



Cycle I Innovative Research Grants Initiative Awards Announced

June 28, 2021 - The National MPS Society's Board of Directors is pleased to announce the results from Cycle I of the Society's 2021 Innovative Research Grants Initiative. Overall, the first Cycle of Grant Proposal submissions, review, and awards identified 5 proposals totaling \$350,000 in award funds. Cycle II is accepting Letters of Intent until July 15, 2021.

Tier I Research Grant Awards

Dr. Heather Flanagan-Steet: Greenwood Genetic Center

"Comparative Analysis of Mechanisms Driving Skeletal Dysplasia"

Dr. Flanagan will be using zebrafish models of MPS II, MPS IVA, and ML II, to identify common pathways involved in bone and cartilage disease, with a specific focus on biological pathways that may characterize future potential drug targets to improve disease progression in these syndromes.

This award is for a total of two years.

Funded with Society support from ML and MPS IVA Research Funds

Dr Calogera (Lilla) Simonaro: Mt. Sinai Medical School

"Investigation of the Endocannabinoid System (ECS) as a Novel Therapeutic Target for the MPS"

Dr. Simonaro will be using the MPS IIIA mouse to investigate changes in the endocannabinoid system in MPS disease. The ECS is a complex biological network in people and animals that is involved in both neurological and inflammatory biology. This is the same system in the body that products like CBD oil interacts with. As part of their study, Dr. Simonaro will be looking at potential drug candidates to both study the changes in the ECS in MPS disease and to see if drugs can intervene and improve aspects of disease. As a heparan sulfate storage disorder, information gained from studying MPS IIIA, may well predict changes in the ECS of other MPS syndromes with heparan sulfate storage including MPS I, MPS II, MPS IIIA-IIID, and MPS VII.

This award is for a total of two years.

Funded with Society support from MPS III and General Research Funds



Cycle I Innovative Research Grants Initiative Awards Announced

Tier II Research Grant Awards

Dr. Michelina Iacovino: The Lundquist Institute

"Role of Autoimmune Disease in MPS Pathology"

It has been known for some time that there is an increase in inflammation in lysosomal storage diseases and in the MPS disorders in particular. There have been persistent questions about what other aspects of the immune system of MPS patients may not be functioning normally. Dr. Iacovino and her collaborator Dr. Lynda Polgreen and colleagues have identified potential aspects of autoimmune activation in MPS III, such that there is a chance that immune modulating drugs might be able to address aspects of their disease. This grant will provide support to help confirm the aspects of this part of MPS disease.

This award, as a Tier II Research Grant Award, is for one year.

Funded with Society support from General Research Funds

Dr. Igor Nestratil: University of Minnesota

"MPS II Brain Phenotypes"

With the potential advent of MPS II Newborn Screening on the horizon, there is a pressing need to predict phenotype as well as to better understand the neuropathology that is evident in MPS II. Sophisticated diagnostic imaging involving magnetic resonance imaging (MRI) is an excellent non-invasive approach to this area of study. Using previously predicted sets of MRIs from MPS II patients, Dr. Nestratil and colleagues aim to identify MRI characteristics that may help distinguish patients with the potential for cognitive signs versus normal cognition. This information may assist in both treatment decisions as well as assist in tracking and improving therapy development by using MRI as a predictive biomarker.

This award, as a Tier II Research Grant Award, is for one year.

Funded with Society support from MPS II Funds



Cycle I Innovative Research Grants Initiative Awards Announced

Fellow-Initiated Research Grant Award

Dr. Brianna Yund: University of Minnesota.

Dr. Julie Eisengart, mentor.

There has long been speculation that mild cognitive issues may exist within a variety of MPS syndromes and clinical forms where overall cognition and function appears intact. Any such issues could be clinically significant. Hence there is a need to evaluate these otherwise non-neuropathic syndromes and forms of MPS disease, for more subtle, but clinically significant issues of cognition and CNS function. Dr. Brianna Yund and her mentor, Dr. Julie Eisengart and colleagues will evaluate patients with non-neuropathic disease drawn from multiple syndromes including MPS I, MPS II, MPS IVA, and MPS VI.

This award, as a Fellow Initiated Research Grant Award, is for one year.

Funded with Society support from MPS VI Funds