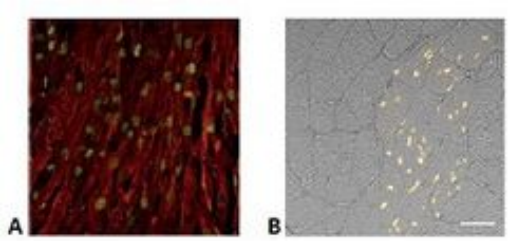
Technology Offer

Human satellite cells as cell therapy for the treatment of muscle waisting disorders

Reference Number 03-00399/32-00019

Challenge

There is a tremendous medical need for the treatment of muscle waisting disorders such as muscular dystrophies or local muscle atrophies (e.g. in urinary incontinence or ventilator-induced diaphragm dysfunction). Various cell-based approaches are currently pursued but generation of skeletal muscle fibers starting either from myoblasts or iPSC as a source of myogenic cells are still challenging. Satellite cells are stem cells of the skeletal muscle. They display an enormous potential for self-renewal and regeneration of skeletal muscle tissue and are thus indispensable for muscle generation. These primary human muscle stem cells can be found in low abundance in a specific stem cell niche of skeletal muscle and *ex-vivo* culturing and expansion is very difficult. Nevertheless, autologous satellite cells are a perfect source for a cell product aiming to regenerate muscle fibers.



A) Significant enrichment of pure myogenic cells after ht. B) HMFFs transplanted after ht result in large human myofibers containing human nuclei.

Technology

The technology provides an efficient method to generate muscle fibers from satellite cells based on human muscle fiber fragments (HMFFs) which are obtained routinely by biopsy. Special culture conditions and hypothermic treatment (ht) result in significant enrichment and expansion of satellite cells associated to HMFFs. The method allows expansion of PAX7 muscle stem cells up to billions while the stem cell character is maintained. Human muscle stem cells obtained by this method can be transplanted in an in vivo model where they generate muscle fibers and repopulate their stem cell niche. Currently, the manufacturing process is adapted to GMP standards, a preclinical testing plan is established and a clinical phase I study for a first indication is planned. This method allows the use of autologous satellite cells, reducing the risk for immunogenic effects, and enables also for long-term storage of this rare cell type. As a further application, these primary muscle stem cells can be an interesting source for screening approaches in drug discovery.

Commercial Opportunity

Available for licensing or co-development

Patent Situation

Patent applications pending in EP, US and JP basing on WO2016/030371, priority from August 25, 2014.

Further Reading

Journal of Clinical Investigation 2014, 124, 10, 4257-4265.
Mol Ther Nucleic Acids. 2016 Jan 19;5:e277.

MDC MAX-DELBRÜCK-CENTRUM FÜR MOLEKULARE MEDIZIN IN DER HELMHOLTZ-GEMEINSCHAFT

Licensing Contact:
Dr Michael Karle
Technology Manager
T: +49 30 948930-02
karle@ascenion.de

Ascenion GmbH
Herzogstraße 64
D-80803 München
T: +49 89 318814-0
info@ascenion.de
www.ascenion.de