

Medical Network

NEWSLETTER Fall 2017

Cure JM Announces Fellowship and Early Career Grants in Juvenile Myositis

Cure JM Foundation has created a new Fellowship Award and is in the process of creating an Early Career Award. The purpose of each grant is to encourage young



clinicians and investigators to pursue or sustain research careers in juvenile myositis.

The Fellowship Award, which is \$60,000 a year for two years, is available to M.D.s who have completed a residency program and who are currently in at least their first year of a pediatric rheumatology program at a center that provides physician training in juvenile myositis. The two-year award is for two subsequent years of fellowship.

The deadline for applications is December 15, 2017 for fellowships starting in the summer of 2018. For the complete announcement, visit www.curejm.org/fellowship. Fellowship grants are made in collaboration with the Childhood Arthritis and Rheumatology Research Alliance (CARRA).

The Cure JM Board has also approved an Early Career Grant of \$225,000 over three years for clinicians/researchers who have completed pediatric rheumatology fellowships and who are seeking support for protected time to pursue research projects in JM.

M.D. or M.D./Ph.D. applicants should have completed their fellowship in pediatric rheumatology. Non-clinician Ph.D.s are also eligible. Although details are yet to be finalized, tentatively eligible candidates will be in their first ten years of post-fellowship practice at a pediatric research hospital or institution, or in the first ten years of a tenure-track appointment at a research institution.

Final guidelines will be published in January. Interested candidates should contact Jim Minow, Executive Director, Cure JM Foundation, at james.minow@curejm.org.

ReveraGen Partnership to Pursue Vamorolone as Potential JM Therapeutic

Cure JM has engaged in a new strategic partnership with ReveraGen, a clinical-stage drug development company with a focus on developing new antiinflammatory therapeutics.

"Investing in a strategic new partnership with ReveraGen is advancing one of Cure JM's key research priorities -- drug development," says Cure JM Executive Director, Jim Minow. "ReveraGen's corticosteroid replacement compound, vamorolone, may be effective in managing Juvenile Myositis—this is what we hope to discover." In pre-clinical and clinical trials for other chronic inflammatory conditions, vamorolone has been well tolerated without the side effects of corticosteroids.

Corticosteroids work well in controlling inflammation, but at a tremendous cost: stunted growth, fragile bones, cataracts, and weakened muscles are some of the side effects.

Vamorolone works by replicating the good, antiinflammatory effects of corticosteroids without the side effects by modifying the chemistry of the drug. Specifically, the aspect of corticosteroids where many genes are turned on is removed in vamorolone. This gene activity is responsible for the majority of side effects in corticosteroids.

Vamorolone is in phase II clinical trials in Duchenne Muscular Dystrophy, and is on the "fast-track" approval process within the FDA. The goal of Cure JM's engagement with ReveraGen is to conduct the preliminary studies necessary to add JM as a targeted indication for vamorolone in future clinical trials.

CARRA Study Group Identifies Four Most Used Biologics in JDM Treatment

A study published by Pediatric Rheumatology Online and conducted by the CARRA Juvenile Dermatomyositis Biologics Workgroup concluded that there are currently four primary biologic drugs in use by pediatric rheumatologists for refractory JDM. The biologic therapies are rituximab, abatacept, tocilizumab, and anti-TNFa drugs.

The report provides clinical experience data through three surveys of over 100 pediatric rheumatologists and through three CARRA consensus meetings. Approximately two-thirds of the respondents indicated that they have prescribed the use of biologics in treating JDM, primarily rituximab. Approximately 10% of clinicians using biologics prescribe them as a monotherapy. Most often, they are used in combination with methotrexate alone, methotrexate and corticosteroids, or methotrexate, corticosteroids, and IVIG.

The results of a second survey supported these findings in considerably more detail with multiple combinations of drugs used with biologics and supported the use of rituximab, abatacept, anti-TNFa drugs, and tocilizumab in that order.

"What we learned over the five years of this study is that while pediatric rheumatologists are increasingly using biologics in treating JDM, such use remains a trial and error process," says Charles Spencer, M.D. of Nationwide Children's Hospital, who leads the CARRA JDM Biologics Workgroup. "We all recognize the need for developing standard protocols, and virtually every survey respondent and study collaborator felt ongoing studies addressing these four biologics in JDM are needed."

The full study can be found at https://www.ncbi.nlm.nih.gov/pubmed/28610606

Cure JM Medical Conference to be held June 29th, 2018 in Washington, D.C.

The 2018 Cure JM Medical Conference will take place on Friday, June 29, 2018 at George Washington University Hospital. The Medical Conference was created by Cure JM to provide clinical education and research updates to pediatric and adult rheumatologists, pediatricians, and medical residents who wish to learn more about diagnosis and care of juvenile myositis and related autoimmune diseases. Objectives of the Medical Conference are to:



- Identify best practices in diagnosing and treating Juvenile Myositis (JM) patients.
- Apply latest developments in myositis research to clinical care.
- Examine specialized management issues relevant to JM patients.
- Discuss JM case studies to make treatment plans to optimize care.

The Medical Conference co-chairs are Lisa Rider, M.D., Deputy Chief of the Environmental Autoimmunity Group at the National Institute of Environment Health Sciences at NIH, and Rodolfo Curiel, M.D., Director of the Myositis Center at George Washington University Hospital.

For a complete schedule of presenters and registration information, please visit www.curejm.org/medicalconference.

Questions? Contact mitali.dave@curejm.org or james.minow@curejm.org

JOIN THE CURE JM MEDICAL NETWORK

This 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 will publish an eNews quarterly to keep you informed of drug trials, funding opportunities, upcoming conferences, and much more.

Thank you for your contributions and dedication to the families and children who are fighting JM! Please contact Beth Barrer at physicians@ curejm.org if you have any questions or would like peer-support information (welcome kits and brochures) from Cure JM to give to your patient families.

Cure JM Funds New Center of Excellence at Seattle Children's Hospital

The Cure JM Board of Directors has approved a \$600,000 three-year grant to establish a JM Center of Excellence at Seattle Children's



Research Institute

Hospital (SCH) in Seattle, Washington. Funding will be used to recruit a world-class JM specialist to lead the new Center and to provide additional training in JM care to the pediatric rheumatology staff at SCH. The Center of Excellence will also systematically engage other pediatric specialties such as cardiology, dermatology, and pulmonology in the care of JM patients.

Funding will also be used to create a new research position in juvenile myositis.

"This new Center of Excellence will be able to advance clinical care in the Northwest as well as stimulate a strong research environment in JM," says Suzanne Edison, Cure JM Board member and co-chair of its Research Committee.

"The grant will open up exciting JM research and innovation potential among the robust academic and biotech community in Seattle, and it will help Seattle Children's Hospital better serve JM patients in a large fivestate area, furthering Cure JM's commitment to families."

The Seattle Center of Excellence will be the third such center to be funded by Cure JM, joining centers at Ann & Robert H. Lurie Children's Hospital of Chicago and George Washington University in Washington, D.C.

Cure JM Announces New Grants

Cure JM has recently funded three new grants in the U.S. and abroad. New grants have been awarded to:

- Zoe Betteridge at the University of Bath in the United Kingdom to validate commercial testing labs to better enable these labs to conduct myositis-specific autoantibody testing. Currently, there are few labs in the U.S. or Europe whose work in autoantibody testing meets a "gold standard" for accuracy and breadth (testing for all known myositis auto antibodies). Dr. Betteridge's project will test and validate commercially available kits for myositis antibodies, potentially increasing the number of lab sites and lowering costs.
 Award amount: \$65,000
- Laura Tasan at the University of Pittsburgh to study the feasibility of Ultrasound as a diagnostic tool for muscle disease in children. If successful, Ultrasound could supplement or replace the use of costlier MRIs to monitor disease activity in JM.
 Award Amount: \$40,000
- Josh Woolstenhulme at George Washington University to better understand the etiology of fatigue and fatigability in JDM using cardiorespiratory measures. The project will measure oxidative stress and mitochondrial function as possible causes of fatigue. This is an important study for patients and families of those with JDM, as it is a common question as to why a patient with JDM continues to feel significant fatigue despite disease control. Award Amount: \$130,000

A fourth grant was also approved, pending further discussions with the Principal Investigator.

Cure JM grants are made in concert with the Childhood Arthritis and Rheumatology Research Alliance (CARRA). CARRA provides independent grant reviewers from its membership. Our thanks to CARRA for assuming this important role.

Deadlines for 2018 Cure JM Grant Cycle Announced

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Letters of Intent and application deadlines for the 2018 Cure JM grant cycle have been announced, with Letters of Intent due May 15 and full applications June 15. The LOI is only to allow for the organization of the review process, and all projects submitting an LOI will be permitted to submit a full application.

Grants can be submitted any time after March 30, when the RFP process opens. Watch for more information to be distributed through the Medical Network, IMACS, CARRA and on the Cure JM web site.

Request for Proposals Begins	March 30, 2018
Letter of Intent Deadline	May 15, 2018
Application Deadline	June 15, 2018
Review of Submitted Applications Completed	September, 2018
Anticipated Date for Notification of Awards	October, 2018
Anticipated Funding Start Date	November 1, 2018

Cure JM Foundation is a 501(c)(3) non-profit organization established in 2003. Cure JM's mission 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, having invested \$12 million and supported over 140 research studies to accelerate the pace of research.



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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

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 newslet	ter.		