# CARRA Registry Newsletter

# Spencer's Journey and the Importance of CARRA -
## A Caregiver Perspective after CARRA's Annual Scientific Meeting

Spencer's vasculitis journey began in the summer of 2021 at a family reunion in Yellowstone. Spencer was feeling quite fatigued and when my dad checked his oxygen (O2) sat it was only 70%. To make a long story short, we ended up being irlifted to the PICU at Primary Children's in Salt Lake City. The fantastic team at Primary Children's set to work and had him on steroids, out of the ICU, and with a diagnosis in hand in just a few days. By the time we got our diagnosis, anti-glomerular basement membrane (GBM) and myeloperoxidase (MPO) positive vasculitis, I think we had seen nearly every specialty in the hospital.

Spencer's diagnosis came on the heels of a very traumatic process. Additionally, it felt so incredibly heavy that there was very little information guiding us in helping Spencer. Luckily we had support from family and incredible rheumatologists, nephrologists, and nurses that spent a lot of time explaining, teaching, helping, supporting, and validating. They got Spencer's disease under control quickly. They saved his life, then his kidneys. When it was time to go home they set us up with the University of Minnesota where we have continued to receive excellent care. It was textbook perfect and yet it was obvious to me that there is so much work to be done.

**The Ableman Family**

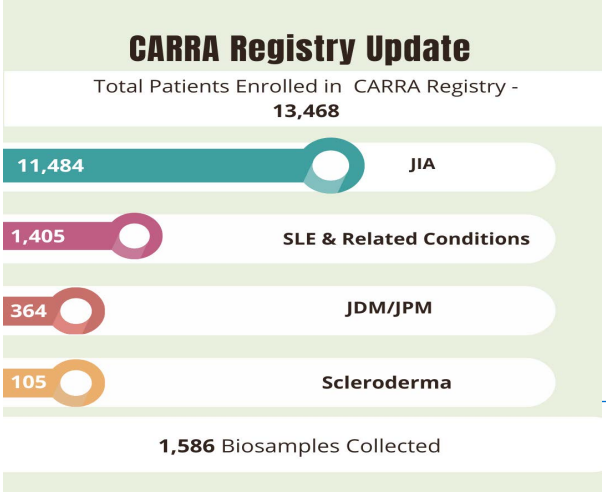
**Author/Caregiver/Mother: Kacey Ableman**

**Vasculitis Patient: Spencer**

The whole process left me with two big takeaways:

1) One, every kid deserves the level of care Spencer received. In Spencer's case we didn't realize we had a ticking clock on his kidneys, or even that his lungs were hemorrhaging. Any breakdown in communication or a lack of access to specialists would have been disastrous. It is heartbreaking to hear stories of patients who don't get the help they need.
2) We need more information. Treating pediatric patients with adult studies is terrifying. I am acutely aware of the shoulders we were standing on - the researchers, patients, and physicians that made the treatments we had available. We must be those shoulders for the next kids. We need more information so they aren't in the dark and can receive even better treatments.

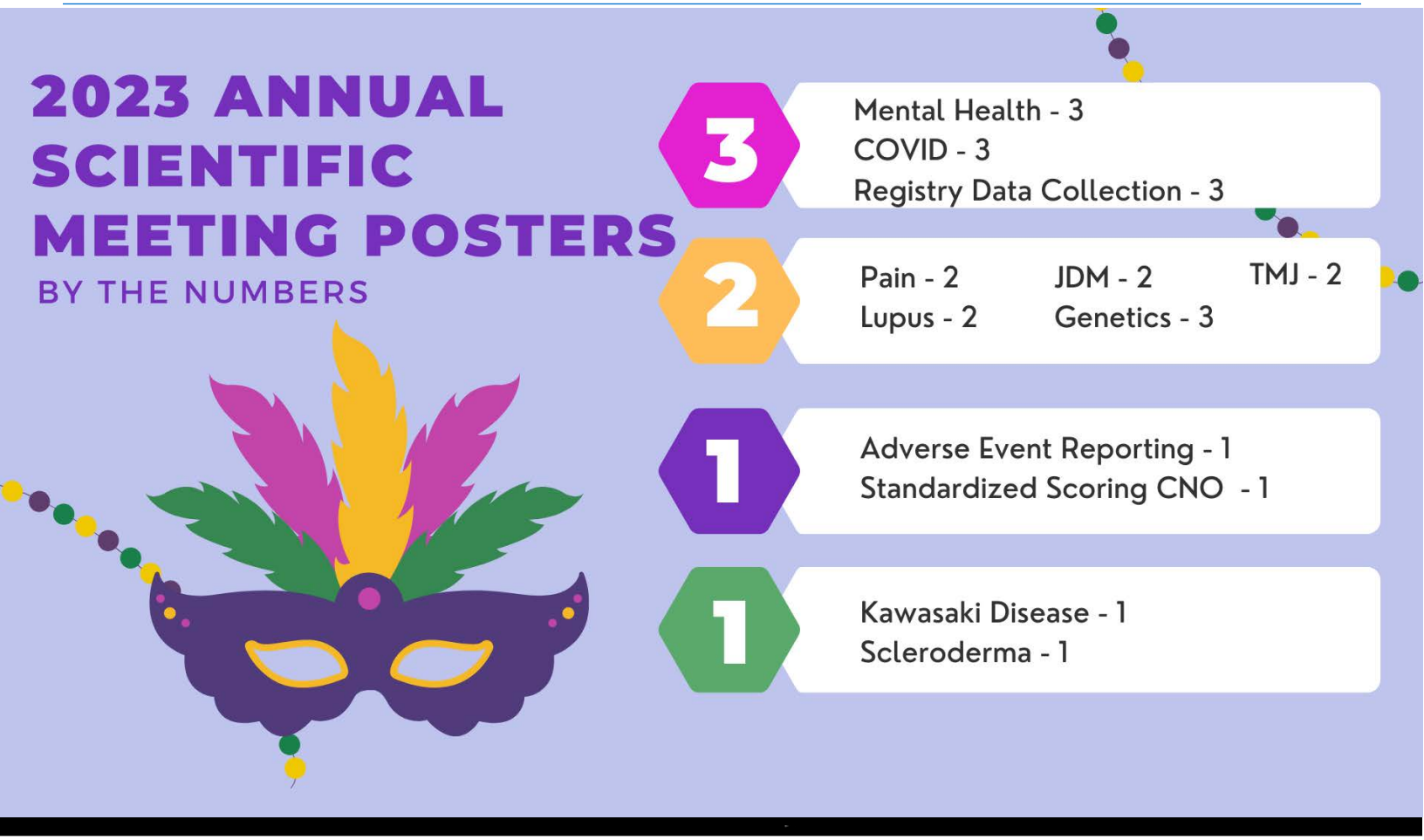
At the CARRA conference, it was so encouraging to see researchers and patients working together to break down barriers to care. Every new shred of information will lead to better and quicker decisions, thus leading to better outcomes. It was so easy to see how the work being done will benefit real families, families who will be going through one of the worst times of their life. Chronic disease is a burden these kids carry so heroically. What I saw at CARRA were professionals with a deep desire to lessen that weight, and the incredible skill to make a real difference. We left inspired to do more, and so profoundly grateful for those who are working so hard to help these amazing kids.




## In this Edition:



CARRA
1050 Connecticut Ave, NW
Suite 500
Washington, DC 20036
202-772-1028
www.carragroup.org




## Juvenile Dermatomyositis ABC Grant
## Juvenile Myositis Pilot: Treatment and Outcomes in Active Skin (JuMP TO Act)

Juvenile Dermatomyositis (JDM) is an orphan disease. Each year, about 3 in 1 million children in the United States are diagnosed with JDM. It can be hard to diagnose, there is currently no cure and better treatments are desperately needed.

JDM effects many parts of the body - especially the skin and the muscles. There are currently no FDA-approved treatments for JDM and even after 1 year of treatment over 1/3 have ongoing skin disease and 2/3 never reach remission. Long-term skin disease and skin damage is common in JDM patients and is associated with low self image, physical pain, and poor quality of life. Rashes can be persistent, painful and disfiguring calcium deposits in the skin called calcinosis. We need better ways to assess and treat children with JDM beyond our present approaches, especially those with ongoing skin disease.

This Advancing Biosample Collection (ABC) Grant we call the Juvenile Myositis Pilot: Treatment and Outcomes in Active Skin Disease (JuMP TO Act), will allow the collection of precious biosamples from this difficult to treat JDM subset children with ongoing skin disease. Together with the data from the Registry, this collection may allow our collaborating researchers to find new genes, proteins and biologic pathways that can help predict how children with ongoing JDM skin disease will respond to treatment so we can provide more effective treatments earlier.

The biggest challenges in performing research on rare diseases like JDM, are the small number of eligible children with JDM and the geographic distribution of children with JDM across the world. The CARRA Registry and Biorepository, and this ABC grant, gives us the resources and infrastructure to perform research in a way that was not previously possible in JDM.

# CDC Awards More Than $4 Million To Support Childhood Lupus Research

## *Lupus Advocacy Efforts Drive Research Funding for the CARRA Registry*

The Childhood Arthritis and Rheumatology Research Alliance (CARRA) announced that the U.S. Centers for Disease Control and Prevention (CDC) awarded a 5-year $4.4 million grant to a team of North American lupus experts to advance critical research on childhood lupus through the CARRA Registry.

The CARRA Registry was the first pediatric lupus registry funded by the CDC in 2019, resulting from nationwide rallying by lupus advocates for increased research funding to study the impact of lupus on children. Prior public funding of childhood lupus research was scarce.

Lupus in children and teens usually occurs more aggressively and severely than in adults. The disease can devastate a child's growth and day-to-day life and even shorten a child's life.

"The Lupus Foundation of America strongly advocates for increased research funding so that targeted treatments for children with lupus are developed and help to improve the quality of their lives," said Patrick Wildman, Senior Vice President, advocacy and government relations, Lupus Foundation of America (LFA). "Lupus advocates have been instrumental in sharing the critical need for childhood lupus research with our nation's leaders. We are grateful for their efforts and for the government's support of this research need."

## *New Funding Aims to Close Gaps in Pediatric Lupus Knowledge*

The CARRA Registry exists as the largest, multi-center observational cohort study in North America following children and young adults with lupus, including more than 1,000 participants with physician-confirmed lupus. Continued funding for the Registry will allow researchers to build on an earlier phase of the CDC grant in which researchers expanded the Registry's enrollment and comprehensive data collection. In that first phase, researchers began studying the natural history and disease trajectories of children with lupus, sociodemographic and disease characteristics, disparities in care and outcomes, and medication adherence.

Principal Investigators Drs. Aimee Hersh (University of Utah Health), Andrea Knight (The Hospital for Sick Children, Toronto), and Mary Beth Son (Boston Children's Hospital) will continue to lead groundbreaking research in the next phase of the grant to address key gaps in lupus knowledge. The CARRA Registry also aims to follow all participants longitudinally for at least 10 years.

"We are thrilled to receive this second round of funding from the CDC to further our mission of advancing meaningful research in pediatric lupus. This grant will use CARRA Registry data to help us better understand the long-term relationship between treatments and lupus disease activity starting in childhood." - Aimee Hersh, MD

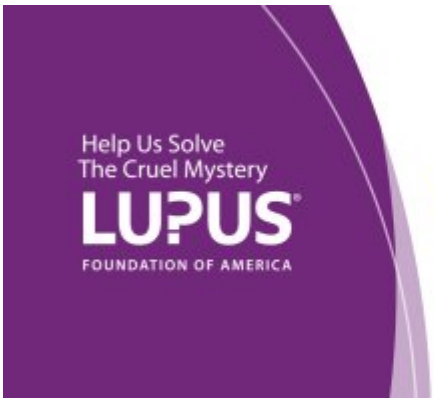
"We are also interested in characterizing neuropsychiatric lupus and patient-centered outcomes important for improving quality of life and reducing disability, both of which are poorly understood in children and young adults with lupus," Dr. Hersh continued. "What's more, we plan to explore disease outcomes in early adulthood once young adults transition from pediatric to adult care."

## *Lupus Foundation of America Leads in Child Lupus Research Funding Advocacy*

Lupus advocates nationwide played a key role in securing the funding that made the grant possible. Advocates, including people with lupus and their families, researchers, and advocacy organizations, worked with the U.S. Congress to increase funding for the National Lupus Registry Program through CDC and to recommend that the program increase its focus on childhood lupus. The Lupus Foundation of America led the charge to establish this program in 2003 and continues to work with the federal government and advocates to expand appropriations for all agencies involved in lupus as part of its comprehensive research strategy.

Between 10 and 20 percent of all cases of lupus develop during childhood. Two out of three children or teens with lupus develop kidney disease, which can cause permanent damage and can be fatal. Lupus also can adversely affect a child's growth, quality of life, as well as longevity.

CARRA researchers strongly expressed their appreciation of the LFA's partnership and advocacy efforts that made this grant possible. and their families who join the Registry as their participation is helping to make this study successful!




# 2023 Outstanding Patient Service Awardee: Ela Chintagunta

The CARRA Outstanding Patient/Caregiver Service Award recognizes outstanding contributions to CARRA by a caregiver or patient. The awardee demonstrates a commitment to CARRA's research through meaningful participation in CARRA-related activities, leadership, and a willingness to engage with members, workgroups, or committees.

This year, the Outstanding Patient/Caregiver Award was presented to Ela Chintagunta. Ela was diagnosed with JIA eight years ago and is currently in her second year at Grinnell College, double majoring in biology and chemistry.

Ela has distinguished herself through her enormous contributions to multiple CARRA workgroups, including JIA Outcomes, Transitions, Mental Health, and many others. Ela has taken a leadership role in these workgroups assisting CARRA members in designing future research projects. In addition, Ela engages her friends to assist with CARRA activities, such as pilot testing surveys to be used in research. Ela even submitted had her own abstract to the American College of Rheumatology (ACR), and it was submitted and accepted for the 2022 ACR meeting

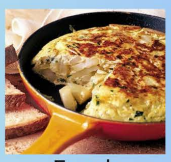
Congratulations Ela, and thank you for your contributions to CARRA!

## Ela's Favorites

**Roles at CARRA:** JIA Outcomes, Transition, Reproductive Health, Mental Health, Pain, Inactive JIA

Movie
Zindagi Na Milegi Dobara


TV Show:
Star Trek



Food:
Spanish Omelette

Hobbies:
Classic Indian Dancing, Volleyball and Badminton

Season:
Winter

Animal:
Sloth

Book:
Organic Chemistry by Clayden, Greeves & Warren

**"You never know how strong you are until being strong is your only choice."**
**- Bob Marley**

## Young Patient Spotlight: Claudette Johnson


Claudette is a Sophomore at Montclair High School in Montclair, New Jersey. She was diagnosed with Localized Scleroderma (LS) at age 9. She enjoys playing piano, reading and competitive diving. Claudette is also an incredible advocate and innovator. She is working on an upcoming CARRA podcast for patients and families.

Claudette polled attendees at the CARRA ASM in New Orleans, LA to see what topics should be first during the new podcast she is working on for CARRA. How patients and families can build community, where to find support, medical trials, the importance of being in the CARRA Registry and so much more will be covered in the podcast. This is just a teaser of what is yet to come!! Look for more information in the August Newsletter.

**CARRA is grateful to the following organizations for their financial support which makes the CARRA Registry possible:**

Arthritis Foundation®
LUPUS FOUNDATION OF AMERICA
CureJM FOUNDATION
SCLERODERMA FOUNDATION
Centers for Disease Control and Prevention
NIH's National Institute of Arthritis and Musculoskeletal and Skin Diseases Patient-Centered Outcomes Research Institute, Rheumatology Research Foundation