Rare Disease Advisory Council

March 25, 2025 | 2:00-3:00pm

Meeting Minutes

Members present: Lesley Bennett, Mary Caruso, Emily Germain-Lee, Colleen Brunetti, Michele Spencer-Manzon, Jim Carson, Joanna Gell, Adrienne Manning.

Members absent: Dorian Long, Craig Miller, Kevin Felice, James Rawlings (Delores Edwards attended in his absence).

Introduction

- Lesley Bennett called the meeting to order at 2:02pm.
- Members of the RDAC introduced themselves and stated their role on the RDAC.
- Lesley summarized the February meeting and the Rare Disease Day event at the Legislative Office Building last month.
- Lesley also announced the chairs of two upcoming subcommittees: Mary Caruso will chair the Advocacy Committee, and Colleen Brunetti will chair the Awareness Committee.

Public Comment

- Kathleen Flynn, representing the National Tay-Sachs & Allied Diseases Association (NTSAD), thanked the group for the invitation to speak at the Capitol last month.
- Rep. Tracy Marra let the group know that HB 6919, a bill adding Duchenne muscular dystrophy (DMD) to the newborn screening panel, got JF-ed to appropriations. She encouraged members to support the bill.

Undiagnosed Diseases Network (UDN) Update

- Michele Spencer-Manzon shared that 6-7 months ago, Yale became a UDN site. They have not yet enrolled patients, but they have received applications for the network, and they are reviewing the applications for enrollment.
- She noted that the UDN is an NIH-funded program, and they may lose funding for some critical components of the program. She will continue to keep the group updated.

Advocacy Committee Update: Legislative Priorities & Testimony

- Mary Caruso updated the group on the Advocacy Committee. They are hoping to get a formal subgroup up and running to help people advocate to their elected representatives in the legislature.
- Some of these priorities include:
    - HB 6920: Patient specific emergency protocols
    - HB 6978: Includes a provision that allows the RDAC to solicit funds
    - HB 7108: Autism and intellectual disability definition changes

        - The group discussed the conflation of autism and intellectual disability, when there are vast differences.
    - SB 1473: Sickle cell gene therapy
        - DSS did submit the application for the CMS Cell and Gene Therapy (CGT) Access Model in time. The agency submitted it on March 6th, and the deadline was March 14th.
    - SB 11/HB 7192: Prescription drug task force, prescription drug affordability board
    - SB 7219: Protection of 504 plans in the Education Committee
        - Colleen shared that a coalition of 18 states have banded together to get rid of 504 protections. The group should look into why states are doing this, so that their advocacy can be based in decisions made across the country.
        - The RDAC discussed the purpose of repealing 504 plans at the national level.
- Mary will send out the remaining bills that RDAC members are engaged with in an email to the group.

Awareness Committee Update

- Colleen Brunetti shared that for this committee, there is interest in mapping rare diseases, spreading news and having events.
- Colleen let the group know that there are seven members of the public interested in serving on the Committee.
- This Committee will aim to start up at the end of April.
- Lesley Bennett brought up the Denise D'Ascenzo Walk to Fight Rare Diseases. She would like to put together an RDAC team for the walk.
    - More information here: https://thedenisefoundation.com/walk-to-fight-rare-diseases
- Alison Leeds brought up that the Connecticut Rare Action Network is planning a family picnic on May 10 at winding trails in Farmington, from 1-4pm. She will share the flier with Lesley to distribute to RDAC members.
- Wayne Cooke brought up Lions Club involvement in advocacy for the rare diseases related to sight.

Closing

- The meeting adjourned at 3:10pm.