

## Cure JM Launches Medical Network

We are excited to announce our recent launch of the **Cure JM Medical Network**. Our NEW information-sharing network was created for you: physicians, nurses, researchers, physical therapists and other healthcare professionals who are committed to improving the lives of patients with Juvenile Myositis. The purpose of this network is to ensure that healthcare providers remain well-informed about research advances, evolving standards of care, and other information of importance to Juvenile Myositis patients and your practice.

**Cure JM invites you to join the Cure JM Medical Network** by filling-out the form on the back page of this newsletter and mailing or faxing it to Cure JM. Or simply register at [www.curejm.org/reg](http://www.curejm.org/reg). As a valued partner, you will receive a complimentary copy of the new e-book, *Myositis and You*. You will also receive our quarterly newsletters to keep you informed of drug trials, research grant opportunities, upcoming conferences, and much more.

Thank you for your contributions and dedication to the families and children who are fighting JM!

Please contact us at [physicians@curejm.org](mailto:physicians@curejm.org) if you have any questions or would like patient support information (welcome kits and brochures) from Cure JM.

## Cure JM Foundation Currently Funded Research

Cure JM has recently approved a round of new grants and is now currently funding sixteen research projects in addition to two Cure JM Centers of Excellence in Chicago and Washington, D.C.

"Our mission at Cure JM is to advance the research leading to improved treatments and a cure for Juvenile Myositis," says Jim Minow, Executive Director. "Increasingly we need to move our basic research to the patient, and many of our recent grants represent an important step in that direction."



*Cure JM Executive Director James Minow*

Targeted areas of research emphasis include **genetics and genomics**, where Cure JM is funding five investigations, plus **drug development**, **biomarker discovery**, and **heterogeneity studies**.

"In the immediate future, Cure JM families will see a dramatic burst of interest and expansion in these areas of scientific discovery" says Minow. "There is a strong history of collaboration within the JM research community. This is a tremendous asset and a great advantage in racing toward a cure."

Understanding the genetics of Juvenile Myositis holds the key to finding improved treatments and perhaps even "precision" treatments tailored to an individual patient. "We really must understand the complex role of genetics and the relationship to environmental factors," in this disease, says Minow, "and this is why we will expand our efforts in this area. It's a promising path to drug development."

**Dr. Claire Deakin** at University College in London is leading a project to study genetic risk factors for JM. By studying large numbers of patients from the United States and the UK, this project will also be able to study genetic risk factors for developing specific features, such as the formation of calcium deposits. The goal is to connect biological outcomes of JM to specific genetic aberrations, perhaps allowing for more targeted interventions. *(continued on next page)*

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Similarly, **Dr. Rebecca Nicolai** at Bambino Gesù Hospital in Rome aims to measure interferon gene expression in JM muscle, potentially leading to the discovery of additional biomarkers to measure disease activity. This is important because the pathogenesis of JM is not understood, although it is likely that aberrant interferon expression plays a major role. Finding potential biomarkers opens the possibility for new drug treatments that inhibit interferon expression in JM.

**Dr. Paul Norman**, a senior research scientist at Stanford University, is investigating the entire HLA genomic region using a new and powerful method he developed. The HLA region is associated with Myositis, and previously uncharacterized genes may interact with HLA genes to become risk factors for disease. Understanding of these risk factors opens the door for new diagnostic indicators, treatment targets and, in the long-term, prevention of the disease through directed gene-therapy approaches.

These three projects are representative of a larger body of work funded by Cure JM (*for more information, see Page 7*). Understanding genetic aberrations or “misspellings” will provide targets for drug and compound testing, another new major research project of Cure JM.

Cure JM has invested in a 3-year drug development program at the **National Center for Advancing Translational Sciences (NCATS) at NIH**, led by **Dr. Jim Inglese**. Cure JM’s funding supports the team’s efforts to screen hundreds of thousands of new drugs, as well as existing drugs currently used in the treatment of other diseases, to determine their possible usage in the treatment of JM. NCATS will then perform follow-up studies on the most promising drugs, with a goal of developing a short list of new and re-purposed drugs that have the potential to improve the prognosis for JM patients.

These four newly-funded projects represent only a portion of Cure JM’s research portfolio. You can learn more by visiting the Cure JM website at [www.curejm.org/research/cure\\_jm\\_research\\_summary.php](http://www.curejm.org/research/cure_jm_research_summary.php). For more information, please contact Jim Minow, Executive Director at [james.minow@curejm.org](mailto:james.minow@curejm.org) or (202) 805-2020.

## 2nd Global Conference on Myositis (GCOM)

Cure JM is honored to be one of the sponsors for the 2nd Global Conference on Myositis (GCOM) held May 5-8, 2017 in Potomac, MD. GCOM’s main agenda is full of rich Myositis content, and we want to highlight a few key sessions sponsored by Cure JM Foundation:

- The Juvenile Myositis Pre-Conference Workshop at GCOM is a session of research presentations for juvenile and adult Myositis investigators, trainees and patient support group leaders to learn important new developments in areas of research, pertaining to Juvenile Myositis, not covered in the main program.
- The Breakout Session, “Visions for the Current State and Future Priorities for Research in Juvenile Myositis” will be held Saturday afternoon. JM thought leaders will present their perspectives followed by facilitated small breakout discussions. The goal will be to identify Juvenile Myositis priorities for translational, clinical, and basic science research, as well as methodologic & resource needs.

This conference is critical in:

- Generating the agenda for future Juvenile Myositis research
- Fostering further collaboration
- Advancing the development of young investigators and trainees

Your participation is greatly valued!

## Research Studies Needing Patients

**Volunteers are urgently needed for the AID Clinical Trial (Abatacept In Juvenile Dermatomyositis).**

The Abatacept clinical trial at George Washington University is currently enrolling patients with Juvenile Dermatomyositis (JDM), **including adults** and children who are at least 7 years old. Study participants must have moderately active disease despite treatment with prednisone and at least one other medication. Travel funds and compensation is available for study participants for five study visits after screening. You can enroll your patients or get more information by contacting Sirlekar Bullocks at [sbullocks@mfa.gwu.edu](mailto:sbullocks@mfa.gwu.edu) or by calling (202) 750-0377.

Please visit the research section on Cure JM’s website for additional studies currently enrolling patients.

# Cure JM Funding Opportunities

## Request for Proposals

Cure JM Foundation (Cure JM) announces its Request for Proposals for 2017 for advancing research to improve medical treatment, patient care, and work toward a cure for Juvenile Myositis. Grants are made in collaboration with the Childhood Arthritis and Rheumatology Research Alliance (CARRA).

Cure JM is a 501(c)(3) non-profit organization established in 2003. The mission of Cure JM is to find a cure and better treatments for Juvenile Myositis, and to improve the lives of families affected by JM. Cure JM is the leading global nonprofit organization funding Juvenile Myositis research. Cure JM has invested \$11 million and supported over 130 research studies to accelerate the pace of research.

CARRA's mission is to conduct collaborative research to prevent, treat and cure pediatric rheumatic diseases. Its approach is that CARRA members will work together to capitalize on opportunities, actively develop a structure and a scientific agenda, and collaborate with potential funding sources to create a research alliance that benefits our patients.

In 2017, Cure JM has committed to fund 2-4 new research grants up to \$75K/year for up to 2 years with preference for individuals who have at least 25% protected research time from their institution.

## Grant Application Process

An electronic letter of intent (LOI) submission form is required 30 days before the deadline for full application and must contain the project title, a brief description of the proposed research, the name, address and email address of the Principal Investigator(s), and any persons who might have a conflict of interest in reviewing the proposal. The LOI must be submitted electronically. The LOI is only to allow organization of the review process, and all projects submitting an LOI will be permitted to submit a full application.

Proposals should be submitted electronically as one single PDF format file and must be received by the submission deadline for applications stated in the research announcement timeline. Please go to Cure JM website for more information and to access electronic forms and applications: [www.curejm.org/research/funding.php](http://www.curejm.org/research/funding.php)

## Timeline

Request for Proposals Begins	March 31, 2017
Letter of Intent Deadline	May 15, 2017
Application Deadline	June 15, 2017
Review of Submitted Applications Completed	September, 2017
Anticipated Date for Notification of Awards	October, 2017
Anticipated Funding Start Date	November 1, 2017

*Super sister Abby (left) pictured with brother and courageous JM warrior, Quentin, along with friend and another brave JM fighter, Maura*



# Cure JM Medical Conference in Austin

Friday, February 17, 2017, 40 medical professionals from North America and Europe came together for Cure JM's 7th Medical Conference, co-sponsored by Cure JM Foundation and Dell Children's Medical Center of Central Texas.

The conference was open to anyone in the medical community with a special focus on those in pediatrics, rheumatology, dermatology, and neurology. It was a FREE CME activity offering 6 AMA PRA Category 1 Credit(s)<sup>™</sup>. At the medical conference, Grand Rounds, "An Overview of Juvenile Myositis in 2017" was presented by **Ann Reed, MD**, Chair, Department of Pediatrics, Duke University School of Medicine. Additional presentations are listed below:



Cure JM Foundation 2017 Medical Conference Attendees in Austin, Texas

- *Welcome and Conference Overview*, **Steven Abrams, MD**, Chair of Pediatrics, Dell Medical School; Director, Dell Pediatric Research Institute and Mitali Dave, President, Cure JM Foundation
- *Labs, Imaging, and What they Mean in Juvenile Myositis*, **Adam Huber, MD**, Staff Pediatric Rheumatologist, IWK Health Centre, Halifax, Nova Scotia
- *Skin Signs of Rheumatological Disorders including Juvenile Myositis*, **Moise Levy, MD**, Physician-in-Chief, DCMC; Chief, Pediatric Dermatology, DCMC
- *Non-Biologic Treatment of Juvenile Myositis: 2017 Update*, **Lauren M. Pachman, MD**, Professor of Pediatrics, Northwestern University Feinberg School of Medicine; Director, Cure JM Program of Excellence in JM Care and Research
- *Emerging Applications of Metabolomics in Precision Medicine*, **Stefano Tiziani, PhD**, Assistant Professor, UT Austin
- *Remarks from Dell Children's Medical Center*, **Mark Shen, MD**, President, DCMC
- *Potential New Treatments with Biologics and Drug Therapies for Juvenile Myositis*, **Lisa Rider, MD**, Deputy Chief, Environmental Autoimmunity Group, National Institute of Environmental Health Sciences, National Institutes of Health
- *Transitioning from Pediatrics to an Adult Medical Home*, **Ankur Kamdar, MD**, Assistant Professor of Pediatrics, UT Health Houston
- *Patient - Provider Experience*, **Ruy Carrasco, MD**, Division Chief, Rheumatology, DCMC
- *Case Studies*, **Kaveh Ardalan, MD**, Lurie Children's Hospital of Chicago, **Colleen Correll, MD**, University of Minnesota, and **Megan Curran, MD**, Lurie Children's Hospital of Chicago



*Cure JM Medical Advisory Committee members (seated, left to right) Dr. Ann Reed, Dr. Adam Huber, Dr. Lauren Pachman, and Dr. Lisa Rider answer family questions during the Conference General Session.*

Several talks from the medical conference will soon be posted on Cure JM's website.

Many of these same physicians and researchers stayed on Saturday February 18 and presented to the 120 Cure JM families who had gathered in Austin for Cure JM's National Family Educational Conference.

At the family conference, many families also participated in the blood and sample collection for Cure JM's upcoming genetic identification program with the Center for Applied Genomics at Children's Hospital of Philadelphia. Some 45 families provided almost 400 samples for whole exome genetic sequencing at CAG, and many families were able to provide samples from the patient, parents, grandparents, siblings, and other family members. Additional families will be participating from home in this study over the next several months.



*The Austin, Texas 2017 **Walk Strong to Cure JM™** Family Fun and Fundraising event was held in conjunction with the 2017 National Conference.*

Cure JM is honored to be named a Top-Rated Nonprofit for the seventh year in a row.”

Shari Hume,  
Vice President and  
Co-Founder of  
Cure JM Foundation

## Cure JM Foundation earns 2017 Top-Rated Award

Cure JM Foundation has been honored with a prestigious 2017 Top-Rated Award by GreatNonprofits, the leading provider of user reviews about nonprofit organizations.



GreatNonprofits staff says, “We are especially thrilled to recognize the great work of your organization, given that less than 1% of eligible nonprofits have received this distinction.”

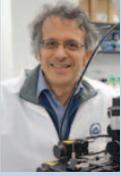
Cure JM made the Top-Rated list thanks to the many positive reviews about our work from donors and the JM community.

According to one recent reviewer, the father of a newly diagnosed teen, “**This foundation provides you answers, compassion, togetherness and friendship so you can get through your day and stay strong for your child.**”

“Cure JM is honored to be named a Top-Rated Nonprofit for the seventh year in a row,” says Shari Hume, Vice President and Co-Founder of Cure JM. “I am humbled and inspired by the unrelenting commitment, the passion, and the grit of our community to find better treatments and a cure. Without a doubt, 2017 will be a year filled with tremendous hope and significant progress in research.”

In the past year, outstanding supporters and volunteers have made it possible for Cure JM to:

- Create a **Cure JM Medical Network** for Healthcare Professionals
- Sponsor its **7th JM Medical Forum**, a one-day conference that brought together physicians, researchers, and medical professionals to learn and share the latest in Juvenile Myositis treatments and research
- Continue **funding its two research and treatment centers** in Chicago and Washington, DC
- Fund promising research at **sixteen additional prestigious research centers** and hospitals
- Begin a **Drug Development Program** at the National Center for Advancing Translational Science (NCATS)
- Start its first five **Regional Chapters** in Chicago, Washington, DC, New York, Texas, and the Pacific Northwest
- Launch a National Walk Program, **Walk Strong to Cure JM™**
- Hold its **11th National Family Conference** in Austin, Texas, bringing together over 120 families to connect with each other and learn about the latest in JM research and treatments from leading physicians and researchers
- Launch an **Early Diagnosis Initiative**
- Create an **E-book of Myositis and You**, a comprehensive guide to Juvenile Myositis, featuring 450 pages with contributions from 80+ experts.



## NCATS Drug Development Program

Jim Inglese, Ph.D.

Principal Investigator, Division of Pre-Clinical Innovation at National Center for Advancing Translational Sciences (NCATS), National Institutes of Health

Cure JM has invested in a 3-year drug development program at NCATS led by Jim Inglese, Ph.D. Cure JM's funding supports the team's efforts to screen hundreds of thousands of new drugs, as well as existing drugs currently used in the treatment of other diseases, to determine their possible usage in the treatment of JM. They will then perform follow-up studies on the most promising drugs, with a goal of developing a short list of new and re-purposed drugs that have the potential to improve the prognosis for JM patients.



## Interferons in Juvenile Dermatomyositis: Pathogenic Role and Correlations with Disease Characteristics at Onset and Long-Term Course

Rebecca Nicolai, M.D.

Division of Rheumatology, Babino Gesu Hospital, Rome, Italy

Dr. Nicolai aims to measure type I and type II interferon gene expression in JDM muscle, potentially leading to the discovery of additional biomarkers to measure disease activity. This is important because the pathogenesis of JDM is not understood, although it is likely that aberrant interferon expression plays a major role. If so, this could open up the possibility for new drug treatments that inhibit interferon expression in JM.



## Novel Genomics Study in Juvenile Dermatomyositis

Paul J. Norman, Ph.D.

Senior Research Scientist, Department of Structural Biology, Stanford School of Medicine

Dr. Norman is investigating the entire HLA genomic region using a new and powerful method he developed that will shed new light on the genetics of JM. Previously uncharacterized genes may interact with HLA genes to become risk factors for disease. Proper understanding of these risk factors opens the door for new diagnostic indicators, treatment targets and, in the long-term, prevention of the disease through directed gene-therapy approaches.



## Heterogeneity of Juvenile Myositis, with a Focus on Therapies and Responses

Takayuki Kishi, M.D., Ph.D.

Environmental Autoimmunity Group, National Institute of Environmental Health Sciences, National Institutes of Health

Dr. Kishi's goal is to investigate clinical response to therapies in patients with JDM from a large JM registry. He will examine the impact of initial medications and other factors on achievement of inactive disease, complete clinical response, and remission. He will also compare different treatment regimens and examine differences in their response rates. These analyses should provide new information on response rates to therapies and predictors of responses to therapies in JDM.

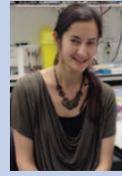


## Physical Activity Monitors as Outcome Measures in Juvenile Myositis

Emily Brunner, D.O.

Pediatric and Adult Rheumatology Fellow, University of Pittsburgh Medical Center, Division of Rheumatology and Immunology

In this pilot study, Dr. Brunner is evaluating the use of Personal Activity Monitors (Fitbits) as a validated measurement tool for physical activity and strength measurement among JM patients. This easy to use tool could be used as a supplement to existing scoring measures and could provide better outcome measurement of a patient's physical function and response to treatment.



## Genetic Risk Factors in Juvenile Dermatomyositis

Claire Deakin, Ph.D.

Post-Doctoral Fellow, University College, London

Dr. Deakin is studying genetic risk factors for JDM using samples from a large number of North American and UK patients with JDM. This project may also be able to determine genetic risk factors for developing specific features, such as the formation of calcium deposits. It will also help us find out whether the age patients are when they get disease influences the role of genetic differences in JDM or its subgroups. The biological effect of these genetic differences will also be studied in order to help us better understand the cause of this disease. This research may lead to more information about the causes of JDM and why certain patients develop certain features.



## The Use of Metabolomics to Develop Novel Biomarkers for Juvenile Dermatomyositis

Jeffrey A. Dvergsten, M.D.

Assistant Professor of Pediatrics, Duke University School of Medicine

Dr. Dvergsten aims to identify aberrant patterns of metabolite expression in blood and muscle of children with JDM to develop new biomarkers for help in diagnosis, assessment of disease activity and response to treatment in JM.



## Plasma Exosomes in Juvenile Dermatomyositis

James Jarvis, M.D.

Professor of Pediatrics, University of Buffalo, NY

Dr. Jarvis aims to understand how blood vessels become injured in JDM. He is culturing exosomes from children with JDM with blood vessel cells to determine if small RNA molecules are capable of causing blood vessel wall damage. By comparing what is seen with JDM exosomes to the exosomes of healthy children, we will have new ways to understand how blood vessels are injured in JDM.



## Validation of Patient Reported Outcomes Measurement Information System (PROMIS) in Juvenile Myositis

Kaveh Ardalan, M.D., M.S.

Attending Physician, Division of Rheumatology, Ann & Robert H. Lurie Children's Hospital of Chicago; Instructor of Pediatrics and Medical Social Sciences, Northwestern University Feinberg School of Medicine

Dr. Ardalan's study aims to validate the use of PROMIS (Patient Reported Outcomes Measurement Information System) to improve Quality of Life data collection. This long-term longitudinal data is needed to assess emotional distress, pain, fatigue, and physical function allowing clinicians to better target interventions —pharmacologic, exercise-related, or psychological. This information would enable JM patients who are struggling with disease management to be more easily identified for intervention.



## Physician Education Program

Megan Curran, M.D.

Attending Physician, Division of Rheumatology, Ann & Robert H. Lurie Children's Hospital of Chicago Assistant Professor, Northwestern University Feinberg School of Medicine; Program Director, Northwestern/McGaw Pediatric Rheumatology Fellowship Program

Dr. Curran aims to better educate pediatricians about JM to increase referral rates to rheumatologists, so that patients can begin treatment without delay. This project includes an online CME module and a brochure mailed to Chicago pediatricians. After program implementation, Dr. Curran will determine if patient referrals increased and if duration of untreated disease decreased.

# Cure JM Family Educational Events

Cure JM is excited to announce expansion of its new series of regional chapters and family educational events. In 2017, Cure JM will expand its regional chapter program to 10 regional chapters.

In each region, local healthcare professionals will be invited to the Family Educational Sessions where a pediatric rheumatologist will present to families on topics of interest to Cure JM families and answer questions from families. In 2017, regional educational events and walks will be organized by Cure JM Chapters in **Seattle, Chicago, Washington D.C., New York City, Dallas, Austin, Houston, Florida** and **Louisiana** with more locations to be announced soon. Contact Shannon Malloy at [shannon.malloy@curejm.org](mailto:shannon.malloy@curejm.org) with any questions.



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*Cure JM Foundation is the leading global nonprofit organization funding Juvenile Myositis research. Cure JM has invested \$11 million and supported over 140 research studies to accelerate the pace of research. Our mission is to find a cure and better treatments for Juvenile Myositis and improve the lives of families affected by JM.*

## Cure JM Medical Network Registration

**To join the Cure JM Medical Network and receive your FREE e-book**, complete the registration form below and fax it to (760) 230-2243, or mail it to Cure JM Foundation, 836 Lynwood Drive, Encinitas, CA, 92024.

You can also register on-line at [www.curejm.org/reg](http://www.curejm.org/reg)

First Name\* \_\_\_\_\_ Last Name\* \_\_\_\_\_ Degree \_\_\_\_\_

Email\* \_\_\_\_\_ Cell Phone \_\_\_\_\_ Business Phone\* \_\_\_\_\_

Medical Specialty or Profession \_\_\_\_\_ Hospital / Medical Office\* \_\_\_\_\_

Office Street Address\* \_\_\_\_\_

City\* \_\_\_\_\_ State/Province\* \_\_\_\_\_ ZIP\* \_\_\_\_\_ Country\* \_\_\_\_\_

Would you like to receive Cure JM Welcome Kits for your patients? If so, how many (up to 50)? \_\_\_\_\_

Would you like to receive Cure JM Awareness Wristbands? If so, how many (up to 50)? \_\_\_\_\_

Yes, I would like to receive more information about Cure JM's FREE Conference for the Medical Community in 2018

Yes, I would like to receive Cure JM's monthly patient newsletter.

The Cure JM Medical Network is an informational network only. Your name will NOT be shared with any other Medical Network member without your consent. Your name will NOT be shared with any other organization or the general membership of Cure JM Foundation, nor will it be listed on Cure JM's website.

\* Required