

SC Rare Disease Symposium



February 28, 2025
Program Agenda

9:30 Check in / Registration

10:00 Welcome & Opening Remarks

Patrick Flume, MD, Endowed Chair, Power-Huggins Endowed Chair for Cystic Fibrosis & Chair for the Rare Disease Advisory Council, Medical University of South Carolina (MUSC)

10:10 South Carolina's Rare Disease State Report Card

Carolyn Sheridan, MPH, State Policy Manager, Eastern Region, National Organization for Rare Disorders (NORD)

10:30 Effective Advocacy

Emily Keeney, Government Relations Advisor, Adams and Reese LLP

11:00 Journey of Discovery: An integrated model of care to find answers and develop treatments for rare genetic diseases

Dr. Mike Lyons, R. Boykin Curry Chair in Translational Genomics and Therapeutics; Director of Clinical Services; Senior Clinical Geneticist, Greenwood Genetics

Dr. Heather Flannagan- Street, Director of Functional Studies, Greenwood Genetics

11:40 Journey of Discovery Q&A

11:50 Lunch

12:15 Personal Stories/Advocacy Presentations

Building Hope: A decade of supercharging research and advocacy for Sanfilippo syndrome

Glenn O'Neill, Founder & CEO of the Cure Sanfilippo Foundation

The Other Side of Impossible

Tarah O'Sullivan, CEO and founder of the Drake Rayden Foundation

12:45 Personal Stories/ Advocacy Q&A

1:00 Off Label but On Target: Perspectives on and Examples of Drug Repurposing for Rare Diseases

Dr. Smith Heavner, Adjunct Assistant Professor, Sr Scientific Director, Real-World Evidence, Data Collaboration Center (DCC)

1:30 Symposium Q&A and Moderated Open Forum

2:00 Adjourn

We'd like your feedback!



We hope you enjoyed the 2025 Rare Disease Symposium!

Your feedback is valuable to us and helps us ensure future events are educational, engaging, and impactful. Please take a few minutes to complete our brief survey. Your insights will help shape the future of our symposiums and further support the rare disease community.

You can access the survey by scanning the QR code or by visiting
<https://redcap.link/rds2025>

Thank you for your participation!