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An *ex vivo* gene therapy strategy to treat Duchenne muscular dystrophy using a novel 3D muscle stem cell culture system and CRISPR/Cas9–mediated genome editing

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Duchenne muscular dystrophy (DMD) is a genetic disorder caused by mutations in *dystrophin* gene and characterized by progressive muscle degeneration and a shortened life span and there is no effective treatment available. Muscle stem cells (MuSCs) exhibit robust myogenic potential *in vivo*, thus they provide a promising curative treatment for DMD and other muscle disorders. Because of scarce native adult MuSCs in muscle tissue and very limited source of biopsies from patients, *ex-vivo* expansion of freshly isolated MuSCs is highly desired to achieve a therapeutic cell dose. Here we develop a soft 3D salmon fibrin gel culture system that can selectively expand MuSCs from a small number of crude skeletal muscle cell preparations and faithfully maintain their regenerative capacity for at least two weeks in culture. Moreover, we used CRISPR/Cas9-mediated genome editing to correct the *dystrophin* gene mutation in MuSCs expanded by this system and restored the skeletal muscle *dystrophin* expression upon transplantation in mdx mice. Our studies established a reliable and feasible platform for *ex-vivo* expansion and direct gene editing of MuSCs, thus greatly advancing MuSC-based gene therapies for various muscle disorders.

Speaker Biography

Wen-Shu Wu has received his Doctoral degree in Cancer Biology from University of Texas MD Anderson Cancer Center and completed his Post-doctoral training at Dana-Farber Cancer Institute, Harvard Medical School. He has held faculty positions at several research institutes. He was an Instructor at Harvard Medical School, a Principal Investigator at Main Medical Center Research Institute and an Assistant Professor of the Program in Cell, Molecular and Developmental Biology at Tufts University School of Medicine. Before joining UIC, he was an Associate Scientist at Children's Hospital Oakland Research Institute (CHORI) and a Principal Investigator at the University of California Berkeley Stem Cell Center. He was recruited to University of Illinois at Chicago as a tenured Associated Professor in 2013.

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