



# ▶▶▶▶ **MSD VIRTUAL SUMMIT 2026**

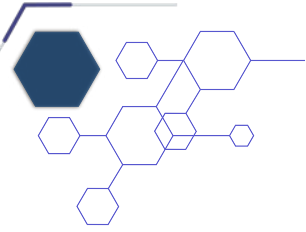
Presented by



**SEPTEMBER 18-19**  
**9 AM - 2 PM CST**

REAL-TIME TRANSLATION  
60+ LANGUAGES | ZOOM

# Welcome to the MSD Virtual Summit



Dear Summit Attendees,

Welcome to the 2026 MSD Virtual Summit!

I'm so glad you're here. This weekend brings together families, researchers, clinicians, and advocates from around the world, all connected by one important goal: creating a better future for those impacted by MSD.

There is still so much work ahead of us, but there is also so much hope. Every conversation, connection, and advancement in research moves us forward.

Thank you for being here, supporting one another, and most importantly, continuing to show up for these incredible families.

I hope you have a wonderful Summit!

With gratitude,

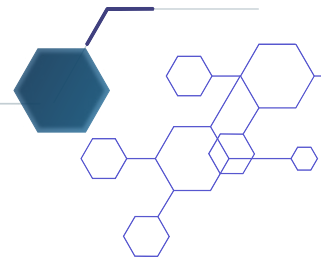
**Brandy Brockus,**

*President, United MSD Foundation*

*Board of Directors*



# Science Day | September 18



## Time (CT)

## Session

## Speakers

9:00 AM

Opening Remarks

Amber Olsen, Founder & Interim  
Executive Director

9:15 AM

### **SECTION 1 | DISEASE CATEGORIZATION**

9:30 AM

Natural History Study Updates

Dr. Laura Adang

10:00 AM

Comprehensive Analysis of Brain MRI in MSD

Dr. Lars Schlotawa

Comprehensive Biomarker Profiling in MSD

Dr. Xinying Hong

### **SECTION 2 | EXPANDING THE SPECTRUM**

10:00 AM

Hypomorphic SUMF1 Genotypes Associated  
with Attenuated MSD

Dr. Siying Lin

10:15 AM

PAG Perspective of the Expanding Spectrum

Brenna Bentley, MS, CGC

### **SECTION 3 | PRE-CLINICAL**

10:25 AM

Advancing Small-Molecule Stabilizers of FGE:  
An Update

Dr. Matthias Baud

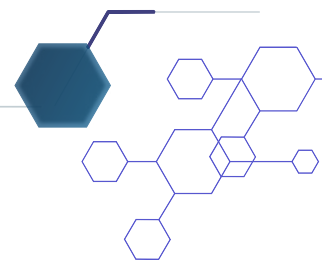
10:35 AM

Crystallographic Fragment Screening  
Identifies Small Molecules That Bind to FGE

Dr. Hartmut Niemann

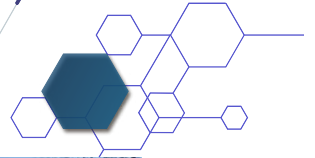


# Science Day | September 18



| Time (CT)       | Session                                                                                                            | Speakers                                                                                  |
|-----------------|--------------------------------------------------------------------------------------------------------------------|-------------------------------------------------------------------------------------------|
| 10:45 AM        | Strategies for the Synthesis & Testing of Small-Molecule Stabilisers of FGE                                        | Dr. Hugh Britton                                                                          |
| 10:55 AM        | Microglia-Based Gene Therapy for MSD                                                                               | Dr. Rebecca Ahrens-Nicklas                                                                |
| <b>11:10 AM</b> | <b>BREAK</b>                                                                                                       |                                                                                           |
| <b>11:15 AM</b> | <b>SECTION 4   THERAPEUTIC PIPELINE &amp; NEWBORN SCREENING</b>                                                    |                                                                                           |
| 11:15 AM        | Clinical Testing of Tazarotene & Identification of Backup Compound Candidates for MSD Treatment                    | Dr. Lars Schlotawa                                                                        |
| 11:25 AM        | CHAMPION Project                                                                                                   | Dr. Lars Schlotawa                                                                        |
| 11:45 AM        | AAV9 Gene Therapy Clinical Trial: Design of a Patient-Centric Clinical Trial for Rare Diseases                     | Dr. Laura Adang & Dr. Rebecca Ahrens-Nicklas                                              |
| <b>12:15 PM</b> | <b>Newborn Screening Panel</b><br>From Incidental to Actionable: Preparing the MSD Community for Newborn Screening | Dr. Laura Adang, Dr. Michael Gelb, Dr. Joe Orsini, Amy Gaviglio, Dr. N. Matthew Ellinwood |
| <b>12:55 PM</b> | <b>CLOSING KEYNOTE:</b><br><b>Making Rare Disease Treatment a Reality</b>                                          | Dr. Peter Marks                                                                           |
| 1:55 PM         | Closing Remarks                                                                                                    | Amber Olsen, Founder & Interim Executive Director                                         |



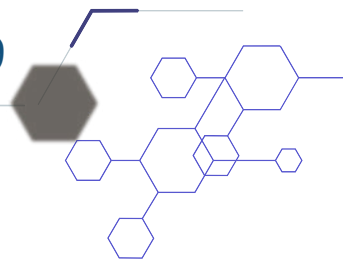


EG

Grant Us Grace



# Family Day | September 19



## Time (CT) Sessions

9:00 AM Opening Remarks

### SECTION 1 | CLINICAL TRIALS

9:15 AM Science Day Recap

10:15 AM Questions & Answers Session

10:30 AM **Clinical Trials: The True Struggles of Participants**

### 11:10 AM BREAK

### SECTION 2 | CAREGIVING

11:25 AM Burnout is NOT Inevitable

12:05 PM Ichthyosis in MSD: Skin Science, Everyday Care, & Family Perspectives

12:45 PM Beyond Calories: The Secret Life of Nutrients

4:30 PM Remembrance + Grounding Activities

4:55 PM Closing Remarks

## Speakers

Amber Olsen, Founder & Interim Executive Director

Dr. Laura Adang, Dr. Rebecca Ahrens-Nicklas, & Dr. Lars Schlotawa

Dr. Amy Waldman

Brad Thompson

Delaney Ding & Malaika Vaz

Anne Kozek

Brenna Bentley

Amber Olsen, Founder & Interim Executive Director





# THE ZEBRA & THE BEAR

In the world of rare disease,  
nothing is more powerful than a mother's love.

A DOCUMENTARY FILM BY PATRICK O'CONNOR



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# 10<sup>TH</sup>

## ANNIVERSARY | MARCH 13, 2027

A DECADE OF HOPE. A BRIGHTER TOMORROW.



# ZEBRA RUN

FOR RARE DISEASE

Event benefits the **United MSD Foundation**

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## Speakers



**Laura Adang, MD, PhD, MSTR**

*Attending Physician, Children's Hospital of Philadelphia*



**Rebecca Ahrens-Nicklas, MD, PhD**

*Assistant Professor, Children's Hospital of Philadelphia*



**Matthias Baud, PhD**

*Associate Professor, University of Southampton*



**Brenna Bentley, MS, CGC**

*Patient & Family Advocate, United MSD Foundation*



**Hugh Britton, PhD**

*Researcher, Baud Group*



**Delaney Ding**

*MD-PhD Pre-Doctoral Fellow*



**Matthew Ellinwood, DVM, PhD**

*Consultant, Connetics Consulting, LLC*



**Amy Gaviglio, MS, CGC**

*Genetic Counselor, CEO & Founder, Connetics Consulting, LLC*



**Michael Gelb, PhD**

*Professor, University of Washington*

## Speakers



**Xinying Hong, PhD**

*Assistant Professor, Children's Hospital of Philadelphia*



**Anne Kozek, MS, RD, LDN**

*Registered Dietitian*



**Serene Siying Lin, MBBS, PhD**

*Clinician Scientist, University of Manchester*



**Peter Marks, MD, PhD**

*Sr. Vice President, Molecule Discovery, & Head of Infectious Disease, Lilly Research Laboratories*



**Hartmut Niemann, PhD**

*Professor, Bielefeld University*



**Joseph Orsini, PhD**

*Research Lab Director*



**Lars Schlotawa, MD**

*Research Consultant, University Medical Center Goettingen, Germany*



**Brad Thompson, LPC-S**

*Counselor & Co-Founder, The HALI Project*



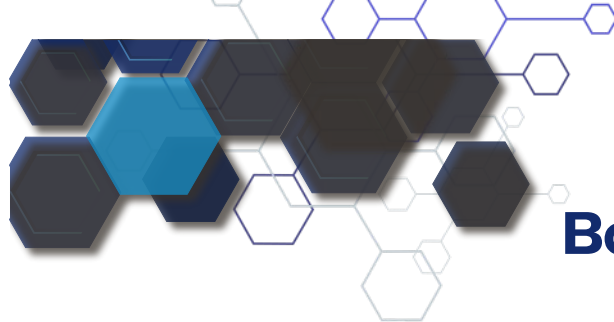
**Malaika Vaz, MS, GC**

*UMSDF Student Ambassador Alumna, Genetic Counseling Graduate*



**Amy Waldman, MD, MSCE**

*Medical Director, Leukodystrophy Ctr, Children's Hospital of Philadelphia*



# THANK YOU TO OUR Board, Staff, & MSD Virtual Summit Committee

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Julie Rosner-Lengele

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Margaret Fish, Bookkeeper  
Felicity Nesham-West, Marketing & Communications Specialist  
Amber Olsen, Interim Executive Director  
Krista Ortega, Student Ambassador Co-Lead

## MSD Virtual Summit Planning Committee

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Sarah Cortell Vandersypen  
Felicity Nesham-West  
Nasikah Ahmed  
Kara Anseth

Insa Borgmann  
Michelle Fox  
Haley Havens  
Natalie Lilien  
Ram Prashanth

*Special thanks to outgoing Executive Director,  
Sarah Cortell Vandersypen for her instrumental  
vision and leadership, and for spearheading the  
planning of the MSD Virtual Summit.*





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FOUNDATION  
MULTIPLE SULFATASE DEFICIENCY

