

## Fetal Treatment Research

Fetal therapy is a very young and rapidly developing science. Essentially all the relevant scientific knowledge, diagnostic techniques, and therapeutic innovations have been developed in the last two decades. And, most of this knowledge comes directly from research ? both experimental basic research in animal models and clinical research in human patients.

The paradigm that launched fetal intervention 25 years ago in San Francisco has served as a model for the development of fetal therapy around the world. This paradigm requires exploration of the pathophysiology of the fetal disease in experimental animal models, and the definition of the natural history and outcome of the untreated disease by careful serial observation of fetuses with the disease. Only when the experimental pathophysiology is worked out, the natural history understood, selection criteria established, and the intervention tested for safety, is a new fetal intervention offered patients.

This mixture of experimental and clinical research has been a constant feature of the Fetal Treatment Center approach for the last 25 years. It has been and continues to be powered by bright, young scientists and physicians from many different disciplines who have been attracted to the challenges and excitement of developing the field of fetal intervention. An overview of the development of fetal therapy and a representative list of the individuals involved and the research topics they covered is available in a recent review article [Harrison MR. *Fetal Diagnosis and Therapy* [1] 2004;19(6):513-24;]

The range of research done at the UCSF Fetal Treatment Center can be appreciated by scanning the titles of publications in the curriculum vitae of Michael Harrison. The best overview of the broad range of research covered in developing fetal therapy worldwide is found in the third edition of *The Unborn Patient* [2].

## Clinical Research

Ongoing clinical research includes three randomized clinical trials supported by the National Institutes of Health. The Management of Myelomeningocele Study (MOMS) [3] trial is a three-center study (UCSF, Children's Hospital of Philadelphia, Vanderbilt University Medical Center) comparing prenatal surgical repair of myelomeningocele to standard postnatal care. The Twin-Twin Transfusion Trial is a multicenter study comparing laser ablation of placental vessels to amnioreduction in the treatment of severe twin-twin transfusion syndrome. We are just completing our second trial on fetal congenital diaphragmatic hernia?this one compares fetal temporary tracheal balloon occlusion with standard postnatal repair of severe CDH.

## Experimental Research

Experimental research continues in the laboratory on a variety of potentially curable fetal diseases, including congenital diaphragmatic hernia [4], gastroschisis [5], twin-twin transfusion syndrome

[6], congenital heart disease [7], and diseases that can be cured by hematopoietic stem cell transplantation [8]. Presently, our most intensive laboratory research is focused on solving the general problem of safe access to the fetus early in gestation, including the problem of membrane separation after intervention and the development of new smaller and better minimally invasive techniques.

## Pediatric Surgery Lab

The pediatric surgery research effort is dedicated to helping improve the health of children. The problems of our small patients also become the motivation for investigators in the basic sciences to study mechanisms of disease and to develop new therapies to cure previously untreatable birth defects.

For more information on the research of the Fetal Treatment Center and Pediatric Surgery visit the UCSF Pediatric Surgery Lab [9]

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### Links

[1]

[http://www.ncbi.nlm.nih.gov/entrez/query.fcgi?cmd=Retrieve&db=pubmed&dopt=Abstract&list\\_uids=1](http://www.ncbi.nlm.nih.gov/entrez/query.fcgi?cmd=Retrieve&db=pubmed&dopt=Abstract&list_uids=1)

[2] <http://www.amazon.com/gp/product/0721684467/qid=1110495850/sr=8->

[1/ref=sr\\_8\\_xs\\_ap\\_i1\\_xgl14/103-6369586-0647803?n=507846&s=books&v=glance](1/ref=sr_8_xs_ap_i1_xgl14/103-6369586-0647803?n=507846&s=books&v=glance)

[3] <https://fetus.ucsf.edu/research/spina-bifida-moms-trial>

[4] <https://fetus.ucsf.edu/cdh>

[5] <https://fetus.ucsf.edu/gastroschisis>

[6] <https://fetus.ucsf.edu/tfts>

[7] <https://fetus.ucsf.edu/congenital-heart-disease>

[8] <https://fetus.ucsf.edu/stem-cells>

[9] <http://pedsurglab.surgery.ucsf.edu>