SCORE Launched to Understand Biology of Pediatric Scleroderma and Identify New Biological Treatment Targets

“When my daughter was diagnosed with scleroderma in 2016, I felt overwhelmed, isolated, and concerned that there was not enough research for children like her. I didn’t know that at that exact moment, a registry was being planned that could shape the treatment and future of all children with scleroderma.” - Mom of a daughter diagnosed with scleroderma

After years of planning and hard work, the Scleroderma Collaborative Research (SCORE) project – “Identifying juvenile scleroderma immunophenotype subsets” has begun and is enrolling patients across 19 Childhood Arthritis and Rheumatology Research Alliance (CARRA) sites in North America (see map).

SCORE participation is open to children and young adults diagnosed with:
• Localized scleroderma (LS, also called morphea) - an autoimmune disease which causes thickening and scarring of the skin, muscles and deeper tissue, or
• Systemic sclerosis (SSc) - an autoimmune disease that, in addition to skin, may also attack the lungs, heart, gastrointestinal system, and/or kidneys.

SCORE’s main goal is to advance juvenile LS and SSc research across North America. More specifically, researchers hope to identify new medicines by studying cells, proteins, and antibodies of children diagnosed with LS or SSc.

SCORE will enroll 150 children with scleroderma (75 LS, 75 SSc) collecting data and blood samples (biospecimens) over a two-year period. So far, the study team has:
• Developed customized forms tailored for both LS and SSc that clinicians use to record information at routine appointments,
• Created electronic databases to store clinical data and biospecimen information,
• Set up a place where the team can process, store, and share blood samples for this study and others in the future.

When the study ends, the patients will continue to be followed in the CARRA Registry for many years.

Since the first patient enrolled in June of 2019, the registry has grown quickly and now has 46 patients – roughly half LS and half SSc.

None of this could be possible without the generous funding provided by the Scleroderma Foundation and the hard work of primary investigator Dr. Kathryn Torok, co-investigators Dr. Suzanne Li, Dr. Anne Stevens, and other CARRA pediatric rheumatology researchers across North America. When the study is complete, SCORE-related data and biospecimens will become part of the CARRA Registry and open LS and SSc enrollment at all participating North American sites (67 academic centers). This will support future research to develop new treatments and improve quality of life for children with scleroderma.

If you are the parent of a child with localized scleroderma/morphea or systemic sclerosis and can be seen at one of the sites shown in the map, be sure to ask your child’s pediatric rheumatologist about enrolling in the SCORE study to improve the lives of all children living with this disease.
Registry Parent/Patient Advisory Committee

CARRA COVID-19 Statement

If you are a parent or patient and have questions about COVID-19 and/or how your participation in a CARRA-sponsored research project is impacted by the pandemic, please reach out to your local provider.

Following local guidelines, most clinics are not doing visits for research. At this time, patients enrolled in the CARRA Registry may be asked to provide information such as a patient global assessment score as part of telehealth or phone visits. Call center visits for long-term follow up are continuing as usual. Please answer calls from Duke University (919 area code) so we can collect this important information.

We are collecting information about patients enrolled in the registry who test positive for COVID-19. Please let your doctor know if you or your child test positive for COVID-19.

For answers to frequently asked questions regarding COVID-19, please visit the Arthritis Foundation’s website (https://arthritis.org/about-us/news-and-updates/coronavirus-and-arthritis-what-you-need-to-know) which is updated frequently as the situation changes. Other condition-specific patient organizations may have COVID-19 information on their website about you or your child’s diagnosis.

Registry Parent/Patient Advisory Committee

JIA mom Melanie Kohlheim chairs the RPAC, which includes 10 members who represent the conditions currently captured in the CARRA Registry. New members will be appointed to the RPAC as new conditions are added to the Registry, current members end their service, or new skills/perspectives are needed to perform RPAC activities.

“We look forward to having expert help at making the registry more patient centered and maintaining patient interest over the years. Melanie and Vincent have put together a terrific group with varying professional skills and interests that will help us continue to enhance the registry and make sure we address patient concerns.” – Laura Schanberg, Registry PI

We are so excited to welcome the patients and parents to the Registry Parent/Patient Advisory Committee, which will make the CARRA Registry even better and more relevant. The RPAC’s ideas and input are incredibly important to the Registry’s mission: what’s important to our patients is at the heart of and the reason for the research we do!” – Yuki Kimura, Registry PI

The RPAC meets monthly and develops the content for three Registry Newsletters each year. If you are a pediatric rheumatology stakeholder (parent, patient, healthcare provider, payor, industry partner, etc.) and have ideas for newsletter articles or questions/comments about the registry, please email Melanie Kohlheim (RPAC Chair) at mkohlheim@carragroup.org.
Registry Retreat

The CARRA Registry is the backbone of many CARRA research projects, including monitoring the long-term safety of medications used for childhood onset rheumatic diseases. The Registry houses identity-protected information about how childhood rheumatic diseases are treated and how they affect patients. The medical team, patients, and families provide information stored in the Registry. The data collected at clinic visits is linked to the blood samples stored in the CARRA Biobank. Studying these samples, along with the clinical data, will help answer questions about the cause of disease and how patients may respond to a given treatment.

The Registry began in July 2015 and after five short years, CARRA celebrated the 10,000th patient enrolled. The CARRA Registry is internationally recognized as a reliable source of clinical data. The hard work of stakeholders, including CARRA members, Duke Clinical Research Institute (DCRI) staff, parents, and patients, as well as funding from the industry partners and the Arthritis Foundation make possible the ongoing success of the Registry.

Always looking to improve Registry operations, CARRA hosted the 2nd annual Registry Retreat on February 23-24 in Dallas, Texas. Over 150 Registry stakeholders gathered to learn about accomplishments, current activities, and future plans. The Retreat brought together parents, patients, scientific investigators, research coordinators, and the support staff that makes the whole program run. Having all the team members gathered was a terrific opportunity to learn about what works and what needs to work better so the CARRA Registry can successfully serve the childhood rheumatic disease community.

Recapture-JIA

— Parent of child with JIA

“Over the course of my daughter’s JIA journey, we have been blessed with very long periods of medicated remission. We are grateful for those periods, but we have never opted to discontinue or reduce her medication. While we would love to give her little body a break from treatments, we don’t know much about her future. If her disease were to flare, it is still unknown whether or not we would be able to get her JIA under control again with the same medication and quickly. If families had these answers, they could better make decisions about continuing, reducing, or withdrawing medications.” — Parent of child with JIA

The Recapture JIA study is important to families because no one wants to take medications forever. There are many struggles for families when it comes to taking medicines. Sometimes doctors and families decide to stop medications when the children are doing well. However, what happens if the child flares when the medicine is stopped?

Recapture-JIA is a research project looking at children who have a flare of JIA after stopping their medicines because their disease was well controlled. The study measures how often JIA flares can be controlled within 6 months of restarting medicine. It will help doctors see which children are more or less likely to get their JIA back under control quickly. Information from this project will help children with JIA, families, and providers make better decisions about the risks and benefits of stopping medications.

Recapture-JIA uses the CARRA Registry for data collection and plans to enroll 150 children by January 30, 2021. Enrollment began November 1, 2019 and as of March 23, 2020, we had already reached 20% of our target. We anticipate significant delays due to COVID-19, but continue to enroll patients already participating in the CARRA Registry who stopped medications and suffered a disease flare. Please talk to your doctor if you think your child might qualify for this study.
Meet your RPAC Member: Kalpana Baniya

My name is Kalpana Baniya, and I'm an 18-year-old lupus patient. I am currently a freshman at Middle Tennessee State University, majoring in Biology. I was born in a refugee camp in a small country named Nepal. My family and I immigrated to the United States when I was 10 years old. As a new resident, language was a huge barrier for my family, as my parents never got the opportunity to attend school. Starting at a young age, I was committed to many responsibilities at home, as I was the only member in the household with the capability of speaking decent English.

In 2013, I was diagnosed with Lupus. My lupus journey was extra complicated because I was fighting the disease alone. My parents didn’t understand lupus. As thirteen years old, I was obligated to explain my life-threatening disease to my parents, although I didn’t understand lupus either.

During my high school years, I started getting involved in extracurricular activities because I wanted to be more than just a Lupus patient. I was a cross-country athlete and a member and officer of many different organizations. In 2018, I got the opportunity to attend a lupus advocacy summit, and that event changed my life. After that event, some of the Nepalese lupus patients reached out to me, asking me to explain to them and their families what lupus is. I was able to help them and that event shaped my perspective into something bigger and brighter. I believe that I convey a perspective of a college student and a foreign lupus patient whose family struggles with the English language and has a difficult time understanding lupus. I am willing to take every opportunity to create better changes for the lupus community. Additionally, I am now working on creating a support group for students with lupus on my campus. Finally, I am passionate about being a voice for students with lupus, an example for a minority community, and helping research.