Families of SMA Canada has awarded a $140,000 research grant to Rashmi Kothary, MD, at the Ottawa Hospital Research Institute for his project, " Muscle satellite cell biology and muscle regeneration in Smn-depleted mice."

Because of a genetic mutation, individuals with SMA don't produce survival motor neuron protein (SMN protein) at high enough levels. We know that this lack of SMN protein means that motor neurons don't work properly, and eventually shrink and die, but researchers are also looking at other ways the lack of SMN protein might impact those with SMA.

By learning more about the different ways that loss of SMN protein affects the body, researchers may be able to identify new ways to treat SMA, or new ways to evaluate whether potential treatments are effective.

Dr. Kothary's project will look at how SMN protein affects muscle satellite cells, which help the body respond to muscle damage.

The grant to Dr. Kothary is funded by Families of SMA Canada.

Meet Dr. Kothary

Who are you?

My name is Rashmi Kothary and I am a Senior Scientist and Deputy Scientific Director at the Ottawa Hospital Research Institute. My formal training was in mouse molecular genetics and I have used this expertise to study neuromuscular development and disease.

How did you first become involved with SMA research?

My introduction to SMA research occurred when I recruited Dr. Christine DiDonato to my laboratory as a postdoctoral fellow many years ago. She had the SMA genetics expertise and we had the mouse modeling expertise. Together, we worked on generating a novel mouse model of SMA that we now use to get a better understanding of the various cell types that are important in the etiology of the disease.

What is your current role in SMA research?

One major aspect of my lab's work is to understand the role played by skeletal muscle in the overall disease burden in SMA.

What do you hope to learn from this research project?

Our goal is to better understand how depletion of Smn impacts on the generation and function of muscle satellite cells, which help muscle respond to damage. The effect of Smn depletion on the capacity of skeletal muscle to regenerate in a mouse model of SMA will also be assessed.

How will this project work?

We will study what aspects of muscle satellite cell biology and muscle regenerative capacity are affected upon Smn depletion using genetic mouse models, single myofiber culture, cell biology, and cell transplantation.

What is the significance of your study?

The proposed studies will provide insight into several aspects of muscle satellite cell biology at multiple stages during disease progression in Smn depleted-mice. This will expand our understanding on the defects in muscle satellite cell,

regenerative potential, and muscle regeneration upon Smn depletion. Identification of novel muscle abnormalities and potential molecular targets will benefit research into the basic and clinical aspects of SMA.

Basic Research Funding

This grant to Dr. Kothary is part of $770,000 in new basic research funding that CureSMA is currently announcing.

Basic research is the first step in our comprehensive research model. We fund basic research to investigate the biology and cause of SMA, in order to identify the most effective strategies for drug discovery. We also use this funding to develop tools that facilitate SMA research.