SQZ Biotech and AskBio Announce Research Collaboration to Create Immune Tolerization Products for AAV Gene Therapies

Scientific leaders in cell and gene therapy collaborate to determine efficacy of SQZ tolerizing antigen carriers (TACs) containing AAV components to overcome patient immune responses to AAV

WATERTOWN, Mass. and Research Triangle Park, N.C., Nov. 7, 2019 — SQZ Biotechnologies (SQZ), and Asklepios BioPharmaceutical, Inc. (AskBio), announced a research collaboration to create tolerizing antigen carriers (TACs) containing AAV (adeno-associated virus) components to solve one of gene therapy’s biggest challenges – the barrier to treatment posed by patients’ immune systems generating neutralizing antibodies toward therapeutic AAVs. SQZ and AskBio will combine their proprietary cell and gene therapy platform technologies to open the door to new treatment paradigms with potential impact across many genetic diseases.

Gene therapies utilizing AAV vectors can be transformative for patients with genetic diseases, but neutralizing antibodies can prevent large populations of patients from benefitting from AAV gene therapies. Patients’ immune systems develop neutralizing antibodies after receiving their first dose of AAV, or they can be pre-existing. This collaboration will strive to give these patients access to novel therapeutics and enable them to take multiple or repetitive doses to gain the full, durable benefit these treatments can provide. Expanding patient eligibility and allowing repeat treatment could change the future of how products are developed and significantly impact the long-term health of millions in need.

“This is a tremendous opportunity to bring together the power of both cell and gene therapy for patients. AskBio has been an innovative leader in gene therapy and shares our patient-centric philosophy. By working together and leveraging the potential of both our platforms, we hope to bring more effective, more durable treatments to patients suffering from devastating rare genetic disorders,” said Armon Sharei, PhD, founder and chief executive officer of SQZ Biotech.

The collaboration between SQZ and AskBio will evaluate the administration of SQZ TACs and AskBio’s gene therapies to potentially address AAV immunogenicity. SQZ is a pioneer in cell therapy, and the company’s knowledge and expertise, as well as their advance capabilities in manufacturing, are critical to this collaboration’s approach to synergizing cell and gene therapies. Preclinical data from SQZ has demonstrated that SQZ TACs specifically inhibit undesired immune responses in multiple contexts, including AAV models. As a leader in the AAV field, AskBio brings expertise in AAV technology, capsid design, clinical processes and manufacturing that would allow for application of these novel methods to overcome immunogenicity. The two companies have a shared goal to increase world-wide access of transformative therapeutics.

R. Jude Samulski, PhD, chief scientific officer and co-founder of AskBio, noted, “AskBio is firmly committed to improving the lives of underserved patients, such as those suffering from Pompe, Huntington’s and various neuromuscular and central nervous system diseases. Addressing AAV immunogenicity is essential to the future of gene therapy as it is one of the most significant limiting factors plaguing the gene therapy space today. SQZ’s pioneering approach to tolerance could offer a solution to this problem.”
“Our collaboration with SQZ is exemplary of our goal to broadly explore potential redosing of AAV gene therapies,” added Sheila Mikhail, chief executive officer and co-founder of AskBio. “We are thrilled to be working with SQZ and are hopeful that this initial research collaboration utilizing two of the most promising therapeutic modalities currently available, cell and gene therapy, will ultimately provide options to improve patients’ immune response to gene therapy.”

About AskBio
Founded in 2001, Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, clinical-stage gene therapy platform company dedicated to improving the lives of children and adults with genetic disorders. AskBio’s gene therapy platform includes an industry-leading proprietary cell line manufacturing process known as Pro10™ and an extensive AAV capsid library. Based in Research Triangle Park, N.C., the company has generated hundreds of proprietary third-generation gene vectors, several of which have entered clinical testing. An early innovator in the space, the company holds more than 500 patents in areas such as AAV production, chimeric vectors, and self-complementary DNA. AskBio maintains a portfolio of clinical programs across a range neurodegenerative and neuromuscular indications with a current pipeline that includes therapeutics for Pompe disease, Limb Girdle Muscular Dystrophy and congestive heart failure as well as out-license clinical indications for Hemophilia (Chatham Therapeutics acquired by Takeda) and Duchenne Muscular Dystrophy (Bamboo Therapeutics acquired by Pfizer). For more information, visit www.askbio.com.

About SQZ Biotech
SQZ Biotech is a privately held, clinical-stage company creating innovative treatments by transforming cells into sophisticated therapeutics. Using its proprietary platform, SQZ has the unique ability to precision engineer virtually any cell type and deliver multiple materials, potentially resulting in powerful, multifunctional cell therapies for a range of diseases with an initial focus on cancer and autoimmune disease. The company’s initial applications leverage SQZ’s ability to generate target-specific immune responses, both in activation for the treatment of solid tumors, and immune suppression for the treatment of immune reactions and diseases. For more information please visit www.sqzbiotech.com.

About SQZ TACs
SQZ tolerizing antigen carriers (TACs) are being developed to induce tolerance to aberrant or unwanted immune activity. TACs are developed from red blood cells (RBCs) SQZ’d with target-specific antigens and piggyback on the natural process of RBC destruction in the body, also known as eryptosis. A process moderated by our liver and spleen, eryptosis causes macrophages to take up aged or senescent RBCs. When our bodies process RBCs for destruction, their components are presented in a tolerogenic manner, reminding our immune systems not to attack our own red blood cells. SQZ TACs drive targeted antigens through this powerful natural mechanism, specifically tolerizing the immune system, potentially stopping undesired immune responses.

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