

## Rare Diseases Advisory Council

October 25, 2024

### **Meeting agenda:**

1. Welcome
2. Introductions
  - a. Halley Harris was introduced as the new Administrative Coordinator
3. Newborn screening process presentation by Tanya Spells, Director of Newborn Screening Follow-up for S.C. Department of Public Health
  - a. Slides shared with council members
  - b. RDAC will post the link to SC newborn screening resources on the RDAC webpage
4. Updates from NORD's RDAC Member Meeting and the Rare Diseases and Orphan Products Breakthrough Summit (Dr. Neena Champaigne and Tara Pittman, attendees)
  - a. Key Take Aways from Members Only Meeting
    - i. The theme for this year's meeting was Equitable Access to Innovation
    - ii. 29 States now have RDACs
      1. Funding is an ongoing issue with initiatives
    - iii. Minnesota working on PA reform with Medicaid to eliminate the burden of refiling every 6-12 months for patients and providers
    - iv. Prescription Drug Affordability
      1. Cost of orphan drugs
  - b. New resource portal for RDAC members and administrators (includes slides from the 2024 member meeting): <https://rdac.rarediseases.org>.
5. SC RDAC Charter
  - a. The adding and removal of members
    - i. When/about what do we vote on? Do we need to wait for in person meetings? How do we define "inactivity" or ground rules for membership?
  - b. Dr. Flume requested volunteers to assist with finalizing charter.
6. Housekeeping and Additional Topics
  - a. Committee member progress on follow-up items from last meeting (see July meeting notes)
    - i. Jonathan Hawayek spoke with Carolyn Sheridan from NORD about helping us find a replacement member to represent RDOs. She will get back to us with potential suggestions.
    - ii. Jonathan Hawayek received codes for prevalence and incidence data available for 1 through 500 and requested for them to be more user friendly. He is expecting them to be ready in the next few weeks.
    - iii. Jonathan Hawayek was given Erica Barnes as administrative contact for Minnesota RDAC.

- b. Ideas on how to handle requests for information from the community
  - i. Looking for referrals/access to information, seeking patient connections for media opportunities, etc.
- c. Administrative Updates:
  - i. Needs assessment is being advertised through the Post & Courier (in print and digitally) across the state through the month of October.
    - 1. 13 ads across the state of SC
    - 2. 200,000 digital banners
    - 3. 10 completers of the survey, 98 people, 57 fully completers
    - 4. Vicki Young suggested sharing ad from Post & Courier and digital banner to advertise at Community Health Center sites, clinical sites, and newsletters
    - 5. Sonya Rigsby suggested partnering with Patient Advocacy groups to share in newsletters. Follow up on obtaining contacts from her.
  - ii. Segra Park in Columbia has been reserved, once again, for next year's SC Rare Disease Symposium to be held on February 28<sup>th</sup> (Rare Disease Day)
- d. Follow Up Items
  - i. Tara will be reaching out to Erica Barnes at University of Minnesota to see if someone from their RDAC is available to speak about the promotion of their needs assessment survey at our January meeting
  - ii. Ginie Daguise to get prevalence and incidence data from Jonathan Hawayek in next few weeks and take them to get epi data from the state (waiting for the full 500)
  - iii. Vicki Young and Patrick Flume to meet and discuss other state data available for review
  - iv. RDAC admin to organize specialized meetings to be held before the end of the year to finalize the charter and discuss the symposium. (Volunteers: Kate Wink, Karen Kemper, Jonathan Hawayek, and Ginie Daguise)

7. Adjourn

**Membership:**

Representation	Member	Presence
Chair	Patrick Flume, MD (MUSC)	P
Department of Health and Environmental Control	Ginie Daguise (SCDHEC)	P
Department of Health and Human Services	Kevin Wessinger (SCDHHS)	P
Medical University of South Carolina	Dan Judge, MD (MUSC, cardiology)	P
Prisma Health -University of South Carolina	Divya Ahuja, MD, MRCP (Infectious Diseases)	P
South Carolina Hospital Association	Kate Wink (Santee Cooper)	P

South Carolina Primary Healthcare Association	Vicki Young (SCPHCA)	P
Biopharma Industry	Jonathan Hawayek (SPARK Therapeutics)	P
Research and Treatment of Rare Diseases (3)	Maysen Mesaros, MS (MUSC, neuroscience) Neena Champaigne, MD (Pediatric Genetics) Chip Norris, PhD (MUSC, connective tissue)	A P A
Patient (2)	Karen Kemper, PhD (scleroderma) 2 <sup>nd</sup> patient N/A	P
Rare Disease Organization	N/A	
Caregiver of person with rare disease	Jason Smith (hypoplastic left heart syndrome)	A
State Health Plan	Tripp Jennings, MD (VP and Clinical Innovations Officer, BCBS South Carolina)	P
Program Coordinator	Halley Harris	P

**Non Member Attendance:** Tanya Spells, Sonya Rigsby, Michelle Meyer, Camerun Washington, Vyndra Smith, Samantha Darwak,