

## Cure JM Research Priorities Provide 'Roadmap to a Cure'

The Cure JM Board of Directors has recently approved a comprehensive set of strategic research priorities intended to guide future grants and investments in support of Cure JM's mission to find a cure, better treatments, and improve the lives of families living with JM.

The research priorities incorporated survey response data from patients and parents of children with JM as well as clinician and researcher review from the Cure JM Medical Advisory Board.

Priorities fall into four broad areas of research: **Accelerating Scientific Discovery** (basic research to better understand the causes of JM); **Expediting Development of Treatments** (improved therapies delivered to patients); **Providing Access to Quality Care** (highest quality care to patients currently and in the future); and **Fostering Collaboration** (sharing knowledge, data, and resources to speed research advances and improve clinical care).

"The Research Priorities will serve as our 'Roadmap to a Cure' for years to come," says Mitali Dave, Cure JM Board President and co-chair of the committee that developed the strategy. "I'm so appreciative of the vision, dedication and hard work the board and our advisors committed to its development."

While Cure JM is funding projects in many of these areas already, the need to expand to larger-scale pre-clinical studies required to support clinical trials is critical to new therapy development. "The task before us now is to find the funding necessary to bring us to the next level in the research and discovery process," says Dave. "Each potential new therapy will cost millions of dollars to move through the development pipeline. Cure JM will need to be financially prepared to be an effective partner with the academic and industry research communities to advance the most promising projects."

CURE JM MISSION:

**A CURE, BETTER TREATMENTS,  
IMPROVED LIVES OF JM FAMILIES**

STRATEGIC RESEARCH PRIORITIES:

**Accelerate Scientific Discovery**

Genomics  
Environmental Factors  
Predictors of Flare  
Mechanisms of Disease  
JM Disease Models

**Expedite Development of Therapies**

Drug Discovery & Repurposing  
Clinical Trials & Patient Participation  
Comparing Effectiveness of Therapies

**Provide Access to Quality Care**

Centers of Excellence (COE)  
Clinical Education Fellowships  
Standards of Care

**Foster Collaboration**

**Biorepositories, Patient Registries, & Natural History Studies  
Pharmaceutical Partnerships**

## 2018 Update on Juvenile Myositis Care and Research Conference

The annual Cure JM Care and Research Conference will be held June 29th at the George Washington School of Medicine and Health Sciences in Washington DC. The day-long program starts at 7:30 a.m. with check in and breakfast. 5.75 AMA PRA credits can be earned.

The conference is open to physicians and the medical community with a focus on those in pediatrics, rheumatology, dermatology, and neurology. There is no cost to attend the conference.

Topics include:

- The Basics of Juvenile Myositis Diagnosis and Care
- Predictors of Response
- JM Complications
- Myositis Autoantibodies and Their Role
- Pathogenesis & Pathophysiology
- Skin Disease
- Calcinosis Treatment
- Mental Health needs.

For a full program and schedule of presenters, and registration information, visit [www.curejm.org/medicalconference](http://www.curejm.org/medicalconference).

For further information, please contact Mitali Dave or Jim Minow at [mitali.dave@curejm.org](mailto:mitali.dave@curejm.org) or [james.minow@curejm.org](mailto:james.minow@curejm.org).

# Vamorolone Under Study as Potential Treatment for JDM

Representatives of the Cure JM Foundation attended a recent planning meeting to focus on the design of a possible clinical trial for a potential steroid replacement for the treatment of juvenile dermatomyositis. The compound, vamorolone, is currently in a Phase II clinical trial in 48 Duchenne Muscular Dystrophy children, and biomarker studies have suggested improved safety relative to prednisone.

Vamorolone is a dissociative steroid that retains anti-inflammatory activities while reducing or eliminating common side effects. Phase I clinical trial data was recently published at [www.sciencedirect.com/science/article/pii/S0039128X1830045X?via%3DiHub](http://www.sciencedirect.com/science/article/pii/S0039128X1830045X?via%3DiHub).

Cure JM is working with vamorolone's sponsor, ReveraGen, to design a biomarker-focused "clinical de-risking" JDM trial. Cure JM Medical Advisory Board member Dr. Lauren Pachman serves as the co-principal investigator on a pending NIH grant with ReveraGen CEO Dr. Eric Hoffman. Other Medical Advisory Board members attending the planning meeting were Dr. Lisa Rider, Dr. Brian Feldman and Dr. Kanneboyina Nagaraju, a ReveraGen co-founder.

## Consensus Treatment Plan for Skin Predominant JDM Developed through CARRA

Treating skin disease as a component of JDM is important as it reflects disease activity and may contribute to morbidity, including physical disfigurement, calcinosis and lipodystrophy. Skin disease is a manifestation of active vasculopathy in JDM that is important to monitor and treat. Persistently active skin disease at three and six months after diagnosis is predictive of continued active disease.

Dr. Susan Kim, associate professor of pediatrics UC San Francisco and member of the Cure JM Medical Advisory Board led a group of pediatric rheumatologists in completing a multi-year study to address the subset of children who have the rash of JDM without significant muscle weakness — skin predominant JDM. Optimal treatments for these children are unknown.

The work of Kim's group led to the development of Consensus Treatment Plans, or CTPs, to provide guidance and standardize approaches to treating skin predominant JDM. Three treatment options were proposed:

- Hydroxychloroquine alone
- Hydroxychloroquine and methotrexate, and
- Hydroxychloroquine, methotrexate and corticosteroids.

The study was published last year in *Pediatric Rheumatology Online* and conducted through the Children's Arthritis and Rheumatology Research Alliance (CARRA) consortium of pediatric rheumatology health care providers. Dr. Kim is Vice-Chair of CARRA's JDM Research Committee.

It is hoped that these CTPs can be applied prospectively, through the CARRA Registry, to determine the best treatment approaches and improve outcomes for skin predominant JDM. Dr. Kim points out that these CTPs represent typical, reasonable treatment approaches used by pediatric rheumatologists. They are not standards of care, and do not replace clinical judgment or decision making between the treating physician and patient. Of note, not all available treatment options were included in the study, including, high dose oral or intravenous corticosteroids and intravenous gamma globulin.

Co-authors include Philip Kahn, Angela Robinson, Bianca Lang, Andrew Shulman, Edward Oberle, Kenneth Schikler, Megan Curran, Lilliana Barillas-Arias, Charles Spencer, Lisa Rider and Adam M. Huber. The full article can be found at <https://link.springer.com/content/pdf/10.1186/s12969-016-0134-0.pdf>



Dr. Susan Kim

# CURE JM SEEKS CHIEF SCIENTIFIC OFFICER

The Cure JM Foundation is seeking a Chief Scientific Officer to provide strategic oversight, guidance, and leadership to the Cure JM research program. The CSO is responsible for the timely and effective implementation of the Cure JM Research Priorities as adopted by the Board of Directors, principally through a grants program supporting basic, translational, and clinical research and Cure JM-funded Centers of Excellence.

The CSO serves as Cure JM's science, research, and medical leader, proactively driving the growth and effectiveness of a grants and clinical research program with ambitious timelines to develop improved treatments for children and adults living with Juvenile Myositis. The CSO position is telecommuting and is full or part-time.

For the complete position description and information on how to apply or nominate a candidate, please visit [www.curejm.org/cso](http://www.curejm.org/cso) or contact Jim Minow, Cure JM Executive Director, at [james.minow@curejm.org](mailto:james.minow@curejm.org).

## Cure JM Chapters Invite Your Participation

Cure JM now has 15 established chapters around the U.S. that have been organized by parents of children with Juvenile Myositis. Chapters hold medical education events each year for families and patients.



Pediatric Rheumatologists or other medical professionals interested in attending or participating in chapter educational events are welcome. If interested, please contact Shannon Malloy at [shannon.malloy@curejm.org](mailto:shannon.malloy@curejm.org) for information on upcoming dates in your areas. Cure JM chapters have been organized in the following locations:

- Massachusetts and New England (MA, RI, NH, VT, ME)
- Greater-New York (NY, NJ, CT)
- Philadelphia (including Southern New Jersey)
- Washington, D.C., (DC, MD, VA)
- Greater Chicago Area (IL, IN, IA, WI, MI)
- Minnesota (MN, IA, ND, SD, WI)
- Ohio/Western Philadelphia (OH, PA, WV)
- Central Texas (Austin, San Antonio)
- Dallas-Fort Worth (including North Texas and OK)
- Houston (including Southeast Texas)
- Florida
- Louisiana (LA, MS, AL)
- Northern California
- Southern California
- Pacific Northwest (WA, OR, UT, MT, ID)

## Cyclophosphamide - an Effective Second-Line Treatment

A recent study of patients treated with Cyclophosphamide showed a reduction in skin disease, muscle disease and global disease 6, 12 and 24 months after starting the drug. The study, published earlier this year in *Arthritis and Rheumatology*, compared long-term skin disease, muscle disease and global disease activities in 56 patients treated with the drug compared to 144 patients not treated with the drug. Treated patients reported no side effects.

Patients who were treated with Cyclophosphamide showed significant improvement in skin disease in particular, according to the study's co-author Dr. Claire Deakin.

In patients with severe Juvenile Dermatomyositis, second-line treatments may be required. Cyclophosphamide is used to treat some connective tissue diseases, but evidence of its efficacy in JDM is limited.

In contrast to biologic therapies that are also used as second-line treatments in JDM, Cyclophosphamide is an inexpensive drug requiring a short treatment course of 6–7 months. As long as infections are monitored, the sustained clinical benefit demonstrated by this study suggests that more frequent use of Cyclophosphamide may be warranted.

The entire study can be found at [onlinelibrary.wiley.com/doi/10.1002/art.40418/pdf](http://onlinelibrary.wiley.com/doi/10.1002/art.40418/pdf). Principal Investigators within the research study group were Dr. Claire Deakin and Dr. Raquel Campanilo-Marques, both of University College London Great Ormond Street Institute of Child Health. Other authors included Dr. Lucy Wedderburn and Dr. Clarissa Pilkington.

# 2018 Cure JM Request for Proposals and Grant Deadlines

Cure JM is now accepting Requests for Proposals for advancing research to improve medical treatments, patient care, and advancements toward a cure for Juvenile Myositis. New research awards are a maximum of \$75,000 per year for up to two years, with preference for individuals who have at least 25% protected research time from their institution.

Applicants should address how their project supports Cure JM's Strategic Research Priorities (see related article).

A one-page letter of intent is required 30 days prior to the deadline for the full application and must contain the project title, a brief description of the proposed research, the name, address and email address of the Principal Investigators, and any persons who might have a conflict of interest in reviewing the proposal. The LOI should be submitted electronically to: [research@curejm.org](mailto:research@curejm.org). All applicants submitting an LOI will be invited to submit a full application.

Proposals should be submitted electronically as one single PDF file to: [research@curejm.org](mailto:research@curejm.org) and must be received by the submission deadline.

The full grant application can be found at [www.curejm.org/grants](http://www.curejm.org/grants).

<b>Letter of Intent Deadline</b>	<b>May 30, 2018</b>
<b>Application Deadline</b>	<b>July 1, 2018</b>
<b>Review Completion of Applications</b>	<b>September 1, 2018</b>
<b>Anticipated Date for Notification of Awards</b>	<b>September 30, 2018</b>
<b>Anticipated Funding Start Date</b>	<b>November 1, 2018</b>

## Interested in the Cure JM Medical Network?

The Cure JM Medical 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 the 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 will publish an eNewsletter quarterly to keep you informed of research advances, new treatment options, drug trials, funding opportunities, upcoming conferences, and other items of interest to the clinical community.

Thank you for your contributions and dedication to the families and children who are fighting JM! Please contact Beth Barrer 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 to give to your patient families.

