

2023 IMPACT REPORT



20 YEARS OF PROGRESS

Dear Cure JM Families and Friends,

As we always say at Cure JM, we are a family of families. No time is that more clear than this year, our 20th Anniversary.



From our founding 20 years ago, by a small group of parents and grandparents, through to today, now 3,000 families strong, our families have always been the inspiration behind our work.

As I reflect on how far we have come in 20 years, I am inspired by your impact to:

- Reduced diagnosis time by 75%, from 12 months to 3 months
- Improve care and treatment protocols, to help patients get the best care
- Invest in research and partnerships to advance new treatments, like rituximab, abatacept, and baricitinib.

As we look into the future, we remain laser focused on:

- Accelerating scientific discovery
- Expediting the development of new and repurposed treatments for JM
- Helping patients get the best care

We could not accomplish any of this without you - our supporters. So, whether you are involved in our chapters, walks, Giving Tuesday, online support groups, or volunteer your time, talent, and treasure in other ways, you are a crucial part of our mission.

As I reflect on how far we have come in 20 years it is clear to me why Cure JM is one of the most effective rare disease organizations anywhere.

We are making progress because of your commitment to our children.

I am grateful to you for being by our side.

With appreciation,

A handwritten signature in black ink that reads "Jim".

Jim Minow
Executive Director



20 YEARS OF PROGRESS



Global

Research Network
and partnerships



\$23 million

Invested in research.

10

Clinical Trials

Completed or
in development

207

Research Studies

Improving treatments
and care



Reduced time to
diagnosis by

75%

From 12 months to 3 months

Early treatment leads to
better outcomes



20

 Clinical Care Network
partnerships

Helping patients get the best care



5

Centers of Excellence

Making significant
institutional investments in
juvenile myositis research



Breakthrough Genetic Studies

To identify genetic risk factors to help
diagnose, treat, and prevent the disease



3,000

Families and patients connected
Because we are stronger together

HOPE IS IN THE RESEARCH



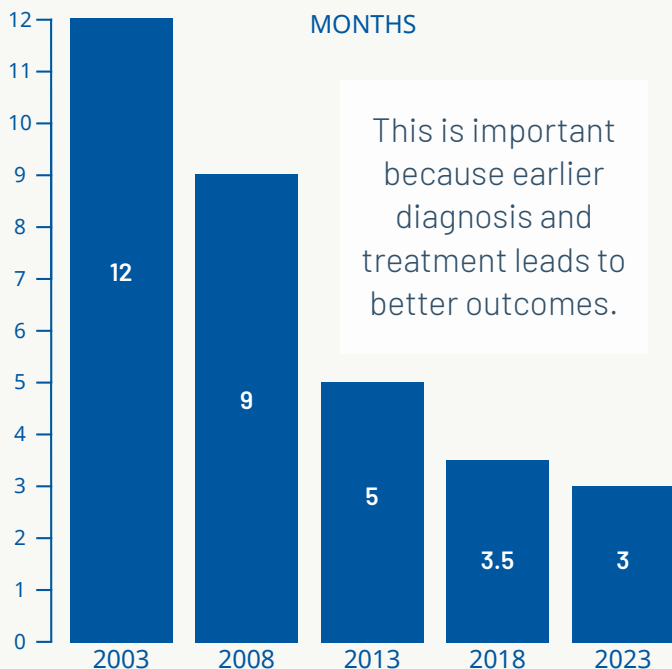
Caroline's family is grateful to the doctors and scientists working so hard to advance critical research.

These accomplishments are a testament to our passionate community of supporters and our dedicated scientists and clinicians.

Although we are proud of our many accomplishments, we still have much work to do.

We will not rest until every patient has better treatments, the best care, and can live their lives to the fullest.

75% REDUCTION IN TIME TO DIAGNOSIS



Young adult JM advocates gathering at Cure JM's Family Conference in Gaithersburg, MD, June 30, 2023.

YOUR DOLLARS AT WORK: 2023 HIGHLIGHTS

In 2023 we remained laser-focused on advancing treatments and care for juvenile myositis.

A Few Highlights from This Year:

- Funding our largest ever new grant cycle, details in the following pages
- Planning for trial of new “anti-steroid steroid”
- Expanded our Clinical Care Network to 20 partner locations, to help kids get the best care
- Resumed in-person events after a pause during COVID
- Launched our young adult Advocates Council, for patients age 18-30 to support each other
- Supported 3,000 families online and in-person

ADVANCING NEW TREATMENTS

Clinical trials for drugs like **rituximab (Rituxan)** and **Octagam 10** for the treatment of myositis are now complete, and these pharmaceuticals have joined the arsenal of treatments available to patients.

We recently completed a trial in **abatacept (Orencia)** and are starting a trial in **baricitinib (Omuliant)** at the NIH in the coming year.

We will remain laser focused – as we have for 20 years – on the advancement of new treatments as quickly as resources allow.





"Thanks to you, the future is brighter for the children and families we serve.

We are so grateful."


Nikki Hahn,
Chair, Cure JM Board of Directors, and parent of a child living with JM

CURE JM CLINICAL CARE NETWORK


Improving care for
the families we
serve.




Dr. Hermine L. Brunner **Dr. Alexei Grom**
CINCINNATI CHILDREN'S HOSPITAL




Dr. Brian Feldman
THE HOSPITAL FOR SICK
CHILDREN (SICKKIDS)



Dr. Marisa Klein-Gitelman
ANN AND ROBERT H. LURIE
CHILDREN'S HOSPITAL OF CHICAGO
CURE JM CENTER OF EXCELLENCE




Dr. Colleen Correll
UNIVERSITY OF
MINNESOTA




Dr. Sara Sabbagh
CHILDREN'S WISCONSIN
MILWAUKEE HOSPITAL




Dr. Jessica Turnier
C.S. MOTT CHILDREN'S HOSPITAL
UNIVERSITY OF MICHIGAN



Dr. Adam Huber **Dr. Bianca Lang**
IWK HEALTH CENTRE AND DALHOUSIE UNIVERSITY



Dr. Susan Sheno
SEATTLE CHILDREN'S HOSPITAL
CURE JM CENTER OF EXCELLENCE



Dr. Megan Curran
COLORADO CHILDREN'S HOSPITAL




Dr. Fatma Dedeoglu
BOSTON CHILDREN'S HOSPITAL



Dr. Susan Kim **Dr. Jessica Neely**
UCSF BENIOFF CHILDREN'S HOSPITALS
CURE JM CENTER OF EXCELLENCE



Dr. Julie Fuller
TEXAS SCOTTISH RITE
HOSPITAL FOR CHILDREN
DALLAS CAMPUS




Dr. Stacey Tarvin
RILEY HOSPITAL FOR
CHILDREN



Dr. Dawn Wahezi
THE CHILDREN'S HOSPITAL
AT MONTEFIORE





Dr. Ruy Carrasco
CHILD NEUROLOGY
CONSULTANTS OF
AUSTIN





Dr. Marietta de Guzman
TEXAS CHILDREN'S HOSPITAL
BAYLOR COLLEGE OF
MEDICINE



Dr. Kelly Rouster-Stevens
CHILDREN'S HEALTHCARE OF
ATLANTA EMORY UNIVERSITY
SCHOOL OF MEDICINE



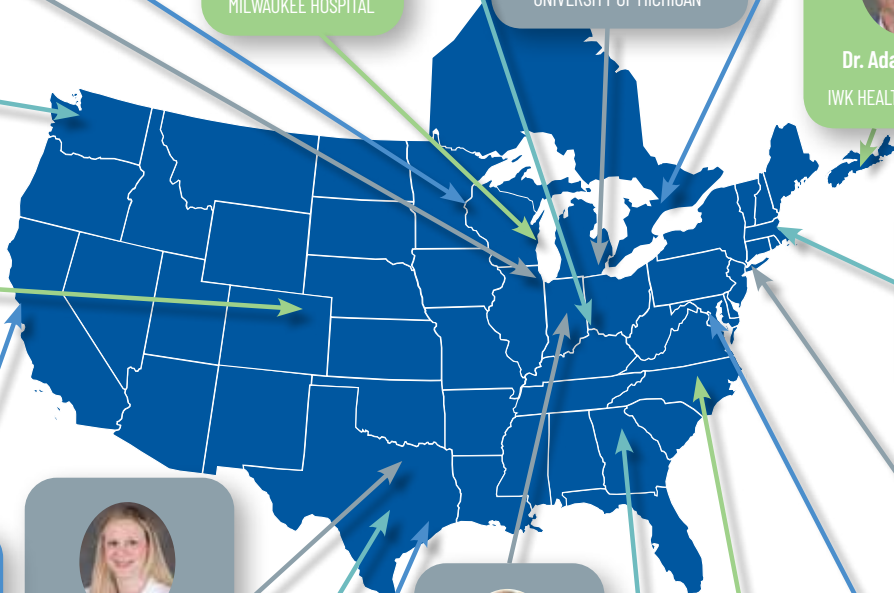
Dr. Rodolfo Curiel **Dr. Hanna Kim**
GEORGE WASHINGTON UNIVERSITY
GW MYOSITIS CENTER
CURE JM CENTER OF EXCELLENCE



Dr. Charris Papadopoulou **Dr. Lucy Wedderburn**
UNIVERSITY COLLEGE LONDON
GREAT ORMOND STREET HOSPITAL FOR CHILDREN



Dr. Kaveh Ardalan **Dr. Jeffrey Dvergsten** **Dr. Ann M. Reed**
DUKE UNIVERSITY SCHOOL OF MEDICINE, CURE JM CENTER OF EXCELLENCE



YOUR DOLLARS AT WORK: CURRENTLY FUNDED RESEARCH

Cure JM is proud to have nearly \$4 million invested in active research commitments.

Our network of researchers spans the globe and is making progress in developing new treatments, understanding the causes of juvenile myositis, and improving care for patients. This research advances our work to improve the treatments, care, and support for juvenile myositis.

As we say, hope is in the research.

Some highlights from our current research grants are:



Brian Feldman, MD, MSc,
FRCPC, Hospital for Sick
Children, Toronto, Canada

To study the effect of **exercise, creatine, and coenzyme Q10 supplements as treatments for muscle weakness** in juvenile myositis.

What is the desired outcome?

That these treatments could supplement traditional treatments to help patients thrive while living with juvenile myositis.



Sarah Tansley BSc, MBChB,
PhD, MRCP. University of Bath,
UK (With Prof. Neil McHugh,
MBChB, MD)

To use specially adapted detection methods to **identify 'new' myositis specific autoantibodies** that have yet to be discovered in approximately 40% of myositis patients.

What is the Desired Outcome?

To aid in understanding how to treat these sub-types of myositis and improve outcomes for the nearly 40% of JM patients who do not test positive for the current autoantibodies.



Jeff Dvergsten, MD, Duke
University, North Carolina
(with George Truskey, PhD and
Lauren Covert, MD)

As JDM is a rare disease, it can be difficult to have enough patients to test new medications. **This project aims to develop a new muscle model that mimics JDM to test the effectiveness of new drugs.**

What is the Desired Outcome?

To find better treatment options for juvenile myositis, with fewer side effects, that could later be tested in clinical trials.



Sara Sabbagh, DO, Medical
College of Wisconsin

For development of a new animal model to **screen new or repurposed drugs for effectiveness and safety in JM**. This is an important step in identifying new treatments for JM.

What is the Desired Outcome?

This study can lead to new, safer, and more effective treatment options with drugs that have been effective in treating other diseases.

YOU ARE THE POWER BEHIND THIS PROGRESS

CURRENTLY FUNDED RESEARCH



Kaveh Ardalan, MD, MS,
Duke University, North Carolina

Funding pre-trial work on vamorolone, an “anti-steroid steroid.” Vamorolone is a new steroid with all the benefits of prednisone and virtually none of the side effects. Cure JM is working with Duke University, the FDA, and the distributors of vamorolone on a clinical trial plan to test the safety and effectiveness of the drug in juvenile myositis patients.

What is the Desired Outcome?

That this project might lead to a clinical trial for an “anti-steroid steroid” (a steroid without the side-effects of current steroids) that could be useful as a first-line treatment option for JM patients.



Melissa Morales, PhD,
Binghamton University,
New York

This project will **test and screen potential medications** on the effect of Type 1 interferon (a prime factor in inflammation) on muscle function and weakness in myositis.

What is the Desired Outcome?

A better understanding of how to target and more precisely treat muscle inflammation in JM patients.



Paul Norman, PhD, Professor,
Biomedical Informatics,
University of Colorado

To shed new light on the genetics of juvenile myositis by using a new method to investigate the HLA genomic region (a well-known region of mutations in the human genome in JDM).

What is the Desired Outcome?

To develop a further understanding of the genetic risk factors for juvenile myositis. This may help develop new diagnostic indicators and treatment targets.



Younghun Han, PhD. Baylor
College of Medicine, Texas

To better understand what drives JDM by identifying which genes and gene combinations predispose patients to JDM.

What is the Desired Outcome?

We aim to expand our knowledge of the causes of JDM to improve treatments and minimize environmental triggers and disease flares.



Chuck Yung Yu, D.Phil.
Nationwide Children's Hospital,
Ohio State University (With
Samantha Coss, MD, PhD)

To study changes in immune cells and proteins in different phases of JDM. We also hope to understand how genetic factors (in particular, a low level of an immune protein known as complement C4) contribute to a higher probability of developing JDM.

What is the Desired Outcome?

To provide new markers to predict disease status and help guide treatment and disease management decisions.



Jayne MacMahon MB, BCh,
BAO, LCRP&SI, MRCPCH.
SickKids, Toronto, Canada

To define a scoring system for a molecule known as interferon to help define how active JDM is.

Interferon plays a crucial role in the immune system and is a prime factor in inflammation.

What is the Desired Outcome?

We know that JDM is a variable disease that affects different patients differently. We aim to develop more individualized approaches to disease management and better outcomes for patients. For example, a scoring system for interferon may help clinicians know when to start and stop different medications.

CURRENTLY FUNDED RESEARCH



Meredyth Wilkinson, PhD,
University College London, UK

Dr. Wilkinson was awarded Cure JM's early investigator grant to study the potential use of drugs categorized as JAK inhibitors. **The drugs being studied included baricitinib (Olumiant), tofacitinib (Xeljanz), and ruxolitinib (Jakafi).**

What is the Desired Outcome?

To help lay the foundation for future clinical trials to bring new, alternative, and supplemental treatments to patients. Alternative treatments have fewer side-effects and can be less disruptive to a patient's life, which results in better long-term outcomes.



Christian Lood, PhD, University
of Washington

This project aims to understand **how mitochondria contribute to disease in JDM patients**. The mitochondria are the "powerhouses" of the cell, responsible for generating most of the cell's energy.

What is the Desired Outcome?

Understanding how mitochondria contribute to JDM may allow us to target this process with new therapies. We also hope to develop mitochondrial-related biomarkers to enable preventive treatment of calcinosis, a difficult complication to treat for some patients, and help improve patients' quality of life.



Joanna Parkes, PhD, Harwell
Science and Innovation
Campus, Nucleic Acid Therapy
Accelerator Hub, UK

This project will study **potential "nucleic acid" therapies for JM**, with the goal to identify novel gene therapy interventions. Gene therapy is an emerging field of research with the potential to revolutionize the way we treat diseases.

What is the Desired Outcome?

It is our hope that this might lead to better treatments and early interventions that might stop JM in its tracks.



Jessica Neely, MD, University
of California San Francisco

Using artificial intelligence to analyze data from three sets of participants: JM patients who have received treatment, JM patients who have not yet received treatment, and healthy volunteers.

What is the Desired Outcome?

That this project will help us identify new treatments (with fewer side effects) for juvenile myositis.

"The research progress I see, and the growing Cure JM community, gives me such hope for the future for all JM patients."

Anna Ramsey, age 26
Chair, Cure JM young adult Advocates Council

CURRENTLY FUNDED RESEARCH: PARTNERSHIPS, REGISTRIES, AND BIOREPOSITORIES

Funding JDM registry and biorepository through CARRA , the Childhood Arthritis and Rheumatology Research Alliance

The CARRA JDM registry is a valuable resource for patients, researchers, and clinicians. It is helping to advance the understanding and treatment of pediatric rheumatic diseases including JDM.

JDM Registry

- A collection of data about individuals with a particular rare disease.
- Registries are used to:
 - understand the disease
 - document different responses to treatments
 - identify risk factors and biomarkers
 - develop and evaluate new treatments
 - develop diagnostic tests
 - facilitate research
- Registries help to overcome the challenges of studying rare diseases— small number of patients & geographic dispersion

Biorepository

- A facility that collects, catalogs, and stores samples
- Samples include
 - Blood
 - Urine
 - Muscle samples
- Plays an important role in advancing research, especially for rare diseases
- Provides researchers with access to a wide variety of samples that would take many years to collect

Partnerships with the International Myositis Assessment & Clinical Studies Group (IMACS), the Global Conference on Myositis (GCOM), and the Childhood Arthritis and Rheumatology Research Alliance (CARRA) JDM Working Group

These partnerships enhance global collaboration by bringing together myositis researchers to work together to advance JM research and clinical care.

What is the Desired Outcome?

It is our goal to increase global collaboration, thereby accelerating the pace of research, facilitate the development of new treatments, and continue to improve care for patients. These factors all combine to improve patient outcomes.

CURRENTLY FUNDED RESEARCH: EMOTIONAL AND MENTAL HEALTH SUPPORT

We are also proud to be funding three new grants to address the mental health and emotional support needs of the families we serve. This program was generously funded by the RB DenUyl Family Foundation in loving memory of Bruce and Eric DenUyl.



Aviya Lanis, MD. Seattle Children's Hospital, Seattle, WA

To develop a narrative medicine intervention program for JM patients. Narrative medicine practitioners collect patient stories and use them to improve communication between patients and healthcare providers. Dr. Lanis will also determine if there is any correlation between this intervention and reduced levels of stress for the patients who participate.

What is the Desired Outcome?

This can help healthcare providers develop more compassion for their patients, help patients feel more understood, and promote healing and recovery.



Colleen Correll, MD, MPH. University of Minnesota, Minneapolis, Minnesota

Along with a pediatric psychologist, and some of the families we serve, Dr. Correll is **creating mental health wellness and resilience-building videos to share with our families.** These videos will offer coping strategies for dealing with the emotional and physical challenges of chronic diseases, teach patients how to manage stress, pain, and fatigue, as well as how to maintain a positive attitude.

What is the Desired Outcome?

Our goal is that these videos can help improve the quality of life for JM patients. Building resilience has been associated with reduced stress, improved sleep, increased physical activity, reduced pain, and improved mood.



Andrea Knight, MD, MSCE. Hospital for Sick Children, Toronto, Canada

To implement and evaluate the impact of **a mental health screening program in JM clinics.** We will support the rheumatology clinic to provide mental health screening for depression, anxiety, and other possible mental health issues that can go along with managing a chronic disease.

What is the Desired Outcome?

To screen for emotional health needs and provide emotional health support in the rheumatology clinic. We also hope to expand this program to other locations of Cure JM's Clinical Care Network in the future. (This grant is also mentioned in the following section because it was funded in part by grants from both funding programs).



CURRENTLY FUNDED RESEARCH: IMPROVING PATIENT CARE

And finally, we are proud to be funding four Mason's Miracle grants to clinician members of Cure JM's Clinical Care Network. The goal of these grants is to improve care to patients. These annual grants are named in memory of Mason Smedley, who showed great courage and strength in his battle with JM.



Dawn Wahezi, MD, MS.
Montefiore Children's,
New York City, New York

To provide the JM clinic with the most up-to-date diagnostic nailfold capillaroscopy (NFC) equipment. NFC means using a microscope to examine the capillaries (tiny blood vessels) in the nailfold (the skin where the nail meets the cuticle). Abnormalities in these blood vessels can indicate that juvenile myositis is active or "flaring". This is important because nailfold abnormalities can serve as a "canary in the coalmine" and indicate that the disease is active, oftentimes before muscle tests or blood tests are able to identify active disease.

What is the Desired Outcome?

It is our belief that providing this equipment will allow clinicians to identify active disease earlier. We believe that the NFC may also help evaluate if the patient is responding to treatments, so that adjustments to the patient's medications can be made early. We hope both factors can combine to improve the patient's quality of life.



Megan Curran, MD. Colorado
Children's, Denver, Colorado

To develop a standardized UV protection educational program for families. This is important because UV protection is a crucial and often misunderstood part of managing juvenile myositis.

What is the Desired Outcome?

To improve patient and family understanding of the importance of UV protection, which we believe will help improve overall patient outcomes. If successful, we hope to replicate this program at other Cure JM Clinical Care network locations.



Andrea Knight, MD, MSCE.
Hospital for Sick Children,
Toronto, Canada

To implement and evaluate the impact of a mental health screening program in JM clinics. We will support the rheumatology clinic to provide mental health screening for depression, anxiety, and other possible mental health issues that can go along with managing a chronic disease.

What is the Desired Outcome?

To screen for emotional health needs and provide emotional health support in the rheumatology clinic. We also hope to expand this program to other locations of Cure JM's Clinical Care Network in the future. (This grant is also mentioned in the preceding section because it was funded in part by grants from both funding programs).



Sheila Angeles-Han, MD, MSc.
Cincinnati Children's,
Cincinnati, Ohio

To validate new measures of JDM disease activity, including nailfold capillary dropout, a urine-protein based biomarker panel as a measure of JDM activity, and to compare at-home childhood myositis assessment score (CMAS) to the in-clinic CMAS scores of patients.

What is the Desired Outcome?

We aim for these programs to help improve standardized care and outcomes for juvenile myositis patients.

**We could not do this work without you and
are so grateful for your support**

POWERING PROGRESS: OUR FUNDRAISING SUCCESS

From Cure JM's first fundraiser – a lemonade stand held 20 years ago – through to today, you are powering our progress.

This is true for most rare diseases: research is powered by friends and families.

Highlights from our 2023 campaigns:

- In July 2023, our signature Walk Strong raised over \$500,000 for research. Next year, our walks will be back in person. Invitations coming in the new year.
- Last year's Giving Tuesday Holiday Challenge raised over \$1,000,000 for research. [CLICK HERE](#) to learn more about this year's campaign..
- Families and friends hosting DIY Fundraisers raised over \$100,000. [CLICK HERE](#) to learn more about these fun events, including a winery dinner, a golf event, and bike rides.

These funds raised were put right to work to improve treatments, care, and support for the families we serve.

*Your
participation
powers
progress.*



Walk Strong to Cure JM®
Washington D.C., 2023

POWERING PROGRESS: OUR LEADERSHIP

Founded by parents and grandparents of children with juvenile myositis, for 20 years Cure JM has been driven by the passion and commitment of our families.

Families remain our driving force today.

"We could not do this work without our families, friends, and communities," says Kristine Alderfer, President, Cure JM's Board of Directors. "We are grateful for your compassion and support. You are changing the world for children diagnosed with juvenile myositis."



Cure JM Board of Directors



Cure JM National Leadership Council



Cure JM Young Adults Advocates Council



Cure JM Grandparents Council

Our mission is carried out by:

- **Our Board of Directors**, which leads our work to fulfill Cure JM's mission.
- **Our Medical Advisory Board**, which provides insight, scientific direction, and expertise to Cure JM's work to advance research and care.
- **Our National Leadership Council**, which leads the charge to raise the funds needed to advance our work.
- **Our Young Adult Advocates Council**, which engages our patients aged 18-30 to advance Cure JM's mission.
- **Our Community Advisory Board**, which provides guidance on ensuring diverse participation in clinical trials and research.
- **Our Grandparents Council**, which provides a platform for grandparents of patients to get involved, share ideas, and support their families.

View the full list of leaders at www.curejm.org/about-us

POWERING PROGRESS: OUR GRANDPARENT SUPPORTERS

Please join us in celebrating the special role that grandparents play within their families and within our community. Since Cure JM was founded by parents and grandparents in 2003, grandparents have always been a special part of Cure JM. We treasure the special role of grandparents and are grateful for their support.

Cure JM is honored to recognize the following grandparents for their lifetime giving

\$500,000+

Marge Coffey
Bruce and Judy Leetz



\$50,000-\$499,000

Michael and Susan Duke
Sheila and Harry Harvey
Nancy Hume
Rita and Richard Jacoby
David and Carol Pearson
Robert and Dixie Slater
Merriane Van Ness

In addition, Cure JM would like to recognize those Grandparents who gave between \$1,000-\$10,000 in our Fiscal Year 2023

(October 1, 2022 through September 30, 2023)

Sheryl and Stuart Chuzmir	Joanne and Jeffrey Hall	Marti Peavey
Kenneth Cutting	Linda and Randy Hart	Ginnette Pimentel
Elinor and Andrew Dahill	Allen and Antoinette Hauser	Joel and Laurel Reed
Phuoc Dang	Rosemary and James Hower	Nan and Dan Schaper
Melanie and David DeKowny/Reinhardt	Rita and Richard Jacoby	Lisa Schreiner
James and Judie Denton	James and Cheryl Jenkins	Mary Shine
Patricia Dobson	Lori and Joe Kania	Laurette and Michael Shulman
Mike and Susan Duke	Annemarie Kessler	Robert Slater
Steve and Trina Edwards	Chuck and Laurel Krider	David Stoms and Enid Pritikin
Mary Ann and Dave Eisenreich	Bruce and Judy Leetz	Christine and Wayne Takahata
Stuart and Susan Ettingoff	Michael and Celene Marsallo	Terry Tobin
Mike and Patricia Fox	Larry McFall	Merriane Van Ness
Josie Garcia	Wayne Mikos	Heather and Mike Weiss
Denny and Patricia Grundy	David and Sharon Naccarati	
Robert and Karen Hahn	Lynn and Skip Neuenswander	

If you have any questions about this list, please contact Betsy.Leon@curejm.org

OUR FINANCIALS



Since inception in 2003

Cure JM has been recognized with awards for its public accountability, program effectiveness, and cost effectiveness.

Cure JM has received the Top-Rated Nonprofit Award from GreatNonprofits.org every year since 2011. GuideStar USA has awarded Cure JM the Silver Seal of Transparency for our commitment to financial transparency and accountability.

Support our Work:

- Give online at www.curejm.org/donate
- Checks can be made out to "Cure JM" and mailed to Cure JM Foundation, P.O. Box 45768, Baltimore, MD 21297.
- To give from your IRA, give a gift of stock, or cryptocurrency, or more ways to give, please contact our Director of Development and Family Engagement, Shannon Malloy, at donate@curejm.org or call us at (202) 596-6267

Cure JM Foundation is a 501(c)(3) non-profit organization. Our tax ID number is 35-2222262.



Top-Rated Since 2011

Emma, age 8, pictured receiving an all-day IV infusion of medication to manage her disease. With your support, we are creating a brighter future for kids like Emma.

Juvenile myositis is a runaway immune response, where the body mistakenly attacks its own healthy tissues and organs. This causes inflammation throughout the body, which can lead to muscle, joint, and skin problems, pain, and fatigue.

Today, juvenile myositis is generally treated with drugs that broadly shut down the immune system. This can be effective, but this also blocks the immune responses necessary to fight off infections, so there are a lot of side effects.

With your support, we are working to change that, by funding research to improve the treatments, care, and support for the families we serve.

With your support, children can imagine a brighter future.



Cure[®]JM

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