Center Awarded Funding from the Department of Defense to Lead Work Towards Identifying Medications to Manage Raynaud Phenomenon

Among patients with systemic sclerosis, Raynaud phenomenon is the most common symptom, with > 95% of individuals experiencing it. It is often the first symptom of scleroderma that patients develop. In a large survey, it was ranked by scleroderma patients as the highest disease-specific symptom affecting their quality of life. Unfortunately, there are no U.S. Food and Drug Administration-approved medications for managing Raynaud symptoms. This is disappointing, as there has been compelling reasoning for many of the drugs that have been tested in trials to have worked, but none have shown benefit in randomized, controlled trials. One of the concerns is that perhaps we need better outcome measures, or methods that we use to assess if a person is responding to a therapy.

Dr. Domsic is working with colleagues from Johns Hopkins (Ami Shah, MD and Laura Hummers MD), and the Royal National Hospital for Rheumatic Diseases in Bath, UK (John Pauling, MD) to develop better outcome measures to be used in Raynaud phenomenon trials. This work was funded by the Department of Defense, through the Congressionally Directed Medical Research Program on Scleroderma. We are excited because we believe it’s a fundamental step in working to have medications approved for Raynaud phenomenon.

Over the last couple of years, this group (with others) has worked to develop a patient-reported questionnaire up through patient focus groups and a panel of scleroderma experts. During the 2019-2020 winter some of our readers may have been asked to fill out questionnaires on Raynaud symptoms. The questionnaire was the first part of the grant project, as measure was further tested. Over the next 1-2 years (cont. on page 2)

Leigh Freno is a Certified Registered Nurse Practitioner at the University of Pittsburgh Physicians Department of Rheumatology Scleroderma Center. She grew up in Indiana County, PA and moved to Pittsburgh, PA in 2009 to complete her degree as a Nurse Practitioner. She recently relocated back to the area after spending five years out of state as a military spouse, having lived in both Jacksonville, FL and Leonardtown, MD. After her husband’s separation from the Navy in October 2018, they decided to move home to be closer to family. Leigh has a wide range of experience in the healthcare field including having worked previously in such specialties as labor and delivery, oncology, outpatient bariatric surgery, as an acute care hospitalist, hospice and palliative care, and family medicine. Leigh lives in the South Hills with her husband Justin, her sons Ethan and Noah, and her cat Olivia. In her spare time, she enjoys being with family and spending time with her boys.

Leigh Freno, CRNP
(cont. from page 1) the 3-center group will be focusing on looking at two types of additional potential outcome measures. First is evaluating a smart phone application to track Raynaud symptoms. The second type looks at non-invasive assessments of blood flow and vascular health in the fingers and hands using infrared thermography and laser speckle imaging. These measures will be tested by observing 160 patients over one year, so that the effect of different seasons on Raynaud activity can be understood better. This is important, as everyone knows that if we start a new medication in March that by June symptoms may be better. But, how much is from the drug, and how much from warmer weather? Learning how much Raynaud activity changes between seasons is key to designing better Raynaud trials, so that we can mathematically account for expected seasonal change. Participants in our observational study will be asked to come in for five visits over the course of a year, which last 1-1.5 hours per visit. As always, we will work hard here at Pittsburgh to combine any research visits with clinic visits. We understand everyone being hesitant to come in right now given Covid-19, but we are hoping for the support of our scleroderma patients in 2021. We all want less discolored and cold fingers in the future!

SPIN-SHARE Platform is Live!

The Scleroderma Patient-centered Intervention Network (SPIN) team is an international organization of researchers, health-care providers, and people living with scleroderma working to develop and provide support programs to those with systemic sclerosis. After a full-scale clinical trial in 2018-2019, the SPIN-HAND toolkit is now available as a free online resource for patients to access in day-to-day life outside of research contexts.

SPIN-HAND offers a variety of exercise modules, such as wrist mobility and finger extension, and video tutorials that demonstrate how to correctly perform the exercises. Modules are offered in two versions: one adapted for those with mild to moderate hand involvement, and one for those with more severe hand involvement, so that patients may choose what level best fits his or her needs. As hand exercises will need to be practiced regularly, SPIN-HAND also offers custom exercise routines, allowing patients to set attainable daily and weekly goals while maintaining motivation throughout the program. To find more information and sign-up for free access, visit: https://tools.spinsclero.com

Pittsburgh Area Scleroderma Support Group

2020/2021 Meeting Schedule
(last Saturday of every month)

<table>
<thead>
<tr>
<th>2020:</th>
<th>2021:</th>
</tr>
</thead>
<tbody>
<tr>
<td>Oct 31</td>
<td>Jan 30</td>
</tr>
<tr>
<td>Nov 28*</td>
<td>Feb 27</td>
</tr>
<tr>
<td>Dec 26*</td>
<td>Mar 27</td>
</tr>
<tr>
<td>Apr 24</td>
<td>May 29</td>
</tr>
<tr>
<td>May 29</td>
<td>Jun 26</td>
</tr>
</tbody>
</table>

*subject to change due to holidays

Where: Meetings will be held via zoom until further notice.

Time: 3:00 - 4:30 pm

Meetings are free of charge.

For more information contact sfgrouppitt@gmail.com

- Meet others affected by Scleroderma
- Share concerns and information
- Give and receive encouragement
- Occasional guest speakers
- Caregivers and family members welcome
Watching Rumbi dance was like seeing the heart burst free from the boundaries of the human body. Her movements held the fluidity of water yet the energy of fire— I could only watch in awe, completely captivated by the strength and charisma of her art.

Rumbi Mupinga has danced her way into people’s hearts from a young age. Born in Harare, Zimbabwe and having moved around the United States since age 5, she originally struggled with finding her place amongst her peers. “At a majority of my schools, I was the minority and I never really fit in with most social groups— even with Black Americans because I was just ‘the African kid,’” she explains. To help bridge that social gap with her high school peers, Rumbi coordinated an African dance for a Black history month program. From that point onwards, her skill and effort to share her love of dance rewarded Rumbi with a path to many friendships and human connection. She has danced at over 200 performances and 100 workshops.

Rumbi’s diagnosis in May of 2016 was accompanied with what she describes as fear of imprisonment in her own body. Rumbi likens her disease to suffocating: “When you are suffocating, you are trapped,” she tells me. “It’s a feeling of being trapped in your own body, unable to move sometimes and in constant pain. Scleroderma doesn’t allow you to live to your best potential... as a dancer, nothing is worse than not being able to dance or keep up with your own movement.”

Rumbi shares with me how attending the University of Pittsburgh & UPMC Scleroderma Center has been “one of the best things that has ever happened... more so than people think.” Her journey for advocacy for those with Scleroderma blossomed through meeting Maureen Laffoon, research coordinator at the Scleroderma Center for over 10 years. “Maureen helped me realize how heavily dance is helping me fight through some of my symptoms,” Rumbi says fondly. Inspired and strengthened by Maureen’s support, Rumbi created an Instagram page “Sclerodancer,” where she has been able to connect with individuals with Scleroderma and other chronic illnesses. She continues to inspire others on her page through sharing her accomplishments and future goals. “My message is simple: dance to just move. It doesn’t have to be perfect, but just keep going, stay positive, and learn how to be a better you.”

Despite the barriers she has faced, Rumbi has never stopped dancing. She founded the African dance organization Barefeet Dance Tribe in Ohio since the age of 19, initially involving local Kent city students. Eleven years later, it has grown into a thriving LLC with Rumbi as the Artistic Director. Although she finds herself unable to keep up with her group as often, Rumbi finds her own way of performing by teaching dance to others. “Surprisingly, without Scleroderma, I wouldn’t have known how to live life better, how to take more breaks, and laugh more with the people that support me... Being able to accept every part of your diagnosis and what it comes with gives you a better outlook on life. I have overcome a lot of adversities and don’t plan on stopping anytime soon!”

Follow Rumbi's journey at: instagram.com/sclerodancer
Patient Gives Back by Organizing Virtual 5K Race to Benefit Scleroderma

Erin Kerstetter wasn’t much of a runner until COVID-19. With little to do, she turned to the outdoors for fresh air, and running quickly changed from a new hobby into an advocacy opportunity for a disease she is learning to live with.

Kerstetter was diagnosed with limited scleroderma in her 30’s. During the time of her diagnosis, Erin knew very little about the disease. After she came to understand what the diagnosis meant, she committed to herself that she would do everything in her power to create more awareness to the scleroderma community and beyond.

Now, four years later, she has turned a life-changing diagnosis into a silver lining. This patient-turned-advocate made it her personal mission to educate and create awareness beyond the scleroderma community. And most recently, that mission included organizing a virtual 5k race to benefit research for scleroderma for UPMC.

“Research is so important in the fight against this rare and complex disease,” said Kerstetter. “UPMC is where my team is and funding their research is important to me. I want to help them in any way I can.”

Erin raised a total of $600 by selling more than 80 t-shirts for the event. The positive response from her family, friends, and strangers was overwhelming.

But Kerstetter’s commitment to supporting this cause goes beyond the virtual 5k. In addition to organizing the event, she leverages various social media platforms—including a Facebook page “Erin Kerstetter & My Journey with Scleroderma”—to give others a place to pose questions and share their own struggles with scleroderma.

“I hope the long-term impact of this donation is, of course, that a cure will be discovered,” said Kerstetter. “I also hope people will continue to become more educated and continue to understand the tribulations scleroderma patients experience.”
**Caleb’s Story—Systemic Scleroderma and Getting a Stem Cell Transplant**

I am an 18-year old with systemic scleroderma and recently received a stem cell transplant at Children’s Hospital of Pittsburgh. I struggled with scleroderma since my freshman year of high school, when I noticed myself getting tired quickly and falling short of my peers in football leading me having to quit the team. My health started declining fast. That summer I lost 25 pounds and started having other symptoms which led to my scleroderma diagnosis.

I underwent multiple tests, had what seemed like an endless amount of information tossed my way. As a 16-year old it was a lot to handle. We discovered that my lungs were affected and started medication, which seemed to work at first, but my next round of pulmonary function tests showed a 30% decrease in my lung function.

Dr. Torok and I discussed my next steps and the best plan of action was a stem cell transplant. Luckily, a new stem cell program had begun at Children’s Hospital of Pittsburgh, led by Dr. Paul Szabolcs. I was very anxious and worried, but was reassured when I met him and the stem cell team. It took many months to get the transplant started. After overcoming multiple setbacks, my mom and I moved to Pittsburgh temporarily and I was admitted to the hospital. There is a certain level of fear when you begin because there are so many opportunities for things to go wrong, but even more I feared what would happen if I did not take action. I went through radiation with only minor difficulty, responded well to medications, and dealt with a feeding tube (which was the hardest part for me - you don’t know just how much you love food until you can’t have it!). I am extremely fortunate because I know how many problems can happen during a transplant; however, one of the most important factors in succeeding is your frame of mind. A positive attitude is invaluable when going through something like this.

There were many improvements in my health during the hospital stay, my skin in particular. I felt so much looser than before, and I remember thinking just how excited I was to resume physical therapy. My energy was also significantly improved. Normally I would need to take a nap, but I began to feel like I could stay awake all day. Needless to say, all this was great and seeing the changes just motivated me even more to make it through.

Getting discharged from the hospital was a day that I will never forget. The doctors and nurses lined up outside, and cheered me on as I exited the wing. Sure I think they do that mostly for the kids that come and go, but even for a young adult like myself it was very special to have the team there to see me out. The staff at Children’s really does treat you like family.

Three months have passed since my “re-birthday”, and things just continue to improve. My energy and mobility are at an all-time high, circulation to my hands has greatly improved, and I am able to do so much more without being bothered by Raynaud’s Disease. Honestly, I feel the best I have since I was first diagnosed back in 2016. For anyone who is skeptical, or afraid to take the leap to a stem cell transplant, don’t be. I can say now that I would do this all over again because of how quickly I felt better. The consequences of uncontrolled scleroderma are scary, in some cases more scary than the risk a stem cell transplant, and there is a small window where this treatment is possible. If you are in a similar situation, please remember that when you have the right team of people, a good attitude, and perseverance, you can achieve a better life!
Our Scleroderma Center is committed to participating in clinical trials. We feel it is a vital step in working together to find treatments for scleroderma. Without clinical trials, our field will not advance. If you are interested in participating in a trial or would like additional information, please contact Maureen Laffoon at 412-648-7871 or laffoonm@pitt.edu.

Currently Enrolling:

Evaluation of Brentuximab Vedotin for Diffuse Cutaneous Systemic Sclerosis: A Phase I/II Multicenter, Randomized, Double-Blinded Safety Study (BRAVOS):

Brentuximab vedotin (name brand Adcetris) is a drug that was developed and has FDA approval for the treatment of lymphoma. This research is being done to evaluate the safety and tolerability of brentuximab in the treatment of diffuse skin disease in scleroderma. Patients must be early in their disease with worsening skin to participate. Two of three patients will receive brentuximab vedotin, and the other individual, placebo. The study lasts 48 weeks and involves 14 visits. Patients will be able to remain on their current scleroderma medications.

An Observational Study of the Effect of Mycophenolate Mofetil (Cellcept) in Early Diffuse Scleroderma (MMF STUDY):

This is a NIH-supported single-center study (being performed only in Pittsburgh) to observe the effect of mycophenolate for the treatment of early diffuse scleroderma. Mycophenolate is one of the most commonly used medications to treat diffuse scleroderma and scleroderma-related pulmonary fibrosis, however we know little about how to predict who will respond well to the medication. Patients whose physician recommends they should be clinically treated with mycophenolate are eligible to be in this study. When in this study your doctor may change your medications at any time. We simply observe the effect of mycophenolate, collect data on its effect on skin, and collect blood and oral swab samples every 3 months for the first year a patient is treated with mycophenolate. This study can easily be combined with regular patient visits.

Optimizing Raynaud Phenomenon Outcome Measures in Systemic Sclerosis (ROSS):

The purpose of this research study is to improve clinical trial design when studying Raynaud phenomenon (RP) in systemic sclerosis (SSc). We are validating a new patient-reported questionnaire, testing a smartphone application to assess Raynaud attack frequency/duration and comparing new imaging techniques for skin blood flow in the hands. The study is over one year, as we recognize Raynaud symptoms change across seasons, and wish to gain a better understanding of how seasons affects these new outcome measures. Ultimately, improving outcome measures could aid in having a drug show a positive change and gain approval for Raynaud treatment. A laser speckle contrast imaging (LSCI) and infrared thermography (IT) machine imaging of their hand. Visits will be 90 minutes on the first day, and thereafter 30-60 minutes.
The observational study will last 48 weeks, although those who start a new medication for Raynaud medication will be asked to follow for a longer period so the effect of the new medication can be captured. There are six study visits within those 48 weeks. At each visit, all subjects will be asked to complete a one week diary the number of Raynaud attack, and on the day of the visit complete a small packet of self-administered questionnaires that collect information on your Raynaud symptoms, function, mood, and quality of life. At all visits except week 36, subjects will undergo non-invasive blood flow imaging using a laser speckle contrast imaging (LSCI) and infrared thermography (IT) machine imaging of their hand. Visits will be 90 minutes on the first day, and thereafter 30-60 minutes. Please read the feature article on page 1 for additional information about this study.

**A Phase 2, Randomized, Placebo-controlled, Double-blind, Open-label Extension Multicenter Study to Evaluate the Efficacy and Safety of KD025 in Subjects with Diffuse Cutaneous Systemic Sclerosis (Kadmon):**

This study examines the safety and effectiveness of KD025 in the treatment of diffuse skin disease in scleroderma. Our Center is one of 25 sites in the United States conducting this study. A study-wide sixty subjects will be randomized (1:1:1) to receive orally administered KD025 200 mg once a day, KD025 200 mg twice a day, or placebo. The study participation is 14 months. (4 weeks for screening, 52 weeks of dosing period, and 4 weeks of follow-up) with a total of 18 visits to our Center. The study will be double-blinded for the first 28 weeks followed by an open-label extension of 24 weeks.

**Investigating methods to help with fatigue (Apollo Study):**

We at the Scleroderma Center are excited to be working with **Apollo Neuroscience™** in exploring whether there may be a wearable device to help with fatigue and other symptoms in scleroderma. In keeping with Covid-19, this small pilot study will be conducted completely remotely, meaning patients will not have to come in to the Center, and everything can be done from their own home. We are hoping to have 20-30 patients with systemic sclerosis and fatigue who are willing to try this wearable device, similar to a bracelet, for one month. We will ask participants to fill out questionnaires regarding fatigue and other symptoms at specific timepoints during the study. The Apollo wearable, developed out of research at Pitt, is a Pittsburgh company founded by a local psychiatrist and neuroscientist.
Sophie Anne’s Fund to Provide Support for Scleroderma Patients

Seeking the best available care and research, many scleroderma patients and their families travel from outside of Western Pennsylvania to the Pediatric Scleroderma Clinic at UPMC Children’s Hospital of Pittsburgh. For some families, the cost of travel and expenses associated with their diagnosis have caused significant financial hardship. In some instances, patients have delayed care or sacrificed participating in promising research, because they don’t have the necessary financial resources.

Because of a generous donation, the Pediatric Scleroderma Clinic at UPMC Children’s Hospital of Pittsburgh is able to offer support to families who need financial assistance to access care. Understanding the challenges families with scleroderma face, Jeff and Martha Seaman wanted to ensure all children have access to necessary clinical care and research.

In honor of their daughter Sophie Anne, who is a patient of the Scleroderma Clinic at UPMC Children’s Hospital of Pittsburgh, Jeff and Martha Seaman established Sophie Anne’s Fund. Sophie Anne’s Fund will support scleroderma patient families who need assistance with expenses such as transportation, lodging and fuel.

If your family needs assistance to access care or participate in research at the Pediatric Scleroderma Clinic, please reach out to scleroderma@chp.edu or call (412) 692-6478.

If you’d like to donate to Sophie Anne’s Fund, helping to support families seeking treatment and research, please reach out to maura.white@chp.edu or call (412) 692-3937.

During the rapidly evolving COVID-19 pandemic, individuals with underlying health conditions such as Systemic Sclerosis, those taking immunosuppressive treatments, and those with lung disease are at increased risk for severe illness from COVID-19. It is important to take the utmost preventative measures and adhere to national, state, and local social distancing restrictions. The Scleroderma Foundation emphasizes practicing the maximum precautions, including:

- Staying at home as much as possible and in accordance with local mandates
- Maintaining social distancing (6 feet apart from individuals you are not living with)
- Employing vigilant hygiene practices

Additional COVID-19 information and recommendations for people with Scleroderma can be found on the Scleroderma Foundation’s website, at: https://www.scleroderma.org
Up-to-date information and tips are also available at: https://www.coronavirus.gov
We would like to thank the following donors for their support of scleroderma research:

- Mr. and Mrs. Michael Anderson
- Friends of Tori Anderson
- Ms. Nadine S. Baldwin
- Mr. Banker Beach Jr.
- Ms. Maureen E. Blair
- Mr. and Mrs. Robert Briggs
- Ms. Michelle Brunii
- Ms. Danielle Bush
- Mr. John C. Caimi
- Ms. Linda L. Canami
- D.C. Cappetta & Associates, Inc.
- Ms. Darlene Carlo
- Mr. John A. Cegalis
- Walter & Marie Coyle Foundation
- Mrs. Dorothea A. Croce
- Mr. and Mrs. Serafino A. Croce
- Mr. Russell Crusan
- Mrs. Barbara Reilly-D'Andrea
- Ms. Bonnie Davis
- Mrs. Vickie DeFrehn
- Mr. and Mrs. Daryl Dehner
- Ms. Hallie Dietsch
- Mr. Dennis M. Domsc
- Mrs. Darlene A. Ebner
- Mr. and Mrs. Richard Edgerly
- Fidelity Charitable Gift Fund
- Ricky Zane Finney
- Ms. Maureen Schorr Fleischmann
- Mr. Ronald G. Fuchs
- Ms. Eva George
- Ms. Janice E. Giannetti
- Mr. and Mrs. Jonathan L. Goldston
- Dr. Timothy Graettinger
- Dr. Caroline Graettinger
- Mrs. Phyllis J. Gray
- Dr. Elaine M. Greifenstein
- Mr. and Mrs. Stanley W. Grove
- Mr. S. Steve Harrison
- Ms. Caryn Hasselbring
- Ms. Carol Heinlein
- Ms. Jeanette M. Hill
- Mr. Scot G. Hughes
- Mr. and Mrs. John Kane
- Mrs. Debra A. Kendall
- Friends of Erin Kerstetter
- Mr. John Kozlowski
- Ms. Irene Kobylarz
- Mr. Hsiang-Jung Kuo
- Ms. Alberta M. Lee
- Ms. Jenny Lin
- Dr. R.C. Lin
- Dr. Show-Bih Lin
- Mr. Frank J. Lotito
- Ms. Carolyn Marks
- Mr. Sheldon Marstine
- Marstine Family Foundation
- Ms. Melanie J. McCarty
- Mr. Steven L. McCarty
- Ms. Nancy A. McDonald
- Dr. and Mrs. Thomas Medsger
- Mr. and Mrs. Mark Mendlow
- Dr. and Mrs. David Merry
- Ms. Nancy M. Munn
- Mr. Dennis Musher
- Ms. Ruth E. Myers
- Mr. A. Richard Nernberg
- Mr. Maurice A. Nernberg
- Mrs. Nancy Nicolella Nernberg
- Mrs. Susan L. Nernberg
- Ms. Elisa A. Nye
- Ms. Michelle Pontious
- Mrs. Nancy Olsen
- Mr. and Mrs. A. Thomas Oyster
- Mr. James L. Passieu
- Mr. and Mrs. Richard J. Pish
- Mr. and Mrs. Peter R. Proffetty
- Ms. Cindy M. Raab
- Mrs. Barbara Reilly-D'Andrea
- Ms. Kathy S. Repp
- Drs. Carolyn and Paul E. Rizza
- Ms. Beverly Raeley Robinson
- Ms. Margaret Romain-Johnson
- Ms. Theresa A. Rondini
- Schertler & Onorato, LLP
- Ms. Elfriede Schramm
- Mr. and Mrs. Dean Schreiber
- Ms. Angela Schuster
- Mr. Brian R. Seylar
- Mr. and Mrs. Theodore S. Sova II
- Mr. and Mrs. Richard Sovchen, Sr.
- Mr. Salvatore Spinosa
- SRU Federal Credit Union
- Ms. Sally T. Stanley
- Ms. Laura Tomko
- Triangle Restaurant/Lounge Turftenders, Inc.
- Ms. Mica Van Fossen
- Mr. and Mrs. Roger A. Vasbinder
- Mr. and Mrs. Joseph Vioi
- Mr. and Mrs. Thomas D. Weaver
- Mr. Thomas D. Weaver
- Ms. Louise Weber
- Ms. Brenda K. Wilson
- Mrs. Barbara A. Worcester
- Shady Lady Studio, LTD

Your contributions to the Scleroderma Center are greatly appreciated and help support research and patient education programs. You can remember or honor a loved one by using the envelope enclosed in this newsletter to send your donation.

For additional information on giving to the Scleroderma Center, please contact Rose Jandrasits at 412-864-1958 or krj13@pitt.edu.
Scleroderma Center
University of Pittsburgh
3500 Terrace Street
BST South 7th Floor
Pittsburgh, PA 15261

SCLEROGERMA CENTER FACULTY AND STAFF

Faculty
Robert Laffayatis, MD
Professor of Medicine
Director

Thomas A. Medsger, Jr., MD
Professor of Medicine Emeritus

Robyn T. Domsic, MD, MPH
Associate Professor of Medicine
Clinical Director

Kathryn S. Torok, MD
Assistant Professor of Pediatrics

Patrizia Fuschiotti, PhD
Assistant Professor of Rheumatology

Fellows
Eleanor Valeni, MD
Pulmonary Fellow

Anna Papazoglou, MD
Rheumatology Fellow

Staff
Eileen Roth, MT
Biological Repository Manager

Maureen Laffoon, BS
Research Coordinator

Jennifer Peat-Fircak, RN
Research Coordinator

Kaila Schollaert-Fitch, MA
Research Coordinator

Kristi Kong, BA
Research Coordinator

Christina Morse, BS
Lab Manager

Tracy Tabib, MS
Senior Lab Specialist

Emily Mirizio, BS
Laboratory Technician

Advisory Group
Everette Curlee Virginia Curlee Gerald Dimmit
Sheldon Marstine Nancy Arthurs McDonald