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