



NIMBUS

THERAPEUTICS

Nimbus and Shire To Co-Develop Therapies for Rare Diseases

Companies Enter Agreement to Co-Develop First-in-Class Small Molecule Therapies for Key Lysosomal Storage Disorders

CAMBRIDGE, Mass. – May 8, 2013 – Nimbus Discovery, LLC, a biotechnology company discovering novel medicines against exciting but previously inaccessible disease targets, today announced a co-development agreement with Shire plc focused on small molecule treatments for several rare genetic diseases known as lysosomal storage disorders (LSDs). The goal of the collaboration is to utilize the Nimbus breakthrough computational chemistry approach to discover and develop novel, disease-altering therapies. Many LSDs remain untreated because of challenges in creating drugs that can effectively reach the disease site. The significant potential of this partnership is the development of the first small molecule agents designed to penetrate inaccessible tissues while offering the convenience of an orally-administered pill.

The Nimbus Shire collaboration is the result of a joint assessment of a series of important rare disease targets with significant unmet medical need. One target was ultimately chosen to be the research focus. Under the terms of the agreement, Nimbus will use its cutting-edge research and development platform to extensively profile molecules against the agreed upon disease target and will deliver a drug candidate that is ready to enter late preclinical studies. Nimbus will control and conduct all related research up to achievement of drug candidate status at which point Shire will have an exclusive option to acquire the program. Shire will then be responsible for all clinical development and future commercialization activities. Nimbus is eligible to receive preclinical, development and commercial stage milestones commensurate with the innovative nature of the research and significant unmet medical need of the disease indications.

“This collaboration validates our computationally-driven, structure-based drug discovery engine and innovative partnering model,” said Rosana Kapeller, M.D., Ph.D., Chief

Scientific Officer of Nimbus. “Nimbus is thrilled to pursue our first alliance with Shire, a company that shares our passion for uncovering breakthroughs in highly sought-after disease targets that have proven inaccessible to traditional industry approaches.”

“Nimbus is ground-breaking in their approach to drug discovery and, in a short period of time, have already assembled an impressive track record in delivering clinical candidates for challenging disease targets,” said Dr. Philip J. Vickers, Senior Vice President, Research and Development, Shire. “As a leader in rare diseases, this partnership is another way for Shire to ensure that we expand into new disease areas and continue to apply cutting edge technologies in this space. This agreement complements Shire’s long-term commitment to bring innovative therapies to patients with rare diseases worldwide.”

This is the first deal announced through Shire’s strategic alliance with Atlas Venture, which identifies investments for early stage venture creation around rare genetic diseases.

About Lysosomal Storage Disorders

Lysosomal Storage Disorders are a group of approximately 50 rare inherited metabolic disorders that are caused by a lack of enzymes that normally eliminate unwanted substances in human cells. Lysosomes act as the “recycling center” of each cell, breaking down unwanted material into simple products for the cell to use for building new material. The lack or malfunction of certain enzymes causes a buildup of the molecules that the enzyme would normally eliminate, and deposits accumulate in many cells of the body. Abnormal storage impairs cell function and can cause damage to the body's cells, which can lead to serious health problems. Lysosomal storage diseases predominantly affect children, many of which die within a few months or years of birth if untreated.

About Nimbus

Nimbus Discovery, a biotechnology company, harnesses cutting-edge computational technologies to uncover breakthroughs in small molecule pharmacology. Nimbus focuses on medically important and highly sought-after disease targets that have proven inaccessible to traditional industry approaches. Nimbus’ robust pre-clinical pipeline includes novel agents for the treatment of cancer, metabolic disease and inflammation. The company is organized as a constellation of small, nimble teams of experienced drug-hunters deployed across program-focused subsidiary companies. Each team is

freed from conventional barriers to scientific success, chartered to create solutions, and geared for program asset deals with leading pharmaceutical companies. Founded in 2009, Nimbus partnered with Schrödinger to invent and apply a physics-based approach that establishes a new standard for rational drug design. Nimbus is backed by world-class life science investors, including Atlas Venture, SR One, Lilly Ventures and Bill Gates. For more information please visit www.nimbustx.com.