

Study Name:	Clenbuterol to Target DUX4 in FSHD (Target FSHD): Open label safety and tolerability study of 3 doses of clenbuterol
Principal Investigator:	Jeffrey Statland, MD – University of Kansas Medical Center
Co-Investigators:	Johana Hamel, MD ; Leo Wang, MD; Stephen Tapscott, MD PhD, Seth Friedman, PhD
Introduction and Purpose of the Study	The primary goal of Target FSHD is to determine if clenbuterol is a therapeutic option for FSHD. This will be determined through a 6-month non-randomized open label study with three sequential cohorts
How many people will participate?	A total of 30 subjects will be enrolled in the study, 10 to each cohort
How do I know if I am eligible?	<p>Have to be:</p> <ul style="list-style-type: none"> • Genetically Confirmed FSHD type 1 or 2 • Between 18 and 75 years of age • Moderately affected • Able to walk 30 feet without the support of another person (participants are able to use orthotics, walking sticks, or canes) • Able to withstand MRI and Muscle Biopsy • Being able to lift your foot against gravity using the front shin muscle (tibialis anterior) or having a leg muscle that can be seen clearly on an MRI for a needle biopsy test <p>Cannot:</p> <ul style="list-style-type: none"> • Pregnant or planning to become pregnant during the study. • Have a serious medical condition that's not well-controlled (like cancer, diabetes, heart, liver, or kidney disease). • Participated in another experimental study within the last 3 months. • Taking medications like beta-blockers, anabolic agents (like testosterone), or diuretics that affect potassium levels. • Taking or planning to take a GLP-1 Agonist during the study.

	<ul style="list-style-type: none"> • Have any condition that would interfere with tests (like MRI, muscle biopsy, or motor function tests) or prevent the use of beta-agonists. • Taking blood thinners or medications that make a muscle biopsy unsafe. • Lactose intolerant or have any issues with lactose. • Taking any medications or treatments that conflict with clenbuterol.
If I am eligible, what will I do?	You will be asked come to site to be screened for the study. Visits will consist of Health-related questionnaires, strength and motor function assessments, physical exams, blood draw, ECG, MRI and Muscle Biopsies. There will be a total of 5 onsite visits within a 6-7 month period. If subject makes it to baseline visit they will be administered Medication that will be taken twice daily while at home.
How much will this cost me?	There is no cost to participate in this study.
Can you help me with my travel expenses?	Yes subjects will receive a \$50 stipend for every onsite visit completed, and reimbursement will be available for travel after approval of receipts.
Who can I contact for more information?	<p>Rebecca Clay- University of Kansas Medical Center Phone: 913.945.9936 rclay@kumc.edu</p> <p>Michaela Walker- University of Kansas Medical Center Phone: 913.945.9920 Mwalker20@kumc.edu</p>

THANK YOU FOR YOUR CONSIDERATION.