

Center for Drug Evaluation and Research (CDER)
Virtual Public Meeting on Patient-Focused Drug Development for Systemic Sclerosis

US Food and Drug Administration

Tuesday, October 13, 2020

LIVE WEBCAST: <http://fda.yorkcast.com/webcast/Play/a992e187a44a4b6eafc1aad859153f4d1d>

AGENDA

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| 10:00-10:05am | Welcome
Robyn Bent, RN, MS, CAPT, US Public Health Service
<i>Office of the Center Director (OCD), Center for Drug Evaluation and Research (CDER), FDA</i> |
| 10:05 – 10:10 am | Opening Remarks
Nikolay Nikolov, MD
<i>Division of Rheumatology and Transplant Medicine (DRTM), CDER, FDA</i> |
| 10:10-10:20 am | Overview of FDA’s Patient-Focused Drug Development Initiative
Theresa Mullin, PhD
<i>Office of the Center Director (OCD), Center for Drug Evaluation and Research (CDER), FDA</i> |
| 10:20 – 10:30 am | Background on Systemic Sclerosis
Dinesh Khanna, MD, MSc
<i>University of Michigan</i> |
| 10:30-10:40 am | Overview of Discussion Format
Robyn Bent, RN, MS, CAPT, US Public Health Service
<i>OCD, CDER, FDA</i> |
| 10:40 – 11:10 am | Topic 1: Health Effects and Daily Impacts
A panel of patients and patient representatives will provide comments to start the discussion on health effects and daily impacts of systemic sclerosis. |
| 11:10 am – 12:15 pm | Large-Group Facilitated Discussion on Topic 1
Patients and patient representatives in the audience are invited to add to the dialogue. |
| 12:15 – 12:45 pm | Break |
| 12:45 – 1:15 pm | Topic 2: Current Approaches to Treatment
Patient perspectives on current approaches to treatment for systemic sclerosis. A panel of patients and patient representatives will provide comments to start the discussion. |
| 1:15 – 2:20 pm | Large-Group Facilitated Discussion on Topic 2
Patients or patient representatives in the audience are invited to add to the dialogue. |
| 2:20 – 2:25 pm | Closing Remarks
Raj Nair, MD
<i>Division of Rheumatology and Transplant Medicine (DRTM), CDER, FDA</i> |

DISCUSSION QUESTIONS

Topic 1: Health effects and daily impacts that matter most to patients

1. Which aspects of systemic sclerosis have the most significant impact on your life? (Examples may include tightening of the skin, painful cold sensitivity in hands and feet, i.e. Raynaud phenomenon, digital ulcers, heartburn, cough, shortness of breath, etc.)
2. Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your systemic sclerosis? (Examples of activities may include daily hygiene, engagement in personal relationships, participation in sports or social activities, completion of school or work activities, etc.)
 - a. How does your systemic sclerosis and its impacts affect your daily life on the best days?
 - b. On the worst days?
3. How has your systemic sclerosis changed over time?
 - a. How long have you had symptoms and how long has it been since you were diagnosed with scleroderma?
 - b. Would you define your systemic sclerosis today as being well-managed?
4. What worries you most about your systemic sclerosis?
5. If you could change one thing about your systemic sclerosis, what would it be?

Topic 2: Patients' perspectives on current approaches to treatment

1. What are you currently doing to help treat your systemic sclerosis? (Examples may include prescription medicines, over-the-counter products, and other therapies including non-drug therapies such as diet modification.)
 - a. How has your treatment regimen changed over time, and why?
 - b. What symptom would you most like to be improved or resolved by treatment?
2. How well does your current treatment regimen treat the most significant aspects of your systemic sclerosis? For example, how well do your treatments improve your ability to do specific activities?
3. What are the most significant downsides to your current treatments, and how do they affect your daily life? (Examples of downsides may include time devoted to treatment, side effects of treatment, route of administration, etc.)
4. Assuming there is no complete cure for your systemic sclerosis, what specific things would you look for in an ideal treatment for your systemic sclerosis?
5. What factors do you consider when making decisions about selecting a course of treatment?
6. Efficacy and safety are important for any treatment. When you think about a treatment for your disease:

- a. Does it make a difference whether:
 - i. the product might improve your most bothersome symptoms or
 - ii. whether the product might preserve organ function, or
 - iii. would you consider improvement in either of those areas equally worth the same level of risk?
- b. Does your acceptance of potential safety risks go up with the potential effectiveness of a product?
- c. Is taking a product with the least safety risk or with more efficacy most important?

SUBMIT A COMMENT TO THE PUBLIC DOCKET

We encourage participants to submit written comments to the public docket by December 15, 2020:
<https://www.regulations.gov/document?D=FDA-2020-N-1934-0001> or visit www.regulations.gov and search for FDA-2020-N-1934-0001.