



Niemann-Pick Type C Clinical Roundtable
Gaining Consensus Regarding Initiation of Treatment for Patients Positively Identified with NPC
Following Newborn Screening

Chaired by Elizabeth Berry-Kravis, MD, PhD
 Principal Investigator, NPC Newborn Screening Initiative
 Professor, Pediatrics, Neurological Sciences and
 Biochemistry; Rush University Medical Center

Organized by The Firefly Fund
 Pam Andrews, Co-Founder and Executive Director
 Allison May Rosen, 3D Communications for Firefly Fund

Agenda
Thursday, May 23, 2019
9:00 am – 3:00 pm
Bethesda Marriott, 5151 Pooks Hill Road, Bethesda, MD

Expert Attendees

Elizabeth Berry-Kravis, MD PhD, *Rush University*
 Simona Bianconi MD, *National Institutes of Health*
 Nicole Farhat NP, *National Institutes of Health*
 Caroline Hastings, MD, *UCSF*
 Jean-Baptiste Le Pichon MD, PhD, *Children's Mercy, KC*
 Marc Patterson, MD, *Mayo Clinic*
 Denny Porter, MD PhD, *National Institutes of Health*

Cindy Powell MD, MS, *University North Carolina*
 Latha Soorya PhD, *Rush University*
 Audrey Thurm, PhD, *National Institutes of Health*
 Allison (Allie) Wainer PhD, *Rush University*
 Melissa Wasserstein, MD, *Montefiore*
 Amy White, MD, *Southwest Pediatrics*
 Kara Woolgar, RN, *Phoenix Children's*

Time	Topic	Speaker
9:00 am	Welcome and Introductions	Elizabeth Berry-Kravis, MD PhD Pam Andrews, Firefly Fund
9:30 am	About NPC and Current Treatment Landscape • Presentation and Q&A	Marc Patterson, MD
10:00 am	NPC Natural History • Presentation and Q&A	Denny Porter MD PhD
10:30 am	Newborn Screening for NPC • Presentation and Q&A	Melissa Wasserstein, MD
11:00 am	Developmental Trajectories in NPC • Presentation and Q&A	Audrey Thurm PhD
11:30 am	Natural History vs Early Intervention • Presentation and Q&A	Elizabeth Berry-Kravis, MD PhD
Noon	Lunch	
12:30 pm	Group Discussion Questions • See attached for discussion topics	All Participants
2:45 pm	Wrap Up and Next Steps	Elizabeth Berry-Kravis, MD PhD
3:00 pm	End	



NPC Community Supporters and Observing Attendees

- Cristin Davidson, PhD, Support of Accelerated Research for Niemann-Pick C (SOAR-NPC)
- Marianne Dwight, Firefly Fund
- Joslyn Crowe, National Niemann-Pick Foundation
- Sean Kassen, Ara Parseghian Medical Research Fund
- Phil Marella, Dana's Angels Research Trust

NPC Industry Supporters and Observing Attendees

- Adepero Shola Adewale, MD, Amicus Therapeutics
- Nick France, Enchi Lui, E-Scape Bio
- Helen Chang, Susan Van Meter, MD, Mallinckrodt
- Michelle Gilmor, Paul Merrigan, Orphazyme
- Darby Thomas, Stride Bio