

16TH ANNUAL GREAT PLAINS

# RARE DISEASE SUMMIT

RARE DISEASES OF THE SKELETAL SYSTEM & CRANIOFACIAL DEVELOPMENT

## THURSDAY MAY 7TH

**3:00 WELCOME**

**3:05 LEVI GERSHKOWITZ**

Founder and CEO  
Living in the Light LLC  
*"Story as Medicine"*

**4:00 ERICA BARNES**

Executive Director  
Minnesota Rare Disease Advisory Council  
*Availability vs Accessibility: Aligning the Ecosystem  
through Effective Policy*

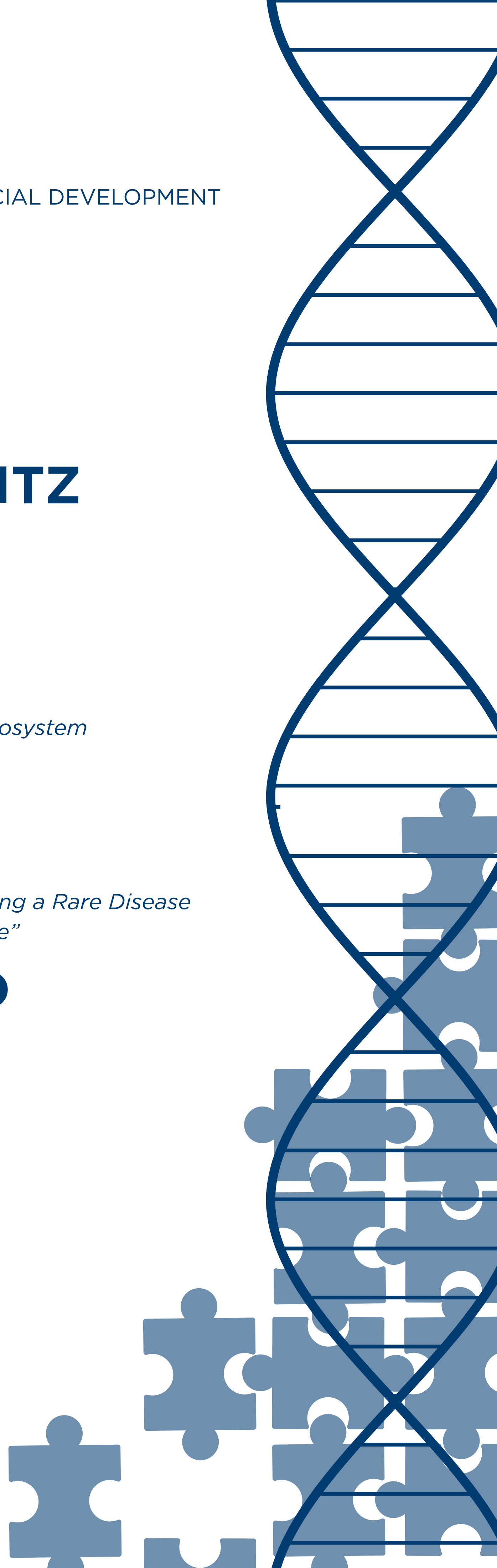
**4:35 TERRI KLEIN**

President and CEO  
National MPS Society  
*"Science, Advocacy, and Community: Leading a Rare Disease  
Organization at the Intersection of All Three"*

**5:10 APPETIZERS AND**

**REFRESHMENTS**

**7:00 CLOSING**



16TH ANNUAL GREAT PLAINS

# RARE DISEASE SUMMIT

RARE DISEASES OF THE SKELETAL SYSTEM & CRANIOFACIAL DEVELOPMENT

## FRIDAY MAY 8TH

### MORNING AGENDA

**8:00 REGISTRATION AND BREAKFAST**

**8:30 JILL WEIMER, PH.D.**

Scientist in the Center for Genetics and Rare Diseases  
Senior Director of Therapeutic Development  
The Sanford Research Institute  
*"Welcome address"*

**MODERATOR: KAMESWARAN SURENDRAN, PH.D**

**8:35 IRFAN SAADI, PH.D.**

Professor of Cell Biology and Physiology  
University of Kansas Medical Center  
*"Modulating Palate Closure Dynamics to Prevent Cleft Palate"*

**9:25 DATA BLITZ**

**10:00 KYLE ROUX, PH.D.**

Scientist, Center for Genetics and Rare Diseases  
Vice President of Biomedical Research  
The Sanford Research Institute  
*"The integrity of the nuclear envelope and its relationship to rare disease"*

**10:35 JAN LAMMERDING, PH.D.**

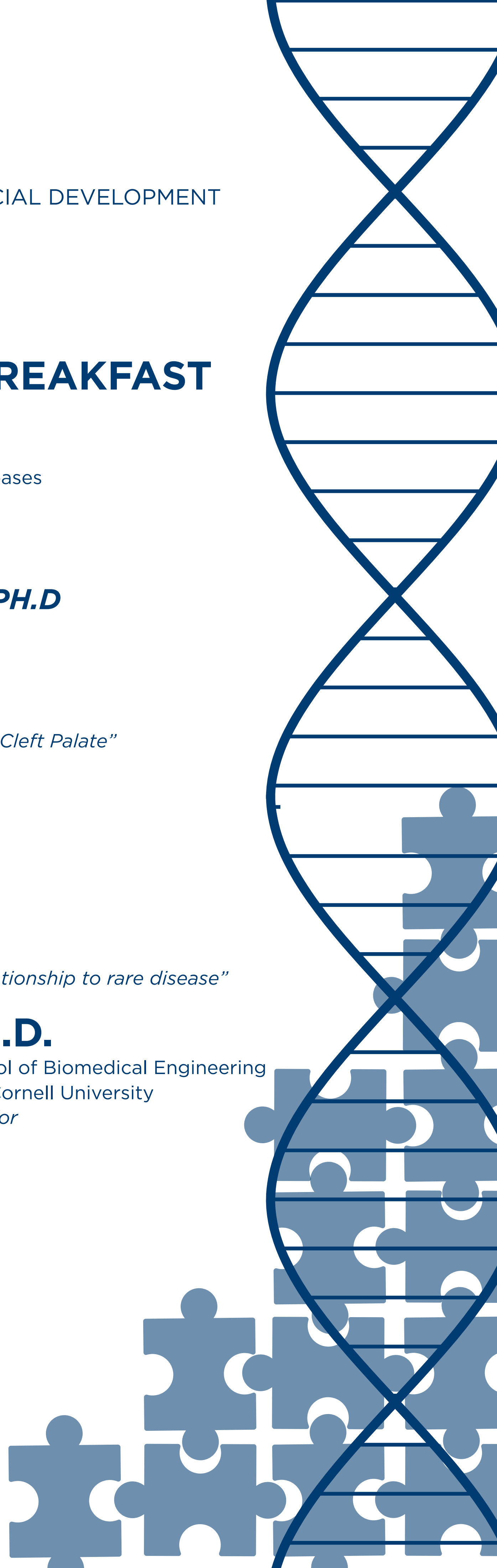
Professor and Associate Director of Meinig School of Biomedical Engineering  
Weill Institute for Cell and Molecular Biology at Cornell University  
*"New Insights and Novel Therapeutic Strategies for Striated Muscle Laminopathies"*

**11:25 BREAK**

**MODERATOR: KYLE ROUX, PH.D**

**11:35 JIANNING TAO, PH.D.**

Associate Scientist & Principal Investigator,  
Cancer Biology & Immunotherapies  
Sanford Research & Sanford School of Medicine,  
University of South Dakota  
*"Pathological Notch Signaling in Musculoskeletal Disease: Mechanisms and Implications"*



16TH ANNUAL GREAT PLAINS

# RARE DISEASE SUMMIT

RARE DISEASES OF THE SKELETAL SYSTEM & CRANIOFACIAL DEVELOPMENT

## FRIDAY MAY 8TH

### AFTERNOON AGENDA

**12:10 DATA BLITZ**

**12:45 LUNCH**

**1:45 JEFFREY BUSH, PH.D.**

Barber Distinguished Professor and Chair of the Department of Cell and Tissue Biology  
Program in Craniofacial Biology at the University of California, San Francisco  
*"Modeling craniofacial morphogenesis and congenital disease through live  
imaging and gene editing of the mouse embryo"*

**2:35 POSTER AND VENDOR SESSION**

**MODERATOR: JIANNING TAO, PH.D**

**3:30 ROLF STOTTMANN, PH.D.**

Professor of Pediatrics  
Nationwide Children's Hospital & The Ohio State University  
*"Genetic Study of Brain and Craniofacial Congenital Anomalies"*

**4:20 ROY MORELLO, PH.D.**

Professor in the Department of Physiology and Cell Biology  
University of Arkansas for Medical Sciences  
*"Skeletal defects in Osteogenesis Imperfecta significantly contribute to  
abnormal lung development and function"*

**5:10 AWARDS & APPRECIATION -  
CLOSING REMARKS**

