

**ADVANCES IN ALPHA THALASSEMIA MAJOR: NO LONGER A FATAL DISEASE**  
Virtual Conference for Providers and Families  
January 8-9, 2021

**DAY 1, January 8, 2021** Pacific Standard Time/Date

4:00 PM **Surviving with Joy – a short film**

4:10 PM **Welcome and overview – Day 1**

*Tippi MacKenzie, MD (UCSF)*

4:20 PM **Family Experience 1**

*Conversations with families about their diagnosis and treatment journeys*

4:50 PM Lessons from Patient Registries

*Introduction: Ali Amid, MD*

Canada Registry of ATM outcomes

*Ali Amid, MD (Children's Hospital of Eastern Ontario, University of Ottawa)*

Hong Kong Registry of ATM outcomes

*Wilson YK Chan, MBBS (Queen Mary Hospital, Hong Kong University)*

UCSF Registry – focus on neurological outcomes

*Marisa Schwab, MD, Billie Lianoglou, LCGC (UCSF)*

Meta-analysis of aggregated registry data

*Tachjaree Panchalee, MD (Siriraj Hospital, Mahidol University)*

**Live Q&A**

5:45 PM **BREAK**

5:50 PM **Prenatal Screening and Diagnosis**

*Introduction: Mary Norton, MD*

Genetic diagnosis of ATM

*John Waye, PhD (McMaster University)*

Prenatal thalassemia screening algorithm

*Mary Norton, MD (UCSF)*

Ultrasound diagnosis of hydrops

*Pornpimol Ruangvutilert, MD, PhD (Siriraj Hospital, Mahidol University)*

**Live Q&A**

January 8, 2021, continued

6:35 PM **BREAK**

6:40 PM **Optimizing fetal therapy for ATM**

*Introduction: Keith Ogasawara, MD*

Managing pregnancies affected by ATM

*Juan González Velez, MD, PhD (UCSF)*

Adoption of IUT as a therapy for ATM in the State of Hawaii

*Keith Ogasawara, MD (Kaiser Permanente Hawaii)*

*In utero* transfusion protocol

*Greg Ryan, MB (Ontario Fetal Centre, University of Toronto)*

*In utero* hematopoietic stem cell transplantation - Phase 1 clinical trial

*Tippi MacKenzie, MD (UCSF)*

7:15 PM **Live panel: Interdisciplinary discussion regarding prenatal care for ATM**

*Moderator: Tippi MacKenzie, MD*

*Juan González Velez, MD, PhD*

*Roberta Keller, MD (UCSF)*

*Keith Ogasawara, MD*

*Greg Ryan, MB*

7:40 PM **Close Meeting**

*Tippi MacKenzie, MD*

7:45 PM **ADJOURN**

**DAY 2, January 9, 2021** Pacific Standard Time/Date

3:00 – **Forum A: Specialists discuss IUT protocol consensus**

3:50 PM *Moderators: Juan González Velez, MD, PhD, Billie Lianoglou, LCGC (UCSF)*

3:00 – **Forum B: Building a patient-family group for ATM**

3:50 PM *Moderator: Sandra Gilbert, PhD (Cooley's Anemia Foundation)*

This session is for patients/families living with ATM

January 9, 2021, continued

- 4:00 PM **Welcome and overview - Day 2**  
*Tippi MacKenzie, MD (UCSF)*
- 4:05 PM **Family Experience 2**  
*Conversations with families living with ATM*
- 4:35 PM **Postnatal therapies for ATM**  
**Introduction: Vip Viprakasit, MD, DPhil**
- Postnatal transfusion protocols  
*Elliott Vichinsky, MD (UCSF)*
- Postnatal hematopoietic stem cell transplantation  
*Alexis Thompson, MD, MPH (Lurie Children's Hospital of Chicago, Northwestern Feinberg School of Medicine)*
- Transfusion and transplantation in Thailand  
*Vip Viprakasit, MD, PhD (Siriraj Hospital, Mahidol University)*
- 5:05 PM **Live panel: Interdisciplinary discussion regarding opportunities and challenges to providing postnatal care for ATM**  
*Moderator: Vip Viprakasit, MD, DPhil*  
*Kay Thwe Aung, MD (Mandalay Children's Hospital)*  
*Maria Cecilia Cruz, MD (Philippine Children's Medical Center)*  
*Supachai Ekwattanakit, MD, PhD (Siriraj Thalassemia Center, Mahidol University)*  
*Sandhya Kharbanda, MD (UCSF)*  
*Aye Aye Khaing, MD (Yangon Children's Hospital)*  
*Alexis Thompson, MD, MPH*  
*Elliott Vichinsky, MD*
- 5:30 PM **Thalassemia International Federation (TIF)**  
*Michael Angastiniotis, MBChB, TIF Medical Advisor*
- 5:35 PM **BREAK**
- 5:40 PM **Live Panel: Discussion with families and providers**  
*Introduction by Craig Butler, Executive Director, Cooley's Anemia Foundation*  
*Moderator: Barbara Koenig, PhD, Director, UCSF Bioethics Program*  
*Melanie Kirby-Allen, MD (Hospital for Sick Children)*  
*Billie Lianoglou, LCGC (UCSF)*  
*Mara Rosner, MD (Johns Hopkins Medicine)*  
*Family Representatives*
- 6:25 PM **Close Meeting**  
*Tippi MacKenzie, MD*
- 6:35 PM **ADJOURN**