

Scleroderma Foundation Launches “Patients as Partners” to Leverage the Voice of the Patient Advocates in Drug Development *and* Clinical Trials

The Scleroderma Foundation is pleased to announce “Patients as Partners,” a new program designed to help our patient community engage with pharmaceutical and biotech companies working on clinical trial designs and other drug development initiatives for scleroderma and related conditions. In addition to educating our pharmaceutical partners on the needs and challenges of the patient community to ensure trial success, the program aims to identify, train and engage people living with scleroderma to serve as advocates in drug development and clinical trial design.

Over the past two years, the Foundation has seen a significant increase in the number of national and international companies working on potential therapies for this disease. With this increased drug development pipeline comes a new opportunity for the scleroderma patient community.

“Pharmaceutical and biotech companies understand that effective clinical trial design and implementation require the input and perspective of the patient community,” says Robert Riggs, Scleroderma Foundation CEO. “The volume of requests for help from industry makes it clear that the Foundation’s members have an important role to play in the development of potential new therapies, which is why the Patients as Partners program is a necessary part of our work.”

Patients as Partners was born out of previous efforts to bring scleroderma patients into the clinical trials development process. “Some of our early engagement opportunities didn’t go as well as we had hoped,” says Riggs. The Foundation was asked by three different companies to do different things – either a focus group, a clinical trial protocol review, even a

session where patient representatives were barraged with questions from scientists wanting to learn more about the “typical” person with scleroderma. “I don’t think either side came out of those early meetings with what we had hoped for,” Riggs explains.

“We didn’t properly prepare our participants, and I don’t think the industry partners were effective in communicating at a lay-person level when presenting scientific data. I left those meetings knowing there was more that we could do as an organization to make sure that we helped advance the development of potential new treatments,” Riggs comments.

Taking the key learnings (both positive and negative) from those first meetings, Riggs partnered with patient advocate Karen Gottesman, who has broad experience as a patient consultant on drug advisory committees for the FDA. “Looking at the number of requests from pharma and seeing this huge unmet need, I reached out to Karen for help and guidance. In so doing, I stumbled on the perfect person to pull this program together,” says Riggs.

Gottesman is known to many within the Scleroderma Foundation community. Aside from her work with FDA advisory boards, she’s been a reviewer for the Department of Defense Peer Review Medical



Patient advocate Karen Gottesman has partnered with the Foundation to launch Patients as Partners program

Patients as Partners *continued...*

Southern California Chapter, where she focused on new program development, with an emphasis on patient and physician education. She is also the author of the Foundation's clinical trials brochure and the book, *The First Year – Scleroderma, An Essential Guide for the Newly Diagnosed*.

Gottesman and Riggs spent several months assessing the regulatory environment and researching the current and potential pipelines of clinical trials and the companies engaging in them. They also looked at the types of engagement activities the pharmaceutical industry was seeking from the Scleroderma Foundation and the role that patient advocates should play in clinical trial design and drug development.

"As a person with scleroderma, my passion is patient empowerment," Gottesman comments. "When Robert and I started looking at the number of potential drugs in early-stage development, we knew we needed to do more in order to be a relevant resource to industry. One of the most obvious needs was to identify and prepare patient advocates to have a meaningful voice in this important process."

Riggs and Gottesman looked at what other organizations were doing and soon realized that there was a real lack of programming. Tapping into this unmet need, the two developed a pilot program based on how patient advocacy organizations, such as the Scleroderma Foundation, can be effective partners in the entire spectrum of new therapies development, from clinical trial design to post-FDA approval.


"There is so much that we, as patients, bring to the table," Gottesman explains. "It's much bigger than just doing focus groups, although that's certainly an important initial first step. We need to make the patient voice heard throughout the process, including early clinical trial design, recruitment and participating in legislative and regulatory processes by providing patient-focused testimony. Patient advocates can also provide important insights into adherence

issues through peer-to-peer education initiatives that can result in better patient outcomes."

With initial funding support through an unrestricted educational grant from Actelion Pharmaceuticals, US, Inc., the Foundation was able to launch Patients as Partners with initial data-gathering efforts in late 2015 and active patient-advocate recruitment efforts in spring 2016.

Gottesman recently joined the program formally as the Foundation's director of pharma & biotech engagement, to spearhead the program. "Her skills and passion have already had a huge impact on the program, and the companies that we're working with are amazed at what the Scleroderma Foundation can bring to the table," says Riggs.

"We're in active conversation with nine companies in the United States and Europe," explains Gottesman. "We are setting up patient advisory boards and roundtable meetings, and we've completed two clinical trial protocol reviews. We are far busier this early into the process than we ever expected."

"It's exciting to be on the forefront of this type of synergy between patient groups and those who are developing the next generation of therapies," says Riggs. "We are starting to see the fruits of decades of work on the part of many people. It's really the coming together of basic scientists (those funded by the Scleroderma Foundation, NIH and other sources), pharmaceutical and biotech industries that develop those scientific findings into potential therapeutics, and clinicians that work on clinical trials. With Patients as Partners, we're bringing the patient voice to the mix in a more formalized way. It's the next step in our mission to find a cure for scleroderma," Riggs says. 

Learn More About 'Patients as Partners'

To learn more about this program and how you can get involved, please contact Karen Gottesman at kgottesman@scleroderma.org