THE MANAGEMENT OF RARE DISEASES
FIFTH ANNUAL RARE DISEASE GENOMICS SYMPOSIUM

BEYOND THE DIAGNOSIS ART EXHIBIT
“TEAGAN” BY JOTA LEAL SCHIZENCEPHALY

March 2 & 3, 2018

Children’s of Alabama
Bradley Lecture Center
4th Floor, Children’s Harbor Building

Friday, 8 am—4 pm: Scientific Focus
Saturday, 9 am—1 pm:
Patient & Caregiver Focus

Friday: $35 Saturday FREE To register, visit ChildrensAL.org/genetics

FREE Parking in the 5th or 7th Avenue Children’s Decks. Please follow the signs.

Questions? Contact Shaila Handattu at shandattu@uabmc.edu

LEARNING OBJECTIVES (Friday, March 2)

Upon completion of this live activity, participants will be able to:

- Describe approaches to surveillance and anticipatory guidelines for patients with rare disease and their families
- Explain how increased understanding of pathogenesis is used to treat rare diseases
- Describe approaches to transition from pediatric to adult care for patients with rare diseases
- Explain the importance of coordination of care to improving outcomes for patients with rare diseases

LEARNING OBJECTIVES (Saturday, March 3)

Upon completion of this live activity, participants will be able to:

- Learn about resources in the state to help with advocacy and management of needs as patients and caregivers

CONTINUING EDUCATION

(Offered for Friday, March 2, ONLY)

CME

Children’s of Alabama designates this live activity for a maximum of 5.75 *AMA PRA Category 1 Credits*™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Children’s of Alabama is accredited by the Medical Association of the State of Alabama to provide continuing medical education for physicians.

BEYOND THE DIAGNOSIS ART EXHIBIT

Selected works from this traveling art exhibit, focusing on the rare disease patient, will be displayed at Children’s of Alabama from February 16 through March 16, 2018. Art has been used for thousands of years to successfully convey a message, whether it be a story or a glimpse into the human spirit. The purpose of this exhibit, presented by the Rare Disease United Foundation, is to encourage a look “beyond the diagnosis” to the patient. Two works from the exhibit are shown in this flyer.

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AGENDA

FRIDAY, MARCH 2

SCIENTIFIC FOCUS

8:00-8:30	**Registration & Breakfast**
8:30-9:00	**Session 1: Genomic Medicine** ~ Bruce Korf, MD, PhD University of Alabama at Birmingham **Session 2: Treatment** Moderator ~ Matthew Might, PhD University of Alabama at Birmingham
9:00-9:45	"Disruption Out of Desperation" ~ Emily Kramer-Golinkoff Co-Founder, Emily's Entourage, Philadelphia
9:45-10:30	"How Model Organism Studies Can Inform Clinical Approaches to Rare Disease" ~ Clement Chow, PhD University of Utah, Salt Lake City, UT
10:30-10:45	**Break**
10:45-11:45	**Keynote Speaker** "Congenital Disorders of Glycosylation: Overview and Focal Points" ~ Hudson Freeze, PhD Sanford Burnham Prebys Medical Discovery Institute, La Jolla, CA
11:45-12:00	**Break/Pick Up Lunch (Provided)**
12:00-1:00	**Session 3: Parent Panel** Moderator ~ Ashley Cannon, MS, PhD University of Alabama at Birmingham **Session 4: Transition of Care** Moderator ~ Nathanial Robin, MD University of Alabama at Birmingham
1:00-1:45	"Transition of Care in Spina Bifida" ~ Betsy Hopson, MSHA Children's of Alabama and University of Alabama at Birmingham
1:45-2:30	"Not a Doorbell Ditch: Cystic Fibrosis and Transition of Care" ~ Brad Troxler, MD University of Alabama at Birmingham **Session 5: Coordination of Care** Moderator ~ Anna C.E. Hurst, MD, MS University of Alabama at Birmingham
2:30-3:15	"Care Coordination for Children with Special Needs" ~ Justin Schwartz, MD Children's of Alabama
3:15-3:35	"Care Coordination at Children's: A Look at the Future" ~ Beth Clark, BSN, MBA Children's of Alabama
3:35-3:55	"Medicaid Health Home Overview" Michael Battle Alabama Care Plan
4:00	**Adjournment**

SATURDAY, MARCH 3

PATIENT & CAREGIVER FOCUS

8:30-9:00	**Registration, Welcome Continental Breakfast**
9:00-9:15	**Symposium Kick Off** "Update on the Genomic Health Initiative and Recent Scientific Symposium" ~ Bruce Korf, MD, PhD University of Alabama at Birmingham "Alabama Rare 2018 Plans" ~ Swapna Kakani Leader, Alabama Rare, and Patient Advocate
9:15-10:40	**Main Lectures** "Palliative Care and Coordination of Care in the Rare Disease Community" ~ M. Garrett Hurst, MD Assistant Professor, UAB Center for Palliative and Supportive Care "Tools to Empower Coordinated Care" ~ Cristina Might, President of NGLY1.org, and Parent Advocate "Fighting for a Cure: Growing an Organization and Interacting with Researchers" ~ Matthew Alexander, PhD Assistant Professor, UAB Department of Pediatric Neurology ~ Scott Griffin, Founder, Hope for Gabe,and Parent Advocate "Empowering the Patient—Your Voice Matters!" ~ Kristin Anthony, Founder, PTEN Hamartoma Tumor Syndrome Foundation ~ Swapna Kakani Leader, Alabama Rare, and Patient Advocate
10:40-11:00	**Break (Coffee, Tea and Snacks Provided)** Q & A Featuring Main Lecture Speakers
11:00-12.00	**Breakout Sessions (Session 1: 11:00-11:30; Session 2: 11:30-12:00; Choose 1 topic per session)** "The Americans with Disabilities Act Disability Discrimination and Workers with Caregiving Responsibilities" ~ Bryan Douglas, Administrative Judge, United States Equal Employment Opportunity Commission "Alabama Medicaid Care Coordination and WaiverPrograms" ~ Carolyn Miller, LICSW, PIP, Health Systems Manager, Managed Care Division, Alabama Medicaid "Overview of Children's Harbor Services" ~ Audrey Lampkin, Director, Children's Harbor Family Center "Advocacy, Acceptance and Understanding: Navigating the School Experience in a Positive Way" ~ James Gallini, JD, Founder, The Gallini Group, LLC
12:00-12:50	**Panel Discussion (featuring Breakout Session speakers)** Moderator ~ Kelly Morris President, Epilepsy Foundation of Alabama, and Parent Advocate
12:50-1:00	**Closing Remarks** ~ Swapna Kakani Leader, Alabama Rare, and Patient Advocate
1:30	**Lunch Will Be Provided**