



Meeting Minutes

Rare Disease Advisory Council (RDAC) Meeting

Date: Wednesday, April 24, 2024

Time: 11:00 AM – 1:00 PM EST

Microsoft Teams Video Conference

Meeting Participants ¹

RDAC Council Member	Present (Y/N)
Rep. Adam Anderson	Y
Eric Biernacki	Y
Dr. Barry Byrne	N
Ann Dalton	Y
Dr. Anita Davis	N
Rebekah Dorr	N
Zana Dupee	N
Jon Hawayek	Y
Kathy Hebda, Vice-Chair	Y
Melissa Jordan, Chair	Y
Dr. Scott McClelland	N
Jessica O'Reilly	N
Dr. Divya Patel	N
India Steinbaugh	Y
Jennifer Sutherland	N
Dr. Mustafa Tekin	Y
Dr. Rajan Wadhawan	Y

Guests: Charles Aiken (UCB Biopharmaceutical), Nicholas Alford (Florida Department of Health), Dr. Pradeep Bhide (Florida State University), Jon Conley (Florida Department of Health), Maggie Dilger (Florida Department of Health), Erik Doroteo (Florida Department of Health), Leslie Dughi (Metz, Husband, & Daughton, P.A.), Brielle Dozier (Artia Solutions), Emily Grosshans (Florida Department of Health), Julie Hardie, Matt Jarju (Mirum Pharmaceuticals), Dierdre McCarthy (Florida State University), Kemi Olabisi (Acadia Pharmaceuticals), John Omick (Traverse Therapeutics), Thomas Perkins (UCB Biopharmaceutical), Mary Petrillo (Johnson & Johnson), Matt Sherwood (UCB Biopharmaceutical), Delilah Thompson, Jade Tindle (Florida Department of Health), Cynthia Vied (Florida State University).

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.

Call to Order, Member Roll Call – *Melissa Jordan, Chair*

The meeting was called to order at 11:01 a.m. EST by Melissa Jordan, RDAC Chair. Roll was taken and a quorum was established.

Approval of Council Meeting Minutes – *Council Members*

Chair Jordan asked for a motion to approve the February 7, 2024, meeting minutes. A motion was made by Representative Adam Anderson to approve the minutes and the motion was seconded by Eric Biernacki. The meeting minutes were approved unanimously.

Subcommittee Breakout Sessions – *Council Members*

Members were released to three separate meeting rooms for 35 minutes to consider annual report recommendations. When the full Council reconvened, subcommittee members provided a summary of their discussions.

2024 Annual Report Recommendations Review – *Council Members*

Chair Jordan opened the discussion by reminding council members the RDAC's Annual Report is due to the Governor and State Surgeon General by July 1, 2024.

The Academic Research Institutions Subcommittee made three recommendations that are included in the appendix. During the discussion of these recommendations, RDAC members offered the following input:

- Using the FL Cancer Center of Excellence designation as a model for a Rare Disease Research Institution designation.
- Creating an objective to encourage grantees to share data and information.
- Adding the ClinicalTrials.gov to the RDAC website.
- Creating a recommendation that newborn screening include whole genome sequencing.

The Health Care Providers Subcommittee made three recommendations that are included in the appendix. During the discussion of these recommendations, RDAC members offered the following input:

- Combine Recommendation 3 with the Academic Research Institutions Subcommittee Recommendation 3, as it relates to promoting education on rare diseases and enhancing support resources for patients, families, caregivers, and add health care providers.

The State Agencies Subcommittee made six recommendations that are included in the appendix. During the discussion of these recommendations, RDAC members offered the following input:

- Consider the possibility a presentation to Florida government officials about RDAC and its work.
- Highlight the RDACs accomplishments in the annual report.

Guest Presentation FSU Institute for Pediatric Rare Disease – *Dr. Pradeep Bhide, PhD, Florida State University*

- Chair Jordan introduced Dr. Pradeep Bhide with the FSU Institute for Pediatric Rare Diseases. Dr. Bhide explained the program is just beginning and plans include a health center, a master's degree program in Genetic Counseling, a genomics diagnosis and research facility, and a biomedical research program.

Future Agenda Items for Discussion – *Council Members*

Chair Jordan mentioned possible presentations from other state RDACs and how those councils surveyed

the rare disease communities in their states. She also mentioned the Academic Research Institutions Subcommittee may want to hear from the Cancer Centers of Excellence Designation group in Florida.

The next council meeting is tentatively scheduled July 17, 2024.

Public Comment

There was no public comment.

Adjournment – *Melissa Jordan, Chair*

The meeting adjourned at 12:56 p.m.

Meeting Appendix: Subcommittee Recommendations

Academic Research Institutions Subcommittee: Dr. Mustafa Tekin explained the Academic Research Institutions Subcommittee's recommendations as follows (notations are in parenthesis for any suggested changes/updates).

- Recommendation 1: Support research institutions in Florida
 - Identify the components that constitute a best practice "Rare Disease Research Institution."
 - Elicit feedback on developed components for a best practice "Rare Disease Research Institution."
 - Centralize developed components of a best practice "rare disease research institution" on a publicly available website.
 - Identify and promote best practices established by rare disease research institutions on a publicly available website.
 - Encourage continued funding for Pediatric Rare Disease Grant Program funding. (*Note: This is a new addition suggestion.*)
- Recommendation 2: Promote the availability of research institutions to individuals with rare diseases.
 - Promote expanded access to genome sequencing for all individuals with rare diseases.
 - Promote timely access to research clinical trials for individuals with rare diseases. (*Note: This objective was reworded to provide clarity.*)
 - Develop a database of research institutions that are available within a geographic location (ZIP code, city, region) on specific rare diseases.
 - Centralize resources on a publicly available website.
 - Create a community of practice amongst research institutions and individuals with rare diseases who are looking to be involved in research studies.
- Recommendation 3: Promote education on rare diseases to health care providers.
 - Identify state and national rare disease experts available to consult with health care providers on complex rare disease cases.
 - Explore the availability and use of technology for consultation between health care providers and rare disease experts.
 - Centralize consultation resources for health care providers on a publicly available website.

Health Care Providers Subcommittee: There was not a quorum of members available for this subcommittee. Based on notes taken by India Steinbaugh through discussions with the facilitator, only technical changes were brought back to the full council.

- Recommendation 1: Promote education on rare diseases to health care providers.
 - Identify existing educational resources, including curriculum in medical and nursing schools, and continuing education courses for health care providers on various topics related to rare diseases, including resources on patient-centered care and holistic medicine.
 - Centralize educational resources on a publicly available website.
- Recommendation 2: Promote advancements in the process of achieving a diagnosis for rare diseases.
 - Identify screening tools for health care providers on rare diseases.
 - Develop resources, such as infographics for health care providers upon suspicion of a rare disease through the process of referral, diagnosis, and treatment.
 - Develop resources, such as infographics for individuals with rare diseases and their families that explains referral, diagnosis, and treatment process for rare diseases.
- Recommendation 3: Promote education on rare diseases to health care providers. (*Note: The subcommittee suggested changing from "health care providers", which implies practitioners to "patients, families, and caregivers. The council concurred with the revision.*)
 - Identify current medical practices and specialists who accept both pediatric and adult individuals with rare diseases.
 - Identify barriers and challenges to medical practices and specialists accepting both pediatric and adult individuals with rare diseases.
 - Develop a list of resources for individuals with rare diseases on how to self-advocate during interactions with health care providers.

- Survey individuals with rare diseases to identify barriers to access to care for testing and treatment.
- Promote the inclusion of advocates, case managers, and interdisciplinary care teams to assist in facilitation during interactions with health care providers.
- Identify how rare diseases affect an individual long-term, such as pain management, household needs, workplace, lifestyle adjustments, and overall quality of life.
- Identify or develop a list of resources for individuals with rare diseases for mental and emotional support.
- Develop a list of resources for individuals with rare diseases, and their families, including but not limited to, patient advocacy groups that support the rare disease community.
- Publicly post a list of resources for individuals with rare diseases for mental and emotional support.

State Agencies Subcommittee: Vice Chair Kathy Hebda shared the following updates and changes to the State Agencies recommendations.

- Recommendation 1: Identify data needs necessary to understand the population with rare diseases in Florida.
 - Define the prevalence/incidence of rare diseases in Florida.
 - Develop a list of rare diseases and identify the distribution of incidents across the state, beginning with the top 50 most prevalent rare diseases. (*Note: The number should be updated to 500. Jon Hawayek is confident Spark will have 51-500 data to share with the council soon.*)
 - Develop a list of International Classification of Diseases, Tenth Edition codes for conditions covered by the Florida Newborn Screening Program. (*Note: This objective has been completed and should be moved to the accomplishments section of the Annual Report.*)
 - Develop a list of resources across the continuum of care that serve individuals with rare diseases such as public services, support organizations, educational support systems, employment services, etc. (*Note: This objective has been completed and should be moved to accomplishments section of the Annual Report. Jon Hawayek will send the document with the listed resources to council staff to keep on file for posting on the future website.*)
 - Identify Florida specific data from national rare diseases using the current available databases in Florida. (*Note: This is an ongoing objective. The Medicaid data has been obtained, but OIR and State Employee data is pending.*)
 - Determine where Florida falls in comparison to other states regarding rates of rare diseases.
- Recommendation 2: Identify areas of improvement for the Department of Health (DOH) on rare diseases.
 - Identify Department of Health programs that serve the population with rare diseases. (*Note: This objective has been completed and should be moved to the accomplishments section of the Annual Report.*)
 - Identify the population with rare diseases the Department serves.
 - Develop a needs assessment survey on the population with rare diseases FDOH serves.
 - Identify barriers and gaps in services that impacts individuals with rare diseases that are serviced by FDOH.
 - Publish data on rare diseases through FDOH data portals, such as FLHealthCHARTS.ORG.
 - Develop training for FDOH staff to better serve the population with rare diseases.
 - Develop an evaluation plan.
- Recommendation 3: Identify areas of improvement for the Agency for Health Care Administration (AHCA) on rare diseases.
 - Identify Medicaid data that can be used to determine the incidence/prevalence of rare diseases in Florida.
 - Identify Medicaid programs that serve the population with rare diseases.
 - Identify the population with rare diseases in Florida Medicaid serves and assess its needs.
 - Identify how current surveys are being utilized to collect feedback from Medicaid recipients with rare diseases in relation to receipt of Medicaid services. (*Note: This objective has been completed and should be moved to the accomplishments section of the Annual Report.*)

- Research potential opportunities to improve Medicaid programs or services and address barriers and gaps.
- Develop training for Agency staff to better serve Medicaid recipients with rare diseases.
- Develop an evaluation plan.
- Recommendation 4: Identify areas of improvement for the Florida Department of Education (FDOE) on rare diseases.
 - Identify FDOE programs that serve the population with rare diseases and identify any barriers or gaps in services. (*Note: This objective has been completed and should be moved to accomplishments section of the Annual Report.*)
 - Develop a resource guide to educate public and private school staff, students with rare diseases, and families on available resources and supports such as Individualized Educational Plans, 504 Plans, and scholarships offered.
 - Develop a communication plan to include the distribution of available resources and supports to meet the needs of students with rare diseases and their families. (*Note: Keep. This will be in collaboration with FDOE, FDOH, AHCA to get education/medical information to schools.*)
 - Develop training for FDOE staff to better serve the population with rare diseases.
- Recommendation 5: Identify areas of improvement for the Florida Office of Insurance Regulation (OIR) on rare diseases.
 - Identify what data on rare diseases currently exist through commercial health insurance plans.
 - Identify the population with rare diseases OIR serves and assess its needs.
 - Identify if current surveys are being utilized to collect feedback from health insurance recipients with rare diseases.
 - Identify OIR programs that serve the population with rare diseases.
 - Evaluate requirements for insurance benefits not to be discriminatory against insurance recipients with rare diseases.
 - Research potential opportunities to improve health insurance programs or services and address barriers and gaps.
 - Develop training for OIR staff to better serve health insurance recipients with rare diseases.
- Recommendation 6: Identify areas of improvement for disaster preparedness and pandemic response for individuals with rare diseases in Florida.
 - Complete a needs assessment of the populations with rare diseases in Florida for natural disaster preparedness and pandemic response.
 - Identify local community resources individuals with rare diseases may contact in the event of an emergency for assistance.
 - Develop educational and training materials on the needs of individuals with rare diseases for emergency shelter coordinators and staff.
 - Develop educational materials for individuals with rare diseases on emergency preparedness planning to be shared with partner agencies, such as information on the Florida Special Needs Shelter Registry, importance of having a checklist for individuals with rare diseases that highlight specific items they may need during a disaster.