

Rare Disease Advisory Council

June 25, 2024 | 1:00-3:00pm

Meeting Minutes

Members present: Lesley Bennett, Jim Carson, Mary Caruso, Kevin Felice, Joanna Gell, Emily Germain-Lee, Dorian Long, Adrienne Manning, Craig Miller, James Rawlings, Michele Spencer-Manzon, Saurabh Vaidya

Members absent: Colleen Brunetti

Introduction

- Lesley opened the meeting at 1:02pm and invited RDAC members to introduce themselves.

Public Comment Period (1:00-2:00)

Summaries of Testimony

- Senator Saud Anwar
 - Sen. Anwar spoke about the importance of having a collective voice in the rare disease community to raise awareness and identify solutions.
- Meg Whitbeck, parent of a child with Gaucher disease
 - As a parent with a ten-year-old son with Gaucher disease, Meg spoke about the educational needs of students with rare diseases. Even if a child is not cognitively impaired, they still need supports – Meg highlighted issues like missed instructional time, missed specials and enrichment, and the social impact of going to school with a rare disease. Many parents do not know what they can do to get supports for their children.
- David Leeds, rare disease patient with HAE
 - David, a patient with Hereditary Angioedema with Normal C1-Inhibitor (HAE), spoke about the ability of rare disease patients to access FDA-approved drugs. He shared that the monthly copayment for his medications is over 30,000.
 - He hopes the RDAC can work on the prior authorization aspects of care.
- Pamela Johnson, HAE patient and caregiver of a son with HAE
 - Pamela spoke about HAE, saying that without treatment 1 in 3 people will die if they do not have access to medication. Her medications cost \$60,000 a month, and her son's cost \$40,000 a month. She spoke about reliance on four copay assistance programs and having to go without medication at times for financial reasons.
 - She testified about co-pay accumulators and co-pay maximizers on the federal level
 - The only benefactors of this model are the benefactors.
 - Connecticut is one of 19 states that has passed legislation to ban co-pay accumulators, but federally there is no legislation in place. Pamela is working at the federal level with Connecticut officials to set something in motion.

- John Flichak, adult living with MOGAD
 - John spoke about his experiences living with Myelin oligodendrocyte glycoprotein antibody-associated disease (MOGAD), including an adverse reaction following a COVID-19 booster shot. He wishes that the hospital staff were able to notify him about the resources and organizations that support individuals with his rare disease.
 - He brought up individuals who do not disclose their disease to their employer, afraid of discrimination.
 - John highlighted the importance of community hospitals in treating patients with rare diseases, before they can get to a larger hospital to isolate the disease and receive more specialized treatment.
 - He also mentioned a few districts in NYC that began teaching about rare diseases in the classroom to raise awareness.
- Mary Caruso, caregiver to two children with Friedreich's Ataxia
 - Mary spoke about her experiences caring for two children with Friedreich's Ataxia, ages 34 and 37.
 - She highlighted the social experiences of her children's peers being afraid of them and ostracizing them.
- Saurabh Vaidya, alongside his son with hemophilia A
 - His son testified about his experiences living with hemophilia A - getting injections and a port.
 - As a parent, Saurabh spoke about the moment he learned that his son had hemophilia A and would need twice weekly infusions for the rest of his life.
 - He emphasized the importance of the support his family received from the hemophilia society.

RDAC Meeting

Roll Call of Members Present

- Members of the RDAC introduced themselves and shared their role on the RDAC.

May Meeting Minutes

- Craig moved to approve the May meeting minutes and Saurabh seconded.
 - The meeting minutes passed unanimously without edits.

Review and Vote on Bylaws

- Craig commented on the structure of the bylaws, including the three standing committees – Executive Committee, Communications and Awareness Committee, and Legislative and Policy Committee.
- Emily Germain-Lee clarified the purpose of the Legislative Policy Committee, in relation to a legislative task force relating to the Katie Beckett Waiver program.
- James Rawlings asked a clarifying question on the appointment to an executive committee, since a lot of members fall as both healthcare providers and patient advocates.

- Lesley and Craig clarified that the executive committee has a breakdown of both co-chairs, a DPH representative, and two members at-large (one from the healthcare sector, and one from the patient advocates).
- The group agreed that how members are classified would rely on which slot they were appointed to fill.
- Michele motioned to vote on the bylaws, Craig seconded

In favor of passing bylaws	Abstained
<ol style="list-style-type: none"> 1. Lesley Bennett 2. Mary Caruso 3. Adrienne Manning (DPH) 4. Kevin Felice 5. Joanna Gell 6. Emily Germain-Lee 7. Craig Miller 8. James Rawlings 9. Michele Spencer-Manzon 10. Saurabh Vaidya 	<ol style="list-style-type: none"> 1. Jim Carson (DOI) 2. Dorian Long (DSS)

- Lesley encouraged members to think about if they would like to run for the executive committee at the next meeting
- Discussion of open and closed voting
 - Michele expressed that she would like the voting for executive committee to be closed (anonymous), or have the names be drawn out of a hat
 - James Rawlings expressed that he would prefer if the voting was open and transparent, so that everyone can know who cast their vote for which person
 - Lesley added that the co-chairs were selected by an open vote, so it would make sense to continue with this precedent
 - The topic of voting will be a continuing discussion

Legislative Update and Information Session

- Rapid Whole Genome Sequencing
 - On July 11 there will be a meeting at UConn to talk about rapid whole genome sequencing, members should get in touch with Lesley Bennett for more details or if they would like to attend.
- Katie Beckett Waiver
 - Lesley asked members if they would be interested in sitting on the Katie Beckett legislative taskforce.
 - Michele will share interested patients and their families to Lesley to be recommended as members on the task force.
- Prescription Drug Affordability Boards
 - Lesley spoke about a bill from the 2024 legislative session (SB 8) that she believes will be an important issue in 2025.

- PDA boards do affordability reviews, and they also use AI or clinical algorithms to measure the drugs. These methods use what is called a QALY (quality adjusted life-year), which often discriminate against the elderly and rare disease patients.
- Typically, the boards have five members, but Lesley would like to propose adding a physician (someone who is actively prescribing these drugs) along with a patient voice on these boards.
 - Minnesota's PDA board has seven members – a rare disease patient from MN's RDAC, and a physician who prescribes.
 - Lesley recapped the activities of several other states' PDA boards for the group's knowledge of the issue.

Rare Disease Survey Draft, Annual Report, & New Business

- Since the council has now received a lot of input from the public over the past three meetings, Michele is going to send out a short, open-ended survey to start putting ideas together on what the council should be focusing on.
 - Group members discussed having an open document versus a survey.
 - Having a survey might rank it by prioritization, which can give the group a better sense of the main issues.
- Michele spoke about the upcoming report in the fall, and asked if any members were interested in working with the co-chairs on the RDAC's annual report.
 - Craig expressed interest.
 - Lesley will approach Colleen as well.
- Lesley expressed that the executive committee will likely take the lead on the report.
- James Rawlings expressed that there should be updates on sickle cell disease in the annual report; Lesley asked if James would be willing to have a provider or researcher speak on sickle cell disease gene therapy at an upcoming meeting.

New Business:

- Lesley asked if members wanted to combine July and August meetings or take off one of the summer meetings; She also proposed that the group combine November and December dates into a single meeting during the first week of December to avoid holiday weeks.
- The group agreed to keep the both the July 23rd meeting, and the August 27th meeting.
- The group agreed to meet on December 10th, 2024 for the November/December meeting.
 - Michele motioned and Saurabh seconded the motion to combine Nov/Dec into one meeting on December 10th. The motion passed unanimously.

Closing

- The council members agreed to move remaining agenda items to the July meeting.
- Dorian motion to close the meeting, Emily seconded the motion.
- The meeting adjourned at 3:07pm.