

Duke Translational Research Institute

#### **ROBERTSON CLINICAL AND TRANSLATIONAL CELL THERAPY (CT<sup>2</sup>) PROGRAM**

#### What is Cerebral Palsy?

Cerebral Palsy is abnormal development of the brain or damage to the developing brain that affects a child's ability to control his or her muscles. This damage can occur before, during birth or early in the child's life. Risk factors include low birth weight, premature birth, multiple births, infections during pregnancy, and complications during birth.

#### How are most cases of CP treated?

There is no cure for CP, but treatment may improve the lives of those who have the condition. It is important to begin a treatment program as early as possible. After a CP diagnosis is made, a team of health professionals works with the child and family to develop a plan to help the child reach his or her full potential. Common treatments include medicines; surgery; braces; and physical, occupational, and speech therapy. To date, there is no approved treatment to alter the damage to the brain.

### **Can Stem Cells help?**

There is much interest in using stem cells to halt or potentially reverse the damage to young brains. At Duke University, Dr. Joanne Kurtzberg and a team of clinicians are testing infusion of stem cells from a child's own umbilical cord blood that was saved at birth. Umbilical cord blood contains stem cells that can potentially facilitate neural cell repair resulting in improved function. Over 400 children have been treated this way at Duke University in early clinical studies.

### What is next with stem cell research for CP?

Since not all parents are able to save their child's umbilical cord blood, our goal is find a cell therapy using stem cells from umbilical cord blood that is universal for all affected children.

## What does it take to move this research program forward?

The research plan needs financial support to find a way to modify the cells from umbilical cord blood so they can be used for all children and not be immunologically rejected. This will also allow additional treatments for those who have depleted their banked cord blood or do not have saved cord blood.

### Why is this research so important?

- Medical costs for children with cerebral palsy alone were 10 times higher than for children without cerebral palsy or intellectual disability
- CP is the most prevalent motor disorder of childhood, affecting 2 to 3 of every 1,000 live births. In the US, approximately 10,000 babies and infants are diagnosed with cerebral palsy each year
- CP cruelly impacts the lives of children and the families who love and care for them

# How can I help?

- Donations to the Robertson Clinical and Translational Cell Therapy Program will go to develop and test a population of cells isolated from umbilical cord blood that can be used universally for all children with CP and related diseases. Current therapies are dependent on a supply of cells from the child's banked cord blood or a related donor. Many families and children do not have these options for a supply of cells. Research hopes to provide a supply of cells that can be used immediately after injury and throughout the course of the disease to prevent further damage or decline.
- Consider a private donation to a research center that you know is actively helping CP children and their families. Federal research dollars are highly competitive and have burdensome timelines; this program at Duke University is ready to initiate.
- Learn about the risk factors of CP and how to plan for a safe pregnancy if you are considering having a baby. Encourage your friends and families to save their child's cord blood in either public or private cord blood banks.

# To learn more about the Duke University Pediatric Blood and Marrow Transplant Center go to:

http://www.cancer.duke.edu/pbmt

Please mail all contributions to:

Duke University Robertson Clinical and Translational Cell Therapy CT<sup>2</sup> Program PO Box 3850 Durham, NC 27705



Dr. Joanne Kurtzberg Director, Carolinas Cord Blood Bank Chief Scientific Officer Robertson Clinical and Translational Cell Therapy Program Director, Pediatric Blood and Marrow Transplant Program

Joanne Kurtzberg, MD, is an internationally renowned expert in pediatric hematology/oncology, pediatric blood and marrow transplantation.

Dr. Kurtzberg pioneered the use of umbilical cord blood as an alternative stem cell source for unrelated marrow transplantation. Over the last two decades Dr. Kurtzberg has established a leading pediatric transplant program which currently treats children with cancer, blood disorders, immune deficiencies, hemoglobulinopathies and inherited metabolic diseases. Recent lines of investigation include the use of autologous cord blood in children with neonatal brain injury and cerebral palsy, as well as preclinical studies isolating oligodendryocytes from cord blood to eventually treat acquired genetic brain injuries.

Dr. Kurtzberg 's lab has developed novel assays to predict cord blood potency from segments attached to cryopreserved cord blood units, and is performing translational research testing cord blood expansion, cellular targeted therapies and tissue repair and regeneration. In 2012, under the direction of Dr. Kurtzberg, the Carolinas Cord Blood Bank received FDA approval for DuCord, a stem cell product derived from umbilical cord blood, for use in transplants between unrelated donors and recipients.