A human iPSC-based cell therapy for Duchenne muscular dystrophy

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Duchenne muscular dystrophy (DMD) is caused by mutations in the dystrophin protein. The current standard of care cannot prevent the loss of mobility in adolescence and death in early adulthood. Sadly new therapeutic approaches, including exon skipping, read-through therapeutics and gene supplementation, have only limited efficacy.

Muscle stem cell therapy is a new treatment option to restore impaired muscle regeneration in DMD patients by injecting healthy muscle stem cells. These stem cells are intended to produce healthy muscle tissue with functional dystrophin protein in the patient and thus to convert the course of the disease to a mild or even asymptomatic state. Based on preliminary work of the researchers, they want to generate a new muscle stem cell population from induced pluripotent stem cells (iPSC) that can colonize and functionally improve 3D patient muscle tissue. In a proof-of-concept study, they will (1) identify the specific subpopulations during muscle stem cell differentiation, (2) test these for functional improvement in a patient-specific tissue model (potency assays), and (3) demonstrate muscle stem cell engraftment and regeneration by molecular analysis of the treated DMD tissues.