Dystrogen Therapeutics Announces That Gene Therapy Successfully Cuts Off Production of Neuron-Destroying Protein in Huntington's Disease

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CHICAGO, April 5, 2019 /PRNewswire/ -- Scientist from Dystrogen Therapeutics Corp. will present data supporting a potential cutting-edge therapy for neurodegenerative diseases caused by trinucleotide repeats, such as Huntington's disease (HD) and spinocerebellar ataxias (SCAs). Early data confirmed that the therapy has the ability to selectively silence only the mutated huntingtin protein, leaving the healthy huntingtin protein undisturbed, allowing it to participate in important processes of the cells' life. The novel treatment, based on RNA interference (RNAi), operates at a different stage of silencing than other therapies currently on the market. The company's approach preferentially silences the expression of a mutant variant of the gene responsible for CAG repeat diseases.

According to the therapy's inventor, in addition to being potentially beneficial for adult patients, this therapy allows for normal cellular development and function of the healthy huntingtin protein, making it a good therapeutic candidate in the pediatric population.

The solution delivers a universal genetic tool used for silencing the expression of the abnormal part of the gene containing expanded CAG repeats occurring also in 8 other genetic diseases caused by polyQ repeats. As a result, the investigators expect to achieve a significant delay in neurodegenerative symptoms. The team will be presenting the results during the 44th FEBS Congress (6-11 July 2019) and in the 24th Annual Meeting of the RNA Society (11- 16 June 2019).

"These findings are potentially significant for the treatment of Huntington's disease and SCA patients, and the ability to selectively silence CAG transcripts in the nucleus may prove to be critical for therapeutic efficacy of gene therapies for these diseases," stated Kris Siemionow, M.D., Ph.D., chief executive officer of Dystrogen. "Whereas most RNAi approaches that target the huntingtin protein are short acting, our long-term silencing effect provides a significant advantage in treatment of trinucleotide disorders."

"Taken together, these findings further support the feasibility of advancing this program through research and into development of a promising gene therapy with the potential to alleviate the toxicity caused by the mutated CAG in HD and SCA," he added. "These data illustrate the potential of our RNAi platform to degrade disease-causing genes, with the prospective to limit off-target toxicity. We are very pleased to have these data presented at a highly relevant conference for the field and look forward to further exploring this opportunity."

About Dystrogen Therapeutics

Dystrogen Therapeutics is a clinical-stage life sciences company committed to developing personalized therapies for rare genetic diseases. The company has two technology platforms, which focus on treating patients with rare diseases such as Duchenne muscular dystrophy, sickle cell anemia, and neurodegenerative disorders such as Huntington's disease and SCA.

In addition to its RNAi platform, the company has developed a chimeric cell therapy platform. Dystrophin expressing chimeras "DEC" are based on ex vivo fusion of allogeneic human myoblast derived from close relative donors with autologous human myoblast received from DMD patient, where chimeric cells maintain the ability to express normal dystrophin protein. DEC cells will increase the number/pool of normal myoblasts and will reduce inflammation and induce replacement of fibrotic tissue thus significantly improving muscle strength and function in DMD patients. The therapy minimizes immune response effect and the need for immunosuppression since the patient will recognize DEC cells as "self" cells. This new approach will be based on delivery and restoration of dystrophin in affected muscles preventing the premature loss of mobility and early mortality of DMD patients. The company is planning on enrolling patients for its DEC chimeric cell therapy Duchenne muscular dystrophy trial. This therapy offers a unique advantage and allows the patient's body and immune system accept the chimeric cell without rejection. Results have demonstrated that increased dystrophin levels correlate with improved functional outcomes. First clinical date from DMD therapy are expected in 2020.

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