Small Molecule Generation of Multinucleated and Striated Myofibers from Human Pluripotent Stem Cells Equivalent to Adult Skeletal Muscle

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SUMMARY

Researchers in the UCLA Department of Microbiology, Immunology and Molecular Genetics have developed a novel means of generating adult skeletal muscle-equivalent myofibers from human pluripotent stem cells.

BACKGROUND

Thousands of people a year are diagnosed with various muscle diseases including neuromuscular disorders, muscular dystrophies, and diseases resulting from metabolic and mitochondrial defects. Generating adult muscle fibers in vitro would allow research surrounding these diseases to more quickly discover therapies and treatments. However, to date no protocol has been identified which generates multinucleated, adult skeletal muscle-equivalent myofibers in vitro. Currently the best protocols used generate mononuclear embryonic muscle cells which are not equivalent to adult skeletal muscle.

INNOVATION

Professor Pyle and colleagues have identified a novel protocol for generating adult skeletal muscle equivalent myofibers in vitro from human pluripotent stem cells using a TGF-beta inhibitor. Following cues active during early human muscle development and utilizing the TGF-beta inhibitor, the myofibers generated are multinucleated, express dystrophin, and equivalent to adult skeletal muscle. Additionally, these adult myofibers provide the appropriate niche for skeletal muscle progenitor cells.

APPLICATIONS

- Generation of adult-like skeletal muscle and/or skeletal muscle progenitor cells for patient-specific autologous transplant
- Deriving patient-specific myofibers for in vitro drug screening and research
- Development of in vitro models of neuromuscular diseases, muscular dystrophies, metabolic and mitochondrial diseases which require functional adult myofibers
- Development of in vitro models of the skeletal muscle progenitor cell niche

ADVANTAGES

- Generation of adult skeletal muscle equivalent myofibers in vitro
- Small molecule use allows for inclusion in defined-media cell culture protocols

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INVENTORS

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OTHER INFORMATION

KEYWORDS

Human pluripotent stem cells, adult myofiber, in vitro muscle disease model, directed differentiation, small molecule inhibitor, TGF-beta

CATEGORIZED AS

- **Medical**
  - Disease: Metabolic/Endocrinology
  - Disease: Musculoskeletal Disorders
  - Research Tools
  - Stem Cell
  - Therapeutics
- **Research Tools**
  - Cell Lines
  - Reagents

RELATED CASES

2016-413-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Three-Step Method For Universal Enrichment, Expansion, And Maturation Of Skeletal Muscle Cells Derived From Human Pluripotent Stem Cells

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