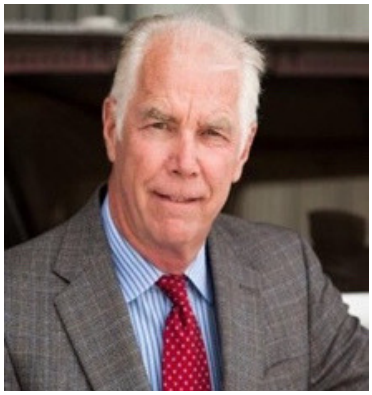
CURE JM STRONG

2018 IMPACT REPORT

CureJM® FOUNDATION

CONTENTS

CURE JM FRIENDS AND FAMILY,



Jim Minow

It's hard to imagine a medical research organization that is more effective in its work than Cure JM. On this, our 15th Anniversary, we can marvel at the progress that has been made in helping children around the world overcome the challenges of juvenile myositis. We'll be sharing a few examples of this progress in the pages that follow—progress that can best be summarized by noting that better treatment regimens, improved standards of care, and Cure JM partnership with leading pediatric research hospitals around the world have all been made possible because of the leadership and financial support our community has provided.

Leadership matters. I've had the privilege of working with a truly extraordinary Board of Directors, Medical Advisory Board, and local Cure JM Chapter and Walk Strong leaders who collectively are the driving force of advancing our mission to find better treatments and a cure for JM. That leadership has resulted in Cure JM launching two new Centers of Excellence at Duke Children's Hospital and Seattle Children's Hospital this year, as well as funding several new initiatives internationally at Hospital for Sick Children in Toronto and Utrecht Medical Center in Belgium, where we are funding a study on how parents can better predict the onset of flares.

Indeed, Cure JM currently has over $5 million in commitments to fund research and improvements in clinical care. In recent years, we've increased our focus on drug development and repurposing existing drugs such as rituximab—effective in managing rheumatoid arthritis—for use in JM. Under the leadership of Medical Advisory Board chair Lisa Rider, M.D., and Lauren Pachman, M.D., two early pioneers in JM clinical care and research, the MAB, along with several other leading clinicians, are creating a plan to test a new drug—vamorolone—for efficacy in JM. Vamorolone is a steroid replacement (one with far fewer side effects than prednisone) that may soon be approved by the FDA for another indication, but which could be available to JM patients if supportive clinical data is available.

I am also pleased to announce the appointment of Andrew Heaton, Ph.D., as Cure JM's first Chief Scientific Officer. A chemist by training, Dr. Heaton brings a strong record of drug discovery to Cure JM, having created three oncology compounds now in phases of clinical trial development. In just the first few months of his appointment, Dr. Heaton is leading us toward newer drug repurposing initiatives that I am confident will result in better treatments for our children at the earliest possible moment.

I would also like to thank Rhonda McKeever for her many years of exceptional leadership as Cure JM's board chair. After more than a decade on the board, Rhonda has passed the leadership torch to Mitali Dave, who is stepping into the chairmanship in 2019. Cure JM would not be the organization it is without have the benefit of their extraordinary dedication.

Finally, my thanks to you—the army or parents, grandparents, friends, and family who make our work possible. All of us on the Cure JM staff and board pledge to do our utmost to turn your dreams for your children into the reality of a world without JM. Together, we can achieve this grand objective.

With appreciation for all you do,

IT SEEMS LIKE IT WAS YESTERDAY

It seems like it was just yesterday that we started Cure JM…but it was 15 years ago!

In the same blink of an eye, as parents, we often reflect on how quickly our kids grow up. These reflections usually include joy, pride, an appreciation from learning the hard way, and a mix of emotional highs and lows.

Like many other families we've known from the early days, this 15 year journey growing Cure JM Foundation has overlapped with our son's courageous battle with Juvenile Myositis. As parents of a child with JM, we've all lived through the good days – seeing our kids more active, tapering off medications, and being eternally grateful for the genuine support of family and friends.

The Hume family.

We've also lived through the bad days – hearing our child's labs are going the wrong direction, battling flares, and trying to encourage a child who asks you, "why me?"

For us, the most positive constant throughout this journey has been the shared experiences with other JM families. From the beginning, Cure JM has been about community. Our JM community shares knowledge, gives comfort, connects our kids to each other, and in general, makes the unbearable bearable.

It is this JM community that helps us power through the bad days and rejoice in the good days. Through Cure JM Foundation, we have found strength and hope in each other. It's that 'never quit' attitude still driving Cure JM Foundation today.

As Cure JM marks its 15th anniversary, we thank all the families, friends and supporters who have made Cure JM Foundation what it is today...a teenager, with a bright and promising future.

- SHARI AND TOM HUME, CO-FOUNDERS OF CURE JM

15 YEARS OF FOCUS AND HOPE

We're celebrating fifteen years of extraordinary progress fueled by the focus and determination of engaged volunteers, leaders, families, patients, physicians and researchers.
Determination is at our core.

In October of 2003, Shari and Tom Hume and Lisa Felix, parents of children with JM, and Harriet Bollar, grandmother of a child with JM, started this foundation. Brought together by circumstance, they founded Cure JM with the same goal that fuels our mission today: to find a cure and better treatments for juvenile myositis and improve the lives of families affected by JM.

The first fundraiser was a modest lemonade stand in Encinitas, California. Soon after, the Foundation established a website to provide information and emotional support for families impacted by the disease.

Since that humble lemonade stand, we've not only grown, but we've made a collective, substantial impact. You'll read about that progress in the pages ahead.

The first Cure JM fundraiser.

A PROMISE KEPT

In 1971, Dr. Lauren Pachman made a promise to a grieving mother.

Lauren Pachman, M.D.

Dr. Pachman was the Head of the brandnew Division of Immunology at Children's Memorial Hospital in Chicago, IL, when the Chief of Staff asked her to see a child in Intensive Care -- a 12-year-old boy suffering from a profound rash, weakness and depression. Dr. Pachman recalled, "I'd never seen that rash before."
Despite the hospital's valiant effo-rts, the young man died. His mother was inconsolable, and Dr. Pachman promised the mourning mother that she would find out why the boy lost his life.
"It's burned into my memory," Dr. Pachman said, "I've kept that promise."

That pledge was the beginning of Dr. Pachman's 48+-year dedication to juvenile myositis (JM), its causes, its treatments, and most importantly, its patients.

Fast forward to the early 2000s, when Dr. Pachman heard from a grandmother in California whose granddaughter had juvenile dermatomyositis (JDM). The grandmother said to Dr. Pachman, "There's not enough information that's available to us about this problem!" Dr. Pachman traveled to California to meet with the grandmother, Harriet Bollar, as well as a couple, Tom and Shari Hume, at one of the first national meeting of the parents of children who had this rare disease. "I will never forget that meeting," Dr. Pachman remembered. "They decided on the name, "Cure JM," because that's what everyone wanted to do." Two skilled rheumatologists from NIH, Dr. Lisa Rider and Dr. Fredric W. Miller III, volunteered to join Harriet and Dr. Pachman in gathering information about JM to help both patients and caregivers.

Another fast forward—four, long years later, this hard-working group finished *Myositis and You: A Guide to Juvenile Dermatomyositis for Patients, Families, and Healthcare Providers*. Published in 2007, the book summarized what was known about JM. It's an essential resource for countless families and physicians around the world. "It took four years of discretionary time to write the book. We hoped to relieve some of the families' anxiety and to help them when they encountered this rare disease."

A PROMISE KEPT, CONT.

In her decades of service, Dr. Pachman witnessed great shifts in diagnosis and treatment. “In the 1970s, we didn’t have any indicators of the severity of disease activity. For example, we didn’t know how to document which muscles were involved, or even where to do a muscle biopsy, for MRI was not yet used for that purpose,” Dr. Pachman recounted. “We didn’t have standards for judging how weak a child is, and it wasn’t until about 2000 that we and others documented that IV prednisone in high doses could effectively dampen the inflammatory response.” There weren't even Pediatric Boards to license Pediatric Rheumatologists until 1992, which Dr. Pachman helped to create.

Dr. Pachman personally contributed a great deal of information that physicians use to diagnose JM. In addition to authoring more than 200 peer-reviewed, scientific publications and chapters, she now directs the JM Translational Research Team at the Cure JM Program of Excellence in JM Research and Care at The Stanley Manne Children’s Research Center, a part of the Ann & Robert H. Lurie Children’s Hospital of Chicago. Her team developed and standardized new approaches to monitor symptoms and signs of disease activity. Today, those standards are used to diagnose JM, both in the U.S., and internationally.

The Pachman Laboratory maintains an extensive repository of bio-samples from more than 600 children and teens with JM. The idea for the registry came as Dr. Pachman saw most of those children as patients. She was struck by the vast differences between them. “Some children were very sick, some didn’t appear sick at all, but went on to become very ill, and some children got better faster than others.” The samples in the repository are connected to a very large database of JM symptoms and treatments. So, the combination of samples and database allow researchers and physicians to improve diagnosis, treatments, and outcomes.

And what about the future? Dr. Pachman sees great promise in biomarkers, which can indicate how well someone will respond to a particular treatment.
“With biomarkers, we may learn when we should switch from a non-effective, time-wasting therapy, to one that actually works.”
Dr. Pachman hopes that research will lead to an understanding of the underlying pathways of inflammation, and how they differ amongst the children, as well as uncover potential agents that may contribute to the inflammatory process “Then, hopefully, we can control the disease.”

Dr. Pachman saw her last patient in 2016. So now, she spends her time working with her Translational Research Team and investigators around the world to identify new genetic and protein biomarkers for JM, using “the informational JM sandbox we created.” In addition to mentoring a PhD candidate, four fellows and junior faculty around the country, she is funded by the NIH to contribute to a clinical trial of a new drug that may cause less side effects than traditional treatments. These efforts are reflected in the ten abstracts bearing her name at the 2018 American College of Rheumatology meeting, and the six manuscripts waiting to be completed. She says, "There is much more work that needs to be done!"
We salute Dr. Pachman for incredible contributions.

"LOOKING BACK, IT WAS ALL MAGIC"

Rhonda and her family.

Back in 2006, Rhonda McKeever was several years into her young daughter's JM journey. The disease's massive impact left her feeling, “impatient.” Rhonda recalls, “Since I could not do anything about what the disease was doing to her, I wanted to somehow make a difference.”
That year she joined Cure JM, and eventually had a conversation with co-founder Shari Hume about running in a marathon to raise money for the foundation.

“Over the next nine months or so, the ideas continued to snowball...in the end it turned into a large, Cure JM team running in the Chicago marathon. Ultimately, we raised over $250,000.” Rhonda continues, “Seeing the powerful impact of the JM families coming together, and the success of that one, small idea, energized me to join the Cure JM Board of Directors, and to lend my leadership talents to drive results for something so near and dear to our family.”

That was one of many firsts for Rhonda and Cure JM. She helped launch the first Cure JM Medical Conference, which has educated hundreds of healthcare providers on the best practices of diagnosing and treating JM, and how to apply research to clinical care.

After many significant contributions, Rhonda was appointed to Chairman of the Board for Cure JM in 2008. Over the next ten years, she contributed her strategic leadership to the organization, and oversaw all operations.

Looking into the future, Rhonda hopes that Cure JM families, friends, and researchers will continue their active role in progress. “We are not bystanders. We make things happen. Anything is possible, including a cure. Let's make it a reality.”

After a decade of innovation and entrepreneurial leadership as Chairman, Rhonda now serves as Chair Emeritus.

She is succeeded by Mitali Dave, who recently reflected on Rhonda’s tangible contributions to Cure JM. “She initiated the pre-cursor to Cure JM's current Walk Strong program, which since its inception has raised $3 million for research. She also led our passionate Cure JM community into contests like Pepsi Refresh, Chase Giving, and CrowdRise's Holiday Challenge, with her motto "We're in it, to win it!" In total, we have raised and won $3.5 million through these contests alone.”

Mitali sums up our collective feelings, “Her energy and optimism will be sorely missed!”

GET TO KNOW OUR CHIEF SCIENTIFIC OFFICER

Cure JM's first Chief Scientific Officer, Andrew Heaton, Ph.D.

Andrew Heaton, Ph.D.

What's your background?

I've had a 20-year career in pharmaceutical discovery, development, and translational studies, and most recently served as the CEO and President of Heaton-Brown Life Sciences and Novogen North America. During my time there, I ushered several oncology compounds from discovery in the laboratory through to Phase I, II and III clinical trials. I am also the senior author on over 100, globally granted-patents, covering a wide range of molecular architectures and diseases and have published multiple papers in peer-reviewed scientific journals.

As for education, I received my Bachelor of Science degree from the University of Tasmania, a Doctorate from James Cook University of North Queensland, an executive MBA from Melbourne Business School, and completed a sabbatical at Imperial College, London.

What drew you to this position?

I have a passion for rare diseases, they are often overlooked by larger pharma companies. I have found the people involved in and touched by rare diseases, from families through to clinicians and research scientists, all seem to share this passion. The Cure JM Foundation is run by some amazing people with the foundation connected and embedded in a global network of clinicians and research groups. These global connections are sharing ideas and bringing forwards fundamental understanding of the disease, which is going to lead directly to better treatments, and one day, a cure.

What is your role as CSO?

I will serve as Cure JM's science and research leader, proactively driving the growth and effectiveness of Cure JM's grants and clinical research programs. I will be playing a pivotal role in unearthing and sourcing cutting-edge genomic and genetic understanding of the disease and dovetailing this knowledge with drug discovery and drug translation/repurposing programs. In addition, I will provide strategic oversight, guidance, and leadership of the Cure JM Research Priorities and serve as the pivotal staff member to Cure JM's Research Committee, Scientific Review Committee and Medical Advisory Board.

What is your vision for Cure JM?

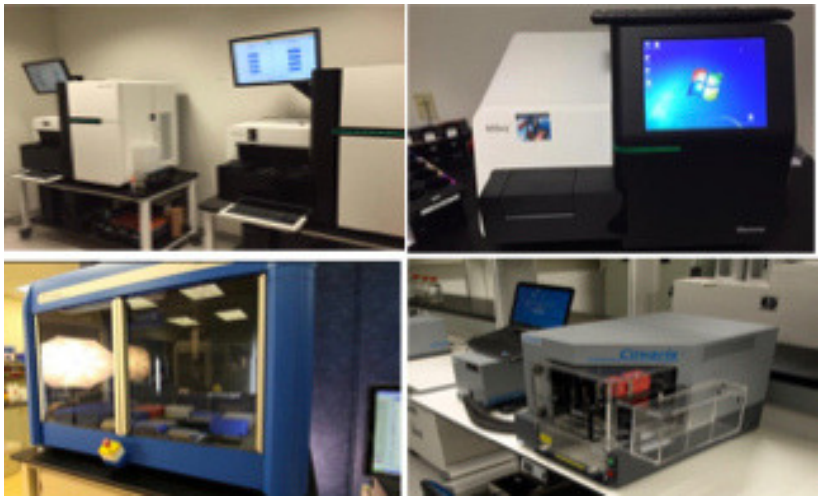
Like everyone involved with the Cure JM Foundation, my ultimate long term goal is to see better treatments and hopefully a cure. While this appears to a be a lofty aspirational goal, I am certain that digging deeper into the fundamental genetic and genomic signals that predispose individuals to JM will underpin all our efforts in making better treatment decisions. Understanding the genomic and antibody specific signals of the disease will also aid understanding the chronicity of the disease. Understanding disease chronicity will also support in more precise treatment decisions.

JM GENETIC RESEARCH UPDATE

We know a lot about the symptoms, diagnosis and treatment of juvenile myositis (JM), yet, we don't have a complete understanding of the underlying causes. Genetics is known to play a role in autoimmune diseases, including JM, and being able to dissect the specific genetic factors that associate with the disease brings us closer to understanding those root causes. Understanding root causes will help us develop new tests, new therapies, and ways to personalize those therapies.

We recognize the promise of genetic research and we've made it a strategic research priority. In 2017, we partnered with the Center for Applied Genomics (CAG) at Children's Hospital of Philadelphia (CHoP) on a study to understand the causes of JM. CAG maintains a pediatric biobank, which contains biospecimen samples, like blood and tissue, paired with medical information. To date, over 100,000 families have voluntarily donated samples into the biobank and shared medical and survey information. This massive store of information makes CAG's pediatric biobank the world's largest of its kind. Researchers use the samples to conduct studies designed to understand the underlying causes of different diseases, both rare and common, that affect children. The end goal of the program is to translate research into new diagnostic tests and new treatments for those diseases.

The Cure JM-invested research is led by Hakon Hakonarson, Ph.D., M.D. and Charlly Kao, Ph.D. The team is using CAG's biobank, sequencing and genotying technologies, and analytical tools to discover which genes associate with JM, and develop an understanding of why mutations in those genes can contribute to the development of JM. The researchers hope the results will lead to new drugs and new treatments that will improve the lives of those living with JM.



Equipment used in the lab to conduct genetic research.

JM GENETIC RESEARCH UPDATE, CONT.

In order to conduct a thorough study, the team is collecting samples from both patients and their families. More samples mean more information, and more information improves the chances of understanding what causes JM. As of January, 2019, about a hundred of Cure JM families have contributed to the study. During the last two National Family Conferences and several Walk Strong® events around the country, families filled out medical and family history paperwork and then gave blood or saliva. Samples were collected from JM patients, their siblings, parents, grandparents, and extended family.

One cannot underestimate the value of these samples. The family pedigree provides important information to analyze and isolate genetic markers passed on through families that may contribute to their autoimmunity; and for families without any apparent or known history of autoimmune disorders, the analysts can look for “de novo” (or new) mutations or variants unique to the affected patients

“When conducting rare disease research, being able to collect samples from so many families is a unique opportunity,” says Dr. Kao. “We were pleased that after just two visits to Cure JM conferences that we already collected samples from 300+ different individuals representing over 70 families. This speaks to the commitment, support, and enthusiasm of the Cure JM organization and their families."

Dr. Kao says that they’ve made progress in the Cure-JM funded research, and plan to report early results in the coming months.

“We currently have encouraging preliminary results, and we are working on sequencing new families to replicate and search for other contributing genetic factors. Families where multiple members have a history of autoimmune disease or where more than one sibling or family member has/had JDM/JM are of particular interest and priority, since the genetic signals/contributions tend to be strongest in these settings. We anticipate at last some of these results will be available this summer to be presented at the Annual Medical Conference and Family Education Event in Chicago, and we project to submit for publication later this year.”

If you want to contribute to this study, you have several options.

• The CAG team travels to many Cure JM events around the country to obtain samples from consenting families. If you’re interested, please contact Cure JM (info@curejm.org), or CAG (https://caglab.org/contact-us.html), to find out if CAG will attend a specific event.

• You can also reach out directly to CAG at CHOP: https://caglab.org/contact-us.html, and the CAG team can arrange the recruitment over the phone, or by mail.

• If you have already contributed to the study, we need you to update some information. Contact us at info@curejm.org for next steps.

GETTING US CLOSER TO PRECISION TREATMENT

"My name is Dr. Cory Stingl, and I am a fellow in pediatric rheumatology and genomics at Duke University in Durham, North Carolina. One of my career goals is to use genomic data to help doctors treating juvenile dermatomyositis (JDM) better predict treatment response, so that children with JDM face fewer limitations from their disease.

Cory Stingl, M.D.

At least 25% of children with JDM will not sufficiently improve with first-line treatment for this disease, which includes steroids, methotrexate, and sometimes intravenous immunoglobulin (IVIG). When children do not respond to these first-line therapies, one potential treatment option is a medication called rituximab. Currently, we do not have a way to predict if a child with JDM will respond to rituximab.

The goal of our study with the Cure JM Foundation is to predict if a child with long-standing juvenile dermatomyositis (JDM) will respond to rituximab before rituximab is started. This knowledge will help doctors choose which patients with JDM should be treated with rituximab, and avoid using it in children who are unlikely to respond (or rituximab “non-responders”). By identifying these “non-responders” early, treatments other than rituximab can be chosen for these patients, thereby reducing the time a child spends with active disease and avoiding long-term skin and muscle damage.

We are using ribonucleic acid (RNA) as an indicator in the blood to determine how a child with JDM will respond to rituximab. RNA tells us what processes are active in cells, some of which are due to JDM. For example, we can see that parts of the immune system are inappropriately active. We think some of the active processes in JDM that we can see using RNA will tell us if a child is going to respond to rituximab or not.

Our study is well under way. RNA samples have been collected and we are working towards analyzing it for indicators of response or non-response to rituximab. We hope our results will help inform future treatment of JDM with rituximab, reduce the number of “non-responders” who are treated with rituximab, and ultimately decrease the time any child spends with active JDM.
We would like thank all the patients that participated in this study and CureJM for the funding without which this research could not happen."

NATIONAL FAMILY CONFERENCE

Travis Kinder, Ph.D., a research fellow at the NIH explains a poster covering the Cure-JM funded, JM Drug Development Program.

Our 12th Annual Family Conference drew families together with researchers working towards progress against JM, connected families with information and support, and linked families with others that share the rare, JM experience.

Those families got a chance to meet and learn from the world's leading researchers, health care providers and subject matter experts on broad topics, from treatments and side effects, to mental health and wellness.

We're always on the lookout for ways to deepen the conference experience and make research more accessible to our families. In 2018, we invited Cure JM-funded researchers to summarize their studies and present that information on large-format posters. The posters were displayed in an open space, where families could wander around and read about each study, its purpose, its methods and its results. Many research authors were there, in-person to answer questions. It's important to make scientific research accessible to our families and it's also important that the information is clear and understandable. So, we translated the studies into plain English and compiled the research into a booklet. The lay summary booklet explains the important, relevant work in easy-to-understand language. The booklet is here: curejm.org/dc2018-recap/pdfs/Lay-Summaries.pdf.

NATIONAL FAMILY CONFERENCE, CONT.

The annual conference is the time when we share our Strategic Research Priorities with our families. Those priorities operate as our “Roadmap to a Cure.” They 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); and Providing Access to Quality Care; and Fostering Collaboration (sharing knowledge, data, and resources to speed research advances and improve clinical care).
These priorities are Cure JM’s guide to the future, and families saw the priorities in action through the reports and presentations from clinicians and researchers. You can watch video recordings of select conference sessions at curejm.org/dc2018-recap/2018-videos.php.

Also, families personally contributed to scientific research and our understanding of JM.
Cure JM invests in a genetic discovery program at the Center for Applied Genomics (CAG) at Children's Hospital of Philadelphia (CHoP), that seeks to identify and understand the genetic markers that play a part in JM. Researchers and phlebotomists worked with families to fill out medical and family history paperwork and then collected blood or saliva. Samples were collected from JM patients, their siblings, parents, grandparents, and extended family. “Having families participate in research at the Cure JM Conference was a great opportunity,” said Kate Ettingoff, President of the Philadelphia Chapter of Cure JM. “I would like to thank CHoP, and all the nurses, phlebotomists, and researchers that are giving so much of their time and energy in support of Juvenile Myositis patients. As a parent, I truly appreciate their time and efforts."

You can read all about this study in the, "JM Genetic Research Update" piece in this report.

A JM family member contributes to CAG's JM genetic research study.

NATIONAL FAMILY CONFERENCE, CONT.

JM research is not relegated to genetic science. We partnered with three healthcare providers, Andrea Knight, MD, MSCE, Dawn Wahezi, MD, and Kaveh Ardalan, MD, MS, to pioneer a focus group study, “Parent Perspectives on Addressing Emotional Health for Patients with Juvenile Myositis.” This session drew together parents to talk about how their children cope with JM. There were three sessions broken out by age: parents of children 6-12; 13-17; 18-21. Each session was moderated, so the researchers could understand how these parents meet the emotional health needs of their children. The researchers will incorporate what they learned into work they are doing on the impact of emotional health on JM patients.

We want you at this year’s conference, just outside of Chicago, at the Hyatt Lodge at McDonald's Campus in Oak Brook, IL. Dates of the conference are June 21th – June 23.

REGISTER FOR THE 2019 CONFERENCE: CUREJM.ORG/CHICAGO

Teens meeting other teens at the Cure JM National Family Conference.

VISIONS FOR CURE JM

Cure JM Chairman, Mitali Dave

"My vision for Cure JM is to provide excellent family support to our Cure JM families through online resources, regional chapters, and annual conferences. No family should face this rare diagnosis alone without the support and collective wisdom of the greater Cure JM family.

Mitali Dave, MBA

At the same time, Cure JM will continue to expand access to quality care through our regional Centers of Excellence in Seattle, Chicago, DC, and Raleigh-Durham, with plans to expand as funds permit. My hope is that these Centers raise the bar for JM patient care, and that they share their best-in-class practices to the larger medical community, so that all JM patients receive the chance they deserve for full and healthy lives. On the research side, my vision for Cure JM is to accelerate scientific discovery and more quickly move new, less toxic, more targeted therapies to patients through precision medicine.

All of the above requires substantial funds. Our small rare disease community must rally together and reach out to our families, friends, colleagues, and acquaintances to share our personal stories and raise the necessary funds. I firmly believe that there is no greater force than the love of parents for their children, and that is what will fuel us to ultimately prevail and find a cure for our kids!"

Nikki Hahn

Cure JM President, Nikki Hahn

"There are three strategic areas where we can make a real difference: fundraising, science, and community.

Fundraising is obvious. Our ability to affect change is limited by financial resources, as research, studies, and drug trials all require funding. We are also focusing on expanding and supporting our chapters and extraordinary local leaders while continuing to encourage and support our family fundraisers.

Science is the reason we are here. With Dr. Heaton's leadership, we will focus our research funding and advisement on drug repurposing and precision decision medicine to provide better, more targeted treatment options with less side effects.

As we evolve into a larger organization, we must maintain the inclusion and warmth that a smaller organization provided, prepared to execute on the ultimate challenge of our vision: to cure JM.
I've never been more confident in the outlook for JM as a disease, in large part due to the work of Cure JM. It's amazing to think of what that lemonade stand in Encinitas, California, by our amazing founders, Shari and Tom Hume started 15 years ago, and the journey it has taken all of us on."

WHAT IS A CENTER OF EXCELLENCE?

Cure JM Centers of Excellence are recognized for the extraordinary care they provide to juvenile myositis patients and families. Their staff and physicians bring the highest level of clinical experience and knowledge to the JM community and are world-class leaders in JM care and research. Cure JM Centers of Excellence also benefit from a string of institutional support from their respective institutions, which assures that Cure JM's financial investment to support clinical care and research is highly leveraged by an institutional commitment to building a robust JM program.

We currently fund four Centers of Excellence that are geographically disbursed around the country:

1. Duke Children's Hospital and Medical Center

- To make an appointment, cal (888) 275-3853

2. Seattle Children's Hospital

- To make an appointment, call (206) 987-2000

3. Stanley Manne Children's Research Institute, affiliated with Ann & Robert H. Lurie Children's Hospital of Chicago

- To make an appointment, call (773) 755-6310

4. George Washington University Myositis Center

- To make an appointment, call (202) 741-3333

The George Washington University Myositis Center treats patients from around the world. Patients must be age 8 and up, and are treated at no charge to the family thanks to Cure JM's support. Email Michelle Best at mbest@mfa.gwu.edu to learn more.

Our current Centers of Excellence.

SUPPORTING THE BEST & BRIGHTEST

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 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.

Understanding Heterogeneity in Pediatric Rheumatic Diseases to Inform Precision Medicine Approaches to JDM
Jessica Neely, M.D.
Clinical Fellow, University of California, San Francisco, Benioff Children's Hospital

Dr. Neely is using a computational approach to identify biomarkers by leveraging existing data to identify transcriptional signatures common among patients with JDM. She is also using a novel sequencing technology that allows examination of gene expression on the individual cell level. This technology holds great promise in identifying new biomarkers for children with JDM and will help us learn more about the cause of disease.

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.

OUR MEDICAL ADVISORY BOARD

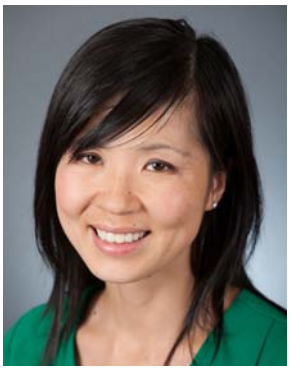
Our Medical Advisory Board (MAB) is comprised of internationally renowned researchers and clinicians in the field of juvenile myositis. The MAB provides insight, scientific direction, and expertise to Cure JM's Board of Directors and Staff. The MAB also consults with clinicians who request guidance in the treatment of a patient.

Lisa G. Rider, M.D.
Chairman, Cure JM Foundation Medical Advisory Board
Deputy Chief of the Environmental Autoimmunity Group, National Institute of Environmental Health Sciences, National Institutes of Health

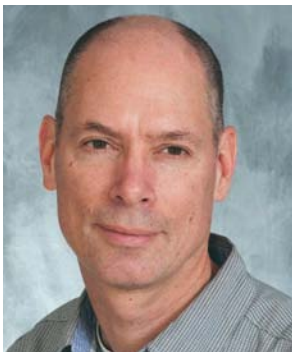
Lauren M. Pachman, M.D.
Professor of Pediatrics, Northwestern University Feinberg School of Medicine

Ann M. Reed, M.D.
Chair of the Department of Pediatrics, Duke University School of Medicine

Adam Huber, M.D.
Pediatric Rheumatologist and Professor of Pediatrics at the IWK Health Centre and Dalhousie University in Halifax, Nova Scotia, Canada



Susan Kim, M.D., MMSc
Associate Professor, Associate Clinical Professor of Pediatrics, University of California, San Francisco Department of Pediatrics



Brian Feldman, M.D., MSc, FRCPC
Professor of Pediatrics & Medicine, Faculty of Medicine, and the DLSPH University of Toronto Head, Division of Rheumatology SickKids

Lucy R. Wedderburn, PhD., MRCP
Professor and Consultant of Pediatric Rheumatology
NIHR Senior Investigator
Director, Arthritis Research UK Centre for Adolescent Rheumatology at UCL UCLH and GOSH

Kanneboyina Nagaraju, DVM, MVSc, Ph.D.
Founding Chair and Professor, Pharmaceutical Sciences, Binghamton University, State University of New York

SINCE 2003 YOU'VE ...

Raised $13 million for research and educational programs

Created resources and connections for over 3,000 patients and families in 45 countries

Funded over 175 critical research studies moving US closer to a cure

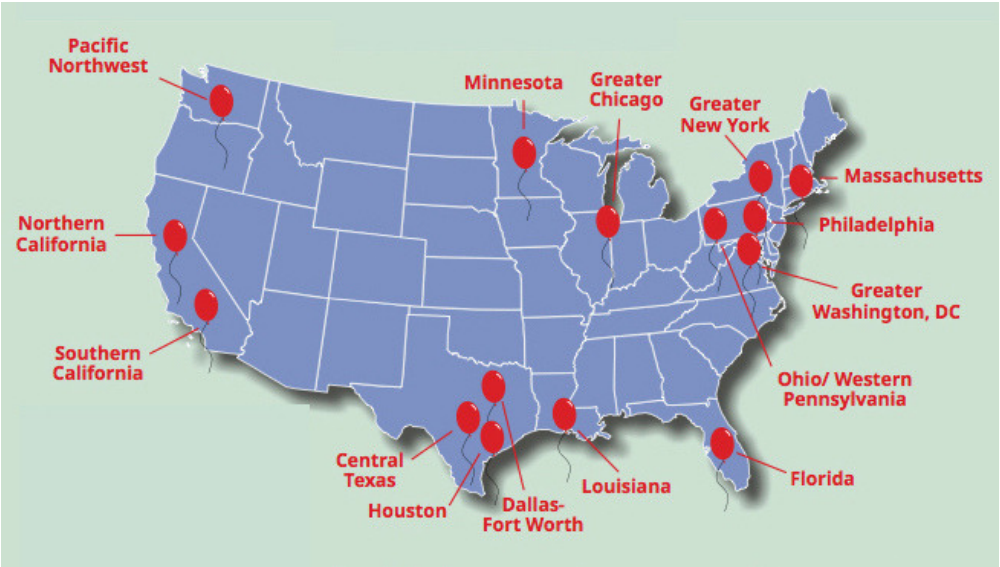
Supported four, major Centers of Excellence to advance JM research and deliver world-class care to JM patients at George Washington University Hospital, Lurie Children's Hospital, Seattle Children's Hospital and Duke Children's Hospital and Medical Center

Aided breakthrough genetics studies at the world-renowned Center for Applied Genomics at Children's Hospital of Philadelphia, as well as at other institutions

Assisted major drug discovery initiatives at the National Center for Advancing Translational Sciences at NIH and in partnership with biotech companies with investigational new autoimmune drugs

Aiding integrated patient registries and biorepositories—critical assets and fundamental building blocks for future drug development and genomic discovery

STRONGER, TOGETHER



We developed Regional Chapters so that our 3,000 JM families have local support.

We have 15 regional chapters that have connected over 900 families and held over 25 events.

Walk Strong to Cure JM® is Cure JM's signature National Walk Program. Families, friends and communities come together to support children, teens, and young adults fighting Juvenile Myositis.

In 2018, we achieved a real milestone. Due to your extraordinary leadership and participation, Walk Strong to Cure JM® hit **$1,000,000 raised**.
Cure JM's regional chapters have held over 25 Walk Strong to Cure JM® family fun events where over 300 Cure JM families and 6,000 attendees participated.
In 2019, Cure JM families are organizing 15 Walk Strong to Cure JM® events. We hope you can join us at one near you!

Tracy Van Ness, DHSc Candidate, MS, PA-C, and family.

Be part of the movement! Join us at a walk near you: curejm.org/walkstrong

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OUR NATIONAL LEADERSHIP

National Leadership Council

Cure JM's National Leadership Council is a collection of individuals that drives Cure JM's success in communities across the country. These families are a crucial part of Cure JM's strategic planning and strategy implementation, and create a platform for leaders to raise the funds required to accelerate research progress. The council is comprised of all regional chapter presidents, walk chairs, and family support network representatives, and fundraising leaders in the Cure JM Community.

The National Leadership Council has seven committees and meets formally on a quarterly basis, including a dinner and half-day strategy session at the National Family Conference.

Kevin Coffey, President, National Leadership Council
Denise Koch, Vice President, National Leadership Council

National Grandparent Council

Merrianne Van Ness, Chair, National Grandparent Council
Susan and Dave Erickson, National Grandparent Council
Sheila and Harry Harvey, National Grandparent Council
Laurel and Chuck Krider, National Grandparent Council
Marge Coffey, National Grandparent Council
Walter McKeever, National Grandparent Council

International Family Support Committee

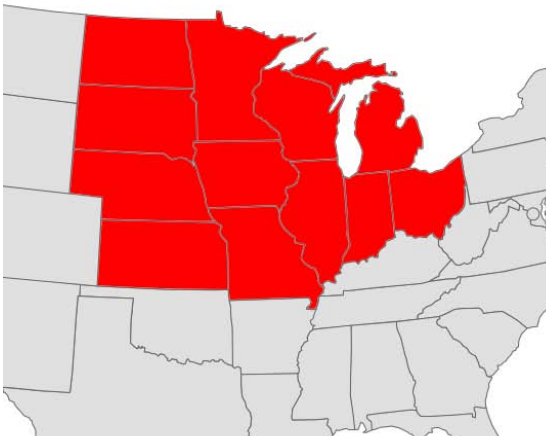
Suzanne Edison, International Family Support Network
Aimee McCloskey, International Family Support Network

Our National Leadership Council.

LEADERSHIP, CONT.

Midwest Chapters, Walks, Fundraising Committee

Zack and Sandi Harrison, Ohio/Pittsburgh Chapter Presidents
Erin Curtis, Ohio/Pittsburgh Chapter Family Outreach Chair
Kathryn Hewitt Dean, Ohio Walk Fundraising Leader
Danielle Harris, Minnesota Chapter President
Joleen Johnson, Minnesota Walk Chair
Julie Garst, Family Support Network, Midwest
Jen Coe, Chicago Chapter President
Karen Berman, Chicago Walk Chair
Denise Koch, Chicago Chapter Family Outreach Chair
Kelly Florido, R.N., Chicago Walk Leader
Denise Hooper, Chicago Walk Fundraising Leader
Rachel Lebensorger, Chicago Fundraising Leader
Sharon Ortega, R.N., Chicago Walk Fundraising Leader
Caesy Sauder, Chicago Walk Fundraising Leader
Tyla McMullen, Wisconsin Fundraising Leader
Lisa Forgas, Former Ohio/Pittsburgh Chapter President



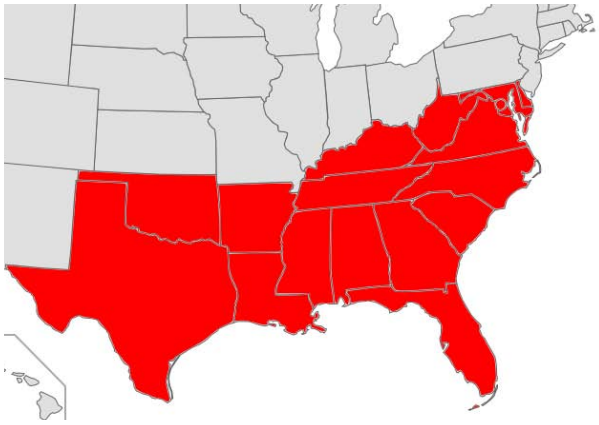
Northeast Chapters, Walks, Fundraising Committee

Justin and Amy Whitney, Maine Family Fundraising Leader
Chris Bokis, Massachusetts Chapter President
Melissa and Keith Corey, Massachusetts Walk Chairs
Stephanie Brown, Massachusetts Family Outreach Chair
Luke and Liz Ryan, Massachusetts Chapter Fundraising Leader
Kate and Dave Ettingoff, Philadelphia Chapter Presidents & Walk Chairs
Michelle Best, D.C. Chapter President
Scott and Shannon Taylor, D.C. Chapter Fundraising
Kevin and Doreen Coffey, New York Chapter Presidents
Tracy and Kelly Van Ness, New York Walk Chairs
Nikki Hahn, New York Chapter and Walk Leader
Ron and Keri Bernstein, New York Chapter Fundraising Leaders
Paul Jacoby, New York Fundraising Leader
David and Kathryn Maher, Connecticut Fundraising Leaders
Colleen and Chuck Marchetta, New York Fundraising Leaders
Allison Moore, New York Fundraising Leader
Leslie Budnick, New York Chapter Fundraising Leader
Glen Westrom, New York Chapter Fundraising Leader
David and Allison Taylor, New York Chapter Fundraising Leaders
Amber Davis, Family Support Network, Military Families
Erin Duval, Family Support Network, Northeast

LEADERS, CONT.

Southern Chapters, Walks, Fundraising Committee

Bud and Ryane Sheffield, Houston Chapter Presidents
Melissa and Tim Shelby, Houston Walk Chairs
John and Jenny Kara, Dallas Chapter Presidents
Danielle Britten, Dallas Chapter Family Outreach and Walk Chair
Sissy Taylor, Central Texas Chapter Co-President
April Duley, Central Texas Chapter Co-President
Cesar De La Canal, Central Texas Chapter, Family Support Network
Amy Gleason, Florida Chapter President
Melissa Hollerand, Florida Walk Chair
Lance Kerwin, Florida Walk Fundraising Leader
Denise Rackauaskas, Former Florida Walk Chair
Ragan Cantrelle, Louisiana Chapter Co-President
Sheila and Harry Harvey, Louisiana Chapter Co-President
Kristen Prescott, Louisiana Chapter Co-President
Jane Amaba, Tennessee, Fundraising Leader
Shari Shobe, Family Support Network, Southeast
Jennifer Howard, Former Dallas Walk Chair



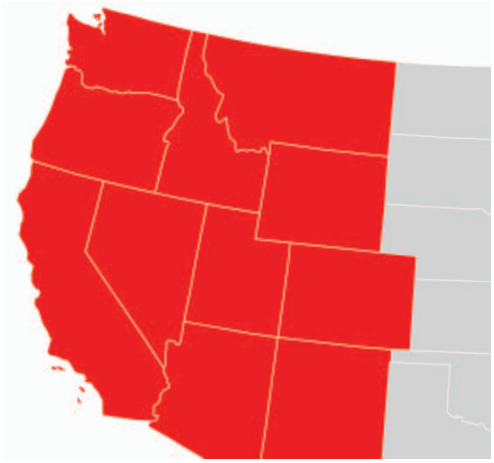
National Legacy Society Committee

Alan Alderfer, National Legacy Society Committee
Walter McKeever, National Legacy Society Committee

LEADERS, CONT.

Western Chapters, Walks, Fundraising Committee

Sue Carpenter, Northern California Chapter President
Brent and Emma Hauser, Northern California Chapter and Walk Fundraising Leaders
Suzy Clement, Northern California Walk Fundraising Leader
Victoria Robinson, Northern California Walk Fundraising Leader
Deema and Oscar Lopez, Southern California Chapter President
Carol Schwartz and Rob Ramsey, Southern California Walk Fundraising Leaders
Joy Garcia and Kelly Kapp, Southern California Fundraising Leaders
Erin Garcia, Southern California Fundraising Leader
Julie Shevlin, Pacific Northwest Chapter President
Jana Sall, Pacific Northwest Chapter Family Outreach Chair
Miranda and Alonzo Alvarez, Pacific Northwest Walk Fundraising Leader
Christy Coss, M.D., Oregon, Family Fundraising Leader
Damon Smedley, Oregon, Family Fundraising Leader
Natalie Strong, Wyoming Family Fundraising Leader
Kathy Yates, Arizona, Fundraising Leader



TO ALL OUR LEADERS,

WE'RE SO GRATEFUL FOR YOU AND YOUR EFFORTS! YOU'RE THE HEART OF CURE JM

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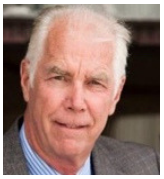

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FOUNDATION

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