

Cure JM 2022 Grant Recipient: Duke Children's Hospital

Lay Summary

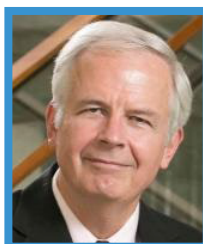
In children, there are rare muscle conditions called inflammatory myopathies that cause weakness and, in the most common one, juvenile dermatomyositis (JDM), a rash. Symptoms may start after a cold or flu-like illness, but unlike these, the symptoms don't subside and require treatment with strong medications that affect the immune system. These medications usually control but do not cure the disease. Researchers have learned much about JDM; however, how it occurs, why it persists, how it causes damage, and how to optimally treat it are questions that remain. The rare nature of JDM makes it difficult to answer these questions because, with rare diseases, there are not enough patients to test all of the hypotheses around the disease. One way to address this issue is to develop a model of the disease in an animal or in cells where the disease behaves like it does in humans. This allows researchers to have many copies of the model to test different therapies to see which are safe and effective. A promising treatment can then begin to be tested in humans with the disease.

In JDM, models have yet to be developed that allow for manipulations focusing on factors that are expected to cause disease. To address this, we have built a 3-dimensional model from healthy human skeletal muscle to learn more about JDM. We have found our model shows changes in muscle similar to JDM when exposed to conditions that contribute to muscle damage in JDM. In this project, we will test additional systems presumed to contribute to disease, and we can then use the model to test new and repurposed drugs as treatment for myositis.



Jeffrey Dvergsten, M.D.

Dr. Dvergsten is a pediatric rheumatologist and physician-scientist who has built his clinical and research career around juvenile myositis. He co-founded Duke's Myositis Clinic, a designated Cure JM Center of Excellence. He also created and manages a repository for research biospecimens from children with inflammatory myopathies.



George Truskey, PhD.

Dr. Truskey is a biomedical engineer and distinguished professor at Duke who has expertise in human vascular and skeletal muscle microphysiological systems. He runs a basic science research lab focused on 3D human tissue models of skeletal muscle and vascular diseases.



Lauren Covert, M.D.

Dr. Covert is a pediatric rheumatology fellow at Duke who is passionately interested in rare autoimmune and autoinflammatory diseases. She has been conducting basic science research focused on the disease mechanisms and therapies for juvenile dermatomyositis in collaboration with Drs. Dvergsten and Truskey during her fellowship.

Impact of Cure JM Funding

Cure JM funding will foster collaborative research efforts, enable our team to build on promising preliminary data, and support us in answering important and distinct clinical questions. Cure JM's support makes understanding this complex and diverse disease possible while empowering a network of patients, clinicians, and scientists to achieve better outcomes and an eventual cure for patients with juvenile myositis.