

# ALPHA THALASSEMIA MAJOR: NO LONGER A FATAL DISEASE

Virtual Conference for Providers and Families

Register

All are welcome  
Registration is free  
CME credit available

January 8, 2021, 4:00 pm - 7:30 pm PST

January 9, 2021, 3:00 pm - 6:30 pm PST

Pacific Standard Time/Date

Check the date and time where you live 

## Highlights!

- Families share diagnosis and treatment journeys
- Optimal practice for prenatal and postnatal treatment
- *In Utero* Transfusion (IUT) protocols
- Strategies to reduce barriers to prenatal treatment globally

## Advances in Alpha Thalassemia Major: No Longer a Fatal Disease

Alpha Thalassemia Major (ATM) was once considered universally fatal *in utero*. If left untreated, a fetus with ATM will develop severe anemia and hydrops and will die before or shortly after birth. However, recent evidence from patient registries has demonstrated that when ATM is diagnosed early in pregnancy, *in utero* transfusions (IUT) can reverse hydrops and enable survival with good neurological outcomes. Still, widespread adoption of IUTs – commonly administered to treat other fetal conditions – has not materialized for patients with ATM.

The goals of this conference are to:

- Demonstrate the impact of prenatal therapy on postnatal and long-term outcomes for alpha thalassemia major (ATM)
- Understand best practices for prenatal and postnatal treatment of ATM
- Bring together families affected by ATM to share their diagnosis and treatment journeys

*In collaboration with the **Cooley's Anemia Foundation** and the **Thalassaemia International Federation (TIF)**, we will hold a special forum dedicated to building a patient group for ATM.*

Two half-day sessions are organized around prenatal screening, diagnosis, and therapies for ATM pregnancies (Day 1); and postnatal management of ATM from birth to adulthood (Day 2). Participants include clinicians, scientists, and families affected by ATM.

**Prenatal topics include** the natural history of ATM, ultrasound diagnosis of hydrops fetalis, genetic diagnosis of ATM, management of pregnancies affected by ATM, protocols for IUT, and an update on the ongoing phase 1 clinical trial of *in utero* hematopoietic stem cell transplant at the University of California, San Francisco. We will hold a specific forum to outline best practices for prenatal management with IUT.

**Postnatal topics include** protocols for chronic transfusions, bone marrow transplantation, and complications of ATM management. Barriers that inhibit access to prenatal and postnatal treatment, and strategies to make therapies more accessible globally will be discussed.

Because ATM disproportionately affects individuals of Southeast Asian ancestry, we planned a schedule that is most convenient for Southeast Asia and the Pacific Islands, while still accommodating North America. The meeting will be recorded and available to families and colleagues in other regions.

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[Register](#)

Registration is free and continuing medical education credits will be available.

Sponsored by the **California Institute for Regenerative Medicine**  and the **Center for Maternal-Fetal Precision Medicine**  at the University of California, San Francisco.

January 8, 2021, 4:00 pm - 7:30 pm PST time/date

4:00 pm

**Surviving with Joy** - a short film

4:05 pm

**Welcome and overview - Day 1**

Tippi MacKenzie, MD (UCSF)

4:15 pm

**Family Experience 1**

Conversations with families about their diagnosis and treatment journeys

4:40 pm

**Lessons from Patient Registries**

Moderator: Ali Amid, MD

**Canada Registry of ATM outcomes**

Ali Amid, MD (University of Ottawa)

**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

5:25 pm

**BREAK**

5:30 pm

**Prenatal Screening and Diagnosis**

Moderator: Mary Norton, MD

**Genetic diagnosis of ATM**

John Waye, PhD (McMaster University Medical Centre, Hamilton Health Sciences)

**Prenatal thalassemia screening algorithm**

Mary Norton, MD (UCSF)

**Ultrasound diagnosis of hydrops**

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

Live Q&A

6:15 pm

**BREAK**

January 8, 2021, 4:00 pm - 7:30 pm PST time/date

6:20 pm

### Optimizing fetal therapy for ATM

Moderator: Keith Ogasawara, MD

#### Managing pregnancies affected by ATM

Juan Gonzalez-Valez, MD, PhD (UCSF)

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

Keith Ogasawara, MD (Kaiser Permanente Medical Center, Honolulu)

#### *In utero* transfusion protocol

Greg Ryan, MB (Mount Sinai Hospital, University of Toronto)

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

Tippi MacKenzie, MD (UCSF)

6:55 pm

### Live panel: Interdisciplinary discussion regarding prenatal care for ATM

Moderator: Juan Gonzalez-Valez, MD, PhD

7:25 pm

### Close Meeting

Tippi MacKenzie, MD (UCSF)

7:30 pm

### **ADJOURN**

January 9, 2021, 3:00 pm - 7:30 pm PST time/date

3:00 -  
3:50 pm

**Forum A: Specialists discuss IUT protocol consensus**  
Moderator: Juan Gonzalez-Valez, MD, PhD (UCSF)  
[Zoom details will be sent to meeting registrants](#)

3:00 -  
3:50 pm

**Forum B: Building a patient-family group for ATM**  
Moderator: Sandra Gilbert, PhD (Cooley's Anemia Foundation)  
[Zoom details will be sent to meeting registrants](#)

4:00 pm

**Welcome and overview - Day 2**  
Tippi MacKenzie, MD (UCSF)

4:05 pm

**Family Experience 2**  
Conversations with families affected by ATM

4:30 pm

**Postnatal therapies for ATM**  
Moderator: Vip Viprakasit, MD, DPhil

**Postnatal transfusion protocols**  
Elliott Vichinsky, MD, Ashutosh Lal, MD (UCSF)

**Postnatal hematopoietic stem cell transplantation**  
Alexis Thompson, MD, MPH (Northwestern Feinberg School of Medicine)

**Transfusion and transplantation in Thailand**  
Vip Viprakasit, MD, DPhil (Siriraj Hospital, Mahidol University)

5:05 pm

**Live panel: Interdisciplinary discussion regarding opportunities and challenges of providing global postnatal care for ATM**  
Moderator: Vip Viprakasit, MD, DPhil

5:30 pm

**Thalassaemia International Federation (TIF)**

5:35 pm

**BREAK**

January 9, 2021, 3:00 pm - 7:30 pm PST time/date

5:40 pm

**Live panel: Discussion with families and providers**

Moderator: Barbara Koenig, PhD, Director  
UCSF Bioethics Program

Introduction by Craig Butler, MA, Executive Director  
Cooley's Anemia Foundation

6:25 pm

**Close Meeting**

Tippi MacKenzie, MD (UCSF)

6:35 pm

**ADJOURN**

# Advances in Alpha Thalassemia Major: No Longer a Fatal Disease

January 8-9, 2021 (San Francisco)

**Tippi MacKenzie, MD, Conference Chair**

Co-Director, Center for Maternal-Fetal Precision Medicine  
UCSF Benioff Children's Hospital  
University of California, San Francisco

**Mary Norton, MD**

Co-Director, Center for Maternal-Fetal Precision Medicine  
UCSF Benioff Children's Hospital  
University of California, San Francisco

**Ali Amid, MD**

University of Ottawa

**Ashutosh Lal, MD**

UCSF Benioff Children's Hospitals

**Billie Lianoglou, LCGC**

UCSF Benioff Children's Hospitals

**Tachjaree Panchalee, MD**

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