Muscular dystrophy appears as an X-linked, recessive disorder. Taking into account that dystrophin (*DMD*) is the human largest gene (79 exons), the variability of the mutations is enormous. The gene for dystrophin can be disrupted by deletions (roughly 65%), duplications (10%), point mutations (10%), or other small rearrangements. The clinical symptoms are progressive muscular atrophy and persistent inflammation within the skeletal muscles.

Proposed Project will be focused on a search for complex and optimal strategy- using both genetically reprogrammed and redirected cells together with anti-inflammatory factor contained within prepeared nanocapsules. The aim of the proposed Project is to establish novel nano-biomedical method for treatment of the muscular dystrophy using stem precursor cells from patients genetically corrected *ex vivo* and applying advanced nanotechnological approach with biodegradable nanocapsules with anti-inflammatory agent. A **biotechnological approach** will enable to receive a large number of patient-specific cells, which will be modified using genetic engineering and genome editing methods in order to obtain the best therapeutic effect. The **nanotechnological approach** will enable the synthesis of innovative, thermosensitive nanocapsules containing an medical agent. The combination of biotechnology and nanotechnology will allow both therapeutic (pro-regenerative) and local anti-inflammatory effects to be achieved without application of systemic pharmacological treatment or other helping cells (e.g. mesenchymal cells). The proposed innovative approach is aimed to provide a more universal method for dystrophy treatment that could be feasible almost in every case of DMD with different types of mutations.

At the first stage of the Project, research will be focused on skeletal muscle stem precursor cells (myoblasts) isolated from dystrophic patients by muscle oligobiopsies. The cells will be genetically reprogramed using novel methodology to obtain induced pluripotent cells. The Project will be then focused onto differentiation of iPS into mesangioplast-like cells (HIDEMs) that will be modified using either genetic engineering or genome editing. The study will propose two therapeutic strategies -one using lentiviral vector carrying microdystrophin and the other one using the genome editing technique leading to utrofine activation. Following the modification HIDEMs, cells will be differentiated into patient-specific, myogenic cells. At this stage, the cells will be transplanted into the *mdx/scid* mice together with the biodegradable nanocapsules with anti-inflammatory agent, prepared during the nanotechnological part of the Project. *In vivo* studies on the *mdx/scid* mouse model will unambiguously identify the most optimal but also universal, therapeutic strategy.