

# CHOP Research Institute Summer Scholars Program



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General, Thoracic and Fetal Surgery

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**Basic Research:** Research most often conducted in a laboratory setting that is designed to enhance our scientific knowledge base (does not involve human subjects)

**Translational Research:** Research focused on bringing concepts in laboratory research directly into medical practice (may or may not involve human subjects)

## Fetal Bone Marrow Transplants and Genome Editing to Prevent Congenital Disease

We are interested in harnessing the normal developmental processes of the fetus to offer potential treatments for congenital diseases. Specifically, we perform bone marrow transplants or CRISPR-Cas9 genome editing in fetuses to take advantage of the developing immune system and proliferative nature of stem cells. By performing the transplant at this age, we avoid having to find a “matching donor” and the toxic/immunosuppressive treatments required for postnatal transplants or the immune responses that pose a barrier to postnatal gene therapy/editing. Target diseases for this research are sickle cell anemia and surfactant protein deficiency among others.

- **Potential summer research project:**  
Projects involve studying ways to optimize donor cell engraftment following in utero bone marrow transplantation in the mouse model or studying the efficiency of genome editing following in utero delivery of CRISPR/Cas9.
- **Students will learn the following techniques:**  
(1) Bone marrow transplant in fetal and adult mice; (2) Flow cytometry to analyze and quantify the presence of donor cells in the blood of mice previously transplanted as a fetus; and (3) Analyzing fetal bone and liver on histology slides to assess for the presence of donor cells after in utero bone marrow transplantation.

Please click [here](#) to read more about Dr. Peranteau