

**TITLE: Assessment of a new mouse model of Sanfilippo syndrome**

**FACULTY MENTOR NAME, EMAIL PHONE NUMBER**

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**FACULTY MENTOR DEPARTMENT - Medicine**

**RESEARCH PROJECT DESCRIPTION** (brief overview of background, hypothesis, methods, role of medical student, funding and relevant publications -- SHOULD NOT EXCEED ~ 250 WORDS)

Sanfilippo syndrome is a lysosomal storage disease characterized by deficiency of N-acetylglucosaminidase. It affects about one in 60,000 births in the US and results in death, primarily in the late teens. The children go from relatively normal to a loss of vision, loss of hearing, loss of musculoskeletal coordination, and loss of communication over a period of 1 to 2 decades. We have constructed a knock in the model of this disease. The mouse is born deficient and we can turn on the gene. The project will involve characterization of the background level of activity in different organs using biochemical assays and assessments of hearing, vision, and coordination. The student will perform the biochemical assays and behavioral tests. The student will also assist with the analysis of the data.

**Heldermon CD**, Qin EY, Ohlemiller KK, Herzog ED, Brown JR, Vogler C, Hou W, Orrock JL, Crawford BE, Sands MS. Disease correction by combined neonatal intracranial AAV and systemic lentiviral gene therapy in Sanfilippo Syndrome type B mice. Gene Ther. 2013 Mar 28. doi: 10.1038/gt.2013.14.

**TITLE: Assessment of maternal fetal Microchimerism**

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During gestation, there is an exchange of stem cells between the maternal and fetal circulations. We have used a mouse model of a lysosomal storage disorder, in order to determine if we can enhance this phenomenon. Many recessive traits can be effectively corrected with as little as 1% of normal enzyme activity. We are attempting to achieve this level of maternal cells in the fetus. We have treated pregnant dams with various factors in order to achieve this.

The students will analyze slides and collate data to determine which interventions were most successful.

**TITLE: Biomarkers Of Breast Cancer**

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**RESEARCH PROJECT DESCRIPTION** (brief overview of background, hypothesis, methods, role of medical student, funding and relevant publications -- SHOULD NOT EXCEED ~ 250 WORDS)

We've created mouse models of breast cancer using primary tumor xenografts from patients. After the growth of tumors, we've harvested blood.

The medical student will analyze this blood using mass spectrometry and ELISA to identify circulating biomarkers of disease.