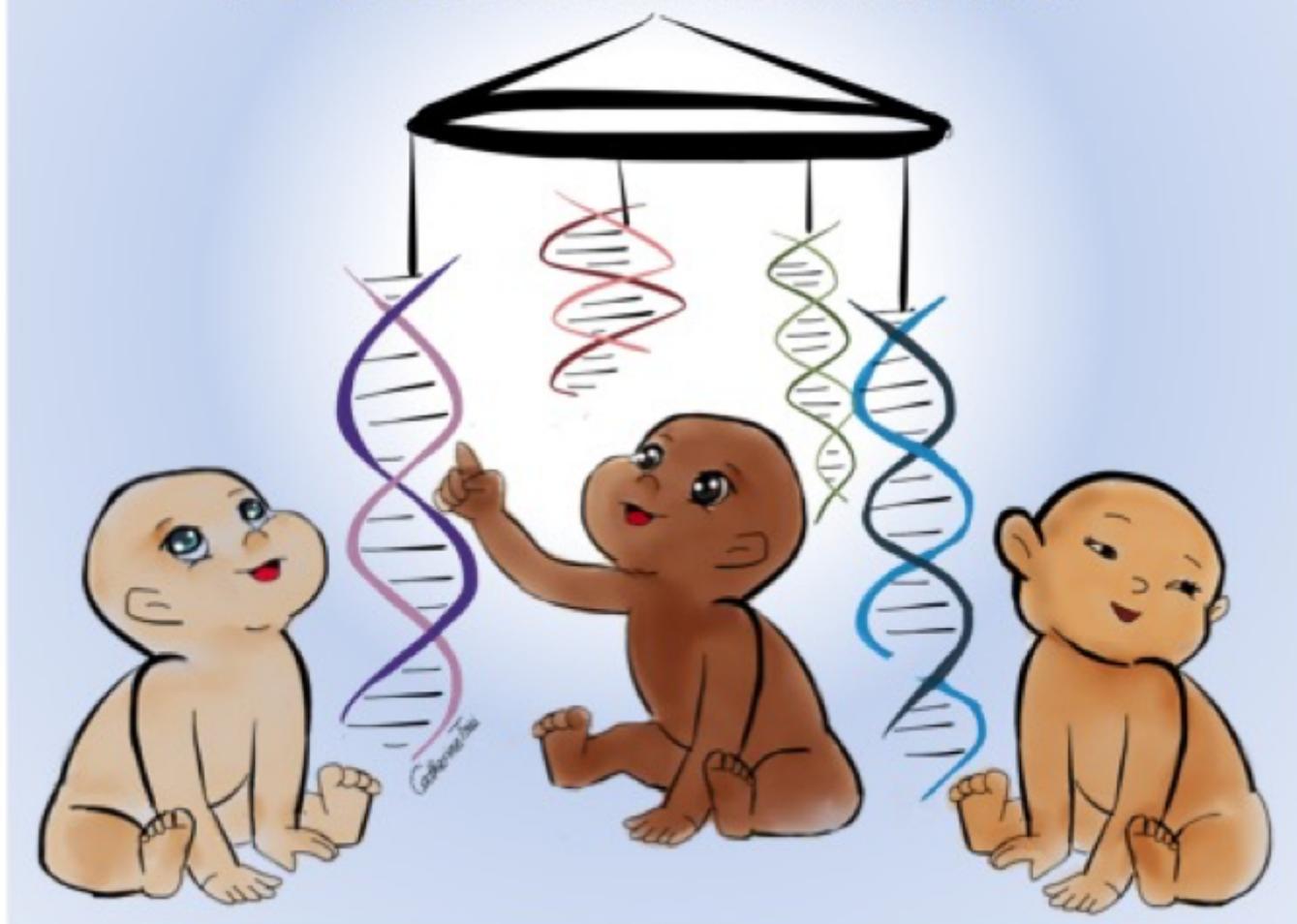


## Intrauterine Therapy for Alpha Thalassemia Major



# UCSF Center for Maternal-Fetal Precision Medicine



[2]

The UCSF Fetal Treatment Center, UCSF Center for Maternal-Fetal Precision Medicine, and UCSF Benioff Children's Hospital Oakland Thalassemia Center's Northern California Comprehensive Thalassemia Center have established the first multidisciplinary center for Alpha Thalassemia Major [1]. The program is designed to address the complex diagnostic,

prenatal, intrauterine, and perinatal management issues affecting a family with an Alpha Thalassemia Major pregnancy. Maternal and fetal complications of an Alpha thalassemia pregnancy are common and can be serious.

Prevention of Alpha Thalassemia Major with genetic counseling has been widely accepted throughout the world, but some families are seeking alternative options. We are offering intrauterine transfusions for fetuses with Alpha Thalassemia Major.

There is growing experience with fetal therapy for alpha thalassemia. Fetuses treated with intrauterine transfusions throughout pregnancy starting at an early gestational age (18-25 weeks) can survive to birth with acceptable morbidity. After birth, the treatment options include bone marrow transplantation or continued chronic transfusions. In the future, we plan to offer in utero stem cell transplantation for this disease.

For more information regarding Alpha Thalassemia Major, fetal intervention, and our multidisciplinary center, please visit:

Conditions & Treatments: Alpha Thalassemia <sup>[3]</sup>

The Northern California Comprehensive Thalassemia Center <sup>[1]</sup>

For more information about this treatment, or to refer a patient, please call 1-800-RX-FETUS (1-800-793-3887) or send us an email at [fetaltreatmentcenter@ucsf.edu](mailto:fetaltreatmentcenter@ucsf.edu) <sup>[4]</sup>.

Stopping a Fatal Blood Disease in the Womb [5] from UCSF: The Campaign [6] on Vimeo [7].

## Phase 1 Clinical Trial: In Utero Hematopoietic Stem Cell Transplantation for Alpha-thalassemia Major (ATM)

In utero stem cell transplantation was developed as a strategy to address the challenges associated with transplantation after birth. In this approach, the mother's stem cells are transplanted into the fetus, taking advantage of the fact that the mother and fetus tolerate each other's cells during pregnancy.

If the transplant is successful and the mother's stem cells are engrafted (incorporated into the baby's own bone marrow), the baby will be able to make normal blood cells. If the transplantation is not fully successful and engraftment is weaker, a booster transplant of the mother's stem cells may be performed after delivery. Since even low engraftment could result in long lasting tolerance to the mother's cells, this booster transplant can improve the baby's ability to make normal blood cells. A booster transplant is expected to be safer than current methods of stem cell transplantation after birth.

Mothers who choose to participate in this clinical trial will have stem cells harvested from their bone marrow. These cells will then be prepared for safe injection and transplanted into the fetus at the same time as an in utero transfusion (IUT). The fetus will have additional blood transfusions until birth. The success of the transplant will be evaluated after birth.

While we believe in utero transplantation can be performed safely, it is possible that it may not be effective. Potential risks of the procedure are that the fetus may become sick after the in utero transplantation or may not survive the therapy. Additionally, the mother may need a blood transfusion after donating bone marrow. In the event that in utero transplantation is not successful, repeated blood transfusions will be performed after birth and stem cell transplantation may be considered.

For more information visit the clinical trial information at [ClinicalTrials.gov](http://ClinicalTrials.gov): In Utero Hematopoietic Stem Cell Transplantation for Alpha-thalassemia Major (ATM) [8]

You can also download our PDF brochure: [Alpha Thalassemia Major Clinical Trial Brochure](#) [9] and read our paper on [Favorable outcomes after in utero transfusion in fetuses with alpha thalassemia major: a case series and review of the literature](#) [10].

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**Source URL:** <https://fetus.ucsf.edu/research/intrauterine-therapy-alpha-thalassemia-major>

### Links

[1] <http://thalassemia.com/services-intrauterine-therapy.aspx#gsc.tab=0>

[2] <http://mfprecision.ucsf.edu/>

[3] <https://fetus.ucsf.edu/alpha-thalassemia>

[4] <mailto:fetaltreatmentcenter@ucsf.edu>

[5] <https://vimeo.com/295220171>

[6] <https://vimeo.com/ucsfthecampaign>

[7] <https://vimeo.com>

[8] <https://clinicaltrials.gov/ct2/show/NCT02986698?cond=Alpha+Thalassemia+Major&rank=1>

[9]

<https://fetus.ucsf.edu/sites/fetus.ucsf.edu/files/wysiwyg/Alpha%20Thalassemia%20Major%20brochure%20for%20pr>

[10] <http://rdcu.be/JpvW>