



Agilis Biotherapeutics Named as Top Company to Watch

Recognition at the Therapeutic Area Partnerships Conference Highlights Company's Growing Position in Gene Therapy

Cambridge, MA, November 19, 2015 7:30 am EST -- (BUSINESS WIRE)—Agilis Biotherapeutics, LLC (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced today that the Company has been chosen as one of the “Top Projects to Watch” at the 10th Annual Therapeutic Area Partnerships (TAP) Conference, taking place November 19-21, 2015, in Boston, MA.

Additionally, Dr. Mark Pykett, President and CEO of Agilis, presented in the Advanced Therapies session of the conference and participated as a panelist at the gene therapy roundtable.

TAP award winners are selected through an industry-wide evaluation process. “Selected companies have been screened using a strict set of judging criteria for the Top 10 award and represent what our committees consider among the most attractive opportunities the industry has to offer,” said Marc Wortman, Editorial Director, Therapeutic Area Partnerships. “Winners have met rigorous criteria, including: unmet medical need, market potential, diversity of indications, strong science, multi-level partnering opportunities (biotech and pharma), potential for new opportunities beyond initial indications and corporate stability. As the industry leader in strategic analysis and transaction tracking, our main goal is to give these companies exposure to potential investors, partners, and acquirers.”

In his presentation, Dr. Pykett highlighted recent advances in Agilis’ gene therapy development efforts in rare CNS diseases and its lead programs in Friedreich’s ataxia and Angelman syndrome. The Company recently announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to the Company’s gene therapy product candidate being

developed for the treatment of Angelman syndrome, the first time that Orphan Drug Designation status has been granted by the U.S. FDA for a drug in the indication of Angelman syndrome.

“We are pleased to have received recognition by TAP as one of the top companies to watch,” stated Dr. Pykett. “Agilis continues to make great strides in its innovative gene therapy platform and to create a highly differentiated business aimed at addressing the significant unmet medical need that patients with rare CNS disorders often face. We believe the TAP honor is representative of the strong outlook for the Company and its growing position in the field of gene therapy.”

About Agilis Biotherapeutics, LLC

Agilis is advancing innovative DNA therapeutics designed to provide long-term efficacy for patients with debilitating, often fatal, rare genetic diseases that affect the central nervous system. Our therapies are engineered to impart sustainable clinical benefits, and potentially a functional cure, by inducing persistent expression of a therapeutic gene. The Company’s technology is aimed at the precise targeting and restoration of lost gene function, while avoiding unintended off-target effects. Our integrated strategy increases the efficiency of developing DNA therapeutics into safe, targeted gene therapies that achieve long-term efficacy and enable patients to remain asymptomatic without continuous invasive treatment. Agilis’ rare disease programs are focused on gene therapy for Friedreich’s ataxia, Angelman syndrome and Fragile X syndrome, rare genetic diseases that include severe neurological deficits and result in physically debilitating conditions. Friedreich’s Ataxia is the most common hereditary ataxia with an estimated 5,000 to 10,000 patients in the U.S. There are an estimated 10,000 to 15,000 people living with Angelman syndrome in the US. Fragile X syndrome is the most common known cause of inherited intellectual disability with an estimated 64,000 patients living in the U.S.

We invite you to visit our website at www.agilisbio.com

Safe Harbor Statement

Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon our current expectations and projections about future events and generally relate to our plans, objectives and expectations for the development of our business. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release.

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