

10th Annual Conference on Advancing Rare Disease Research, Therapy, and Patient Advocacy

Friday March 1, 2019 | 5:00-6:30pm

Dinner

Introductions | Kasturi Haldar, Ph.D., Director of CRND

Welcome and Invocation | Dean Mary Galvin

Keynote speaker | Mr. Matt Boler

Student Awards | Barb Calhoun | Jill McCormack

7:00pm | Notre Dame vs Penn State Hockey Game

Saturday March 2, 2019

7:30am | Breakfast

7:55am | Glee Club Rare Disease Song

8:00am | Welcome | Deputy Chief Suzanna Fitzberg (Mayor's office)

8:15-8:45am | Keynote: Rare Tumors: Advancing Preclinical Models | Xin Lu, Ph.D., John M and Mary Jo Boler Assistant Professor, Boler-Parseghian Center

8:45-9:45am | Discovery, Translation and Development

Kasturi Haldar, Ph.D., Notre Dame

John Crowley, CEO, Amicus

Elizabeth Berry Kravis, M.D., Ph.D., Rush Medical Center

Steve Aselage, Board member, Retrophin

Diana Wetmore- Harrington, Ph.D., V.P., and Director, Harrington Institute

Brian Blagg, Ph.D., Notre Dame

9:50-10:45am | Community Resources and Empowering Rare Disease Patients

Rebecca Wingert, Ph.D., Notre Dame

Gillian Shaw, Senior Fellows at EnFocus

Nicole Boice, Founder, Global Genes

Suhail Alam, Ph.D., Notre Dame

John Koren, Ph.D., Notre Dame

Siyuan Zhang, Ph.D., Notre Dame

Kunigunda Szentcs, IDEA Center Notre Dame

10:45-11:15am Coffee, Tea, and Poster session



10th Annual Conference on Advancing Rare Disease Research, Therapy, and Patient Advocacy

11:15-12:00pm Patient Advocacy and Health Promotion

Mary Alice Reid, M.D., Beacon Pediatrics
Karen Quandt, R.N., NPC Patient Family & Advocate
Sean Nohelty, Tarah O'Sullivan Patient Foundations for NKH
Daniel Fulkerson, M.D., Beacon Health
Francis Fitzgerald, Rare Disease Foundation, ND'18
Rob Long, Uplifting athletes

12:15-12:30pm | 'One Minute Teaser Talks'

- Neurological disorders
- Hematological and Respiratory Diseases-
- Rare Tumor Biology
- Intellectual Disabilities
- Lysosomal Storage Disorders
- Musculoskeletal and other rare disorders-
- Rare ND Club
- Global Genes
- Uplifting Athletes

12:30-2:00pm | Lunch and Posters

2:00-3:30pm | Rare Diseases in our Community

- Angelman Syndrome
- Von Hippel Lindau
- Leber's Hereditary Optic Neuropathy
- Coats Disease
- Rett Syndrome
- Spinal Muscular Atrophy with Respiratory Distress

3:30-3:45pm | Break and Posters

3:45-5:00pm

- Neurofibromatosis Type 1
- Mast Cell Activation Syndrome/ Eosinophilic Esophagitis
- Sickle Cell Disease
- Shprintzen Goldberg Syndrome
- Non ketotic Hyperglycemia