CureJM Grant recipient:

NATA UK

Lay Summary

During an infection or injury, a protein called interferon beta signals to the immune system, creating a cascade of events in the immune system that leads to "inflammation." Inflammation is typically beneficial, with immune cells acting like first responders and helping trap bacteria and agents that are foreign to the body. In JDM patients, this interferon signal is strong even when no infection is present, causing damage by the immune system to muscles and skin. The goal of this project is to selectively decrease interferon levels and thereby decrease inflammation. The NATA approach is to slow down the cellular response that manufactures interferon. The machinery being targeted is called messenger ribonucleic acid, or mRNA. Blocking the mRNA will stop the mechanism that produces interferon, thereby reducing inflammation and tissue damage.

A panel of molecules designed by a computer to block the cellular machinery for interferon will be manufactured and then tested against cells that have an overproduction of interferon. A feedback process will be followed to generate a molecule that can selectively target just the interferon system. Once a lead compound has been selected, it will be tested against cells isolated from JDM patients to determine how active it is at slowing down JDM.

The lead nucleic acid compound will then be tested in primary cells from JDM patients to evaluate its performance in cells isolated from patients with JDM.

Key People

Dr. Joanna Parkes

I first became aware of myositis in the final year of my Genetics degree at the University of Manchester, UK. In 2014 I went on to secure several awards to complete a Ph.D. investigating various aspects of myositis. After completing my Ph.D., I was awarded a Myositis Association Mentored Research Fellowship to attend Binghamton University (BU), New York, and continued my myositis research. At the BU School of Pharmacy and Pharmaceutical Sciences, I undertook studies into the response of myositis muscle to the biologic drug rituximab, and the impact new drugs and interferon beta have in cells from myositis patients.

As part of my research at BU, I also investigated the immune response to gene therapy in Duchenne Muscular Dystrophy; this introduced me to the field of nucleic acid therapeutics (NATs). NATs are a relatively new class of drugs that treat diseases by changing molecular machinery.

In 2022, I took a position as a research scientist in the biology department of the recently established Nucleic Acid Therapy Accelerator (NATA) hub at the Harwell Science and Innovation Campus in Oxfordshire, UK. NATA is a UK national research initiative with a mission to accelerate the development of nucleic acid therapeutics. I was responsible for generating a plan for developing a "NAT" therapeutic for juvenile dermatomyositis (JDM).

Impact of Cure JM Funding

At NATA, I work across projects with industry and academic collaborators. This Cure JM grant gives me the resources and research time to dedicate to this project. I am grateful for the opportunity to continue my myositis research career, lead my own research project, and ultimately contribute to developing sorely needed treatments for JDM.