

VOICE

SCLERODERMA

The magazine exclusively for Members
of the National Scleroderma Foundation.

SPRING 2024

MOVING MISSION FORWARD

Our strategic framework to
maximize the impact on
complex issues facing the
scleroderma community

- **MY CAUSE MY CLEATS**

Elevating Disease Awareness in Chicago through the NFL

- **ANNOUNCING THE 2024
RESEARCH GRANT RECIPIENTS**

Meet the newest class of investigators

- **ADVANCEMENTS IN CAR-T
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Revolutionizing the treatment landscape





2024 NATIONAL SCLERODERMA CONFERENCE



July 19 – 21, 2024

scleroderma.org/conference
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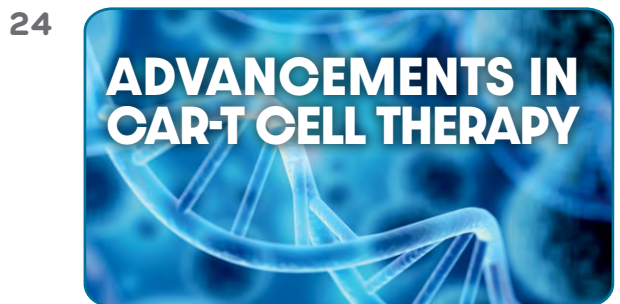


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ABOUT US

The National Scleroderma Foundation was founded in 1998 to advance medical research, promote disease awareness, and provide support and education to people with scleroderma, their families and support networks.

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A BRIGHT FUTURE AHEAD

Happy Spring everyone! As we move forward in our journey to overcome scleroderma, I wanted to share with you the sense of excitement that permeates the leadership of the National Scleroderma Foundation. This includes the volunteers who serve on our Board of Directors, Medical and Scientific Advisory Board, and our Patient Advisory Board. Our steadfast commitment to the mission, coupled with constantly renewed energy, enthusiasm, and dedication, propels us towards what is certain to be an impactful future.

Our Foundation's sense of purpose is fueled by our community's collective determination to create a brighter tomorrow for those living with this complex autoimmune disease. The Foundation has long served as a beacon of hope for those affected by scleroderma. While we all experience the challenges that people living with scleroderma face, we are unwaveringly optimistic about the strides we are making together.

We recognize the strength derived from a united front, and we are committed to fostering a sense of belonging among those affected by scleroderma. By expanding our community, and applying your insights and feedback, we are building a comprehensive network of support, deep understanding, and shared experiences that will make a significant difference in the lives of individuals facing the challenges of scleroderma.

We encourage you, our valued community members, to actively participate in this shared journey. By coming together, we can create a resilient and supportive environment that empowers each person to face their diagnosis with courage and hope.

Our commitment is not merely administrative; it is deeply personal and heartfelt. Every member of our leadership team and our network of volunteers is dedicated to making a tangible difference in the lives of those we serve. The enthusiasm of these servant leaders is contagious, and we invite you to join us in this endeavor. Whether you are a patient, caregiver, or supporter, your involvement is crucial.

Together, we can transform challenges into opportunities and realize our vision of finding a cure.

I hope you enjoy this issue, which highlights the important work of the Foundation across all facets of our mission.

A handwritten signature in black ink that reads "Kevin Boyanowski".

Kevin Boyanowski
Chair, Board of Directors



OUR FRAMEWORK FOR THE FUTURE



Mary J. Wheatley,
IOM, CAE

As the leading patient advocacy organization serving people living with scleroderma in the United States, the National Scleroderma Foundation is positioned to play a key role in addressing the challenges of the future. To that end, the Board of Directors has approved a strategic framework that enables us to focus the Foundation's efforts toward maximizing the impact we can have on complex issues facing those living with scleroderma, their families and support networks as well as the medical and scientific communities.

This framework is meant to serve our organization in an ever-changing world and provide flexibility in scope and scale, while providing a clear vision of where we want to go, and where we need to be, to best serve the scleroderma community. Rooted in our 2024 strategic framework, our vision is delineated by six pillars that encapsulate our aspirations, guiding us toward a future marked by progress and unity.

1. Increased Collaboration

Collaboration serves as the cornerstone of our framework. We are committed to fostering an environment that encourages partnerships among researchers, healthcare professionals, patient communities, and advocacy groups. By forging alliances and synergies, we aim to accelerate the pace of discovery, pooling expertise and resources to drive groundbreaking advancements in scleroderma research and care.

2. Clear and Open Communications

Clear and open communication is pivotal in fostering trust and unity. We will facilitate clear, accessible, and open channels of communication. This commitment spans interactions within our organization, with the community we serve, and among our diverse stakeholders. By fostering dialogue and transparency, we aim to build strong, enduring relationships built on trust and understanding.

3. Build on Our Reputation as a Trusted Source

In an era inundated with information, we will emerge as the beacon of trust and credibility. Our dedication to accuracy, reliability, and integrity in disseminating information underscores our commitment to becoming a trusted source for scleroderma-related resources, guidance, and support. We aspire to be the go-to authority, empowering individuals with credible information to make informed decisions about their health and well-being.

4. Create a Diverse and Unified Community

Our vision encompasses a community that celebrates diversity and embraces unity. We will create an inclusive environment that values and respects the

unique experiences, backgrounds, and perspectives of all individuals affected by scleroderma. By fostering a sense of belonging and solidarity, we aim to unite our community in a collective journey towards empowerment, support, and resilience.

5. Embody Innovation

Innovation propels progress. We are committed to embracing innovation across all facets of our endeavors, from research methodologies and treatment approaches to outreach strategies and advocacy efforts. By fostering a culture that encourages creativity, experimentation, and the adoption of innovative technologies, we strive to drive transformative change in the fight against scleroderma.

6. Build a Culture of Philanthropy

Philanthropy forms the bedrock of our mission. The Foundation aims to cultivate a culture of giving and generosity, inspiring individuals, corporations, and philanthropic entities to support our cause. Through philanthropy, we aim to expand our reach, amplify our impact, and ensure sustainable initiatives that continue to advance research, provide support, and advocate for the needs of those affected by scleroderma.

As we chart the course forward guided by these pillars, our commitment is steadfast. Together, with our community, partners, and supporters, we look forward to a future where the burden of scleroderma is alleviated, where innovation leads to breakthroughs, and where unity and collaboration pave the way toward a world free from the constraints of this disease.

A handwritten signature in black ink, appearing to read 'Mary J. Wheatley'.

HELP US PROMOTE DISEASE AWARENESS IN JUNE

The National Scleroderma Foundation offers hope to members of the scleroderma community. Whether you're a newly diagnosed individual, long-time warrior or supporting someone affected by this disease, your unique journey is important, and you're never on it alone. This year's awareness month theme is #sclerodermamatters.



Whether you're living with scleroderma or supporting someone affected by it, your story matters. This year's awareness month will highlight unique journeys from across the scleroderma community.

Request a Proclamation or Building Light Up in your area.

Each city and county will have its own guidelines and procedures for signing proclamations or securing buildings, bridges, homes, businesses, stadiums, other local points of interest, and landmarks requests for lighting it teal.

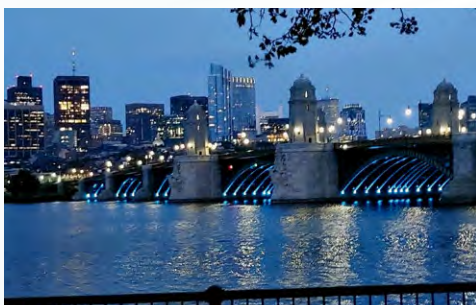
The easiest way to research your city or county guidelines is to look on its website and search for the term "proclamation" or "building light-ups." Some of the larger cities and counties will have guidelines listed. Smaller cities and counties may provide contact information on the website to call or email for information.

To expedite the process, the following information is usually required when submitting a proclamation and light up of buildings, landmarks, and points of interest. We hope to have Teal around the country.

- The purpose of the request (June Awareness Month)
- The date(s) of the day, week, or month of the event to be requested (you can request whichever you would like: June 29th Scleroderma Awareness Day or the month of June)
- A brief history of the Foundation www.scleroderma.org/about-us/
- The name, daytime telephone number, and email address of the contact person.

The wheels of the government turn slowly, so be sure to begin the request process at least two months before your event date or as soon as possible.

- Timing is key if you want to have the request announced at a city council or county commissioner meeting. Do not hesitate to follow up to check the status of your request and upon doing so, offer to provide any additional information the official may need.
- Track your successes at bit.ly/2024tealtracker.
- Make your local community aware by sharing pictures on social media. Tag the National Scleroderma Foundation, your Chapter, and add the tag #sclerodermamatters so our entire community can see how you have raised awareness or shined a light on scleroderma!



Help light up landmarks around the country or secure a proclamation during Scleroderma Awareness Month in June and on Scleroderma Awareness Day June 29th!

MY CAUSE

my cleats



Families affected by scleroderma play a unique role in raising awareness. Whether you are caring for someone living with scleroderma or living with the disease yourself, the support of your family can make the impossible feel doable. A family affected by scleroderma might take on the challenge together and show the world how to live fully with scleroderma. Emily Rooney understands the importance of disease awareness and elevates the voices of scleroderma through her platform in honor of her step/bonus mom, Pat Rooney.

OVER THE LAST 30 YEARS, PAT HAS VALIANTLY

battled scleroderma. Although her journey has been challenging, Pat never ceases to maintain a positive attitude and is a bright light in the lives of those around her. No matter the pain Pat faces, she continues to move forward with a smile on her face. According to her doctor, Pat has continued to beat the odds, and he attributes it to her positive spirit and willingness to fight.

This year, Emily had the unique opportunity to support Pat and her journey with scleroderma through Emily's role on the Chicago Bear's staff. During the Bears season, Rooney painted her Nike Air Force 1 shoes blue and teal for the Bears vs. Lions home game at Soldier Field to raise awareness for the National Scleroderma Foundation and honor Pat through the My Cause My Cleats program. The bright teal laces were the cherry on top of colorful sneakers adorned with the Foundation's signature colors.

As part of the National Football League (NFL)'s My Cause My Cleats program, the Chicago Bears ownership has allowed staff members to participate by painting and wearing shoes that represent and bring awareness to a particular cause or charity of the staff members choosing. Through Rooney's efforts, the National Scleroderma Foundation reached the fields of the NFL for the first time in the

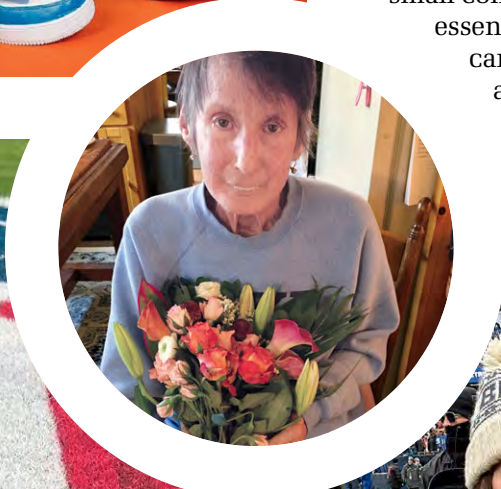
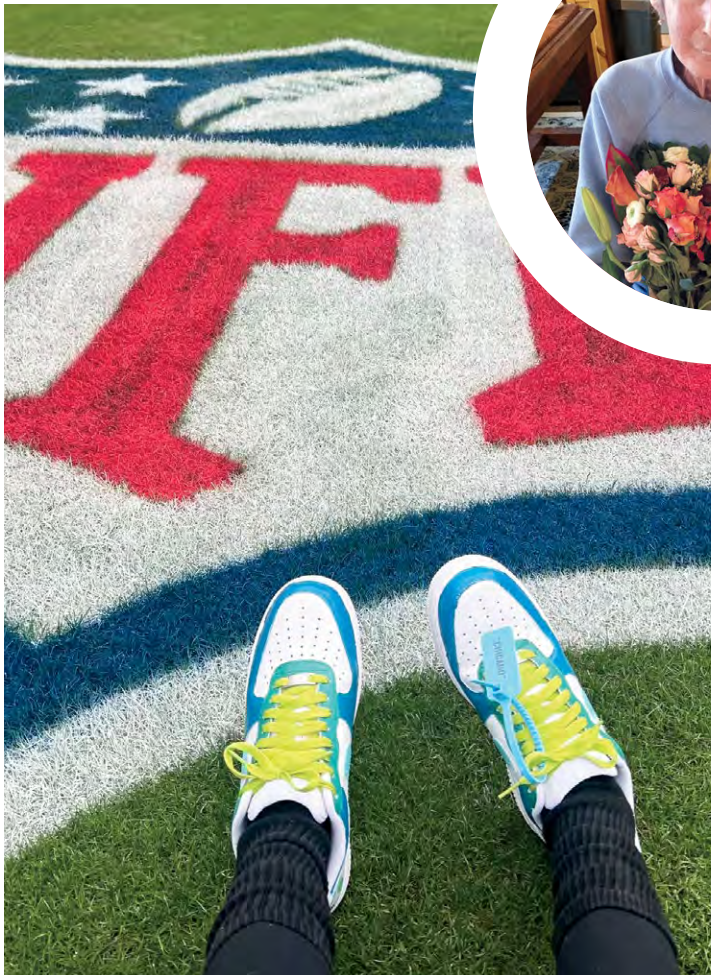




Foundation's history. For Rooney, choosing the National Scleroderma Foundation was a choice that resonated close to her heart. The teal sneakers represented a future of hope and inclusion for the Foundation.

Scleroderma awareness is vital to the National Scleroderma Foundation's work and a substantial challenge as the disease is widely unknown. For many living with scleroderma, it can feel isolating when your community does not understand your experience or disease. Each opportunity for scleroderma awareness brings us one step closer to a cure and to making the scleroderma community feel seen.

Rooney's ability to shine a light on scleroderma shows the power of raising your voice. Even small conversations can help link others to essential resources and support. Although it can feel scary to share your story, talking about your journey with scleroderma can empower others and show them they are not alone.



ARE YOU LOOKING FOR WAYS TO SHARE YOUR STORY?

Visit our website or email us at communications@scleroderma.org.



PEGGY COLLINS

MANY PEOPLE KNOW PEGGY COLLINS

as a vibrant member of our scleroderma community. Since joining the National Scleroderma Foundation in 2018, she has risen to many leadership roles within the Foundation. Her work as a leader has helped individuals nationwide find the resources and support they need from the Foundation.

Her tenacious attitude comes from her life as a devoted mother. As a single mom of three, she knows the value of working hard and prioritizing what matters. Peggy says, “I worked my butt off in jobs that weren’t my passion but were a means to an end in supporting my boys. I did everything from clerical to job scheduling for a communications company.” These skills would later make her a substantial asset to the Upper Great Lakes Chapter.

Her journey with scleroderma has not been easy. On June 5, 2012, Peggy was diagnosed with systemic sclerosis. She notes, “It’s funny how you always remember the day.” For four months, she managed her disease alone in silence to ensure her youngest son was adequately taken care of at home. Eventually, she needed to have surgery and had to address her diagnosis with her children. While her surgery helped some, it was not enough for Peggy’s busy life, and she could no longer work.

After seven years of significant challenges, it was apparent to Peggy she needed to get involved with the scleroderma community. She started as a support group leader with the Autoimmune and Scleroderma Support Group and then came to the Foundation at the end of 2018. At that time, she was clear with other leaders that she needed to bring her faith to the table and was told, “As long as she wasn’t hitting anyone over the head with it, it was fine.”

Five years later, Peggy serves as Chair of the Upper Great Lakes Chapter, which encompasses all Foundation grassroots work in Michigan, Minnesota, and Wisconsin. She has been instrumental in helping merge the former Michigan and Minnesota Chapters and bringing in the Wisconsin constituents to the Chapter. She was elected Chair of the Chapter Leadership Council last spring and has served in that

leadership role since July 1, 2023. As the Chair of the Chapter Leadership Council, she is also a member of the National Board of Directors. Peggy serves as a National Patient Advisory Board member and, most importantly, continues her work as a support group facilitator in her Chapter. She was a tremendous asset in planning and hosting the 2023 Leadership Weekend in New Orleans in October, and showed off many of her event-planning skills during that time!

When asked what the most meaningful work in the Foundation is for her, Peggy shared “Connecting with all of the patients and staff. Connections keep everything going.” Peggy spends much of her time speaking with other people living with scleroderma, and recently, someone told her that she told them “exactly what they needed to hear when they needed to hear it.” This particular friend was struggling with that part of her disease, and Peggy told her to “set a timeline for wallowing, and then she had to move forward.” That one bit of advice helped her friend so very much. It’s not just about how Peggy is helping her scleroderma friends. She says,

“I GET SO MUCH FROM THE CONNECTIONS, TOO – THE SUPPORT GOES BACK AND FORTH OVER AND OVER AGAIN.”

If Peggy could give a newly diagnosed person one piece of advice, she says it would be this: “Forget the time limit your doctor put on you. Still, so many doctors prescribe timelines. I straight up tell people to forget that. Nobody knows when you are going to die. I was given one year, and that was 11 years ago. I have a close friend who was diagnosed at 12, and she is now 42. Get rid of that timeline, live your life, and don’t be afraid of getting out there.”



FALLING IN LOVE WITH GEORGIA



IF YOU HAVEN'T HAD THE PLEASURE OF VISITING

Georgia in October, you're truly missing out on something beautiful. This is the time when nature paints its canvas with vibrant hues of red, orange, and gold, creating a breathtaking spectacle that will leave you in awe.

But there's more to October in Georgia than just stunning landscapes. It's also a time for coming together and making a difference. One remarkable event that embodies this spirit is the golf outing organized as a fundraiser by Peggy (Basile) Levengood and her family. The Basile family hosted the 11th Annual Kathleen Basile Memorial Golf Tournament on October 19, 2023, at the Hamilton Mill Country Club in Dacula, Georgia. Golfers enjoyed a day filled with the stunning beauty of this meticulously designed golf course. It comes as no surprise that it has consistently been recognized as one of Atlanta's Top 10 golf courses.

In January 2009, Peggy lost her beloved sister Kathleen Basile to scleroderma, after a long 9-year struggle with the disease. Her family has made it their mission to raise money for research to find a cure for scleroderma. Their first fundraising event started with a golf tournament eleven years ago. They have dedicated this event to honoring Kathleen and raising awareness about this disease every year since, with a brief hiatus during the pandemic years.

Kathleen was an extraordinary young woman who touched the lives of many with her love and kindness. Her family held her dear to their hearts, and she was adored by her nieces and nephews. She played an active role in her church's theater ministry and was a dedicated employee at Kroger.

Over the years, this event has proven to be a beacon of hope and unity for the scleroderma community. Since its inception in 2009, this event has raised more than \$100,000 for scleroderma research. These funds have played a crucial role in furthering research efforts, supporting patients, and spreading awareness about this debilitating disease.

Peggy and her late sister, Doreen, were instrumental in establishing the Foundation's Southeast Chapter. The entire family is dedicated to the cause, joining in to help any way they can. They organize and work the golf event, and the family's *Stepping Out to Cure Scleroderma and Hot Cakes 5k* event in the summer, too. And of course, they help secure attendees and donations for each.

Join us next fall in Georgia as we continue to honor Kathleen Basile's memory and contribute towards creating a brighter future for those affected by scleroderma.

Should you have any questions about the event, please contact us at SEchapter@scleroderma.org.

Dedicated volunteers bring patient education TO THE MID-ATLANTIC REGION

JoAnne LaPergola



THE MID-ATLANTIC CHAPTER HAS A LOT TO BE

grateful for this year, especially the volunteers and educational opportunities available to members of our community! In addition to a robust network of volunteers across the region, the chapter is grateful to be supported by dedicated healthcare professionals from our Designated Research and Treatment Centers, who are team players willing to do what is necessary to help the scleroderma community.

The Chapter presented two remote educational events with the support of the Johns Hopkins Scleroderma Center. Dr. Fredrick Wigley's presentation included information on important scleroderma symptoms and what to look for. Dr. Wigley also shared how Johns Hopkins embraces this rare disease, while Dr. Mathai shared his experience as a pulmonary specialist.



In October, the Chapter partnered with the University of Pittsburgh Medical Center to present the first in-person educational program in the region since 2019. The speakers were very generous with their time and shared exceptional presentations with a period for questions and answers as well. Dr. Robyn Domsic spoke on Raynaud's phenomenon, Dr. David Levinthal spoke on GI manifestations in systemic sclerosis, Dr. Kathryn Torok spoke on Pediatric scleroderma, Dr. Belinda Lebron spoke on Pulmonary hypertension, Dr. Kevin Gibson spoke on Interstitial Lung Disease, and Dr. Christina Padilla spoke on current research and how patients advance research. Fifty attendees enjoyed the amazing day of connection and learning with our amazing Pittsburgh medical community. Support for this event was provided by a generous bequest.



In November, Dr. Peter Merkel and his team took the stage at the University of Pennsylvania to share information regarding all areas of scleroderma and how the scleroderma community is benefiting from new oncology discoveries. The speakers included Dr. Peter Merkel, Chief Division of Rheumatology, Dr. Nora Sandorfi, Associate Prof of Clinical Medicine, Dr Chris Derk, Prof of Clinical Medicine Div of Rheumatology, Nasheed Hossain, CAR-T and Autoimmune Diseases, and finally Nikhil Jiwrajka, Oncologist Biospecimens. Forty-eight members of the scleroderma community joined us for this special day.



Our Chapter fully enjoyed our fall programming, and we are already excited for and planning our events for next year.

The Mid-Atlantic chapter works in the District of Columbia, Maryland, Virginia, Delaware, South and Central New Jersey, Pennsylvania, and West Virginia, to offer local scleroderma educational opportunities for people affected by scleroderma and fundraisers to drive our mission. Connect with us at www.scleroderma.org/MidAtlanticChapter/ and learn how you can join our team!

Cirilo DeJesus

SHARES HIS JOURNEY WITH SCLERODERMA



When Cirilo DeJesus was nine years old, his mother noticed a dark spot on his leg after he came in from playing outdoors.

“Cirilo, go take a shower,” she must have said. “But mom, I just took one!” Cirilo replied. “Well you must not have done a good job of cleaning yourself – look at that dirt on your leg! Go take another.”

Despite a second wash, the dark spot didn’t go away.

“It looked like a birthmark,” Cirilo recalled. “They thought it was cancer. Eventually, we went to a specialist who did a biopsy and that’s when they determined it was scleroderma. They told my parents I would die within a year.”

More than 50 years later, Cirilo has made the courageous decision to break his silence and share his journey with our community.

“I read an email from the Foundation about Tiffany Shank and how she was bullied growing up because of her scleroderma. I got bullied in school too. I played football but it was tough. They had different names for me. At first, I was skeptical about sharing my story and didn’t feel comfortable about it, but the thought kept bothering me every day, so I knew I had to. I know there are kids who have the same disease and I hope my story will help them feel less isolated and alone.”

After his diagnosis, Cirilo endured a lot of pain in his left leg. He suffered from strong cramps that ran from the top of his leg to his feet which caused deformities in his toes, and his leg shortened by 2-3 inches. The cold weather where he lived in Trenton, New Jersey, also exacerbated his pain. The medications doctors gave him caused strong side effects, and after a while, he told his mother that he could not take any more.

Cirilo’s difficulties with the cold ultimately led his parents to move their family to their hometown in Rincón, Puerto Rico, when he was 15 years old. Though he was hesitant about the move because he didn’t know a lot of Spanish and he would have to start school all over again, living in Puerto Rico proved to be a fresh start.

“I did everything I always wanted to do. I always wanted to work in banking and I did that. I always wanted to get married and I did that. I always wanted to have kids and I did that. I have accomplished a lot in my life and I’m fine,” Cirilo said.

Though Cirilo’s childhood began with great trauma and grief, he has lived life to the fullest. A proud husband and father of two – a 27-year-old daughter and a 26-year-old son – he relishes in the riches of family life which has helped him heal from the pain of scleroderma.

“Yes, I have rough times...but I didn’t crawl into a hole and bury myself...I tried. So now, I can help people understand that they’re not alone and that there are other people who made it through. If there are any kids living with scleroderma reading this, know it is possible that you can grow up and live a happy life. I’m 62 years old and I’m still alive. To me, I’m still alive.”



“ I WOULD CRY AT NIGHT, I REMEMBER MY MOTHER WOULD WAKE ME UP AND ASK ME WHY I WAS CRYING. ”

SPOTLIGHT ON SUPPORT:

DEBBIE CHARLTON



DEBBIE CHARLTON SERVES AS A Support Group Facilitator, and is Chair of the Texoma Chapter Advisory Council. In February 1999, Debbie was on a ski trip with her family. Her ankles and hands were swollen. She could not even ski on the bunny slope. After that trip, she knew she needed to see a doctor. None of this was normal for her, as she was an avid jogger and played volleyball with her company. In March of that year, Debbie was diagnosed with Scleroderma. She was given a year and a half for survival. Upon hearing the news, she was so scared. She had two children to take care of, a husband, and a job she loved. When she shared the news with her mother, her mother said,

“YOU MAY HAVE A DIAGNOSIS BUT SPIRITUALLY YOU ARE HEALED”

That has kept her afloat all these years, 25 years to be exact.

Debbie shared that being a support group facilitator in San Antonio is therapeutic for her to help other scleroderma warriors realize that there is hope and how to advocate for themselves for their medical care. It has also awarded her the opportunity to build a strong support network within the community, and she is a guiding force when supporting the group's members. She attributes her faith, optimism, family, and scleroderma brothers and sisters to where she is today.

For a while, she grieved about not being able to work or enjoy the activities prior to being diagnosed with scleroderma. The word modification came up frequently. She made modifications along the way. Not being able to jog anymore, she replaced that with small hikes with her daughter. Debbie enjoys gardening. While she can't carry a bag of soil, she has learned to ask for help, another modification.

Debbie is passionate about volunteering for the National Scleroderma Foundation and spreading awareness wherever she can. She often goes out head to toe in her teal Foundation gear. She does this to spread awareness, have conversations with people in her community to share information about scleroderma.

By nature, Debbie is an optimistic person, while she admits she has bad days. When that happens, she allows herself to relax, partake in self-care, gives herself grace and often puts her air pods in, goes to the backyard and dances and sings. Her neighbors love this!

Debbie and her family are celebrating her 25-year anniversary of being diagnosed with scleroderma by going to their favorite place on the river. She will fish, paddle board with her daughter, eat some hamburgers with her son, watch sports with her husband, and reflect on how far she and her family have come and continue to be grateful and modify as needed.



NATIONAL SCLERODERMA CONFERENCE AND KIDS GET SCLERODERMA, TOO!

JULY 19-21, 2024

WE ARE PROUD TO ANNOUNCE THAT

the 2024 National Scleroderma conference and the Kids Get Scleroderma, Too! conference will be held in Seattle, Washington. This year's conferences will be fully in-person and held July 19-21 at the Hyatt Regency in Bellevue, on Seattle's Eastside. Whether you are newly diagnosed or have been living with scleroderma for many years, the adult and KGS2! conferences are a great way to learn about the disease and become connected with others who are living with the same experiences.

The conferences will include scleroderma focused sessions and panel discussions on topics such as Lung involvement, nutrition, understanding clinical trials, living your best life with a chronic disease, Morphea, managing ulcerations, juvenile scleroderma research updates and much more. These topics will be presented by world renowned scleroderma experts such as Dr. Virginia Steen, Dr. Dinesh Khanna, Dr. Maureen Mayes, Dr. Tracy Frech and many others. We typically offer 50 plus educational sessions so there will be something for everyone. Don't miss your chance to engage with scleroderma experts and view these sessions live.

The Kid Get Scleroderma, Too! conference is geared toward kids and teens ages 5-17 who are living with scleroderma and their families/caregivers. This event is the ideal place for your child to meet others affected by scleroderma and make long-lasting friendships. You and your family will also have the opportunity to learn about current therapies from top pediatric rheumatologists and hear from young adults that have navigated juvenile scleroderma. Registration for the Kids Get Scleroderma, Too! conference is now live and can be found on our website at scleroderma.org/kids-get-scleroderma-too-conference/. Don't miss out on this fun and educational event for the entire family.

Education sessions and great connections aren't the only things that this event offers. As always, we will open the conference with the research poster hall. The research poster hall is the best way to see what new and exciting research is coming down the pipeline. You can even ask your questions to the researchers themselves between the hours of 4-7pm on Friday, July 19. While you're exploring the halls and making new friends, don't forget to stop by our Exhibit Hall to meet our sponsors and engage with partner organizations. We will also have a National Scleroderma Foundation booth where you can meet up with your friends and learn all about upcoming events and how to get involved with the Foundation.

We understand that cost can be an issue, so we are offering a scholarship to individuals in need of financial assistance. The scholarship awards cover the partial or full cost of travel, lodging and registration for the event. We want to ensure that as many people as possible will have access to these high-quality educational resources and networking opportunities. The Scholarship application can be downloaded from the conference webpages at scleroderma.org/national-conference/. All completed scholarship applications must be submitted to scholarships@scleroderma.org by the deadline, March 20, 2024.

Registration is now open, so you can register for the National Scleroderma Conference at scleroderma.org/national-conference/. Additional information on hotel room rates, transportation and the scholarship program can be found on our website Scleroderma.org. We hope to see you July 19-21, for this year's National Scleroderma Conference in Seattle Washington. Please email info@scleroderma.org or call 800-722-4673 with any questions.



NAVIGATING GI ISSUES IN SCLERODERMA



Tracy Frech, MD, MS



People living with systemic sclerosis may experience abnormalities in the digestive system and gastrointestinal (GI) tract. The overproduction of collagen, a hallmark of scleroderma, can lead to thickening and fibrosis (scarring) of tissues. This process can weaken muscles, resulting in abnormally slow movement of food (dysmotility) during digestion.

The journey of food from the mouth to the stomach involves the esophagus, a tube responsible for this transport. Normally, the lower esophageal sphincter acts as a gate, allowing food to enter the stomach and promptly closing to prevent reflux. However, in systemic scleroderma, this gate may not close properly, leading to acid backwash and a burning sensation (heartburn) as food and acid return to the esophagus. The acid can cause scarring and narrowing (stricture) of the lower esophagus.

The Connection Between Scleroderma and Gut Health

Scleroderma involves vasculopathy, affecting blood flow, and GI issues may result from decreased blood supply to the nerves essential for bowel motility. Additionally, emerging evidence suggests that the balance of “good bacteria” in the gut plays a crucial role in regulating immunity.

Screening for Gastrointestinal Involvement

Healthcare providers actively screen for vascular, fibrotic, and scarring skin and internal organ manifestations in individuals with systemic scleroderma regardless of autoantibody subtype. Determining disease duration and tailoring screening approaches to each patient is essential, particularly regarding GI tract symptoms. The GI tract is the most commonly affected internal organ, presenting in 10% of patients initially and nearly all patients over time.

Managing Common Gastrointestinal Issues

Addressing acid reflux and heartburn in scleroderma may involve lifestyle adjustments. Avoiding alcohol, greasy/fatty foods, spicy foods, chocolate, tobacco, and

caffeine can be beneficial. Antacids, both liquid and tablet forms, can neutralize acids and reduce heartburn, but consultation with a healthcare provider is crucial due to potential side effects.

Physicians may prescribe medications like proton pump inhibitors, H-2 blockers, or metoclopramide to manage acid production and improve motility. Gravity-assisted measures, such as maintaining an upright position after meals and elevating the head of the bed, can help prevent reflux. Weight management and avoiding tight-fitting garments are also recommended.

Addressing Specific Gastrointestinal Challenges

Abnormal motility and esophageal narrowing may cause swallowing difficulties. Eating slowly, chewing thoroughly, consuming softer foods, and avoiding foods prone to sticking in the throat can aid digestion. For significant esophageal narrowing, periodic dilation may be necessary.

Damage to the small bowel muscles in scleroderma can lead to ineffective food movement, bacterial overgrowth, diarrhea, and malabsorption. Treatment options may include antibiotics, fat-soluble vitamin supplements, iron supplements, and dietary adjustments.

In the colon, weak or scarred muscles can result in constipation. Maintaining a high-fiber diet, adequate fluid intake, and regular exercise can help prevent constipation. Stool softeners and bulking agents may be recommended by healthcare providers.

Ongoing Research

Continued research is essential for gaining a deeper understanding of the complexities of scleroderma and its impact on the gastrointestinal system. Ongoing investigations aim to identify new therapeutic strategies and improve the overall management of GI complications associated with systemic scleroderma.



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2024 KIDS GET SCLERODERMA TOO! CONFERENCE

July 19 – 21, 2024



scleroderma.org/KGS2

info@scleroderma.org or (800) 722-4673

The power of CONNECTION

Mary Alore

HAVE YOU EVER BEEN IN A ROOM FULL OF PEOPLE AND STILL FELT ALONE? I KNOW I HAVE.

In 2011, I felt highly isolated, scared, and alone after being diagnosed with Diffuse Systemic Sclerosis, scleroderma.

Before my diagnosis, I visited many healthcare providers who didn't know what was wrong and said, "I'm sorry, but we can't help you."

After diagnosis, I encountered family, friends, and others who couldn't relate to what I was going through and said, "But you don't look sick."

In search of support, I attended an autoimmune support group meeting. Sitting in a large room with round tables full of people, I thought, "Wow, I'm not alone after all." I sat at a table, introduced myself, and explained that I had recently been diagnosed with scleroderma. I soon learned nobody at my table or in the room had scleroderma. The rarity, isolation, and unknown of scleroderma hit me hard.

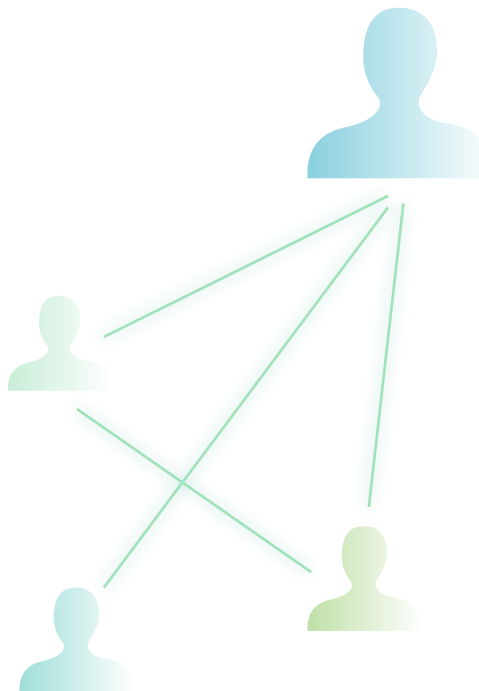
At my appointment with Dr. Dinesh Khanna, my rheumatologist, I shared my experience with the support group. He offered to connect me with other patients seeking similar support.

He connected me with Anita Devine and Sheri Hicks, which collectively transformed our experiences from lonely and isolated to connected and understood. We offered each other real-life experiences and provided the support missing from family, friends, physicians, or therapists.



That one connection was the catalyst for starting the Michigan Medicine Scleroderma Peer Mentoring program.

The Peer Mentoring Program provides support to people living with scleroderma, particularly those who are newly diagnosed. Trained Peer Mentors help bridge the gap between patients and healthcare providers by facilitating connections and support for both sides.



Pictured left to right - Mary Wheatley, Dr. Puja Khanna, Mary Alore, Dr. Janet Poole, Dr. Susan Murphy, and Dr. Yen Chen.

Mentors listen to patients and caregivers to:

- Provide information, guidance, and emotional support.
- Help patients confront challenging issues and emotions.
- Encourage patients to partner in their care.

In addition to Peer Mentoring, some mentors work closely with researchers at Michigan Medicine. A few Peer Mentors participated in the Resilience-Building Energy Management Program (called RENEW) research study as Peer Health Coaches. Peer Health Coaches led participants through goal-focused health-related learning modules and provided guidance and social support.

The results of the RENEW study were terrific. After 12 weeks, study participants reported less fatigue, pain interference, depressive symptoms, and increased resilience.¹

Participants greatly valued peer health coaching. Connecting with someone living with scleroderma and understanding the challenges of the disease fostered a sense of belonging. Study participants felt like they were in the “same boat” and found this support irreplaceable. Additionally, they appreciated emotional and practical support, helping them cope with isolation.

Whether from personal experience or research findings, the results are clear: there is power in connection. Peer Mentors offer valuable connections and practical support for those navigating the scleroderma journey.

The power of a single connection can create a ripple effect of change that impacts lives today and tomorrow. Let us embrace the power of connection to support each other, especially when navigating challenges in our everyday lives, such as living with scleroderma.

¹ Murphy S, Chen Y, Alore M, Hicks S, Pape A, Hassett A, Kratz A, Whibley D, Harper A, Huang S, Jay G, Bolde S, Khanna D. 2024. Effects of a resilience-building energy management program on fatigue and other symptoms in systemic sclerosis: a randomized controlled trial. *Arthritis Care Res.* <https://doi.org/10.1002/acr.25253>

FUNDING INNOVATIVE, PEER-REVIEWED RESEARCH

SINCE ITS FOUNDING, IN 1998, THE National Scleroderma Foundation has committed more than \$33 million to advancing medical research in scleroderma. Continued research is critical to accelerating scientific discovery, developing new treatments, providing hope to individuals living with the disease and ultimately finding a cure for scleroderma.

This year, the Foundation is proud to announce 2024 funding for seven grant awards to new and established investigators, totaling \$1.4 million.

The Foundation's peer-reviewed research grants program prioritizes scientific merit and provides funding for both early career and established investigators. The program is administered by the Foundation's Research Committee. The committee makes funding recommendations to the Board of Directors annually after a rigorous peer review process.

The review panel is composed of a team of highly respected scientific experts who carefully review, critique, and score all applications based on the National Institutes of Health's guidelines and ranking system. Only those proposals of the highest scientific and technical merit are recommended for funding.

Once awarded a grant from the Foundation, recipients must submit annual reports on their progress. All reports are reviewed by the Foundation's Research Committee to ensure compliance with programmatic, scientific, and fiscal and administrative policies and requirements.

This spring, seven researchers will begin work on their Foundation-funded projects. For this round of funding, the Foundation received 26 proposals from investigators representing 18 institutions. All funded projects were rated as "high impact" by reviewers, and the success rate for FY 2024 applicants was 28% (compared to 29% last year).

The FY 2024 portfolio includes three New Investigator Awards and four Established Investigator Awards, and a good mix between basic and translational focused science. The portfolio also includes research focused on the pediatric population.

Did you know you can designate your gift to the Foundation to research by calling us today at 1-800-722-HOPE!

Announcing

OUR NEWEST CLASS OF INVESTIGATORS!



Ramadan Ali, PhD, University of Michigan

Mechanistic connections between NETs and scleroderma vasculopathy

- Mark Flapan New Investigator Award



Brendon Baker, PhD, University of Michigan

Metabolic and epigenetic targeting of stromal cell-matrix crosstalk for reversing fibrosis in scleroderma

- Debra Lurvey Memorial Research Grant
- Established Investigator Award



Laura Polivka, MD, PhD, Cedars-Sinai Medical Center (Los Angeles)

Exploring the pathogenesis of severe juvenile systemic sclerosis

- New Investigator Award — This grant is supported in part by the Marta Marx Fund for the Eradication of Scleroderma



Maria Teves, PhD, Virginia Commonwealth University

Advancing scleroderma treatment: a study using primary cilia as a therapeutic target

- Walter & Marie Coyle Research Grant
- Established Investigator Award



Md Nurunnabi, PhD, The University of Texas at El Paso

Investigating nanoparticle enabled cell specific apoptosis induction and fibrosis treatment

- New Investigator Award



Mohammed Osman, MD, PhD, University of Alberta

Evaluating a role for polysialic acid as a diagnostic, prognostic and therapeutic target in systemic sclerosis

- Established Investigator Award



Reshmi Parameswaran, MS, PhD, Case Western Reserve University

BAFF CAR-T treatment for systemic sclerosis

- Established Investigator Award

Congratulations to our newest funded investigators! You can learn more about these projects at scleroderma.org/research.

HIGH INTENSITY IMMUNOSUPPRESSION IN JUVENILE SYSTEMIC SCLERODERMA:

BALANCING RISK AND REWARD

Ivan Foeldvari, MD



THIS WINTER, I HAD THE PLEASURE OF

hosting the 16th Hamburg Symposium on Juvenile Scleroderma in Hamburg, Germany. Thanks to generous support from the National Scleroderma Foundation and the Scleroderma Clinical Trial Consortium, this international multidisciplinary

workshop brought together

pediatric and adult rheumatologists, dermatologists, pulmonologists, cardiologists, and transplant specialists with a research focus in systemic scleroderma, as well as representatives from the patient community.

Juvenile systemic sclerosis (jSSc) is a rare condition that affects approximately 3 in every 1,000,000 children and presents significant challenges in terms of management and therapy. The primary focus of this workshop was to determine which jSSc patients might benefit from autologous haemopoietic stem cell transplant (AHSCT) or cellular therapies, given the complexities of the disease and the evolving landscape of treatment options.

Key presentations at the symposium provided insights into current approaches and ongoing research in this field. The symposium also delved into organ-

specific considerations for AHSCT in jSSc, with working groups focusing on skin involvement, musculoskeletal issues, interstitial lung disease, cardiac complications, digital vasculopathy, renal involvement, and gastrointestinal problems. Experts discussed the challenges of defining prognostic factors for severe disease and determining appropriate indications for AHSCT in different organ systems.

At the end of the workshop, we held a consensus meeting with all participating experts and patient representatives. The aim of the consensus meeting was to develop guidance for HSCT and Cellular Therapies for patients with jSSc. In the consensus meeting we discussed in which extent the adult data and the criteria can be extrapolated to juvenile patients and its limitations. We formulated a guidance regarding HSCT and CT in jSSc patients, based on nominal group technique, which will be submitted for publication shortly.

This guidance will help to standardize the inclusion criteria worldwide and make the results of future procedures more comparable. We hope that HSCT and CT will have standardized exclusion criteria and transplant protocols to enable data collection, interpretation and improve outcomes and care of all jSSc patients.

Overall, the symposium highlighted the complexities of treating jSSc and the need for personalized approaches that balance the potential benefits of high-intensity immunosuppression with the associated risks. By collaborating across disciplines and leveraging ongoing research efforts, the hope is to advance towards more effective and standardized treatments for this challenging condition.

Visit www.scleroderma.org/pediatric/ to learn more about pediatric scleroderma. You can learn more about Dr. Foeldvari's work at www.juvenile-scleroderma.com.

¹. Foeldvari I, Torok K, Anton Lopez J, et al. Best clinical practice in the treatment of juvenile systemic sclerosis: expert panel guidance - The result of the International Hamburg Consensus Meeting December 2022. Expert Review of Clinical Immunology. 2024.

Announcing

THE NEERJA MARWAHA BHAGAT RESEARCH SCHOLAR FELLOWSHIP



THE NEERJA MARWAHA BHAGAT RESEARCH

Scholar Fellowship was established earlier this year to honor the legacy of a longtime leader of the National Scleroderma Foundation Mid-Atlantic Chapter.

Neerja Marwaha Bhagat served as Chair of the Chapter for more than a decade and was a tireless advocate for people living with scleroderma. She led the planning of the chapter's *Stepping Out to Cure Scleroderma* walks and helped build much-needed awareness about scleroderma in her community.


“BY ESTABLISHING THIS FELLOWSHIP, OUR HOPE IS THAT IMPORTANT RESEARCH IN SCLERODERMA WILL BE STIMULATED BY BRIGHT NEW MINDS IN THE FIELD.”

shared Dr. Marwaha. “Our family is honored to partner with the National Scleroderma Foundation to support research scholars interested in pursuing novel ideas in scleroderma.”

Neerja’s legacy lives on through her many friends and her extended family, including her husband Dr. Pradip Bhagat, her two children, Deepak and Nishi, her two brothers, Dr. Vijay Marwaha & Dr. Ajay Marwaha and close family including Drs. Subhash & Hedwig Marwaha and Andrew and Raj.

Each year, one scholar will be honored as the Neerja Marwaha Bhagat Research Scholar in recognition of their drive to discover the cause and cure of scleroderma.

LEARN MORE ABOUT THE FELLOWSHIP AT [SCLERODERMA.ORG/RESEARCH](https://scleroderma.org/research).



ADVANCEMENTS IN CAR-T CELL THERAPY

An Exciting Frontier in Scleroderma

Systemic sclerosis or scleroderma, a complex autoimmune disorder characterized by fibrosis and vascular abnormalities, poses significant challenges in terms of effective treatment. The current standard of care for scleroderma primarily involves immunosuppression, which can impair immune response and lead to increased infections and other serious health risks. There are currently no approved therapies to treat the overall disease. However, in the last decade, several cell-based therapies have been used to treat autoimmune diseases, including scleroderma. There is growing excitement about the potential of cutting-edge therapies, such as Chimeric Antigen Receptor T-cell (CAR-T cell) therapy, in revolutionizing the treatment landscape for scleroderma.

UNDERSTANDING CAR-T CELL THERAPY

CAR-T cell therapy is a groundbreaking immunotherapy that harnesses the power of a patient's own immune cells to target and combat specific abnormalities in the body. In the context of scleroderma, this therapy aims to address the dysregulated immune response and fibrotic processes characteristic of the disease.

HOW CAR-T CELL THERAPY WORKS

CAR-T cell therapy begins with the extraction of T cells from the patient's own blood. T cells are a type of immune cell that plays a crucial role in orchestrating the body's defense mechanisms. The collected T cells are then genetically modified to express chimeric antigen receptors (CARs) on their surface. These CARs are designed to recognize specific antigens associated

with scleroderma, creating a targeted and personalized treatment approach.

Modified T cells are cultured and expanded in the laboratory to increase their numbers significantly. This step ensures an ample supply of engineered T cells with enhanced targeting capabilities. The expanded and modified CAR-T cells are then infused back into the patient. Once in the bloodstream, these engineered cells seek out and destroy cells displaying the targeted antigens, which, in the case of scleroderma, may include those involved in the fibrotic and autoimmune processes.

POTENTIAL BENEFITS OF CAR-T CELL THERAPY IN SCLERODERMA

CAR-T cell therapy offers a highly specific and targeted approach to treating scleroderma by focusing on the antigens associated with the disease. This precision minimizes collateral damage to healthy cells, reducing the risk of side effects.

By reprogramming the patient's T cells, CAR-T therapy aims to modulate the immune response, addressing the underlying autoimmune component of scleroderma. This modulation may help restore immune balance and mitigate the fibrotic processes associated with the disease.

This therapy allows each person's T cells to be genetically modified based on their unique disease profile, making CAR-T cell therapy a personalized and tailored treatment strategy. This individualized approach holds promise for improved efficacy and reduced adverse effects.



Dinesh Khanna,
MD, MSc



Monalisa Ghosh, MD

Current CAR-T treatments are targeting B cells in people with B-cell cancers, but future targets may include other antigens involved in scleroderma and other autoimmune diseases.

POTENTIAL SIDE EFFECTS OF CAR-T

CAR-T involves upfront intense immunosuppression therapy that can lower the immune response to infections. The body can have an intense inflammatory response to the CAR-T products that may be associated with cytokine release syndrome with fever, difficulty breathing that may require close follow up to aggressive treatment. In addition, CAR-T can rarely also cause neurological symptoms of confusion, headaches, and weakness. In addition, giving T cells back that are activated can potentially cause flare of the disease, although it has not yet been seen in the few patients treated outside US.

WHO IS ELIGIBLE FOR CAR-T

Like stem cell transplant, CAR-T therapy is designed to target early scleroderma where inflammation/ autoimmunity predominates the disease. Patients that are candidates for CAR-T include those with disease duration within 7 years and progressive skin disease or internal organ involvement who are not responding to traditional immunosuppressive therapy such as mycophenolate mofetil, methotrexate, and other therapies. It is unknown if CAR-T therapy will be effective in those with long standing disease where fibrosis predominates in scleroderma. Future CAR-T therapies may target those with fibrosis, but current trials are focused on early scleroderma. More information is available at clinicaltrials.gov and through the National Scleroderma Foundation.

CHALLENGES AND FUTURE DIRECTIONS

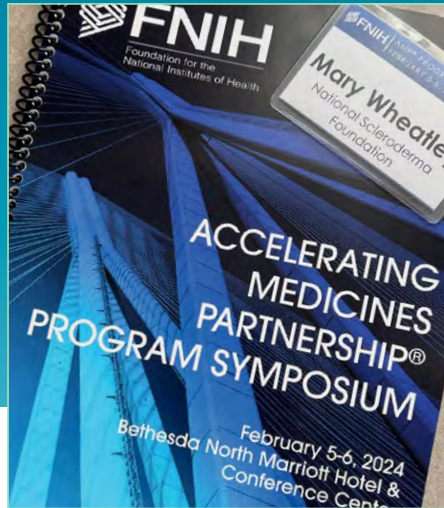
While CAR-T cell therapy shows immense potential, challenges remain. The complexity of scleroderma and the need for a deeper understanding of the disease's underlying mechanisms pose obstacles to the widespread implementation of this therapy.

Ongoing research is essential to refine CAR-T cell therapy for scleroderma, optimize treatment protocols, and identify the most effective antigen targets. Collaborative efforts between clinicians, researchers, and pharmaceutical companies are crucial in advancing this promising frontier in scleroderma treatment.

CAR-T cell therapy is certainly a promising treatment, offering a novel and targeted approach to address the underlying causes of scleroderma. As research progresses and clinical trials unfold, the potential for CAR-T cell therapy to transform the lives of individuals living with scleroderma becomes increasingly tangible. With ongoing advancements, this groundbreaking therapy may emerge as a game-changer in the quest for more effective and personalized treatments for scleroderma.

ACCELERATING MEDICINES PARTNERSHIP CELEBRATES 10 YEARS

Mary Wheatley, IOM, CAE



TEN YEARS AGO, THE FOUNDATION

for the NIH (FNIH) announced the inception of the Accelerating Medicines Partnership (AMP). To celebrate ten years of progress, the FNIH hosted a symposium in Bethesda in February.

Since its inception in 2014, the AMP program has expanded from its initial three programs in RA/Lupus, Type 2 Diabetes and Alzheimer's, to nearly a dozen programs, all with robust data sets and insights relevant to people living with these diseases in the real world.

The initial programs set out to dissect the molecular basis of these diseases. The rationale was that by “deconstructing” these diseases, and studying them at a cellular level, we would learn more about the pathogenesis of each, including the distinctions and similarities.

In the Autoimmune and Immune-Mediated (AIM) program, several discoveries were made that are impacting patient care today. The AMP also marked an inflection point in biomedical research, where the use of animal models has evolved from trying to model human disease, to now using humans (tissue, cells, data) to understand disease and using animal model to test therapeutic effect against a target. It is important to note this is thanks in large part to those who so generously donated samples to the AMP projects.

During a panel on the impact of the programs over the last ten years, Dr. Lindsey Criswell summarized it well,

“These programs impacted how we do science and treat patients.” She also noted, importantly, that these projects could lead to a molecular taxonomy of disease. This, in stark contrast to the siloed disease management of today, which is situated by clinical presentation or system (e.g., cardiac or pulmonary disease).

The Symposium also hosted a session on AMP Systems Biology of Inflammation. This program is leveraging large datasets to identify shared pathways across diseases. This approach will continue to support data sharing and team science and help us learn more about the similarities across diseases.

There is tremendous unmet need in the area of autoimmune disease. While there have been many therapies approved in recent years, by and large, they treat the symptoms, not the underlying conditions. They also affect each person differently, given we all have our unique biology and genetic makeup.

As science progresses within each of these areas, and across the AMPs, academia and industry, the possibilities are truly awe-inspiring. Opportunities abound, chief among them expanding recruitment efforts to be more representative of those who are living with these diseases and will ultimately benefit from the therapies developed.

National Scleroderma Foundation is a proud partner of the Foundation for the National Institutes of Health and we were delighted to sponsor this meeting.

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Our vision is to be a relentless force in finding a cure for scleroderma. Thank you for your partnership in helping advance our shared mission and provide critical support for people living with scleroderma in the US.