Duchenne muscular dystrophy (DMD) is a dangerous genetic disease. X-linked genetic disorder, which may develop due to spontaneous or inherited mutations in the dystrophin gene. Only boys with a frequency of 1 in 3500 births suffer from it. In DMD, mutations in the genes encoding dystrophin - one of the muscle proteins - lead to disorders of the skeletal muscles, their degeneration, in the course of the disease, the next stage is the weakening of postural, respiratory and often myocardial muscles. Unfortunately, there is no effective medicine for this disease.

The aim of the study will be to apply and evaluate a new treatment method which is SM-SPCs and AT-MSC stem cell therapy.

We propose a method of DMD treatment consisting in the stem cells properties. Stem cells/progenitor cells of skeletal muscles (SM-SPCs) seem to be a proper candidate for cell therapy, they have the ability of damaged muscles rebuilding and form new contractile apparatus. The use of SM-SPCs co-transplantation together with a population of mesenchymal cells (AT-MSC) with anti-inflammatory properties may further improve the therapeutic effect. The cells will be implemented into dystrophic muscles by injection.

The tests will have a preclinical character and will be performed on laboratory animals - mice developing muscular dystrophy. The therapeutic effect will be assessed using a number of advanced methods: functional tests carried out on animals using telemetry, molecular biology analysis and electromyography evaluation of directly treated muscles. The collected data and results will allow the evaluation of the proposed therapy and could make a possibility of application in people.