



Rare Disease Advisory Council Meeting Minutes

Wednesday, April 23, 2025
11:00 a.m. EDT
Virtual

Welcome/Introductions:

The meeting was called to order at 11:06 a.m., EDT by Melissa Jordan, MS, MPH, Assistant Deputy Secretary for Health for the Florida Department of Health, and Chair of the Rare Disease Advisory Council (Council). Chair Melissa Jordan gave a summary of the meeting agenda.

The following members or designees were in attendance: (Quorum was established)

Melissa Jordan, MS, MPH, Assistant Deputy Secretary for Health, Florida Department of Health (Chair)
Eric Biernacki, JD, Managing Shareholder, Andrews Biernacki Davis
Ann Dalton, Assistant Deputy Secretary for Medicaid Policy, Agency for Health Care Administration
Jonathan Hawayek, MBA, Director, State and Local Government Affairs, Spark Therapeutics, Inc.
Anita Davis, PT, DPT, CNPT, Physical Therapist, Brooks Rehabilitation
Alexis Bakofsky, MA, Deputy Commissioner, Florida Office of Insurance Regulation
India Holroyd, MPH, Cabinet Affairs Director, Florida Department of Agriculture and Consumer Services
Colleen Bartlett, DNP, BayCare Chronic Complex Clinic
Mustafa Tekin, MD, University of Miami Miller School of Medicine
Barry Byrne, MD, PhD, UF Health Advanced Therapeutics and Powell Gene Therapy Center
Rajan Wadhawan, MD, Advent Health

Guests and Staff

Jon Conley, Strategic Initiatives Manager, Florida Department of Health
Yolanda Bonds, Rare Disease Advisory Council Staff Liaison, Florida Department of Health
Kristine Craze, Contractor, Florida Department of Health
Christina Samper, Regulatory Advocacy Manager, AdventHealth
Bridgette Morton, PhD, Deputy Director, Public Health Research, Florida Department of Health
Gavin Grigg, Division of Children's Medical Services, Florida Department of Health
Aleska Fernandez, Corporate and Investment Banking Intern, Citi

Business

1. Approval of Meeting Minutes:

Chair Melissa Jordan asked for a motion to approve the minutes from February 20, 2025. The meeting invitation included a copy of these minutes. Jonathan Hawayek made a motion to approve the February minutes, and Alexis Bakofsky seconded the motion. The members approved the minutes with no objections.

2. Discussions

A. Subcommittee Reports on Council Recommendations

Academic Research Institutions Subcommittee – Dr. Mustafa Tekin said the Subcommittee reviewed last year's recommendations and those from earlier this year. He said the group identified several goals that had not been met regarding rare disease research and clinical care. The Subcommittee agreed to address these gaps by recommending the creation of a Florida-based Cancer Center of Excellence program as high priority. The recommendation is to recognize centers that address multiple rare diseases through basic, translational, and clinical research.

Goals for the Cancer Center of Excellence in Rare Disease Research would include:

- To create sub centers doing research, studies, clinical care or translational studies
- To create a repository for patients, families, physicians and researchers to identify clinical trials in the state by sites that are closer in proximity and appropriate for the patients
- To promote interaction among rare disease researchers
- To identify funding sources to increase successes of developing treatments and interventions
- To provide families a resource for access to information regarding clinical trials so they can identify sites that may be closer to them or more appropriate

Dr. Byrne mentioned the Florida Legislature is considering a proposed Sunshine Genetics Act pilot to assist health care providers by identifying rare diseases in newborns and children more rapidly, accurately, and efficiently. If implemented, it provides an opportunity for Florida to be a national leader in early diagnosis and therapy that exists for single gene defects and common problems approached by gene therapy.

Health Care Providers Subcommittee – Dr. Anita Davis mentioned the Subcommittee has struggled to reach a quorum recently. She then summarized their review of the 2024 recommendations and efforts to streamline future recommendations. The Subcommittee identified the following needs concerning health care providers:

- Awareness to diagnosing and treatment, educating the health care providers
- Provide educational resources and referrals to other providers
- Streamline a list of screening tools (resources, articles, Continuing Medical Education websites)
- Ways to disseminate the resources and tools

Ann Dalton raised similar priorities from the State Agencies Subcommittee regarding screening for rare diseases and referrals to specialists. She suggested the possibility of collaborating with the Health Care Providers Subcommittee and outreach to the Agency for Health Care Administration (AHCA) and the Medicaid Program.

State Agencies Subcommittee – Chair Melissa Jordan spoke for the State Agencies Subcommittee regarding the idea of making more information available through a website or through web resources with the assistance from Florida Department of Health (DOH) staff and Rare Disease Advisory Council (Council) members in collecting data that would be helpful for individuals and families. DOH staff could assist in pulling together an inventory of useful data to share with the rare disease community from various sources including Department of Education, AHCA, Medicaid, and DOH programs. She reminded the group about the completion of the top 50 rare disease data listed in the last report as well as the focus of the top 500 rare diseases and conditions that will be in the 2025 annual review.

Chair Melissa Jordan mentioned the efforts to go through each recommendation and streamline them to ensure they are realistic and achievable.

Dr. Colleen Bartlett offered her services to engage students who are looking for internships and other opportunities to gain experience in health care and public health by assisting the Council in its efforts.

Chair Melissa Jordan would like to postpone the votes on recommendations until DOH staff compiles information in a document. Jon Conley mentioned that a survey will go out within seven days for formal voting on information to be included in the annual report.

B. Andrew John Anderson Pediatric Rare Disease Grant Program - Application Prioritization

Chair Melissa Jordan shared a brief overview of the Guidelines and Conflict of Interest Policy that the Council approved on October 7, 2024. Staff reviewed the statutory requirements for evaluating

applications. While member recusal is based on the honor system, failure to comply could result in application denial if a member fails to disclose a conflict or does not recuse themselves from the review process. Documents were included in the meeting invitation.

Bridgette Morton, Deputy Director of Public Health Research for the Florida Department of Health, explained the review process and the system for rating applications. Dr. Mustafa Tekin asked a question about presenting the list of applicants to determine any conflicts for Dr. Mustafa Tekin in the voting process.

Council members were asked to recuse themselves and temporarily leave the meeting if they were associated with the applicants. Based on the guidelines, members are to explain their recusal and exit. Dr. Mustafa Tekin announced his association with University of Miami and exited the meeting. All other members were present during the discussion and voting.

Chair Melissa Jordan asked that the members would be recused of the entire discussion based on the broad conversation of all applicants. Members could return at the stated time per Chair Melissa Jordan. She explained how the list could be prioritized based on the following three factors: 1. Other funding exists for the project, 2. Amount of the grant, and 3. External rating scores. After the discussion, Chair Melissa Jordan summarized the recommendation and put it to a vote.

Chair Melissa Jordan: Based on the discussion and vote, the Council put forward application #50006 as the pediatric rare disease program grant recommendation for the current fiscal year, including an alternate application, #50004, in case the primary awardee secures funding prior to these grant funds becoming available.

Dr. Rajan Wadhawan offered a motion to accept the recommendation, and Eric Biernacki seconded the motion. Members who voted to accept the recommendation were:

Chair Melissa Jordan
Dr. Rajan Wadhawan
Jonathan Hawayek
Eric Biernacki
Dr. Anita Davis
Dr. Colleen Bartlett
Alexis Bakofsky
India Holroyd

There were no objections.

Chair Melissa Jordan mentioned two institutions that were funded last year that returned to report updates at the fall meeting. She is soliciting ideas on how the Council can continue to highlight institutions and gather updates on how the funds are impacting the world of research.

3. Public Comment/Open Discussion

No public comments were received.

4. Adjournment

Chair Melissa Jordan asked for a motion to adjourn. Dr. Rajan Wadhawan offered the motion, with a second from Eric Biernacki. The meeting concluded at 12:20 p.m., EDT.

Commented [MA1]: Is this supposed to be him?

Commented [YB2R1]: Yes, I mentioned his name in the same sentence.

"Dr. Mustafa Tekin asked a question about presenting the list of applicants to determine any conflicts for Dr. Mustafa Tekin in the voting process." He is the one that asked the question. Please advise.