

VOICES OF THE PEOPLE – THE ALSTRÖM SYNDROME JOURNEY

ASI Externally-Led Patient-Focused Drug Development Meeting

September 22, 2022

- 07:00 – 08:30 **Breakfast for Participants in the Duncan Room**
- 08:30 – 08:40 **Welcome and Overview of the EL-PFDD:** Sheila Farrell, MD, US Food and Drug Administration
- 08:40 – 08:45 **Welcoming Remarks:** Robin Marshall, Executive Director, Alström Syndrome International
- 08:45 – 08:55 **The Genetics of Alström Syndrome:** Clair A. Francomano, MD, Chair, Scientific Advisory Board
- 08:55 – 09:05 **The Natural History of the Disease:** Chase A. Palmer, PharmD, Board of Directors
- 09:05 – 09:25 **Overview, Demographic Polling:** Anne D. Nordstrom, MBA, PhD
- 09:25 – 09:55 **Panel 1: Quality of Life/Burden of Disease:** Dona Algama, Katelyn Denbow, Callie Marshall, Chad Potter
- 09:55 – 10:05 **BREAK IN THE HALLWAY**
- 10:05 – 10:35 **The Little Warrior – Living from Crisis to Crisis: Prerecorded presentation about Dannie Grace Priebe’s turbulent early life.** Narrated by Sarah Durfey
- The Three Amigos: Prerecorded presentation demonstrating genetic variability and different journeys even within families.** Narrated by Jennifer Potter
- Jack’s Tale: Prerecorded presentation about Jack and the early years on his Alström Journey.** Narrated by Ed and Erin Conlon
- 10:35 – 10:50 **Panel 2: Quality of Life: Overcoming the Odds and the Quest for Independence:** Hannah Wedel, Doula Jarboe, Adam Kozarewicz, Milan Boekhoudt
- 10:50 – 12:00 **Polling and Discussion with the Global Alström Community:** Conducted by Anne Nordstrom, MBA, PHD and facilitated by Clair Francomano, MD and Alex Levin, MD
- 12:00 – 13:00 **BUFFET LUNCHEON IN THE DUNCAN ROOM**
- 13:00 – 14:00 **Coming of Age and Experiencing Setbacks:** Prerecorded presentation by Milan Boekhoudt
- A Life of Independence and Marriage:** Prerecorded presentation by Doula Jarboe
- A Survivor and Man of the House:** Prerecorded presentation by Adam Kozarewicz
- The Rough Road to a Good Education:** Prerecorded presentation by Hannah Wedel
- Two Tales of Surviving the Burden of the Disease - Courageous Mothers and Daughters:** Prerecorded presentations by Katelyn and Gina Denbow and Callie and Shelly Marshall
- 14:00 – 15:30 **Panel 3: It’s Complicated - Issues of Diagnosis, Burden of Treatment, Access to Clinical Trials:** The Assembled Alström Parents, Adults and Children
- Polling and Discussion with the Global Alström Community:** Conducted by Anne Nordstrom, MBA, PHD and facilitated by Clair Francomano, MD and Alex Levin, MD
- 15:30 – 15:40 **Closing Remarks:** Robin Marshall, Executive Director, Alström Syndrome International