Press Release

CYTOO's partner Astellas Gene Therapies to present results of an AGT/ CYTOO collaboration on clinical candidate selection to treat DMD at the 2nd Gene Therapy for Muscular Disorders Summit

Grenoble, France, 5th April, 2022 – CYTOO and Astellas Gene Therapies (AGT) entered into a research collaboration in 2020 to select AGT's AAV gene therapy clinical candidate to treat Duchenne Muscular Dystrophy (DMD). Today, AGT will present the results of this collaboration at the 2nd Gene Therapy for Muscular Disorders Summit in Boston.
DMD is a rare and life-threatening genetic disorder that affects children–approximately 1 in 3,500 to 5,000 boys–and families. It is caused by mutations in the dystrophin gene that results in progressive muscle degeneration and weakness. By the early teens, most individuals with DMD have lost the ability to walk unassisted and their heart and respiratory muscles have also weakened. Without therapy, individuals with DMD usually die from cardiomyopathy and respiratory failure in their second decade of life.

CYTOO pioneered MyoScreen™, an *in vitro* R&D platform that enables the evaluation of therapies in patient-derived primary skeletal muscle cells under physiological conditions. The MyoScreen platform is compatible with most AAV-based gene therapies and facilitates the development of quantitative, image-based assays monitoring the activity and mechanism of action of these therapies. The collaboration between AGT and CYTOO assessed the activity of several AAV-mediated exon skipping candidates using a quantitative cell profiling assay developed at CYTOO. This assay employs AI-assisted comparisons of images from myotubes from healthy and disease donors to establish profiles that phenotypically distinguish healthy and patient cells. These profiles are then used to determine a Health-Score™ (% phenotypically healthy cells out of total) that evaluates the ability of therapeutic candidates to revert disease-associated phenotypes in patient-derived myotubes.

AGT's investigational exon skipping gene therapy approach to treat DMD uses an AAV vector, encoding modified U7 small nuclear RNAs (snRNA), to deliver an antisense sequence designed to induce cells to skip over faulty or misaligned sections of genetic code in the dystrophin gene, with the goal of restoring meaningful levels of a functional dystrophin protein. For the treatment of DMD, this approach has the potential to provide significant advantages over microdystrophin gene replacement strategies that produce a substantially truncated protein, which may limit the degree and durability of disease correction, as well as existing antisense oligonucleotide (ASO) therapies whose efficacy is limited by poor biodistribution to muscle tissue.

Luc Selig, CYTOO's CEO, said, "The data to be presented today show that MyoScreen is a unique platform to demonstrate and quantify the functionality of restored dystrophins in DMD myotubes treated with AGT's AAV-mediated exon sipping." Luc added: "We strongly believe that the ability to evaluate muscle therapies in patient-derived cells with quantitative and functional assays provides a much-needed alignment of preclinical discovery with clinical endpoints."

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About CYTOO

CYTOO is a drug discovery biotech company focusing on disorders affecting muscle health. CYTOO pioneered MyoScreen™, an *in vitro* R&D platform that enables testing muscle therapies in primary patient-derived skeletal muscle cells. Using innovative, image-based, high-throughput assays and quantitative functional assays, MyoScreen aids therapeutic discovery at all stages, from target identification to the development of clinical candidate potency assays. Through R&D partnerships, CYTOO has worked on more than 50 projects comprising RNA and gene therapies. To better support CMC related activities, CYTOO is establishing a GMP environment that will be operational in 2023.