



Rare Disease Advisory Council Meeting Minutes

Monday, October 7, 2024

12:00 p.m. EST

Hybrid Meeting – Florida State University College of Medicine and Microsoft Teams

Welcome/Introductions:

Melissa Jordan, Chair, opened the meeting and Alma Littles, M.D., Dean of Florida State University's College of Medicine, welcomed the group.

The following members or designees were in attendance:

Eric Biernacki
Dr. Barry Byrne
Ann Dalton
Dr. Anita Davis
Zana Dupee
Jonathan Hawayek
Kathy Hebda, Vice-Chair
Melissa Jordan, Chair
Dr. Divya Patel
India Steinbaugh
Dr. Mustafa Tekin
Dr. Rajan Wadhawan

Guests and Staff:

Yolanda Bonds, Florida Department of Health
Jon Conley, Florida Department of Health
Dr. Shanta Dhar, University of Miami Miller School of Medicine
Maggie Dilger, Florida Department of Health
Marnie George, Johnson and Blanton
Gavin Grigg, Florida Department of Health
Anna Grace Lewis, Metz, Husband & Daughton
Aimee Lyons, Lewis, Metz & Daughton
Ashley Arthur, GeneDX
Matt Harju, Mirum Pharmaceuticals
Fran Hokkanen, NORD Rare Action Network
Dean Alma Littles, Florida State University College of Medicine
Victoria Mohebpour, Florida Department of Health
Meigs Lamb, Florida Department of Health
Bridgette Morton, Florida Department of Health
Anna Simmons, Florida Department of Health
Dr. Yi Zou, Florida State University College of Medicine
Lisa Zuccarello, Kite Pharma

1. Approval of July 2024 Meeting Minutes:

Dr. Barry Byrne motioned to approve the July 17, 2024, Meeting Minutes, and Dr. Mustafa Tekin seconded. All were in favor and the motion carried.

2. Presentations:

The Council heard presentations by the following Principal Investigators of the 2024 Rare Pediatric Diseases Research Grant Awardees:

- a. Yi Zhou, PhD – The Florida State University College of Medicine – Study of Developmental and Epileptic Encephalopathy using YWHAG Mutation Models

b. Shanta Dhar, PhD – The University of Miami Miller School of Medicine – Cardioliipin Enhancing Mitochondria Acting Nanoengineered Tools for Understanding Role of Oxidative Stress and Inflammation in Barth Syndrome

3. Review of Pediatric Rare Disease Grant Application Guidelines and Conflict of Interest Draft:

Department staff provided an overview of the Pediatric Rare Disease Grant Application Guidelines and Conflict of Interest Draft created by the Council's Workgroup on Grant Guidelines. Clarification was provided that a third-party vendor will review all grant applications before sharing scoring results with the Council. Any Council member applying for the grant will recuse themselves from the Council's voting process. Department staff noted there will need to be a meeting in Quarter 1 of 2025 to review applications, and Council members expressed a desire to end the application submittals in January 2025 to account for the busy winter holiday season. There was discussion on the Council's ability to make funding recommendations during its review process.

Jon Hawayek motioned to approve the Pediatric Rare Disease Grant Application Guidelines and Conflict of Interest Draft, and Dr. Byrne seconded. All were in favor and the motion carried.

4. Public Comments/Open Discussion:

No public comments were received.

5. Adjournment:

The meeting adjourned at 1:02 p.m. EST.