FW: Urgent HFT request from HHS

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Date: Sat, 04 May 2019 12:54:46 +0000
Attachments: NIH HFT Overview & Examples May 2019.2.docx (24.28 kB)

Hi Larry,

Apologies – I tried to send this last night, but apparently it didn't go through. The attached are the HFT examples requested by Brett. If it looks OK to you, I will send over. Thanks! Cheers, Carrie

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NIH-funded Research using Human Fetal Tissue

NIH conducts and funds basic, preclinical, and clinical research involving the analysis or use of human fetal tissue for a wide range of diseases and conditions. Studying human fetal tissue allows researchers to understand the processes, abnormalities, and pathologies unique to early human development. In FY2019, NIH supported 200 grants and projects that involve research with human fetal tissue. Several examples of NIH-supported research using human fetal cells and tissues are described below.

Brain Development

The BrainSpan Atlas of the Developing Brain, a partnership among the Allen Institute for Brain Science, several academic institutions, and NIH, has created a comprehensive 3-D brain blueprint. The Atlas details the anatomy of the brain's underlying structures, and exactly where and when particular genes are turned on and off during mid-pregnancy – a time during fetal brain development when slight variations can have significant long-term consequences. The BrainSpan Atlas has been used to identify genetic networks relevant to autism and schizophrenia; in both studies, the fetal pattern of gene expression revealed relationships that could not be detected by studying gene expression in the adult brain.

Alzheimer's Disease

Alzheimer's disease affects 5.8 million people in the U.S. NIH is currently funding a project that has already demonstrated that human fetal neural cell lines transplanted into a mouse model of Alzheimer's disease improved cognition and reduced the level of amyloid plaque in the mouse brains.

Spinal Cord Injury

As many as 450,000 people in the U.S. have a spinal cord injury and 11,000 spinal cord injuries occur in the U.S annually. NIH has supported a spinal cord injury research project at the [redacted] for many years. The researchers used human fetal spinal cord neural progenitor cells to show spinal cord regeneration in a rodent model in 2016. The researchers then generated similar neural progenitor cells from human embryonic stem cells, which they compared to the fetal cells, and demonstrated that the embryonic cell-derived neural cells also functioned in a rodent model in 2018. In 2019, these researchers demonstrated spinal cord regeneration from a human fetal cell line in a non-human primate model.

Amyotrophic Lateral Sclerosis

Amyotrophic Lateral Sclerosis (ALS, also known as Lou Gehrig's disease), is a group of progressive rare neurological diseases that mainly involve the neurons responsible for controlling voluntary muscle movement. There is no cure. CDC estimates that over 17,000 people in the U.S. have ALS. NIH has funded multiple grants for preclinical and clinical research in the development of a cell-based therapy to treat ALS using human fetal neural cells. Most recently

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NIH funded a Phase 1b clinical trial. Neuralstem Inc is continuing development of the cell product, NSI-566, which has received orphan designation by the FDA.

Neuralstem is also conducting clinical trials with the same cell product for other indications – spinal cord injury (phase I results published) and stroke (neither is NIH funded). The Department of Defense also awarded a Phase 1 Small Business Innovation Research contract to Neuralstem in 2018 to develop the product as a candidate treatment for severe Traumatic Brain Injury.

Age-Related Macular Degeneration

Age-related macular degeneration (AMD) is estimated to affect 196 million adults worldwide by 2020; for the "dry" form, there is no treatment. A researcher in the NIH intramural program is developing a human retinal pigment epithelium (RPE) potential therapeutic from induced pluripotent stem cells (iPSCs). Human fetal RPE cells are being used to validate the iPSC-derived RPE cells. Recent findings from the NIH laboratory demonstrate that clinical-grade iPSC-derived RPE patches rescue retinal degeneration in rodents and pigs. NIH plans to file an Investigational New Drug application with FDA soon.

Zika Virus

There were 72 cases of Zika virus disease in 2018 in the U.S., and 148 in U.S. territories, according to CDC. The Zika virus can cause fetal birth defects, including microcephaly (a head size much smaller than normal).

An NIH-funded research team identified types of human brain cell types infected by the Zika virus and screened compounds using cultures from human fetal brain cells to find that an approved antibiotic inhibits the virus in one cell type. NIH also funded a pilot project to develop a cell culture system to study Zika virus infection with different human fetal neural cell lines.

NIH is currently funding a Zika in Infants and Pregnancy prospective study of ~6,000 pregnant women in Brazil, Colombia, Puerto Rico, Nicaragua, Guatemala, and Peru. The researchers follow women throughout their pregnancies for Zika infection and congenital anomalies and collect and study human fetal tissue from lost pregnancies. NIH is also funding research using human fetal tissue to examine how certain cells of the immune system may control Zika infection during pregnancy.