SCLERODERMA VOICE

The magazine exclusively for Members of the National Scleroderma Foundation.

SUMMER 2023

• 25 YEARS OF INNOVATIVE RESEARCH IN SCLERODERMA

Committing $30.8 Million dollars since 1998.

• GETTING TO THE HEART OF IT ALL

From grantee to mentor – shepherding the next generation of researchers.

• LINKING THE GLOBAL COMMUNITY IN REAL-TIME

Amidst COVID-19 hardship, the scleroderma research community joins to share new information and insights.

You could change the future of systemic sclerosis research.

Rare Disease Research Study: Systemic Sclerosis

23andMe has launched a study with the ultimate goal of improving the lives of people living with systemic sclerosis and you may be able to help. Your genetic information and insights may lead researchers to future treatment discoveries.

How it works

Participation in the study is simple and can be done entirely from home. If you are eligible and choose to enroll, you will receive a 23andMe Health + Ancestry kit at no cost, provide a saliva sample, and be invited to answer online survey questions.

Get started

It's time to push systemic sclerosis research forward, together. To learn more about our study, eligibility requirements and how you can help, you can visit our website.

https://www.23andme.com/systemic-sclerosis-NSF

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SCLERODERMA VOICE

25th ANNIVERSARY National Scleroderma Foundation

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MERCK

The magazine exclusively for members of the National Scleroderma Foundation.
This resource was made possible in part through an independent grant from Merck & Co., Inc.

ABOUT US

HISTORY: 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.

CHANGE OF ADDRESS: To ensure timely delivery, please email development@scleroderma.org with the subject line "Change of Address." You may also call us toll-free at 800-722-4673 or write us at 300 Rosewood Drive, Suite 105, Danvers, MA 01923.

ADVERTISING DISCLAIMER: The Foundation in no way endorses any drugs or treatments reported in this magazine. Any references to products, services, treatments or health care providers in this magazine are not a recommendation or endorsement of products, services, treatments, providers or treatment centers. Information is provided to keep readers informed. Because the manifestations and severity of scleroderma vary among individuals, personalized medical management is essential. Therefore, it is strongly recommended that all care options, including but not limited to, all drugs, treatments and/or products, be discussed with the reader's physician(s) for proper evaluation and treatment.

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DOING OUR PART TO ADVANCE RESEARCH

I've always been fascinated by research... not only is it necessary for us to understand what works, and what doesn't, but it is research that leads to new discoveries. Without research, my wife Mariann wouldn't be able to take our daily walks or play with our granddaughter. Because of research, her doctors have prescribed a regimen that works for us to improve her quality of life.

The National Scleroderma Foundation has always had a commitment to advancing medical research. Since 1998, the Foundation has been the leading nonprofit funder of peer-reviewed research to discover the cause, understand the mechanisms, and overcome scleroderma forever.

As we look back over the last 25 years, it's exciting to consider the impact of some of the work we've funded. Some of the earliest work we supported in the late nineties laid the groundwork for the therapies of today. This includes work by some of the best and brightest scleroderma researchers of our time, including Arnold Postlethwaite, Paul Utz, Virginia Steen, Thomas Medsger, Richard Silver, John Varga, Peter Merkel, Maria Trojanowska, Dinesh Khanna, and many more.

Thanks to the passion and dedication of decades of volunteer leaders, scientists and supporters over the last 25 years, great strides have been made. Yet despite these advancements, the challenges in scleroderma today are greater than ever, requiring each of us to do our part to help.

I consider it a privilege to participate in clinical research. I regularly check the "yes" box when asked if willing to participate. To date, I've given blood, filled out questionnaires, and sat on numerous advisory panels. I know that my participation can help lead to powerful solutions for a healthier future for all of us, whether it's developing a new vaccine or treatment or measuring the efficacy of an existing drug or intervention.

Individual participation in research is not only critical, it's exciting to play an active role in something that for years has been somewhat shrouded in mystery. As medicine becomes more personalized, it's increasingly important that we continue to build a pool of interested participants. Our scleroderma community has always been such a wonderful source of inspiration to me in that way. Whenever I share what we're supporting in our research portfolio, or what exciting projects our corporate partners are working on, almost always, the next question is: How can I help?

And I'm glad to say that we're making it easier than ever for you to help by participating in research yourself. We're excited to launch a new partnership with Carebox this summer. This will allow us to provide a more user-friendly, patient-centric, clinical trial engagement platform to empower families to find and quickly access new therapies in development found only in clinical trials. We hope this will make it easier than ever for you and your loved ones to find and match with scleroderma related clinical trials.

Visit scleroderma.org/research today to learn more.

Kevin Boyanowski
Chair, Board of Directors

OUR COMMITMENT TO RESEARCH IS *Unwavering*

Mary J. Wheatley, IOM, CAE

For the past 25 years, the National Scleroderma Foundation has been a leading supporter of scleroderma research. Our research program has made significant progress in understanding the causes and mechanisms of scleroderma, and in developing new treatments. Some of the key research areas that the Foundation has supported include:

- **Understanding the genetics of scleroderma:** Foundation supported research looking at the genes that are involved in scleroderma has led to a better understanding of the disease and has identified new targets for treatment.
- **Improving the diagnosis and care of scleroderma:** Projects focused on the development of new diagnostic tools and guidelines for scleroderma have a direct impact on clinical practice and care. This work may lead to shorter diagnostic journeys for people living with scleroderma.
- **Developing new treatments for scleroderma:** Many of our projects have identified novel therapeutic targets that may lead to the development of new drugs and other therapies for scleroderma.

The Foundation's research program has made a significant impact on the lives of people affected by scleroderma. Through its research programs, the Foundation is helping to improve our understanding of the disease, develop new treatments, and improve the diagnosis and care of people living with scleroderma. By sharing the outcomes of Foundation funded work at meetings and in publications, our research community is expanding the medical knowledge and literature on scleroderma in the US.

We are unwavering in our commitment to continued support of scleroderma research. With each new project, we are one step closer to realizing our vision of finding a cure for scleroderma and improving the lives of people affected by the disease.

Since 1998, you have helped the National Scleroderma Foundation create a legacy of impact in scleroderma research that continues to pay dividends to those impacted by this disease. I hope you enjoy learning more about the work you're helping to support in this issue of the magazine. I can't wait to see what happens in our next 25 years.

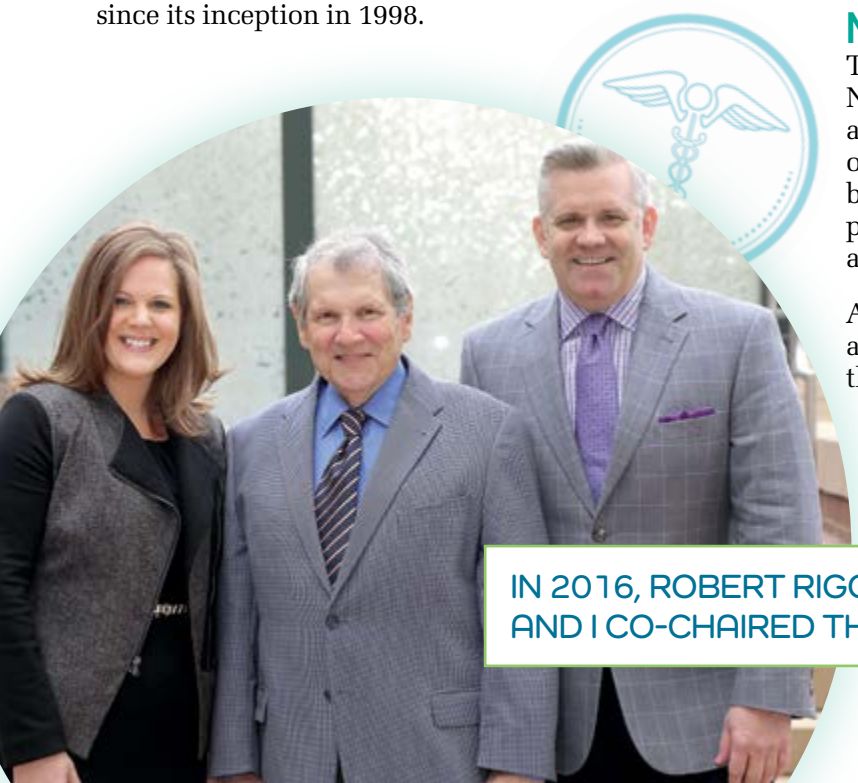
The National Scleroderma Foundation is a non-profit organization that relies on the support of individuals and organizations to fund its research program. If you would like to help support scleroderma research, you can make a donation online at scleroderma.org/donate or by calling 1-800-722-3777. Your donation will help to fund important research that may lead to new treatments and a better understanding of scleroderma. Thank you for your support.

25 years of INNOVATIVE RESEARCH IN SCLERODERMA

IN 1998, THE FOUNDATION FUNDED

its first grants. Ten grants at $50,000 each were supported that year for a total commitment of $500,000. Drs. Arnold Postlethwaite, John Varga, Paul Utz, and Virginia Steen were among that initial cohort of scleroderma investigators supported by the Foundation's peer-reviewed research grants program. Their work continues to shape the landscape of scleroderma research globally.

Since that time, the portfolio has grown to support more than 170 investigators at 78 institutions all over the world. The Foundation has committed $30.8 Million to its peer-reviewed research grants program since its inception in 1998.



Nearly a decade later, in 2007, scleroderma gained national visibility as the condition became eligible for Department of Defense research funding due to a Congressional Act that noted the impact of scleroderma on members of the military.

The Foundation's first Early Career Investigator Conference was held in 2013 and continues today. This program fosters the next generation of scleroderma researchers and provides career mentoring in a supportive environment. Along with their peers and colleagues, a committed group of scleroderma experts provide real-time feedback on everything from project design to sample size to data analysis.

NIAMS COALITION

The Foundation is a longstanding member of the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) Coalition. Member organizations raise awareness about research into the basic understanding, causes, incidence, treatment, and prevention of diseases of the bones, joints, muscles, and skin.

As such, we are key partners of the NIAMS and serve as the voices of the patients and professionals for whom the institute works.

IN 2016, ROBERT RIGGS (FORMER CEO OF THE FOUNDATION) AND I CO-CHAIRED THE COALITION TOGETHER.

In 2020, thanks to our passionate community of advocates, the DoD Congressionally Directed Medical Research Program designated $5 million for scleroderma research. With this new program, the Department of Defense made a commitment and patient perspectives on the treatments used at the time. You can read about the research supported under this program at cdmrp.health.mil/srp/awards/awards.

PATIENT-FOCUSED DRUG DEVELOPMENT MEETING

In 2020, the FDA Center for Drug Evaluation and Research (CDER) hosted a Virtual Public Meeting on Patient-Focused Drug Development for Systemic Sclerosis. Approximately 250 people attended the virtual meeting, including representatives from the Foundation.

The patient input generated through this meeting was meant to strengthen FDA's understanding of the daily burden of systemic sclerosis and patient perspectives on the treatments currently used to treat it. Participants highlighted the physical, emotional, and social toll systemic sclerosis takes on daily life, emphasizing the need for new treatment options. Several key themes emerged, including the broad range of symptoms experienced; the physical impact on activities of daily living; and experience with different medicines. This input may help companies who develop drugs understand how to measure those aspects of systemic sclerosis that are important to patients.

In 2021, despite economic uncertainty felt around the globe, the Foundation's board of directors made a commitment to double its research funding, which attracted a record number of applications. Funding was approved in total of $2.4 Million for research grants that cycle, including for projects focused on pediatric scleroderma and lung involvement.

(continued on page 22)

25 YEARS OF RESEARCH FUNDING

BY THE NUMBERS

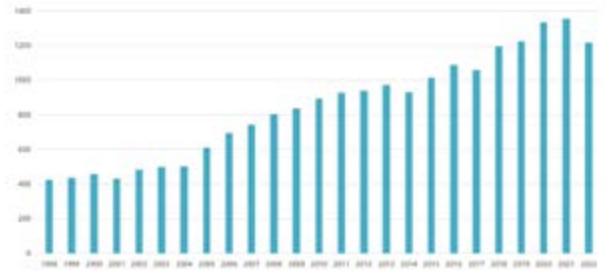
171
Investigators

315%
ROI

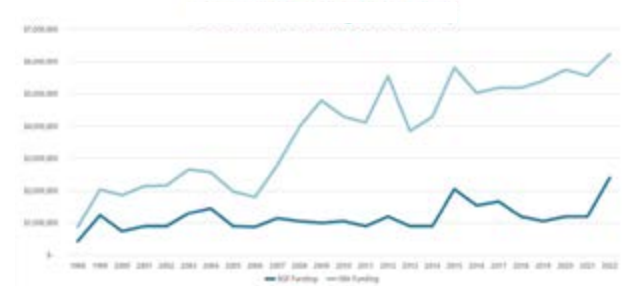
78
Institutions

$30M
Funded

INCREASE IN SCLERODERMA PUBLICATIONS 1998-2022



INCREASE IN SCLERODERMA RESEARCH FUNDING 1998-2022



NIH $97M
In additional NIH Funding

IMPROVING QUALITY OF LIFE *through medical advancements*

Meet Josie Garcia, a 58-year-old Northern California native who was diagnosed with systemic diffuse scleroderma, Raynaud's, Interstitial Lung Disease, GERD, and Myositis in 2012, and Diffuse Cutaneous Systemic Scleroderma in 2020.

Josie suffered from advanced skin tightening, debilitating and severe itchiness and nerve pain. Her skin score in 2020 was 41 (out of 51), DLCO at 59%, ulcers on her arms, and range of motion greatly decreased, impacting and diminishing her quality of life. She shared, "I didn't think the disease had significantly progressed until my rheumatologist asked me to consider an autologous stem cell transplant, HSCT (Haematopoietic Stem Cell Transplantation), especially since I was 8 1/2 years post-diagnosis."

Due to her specialist team's location at Stanford Hospital (a 2+ hour commute from Sacramento area to Palo Alto), she chose to have her transplant there. Josie was the second scleroderma patient to undergo this treatment at Stanford: "My medical team was beyond outstanding. Their bedside manner was caring, compassionate, and empathetic. I called them my angels here on earth. They answered every question, left no stone unturned, and kept me apprised of everything at every step of the way. From initial consultation, through transplant, (including up to present day), they still are deeply involved with my care. My new re-birthday was April 5, 2021!"

The procedure itself took only eight minutes to reinfuse her stem cells: "It was fairly anticlimactic with all the necessary preparation to get to transplant day." Josie was hospitalized for three weeks afterward. She credits her strong faith to carrying her through this process. Initially, she was nervous and convinced she wouldn't proceed, but through her faith and prayer along with many preliminary tests, she decided to move forward.

> "THIS MEDICAL ADVANCEMENT IS A MIRACLE! IT GAVE ME MY QUALITY OF LIFE BACK."

She credits her success to not only her medical team but also to the emotional support from her family, friends, church, golf and local communities, along with people she didn't even know. She credits her husband immensely – he attended appointments with her regularly and was a sounding board to discuss treatment plans: "It was such a comfort having him there with me."

Two years, later, she has great, good, and not-so-good days: "Without the pioneers ahead of me to trailblaze the way, I wouldn't be where I am today. I'm so thankful and grateful to be living my best life, privileged when I can help others through their medical journeys. And I will continue to spread awareness as often as I can to one day find the cure for scleroderma."

"I BELIEVE THAT EVERY SINGLE MOMENT OF EVERY DAY IS A GIFT NEVER TO BE TAKEN FOR GRANTED."

BUILDING THE FUTURE OF SCLERODERMA RESEARCH, *one cell at a time*

I HAVE A LIFELONG INTEREST IN studying autoimmune disease. I've long been fascinated by the way the body knows to attack a virus, but other times attacks its own healthy cells or tissue. Benaroya Research Institute has provided an excellent opportunity for this work because we look at all types of autoimmune disease. Benaroya scientists are working on discoveries that we hope will allow us to rebalance the immune system in all autoimmune and immune system diseases. Our ultimate goal is to achieve a healthy immune system for each individual.

> "RECENTLY, I'VE BECOME MORE INTERESTED IN SCLERODERMA. THE SKIN IS THE LARGEST ORGAN AND PROTECTS US FROM THE OUTSIDE WORLD. WHAT HAPPENS WHEN THIS BARRIER IS COMPROMISED?"

What we've learned in the last few years, because of some really cool technological developments, is that there's an incredible amount of diversity in our skin. And it's so much more complex than we originally thought it was. But we really don't understand the immune signals that drive that complexity. What we're really hoping to get after in this project is to understand what the specific immune cell populations are that are driving this complexity, and if we can understand what is driving these disease processes. And that's really important, because we could then start to tailor drug regimens and understand what chemical interventions or drug interventions might be helpful to get at some of these issues.

I'm grateful to the National Scleroderma Foundation for funding my project entitled *Cutaneous T cell dependent regulation of scleroderma-associated fibroblasts.* Through this project, we hope to learn more about immune signaling in cells.

One particularly novel aspect of this project is that we are using 3D engineered skin, which is this skin-like gel that mimics the architecture of human skin so we can monitor how helper cells are regulating the immune response, and begin to understand when, how and even why it is triggered.

We know that scleroderma presents so differently in each person. By identifying the individual components of the immune system that may drive disease, perhaps we can unravel some of that complexity. If we can figure out what drives these disease processes, we can start to figure out how to treat the disease, and eventually even how to prevent it.

Ten years from now, I hope that with the ongoing technological advances we are able to really recreate aspects of healthy and diseased tissue in the lab so that we can really study the smallest, most specific aspects of disease. And not just for scleroderma, but for all autoimmune diseases. We have this idea here at the Benaroya Research Institute, that if we can figure out the mechanisms that drive one autoimmune disease, we can start to really understand how to correct and reverse and even cure many autoimmune diseases.

This incredible technological work is innovative work that we're doing, and it's also very expensive work. When you support the National Scleroderma Foundation, you're making a direct investment in the future of scleroderma research. Thank you for continuing to fund the innovative science that is necessary to understand more about this disease, and the projects that are bringing us closer to a cure.

PETER MORAWSKI, Ph.D.
Research Assistant Member
Benaroya Research Institute at Virginia Mason Franciscan Health
@Peter_Morawski

Meet our Foundation-supported researchers

FROM THE CLASS OF 2023

This spring, I had the pleasure of sitting down with each of our newly funded investigators to learn more about the important work they're doing to help us achieve our vision to find a cure for scleroderma. We are so fortunate to have the best and brightest researchers focused on our disease area. I continue to be inspired by the passion and dedication of the scleroderma research community. After learning more about the work the Foundation is supporting this year, I'm sure you will be too. Each one shared their gratitude for the Foundation's support of their work, which is only made possible by the generosity of our donors. Thank you for investing in a future free from scleroderma. You can learn more about all the projects the Foundation is supporting by visiting our website at scleroderma.org/research.

MARY WHEATLEY, IOM, CAE
Chief Executive Officer

This year's class of researchers and their Foundation-supported projects are included below.

- **Andreea Bujor, M.D., Ph.D., Boston University**
  Spatial frequency domain imaging as a new method to quantify skin changes in scleroderma
- **Rafael Contreras, Ph.D., University of Minnesota**
  Centromeres, Chromosome Instability, and cGAS-STING activation in Scleroderma Fibrosis
- **Roxane Darbousset, Ph.D., Boston Children's Hospital**
  Platelets as driver of NET formation in systemic sclerosis
- **Suzanne Li, M.D., Ph.D., Hackensack University Medical Center**
  Developing Classification Criteria for Juvenile Systemic Sclerosis: An International Effort to Enable Pediatric-focused Clinical Trials
- **Justin Lui, M.D., Boston University**
  Cardiac Strain Phenotyping of Systemic Sclerosis-related Pulmonary Hypertension
- **Peter Morawski, Ph.D., Benaroya Research Institute at Virginia**
  Mason Cutaneous T cell dependent regulation of scleroderma-associated fibroblasts
- **Elena Netchiporouk, M.D., MSc, The Research Institute of the McGill University Health Centre**
  Investigating the Impact of the Environment on Systemic Sclerosis in Canada
- **Natalie Saini, MSc, Ph.D., Medical University of South Carolina**
  Determining the somatic mutation burden and its consequences in scleroderma-lung disease

INVESTIGATOR Q&A

ANDREEA BUJOR, M.D., Ph.D.

My first scleroderma patients in medical school left a long-lasting impact on me. But I would say the most important thing or person that made me study scleroderma was my longtime mentor, Dr. Trojanowska, whom I met by chance many years ago. She's the one who offered me my first research position in her lab, and she continues to inspire me to study scleroderma. And again, I have to acknowledge my patients. My patients are really an inspiration to me, they keep me going when things get hard. I'm very impressed with their resilience and with their willingness to help the research in any way they can.

RAFAEL CONTRERAS, Ph.D.

I'm very excited with clinical trials that they're offering to treat viruses, like the Interleukin-6 trials. I think that targeting interferon beta is also a very good option. And hopefully, we can also target also what I'm proposing: the signal system pathway, which could block interleukin-6 and interferon beta at the same time.

NATALIE SAINI, MSC, Ph.D.

I think for me, the biggest factor was the fact that Dr. Feghali-Bostwick has her lab right here [at MUSC]. And the first time I spoke to her, we discussed scleroderma, and I was interested in these patients and their genetic mutations that may hold clues. This was something that people haven't been able to address before because they haven't had the tools to do so. When I spoke to Carol about it, we talked about how it's possible that scleroderma patients have increased mutation. And we did a little pilot, and we found that that was true. And that was pretty much the impetus into trying to figure out what these mutations are doing, and why these patients are getting such high rates of them.

It was wonderful to hear that our community of scleroderma clinicians and researchers are so committed to finding solutions for their patients, and that we have such inspiring mentors in our mix, too!

FOUNDATION SUPPORTED RESEARCHERS

ROZANE DARBOUSSET, Ph.D.

The neutrophil aspect of my project is the most interesting to me. People in the field of scleroderma, when we talk about neutrophils, it's not really something that is obvious. Neutrophils are more involved in immunity and fighting infections, and in scleroderma they have been for a long time ignored. So for me it's bringing something new to the table in scleroderma and that's really exciting.

SUZANNE LI, M.D., Ph.D.

In 2005, I saw a four-year-old girl in my office that had a linear scleroderma lesion on her leg, and I was worried that she could get a leg length difference or have joint problems. I wanted to understand the best way to help her. It took more than a year, working with a team of specialists including other pediatric rheumatologists and radiologists, to try to see if we could study this some more to really understand what the best treatment was. It really made me realize that we need better outcome measures to better characterize the disease and to be able to help more people.

JUSTIN LUI, M.D.

I think the biggest issue is recognizing that there are so many different ways that scleroderma can present. It's just not just a one size fits all or one kind of disease that everyone has. It's a disease of many shapes and forms, which can be challenging to fully understand. The research in this field has really blossomed, and that's very promising. There are so many amazing and diverse investigators studying it from various angles, backgrounds, perspectives, and expertise.

How the Medical and Scientific Advisory Board SUPPORTS THE MISSION

By Virginia D. Steen, M.D.
Chair, Medical & Scientific Advisory Board, National Scleroderma Foundation

FOR THE LAST six years, I have had the pleasure of chairing the Foundation's Medical and Scientific Advisory Board (MSAB). This group supports the leadership of the Foundation by advising the Board of Directors on matters requiring scientific judgment, including research and patient care. The MSAB serves as a connector to the medical and research communities and takes a leading role in fostering physician education, mentoring young investigators, and assisting with the development of Foundation programs designed to accomplish these goals.

> "I AM GRATEFUL TO WORK WITH THIS GROUP OF FRIENDS AND COLLEAGUES REPRESENTING A BROAD SPECTRUM OF RESEARCH INTERESTS, EXPERTISE, AND EXPERIENCES."

The MSAB includes representation from rheumatology, pulmonology, physical therapy, pharmacology, pediatrics and dental health. Members are engaged in everything from very basic science to clinical trials to patient care. We are fortunate to have faculty from premiere research and treatment centers across the country serving on the MSAB, with robust NIH funding portfolios.

Throughout the year, the MSAB develops Continuing Medical Education (CME) programming for the Foundation to assist in educating physicians who are not experts in scleroderma on the signs and symptoms of the disease in order to assist in earlier diagnoses. These programs are offered free of charge through our partnership with CME University (visit cmeuniversity.com/ for more information and to search our courses).

Members of the MSAB also serve as subject matter experts for Foundation publications, information and education for the scleroderma community. In addition, we are honored to play a role in the planning and execution of the National Scleroderma Conference and Kids Get Scleroderma, Too! Conference.

Speaking at chapter educational events is another highlight for members of the MSAB. By engaging with Foundation members, we are able to share important information about disease management while advancing the Foundation's mission.

- Virginia Steen, M.D., *Chair*
- Tracy Frech, M.D., MS, *Chair, Patient Education Committee*
- Richard Silver, M.D., *Chair, Research Committee*
- Lorinda Chung, M.D., *Chair, Physician Education Committee*
- John Varga, M.D., *Immediate Past Chair*
- Shervin Assassi, M.D.
- Jessica Farrell, PharmD
- Jessica Gordon, M.D.
- Kristin Highland, M.D.
- Laura Hummers, M.D., ScM
- Heidi Jacobe, M.D.
- Dinesh Khanna, M.D., MS
- Robert Lafyatis, M.D.
- David Leader, D.M.D., M.P.H.
- Maureen D. Mayes, M.D., M.P.H.
- Janet Poole, Ph.D., OTR/L
- Lesley Ann Saketkoo, M.D., M.P.H.
- Robert Spiera, M.D.
- Kathryn Torok, M.D.
- Maria Trojanowska, Ph.D.
- Carol Feghali-Bostwick, Ph.D., *Board Liaison*
- Karen Gottesman, *Patient Advisory Board Liaison*

LEARN MORE ABOUT THE MSAB AT SCLERODERMA.ORG/MEDICAL-SCIENTIFIC-ADVISORY-BOARD/.

Fostering the Next Generation of SCLERODERMA RESEARCHERS

THE NATIONAL SCLERODERMA

Foundation's commitment to fostering the next generation of scleroderma researchers led to the launch of the Pre-Doctoral Summer Fellowship Award Program. The Summer Fellowship Award Program is designed to recognize Ph.D. students conducting research related to scleroderma and cultivate their early interest in the cause of the disease: "The National Scleroderma Foundation is committed to training the next generation of scleroderma researchers, ensuring a pipeline of future investigators," says Foundation Board of Directors Vice-Chair Carol Feghali-Bostwick, Ph.D.

The 2021 inaugural class of award recipients was named in memory of Dr. Arnold Postlethwaite, an esteemed academician and physician who dedicated his life to advancing the treatment of rheumatic diseases, including scleroderma. The Foundation continues the award program annually, and each year one recipient is recognized as the Dr. Arnold Postlethwaite Summer Fellowship. The endowed memorial fellowship was established by the family of Dr. Arnold Postlethwaite and honors his career as a scleroderma specialist and his 22-year relationship with the National Scleroderma Foundation, where he served on the Medical & Scientific Advisory Board and as a support group leader.

Students may submit a proposal on research relevant to adult or pediatric scleroderma. Applications are accepted annually and due each year at the end of April, with awards given out at the beginning of June. Interested students can learn more on our website scleroderma.org/fellowship.

The success of the Foundation's research mentor programs is seen in the growth and success of those who have engaged with the Foundation as students and young investigators starting their career in scleroderma research. In 2022, the Dr. Arnold Postlethwaite Fellowship Award was given to Vivek Jani, a graduate student from Johns Hopkins University School of Medicine, working on his Ph.D. in Biomedical Engineering. Under the guidance of his mentor, Monica Mukherjee, M.D., M.P.H., he submitted his research project titled: Machine and Deep Learning for the Early Detection of Scleroderma-Associated PAH. Dr Mukherjee started as a young Investigator, participating in the Foundation's Early Career Investigator Workshop mentor program and receiving a National Scleroderma Foundation New Investigator grant award. Dr. Mukherjee, now a well-established investigator, who the Foundation recently awarded an Established Investigator grant, is dedicated to mentoring the next generation of scleroderma researchers. A full circle success! Their story is featured on page 30.

(continued on page 30)

Bringing the voice of the patient TO CLINICAL TRIALS

For the last decade, the National Scleroderma Foundation has been bringing the voice of those affected by scleroderma into the process of drug development. The Foundation launched *Patients as Partners,* a program designed to help our community engage with pharmaceutical and biotech companies working on clinical trial designs and other drug development initiatives for scleroderma and related conditions. Developed out of a gap and need for our industry, Patients as Partners became a premier program that many industry partners look to, to help guide them during critical stages of development.

Patients as Partners is designed to work in concert with industry partners on a variety of needs across all areas of drug development, from clinical trial protocol development through FDA approval and post-approval activities. The Foundation compiles patient advisory boards through recruiting, training and preparing members from our community to be strong advocates.

The Foundation has a multi-year track record of working successfully with numerous industry partners on national and global initiatives. Daniel Martinez, Manager for Patient Advocacy at Horizon Therapeutics, has the following to share about their experience with the program: "the Patients as Partners program was an excellent way for Horizon to gain access to patients and caregivers so that we could elevate the patient voice throughout our clinical trial process."

Our community of patients validates the important role they can have while participating in clinical trials shared by the following testimonies. And we believe that Patients as Partners is a valuable asset for both the industry partner, but most importantly, for those battling scleroderma.

"I chose to participate in a clinical trial in 2012 because my disease was rapid and aggressive. The approved drugs at the time were not slowing down my disease, let alone curing it. The risk that I would die from scleroderma had become much more serious once interstitial lung disease was added to my diagnosis. That mortality risk made the risk of experimental treatment sound far more appealing than doing nothing. Also important to me was my found purpose for my remaining life: by becoming part of the research, and part of the awareness of that new treatment option, whether I lived or died, whether I improved or didn't improve, my life had meaning by adding to the database of knowledge of what to do for future patients. Medical researchers can't do their work alone in a lab, real answers can only be found with real patients. They needed me and I needed them. That's the best kind of teamwork I have ever experienced!" -Cyndy Martin

"I think the trial was important to me personally because it gave me the unique chance to see if a cutting edge treatment—a heart-friendly stem-cell transplant protocol—could help fight my severe scleroderma in ways that conventional treatments could not. And while my scleroderma became active again later, I went into the trial knowing my experience will help inform future efforts to bring this potentially life-saving treatment to many, many other scleroderma patients in the future." – Nikhil Bhat

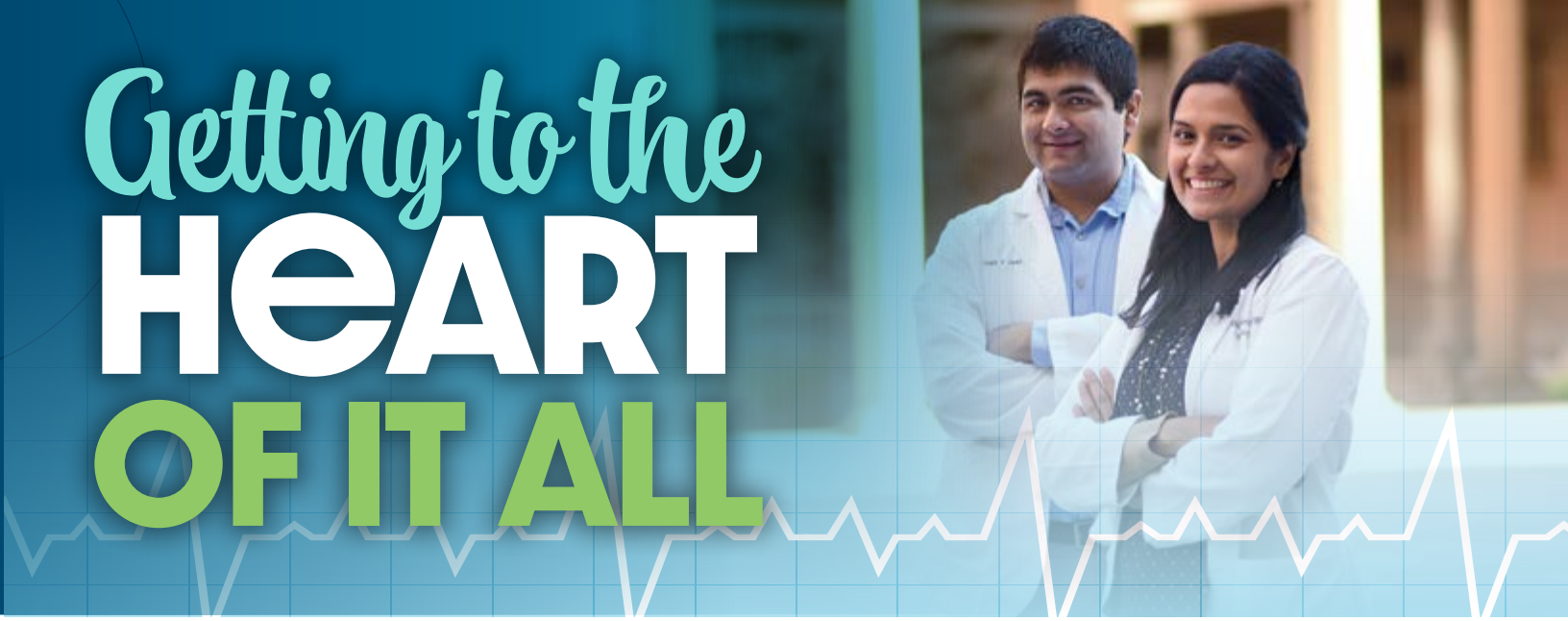
Cyndy Martin

Nikhil Bhat

Daniel Martinez

TO LEARN MORE ABOUT PATIENTS AS PARTNERS PLEASE VISIT SCLERODERMA.ORG/PATIENTS-AS-PARTNERS/

Getting to the HEART OF IT ALL



AN EARLY START

Monica Mukherjee, M.D., M.P.H., is currently an Associate Professor of Cardiology at Johns Hopkins University, and an established expert in the cardiac manifestations of scleroderma. Since 2012, Dr. Mukherjee has been interested in cardiac remodeling, particularly in the right side of the heart.

"I joined Johns Hopkins in 2012, and initially was interested in studying how the right side of the heart remodels in different disease states. In one disease, known as pulmonary hypertension, the right side of the heart remodels in order to pump against higher pressures. Its ability to do so is an important indicator of patient prognosis. Moreover, there are five different groups of pulmonary hypertension, each of which may lead to unique phenotypes of right ventricle remodeling. In scleroderma, there are three main types of pulmonary hypertension, which may occur and lead to adverse changes in how the right ventricle functions, further complicating this already complex disease.

When I first became Hopkins faculty, I was quickly impressed by the robust scleroderma and pulmonary hypertension centers, and was motivated to expand my research into this very important disease. With my background in advanced cardiac imaging, I had several ideas on using novel echocardiographic approaches as a yearly screening tool for cardiac manifestations in scleroderma. I began sharing my ideas with the Chief of Rheumatology, Dr. Ami Shah, and the head of the Scleroderma Center, Dr. Fredrick Wigley. That work led to a lasting collaboration with the Johns Hopkins Scleroderma Center, and an opportunity to apply for my first National Scleroderma Foundation grant."

Dr. Mukherjee received the Early Investigator Award in 2016, funded by the Foundation, which further fueled her desire to improve cardiovascular outcomes for people diagnosed with scleroderma.

"The tools that we were previously using in the field to look at heart remodeling in scleroderma were not sophisticated enough to pick up subtle changes in cardiac function suggestive of worsened prognosis. We know once scleroderma manifests, the outcomes are poor, so the question became 'how can we improve existing screening tools and echocardiography to detect cardiac changes in scleroderma early'?"

Through a new tool called speckle tracking strain used in conjunction with standard echocardiograms, Dr. Mukherjee and her colleagues detected abnormalities in how the right ventricle in the heart of scleroderma patients contracts.

> **"THE FOUNDATION GRANT I WAS AWARDED IN 2016 REALLY GAVE ME FOCUSED TIME TO EXECUTE VERY SOPHISTICATED RESEARCH"**

This research helped us see that there were subtle abnormalities in how the right heart contracts that we were unable to appreciate with conventional measures alone. Speckle tracking strain relies on using a standard echocardiography and following pixels or speckles in the image, derived from the interaction of the ultrasound beam with the heart tissue. Then, using a specialized software, the degree and speed of movement are used to quantify how a particular segment of the heart is contracting. This method is a more sensitive and non-invasive way to measure how different segments of the heart contract, and may unmask subclinical abnormalities that have clinical significance. Our first major study, funded by the Foundation, led to one of our biggest articles that was published in one of Cardiology's most esteemed journals called Circulation Cardiovascular Imaging."

Since then, Dr. Mukherjee has cemented herself as a leader in the field of multimodality imaging of the right heart, with a particular emphasis in right heart dysfunction in scleroderma and pulmonary hypertension.

Through ongoing collaboration and support from the National Scleroderma Foundation, Dr. Mukherjee was recently awarded the Established Investigator Award, highlighting the role that the Foundation has played in her successful transition from early to established investigator.

CROSSING PATHS

Two years after Dr. Mukherjee was awarded her first Foundation grant, Mr. Vivek Jani began the Medical Scientist Training Program (MSTP) at Johns Hopkins University, an esteemed seven-to-eight-year program that will result in him earning two postgraduate degrees—a Doctorate of Medicine (M.D.) and a Ph.D.. Mr. Jani came to Hopkins already equipped with a master's degree in Biomedical/Medical Engineering from the University of California San Diego, where he also completed his undergraduate studies in the same field.

"I'm an engineer and came to Hopkins very interested in cardiac mechanics, which is the study of how the heart contracts and the factors that affect contractility. After some clinical training, my interests shifted more towards studying how we can understand what's going wrong with the heart as a pumping organ and to better leverage that data and make more accurate predictions for patients that will help them more long term. This is why one of my mentors, Dr. David A. Kass, M.D., Director of the Institute of CardioScience (ICS), introduced me to Dr. Mukherjee."

In 2022, Mr. Jani was awarded the National Scleroderma Foundation's inaugural Dr. Arnold Postlethwaite Summer Fellowship Award, which encourages and fosters the next generation of researchers by cultivating interest in the cause and cure of scleroderma under the mentorship of an established scleroderma researcher. Mr. Jani was awarded the fellowship for his project titled Machine and Deep Learning for the Early Detection of Scleroderma-Associated PAH with Dr. Mukherjee as his mentor.

"Our work started from the idea that attention to differences in cardiac remodeling in different scleroderma patients provides an avenue for the application of precision medicine approaches for screening, diagnosis, and treatment of this syndrome. The recent explosion of artificial intelligence (AI) and machine learning technologies have allowed for their application to clinical problems. With this in mind, we decided to integrate novel machine learning techniques with sophisticated echocardiographic techniques, like speckle tracking, for the development of state-of-the-art algorithms that identify disease-specific features of scleroderma to better predict outcomes for patients."

WHAT'S NEXT

Research at the Foundation is multifaceted. We aim not only to advance medical and scientific research on scleroderma in the present day through our peer-reviewed research grant program, but we also connect researchers to each other through Global Research Webinars and support efforts to mentor the next generation of scleroderma researchers like Mr. Jani.

Establishing our Pre-Doctoral Fellowship Program to spark the interest of graduate students who may not have otherwise considered a career in scleroderma research was a critical step in building a pipeline of future investigators. Our robust research programs at the Foundation ensure that progress is always being made toward finding a cure, and our hope is that young researchers like Mr. Jani, who are being mentored by established investigators like Dr. Mukherjee, will follow in their footsteps and become established investigators themselves.

Because of Dr. Mukherjee's mentorship, Mr. Jani has begun carving out his own path as a researcher.

"I want to pursue Cardiology as a clinical field and pursue research," he said. "Scleroderma is a great place to start because there's a huge need. Plus, there are many factors in scleroderma affecting the heart, and the right side of the heart specifically."

The Foundation is proud to have supported Dr. Mukherjee and Mr. Jani through their mentoring relationship. Together, the dynamic duo is making strides in refining screening and early detection of cardiac manifestations in scleroderma by integrating advanced AI with speckle tracking echocardiography. We are grateful to Dr. Mukherjee for helping to mentor the next generation of scleroderma researchers, and we are equally grateful to her and Mr. Jani for the progress they have made for patients.

"There's been a significant change in mortality rates from scleroderma and PAH," said Dr. Mukherjee. "As recently as 10 years ago, many scleroderma patients with pulmonary hypertension were given a prognosis of four years after their diagnosis. Now, we're approaching 8-10 years in some groups, in part driven by improved awareness, screening, and early initiation of treatment. The research we're doing is impacting the health and wellness of this community. Sometimes it feels like the tunnel is very dark, but I think that the recent change in mortality trend is evidence that early screening and detection for cardiac involvement in scleroderma is happening and is leading to improvements. This hope is important to hold onto."

"I echo all that," Mr. Jani said.

Teen supports cousin BY RAISING MONEY FOR SCLERODERMA RESEARCH

17-YEAR-OLD TAYLOR RIENER RAISED nearly $6,000 for scleroderma research in honor of her cousin who was diagnosed with diffuse systemic scleroderma in 2019 after experiencing symptoms for several years.

From a small town in Idaho, Taylor grew up playing sports with her family and decided to host a high school volleyball game to raise funds for the National Scleroderma Foundation. She called it: "Dig for Derma!"

"I had the girls on the volleyball team (C, JV, and Varsity) sell paper volleyballs for $1, $10, and $25. Then, I personally went to businesses in town and asked for donations."

Taylor's cousin was able to come home from college in time to watch the 'Dig for Derma' match, which was a total success! The varsity team won in three sets, and, most importantly, Taylor helped hundreds of people learn about scleroderma by supporting our community.

"Even though I didn't want the attention," said Taylor's cousin, "I knew that it was important to raise money for the cure to help everyone. It is a way to give hope to everyone affected by scleroderma and every bit helps."

One of the National Scleroderma Foundation's mission pillars is research. The Foundation invests in research to understand the mechanism, discover the cause, and overcome scleroderma forever. Through research, we will find a cure, and we are so grateful for the Riener family who championed the Foundation's mission and adopted it as their own.

> "I AM VERY CLOSE WITH MY COUSIN. AFTER HIS DIAGNOSIS AND AFTER LEARNING THAT THERE WAS NO CURE, I WANTED TO DO SOMETHING TO CHANGE THAT."

"I want others to know about this disease since it isn't common," Taylor said. "And I want people to support advancing medical research. I hope the Foundation can someday find a cure and help save lives."

Thank you, Taylor, and everyone who supports scleroderma research.

To support scleroderma research today, go to: scleroderma.org/supportresearch

Sitting down with PROFESSOR IDO AMIT

Professor Ido Amit is Principal Investigator at the Amit Lab at the Weizmann Institute of Science located in San Francisco, California.

Shenna Gianetta, Manager of Research for the National Scleroderma Foundation oversees the research programs at the National Scleroderma Foundation.

Gianetta: Why did you become interested in scleroderma research?

Amit: Scleroderma is a debilitating and sometimes deadly disease that especially affects young women. Yet its causes remain an unsolved enigma. My lab started wrestling with this mystery together with Prof. Chamutal Gur from Hadassah Medical Center in Jerusalem, who has a personal interest in this disease as a rheumatologist, and after two of her family members developed scleroderma, with different levels of severity.

Gianetta: How has the scleroderma community (individuals living with the disease) impacted you both personally and professionally?

Amit: We have a strong connection with many scleroderma patients in Israel and the Palestinian Authority and with the clinicians treating them. We feel it is our obligation to discover remedies for this terrible disease. We are also working on encouraging collaboration with pharma companies to develop new treatments for scleroderma and advancing clinical trials to bring new hope for the people with the disease.

Gianetta: What is the most important question you want to address with this project? Can you describe for us why your research project is promising for those living with scleroderma?

Amit: Scleroderma is one of the most frustrating disorders to treat. We can alleviate some of the patient's symptoms, but usually, we cannot significantly affect the cause of the disease, block its progression, or reverse its course. One of the main reasons is that the cause of the disease is still largely unknown, and the heterogeneity in patients' clinical manifestations is huge.

Our research was conducted with the aim of zoning in on the first domino tile that starts the scleroderma cascade. To do that, we collected skin samples from nearly 100 scleroderma patients and from more than 50 healthy volunteers who served as a control group.

Scleroderma is considered an autoimmune disorder – that is, one in which the immune system turns on the person's own body. However, when we used our advanced single-cell technologies, the analysis failed to reveal a characteristic and global pattern of immune abnormalities.

Instead, we found out it was the patients' fibroblasts that differed significantly from those of the controls. Aside from roles in growth and wound healing, fibroblasts are usually thought to be mere "bricks" holding cells in place. Our study

challenges this humdrum picture. We found that fibroblasts can be divided into about ten major group types, and these groups can be further broken into some 200 subtypes.

Most importantly, we identified a subtype of fibroblasts whose concentration drops sharply in the early stages of scleroderma. Identifying these Scleroderma-Associated Fibroblasts, abbreviated as ScAFs (also for scaffold), opens the way towards developing a future therapy for scleroderma; they may also help predict the course of the disease, making it possible to adjust the treatment to each individual patient.

Gianetta: What would you say is a particularly innovative aspect of this project?

Amit: To discover the rare subset of cells involved in the development of scleroderma, we utilized the state-of-the-art single-cell technologies developed in our lab. These technologies, known as single-cell genomics, enable us to study each cell's unique identity and identify previously unknown rare subtypes of cells and understand their activity at a single-cell resolution. In addition to single-cell RNA sequencing, we also combined other layers of analysis, such as examining epigenetic regulation of each cell using a technique called single-cell ATAC-seq. This allowed us to identify specific molecular pathways that drive healthy ScAFs into the scleroderma disease state. These pathways can be used to develop novel therapies for scleroderma. The utilization of these novel technologies on samples and clinical data from close to 100 patients in the largest-ever study of its kind for scleroderma is highly innovative and allowed us to reach our important discoveries.

Gianetta: What is the overall importance of this project? How do you see this work impacting the field?

Amit: Our project shows how integrating the latest basic research techniques with a large database of medical data can shed new light on diseases whose origins are currently obscure. It reveals the enormous potential of meticulous tissue analysis using advanced single-cell technologies for uncovering disease dynamics. The identification of the ScAFs and other pathogenic skin stromal cells (e.g., pericytes, vascular cells) that changed in the early disease state, and how they interact with each other, provides a new understanding of what scleroderma is. It might be possible to design a therapy that will leverage the unique signaling repertoire of these cells slowing the progression of the disease.

Gianetta: What would you consider to be your most significant research accomplishment(s) to date? – and why? Do you feel it changed the way people approach the field?

Amit: My most significant research accomplishment is the harnessing of state-of-the-art molecular and computational technologies to create an impact on patients' health and to develop personalized medicine. These advanced technologies provide us with the ability to understand changes at a single-cell level and connect these changes to the clinical condition of different patients in order to identify new drug targets.

It is exciting to see how the field of single-cell research is rapidly expanding in recent years, transforming the way biological and medical science is being conducted and enabling the discoveries of new treatments.

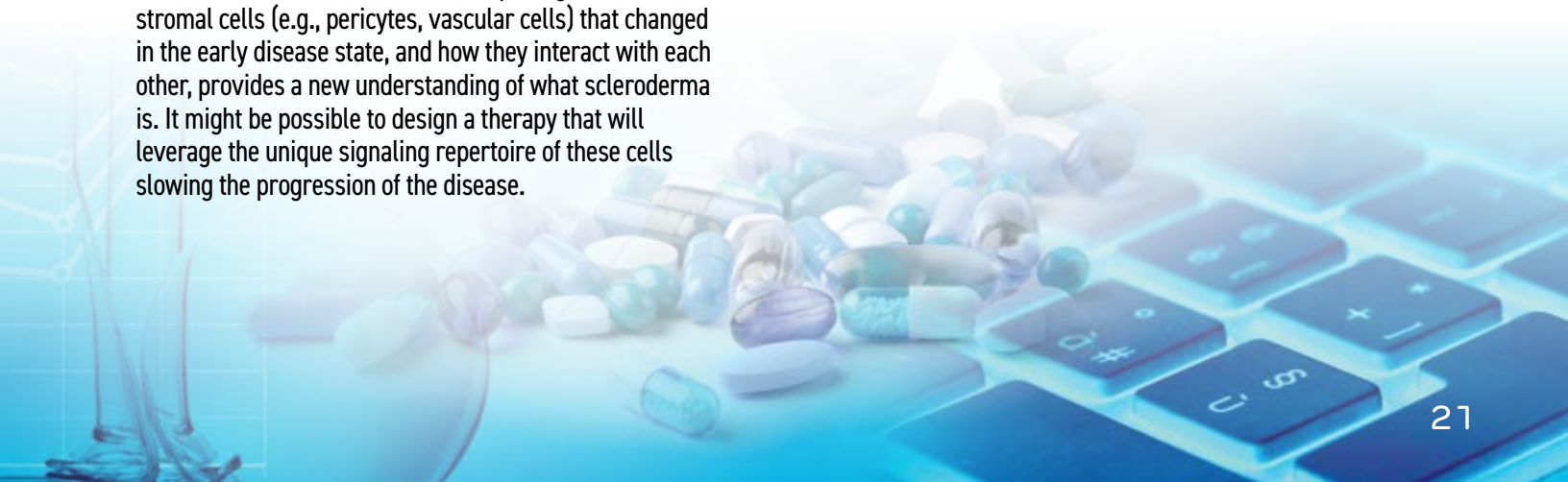
Gianetta: What do you consider the biggest issues in scleroderma research today?

Amit: The biggest challenges in scleroderma research today are the ability to identify which patients are at the greatest risk of deterioration and to develop novel treatments to stop this terrible process.

Due to the fact that scleroderma is a complicated disease involving a broad spectrum of clinical features that encompass vascular, immune, and fibrotic manifestations, the research and drug development has to meet all these aspects. Another important challenge is to design relevant animal and human ex-vivo models of scleroderma that will enable faster development of effective and specific treatment for the disease.

Gianetta: What about the most promising directions?

Amit: Our research so far has enabled the discovery of previously unknown pathways related to the development of scleroderma. We are thrilled to test the utilization of these new pathways on ex-vivo human skin and animal models we are working on developing, in the hope of turning those into treatments which will improve the lives of patients in the near future.



(25 years of innovative research continued from page 8)

In 2021, the Foundation created a named award to honor the memory of Arnold Postlethwaite, M.D., a distinguished scleroderma researcher and longstanding member of the Foundation's Medical & Scientific Advisory Board. The Dr. Arnold Postlethwaite Summer Fellowship supports Ph.D. students interested in scleroderma research.

FNIH BIOMARKERS CONSORTIUM

In 2022, the Foundation joined the Biomarkers Consortium, which is a program of the Foundation for the NIH which brings together partners to identify, develop and qualify potential biomarkers to improve drug development and regulatory decision-making. The identification of new biomarkers is necessary to help us predict, prevent and provide the right treatment to the right people at the right time. To meet this need, the Biomarkers Consortium supports projects which serve to develop promising biomarkers in order to help accelerate the delivery of successful new technologies, medicines and therapies for prevention, early detection, diagnosis and treatment of disease.

SCLERODERMA RESEARCH TODAY

Scleroderma research today is utilizing exciting new technologies for everything from simulating tissue to sequencing genomes. With the advent of artificial intelligence, the possibilities truly are endless. This year, the Foundation has committed more than $1.8 Million to research initiatives, including a new group of projects you can read all about in this issue.

SCLERODERMA ADVOCATES *in action*

RIGHT NOW, YOU CAN TAKE ACTION TO SUPPORT PEOPLE LIVING WITH SCLERODERMA - IT ONLY TAKES A FEW MINUTES TO MAKE AN IMPACT.

MEET WITH CONGRESS

Sit down with your members of Congress at home or in Washington, DC.

DIGITAL ADVOCACY

Tweet or email your elected officials about important scleroderma policy issues.

TELL YOUR STORY

When you tell your scleroderma story, you serve as a role model for others with the disease.

LEARN MORE BY REACHING OUT TO US BY EMAIL AT INFO@SCLERODERMA.ORG

Linking the GLOBAL COMMUNITY IN REAL-TIME

GLOBAL SCLERODERMA RESEARCH NETWORK E-SEMINARS

John Varga M.D.

Frederick G L Huetwell Professor of Internal Medicine – Director, sclerolab – Chief, Division of Rheumatology – Department of Internal Medicine – University of Michigan

THE ARRIVAL OF THE COVID-19 PANDEMIC HIT

the scientific community hard. Investigators were told to stay home, and laboratories went dark. Scientific meetings, symposia, conferences, and workshops, the lifeblood of scientific progress, were canceled. It seemed as if research ground to a halt, leaving scleroderma investigators bewildered, temporarily adrift, frustrated, and isolated. But the work had to go on, and after a brief pause, research laboratories cautiously re-opened their doors. What was missing was the opportunities to share new data, information, and insights. Science flourishes on the open exchange, intense dialogue, and robust debate. These are activities that are essential as a "reality check" in science, and also as tools for building consensus, validation, and implementation, as well as sharing the latest advances in fast-moving fields, such as scleroderma research. These activities are also essential for fostering communities of investigators sharing the same passion, focus, tools, and knowledge. The absence of opportunities to come together in person and face-to-face was keenly felt by scleroderma researchers everywhere.

As the scleroderma research community shared their frustration about the void, an idea emerged: why not meet together virtually? The idea was to use the internet for regular scientific presentations that would be open and available to all interested in keeping up with the latest advances. Moreover, because Zoom and other platforms have become inexpensive, accessible from anywhere, and easy to use, such webinars could reach a global audience, and in this way augment rapid information diffusion. Even better, such an open format might even help to stimulate out-of-the-box thinking, inspire new researchers and trainees, strengthen the global community of scleroderma investigators, and foster new collaborations.

Fortunately, the National Scleroderma Foundation quickly and fully embraced the concept and committed to running the new program. While today many of us take webinars for granted, this format was still relatively new and untried for the purpose of global communications three years

(continued on page 30)

Meet SHENNA GIANETTA

THE NATIONAL SCLERODERMA

Foundation is known for its strong support of scleroderma research. Shenna Gianetta, Manager of Research for the National Scleroderma Foundation, ensures that all of the Foundation's research programs continue to run at top speed. Her passion and dedication to the scleroderma community is shown in the many projects and programs that she leads year after year.

The Foundation's research programs have seen steady growth since Shenna joined the Foundation in 2011. The Peer-reviewed research grant program, which has jumpstarted the careers of many scleroderma experts, has increased the amount of grant awards from $150,000 to $200,000. The Foundation's mentorship program has expanded to include quarterly meetings and the Early Career investigator program was able to successfully transition to a virtual format during the pandemic.

The National Scleroderma Foundation is the leading non-profit supporter of peer-reviewed research to discover the cause, understand the mechanism, and ultimately find a cure for scleroderma. Although there is a lot of work that needs to be done to find a cure, over the years, significant advances in research and treatment have been made to improve the lives of those individuals living with scleroderma. Shenna has played a significant role in expanding the Foundation's reach by pioneering new programs and pulling together the research community.

In 2021, she helped launch two amazing programs for the research community: The Advances in Scleroderma Research Global Webinars series and the Pre-Doctoral Summer Fellowship Award Program. The webinars take place four times annually and focus of bringing researchers from around the world together to discuss scleroderma and related research.

"It's really making a difference in building, strengthening, and expanding the reach of the scientific research community. Through the increased dissemination of knowledge, new collaborations will help to accelerate advances in scleroderma research."

The Pre-Doctoral Summer Fellowship awards demonstrate the Foundation's commitment to fostering the next generation of scleroderma researchers through cultivating students' early interests in working to discover the cause and cure of scleroderma. When asked what her favorite part of working at the Foundation is, she responded:

"Being able to work with individuals, whether Foundation staff, volunteers, physicians, or research scientists, that have such a tremendous amount of passion and commitment to the scleroderma community and the Foundation's mission."

The future of scleroderma research looks bright, and the National Scleroderma Foundation is happy to have Shenna Gianetta leading the charge. For more information on the many research programs that the Foundation supports, visit scleroderma.org/research.

> "AS AWARENESS HAS GROWN OVER THE YEARS, WE HAVE RECEIVED GRANT APPLICATIONS FROM A GREATER NUMBER OF INSTITUTIONS, BROADENING AND STRENGTHENING OUR COMMUNITY OF SCLERODERMA RESEARCHERS."
>
> *Shenna Gianetta*

National Scleroderma Foundation

Support groups

Support Groups and Leaders, by State, as of April 30, 2023

Alabama
Birmingham
Jo Ann Bokenkamp

North Alabama
Sarah Logan

Arizona
Mohave Valley
Carol Hayward
Southern Arizona/Tucson
Pat Gould
Phoenix/Glendale
Holly Roberts

Colorado
Southern Colorado/Caregiver Connection
Gary Keschl
Denver
Barb Frodin
Western Slope
Rocky Mountain Chapter
Northern Colorado
Connie Osborn

Connecticut
Eastern Connecticut
Tri-State Chapter
Yale/New Haven
Tri-State Chapter

Delaware
Dover
Jennifer Cropper

Florida
Central Florida
Jan Gura
Jenn Scheinberg
New Smyrna Beach/Volusia County
Eileen Savell
Tampa Bay Area
Arlene Sweeney-Cornwall
Palm Beach/Broward/Dade Counties
Desiree James
Jessica Massengale
Meryl Parr
Beth Taber
Jacksonville
Hannah Bose
Lorraine Meide

Georgia
Peachtree City
Demetra Newton
Vernita White
Savannah
Robin Edwards

Iowa
Des Moines Area
Jill Connolly
Karen Fragale

Kansas
Topeka Area
Emily Morris

Louisiana
Baton Rouge
Del Anselmo
Many
Tina Dowden
New Orleans
Joy Mitchell

Maine
Statewide
Sandy Lunner
South Berwick
New England Chapter
York
Ellen Gregory

Massachusetts
Boston
Mary McClay
Fall River/Bristol County
Donna Bernier
North Shore
Roberta Mauriello
Worcester
Nancy Velleco

Michigan
The Autoimmune and Scleroderma Support Group
Nancy Stephens
Peggy Collins
Dryden
Michigan Chapter

Minnesota
North Metro
Deanna Hokanson
West Metro
Elaine Robashkin

Missouri
Poplar Bluff Area
Fran Atwell
Springfield Area
Gerry Robertson
St. Louis Area
Debbie Morris

Montana
Statewide
Montana Chapter

Nebraska
Statewide
Karen Fragale
Renee Hyde

New Hampshire
Central New Hampshire
Cathy Legere
Don Legere
Southern New Hampshire
Carla King
Jean Chapman

New Jersey
Bergen County
Tri-State Chapter
Northern New Jersey
Tri-State Chapter
Young Adults/Ages 18 to 30's
Amanda Lippincott

New Mexico
Albuquerque/Teens and Parents
Debra Droux
Jackie Martinez
Maria Tafoya

New York
Albany
Tri-State Chapter
Buffalo
Paulette Reed
Long Island/Telephone Support
Evamarie Gilbes-Cole
Nassau/Queens
Evamarie Gilbes-Cole
New York City
Zeba Hyder
Tiese Mahabir
Orange County
Barbara Celnick
Jodi Lyn
Rochester
Marilyn Sibley
Diane Reynolds

New York (cont.)
Tri-State Chapter Wide
April Roberts

North Carolina
Fayetteville
Donna Thomas

Ohio
Youngstown Area
Leni Schulz
Cleveland
Lucille Miller
Columbus
Allison Fish

Oklahoma
Norman
Karla Shelby

Oregon
Statewide
Christina Fidalgo

Pennsylvania
Doylestown
Kelly A. Kelly
Enola/Central PA
Dan Caruso
Pittsburgh
Caroline Graettinger
Tim Graettinger
Reading
Dawn Batzel
Williamsport
Jayne Young

South Carolina
Charleston
Sarah Budd
Patti Newman
Piedmont
LaKesha Williams

South Dakota
Sioux Falls
Annie Schock

Texas
Dallas/Fort Worth
Serita Gibson
Lilly Witherspoon
Carla Sutton
San Antonio
Deborah Charlton
Florence Diaz
Christy Lopez

Utah
Greater Salt Lake City
April Torres

Vermont
Brattleboro
Ilene Wax
Burlington
Blythe Leonard

Virginia
Fredericksburg/Richmond
Christine Hamblin
Norfolk/Phone Support
Blythe Leonard

West Virginia
Huntington
Karen Baker
Weirton/Telephone Support
Sandra Fennych

Virtual Support Groups

Black, Indigenous, People of Color (BIPOC)
Marilyn Sibley
Zynovia Hetherington
Caregivers
Debbie Haussler
Localized for Adults and Parents
Barbara Burke
Angela Bledsoe
Kira Kistner
Newly Diagnosed Support Group
Ron Sasso
Parents of Children with Scleroderma Support Group
Tracy Duvall
Pamela Pour
Scleroderma Young Adults Seeking Connection (SYNC) Support Group
Amanda Lippincott
Tiare Tolzmann
Spanish Speaking/Bilingual
Monica Ramirez
The 20%: A Virtual Support Group for Males Living with Scleroderma
Michael Bessert
Erion Moore

Every effort has been made to ensure this list is up to date and accurate as of April 30, 2023. For any corrections please email info@scleroderma.org.

The Patient Advisory Board Shares a Vision OF A WORLD WITHOUT SCLERODERMA

By Evamarie Cole and Karen Gottesman

PEOPLE LIVING WITH SCLERODERMA

have always been central to the mission of the National Scleroderma Foundation. The Foundation was created 25 years ago with a mission to advance medical research, promote disease awareness, and provide support and education to people with scleroderma, their families and support networks.

As Foundation leadership reviewed the governance and operations of the organization as part of the 2020 strategic plan, it became clear that while people living with scleroderma were well-represented in leadership positions throughout the Foundation, there was not a group specifically charged with representing the needs and voice of the patient community. The Patient Advisory Board was created by the Board of Directors with an update to the Foundation bylaws in December 2021, and was populated through an open call for nominations in 2022.

The Patient Advisory Board works closely with the Medical and Scientific Advisory Board, and the national Board of Directors, as well as Foundation staff and chapters, to work toward our shared vision of being a relentless force in finding a cure for scleroderma.

Additionally, the Patient Advisory Board serves several vital functions to advance the work and mission of the Foundation by advising the Board of Directors on matters related to patient support, education, and research.

These include serving as a link between the Foundation and the patient community; taking a leading role in disseminating patient information and education; developing timely educational programming; and establishing research priorities. In addition, the Board is charged with mentoring new and potential leaders and assisting with the development of Foundation programs and services designed to achieve the mission priorities.

We are honored to be the inaugural Patient Advisory Board Co-chairs which includes nine founding members, listed below, and we're delighted to be expanding our ranks this summer with the addition of several new members. To facilitate a more seamless nominations process, we do accept rolling nominations from interested candidates, whether you are a patient, caregiver or passionate about our cause.

If you are interested in joining us, please complete a nomination form online at bit.ly/PABrolling.

Evamarie Cole,
Co-Chair
New York

Karen Gottesman,
Co-Chair
California

CURRENT PATIENT ADVISORY BOARD MEMBERS

Melody Breen
South Carolina

Peggy Collins
Michigan

Carla King
Massachusetts

Amanda Lippincott
New Jersey

Ann Mogilevsky
Minnesota

Monica Ramirez
Florida

Ron Sasso
South Dakota

Finding scleroderma-related clinical trials has never been easier.

The National Scleroderma Foundation has partnered with Carebox to launch a new custom trial finder just for you right from our website.

Learn more at www.scleroderma.org

Know Your Options

Find clinical trials and access the most cutting-edge treatment plans while helping to pave the way for future improvements.

Scleroderma-Specific

Designed specifically for scleroderma patients with advanced search filters and criteria that include biomarkers, comorbidities and previous treatments.

Dynamic

Patients can return to their questionnaire at any time to update their information and find clinical trials that match the new criteria.

carebox

Carebox Connect is a service that is free and confidential. Patients are under no obligation to participate in clinical trials they are matched with. Matching does not guarantee enrollment. Patients can discontinue Carebox services at any time.

National Scleroderma Foundation

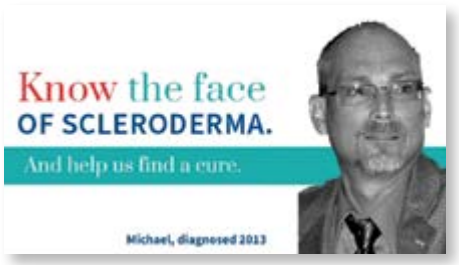
OUR *vibrant* COMMUNITY

Michigan:

This past March, the Michigan and Minnesota Chapters kicked off its monthly Lunch & Learn educational series with guest speakers Nina Rammessar, M.D., and Rachel Lando. Dr. Rammessar discussed different tests and labs often used for patients with scleroderma, followed by a robust Q&A session. Afterward, Rachel Lando took the participants through an adaptive yoga session.

On April 19, the Chapter hosted its next session with guest speaker, David Leader, D.M.D., M.P.H., who discussed dental issues associated with scleroderma. Dr. Leader is a general dentist who has treated patients with scleroderma since 1983, and has received the National Scleroderma Foundation's Doctor of the Year Award. The Lunch & Learn series will continue on the third Wednesday of each month. These webinar sessions are free and open to everyone!

Visit scleroderma.org/michigan to learn more.



Minnesota:

In-person Stepping Out to Cure Scleroderma walks are returning to Minnesota. This year, the Minnesota chapter is planning its first in-person walk since 2019 at Rosland Park in Edina, Minnesota, on June 24. Walkers will circle the lake on a dedicated trail while socializing with other members and supporters of the scleroderma community. The Chapter is excited to be together again in person after a long time apart. The in-person walk will also allow the Chapter to share its vision and future plans for supporting individuals affected by scleroderma, their families, and caregivers. Please come out and join the Chapter on June 24, 2023!

Registration Link: scleroderma.org/steppingout

Southeast Florida:

The Southeast Florida Chapter is excited to announce its Coconut Creek Stepping Out to Cure Scleroderma walk exceeded its goal by over $8,000. The Chapter had set an initial goal of $55,000 and reached a final total of over $63,000. The Chapter would like to thank all volunteers who continue to support and help our Chapter grow. Volunteers not only felt inspired by the Chapter's event, but many learned new information about scleroderma!

Ohio:

Ohio is building awareness and advocacy for Scleroderma!

Across the country, individuals are encouraged to reach out to local cities and townships to secure proclamations for Scleroderma Awareness Month in June. The Ohio Chapter is excited to announce they have secured a proclamation from the State of Ohio Governor, Mike DeWine, which declared the entire month of June as National Scleroderma Awareness Month in Ohio. Furthermore, several other proclamations have been secured around the state and the Chapter received approval from the Terminal Tower in Cleveland, Ohio, to be lit up in Teal on June 29th, National Scleroderma Day. Ohio has been and continues to be a strong advocate for disease awareness and hopes you will join its chapter members in raising awareness for scleroderma.

New England:

The 7th Annual Stepping Out to Cure Scleroderma Deer Island Stroll was held on June 10, and took place at the beautiful MWRA park on Deer Island in Winthrop, Massachusetts. The walk's venue showcased the serene views of Boston Harbor and its skyline. This Walk/stroll has grown immensely over the years and was founded by Maria Bartoszewicki, daughter of New England's former board president, Don Legere. scleroderma.org/stroll23

Delaware Valley:

On March 4, a group of volunteers from the Delaware Valley Chapter rallied together and brought back the Sweet Briar Walk. These individuals worked tirelessly to support and advocate for those affected by scleroderma by raising awareness of the Sweet Briar Walk through radio, TV, churches, and community groups.

The walk took place at Sweet Briar College on March 4, and was well executed with the help of the college staff. This year's event raised awareness in Virginia by bringing together over 150 individuals and raising over $15,000 in donations. The Delaware Valley Chapter would like to thank all the dedicated volunteers who made this walk possible.

South Carolina:

On May 20, 2023 in Greenville and on May 21, 2023 in Columbia the South Carolina Chapter hosted their Stepping Out to Cure Scleroderma awareness walks. Friends, families and individuals living with scleroderma gathered together in person and virtually to support the Chapter. As a hybrid event, individuals who could not attend in-person were encouraged to walk, dance or meet up for coffee. The Chapter recommended virtual walkers participate in whatever way they would like. For the in-person participants, there was a stroll along the paved pathways and then a gathering under the pavilion for walkers to socialize. If you were unable to attend, but would still like to support the South Carolina Chapter's Stepping Out to Cure Scleroderma Walk, visit scleroderma.org/steppingout/.

On April 1, 2023 the South Carolina Chapter hosted their 13th Annual Amy K. Parrish Education Day.

Heartland:

The Heartland Chapter hosted its Stepping Out to Cure Scleroderma Des Moines walk on June 3. Members of the Heartland Chapter came together and enjoyed the in-person walk at at Grandview University in Des Moines, Iowa. Thank you to everyone who participated and helped bring awareness for scleroderma!

Tri-State:

It has been almost three years since the Tri-State Chapter's support groups have joined together in-person. The Chapter is happy to announce the return of in-person support group meetings. These meetings are a great opportunity to reconnect in person and enjoy collaboration and support that comes from being able to sit across from one another. The Chapter asks for support group participants to bring ideas, topics and activities they would like to see in support group meetings.

The Tri-State Chapter will be seeking volunteers as it transitions back to in person meetings. There are many ways to volunteer within support groups and the community. Although the return to in person meeting is exciting, the Chapter understands that the safety of members is the first priority. Precautions will be taken to ensure everyone's safety. If you have questions or would like to volunteer, please email tri-state@scleroderma.org

Texas Bluebonnet:

On April 15, the Texas Bluebonnet Chapter hosted its 2023 Stepping Out to Cure Scleroderma Dallas & North Texas Walk. The event took place at Bear Creek Park in Keller, Texas. Along with the walk, the Chapter hosted a raffle and silent auction, DJ kids' music games and fun family music. The Chapter enjoyed seeing everyone coming together to raise awareness and support for the scleroderma community. If you would like to participate in a Stepping Out to Cure Scleroderma walk in Texas, you can join the Texas Bluebonnet Chapter at its Houston/South Texas walk on May 6, at Sugar Land Memorial Park and its San Antonio/Central Texas walk on June 3, 2023, at Mission Park (Dance Pavilion).

Rocky Mountain:

The Rocky Mountain Chapter hosted its Denver Scleroderma Education Day on April 8. The Chapter was pleased to welcome Kristen Demoruelle, M.D., Ph.D., Liudmila Kastsianok, M.D., and Mehrnaz Maleki Fischbach, M.D. to the Denver Education Day Event at UCHealth. During this event, the group discussed updates in scleroderma research, treatments, and offered support group sessions. This program was a great opportunity for individuals living with scleroderma to meet one another, along with caregivers, family members, friends and health care professionals! The event took place at Bruce Schroffel Conference Center in the Anschutz Inpatient Pavilion Two.

Missouri:

The Missouri Chapter hosted its Kansas City Stepping Out to Cure Scleroderma walk on June 3. The walk took place in a new location at the Macken Park in North Kansas City, Missouri. The day was filled with fun activities as the Chapter also hosted a raffle and silent auction during the walk event. After the walk, the Chapter provided lunch for walkers to spend time connecting with one another. If you would like to participate in a Stepping Out to Cure Scleroderma walk in Missouri, the Chapter is hosting a walk this Fall in St. Louis. Details of the Stepping Out to Cure Scleroderma St. Louis Walk will be announced on scleroderma.org/steppingout.

Oregon:

The Oregon Chapter hosted their monthly virtual support group on March 11. The group watched a video presentation from the 2022 National Conference. The video focused on common systemic sclerosis medication concerns and questions and was presented by Jessica Farrell, PharmD. After the video, the group discussed the content and asked questions. If you are interested in joining, the Chapter's virtual support group meets on the second Saturday of every month. To learn more, email ORchapter@scleroderma.org.

(Fostering the next generation of scleroderma researchers continued from page 15)

2021 AWARD RECIPIENTS:

Elizabeth Caves, Yale University
Mentor: Valerie Horsley, Ph.D.
Project Title: Understanding the role of resident adipocyte fatty acids in dermal fibrosis

Wei Dong, University of Tennessee HSC
Mentor: Weikuan Gu, Ph.D.
Project Title: The potential role of-Caryophyllene in the treatment of systemic sclerosis

Noelle Kosarek, Geisel School of Medicine at Dartmouth
Mentors: Michael L. Whitfield, Ph.D., Patricia Pioli, Ph.D.
Project Title: Using Single Cell RNA-sequencing to Elucidate the Mechanisms of Fibrosis in a Systemic Sclerosis 3D Human Tissue Model

Joe Mouawad, Medical University of South Carolina
Mentor: Carol Feghali-Bostwick, Ph.D.
Project Title: The Role of Extracellular Vesicles in the Propagation of Scleroderma-Associated Lung Fibrosis

Helen Warheit-Niemi, University of Michigan
Mentor: Bethany Moore, Ph.D.
Project Title: Dissecting the role of neutrophil function and aging in pulmonary fibrosis.

2022 AWARD RECIPIENTS:

Dillon Popovich, Dartmouth College
Mentor: Michael L. Whitfield, Ph.D.
Project: Pathway-Driven Drug Repositioning in Systemic Sclerosis from Omics Data

Adegboyega Adewale, Medical University of South Carolina
Mentor: Carol Feghali-Bostwick, Ph.D.
Project: The role of the IL-6-IGF-II axis in Systemic Sclerosis-Associated Lung Fibrosis

Suneeti Madhavan, Case Western Reserve
Mentor: Dr. Radhika Atit
Project: Adipocyte lipolysis is activated by Wnt-induced ER stress

Rithika Behera, University of Pittsburgh
Mentor: Robert Lafyatis, M.D.
Project: Understanding FOSL2 mediated regulation of dermal myofibroblasts in systemic scleroderma

Vivek Jani, Johns Hopkins School of Medicine
Mentor: Monica Mukherjee, M.D., M.P.H.
Project: Machine and Deep Learning for the Early Detection of Scleroderma-Associated PAH

Vrinda Dambal, Boston University
Mentor: Maria Trojanowska, Ph.D.
Project: Characterization of secretome of GATA-6 deficient Pulmonary Arterial Endothelial cells and to check its effect on activation of fibroblasts in SSc-PAH model

(Linking the global community in real-time continued from page 23)

ago. The Foundation set up the infrastructure for the webinars. Importantly, it was agreed that the webinars would be global in reach, open and gratis to all researchers, and free from commercial influences or sponsorship. An international and diverse Steering Committee was established to plan the program, and the Foundation reached out widely to advertise.

I am very pleased to report that this innovative program is now in its third year, and has been successful beyond expectations. Webinars occur every three months, and each session features three scientific presentations. Speakers come from many countries and present the very latest significant scientific advances in scleroderma. The sessions are broadly advertised through the Internet and social media, and registration is free of charge and open to anyone in the research community that is interested. Recent presentations covered innovative cellular therapies and vaccination for fibrosis, the discovery of novel targets for therapy, environmental factors that cause fibrosis, collagen biosynthesis, bioengineering, parallels between aging, cancer and scleroderma, genetics, and many many other timely and exciting topics.

Many of these presentations were made by scientists working outside the scleroderma field – underlining the critical importance of cross-disciplinary exchange of ideas and convergence of information. Up to 400 participants registered, and the presentations are followed by lively discussions generating exciting new collaborations. A novel feature is that each presenter is introduced by an early-stage scleroderma investigator who moderates the session and subsequent questions and answers. Notably, our registrants come from over 35 countries and all five continents and include investigators from hospitals, universities, government agencies, as well as pharma and biotech companies – a testament both to the high level of interest in scleroderma, as well as the excitement about the virtual format.

Scleroderma is challenging - for those who live with it every day, and for those dedicated to finding cures. The diverse and international community of scleroderma investigators benefits enormously from the rapid dissemination of information and open exchange of ideas and resources. As they say, it takes a village – and all can contribute and make a difference!

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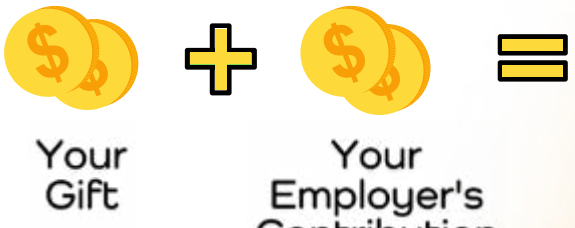

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