Rare Diseases Advisory Council Quarterly Meeting

April 11th, 2025
9:00 AM-10:00 AM

Meeting agenda:

- Welcome & Introductions
    - Yvonne Donald, M.A., CSCEC (James R. Clark Memorial Sickle Cell Foundation)
    - Cara O'Neill, MD, FAAP (Cure Sanfilippo Foundation) | Not Present
    - Kimberly Brown (RDAC coordination)

- Annual Report

  The RDAC annual report is due on June 30, 2025. The group discussed the SC Report Card from NORD, evaluating the state of South Carolina in comparison to surrounding states, and deciding which policy recommendations to prioritize in the report. The categories discussed include:
    - Medicaid Financial Eligibility
    - Medical Nutrition
        - The group discussed potential ways to impact medical nutrition outside of recommendations to the legislature, with specific consideration for what Florida has implemented.
        - Next steps:
            - Reach out to NORD to better understand what Florida has implemented and share with Dr. Wessinger.
            - Was able to find the medical coverage guideline for enteral formulas on the Florida Blue website which references the state mandate for coverage in this area.
    - Newborn Screening
        - The group discussed ongoing work and expected traction in the area of newborn screening. The group discussed monitoring an ongoing pilot in Florida which offers free genetic testing for newborns.
        - Next Steps:
            - Dr. Champaigne has reached out to the NBS Advisory Committee members to see if we can have and RDAC member join the committee. Tara and Dr. Flume have been cc'd on the email and will coordinate next steps after receiving a reply.
    - Prescription Drug Out-of-Pocket Costs
        - The group suggested that this be a prioritized recommendation in the annual report.
    - Protecting Patients in State Medicaid Programs
    - Protecting Patients in State Regulated Insurance
        - Next steps:
            - Here is a helpful description on short term limited duration insurance (STLDI) plans. In summary, they are short term plans that

are not regulated by the ACA or other federal insurance rules, meant to fill a gap in coverage (not like COBRA, which allows for a continuation of an existing employer insurance coverage). They are not comprehensive and are mostly unregulated at the federal level because they are not considered individual health insurance like ACA compliant plans sold through Marketplace. There was a finalized federal rule that came out in March of 2024 limits new STLDI plans, sold or issued on or after September 1, 2024 to three-month terms, and caps total durations (ie. renewals) at no more than 4 months. States can have stricter rules on STLDI plans than the federal rule, but not more lenient. SC had stricter regulations than federal rules from 2018- Aug 2024 but now federal rules are tighter with the changes that took place in Sept 2024.

- - - - - You can find a summary of the recent federal rule change, the reasons for the change, and where certain states fall in their restrictions of STLDI plans here.
      - NORD's suggestions for improving a grade in this area is to more strictly regulate short-term plans (beyond federal rules). Examples of policy options for doing so include: banning short term plans, further limiting plan duration, limiting "stacking" of different STLDI plans, implementing preexisting condition protections, limiting premium rating factors, limiting who can enroll, prohibiting rescissions..
        - Here is the link to NORD's webpage describing STLDI and their rubric for grading
  - Rare Disease Advisory Council
  - Step Therapy (Fail First)
    - The group discussed benefits and challenges related to step therapy and step therapy policy.
    - Next steps:
      - Reach out to NORD to see if they have data/evidence to demonstrate the hindrance of response time.
  - Telehealth
    - The group discussed the benefits and challenges related to telehealth.
    - Next steps:
      - The group suggested that this be a prioritized recommendation in the annual report, pending what Jon Hawayek finds out regarding context of previous legislative discussions related to the Interstate Medical Licensure Compact (IMLC).

- Charter & Succession Planning
  - Did not get to discuss this item. The charter was approved by council via electronic vote and RDAC will now adhere to those guidelines going forward.

- Adjourn

Mission: The council shall advise the Governor, the General Assembly, and other stakeholders on research, diagnosis, treatment, and education related to rare diseases

Definition: For purposes of this council, a rare disease is defined as one affecting fewer than 200,000 persons combined in a particular rare disease group

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)	A
Greenwood Genetics	Steve Skinner, MD (President & CEO)	P
Prisma Health -University of South Carolina	Divya Ahuja, MD, MRCP (Infectious Diseases)	A
South Carolina Hospital Association	Kate Wink (Santee Cooper)	A
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)	P P A
Patient (2)	Karen Kemper, PhD (scleroderma) Bridget Downing	P P
Rare Disease Organization	Yvonne Donald, M.A., CSCEC (James R. Clark Memorial Sickle Cell Foundation)	P
Caregiver of person with rare disease	Cara O’Neill, MD, FAAP (Cure Sanfilippo Foundation)	A
State Health Plan	Tripp Jennings, MD (VP and Clinical Innovations Officer, BCBS South Carolina)	P

Responsibilities of the Advisory Council:

- Solicit comments from stakeholders, including patients and patient caregivers in South Carolina impacted by rare diseases, to assess the needs of rare-disease patients, caregivers, and providers in the State;
- Consult with experts on rare diseases to develop recommendations to improve patient access to and quality of rare-disease specialists, affordable and comprehensive health care coverage, relevant diagnostics, timely treatment, and other needed services;

- Research and identify priorities related to treatments and services provided to persons with rare diseases in South Carolina and develop recommendations that include safeguards against discrimination for these populations on such issues, including disaster and public health emergency-related planning;
- Publish a list of existing, publicly accessible resources on research, diagnosis, treatment, and education relating to the rare diseases in South Carolina;
- Identify and distribute educational resources to foster recognition and optimize treatment of rare diseases in South Carolina; and
- Identify best practices to reduce health disparities and achieve health equity in the research, diagnosis, and treatment of rare diseases in South Carolina.
- Report annually (by June 30) to the Governor, the Chairman of the Senate Finance Committee, the Chairman of the Senate Medical Affairs Committee, the Chairman of the House Ways and Means Committee, and the Chairman of the House Medical, Military, Public and Municipal Affairs Committee.