

Rare Disease Advisory Council

March 26, 2024 | 2:00-3:00pm

Meeting Minutes

Members present: Lesley Bennett, Colleen Brunetti, Jim Carson, Mary Caruso, Kevin Felice, Joanna Gell, Emily Germain Lee, Dorian Long, Craig Miller, Miriam Miller, Michele Spencer-Manzon, Saurabh Vaidya

Others present: Wayne Cooke, Kevin Friert, Barry G, Carolyn Macica, Phil Marella, Nicole Nieves, Lisa Roy, Carolyn Sheridan, Myrna Watanabe

Introduction

- Members of the RDAC introduced themselves and stated their role on the RDAC
- Michele previewed the meeting and had Lesley summarize the discussion from the February meeting
- The meeting minutes were approved for February

Public Comment Period

- Wayne Cooke invited members to attend the walk to fight rare diseases at 10:00am on April 27, at Quinnipiac University campus – many organizations in the rare community will be present
 - Registration/sign up at jbsports.com or the Denise D’Ascenzo foundation website
 - Wayne Cooke spoke about the Lion’s club in Branford, CT – the Lions will be at the walk to fight rare diseases
 - As the largest civic organization, they have a long history of supporting vision, which has an overlap with rare conditions that impact sight
- Carolyn Macica shared a save the date for a Connecticut Children’s rare disease event:
 - 10th Annual Rare Disease Symposium, Friday October 18, 2024, from 1:00-5:00pm
 - Theme: pediatric to adult transition care
 - Carolyn has one spot left for the patient panel on the transition to adult care – reach out to rarediseaseday@quinnipiac.edu to contact her
- Carolyn Sheriden, representative at the National Organization for Rare Disorders (NORD) spoke about her work in helping states establish RDACs across her nation, and the supportive resources that NORD provides
- Kevin Friert provided information on Rare New England- they are hosting a Rare New England Annual Conference on Saturday, October 19, 2024
 - They are looking for a patient or parent to speak, reach out to kevin@rarenewengland.org for more information

Update on Rare Disease Day (February 28, 2024)

- Members spoke about how the event was smaller than in previous years, but it allowed for good conversation with legislators, and it was great to connect with rare disease patients

- Colleen emphasized the impact of the children who spoke, and expressed that the RDAC should look to hear from the voices of children more often
- The council expressed enthusiasm for hosting the event again next year

Jackson Labs Rare Disease Forum (March 7, 2024)

- Michele spoke about the Rare Disease Forum she attended that was sponsored by BioCT – really successful event with various panels representing a diverse array of expertise
- Themes and takeaways from Michele: reducing the time it takes to sequence, exome sequencing, diagnostics and therapies, the need to collaborate more in Connecticut, getting companies excited about working on rare diseases
- Craig added to Michele’s comments, saying that the event reinforced the vibrant scientific ecosystem in the state, and sparked optimism
- The group discussed increasing collaboration across industry to make progress in rare disease work

Legislative Updates

- SB 175: funding for the RDAC
 - has passed out of committee
 - The language contains \$50,000 dollars, and if the bill passes it will enable the RDAC to use outside funds
- HB 5367: Medicaid coverage on rapid whole genome sequencing for critically ill children
 - Added pediatric ICUs, defined the age as 0-12 months, and provided parameters to the DSS commissioner
- SB 206: Katie Beckett waiver expansion
 - Going before the appropriations committee
- SB 307: Medicaid coverage of Biomarker testing
 - Increasing the transparency of prior authorization
- SB 8: prescription drugs
 - Creates a prescription drug accountability board
 - There is no patient voice on this board, which could be dangerous down the road
- HB 5200: Access to diagnostic medical equipment
 - Mary Caruso has been following this bill; received a joint favorable report 3/22

Bylaws

- Miriam encouraged the group to think about the sustainability of the group and not be overly prescriptive in the bylaws
 - Colleen added to this comment, saying that some parts of the bylaws should be broken out into policy, rather than bylaws
- Craig, Colleen, and Lesley will coordinate the revisions of the bylaws to present on next meeting

New Business

- The group is interested in hosting a forum or public meeting during the fall of 2024, potentially in the Legislative Office Building

- Michele expressed that the RDAC should look to form subcommittees that leverage the differing expertise of members
 - It was recommended that the RDAC has standing committees, and then from there, ad hoc committees for short-term goals (for example, having a scientific forum)

First Public In-Person Meeting Update

- Michele expressed the importance of in-person the RDAC meetings, and offered to host at Yale for the April 23 meeting
 - Hybrid meetings will always be made possible
- Space in JAX is reserved for the RDAC May 28 meeting

Closing

- The Michele and Saurabh motioned to adjourn the meeting at 3:02pm