



The Silverstein Foundation, REGENXBIO, and OrbiMed Launch Prevail Therapeutics

NEW YORK – August 9, 2017 – The Silverstein Foundation for Parkinson’s with GBA, REGENXBIO Inc. (“REGENXBIO”), and OrbiMed today announced the formation and launch of Prevail Therapeutics, Inc., (“Prevail”), a new company focused on discovering and developing novel biologic therapies for Parkinson’s Disease and other neurodegenerative diseases. Parkinson’s Disease is a progressive neurodegenerative disorder that affects more than 10 million people worldwide¹. Gene mapping in recent years has led to the identification of new genes and risk factors for Parkinson’s, including the glucocerebrosidase (GBA1) gene mutation. First observed in 2004, GBA1 is the most common of currently known Parkinson’s mutations, and it is estimated that up to 10 percent of people with Parkinson’s in the United States carry this mutation². Recent discoveries around the link between the GBA1 gene, glucocerebrosidase (GCase) protein production, and accumulation of alpha-synuclein in the brain may have implications for the broader Parkinson’s population², as well as in other neurodegenerative diseases. Prevail Therapeutics has assembled a world-class team to discover and develop novel disease-modifying treatments for patients.

In conjunction with its launch, Prevail has entered into an exclusive worldwide license agreement with REGENXBIO to develop and commercialize gene therapy products using REGENXBIO’s NAV AAV9 vector for the treatment of Parkinson’s Disease and other related neurodegenerative diseases.

“As a leader in gene therapy, we are pleased to collaborate with Prevail in the development of life-changing therapies for patients suffering from serious neurodegenerative diseases for which better treatment options are needed,” said Ken Mills, president and CEO of REGENXBIO. “We view the formation of Prevail as an important part of our mission to enable the use of our NAV Technology Platform to develop successful new therapeutics for patients suffering from severe diseases with significant unmet need.”

The Company was co-founded by Asa Abeliovich, M.D., Ph.D., faculty at Columbia University and co-founder of Alector, who will lead Prevail as Chief Executive Officer. “Current treatments for Parkinson’s Disease are focused on alleviating symptoms,” Dr. Abeliovich said. “Prevail is focused on developing disease-modifying therapies that slow, stop, and reverse the neurodegenerative process.”

Jonathan Silverstein, partner and co-head of Private Equity at OrbiMed, added, “We are proud to partner with REGENXBIO and Dr. Abeliovich in founding Prevail. We believe REGENXBIO’s NAV Technology Platform provides us with a promising opportunity to develop potential life-changing therapies for patients suffering from Parkinson’s Disease and other neurodegenerative diseases. Dr. Abeliovich’s leadership and deep expertise in the molecular mechanisms of neurodegeneration uniquely position Prevail to make a significant mark in developing innovative

biologic therapies for Parkinson's Disease and other central nervous system diseases with huge unmet medical needs.”

About Prevail Therapeutics

Prevail Therapeutics is a biotechnology company focused on developing novel biologic therapies for Parkinson's Disease and other neurodegenerative diseases. Prevail was launched in 2017 by The Silverstein Foundation for Parkinson's with GBA, REGENXBIO, and OrbiMed, and is headquartered in New York, NY.

About The Silverstein Foundation for Parkinson's with GBA

The Silverstein Foundation is a 501(c)(3) non-profit organization founded in 2017 focused on investing in cutting edge research with the goal of discovering new therapies for the treatment of Parkinson's Disease in glucocerebrosidase (GBA1) mutation carriers. For more information, please visit www.silversteinfoundation.org.

About REGENXBIO Inc.

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV® Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

About OrbiMed

OrbiMed is a leading investment firm dedicated exclusively to the healthcare sector, with over \$14 billion in assets under management. OrbiMed invests globally across the spectrum of healthcare companies, from venture capital start-ups to large multinational companies utilizing a range of private equity funds, public equity funds, royalty/debt funds and other investment vehicles. OrbiMed maintains its headquarters in New York City, with additional offices in San Francisco, Shanghai, Mumbai and Herzliya. OrbiMed seeks to be a capital provider of choice, with the flexibility to provide equity and debt capital solutions that are tailored to the unique needs of our portfolio companies. The firm's global team of over 80 professionals brings the resources and experience required to be an exceptional long-term partner in building world-class healthcare companies. www.OrbiMed.com.

1 “Statistics on Parkinson's.” *Parkinson's Disease Foundation*. Retrieved from http://www.pdf.org/parkinson_statistics

2 “GBA1 and Parkinson's Disease.” *The Michael J. Fox Foundation for Parkinson's Research*. Retrieved from <https://www.michaeljfox.org/understanding-parkinsons/living-with-pd/topic.php?gba1-parkinsons>

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