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 | Research and Therapy
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 | Resources and Empowerment
Rebecca Wingert, Ph.D., Notre Dame
Suhail Alam, Ph.D., Notre Dame
John Koren, Ph.D., Notre Dame
Siyuan Zhang, Ph.D., Notre Dame
Kunigunda Szentes, IDEA Center Notre Dame
Gillian Shaw, Senior Fellows at EnFocus
Nicole Boice, Founder, Global Genes
10:45-11:15am Coffee, Tea, and Poster session

11:15-12:00pm Patient Health & Advocacy
Mary Alice Reid, M.D., Beacon Pediatrics
Karen Quandt, R.N., NPC Patient Family & Advocate
Tarah O’Sullivan & NKH Patient Advocates
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 Poster take down.

3:45-5:00pm
• Neurofibromatosis Type 1
• Mast Cell Activation Syndrome/ Eosinophilic Esophagitis
• Sickle Cell Disease
• Shprintzen Goldberg Syndrome
• Non ketotic Hyperglycinemia