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(54) Title: METHOD FOR GENE REPAIR IN PRIMARY HUMAN MUSCLE STEM CELLS (SATELLITE CELLS) IN VITRO
AND GENETICALLY REPAIRED HUMAN MUSCLE STEM CELL

(57) Abstract: The invention relates to a method for gene repair in primary human muscle stem cells (satellite cells) in vitro comprising the following steps: providing a sample of an isolated muscle-fiber containing tissue sample collected from at least one patient with a monogenic muscle disease, wherein the monogenic muscle disease is caused by at least one mutation in at least one gene encoding for at least one muscle protein; isolating and cultivating primary stem cells from said muscle-fiber containing tissue sample, and correcting the at least one mutation in the at least one gene encoding for at least one muscle protein in the cultivated primary stem cells by targeted modification of the at least one mutation by gene editing using CRISPR/Cas-based tools. The invention relates also to genetically repaired human muscle stem cell obtained in such a method.



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