



*Externally-led Patient Focused Drug Development Meeting for Pyruvate Kinase Deficiency
College Park Marriott Hotel and Conference Center, Hyattsville, MD
Friday, September 20, 2019*

- 9:30 am Registration and light continental breakfast
- 10:00 to 10:10..... Opening Remarks
*Peter Saltonstall, President & CEO, NORD
Dore Peereboom, Foundation for Rare Blood Diseases (SZB)*
- 10:10 to 10:25..... Clinical Overview of PK Deficiency
Rachael Grace, M.D., Hematologist/Oncologist, Boston Children's Hospital
- 10:25 to 10:45..... Welcome Remarks
*Wilson Bryan, M.D., Director, Office of Tissues and Advanced Therapies, CBER,
FDA*
- 10:45 to 10:55..... Introduction and Meeting Overview
Larry Bauer, Sr. Regulatory Drug Expert, Hyman, Phelps, & McNamara, P.C., Meeting Moderator
- 10:55 to 11:05..... Overview of Discussion Format & Demographic Polling Questions
- 11:05 to 11:30..... Topic 1: Living with PK Deficiency—Burdens and Symptoms
*Rebecca Herzog, Caregiver
Katine "Cathy" Miller, Patient
Ai Bee Poh, Caregiver
Tamara Schryver, Patient
Alejandra Watson, Caregiver*
- 11:30 to 12:30..... Polling Questions and Facilitated Audience Discussion on Topic 1
Larry Bauer, Meeting Moderator
- 12:30 to 1:30.....Lunch
- 1:30 to 1:55..... Topic 2: Current & Future Treatments
*Zach Adamson, Caregiver
Elizabeth "Libby" Garrett, Patient
Jennifer King, Caregiver
Tina Knapp, Caregiver
Jonathan Watson, Pediatric Patient*

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1:55 to 2:55..... Polling Questions and Facilitated Audience Discussion on Topic 2
Larry Bauer, Meeting Moderator

2:55 to 3:15..... Closing Remarks
Lucas Kempf, M.D., Medical Officer, Rare Diseases Program, CDER, FDA

3:15 to 3:20..... Farewell Remarks & Next Steps
Michelle Adams, Director of Federal Policy, NORD

Adjourn

3:30 to 5:30.....Closing Reception
Sheppard's Art Gallery, College Park Marriott Hotel and Conference
Center