DUX4 surexpression and silencing in a murine model

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FSHD is a muscular dystrophy characterized by muscle atrophy. It is caused by mis-expression of DUX4 transcription factor in skeletal muscle cells. Potential therapeutic agents (AOs, siRNA) inhibiting DUX4 expression in muscles showed promising results in vitro. Development of animal models to assess these antisense agent toxicity and efficacy constitutes a next step towards clinical trials for FSHD. However, no transgenic mouse expressing DUX4 and presenting a myopathy is available. So, we established two naked DNA injection methods in mouse hind limb muscles to express DUX4 and create muscle damage. Afterwards we will investigate the efficacy of therapeutic agent against DUX4 in these models.