

# Michigan Researchers Develop Disease-Specific Stem Cell Lines

National Hemophilia Foundation's E-Notes, May 2011

Great Lakes Hemophilia Foundation  
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Researchers from the University of Michigan (U-M) in Ann Arbor have developed the state's first embryonic stem cell lines that carry genes linked to specific inherited diseases, including hemophilia.

On April 4, U-M announced the creation of two stem cell lines: one carries the gene for hemophilia B (factor IX deficiency) and the other for Charcot-Marie-Tooth disease, a neurological disorder that causes progressive degeneration of the foot, lower leg and hand muscles.

Stem cells are unspecialized cells that can renew themselves for prolonged periods. They can also develop into many different cell types, making them a potentially renewable source of replacement cells that could be used in the future to treat many conditions. The creation of such stem cell lines will open the door to an enhanced understanding of the origin and progression of congenital disorders, and hopefully to new treatments based on those findings. "These stem cell lines hold so much promise for medical science, and for this reason, they will be of tremendous interest to researchers around the world," said Eva L. Feldman, MD, PhD, director of U-M's A. Alfred Taubman Medical Research Institute.

Access to embryonic stem cells was first made possible in Michigan in November 2008, when voters approved Proposal 2, a state constitutional amendment. The law allows investigators to generate new lines from unused embryos donated by fertility clinics. Instead of discarding the embryos, which in some instances carry the genes responsible for congenital disorders, scientists now have a new approach for advancing research.

The amendment led to a partnership between U-M's Consortium for Stem Cell Therapies (CSCT) and Detroit-based Genesis Genetics, a company specializing in pre-implantation genetic diagnosis (PGD), a test used to identify days-old embryos carrying disease-causing genetic mutations. This partnership now gives patients the option of donating embryos that test positive for a genetic disorder to the CSCT.

While the use of disease-specific embryonic stem cell lines looks promising, scientists are still in the relatively early stages of investigation. It will take years of preclinical and clinical research before actual treatments for diseases, such as hemophilia, become available.

"We are producing tools that can be of immeasurable aid to scientists studying such disorders as hemophilia and Huntington's disease. And we are just beginning to scratch the surface of this new scientific frontier," said A. Alfred Taubman, founder and chair of the A. Alfred Taubman Medical Research Institute.

*Source: University of Michigan news release dated April 4, 2011*