



SSF Medical and Scientific Advisory Board

Chair

Theresa Lawrence Ford, MD, FACR

Members

Esen Akpek, MD
Penny A. Asbell, MD, FACS, MBA
Herbert S. Baraf, MD, MACR
Richard Brasington, MD, FACP
Michael Brennan, DDS, MHS
Steven E. Carsons, MD*
Nancy L. Carteron, MD, FACP
Troy Daniels, DDS, MS*
Denise L. Faustman, MD, PhD
H. Kenneth Fisher, MD, FACP, FCCP
Gary Foulks, MD, FACS
S. Lance Forstot, MD
Philip C. Fox, DDS*
Robert I. Fox, MD, PhD, FACP*
Tara Mardigan, MS, MPH, RD
Austin Mircheff, PhD
John Daniel Nelson, MD, FACS
Kelly Nichols, OD
Athena Papas, DMD, PhD
Ann Parke, MD
Andres Pinto, DMD
Nelson Rhodus, DMD, MPH
Vidya Sankar, DMD, MHS
Daniel Small, MD, FACP
Neil Stahl, MD
Frederick B. Vivino, MD, FACP
Jeffrey Wilson, MD, FACP

Associate Members

Simon J. Bowman, PhD, FCRP
Janine A. Clayton, MD
Arthur Grayzel, MD, FACP*
Roland Jonsson, DMD, PhD
Stuart S. Kassin, MD, FACP*
Robert Lebovics, MD
Michael Lemp, MD*
Xavier Mariette, MD
Haralampos M. Moutsopoulos, MD*
Manuel Ramos-Casals, MD, PhD
James J. Sciubba, DMD, PhD*
Harry Spiera, MD*
Leo Sreebny, DDS, MS, PhD*
Athanasios G. Tzioufas, MD
Ira J. Udell, MD*
Claudio Vitali, MD
Daniel J. Wallace, MD
Pierre Youinou, MD, Dsc

*Counselor



SSF Advancing Forward: Promoting Clinical Trial Development

by Theresa Lawrence Ford, MD, FACP

Chair, SSF Clinical Trials Consortium; Chair, SSF Medical and Scientific Advisory Board; and CEO and Medical Director, North Georgia Rheumatology Group, PC, Lawrenceville, Georgia

What a journey this has been. It hardly seems possible that three years ago we began directing efforts promoting the development and availability of clinical trials for patients suffering from Sjögren's disease. We are very proud of the progress made by the Sjögren's Syndrome Foundation (SSF) Clinical Trials Consortium (CTC) which first convened in May of 2014. I serve as the Chair of the CTC which is an international initiative whose mission is to increase the availability and the accessibility of therapies for treating Sjögren's. We started with a small international steering committee. However, we have expanded to include many members who are leaders in the Sjögren's community. Three major goals were developed to accomplish our mission:

- To support and promote objectives that facilitate the design of clinical trials;
- To increase industry partnerships with the SSF;
- To engage in dialogue with government agencies that oversee therapy approval. (i.e. FDA, EMA).



Theresa Lawrence Ford, MD, FACP



The latest initiatives of the SSF CTC are currently underway. The SSF is excited to be launching the first-ever online platform to train clinical trial investigators and, eventually, educate clinicians from multiple specialties who manage and treat Sjögren's patients. Called STEP, or "Sjögren's Training and Education Platform," this unique program will lead to more clinical trials in Sjögren's and higher quality trials. Ultimately, this new program will lead to greater interest in developing new therapies in Sjögren's on the part of pharmaceutical companies, because internationally-accepted training and models for trial execution will be easily available and accessible.

Currently, pharmaceutical companies must develop their own training programs for investigators leading their clinical trials. This approach takes a great deal of time and money and most often does not lead to the best and most consistent training. With STEP, clinical trial investigators will

share the same training. The STEP program will offer an online portal for clinical trial investigators to take an online course to learn how to collect data for clinical trials.

The design of this online platform will ultimately help speed the training of investigators and ensure that all investigators, no matter which clinical trial they are working on, will be collecting data in the same manner. Consistency in the way clinical trials are executed from one center to another and even from one trial to another is critical for determining if a therapy really works. Better methods for training was clearly identified by our corporate members and the SSF Clinical Trials Consortium.

STEP Initiatives Move Forward

Under STEP, we plan to:

1) Develop an online training program for current Sjögren's-specific outcome measures

STEP participants will be trained to use the leading international model for objective measurements in Sjögren's (ESSDAI) and its counterpart for patient-reported measurements (ESSPRI) that were recently developed specifically for Sjögren's. These measures are now the most frequently used indexes to gauge the effectiveness of therapies for Sjögren's. **This initial phase will be launched in the late fall or early next year.**

Once the basic platform is developed, the SSF will partner with pharmaceutical companies to develop personalized platforms for each company and its clinical trials. Investigators will take the online qualifying test to ensure that they meet the requirements for rating disease activity in Sjögren's patients according to standard testing devised by our SSF key opinion leaders and, in addition, according to requirements for a company's specific trial.

2) Develop online training programs for additional outcome measures that can be used in Sjögren's trials

Future plans to expand our offerings through the STEP platform include training on multiple outcome measures that might be used in clinical trials in Sjögren's. For example, outcome measures already exist for specific symptoms in Sjögren's, such as fatigue and cognitive function (or "brain fog"), and our hope is to add these to our online training either as they currently are or changed slightly to better fit Sjögren's. This will greatly expand our educational offerings to companies and clinical trial investigators to ensure successful clinical trial design.

3) Offer educational videos demonstrating how to perform Sjögren's-specific tests that can be used for clinical assessment of patients in a clinician's office and/or for use in clinical trials

Gaining knowledge and consistency in conducting Sjögren's-specific tests is critical for healthcare professionals. Properly and consistently performing tests such as a lip biopsy (and reading these biopsies), the Schirmer's test to measure dry eye, unstimulated and stimulated salivary flow measures, and skin biopsies for small fiber neuropathy will help medical providers in their diagnosis and treatment of Sjögren's.

Online Forum Will be Launched for Clinical Trials

A fourth priority set by the SSF CTC is to create an online communication forum for physicians and other support staff who are engaging in clinical trials. We are excited about this special venue for discussions among those involved in executing clinical trials and those who are considering becoming a clinical trial site. **The SSF expects to launch this forum this fall.**

SSF National Survey Spreads Awareness and Assesses Burden of Illness

An important part of the overall effort to ensure new therapies are developed for Sjögren's patients has been to increase education and awareness in the healthcare professional sector. In addition, a major goal has been to work closely with the pharmaceutical industry to demonstrate the tremendous disease burden that Sjögren's patients suffer and encourage them to investigate potential therapies for Sjögren's and engage in clinical trials. To demonstrate the challenge of the signs and symptoms experienced and the resulting functional limitations for patients, the Sjögren's Syndrome Foundation (SSF) conducted an extensive national patient survey.

The "Living with Sjögren's" survey was conducted in the United States by Harris Poll on behalf of the SSF between May 11 and July 11, 2016. Nearly 3,000

Editor

Katherine M. Hammitt, MA

Medical & Scientific Editor

Nancy Carteron, MD, FACP

Co-Medical & Scientific Editors

Vidya Sankar, DMD
S. Lance Forstot, MD

At-Large Medical & Scientific Reviewers

Esen K. Akpek, MD
Herbert Baraf, MD, MACR
Jeffrey Wilson, MD, FACP

SSF CEO

Steven Taylor

e-mail: sq@sjogrens.org

www.sjogrens.org



The *Sjögren's Quarterly*® newsletter is published by the Sjögren's Syndrome Foundation Inc., 10701 Parkridge Blvd., Ste. 170, Reston, VA 20191. Copyright ©2017 Sjögren's Syndrome Foundation Inc. ISSN 0899-637.

DISCLAIMER: The Sjögren's Syndrome Foundation Inc. in no way endorses any of the medications, treatments, or products mentioned in advertisements or articles.

patients (96% women), aged 18+ years in the U.S. and who reported having been diagnosed with Sjögren's by a medical professional or doctor, shared their experiences with the disease and its physical and emotional effects on their lives. This nationwide survey examined the variety and severity of experiences Sjögren's patients have and the impact on their quality of life. Not only the physical but the mental and financial burdens were addressed as well. The survey was highlighted during Sjögren's Awareness Month in April 2017, and a summary of findings can be viewed on the SSF website at <http://www.sjogrens.org/home/about-sjogrens/living-withsjogrensurvey>.

SSF Five-Year Breakthrough Goal Makes Major Progress

The SSF Five-Year Breakthrough Goal "to shorten the time to diagnose Sjögren's by 50% in 5 years!" started in 2012, and, four years into the goal, a 2016 survey showed that for patients diagnosed in 2015, the time to receive an accurate diagnosis of Sjögren's was brought down substantially from six years to three. In addition to increasing education and awareness in the healthcare professional sector, SSF initiatives have included increasing awareness in the public domain and increasing involvement from industry, stakeholders and partners. Our goals center around ensuring that Sjögren's patients are recognized by physicians for diagnosis and treatment and, subsequently, that Sjögren's patients are considered for clinical trials.

SSF Clinical Trials Consortium Paves the Way

To accomplish our goals, the CTC has engaged in regular discussions to assess barriers to clinical trials in Sjögren's. We scheduled teleconferences, as we did for our first meeting in May 2014, and face-to-face meetings, as we have done during the past three November SSF luncheon meetings during the American College of Rheumatology (ACR) annual conferences. The CTC met twice last fall, during the Biologic and Targeted Therapeutics in Sjögren's (BATTS) conference in Oklahoma at OMRF and again at ACR to discuss the SSF meeting with the FDA and the needs of companies and KOLS. New priorities were set as a result and included the development of the online educational platform called STEP.

In March 2016, we hosted a face-to-face CTC meeting of U.S. members and SSF corporate partners. During that meeting, launching a dialogue with the FDA was set as the initial top priority. The SSF built on discussions begun at a November 2014 SSF meeting during the ACR, when a regulatory update was given by the FDA and ensuing discussion raised the need for an FDA guidance document for drug approval in Sjögren's. Continuing the dialogue with the FDA last July, we dis-

cussed numerous options with the FDA that we could pursue with their input. Flexibility was encouraged.

The SSF CTC currently meets regularly with KOLS around the world to determine barriers to clinical trials in Sjögren's and strategies to tear down those barriers. Many barriers exist in getting new therapies to market, but tremendous progress has been made and current initiatives are underway. There has been increased interest and subsequent plans for new clinical trials. Development of biomarkers and novel diagnostics are in the pipeline and a priority for SSF research grant support. We now have internationally-accepted classification criteria, and internationally-accepted outcomes measures are being utilized with ongoing updates and improvements. (Clinical trial design for Sjögren's has made a major leap forward with the final validation of the EULAR-endorsed outcome measures, ESSDAI and ESSPRI.)

Industry Lends a Hand

Our industry partnerships are increasing as well as awareness that we can assist from the earliest stages of initial interest and discussions through identification of potential therapies, trial design, introduction to KOLS and potential trial sites and subsequent patient recruitment. Also, the SSF can help companies navigate government agency processes and post-approval marketing.

In turn, companies have offered critical input into the SSF CTC goals and initiatives. They bring a unique perspective to the barriers we all face, help us develop strategies to address them, and provide support for our efforts.

This journey has been both challenging and rewarding. Through the efforts of many, we are actively moving forward getting closer to our goals. The SSF thanks each one of you for your support in our continuing efforts. ■