DYSTROGEN THERAPEUTICS

August 18, 2020: *FDA has granted Dystrogen Therapeutics Rare Pediatric Disease (RPD) Designation and Orphan Drug Designation for the company's product candidate: Dystrophin Expressing Chimeric (DEC) Cell Therapy DT-DEC01*

Dystrogen Therapeutics, a biotechnology company advancing innovative therapies for rare genetic diseases that affect the musculoskeletal system, has announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) Designation and Orphan Drug Designation for the company's product candidate Dystrophin Expressing Chimeric (DEC) Cell Therapy DT-DEC01 for the treatment of Duchenne Muscular Dystrophy (DMD), a rare pediatric disease.

"We are pleased that FDA granted our request for rare pediatric designation for our cell therapy candidate for DMD, a devastating, life-limiting disease," said Dr. Maria Siemionow, MD, PhD, CMO of Dystrogen Therapeutics. "Our mission for these patients is to provide a treatment option where none currently exists to improve their lives and reduce the burden on their caregivers. The Rare Pediatric Disease and Orphan Drug designations provide significant incentives in seeking marketing authorization in the United States that support our business strategies moving forward. RPD designation enables the potential award of a Priority Review Voucher, if the marketing application for DEC Therapy is approved."

About Rare Pediatric Disease Designation

According to a recently revised FDA statue (Advancing Hope Act of 2016 (Public Law 114-229), as defined in section 529(a)(3) of the Federal Food, Drug, and Cosmetic Act (FD&C Act) (21 U.S.C. 360ff(a)(3)), a rare pediatric disease is *"a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals from aged birth to 18 years, including age groups often called neonates, infants, children, and adolescents."* Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application (NDA) or biologics license application (BLA) for a rare pediatric disease may be eligible for a voucher which can be redeemed to obtain priority review for a subsequent marketing application for a different product. The Priority Review Voucher may be used by the Sponsor or transferred an unlimited number of times. The U.S. Congress is currently considering legislation to extend the priority review voucher program and/or grandfather products that have already been granted pediatric designation but have not yet received marketing authorization.

About FDA Standard Review and Priority Review Designations

Prior to approval, each drug marketed in the United States must go through a detailed FDA review process. In 1992, under PDUFA, the FDA agreed to specific goals for improving the review time of NDAs and BLAs and created a two-tiered system of review times - Standard Review and Priority Review. Standard Review can be accomplished in a ten-month time frame from the time the application is filed by the FDA, which typically occurs approximately 60 days following submission of the application. A Priority Review designation is given to drugs that offer major advances in treatment, or provide a treatment where no adequate

therapy exists. The FDA goal for reviewing a drug with Priority Review status is six months from the time the application is filed by the FDA.

About Orphan Drug Designation

Under the FDAs Orphan Drug Designation program, orphan drug designation is granted by the FDA to novel drugs or biologics that treat rare diseases or conditions affecting fewer than 200,000 patients in the U.S. The designation allows the drug developer to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of the drug, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical trial design assistance, and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

About Dystrogen Therapeutics

Dystrogen Therapeutics has developed an engineered chimeric cell therapy which has been previously shown to improve cardiac and pulmonary parameters, as well as restore muscle function, in pre-clinical studies. For Duchenne's muscular dystrophy, the company has developed Dystrophin Expressing Chimeras "DECs". Using the company's proprietary technology, DECs are created by an ex vivo fusion of allogeneic human myoblast from a healthy donor with autologous human myoblast received from DMD patient. DECs have been shown to maintain the ability to express normal dystrophin protein in previously published pre-clinical studies. The proposed therapy does not target any specific gene mutation, and it does not require genome editing, and as a result it can be applied to all muscular dystrophies without limitations.

Safe Harbor Statement

Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon our current expectations and projections about future events and generally relate to our plans, objectives and expectations for the development of our business. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release.