

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 is 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