



Ultragenyx Acquires Global Rights to AAV Gene Therapy ABO-102 for Sanfilippo Syndrome Type A (MPS IIIA) from Abeona Therapeutics

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ABO-102 / UX111 is being evaluated in ongoing pivotal Transpher A trial in patients with MPS IIIA

Interim data featured in encore oral presentation at American Society of Gene & Cell Therapy (ASGCT) today

NOVATO, Calif. and NEW YORK and CLEVELAND, May 17, 2022 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) and Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced an exclusive license agreement for AAV gene therapy ABO-102 (now UX111) for the treatment of Sanfilippo syndrome type A (MPS IIIA). Under the terms of the agreement, Ultragenyx will assume responsibility for the ABO-102 program and in return Abeona is eligible to receive tiered royalties of up to 10% on net sales and commercial milestone payments following regulatory approval.

"Based on promising data from Abeona's clinical program, regulatory feedback to date, and our experience developing treatments for other MPS diseases, we believe ABO-102 has the potential to be a transformative therapy for patients with MPS IIIA," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "Our team's expertise in MPS and gene therapy clinical development makes this program a seamless integration, and it has the potential to be our first gene therapy to market. The Sanfilippo community has been waiting too long for a first treatment and we believe we can help accelerate this program."

"Data from the ongoing Transpher A trial demonstrate ABO-102 holds significant potential to improve outcomes for patients with MPS IIIA who experience relentlessly progressing neurodevelopmental and physical decline that is life-threatening at a very young age," said Vish Seshadri, Ph.D., Chief Executive Officer of Abeona. "We believe that Ultragenyx, with deep expertise in rare, genetic, metabolic lysosomal storage disorders and a demonstrated commitment towards MPS diseases, is the ideal partner to eventually bring ABO-102 to patients."

Abeona has completed a successful Type B meeting with the U.S. Food and Drug Administration (FDA) regarding the pivotal Transpher A trial to support filing and approval for ABO-102 for the treatment of patients with MPS IIIA. Interim results from the Transpher A trial presented in an encore presentation at the American Society of Gene & Cell Therapy (ASGCT) today demonstrate that neurocognitive development was preserved in children treated before 2 years old or with a development quotient (DQ) > 60 (n=10) within normal range of a non-afflicted child after treatment with ABO-102 (3×10^{13} vg/kg). The interim results also showed continued or stabilized cognitive function and behavioral progress using standard developmental assessments. Some of these patients have reached 24-months post treatment and stabilization or increase in cortical gray matter, total cerebral, and amygdala volumes have been observed. Statistically significant reduction in liver volume was seen with ABO-102 treatment. Dose-dependent and statistically significant reductions in cerebrospinal fluid and plasma heparan sulfate, demonstrating replacement of enzyme activity consistent with levels required for disease correction in the central nervous system, have been sustained in treated patients for two years after treatment. ABO-102 has been well-tolerated with no treatment-related serious adverse events and no clinically meaningful adverse events reported.

"MPS IIIA is characterized by severe neurodegeneration with debilitating symptoms for which there is currently no treatment," said Kevin Flanigan, M.D., director of the Center for Gene Therapy at Nationwide Children's Hospital in Columbus, Ohio, and Transpher A study principal investigator. "The promising results to date suggest a single intravenous dose of ABO-102 AAV-based gene therapy has the potential to help children with MPS IIIA sustain neurocognitive development when they are treated during early stages of their disease."

About ABO-102 / UX111

ABO-102 (now UX111), is a novel gene therapy in Phase 1/2 development for Sanfilippo syndrome type A (MPS IIIA), a rare lysosomal storage disease with no approved treatment that primarily affects the central nervous system (CNS). ABO-102 is dosed in a one-time intravenous infusion using a self-complementary AAV9 vector to deliver a functional copy of the SGSH gene to cells of the CNS and peripheral organs. The therapy is designed to address the underlying SGSH enzyme deficiency responsible for abnormal accumulation of glycosaminoglycans in the brain and throughout the body that results in progressive cell damage and neurodevelopmental and physical decline. The ABO-102 program has received Regenerative Medicine Advanced Therapy, Fast Track, Rare Pediatric Disease, and Orphan Drug designations in the U.S., and PRIME and Orphan medicinal product designations in the EU.

About the Transpher A Study

The Transpher A Study (ABT-001) is an ongoing, two-year, open-label, dose-escalation, Phase 1/2 global clinical trial assessing ABO-102 for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA). The study is intended for patients from birth to 2 years of age, or patients older than 2 years with a cognitive developmental quotient of 60% or above. ABO-102 gene therapy is delivered using AAV9 technology via a single-dose intravenous infusion. The study primary endpoints are neurodevelopment and safety, with secondary endpoints including behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, heparan sulfate levels in CSF, plasma and urine, and brain and liver volume.

Further details can be referenced here: <https://clinicaltrials.gov/ct2/show/NCT02716246>

About Sanfilippo syndrome type A (MPS IIIA)

Sanfilippo syndrome type A (MPS IIIA) is a rare, fatal lysosomal storage disease with no approved treatment that primarily affects the CNS and is characterized by rapid neurodevelopmental and physical decline, often by age three. MPS IIIA has a global incidence of one in 100,000 with a median life expectancy of 15 years.

Children with MPS IIIA present with progressive language and cognitive decline and behavioral abnormalities. Other symptoms include sleep problems and frequent ear infections. Additionally, distinctive facial features with thick eyebrows or a unibrow, full lips and excessive body hair for one's age, and liver/spleen enlargement are also present in early childhood. MPS IIIA is caused by genetic mutations that lead to a deficiency in the SGSH enzyme responsible for breaking down glycosaminoglycans, which accumulate in cells throughout the body resulting in rapid health decline associated with the disorder.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, for which there are typically no approved therapies treating the underlying disease.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at: www.ultragenyx.com.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Abeona's lead clinical program is EB-101, its investigational autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development. The Company's development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated gene and cell therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and potential commercial production of AAV-based gene therapies. For more information, visit www.abeonatherapeutics.com.

Ultragenyx Forward-Looking Statements and Use of Digital Media

Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations and projections regarding its business plans and objectives, the therapeutic potential and clinical benefits of its products and product candidates, expectations regarding the safety and tolerability of its products and product candidates, and future clinical developments or commercial success for its products or product candidates are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the ability of Ultragenyx and its third party partners to successfully develop product candidates, including ABO-102 / UX111, the effects from the COVID-19 pandemic on the company's clinical and commercial activities and business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, the potential for any license or collaboration agreement, including the company's license agreement with Abeona as described in this press release, to be terminated, uncertainty and potential delays related to clinical drug development, the company's ability to achieve its projected development goals in its expected timeframes, risks and uncertainties related to the regulatory approval process, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 5, 2022, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's investor relations website (<https://ir.ultragenyx.com/>) and LinkedIn website (<https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/mycompany/>).

Abeona Forward-Looking Statements

This press release contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. We have attempted to identify forward-looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition; continued interest in our rare disease portfolio; our ability to commercialize our EB-101 product candidate; obtaining a strategic partnership to take over development activities for ABO-102; our ability to enroll patients in clinical trials; the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies; the impact of competition; the ability to secure licenses for any technology that may be necessary to commercialize our product candidates; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; risks associated with data analysis and reporting; reducing our operating expenses and extending our cash runway; our ability to execute our operating plan and achieve important anticipated milestones; and other risks disclosed in the Company's most recent Annual Report on Form 10-K and other periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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