GENES AND THERAPY – 19th JANUARY 2022

First patient dosed with novel chimeric cell therapy for Duchenne Muscular Dystrophy

Dystrogen Therapeutics dosed the first patient, a 6-year-old boy, in its Phase 1 Human Pilot Clinical Study of DT-DEC01, Dystrophin Expressing Chimeric cells, or DEC, for the treatment of Duchenne Muscular Dystrophy (DMD). This is a non-randomized trial enrolling boys between the ages of 5 and 18. The trial investigates the safety, tolerability, and efficacy of DT-DEC01. DT-DEC01 is a chimeric cell therapy that has yielded positive functional responses in preclinical studies. The DEC cells produce clinically significant levels of dystrophin, do not trigger immune response, and do not involve genetic manipulations, thereby reducing risk of off target mutations.

Fabry Disease gene therapy trial to be discontinued

Avrobio will discontinue recruiting more patients to its gene therapy trial for Fabry Disease. This is after the Phase II trial results showed variable engraftment and declining levels of the alpha galactosidase A enzyme, for which the gene therapy was performed with AVR-RD-01. The Phase I trials had shown positive favourable outcomes. Avrobio will now prioritize its focus on lysosomal disorder pipeline.

Positive outcomes from largest ongoing hemophilia A clinical trial

BioMarin's ongoing global Phase 3 GENEr8-1 study of valoctocogene roxaparvovec, an investigational gene therapy for the treatment of adults with severe hemophilia A, yielded positive results. The therapy restored levels of the blood clotting protein to that of mild hemophilia and trial participants showed very few bleeding episodes over two- or three-years' time. Its Marketing Authorization Application (MAA) is currently under review by the European Medicines Agency (EMA) and the company plans to submit regulatory submission to the FDA sometime in the second quarter of this year.

Duchenne Muscular Dystrophy gene therapy trial yields functional improvements in patients

Sarepta Therapeutics' ongoing single dose gene therapy trial SRP-9001-102 (delandistrogene moxeparvovec), part 2 of the SRP-9001 trial for Duchenne Muscular Dystrophy yielded positive results in patients with improvement in functional responses. Participants will be monitored for five years after treatment. SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein.

GMP plasmid DNA approval to bolster faster gene therapy development

The U.S. FDA provided acceptance of Andelyn Biosciences' GMP plasmid DNA. This will allow the gene therapy CDMO to integrate client's manufacturing process and condense timelines. Andelyn provides research and clinical grade plasmids to its clients, which is an important entity to develop gene therapies.

Sickle cell disease gene-edited cell therapy rights transferred wholly to Sangamo

The rights and obligations for SAR445136, a zinc finger nuclease gene-edited cell therapy candidate in development by Sangamo and Sanofi for the treatment of sickle cell disease will be transferred wholly to Sangamo over the first half of 2022. The preliminary Phase 1/2 clinical data for the trial is encouraging and the final patients in the trial will be dosed in the third quarter of this year.

Bayer and Mammoth Biosciences collaborate over CRISPR gene editing technology

Bayer and Mammoth Biosciences has entered a strategic collaboration through which Bayer will harness Mammoth's CRISPR gene editing technology to develop in vivo gene editing therapies. Mammoth will receive USD 40 million upfront and more than USD 1 billion upon achieving certain

milestones. This collaboration comes after Bayer bought BlueRock and AskBio to expand its gene and cell therapy pipeline.

New instrument for the therapeutics field

A new instrument launched by Halo Labs allows the identification and quantitation of excipient degraded products, an important concern in developing protein, drug, cell and gene therapies.