricular infusion. Tralesenidase alfa has been granted orphan drug designation by the European Commission and the U.S. Food and Drug Administration. For additional information regarding the investigational product tralesenidase alfa, please contact BioMarin Medical Information at medinfo@bmrn.com until the end of December 2019. Beginning January 2020, please contact Allievex Medical Information at medinfo@allievex.com.

About Sanfilippo Syndrome Type B

Sanfilippo syndrome type B, a lysosomal storage disease, is caused by deficiency in the enzyme alpha-N-acetylglucosaminidase (NAGLU), one of the four enzymes required for heparan sulfate (HS) degradation. The first symptoms generally appear between the ages of two and six years and consist of behavior disorders, intellectual deterioration, sleep disorders and, in some cases, mild dysmorphism. With time neurological involvement becomes more prominent with progressive loss of motor milestones and communication problems. The prognosis is poor with death occurring in most cases in the late teens or early 20s.

About Allievex Corporation

Allievex, a clinical-stage biotechnology company founded by Pappas Capital, is developing novel therapies for the treatment of rare pediatric neurodegenerative diseases. Allievex's lead product candidate is tralesenidase alfa, an investigational enzyme replacement therapy using a novel fusion of recombinant human alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2) for the treatment of children with Sanfilippo syndrome type B or mucopolysaccharidosis IIIB (MPS IIIB). For more information, please visit www.allievex.com.

About Pappas Capital

Founded in 1994, Pappas Capital invests exclusively in innovative life sciences companies with a focus on biotechnology, biopharmaceuticals, drug delivery, medical devices and related ventures, across the United States and Canada. Pappas Capital has raised more than \$540 million and has guided the launch or development of more than 85 companies. In addition to its Pappas Ventures funds, the firm manages customized investment vehicles for third parties through its Specialized Fund Management Group and works with academic institutions in developing innovation through its Translational Medicine Research Initiative. For more information, please visit: www.pappas-capital.com.

About Novo Holdings A/S

Novo Holdings A/S is a private limited liability company wholly owned by the Novo Nordisk Foundation. It is the holding and investment company of the Novo Group, comprising Novo Nordisk A/S and Novozymes A/S, and is responsible for managing the Novo Nordisk Foundation's assets.

Novo Holdings is recognized as a leading international life science investor with a focus on creating long-term value. As a life science investor, Novo Holdings provides seed and venture capital to development-stage companies and takes significant ownership positions in growth and well-established companies. Novo Holdings also manages a broad portfolio of diversified financial assets. Further information: www.novoholdings.dk.

Investor Contact:

Thomas P. Mathers
Allievex Corporation
617-642-8677
tom@allievex.com

Media Contact:

Amanda Klein/Alex Jeffrey
Gasthalter & Co.
212-257-4170

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