

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.

Given the prevalence of ATM in Southeast Asia, the conference is scheduled so healthcare providers and families this region can participate during daytime hours.

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

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[Register](#)

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 Wayne, PhD (McMaster University, 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

Introductions: 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 (Mount Sinai Hospital, Ontario Fetal Centre)

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: Tippi MacKenzie, MD

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 González-Velez, 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 (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
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Elliott Vichinsky, MD

UCSF Benioff Children's Hospitals

Ashutosh Lal, MD

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Billie Lianoglou, LCGC

UCSF Benioff Children's Hospitals

Marisa Schwab, MD

UCSF Benioff Children's Hospitals



Ali Amid, MD

University of Ottawa

Vip Viprakasit, MD, DPhil

Siriraj Hospital, Mahidol University

Tachjaree Panchalee, MD

University of California, San Francisco
Siriraj Hospital, Mahidol University

UCSF Center for Maternal-Fetal
Precision Medicine

CIRM
CALIFORNIA'S STEM CELL AGENCY

UCSF Benioff Children's Hospitals
Fetal Treatment Center
Northern California
Comprehensive Thalassemia Center

Cooley's Anemia
FOUNDATION

TIA
THALASSAEMIA
INTERNATIONAL
FEDERATION

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