Rare Disease Day
Thursday, February 28, 2019
12:00-1:00pm
State Capitol, Eureka Room
*Lunch will be served

AGENDA

I. Welcome (5 minutes)
   - RD Caucus Legislator

II. Documentary Screening, 10 minutes
   *Tess in not Alone, a USP7 story, Directed by Bo Bigelow*
   When Tess was diagnosed with a little-known gene mutation, her dad sought answers online. He found a researcher of USP7 gene mutations and learned Tess was the eighth known patient in the world. Today they have found 45 patients across the globe.

III. Living with Rare Disease, Patient Perspectives, 15 minutes
   Moderated by Angela Ramirez Holmes, Founder/President Cal Rare
   Patient 1: Jacob Fraker, Cystic Fibrosis patient
   Patient 2: Christine Waggoner, Cure GM1 Foundation, mother of Iris
   Patient 3:

IV. Documentary Screening, 5 minutes
   *Menkes Disease: Finding Help and Hope, Directed by Daniel DeFabio*
   Oscar nominated actress Mary McDonnell narrates this look at a rare disease with a life expectancy of 3-10 years and how it changed Lucas and his family as they went through a clinical trial at the NIH.

V. Panel: Innovation in Rare Diseases (a look ahead for 2019), 20 minutes
   - Moderated by Oliver Rocroi, Senior Director of State Government Relations, California Life Sciences Association (CLSA)
   - Panelist 1
   - Panelist 2
   - Panelist 3

VI. Closing Remarks, 5 minutes
   - RD Caucus Legislator