

# Prenatal Administration of Stem Cells for the Treatment of Sanfilippo Syndrome

**R**esearchers at the University of South Florida have developed a novel method of treating a fetus having Sanfilippo syndrome using stem cells from umbilical cord.

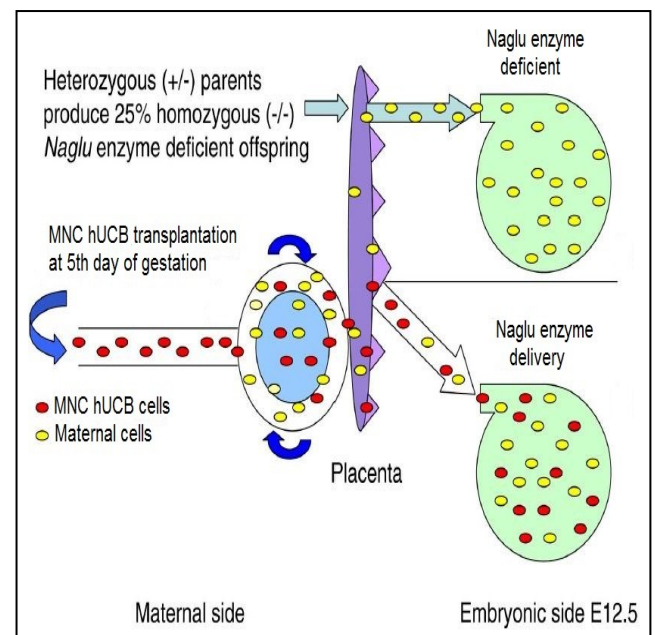
Sanfilippo is a rare genetic condition that causes fatal brain damage. It is referred to as a childhood disease because most patients never reach adulthood. Sanfilippo affects mostly the Central Nervous System (CNS). Over time, brain cells fill up with waste that the body is unable to process. As the brain gets progressively damaged, children experience hyperactivity, sleeplessness, loss of speech and cognitive skills, mental retardation, cardiac issues, seizures, loss of mobility, dementia and finally death, usually before adulthood. There is currently no treatment or cure available to families diagnosed with this devastating disease. Sanfilippo disease can be diagnosed prenatally, but current therapies (e.g., bone marrow transplant) are administered postnatally and only treat symptoms or halt disease progression, rather than reverse disease processes. Hence, there is a need for a treatment to cure Sanfilippo syndrome.

Our researchers have developed a method of injecting human umbilical cord blood cells, into a pregnant mammal to induce stem cell transmigration into embryos lacking proteins or only capable of synthesizing abnormal proteins. Pluripotent UCBCs, once delivered, replace the function of cells with the abnormal or missing protein and restore the normal cell phenotype, eliminating signs of Sanfilippo disease. This invention can be employed in a variety of congenital disorders, including neurological disorders, autoimmune disorders, vascular disorders involving inflammation. Hence, this method is effective for the treatment of Sanfilippo syndrome.

### ADVANTAGES:

- **Earliest intervention**
- **Avoids trauma to the fetus**
- **Administered prenatally to cure disease**

### *Umbilical Cord Stem Cells for the Treatment of Sanfilippo Syndrome*



### *Prenatal Delivery of Deficient Naglu Enzyme by Transplantation of MNC hUCB Cells into the Blood Circulation of a Mouse Modeling MPS III B*