Muscle mass influences upon response to therapy

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Meet the Inventors

Koeberl, Dwight
Kishnani, Priya

Contact For More Info

Thomas, Dennis
919-681-7580
dennis.thomas@duke.edu

Department

Pediatrics (Dept. & CRU)

Duke University is seeking a company interested in commercializing a drug treatment method for enhancing lysosomal storage disease therapy by modulating cell surface receptor expression. Lysosmal storage diseases (LSDs), including Pompe disease, Gaucher disease, Farby disease and Niemann-Pick disease, are inherited metabolic disorders that result from the malfunction of the lysosomes. The patients suffer from developmental delay, seizures, organ problems to death. Currently, there is no cure for LSDs and the only non-invasive treatment is enzyme replacement therapy (ERT). Though efficacious in some cases, ERT can be ineffective in others due to high requirement of enzymes but inherent low expression of cell surface receptors as well as a variety of other reasons. A method to trigger high expression of enzyme receptors will greatly enhance the efficiency of ERT and hence create a combined market of $2 billion for LSDs1.