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\$15 Million Award to Go Toward Exploring New Treatments for Autism, Other Brain Disorders

By Duke Medicine News and Communications

DURHAM, N.C. -- Duke Medicine has been awarded \$15 million to support an innovative research program that explores the use of umbilical cord blood cells to treat autism, stroke, cerebral palsy and related brain disorders.



The award from The Marcus Foundation, an Atlanta-based philanthropic organization, will fund the first two years of a planned five-year, \$41 million project by Joanne Kurtzberg, M.D., chief scientific and medical officer of Duke's Robertson Cell and Translational Therapy Program, and Geraldine Dawson, Ph.D., director of the Duke Center for Autism Diagnosis and Treatment.

"I am excited about this unprecedented opportunity," said Victor Dzau, M.D., chancellor for health affairs and president and CEO of Duke University Health System.

"Joanne Kurtzberg has done groundbreaking work on cord blood transplantation at Duke, and Geri Dawson brings an enormous wealth of knowledge and experience of autism," Dzau said. "Together they will explore innovative approaches to treating these challenging brain disorders. This research holds the promise of truly transformational discovery, and we are deeply grateful to The Marcus Foundation for making it possible."

Kurtzberg and Dawson hope to develop cell-based therapies that can potentially restore brain function in people with the disorders, for which there currently are no cures. If successful, the study could identify therapies for further evaluation in clinical trials to potentially decrease disabilities and improve the quality of life for millions of children and adults.

"Duke University and Health System greatly appreciate the vision of The Marcus Foundation," said Duke President Richard H. Brodhead. "This gift will enable the expertise of Duke's medical researchers to be focused on new approaches and treatments, with the goal of bettering the lives of millions."

The project will consist of a series of clinical trials using umbilical cord blood cells to treat a total of 390 children and adults with autism, 100 children with cerebral palsy and 90 adults with stroke. Based on previous research, Kurtzberg and Dawson hypothesize that cord blood may promote repair of dysfunctional or damaged areas of the brain.

"Funding for this type of research is very scarce, so the only way we can truly make

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progress is with support from private philanthropic organizations like The Marcus Foundation,” said Nancy Andrews, M.D., Ph.D., dean of Duke University School of Medicine. “With the foundation’s help, we hope to give untold numbers of people with autism and related disorders hope for a better outcome.”

There are approximately 2 million people in the United States with autism spectrum disorder, a group of conditions affecting social communication and behavior. Stroke kills an average of nearly 130,000 in the U.S. every year, while cerebral palsy currently affects an estimated 764,000 children and young adults.

The initial phase of the program -- a preliminary trial involving 20 pediatric subjects with autism using their own banked cord blood -- is already under way. It will conclude with Phase II trials using donated cord blood in children with autism and cerebral palsy and adults with stroke.

“The whole program has enormous potential,” said Kurtzberg, who is also director of the Pediatric Blood and Marrow Transplant Program and the Carolinas Cord Blood Bank. “Autism, stroke and cerebral palsy are all neurologic conditions that impair function and quality of life for these children and adults. If we can make that better, it will have a huge personal and societal impact.”

Cord blood cells are collected without risk to the mother or baby from the placenta, which is otherwise discarded as medical waste after a baby is born. After collection, the cells can be frozen and stored for future use in blood stem cell transplantation or cellular therapies.

Kurtzberg’s previous research has shown that cord blood cells can reduce inflammation and signal normal cells to repair damage in areas of the brain affected by inherited pediatric brain diseases. A recently published Duke study indicates that treating children with their own cord blood cells can have similarly beneficial results in cases of hypoxic ischemic encephalopathy.

Building on those research results, the new study will try to determine whether cord blood cells will have the same effect in cases of autism and stroke. Using methods developed by Dawson, the study will examine whether the therapy not only improves behavioral outcomes in children and adults with autism, but also reshapes the patterns of brain activity.

Kurtzberg and Dawson also will explore the key question of whether the beneficial effects of cord blood can be achieved by treating patients with donated cord blood rather than only with their own banked blood cells. If that is found to be true, it could make cord blood treatment available to the largest numbers of patients in need.

“We all wanted to have a treatment that, if it’s effective, would be accessible to everyone,” said Dawson, who was the founding director of the University of Washington Autism Center and then chief science officer at Autism Speaks before joining the Duke faculty in August 2013.

Established by Bernie Marcus, the co-founder of The Home Depot, The Marcus Foundation has a long-established interest in autism, stroke, cerebral palsy and other neurological conditions, and in stem cell research. It focuses on biomedical research projects that are close to clinical application, that may bring novel therapies to bear on disorders without existing treatments, and that are unlikely to receive funding from traditional sources such as the National Institutes of Health.

Marcus also opened the Marcus Autism Center in Atlanta in 1991 and donated the funds to start the Autism Speaks advocacy organization in 2005.

Families interested in enrolling a child in the study should contact Duke at cordbloodtherapyinfo@dm.duke.edu or (844) 800-CORD or (844) 800-2673.

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