Patient preferences and priorities for future therapies: An externally led patient-focused drug development meeting on sarcoidosis

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Introduction

The identification of patient preferences and priorities are a crucial element in the process of developing therapies that target outcomes important to the unique disease community.

This is especially true for sarcoidosis, where current treatments are not sufficient to address the challenges reported by patients or manifestations affecting multiple organs.

Sarcoidosis is an inflammatory disease characterized by the formation of granulomas in one or more organs of the body and is estimated to impact between 150,000 and 200,000 individuals in the U.S.[1]

As a rare, multi-system disease with considerable variability in disease course and severity, it can be difficult to capture the impact of sarcoidosis on patients and families. The Foundation for Sarcoidosis Research (FSR) is the leading international organization dedicated to finding a cure for sarcoidosis and improving care for sarcoidosis patients through research, education, and support. Since its establishment in 2000, FSR has fostered over $7 million in sarcoidosis-specific research efforts.

Methods

In October 2024, the Foundation for Sarcoidosis Research (FSR) hosted a virtual Externally Led Patient-Focused Drug Development (EL-PFDD) meeting for sarcoidosis.

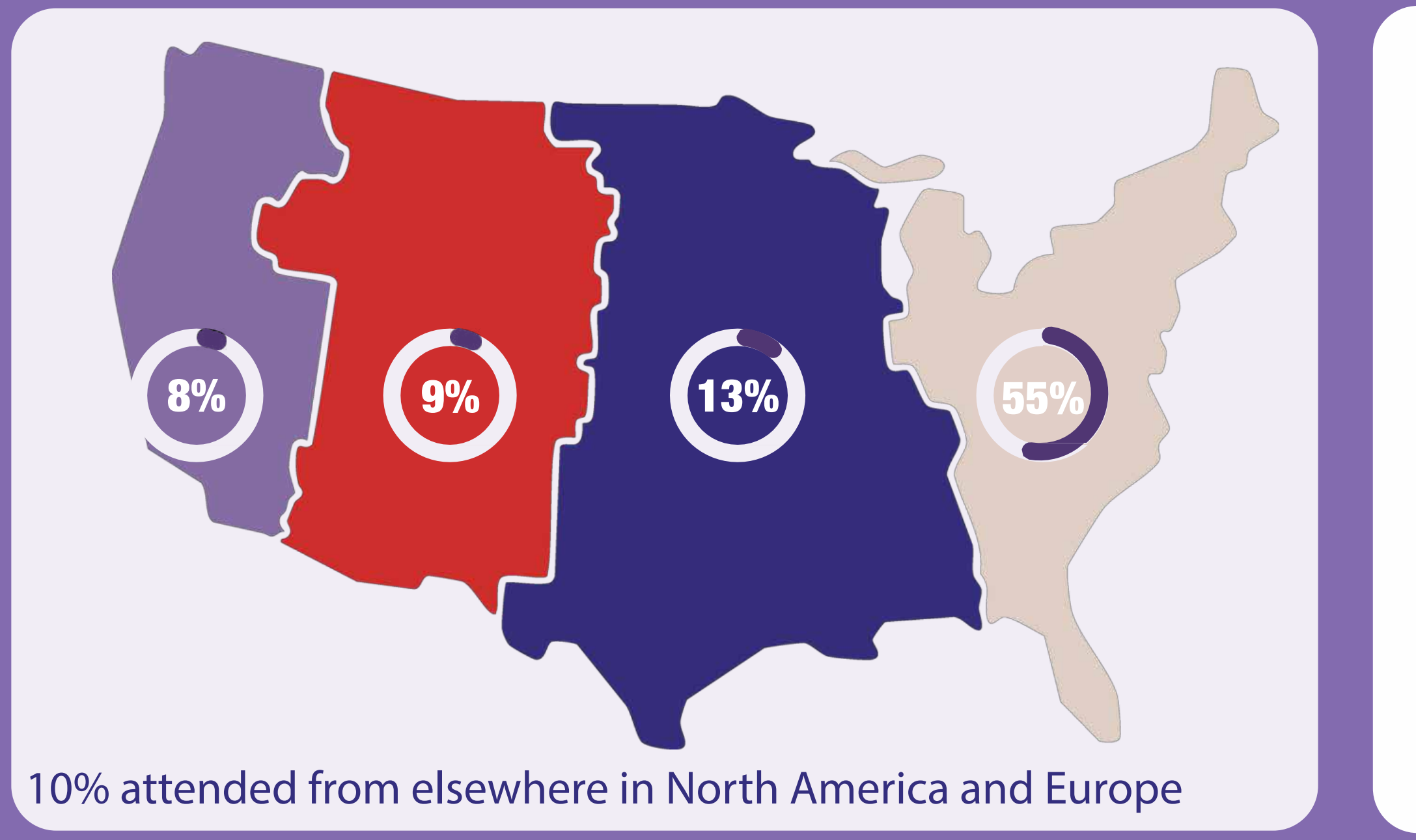
343 individuals with sarcoidosis, caregivers, family members, researchers, clinicians, government officials, and others attended. The meeting format included pre-recorded patient stories, live polling and call-ins, written comment submission, and live topic-specific panel discussions.

Participants shared perspectives on the daily impact of sarcoidosis, as well as needs and priorities for future approaches to treatments.

Results:

- Live participant polls revealed a predominantly female patient audience (79%), with the majority above 45 years of age (46% 55 -64 years, 25% 65+ years, and 15% 45-54 years).
- The meeting resulted in documented insights into the challenges resulting from sarcoidosis diagnosis, tolerance of current treatments and their side effects, and disease management.
- Additionally, facilitated discussions revealed lived experiences on these topics and provide a rich set of qualitative information to contextualize sarcoidosis and its unmet medical needs.

Figure 1: Meeting Attendee Location



10% attended from elsewhere in North America and Europe

Figure 2: Organs Involved

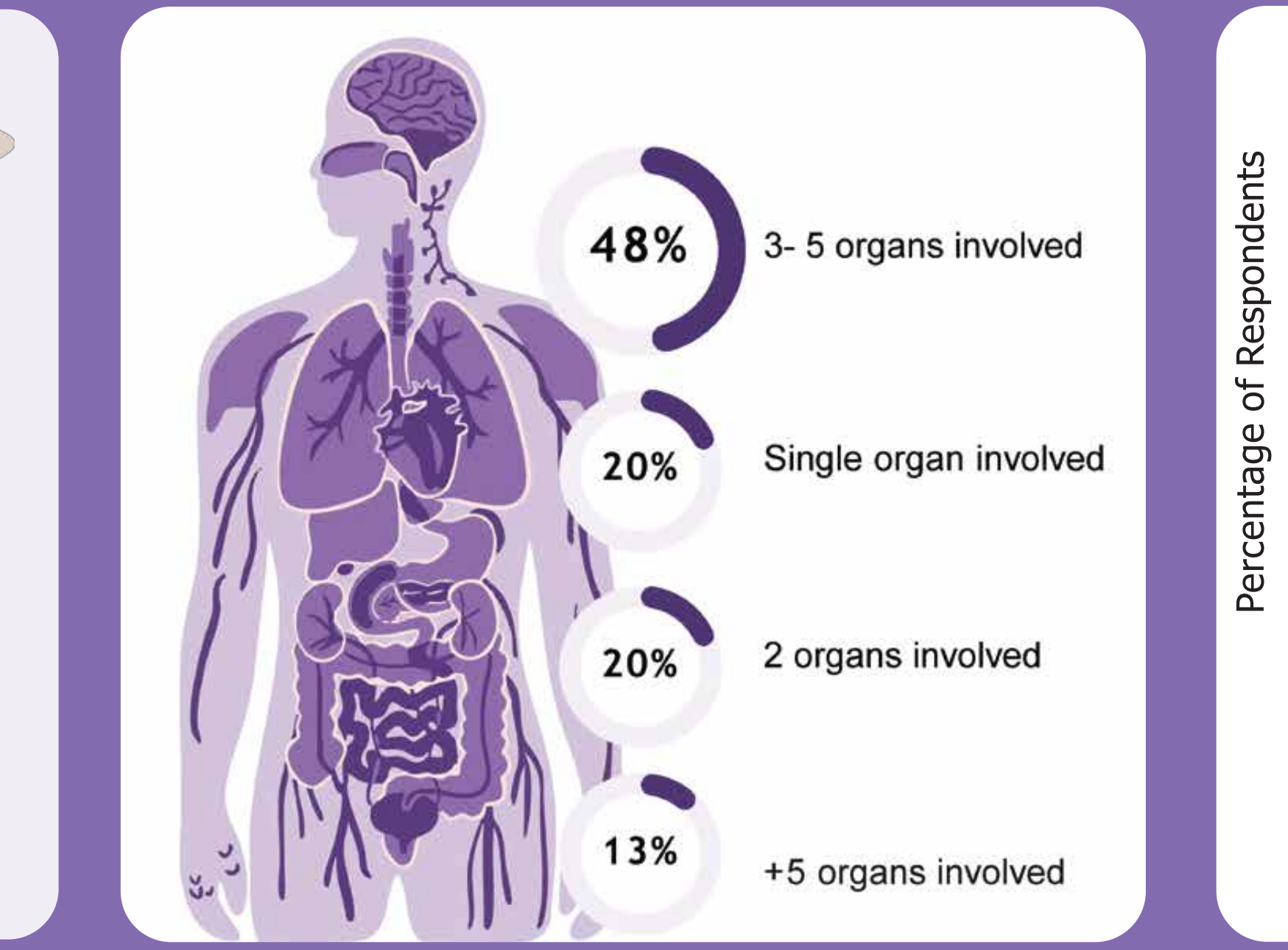
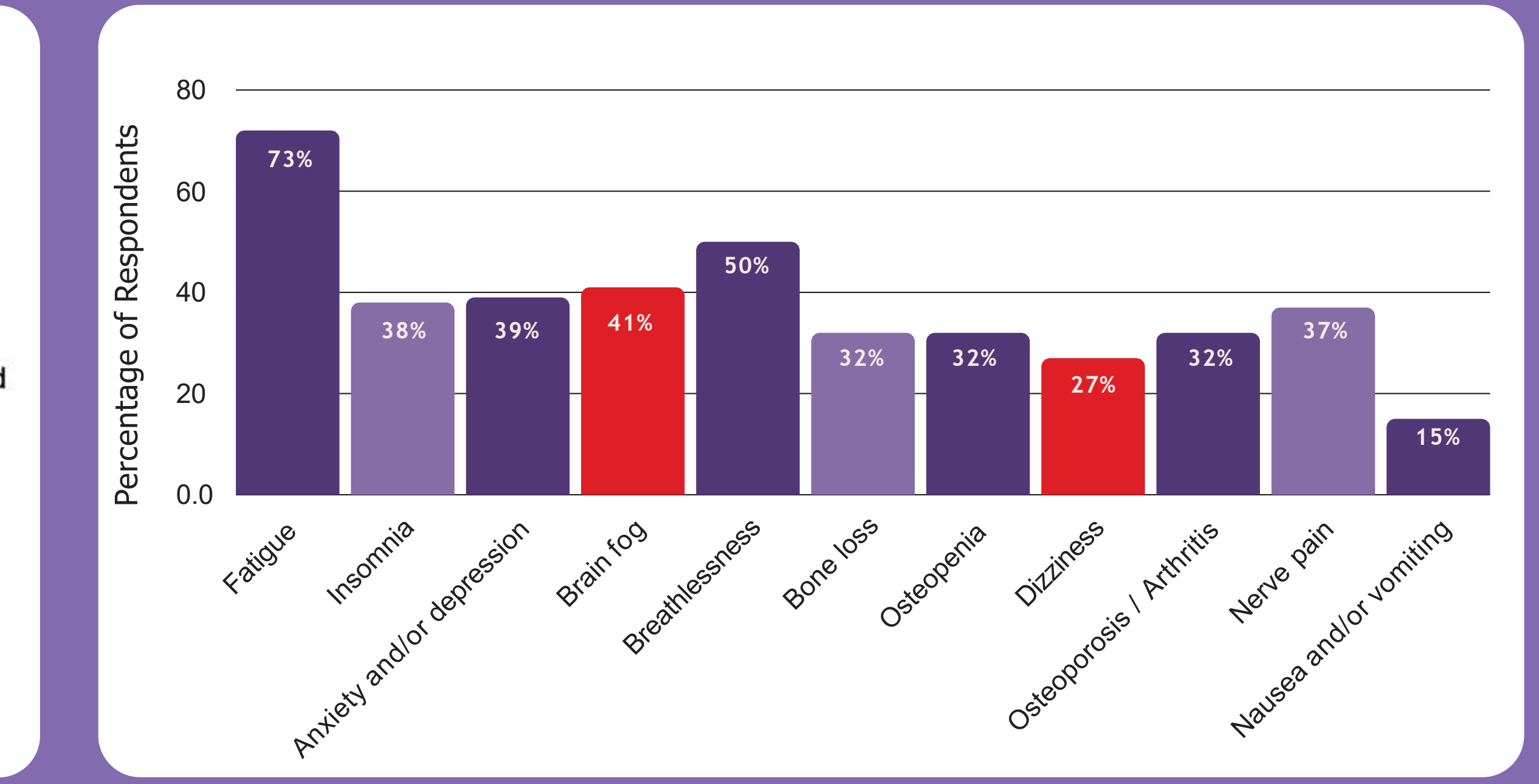


Figure 3: Which of the following symptoms have most impacted your life?



> *"I would like something that could make chronic sarcoidosis stable; so, I don't lose more parts of myself. That's the frustrating part for me, it started in one area, and then has taken different body parts, to where I'm having to use replacements. I feel like I'm losing pieces of myself as the years progress. I want something that could at least stop the progression."*

> *"A therapy that addresses fatigue is incredibly important. It's one of the most debilitating aspects of this disease."*

> *"Because it was a double-blind single-site trial, there was a chance I could receive the placebo. Would my sarcoid get worse? That was a big concern."*

Table 1: What have been the most challenging side effects of you or your loved one's current treatments? Select ALL that apply

Response options	Percent
Weight gain	**20.9%**
Insomnia	**19.0%**
Irritability	**17.1%**
Mobility challenges	**16.5%**
Damage to other organ (liver damage, heart disease,	**14.6%**
Incontinence	**10.1%**
None or not applicable	**1.9%**

Table 2: What would make you interested in participating in a clinical trial for sarcoidosis?

Response options	Percent
Therapy could slow or stop progression	**29%**
The trial allows for multiple organ manifestations of sarcoidosis	**18%**
Therapy addresses fatigue	**18%**
Therapy doesn't require me to stop current therapies or go on placebo	**13%**
Therapy addresses nerve pain	**5%**
Therapy addresses breathlessness	**6%**
I would never be interested in a clinical trial	**0%**

Conclusions

The EL-PFDD meeting on sarcoidosis brought together members of a rare disease community and provided unique insights that should be considered in drug development efforts.

- Participants joined the meeting from all over the country and the world, with most joining from the Eastern coast of the U.S.
- Nearly half of the patients impacted by sarcoidosis who attended reported complex disease involving 3-5 organs.
- Therapies are desired that slow or stop progression of sarcoidosis.

Key takeaways from the facilitated dicussion include:

- Inaccurate and imprecise diagnostic tools and the lack of biomarkers translate to lost years of care and irreversible damage to organs.
- Better options are needed other than long-term, high-dose steroids as the primary pathway to treatment.
- New therapies are needed that have different mechanisms to attack the inflammation and address the symptoms of sarcoidosis.
- Collaboration is needed to create new models of acceptable data, trial requirements, and endpoint selection that are responsive to the patient's needs.

> *"It's more than just the physical toll. The emotional and psychological weight is heavy too. The disease has distanced me from the life I once had."*

> *"I was forced to quit my job. The chronic pain, fatigue and complexity of managing my doctor's visits and treatments make it impossible for me to work."*

Acknowledgements and Resources:

EL-PFDD

FSR Homepage
StopSarcoidosis.org

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References
[1]https://www.stopsarcoidosis.org/what-is-sarcoidosis/