



The Honorable Jan Schakowsky
Ranking Member
Select Investigative Panel on Infant Lives
U.S. House of Representatives
Washington, DC 20515

APR 06 2016

Dear Ranking Member Schakowsky:

Thank you for your recent letter regarding medical research using human fetal tissue. The use of fetal tissue in medical research has been an instrumental component of our attempts to understand, prevent, and treat a number of conditions and diseases that affect millions of Americans. Scientists have been working with human fetal tissue since the 1930s. For example, human fetal tissue is an important resource for researchers studying retinal degeneration, pregnancy loss, human development disorders such as Down Syndrome, and early brain development. Fetal tissue has also served as a critical resource for the development of models of human disease, such as HIV/AIDS, which has devastating effects on the human immune system. Importantly, cell lines derived from fetal tissue have also played an essential role in the creation of new vaccines and remain valuable in important efforts such as the pursuit of a vaccine for Ebola.

As described more specifically below, human fetal tissue is a critical resource for a number of areas of research. Fetal tissue has a unique biological flexibility compared to more differentiated adult tissue allowing cells from fetal tissue to often more easily adapt and grow in new environments, which makes it a better research resource for specific lines of scientific inquiry. In some cases, advanced cellular technologies such as induced pluripotent stem cells (iPSCs) are being explored as new model systems for complementing or potentially replacing studies with fetal tissue. However, even if a new model system is developed, it still must be validated against an established model system. This validation process is critical to ensuring that the new model system can replicate biological functions and disease states, ultimately allowing it to serve as a suitable alternative to existing models. Currently, research involving human fetal tissue is essential to the following areas of research:

Understanding human development

Human fetal tissue is critical for understanding how typical fetal development occurs and addressing diseases and conditions that affect the health of developing infants. For example, scientists are using fetal tissue to study the immune systems of the fetus and mother, and any incompatibilities arising due to infection or inflammation that may lead to rejection, miscarriage, or preterm birth. Fetal tissue is being used to identify biomarkers in maternal blood serum to help predict early pregnancy loss and allow for possible interventions to prevent such events.

Additionally, fetal tissue is also being used to study the origin and genetic roots of structural birth defects that are the leading cause of infant deaths.

Informing our understanding of brain disorders

A major focus of HHS-funded neuroscience research is to study crucial processes underlying typical and atypical brain development. Many of these processes contribute to the functions that give rise to unique human behaviors and studying such processes requires the use of human tissue. Fetal tissue affords researchers with the opportunity to answer specific questions that cannot be answered using other model systems because tissue contains information about structural features and brain architecture that other types of cells may not necessarily provide. Topics of study include critical windows of human brain development during pregnancy, when developing brains are especially vulnerable to injuries that lead to cerebral palsy and lifelong cognitive disabilities; how mutations lead to conditions like Down Syndrome or Miller-Dieker Syndrome; and, how nerve cells in the brain are affected by preterm birth, potentially contributing to epilepsy and various cognitive disorders.

Working toward treatments for retinal diseases

Human retinal degeneration studies rely on a well-established model using human fetal retinal pigment epithelium (RPE) tissue. Within the retina, RPE is the key tissue layer that regulates and nourishes the light-sensitive nerve cells. Damage and destruction of the RPE can lead to many neurodegenerative diseases, including age-related macular degeneration, the leading cause of irreversible blindness in the United States. Human fetal RPE tissue best represents the RPE found in the intact eye and thus allows for important comparisons in current work to develop cell-based therapies using induced pluripotent stem cells (iPSCs).

Historical use in vaccine development

The ability to propagate and grow sufficient virus to make cost efficient vaccines has been aided by the development and use of human cell lines of fetal origin. The poor growth characteristics of viruses, coupled with the inability to keep non-fetal, healthy human cells growing in culture for extended periods of time, made these well characterized and perpetually growing cell lines the best option for the production of many viral vaccines. Furthermore, current vaccines used to prevent hepatitis A, rubella, varicella (chickenpox), and zoster (shingles) in the United States are produced in long established human cell lines of fetal origin.

Current vaccine development

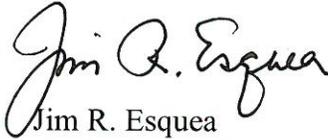
Scientists at the National Institutes of Health's National Institute of Allergy and Infectious Diseases (NIAID) Vaccine Research Center (VRC), in collaboration with GlaxoSmithKline, developed an experimental vaccine that uses the chimpanzee adenovirus type as a carrier to express an Ebola virus protein designed to stimulate protective immune responses. The experimental vaccine is produced in a cell line derived from fetal tissue that was obtained by the VRC from a non-profit repository in 2009. A NIAID-sponsored clinical trial, Partnership for Research on Ebola Vaccines in Liberia (PREVAIL I), is now underway in Liberia to test the

safety and immunogenicity of this vaccine candidate as well as another candidate. Interim results from this trial showed that both experimental vaccines appear to be safe, and, in May 2015, NIAID announced the successful enrollment of all 1,500 participants in the Phase 2 portion of the PREVAIL I trial. A safe and effective Ebola vaccine will be a critically important tool to help prevent Ebola virus disease and contain future outbreaks.

In closing, it is important to emphasize that one can never be certain where the next cure or treatment will come from, and maximizing researchers' access to diverse tools, methods, and experimental systems is critical for enhancing the likelihood of success in advancing the HHS mission. It is impossible to predict what types of cells or systems will be necessary for answering particular research questions or developing new treatments and cures. Thus, human fetal tissue is likely to remain a unique and invaluable resource for studying both typical and atypical processes early in development, elucidating the pathogenesis of infectious disease, advancing our understanding of a wide range of conditions, and developing new treatments and cures.

We hope you find this information helpful. Thank you for your interest in the important work of our Department.

Sincerely,



Jim R. Esquea
Assistant Secretary for Legislation

cc: The Honorable Marsha Blackburn
Chairman