

#### Funding myositis research

The Myositis Association's mission is to find a cure for inflammatory and related myopathies, while serving those affected by these diseases.

Scientists and physicians volunteer their time and expertise to assist in evaluating research applications and to provide guidance to TMA.



#### **TMA Research Committee**

- Lisa Christopher-Stine, Johns Hopkins Myositis Center
- Brian Feldman, MD, The Hospital for Sick Children
- Gerald Hengstman, MD, PhD, Netherlands
- Jerry Mendell, MD, Columbus Children's Research Institute
- Lawrence Pestronk, MD, Washington University
- Lawrence Phillips, MD, University of Virginia
- Robert Wortmann, MD, Dartmouth Medical Center
- Steven Ytterberg, MD, Mayo Clinic Rochester



#### **TMA Research Awards**

#### **Research Grants**:

"Seed monies" -- grants of up to \$50,000 per year for 2 years. These are grants for new or innovative research projects.

Established projects – grants of up to \$100,000 per year for 2 years.

#### **Research Fellowships**:

Research Fellowships -- \$50,000 per year for 2 years. These are designed to encourage promising postdoctoral investigators to begin a career in the study of inflammatory myopathies.



#### TMA Research Funding So Far

# In 7 years, TMA has awarded research funding of over \$3 million.

The range of research has been extended to include U.S. and international centers along with international MAB members.

Recent funding has focused on improving therapies.



#### Institutions Receiving Funding

- Friedrich Bauer Institute, Germany
- USC, Keck School of Medicine
- Thomas Jefferson University
- Children's Memorial Hospital (Chicago)
- Baylor University College of Medicine
- National Institutes of Health
- Johns Hopkins Medical Center
- St. Elizabeth's Medical Center



## Institutions Receiving Funding

- University of Oklahoma
- Mayo Clinic, Rochester
- Ichikawa General Hospital, Japan
- Istituto G. Gaslini, Genova, Italy
- Karolinska Hospital, Stockholm
- Ludwig-Maximilians-University in Munich, Germany



## Institutions Receiving Funding

- University of Pittsburgh
- Columbus Children's Research Institute
- Institute of Neurology, Rome, Italy
- University of Kentucky



#### **Funded Investigators**

- Sabine Krause, MD, PhD
- Valerie Askanas, MD, PhD
- Carol M. Artlett, BSc, PhD
- Lauren Pachman, MD
- Xiao-Feng Yang, MD, PhD
- Gulnara O. Mamyrova, MD, PhD
- K. Nagaraju, MD
- Ingrid Lundberg, MD, PhD



#### **Funded Investigators**

- Henry Querfurth, MD
- James Jarvis, MD
- Consuelo Lopez, MD
- Satoshi Okada, MD
- Angelo Ravelli, MD
- Stina Salomonsson, MD
- Hanns Lochmueller, MD
- Nicola Ruperto, MD



#### **Funded Investigators**

- Terry Oh, MD
- Eun-Ha Kang, MD
- Brian Kaspar, PhD
- Jerry Mendell, MD
- Christa M. Studzinski, PhD
- Massimiliano Mirabella, MD, PhD



- The MHC Processing and presentation machinery: clues for the immunopathogenesis of idiopathic inflammatory myopathies
- The role of excess cholesterol in the disease process of inclusion-body myositis.
- Characterization of Maternal Microchimeric Cells in Males with Juvenile Myositis
- Are lymphocytes activated at 10 years after diagnosis of Juvenile Dermatomyositis?



- RNA Exosome-specific Immune Responses and Autoimmune Myositis
- Completion of the Database for Childhood Myositis Heterogeneity Study
- Mechanisms of muscle fiber damage and dysfunction in myositis
- Juvenile myositis in Native Americans



- The role of Chimeric Dendritic cells in the inflammatory process of Juvenile Dermatomyositis
- The role of granulysin in muscle fiber injury in idiopathic inflammatory myopathies
- A multicenter study of the long-term outcome of juvenile idiopathic inflammatory myopathies
- Muscle weakness experienced by patients with IIM-is there a connection between IL-1a and muscle function?



- Study of the molecular pathogenesis of inclusion body myositis.
- Study of how the immune system affects muscle function in patients with polymyositis and dermatomyositis
- To recruit patients and medical centers from 46 countries to determine the treatment regimen associated with the lowest occurrence of flare and the lowest drug related toxicity in juvenile dermatomyositis.



- Characterization of CD4+CD8+ double positive T cells in dermatomyositis
- Gene therapy to carry a follistatin gene into quadriceps muscle to enlarge and strengthen the quadriceps
- To classify the condition of myositis patients worldwide in order to assess the most effective treatments
- Explore a stem-cell based muscle regenerative therapy for inclusion-body myositis.

# Recent Funded Projects are Focused on Treatments

- To study whether gene therapy can be used to increase muscle mass and strength in humans
- To study whether a new high-tech "smart brace" device is effective for people with IBM and knee instability
- To study a drug that improves cognition in Alzheimer's patients and reduces amyloid-beta to see whether it will also work to reduce the effects of amyloid-beta in the IBM-experimental human muscle culture model.
- To examine the ability of diet and exercise to prevent, halt, or reverse the disease process of IBM.



#### The Myositis Association

- www.myositis.org
- tma@myositis.org
- 1-800-821-7356
- The Myositis Association 1737 King Street, Suite 600 Alexandria, VA 22314