GENES AND THERAPY – 8th DECEMBER 2021

Krystal Biotech announces positive results from gene therapy trial for rare skin disease

Krystal Biotech announced positive topline results from the pivotal GEM-3 trial of investigational beremagene geperpavec (B-VEC), now known as VYJUVEK™, for the treatment of dystrophic Epidermolysis Bullosa (dystrophic EB). VYJUVEK™ is an investigational non-invasive, topical gene therapy designed to deliver two copies of the *COL7A1* gene when applied directly to dystrophic EB wounds. Dystrophic EB is a rare and severe monogenic disease caused by a mutation in the *COL7A1* gene. The lack of a functional COL7A1 protein leads to absence of anchoring fibrils which causes extremely fragile skin that blisters and tears from minor friction or trauma.

AviadoBio™ joins as a new member on the list of gene therapy companies

AviadoBio Ltd announced on 2nd December, the successful completion of an $80 million (£58.6 million) Series A financing round, following an initial $16.5 million (£12 million) seed financing. The funds will be used to advance AviadoBio's lead program in frontotemporal dementia (FTD) into the clinic, progress its preclinical assets, including for amyotrophic lateral sclerosis (ALS), whilst continuing to expand its industry-leading team. AviadoBio's technology was developed in the laboratory of Christopher Shaw, Professor of Neurology and Neurogenetics at King's College London's Institute of Psychiatry, Psychology & Neuroscience, with the support of the UK Dementia Research Institute, the My Name'5 Doddie Foundation and the Van Geest Foundation and, incubated by F-Prime Capital and JJDC.

BrainStorm Cell Therapeutics and Catalent complete technology transfer for NurOwn® manufacturing

BrainStorm Cell Therapeutics and Catalent has completed the technology transfer for NurOwn® manufacturing at Catalent's facility. NurOwn is BrainStorm's autologous cellular therapy being developed for the treatment of amyotrophic lateral sclerosis, progressive multiple sclerosis and other neurodegenerative diseases. NurOwn® technology platform (autologous MSC-NTF cells) uses autologous, bone marrow-derived mesenchymal stem cells (MSCs) that have been expanded and differentiated ex vivo. MSCs are grown under patented conditions that induce the cells to secrete high levels of neurotrophic factors (NTF).

Astellas Pharma and Dyno Therapeutics collaborate to develop next-generation AAV gene therapy vectors

Astellas Pharma and Dyno Therapeutics has collaborated to develop next-generation adeno-associated virus (AAV) vectors for gene therapy directed to skeletal and cardiac muscle using Dyno's CapsidMap™ platform. Dyno's CapsidMap uses in vivo experimental data and machine learning to create novel AAV capsids. Under the agreement, Dyno will design novel AAV capsids with improved functional properties for gene therapy, while Astellas will be responsible for conducting preclinical, clinical and commercialization activities, including manufacturing, of gene therapy product candidates using the novel capsids.

Amarna Therapeutics raises funds for first-in-human clinical trial of hemophilia B

Amarna Therapeutics works towards developing gene therapies based on simian virus 40 (SV40)for a range of rare and prevalent diseases. It has secured €5 million funding to advance its lead gene therapy for the treatment of hemophilia B towards a first-in-human clinical trial, and to progress its R&D pipeline in selected autoimmune diseases and chronic inflammation.

Atamyo Therapeutics receives approval to start clinical trials in Europe for ATA-100 gene therapy

Atamyo Therapeutics has received its first authorization for clinical trial applications (CTA) in Europe for ATA-100, its gene therapy intended to treat girdle muscular dystrophy type 2I / R9 (LGMD2I / R9) linked to the protein FKRP (Fukutin-Related Protein). ATA-100 provides a normal copy of the gene for the production of the FKRP protein. This therapy is the result of research by Doctor Isabelle Richard, General Scientific Director of Atamyo, Research Director at CNRS and in charge of the Progressive Muscular Dystrophies laboratory at Généthon. LGMD2I / R9 is a rare genetic disorder where patients suffer from progressive muscle weakness leading to loss of walking.

Taysha Gene Therapies announces positive results for gene therapy for SLC13A5-related epilepsy

Taysha Gene Therapies announced positive preclinical data for TSHA-105, an AAV9-based gene therapy in development for SLC13A5-related epilepsy at the American Epilepsy Society Annual Meeting. SLC13A5-related epilepsy results from a mutation in the *SLC13A5* gene that prevents citrate from being taken up into neurons in the brain. TSHA-105 is a self-complementary vector encoding a codon-optimized human *SLC13A5* gene which when delivered in the cerebrospinal fluid, significantly

decreases plasma citrate levels in SLC13A5 KO mice and reducesepileptic activity. An IND/CTA filing for TSHA-105 for the treatment of SLC13A5-related epilepsy is expected in 2022.