



Agilis Biotherapeutics Achieves FDA “Rare Pediatric Disease” Designation for AADC Gene Therapy Candidate

FDA Designation Enables Potential Award of Priority Review Voucher Upon Marketing Approval

Cambridge, MA, November 17, 2016, 7:30 am EST (BUSINESS WIRE) — Agilis Biotherapeutics, LLC (Agilis), a biotechnology company advancing innovative gene therapies for rare genetic diseases that affect the central nervous system (CNS), announced today that the Food and Drug Administration (FDA) has granted a Rare Pediatric Disease (RPD) designation for its gene therapy product candidate, AGIL-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency. The RPD designation supplements the Orphan Drug Designation granted by the FDA and announced by Agilis earlier this year. The Orphan designation allows Agilis to leverage the FDA’s Priority Review pathway, hastening patient access to AGIL-AADC by shortening the review of the marketing application by as much as four months. The complementary RPD designation now gives Agilis the ability to qualify for a Priority Review Voucher (PRV) upon approval of AGIL-AADC. PRV’s can be redeemed or transferred to a third party to receive Priority Review on a subsequent marketing application for a different product. This voucher will be requested at the time of AGIL-AADC’s marketing application and awarded upon approval of AGIL-AADC.

“We are pleased that FDA granted our request for rare pediatric designation for our gene therapy candidate for AADC deficiency, a devastating, life-limiting disease,” said Mark Pykett, CEO of Agilis. “Our mission for these patients is to provide a treatment option where none currently exists to improve their lives and reduce the burden on their caregivers. The Rare Pediatric Disease and Orphan Drug designations provide significant incentives in seeking marketing authorization in the United States that support our business strategies moving forward. RPD designation enables the potential award of a Priority Review Voucher, if the marketing application for AGIL-AADC is approved.”

AADC deficiency is a rare CNS disorder arising from a reduction in the enzyme aromatic L-amino acid decarboxylase that results from mutations in the dopa decarboxylase (DDC) gene. This reduction leads to deficits in the neurotransmitters dopamine, norepinephrine, epinephrine, serotonin and melatonin. In its profound forms, AADC deficiency causes severe developmental delays, the inability to develop motor strength and control (global muscular hypotonia/dystonia) resulting in breathing, feeding, and swallowing problems, frequent hospitalizations, and the need for life-long care that ultimately culminates in premature death within the first decade of life.

AGIL-AADC, an adeno-associated virus (AAV) vector containing the human gene for the AADC enzyme, is administered as a one-time treatment and 18 subjects with AADC deficiency have received AGIL-AADC to date in two prospective clinical cohorts using a single administration of the gene therapy. Subjects treated with AGIL-AADC have exhibited substantial gains in motor function and cognitive scales over multiple years following the single gene therapy treatment, as well as *de novo* dopamine production visualized by F-DOPA PET imaging and the emergence of dopamine metabolite biomarkers. In contrast, untreated subjects routinely show continued deterioration as the disease progresses, as observed in natural history cases. Recently, the Company announced that a third clinical cohort for AGIL-AADC has been initiated, a Phase IIb trial in which the first subject has been dosed.

About Rare Pediatric Disease Designation

According to a recently revised FDA statute (Advancing Hope Act of 2016 (Public Law 114-229), as defined in section 529(a)(3) of the Federal Food, Drug, and Cosmetic Act (FD&C Act) (21 U.S.C. 360ff(a)(3)), a rare pediatric disease is *“a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals from aged birth to 18 years, including age groups often called neonates, infants, children, and adolescents.”* Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application (NDA) or biologics license application (BLA) for a rare pediatric disease may be eligible for a voucher which can be redeemed to obtain priority review for a subsequent marketing application for a different product. The Priority Review Voucher may be used by the Sponsor or transferred an unlimited number of times. The U.S. Congress is currently considering legislation to extend the priority review voucher program and/or grandfather products that have already been granted pediatric designation but have not yet received marketing authorization.

About FDA Standard Review and Priority Review Designations

Prior to approval, each drug marketed in the United States must go through a detailed FDA review process. In 1992, under PDUFA, the FDA agreed to specific goals for improving the review time of NDAs and BLAs and created a two-tiered system of review times - Standard Review and Priority Review. Standard Review can be accomplished in a ten-month time frame from the time the application is filed by the FDA, which typically occurs approximately 60 days following submission of the application. A Priority Review designation is given to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. The FDA goal for reviewing a drug with Priority Review status is six months from the time the application is filed by the FDA.

About Orphan Drug Designation

Under the FDA's Orphan Drug Designation program, orphan drug designation is granted by the FDA to novel drugs or biologics that treat rare diseases or conditions affecting fewer than 200,000 patients in the U.S. The designation allows the drug developer to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of the drug, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical trial design assistance, and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

About Agilis Biotherapeutics

Agilis is advancing innovative gene therapies designed to provide long-term efficacy for patients with debilitating, often fatal, rare genetic diseases that affect the central nervous system. Agilis' gene therapies are engineered to impart sustainable clinical benefits and potentially a functional cure by inducing persistent expression of a therapeutic gene through precise targeting and restoration of lost gene function, while avoiding unintended off-target effects, thereby providing safe gene therapies that achieve long-term efficacy and enable patients to remain asymptomatic without continuous treatment. Agilis' rare disease programs are focused on gene therapy for AADC deficiency, Friedreich's ataxia, Angelman syndrome, rare genetic diseases that include severe neurological deficits and result in physically debilitating conditions.

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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