



## Rare Disease Advisory Council Meeting Minutes

**Thursday, February 20, 2025**

11:00 a.m. EST

Virtual

### **The following members or designees were in attendance:**

Melissa Jordan, Deputy Assistant Secretary, Florida Department of Health

Alexis Bakofsky, Chief of Staff, Florida Office of Insurance Regulation

Adam Anderson, Representative, Florida House of Representatives

Colleen Bartlett, BayCare Chronic Complex Clinic

Dr. Barry Byrne, Associate Chair of Pediatrics and Director of the Powell Gene Therapy Center at the University of Florida

Ann Dalton, Bureau Chief of Medicaid Policy, Agency for Health Care Administration

Rebekah Dorr

Jonathan Hawayek, Spark Therapeutics

Dr. Blake Shay, Pharmacy Manager at St. Joseph's Women's and Children's Hospital, BayCare Health System

Jennifer Sutherland

Dr. Mustafa Tekin, Professor and Chair, Department of Human Genetics, University of Miami Miller School of Medicine

India Steinbaugh Holroyd

### **Guests and Staff:**

WFSU-TV The Florida Channel

Jon Conley, Florida Department of Health

Yolanda Bonds, Florida Department of Health

Anne Grace Depaolo, Metz Husband & Daughton PA

Christina Connelly-Hooker, Takeda Pharmaceuticals

Kristine Craze, Florida Department of Health

Christina Samper, Advent Health

Matt Harju, Mirum Pharmaceuticals

Aleska Fernandez (Unverified)

### **Welcome/Introductions:**

The meeting was called to order at 11:04 am ET by Melissa Jordan, Assistant Deputy Secretary for the Department of Health, and Chair, Rare Disease Advisory Council (Council). Ms. Jordan welcomed members to 2025 and outlined the purpose of the meeting. She also highlighted Rare Disease Day, which was February 28, 2025, referencing materials from National Organization for Rare Disorders website that were included in the meeting packet, and provided an update on Pediatric Rare Disease Grant program.

Yolanda Bonds, Council Coordinator took roll call and confirmed the presence of quorum.

### **Business**

#### **1. Approval of December Minutes:**

Chair Jordan asked for a motion to approve the minutes from December 11, 2024. Motion by Jonathan Hawayek, seconded by Dr. Mustafa Tekin.

## **2. 2025 Annual Report/Subcommittee updates:**

Chair Jordan explained to the members that starting with today's meeting, she requests that subcommittees chairs provide updates from their respective meetings.

Dr. Tekin, Chair of Academic Research Institutions subcommittee reported that his most recent meeting was canceled due to office closure (weather). However, he mentioned the issue surrounding NIH grants and the lack of federal funding which could impact states as they continue to research rare diseases.

Chair Jordan, who chairs the State Agencies subcommittee, gave a brief introduction and overview of Florida Health Charts data portal, pointing out rare disease data that has been added statewide on county and some sub county levels. She encouraged members to visit and explore the site and suggested the Council work to identify additional data for the site that would be useful.

## **3. Discussion Topics**

### **a. Rare Disease Workforce Needs**

Chair Jordan mentioned the collaboration with academic partners to work with graduate or master level students or perhaps assist with an internship for someone interested in doing a project with the Council. DOH can send emails to the public health schools that might be interested in working with the Council with assessments or surveys.

### **b. Leveraging Council Resources**

Mr. Conley highlighted the composition of the Council, from the perspective of having a combination of researchers, care providers, policy makers and individuals impacted by rare disease and mentioned the need for the Council to maximize and leverage available resources based on the range of Council expertise. He encouraged the members to lean into their unique networks of colleagues, institutions and organizations to support the Council's initiatives and recommendations.

Members discussed the use of other state RDAC surveys to use as templates, particularly Pennsylvania and Colorado, instead of creating a Florida survey from scratch. Jonathan Hawayek volunteered to survey other state RDACs to determine best practices that may assist Florida in survey creation and distribution.

Mr. Conley mentioned that there is a State Agencies subcommittee, and a joint meeting between Academic Research and the State Agencies subcommittee might provide opportunities to refine the work in developing the survey mechanism.

### **c. Annual Report Work Process**

Mr. Conley provided an overview of the process that the Department of Health would be using to facilitate the work on the annual report. He mentioned that staff will be scheduling subcommittee meetings in March and April to focus on refining, redeveloping, and/or modifying the current recommendations. The subcommittee chairs will prioritize and finalize this information and report it to the Council for a final approval by mid-May. Routing of the report will begin by the end of May.

### **d. Update on the Top 51-500 most prevalent rare diseases**

Jonathan Hawayek presented the top 51-500 prevalent rare diseases to the Council. He shared that the data was collected and included ICD10 and NDC codes. The data were compiled from national and international sources as to prevalence and incident rates for each condition.

## **4. Next Steps/Subcommittee Meeting Dates**

Chair Jordan proposed subcommittees meet once every other month to encourage full participation, productive and interactive meetings. Mr. Conley mentioned that subcommittee meetings may need to continue meetings monthly until the annual report process is completed.

**Reminder:** Chair Jordan mentioned in April the Council will hold a meeting to review and prioritize the pediatric rare disease grant applications once a final timeline for the scientific review is complete.

**5. Public Comments/Open Discussion:**

No public comments were received.

**6. Adjournment:**

Chair Jordan asked for a motion to adjourn. Rep. Anderson offered the motion, Dr. Tekin and Jonathan Hawayek seconded the motion. The meeting concluded at 12:55 pm