Advances in

ALPHA THALASSEMIA MAJOR: NO LONGER A FATAL DISEASE

Virtual Conference for Providers and Families

Register

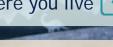
All are welcome
Registration is free
CME credit available

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





- 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

Conference Summary

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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DAY 1 Advances in Alpha Thalassemia Major

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, 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



DAY 1 Advances in Alpha Thalassemia Major

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



DAY 2 Advances in Alpha Thalassemia Major

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 4:05 pm	Welcome and overview - Day 2 Tippi MacKenzie, MD (UCSF) 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 5:35 pm	Thalassaemia International Federation (TIF) BREAK



DAY 2 Advances in Alpha Thalassemia Major

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

Organizing Committee

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
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Northern California Comprehensive Thalassemia Center



