Rare Disease Advisory Council Meeting Minutes

Wednesday, July 17, 2024
11:00 a.m. EST
Virtual via Microsoft Teams

1. **Welcome/Introductions and Roll Call:**
   Council members and guests were welcomed, and roll was called.

The following members[1] or designees were in attendance:
Representative Adam Anderson
Eric Biernacki
John Mattson *(designee for Ann Dalton, Agency for Health Care Administration)*
Dr. Anita Davis
Rebekah Dorr
Jonathan Hawayek
Kathy Hebda, Vice-Chair
Melissa Jordan, Chair
Dr. Scott McClelland
Dr. Divya Patel
India Steinbaugh

Guests and Staff:
Yolanda Bonds, Florida Department of Health
Robert Brooks, Florida Department of Health
Jon Conley, Florida Department of Health
Maggie Dilger
Aleskia Fernandez
Jennifer Garzia
Meigs Lamb, Florida Department of Health
Anna Grace Lewis
Paula Libassi
Holly Murphy
Kemi Olabisi
John Omick
Lisa Rawlings
Christina Samper

Opening Remarks:
Chair Melissa Jordan announced the 2024 RDAC Annual Report was submitted on time to the Governor and shared with the council. She thanked the council for its work over the past year.

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[1] The statute defines specific individuals for the RDAC and several are vacant including: a geneticist practicing in this state; a registered nurse or advanced practice registered nurse who is licensed and practicing in this state with experience in treating rare diseases; a hospital administrator from a hospital in this state which provides care to individuals diagnosed with rare diseases; a pharmacist who is licensed and practicing in this state who has experience with drugs that are used in the treatment of rare diseases; a representative of the Office of Insurance Regulation.

Chair Jordan updated council members that staff had been researching collaborations with other state RDACs regarding website development, patient survey creation/dissemination, and additional processes that have been successful.

Minnesota's Rare Disease Advisory Council has had the largest response rates to date on its patient survey with more than 1000 participants. Chair Jordan noted a possibility of having a member from the Minnesota RDAC present at a future Florida RDAC meeting. Staff shared Minnesota's website in the Teams chat for council members and the public to view. Members were encouraged to give feedback.

Chair Jordan also asked the council to think about resources within the group that could help facilitate previously set goals. She posed the idea of collaborating with a graduate school to recruit master's students with an interest in rare diseases to work with the council.

2. **Approval of Meeting Minutes:**
   Jon Hawayek made a motion to approve the April 24, 2024, meeting minutes, and Representative Adam Anderson seconded. The motion carried and the minutes were approved unanimously.

3. **Discussion Topics:**
   a. **2024 Annual Report Review** – Jon Conley, Strategic Initiatives Manager, Florida Department of Health
   Jon Conley provided a brief overview of the 2024 Annual Report.

   Mr. Hawayek updated the RDAC that the data set of the top 51 – 500 most prevalent rare diseases globally should be received in the next 60 days and would be shared with the council when available. Mr. Hawayek asked if the council could ensure policy makers are aware of the work of the council and the Annual Report. Representative Anderson echoed Mr. Hawayek's interest in finding ways tor present a 2024 Annual Report summary and asked if Chair Jordan or Mr. Conley could present at a future health care committee during committee week. Chair Jordan agreed that a presentation to share the implementation of the statute and the continued effort tasked by the legislature would be helpful.

   Vice-Chair Kathy Hebda complemented the hard work of the council.

   b. **Update on Andrew John Anderson Pediatric Rare Disease Grant Program** – Robert Brooks, Director of Public Health Research, Florida Department of Health

   Robert Brooks updated the council on the Andrew John Anderson Pediatric Rare Disease Grant Program outlined in Section 381.991, Florida Statutes. The purpose of the grant program is to advance the progress of research and cures for rare pediatric diseases by awarding grants through a competitive, peer-reviewed process. Grants will be awarded by the Department after consultation with the Rare Disease Advisory Council based on scientific merit, as determined by a competitive, peer-reviewed process to ensure objectivity, consistency, and high quality.

   Mr. Brooks explained that the grant program's funding is $500,000 annually and is based on funds available. The program is for research specific to rare pediatric disease and to advance innovative approaches for research and treatment of rare pediatric diseases. Awards will be available exclusivity to Florida-based institutions and preference may be given to proposals that foster collaboration among institutions, researchers, and community practitioners. Any balance at the end of the fiscal year is available for carrying out the purposes of the trust fund.

   Mr. Brooks also noted that the Rare Pediatric Disease Research Grant Program Funding Opportunity Announcement was published in January 2024. After the announcement, there were 29 applications reviewed and scored based on: Overall Impact, Health Impact, Pediatric Rare Diseases Relatedness, and Infrastructure. Two grants were awarded based on overall scores and scientific merit.

For Fiscal Year (FY) 2023-2024, the application priorities were: Reduction of mortality and morbidity in relation to disproportionately impacted individuals; improved screening accuracy and detection in high-risk groups; creation of consortia to collaboratively work on rare pediatric diseases; research with a focus on prevention and improved treatment of care delivery that contributes to a reduction in deaths; research that contributes to reductions in deaths due to rare pediatric diseases resulting from disproportionately impacted individuals due to race, ethnicity, or income; improved screening accuracy, detection of high-risk subgroups, and/or improved implementation of a screening program that results in an increase in early detection or prevention. The total funding requested in FY 2023-24 application was $8,694,235.

Notice of the funding opportunity and the application cycle for FY 2024-25 should be released soon. The RDAC's responsibilities include establishing and following rigorous ethical guidelines and adhering to a strict policy regarding conflicts of interest. The Department will engage a vendor for peer review of applications and will consult with the RDAC regarding final awards. The Department will then announce the awards and finalize agreements.

Representative Anderson asked if the third-party peer review group is the same for other grant programs within the Department, and Mr. Brooks confirmed it is. Mr. Brooks explained the benefits of using the same reviewers, which includes helping find subject matter experts on rare applications.

Chair Jordan called attention to the council having a part in the grant process. She explained the requirement of the council to develop guidelines on the review process that would ensure fairness and ensure there were no conflicts of interest on any member of the council or their organizations. She noted there would be time later in the meeting to discuss creating a subcommittee specific to this new task of the council.

c. **Medicaid Whole Genome Sequencing Update** – John Mattson, MGHP, FCCM, PMP, Agency for Health Care Administration

Mr. Mattson reviewed the 2023 General Appropriations Act which was effective January 1, 2024. The Agency for Health Care Administration will reimburse for rapid whole genome sequencing provided to Medicaid recipients who are: 20 years of age or younger; have a complex or acute illness of unknown etiology that has not been caused by environmental exposure, toxic ingestion, an infection with normal response to treatment, or trauma; and are receiving inpatient treatment in a hospital ICE of high-acuity pediatric care unit.

The following three codes were covered beginning January 1, 2024:

CPT Code	Description	Fee Schedule
81425	Test for detecting genes associated with disease, genome sequencing analysis	$3,018.72
81426	Test for detecting genes associated with disease, genome sequencing analysis, each additional comparator genome	$1,625.97
81427	Reevaluation test of previously obtained genome sequencing	$1,402.59

He noted this service will be reimbursed at a rate in addition to the hospital inpatient reimbursement for diagnostic related group (DRG) payment, in accordance with the 2023 General Appropriations Act. For fee for service (FFS) claims, independent laboratories and hospitals will be reimbursed for rapid whole genome sequencing with a Date of Service on or after January 1, 2024, that have a corresponding inpatient claim billed with revenue code 310 (OPH-PATHOLOGY/GENERAL). SMMC plans have the flexibility to reimburse covered services through additional codes and to negotiate mutually agreed upon reimburse rates for rapid whole genome sequencing with their network of contracted providers.

Mr. Hawayek asked if Mr. Mattson was aware of any managed Medicaid plans that have chosen to be less restrictive. Mr. Mattson explained they had not had any complaints where the costs are not being paid on the fee schedule. One complaint they have received is that DRG payment is not sufficient to cover some of these more expensive testing codes.

d. **Discussion of Creating Additional Subcommittees** – RDAC Members
Chair Jordan opened up the discussion of potential new subcommittees to tackle council goals relating to a website/repository, patient survey, data, and the new Andrew John Anderson Pediatric Rare Disease Grant Program (which would need to meet by mid-August).

Dr. Rajan Wadhawan suggested the Academic Research Institutions Subcommittee could provide insight on a potential grant program subcommittee. Chair Jordan suggested staff should send out information that could gauge interest and ensure there are not conflicts of interest.

Representative Anderson volunteered to be on the subcommittee that would support the Andrew John Anderson Pediatric Rare Disease Grant Program.

Mr. Hawayek volunteered to serve on the data and repository subcommittees.

Mr. Conley said a council membership survey would be distributed after the meeting to ask for volunteers and further suggestions to determine subcommittees.

Dr. Anita Davis asked for clarification on the skill sets needed to serve on each subcommittee. Council staff will create guidance that will accompany the survey.

e. **Member Updates** – RDAC Members

There were no member updates provided.

4. **Public Comments/Open Discussion:**
Paula Libassi thanked the council for their hard work in the chat.

5. **Closing Remarks**
Melissa mentioned the following tentative meeting dates:
8/14/2024 – Health Care Providers Subcommittee Meeting
8/15/2024 – State Agencies Subcommittee Meeting
8/16/2024 – Academic Research Institutions Subcommittee Meeting
10/7/2024 – Possible hybrid meeting during FSU Discovery Days

Representative Anderson mentioned the opening of the Pediatric Rare Disease Institute during FSU Discovery Days and possible sponsorships.

6. **Adjournment:**
Mr. Hawayek motioned to adjourn the meeting, the motion was seconded, and the meeting was adjourned at 12:06 p.m. EST.