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
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  
*Michele 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