Duke Studies New Approach in Fetal Transplants for Metabolic Disorders

October 13 2009

(PhysOrg.com) -- Researchers say a new development in cord blood transplants for inherited metabolic disorders may be curative for some babies who are treated while still in the womb.

Joanne Kurtzberg, MD, professor of pediatrics and pathology and director of the Pediatric Blood and Marrow Transplant Program at Duke University Medical Center, says the new approach uses a small, select number of therapeutic stem cells that have been treated to hasten and improve the process of engraftment.

Kurtzberg is formally studying the procedure in a pilot trial open to newly pregnant mothers known to be at risk for having children with lethal metabolic disorders.

Metabolic disorders -- also known as lysosomal storage disorders -- include several dozen rare, inherited genetic diseases marked by the absence of specific enzymes the body needs to break down and get rid of metabolic byproducts of energy production.

Left untreated, metabolic disorders can lead to bone, brain, and central nervous system problems and early death.

For the past decade or so, physicians have experimented with cord blood transplants after birth as a way of treating these diseases, and in many cases, treatments have been successful. Kurtzberg says the timing of the transplant is critical.

“The idea is to give the baby cord blood stem cells from a healthy donor that have the potential to provide healthy genes that can replace the ones that aren’t working properly in the baby’s own cells,” says Kurtzberg. Generally, she says that the earlier the treatment, the greater the chance the donor cells will work, so transplants before the baby is born are ideal.

The fetal transplant itself is fairly simple. After a physician diagnoses the presence of one of the genetic diseases and the parents consent to treatment, donor cells are injected directly into the baby’s abdomen at 12 to 14 weeks into the pregnancy.

After that, there is an extended period where the transplant needs to “take,” or engraft. At birth, the baby will be tested to see if donor cells are present and if they’re already working to fix the malfunctioning genes.

If not, the baby would be eligible for a conventional transplant with chemotherapy within the first few weeks of life.

Kurtzberg says the trial is open to pregnant women who are at risk of having a baby with Krabbe Disease, metachromatic leukodystrophy (MLD), Pelizaeus-Maerzbacher Disease (PMD), Tay-Sachs disease, or Sandoff Disease.

Donor cells will be manufactured by Aldagen, Inc., a biopharmaceutical company in Durham and a partner in the study. Kurtzberg has no interest in the company.

Provided by Duke University (news : web)