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Phase I Clinical Trial Examines Safety of Using Cord Blood Stem Cells for Treating Children with Autism Spectrum Disorder

Preliminary Findings Indicate Potential Efficacy of Cord Blood to Improve the Lives of Children with Social and Behavioral Challenges Associated with ASD

WALTHAM, Mass. – April 5, 2017– Novel research results from Duke University's Robertson Clinical and Translational Cell Therapy Program published today in the April issue of *Stem Cells Translational Medicine* indicate promising developments related to using a baby's own cord blood stem cells to treat Autism Spectrum Disorder (ASD).

Drs. Joanne Kurtzberg, MD, Director, Carolinas Cord Blood Bank and Pediatric Blood and Marrow Transplant Program; Chief Scientific Officer, Robertson Clinical and Translational Cell Therapy Program and Geraldine Dawson, PhD, Professor of Psychiatry and Behavioral Sciences; Professor of Pediatrics and Psychology and Neuroscience; Director, Duke Center for Autism and Brain Development, led the Phase 1 clinical trial. ViaCord, PerkinElmer's private cord blood and cord tissue preservation business, helped to fund the study and provided nearly one-half of the cord blood units used by the participating families.

"We are pleased that this study demonstrated the safety of treating children with ASD with their own cord blood," said Kurtzberg. "We're also encouraged that, while small and non-randomized, there were observed improvements in a majority of the children reported by clinicians and parents. Without the support of ViaCord and later, the Marcus Foundation, the study would not have been possible. We are now hoping to replicate these preliminary results in a Phase II randomized clinical trial for which enrollment is nearly complete."

The trial was designed to determine the safety of an intravenous infusion of autologous umbilical cord blood for the treatment of pediatric patients with ASD. A secondary research endpoint to evaluate the feasibility of outcome measures was based on the change in each child's socialization and communication abilities and autism symptoms, measured via parent report, direct observation, and clinician judgment. The study also incorporated novel methodologies using eye-tracking that assessed how the child processed social information. The results indicated that the treatment is safe and preliminarily indicated improvements in symptoms across all domains in the majority of children receiving an adequate cell dose.

"When my son was just a year old, he was overly sensitive to noises like lawn mowers and was not babbling at all," said Kristina, a mother whose child participated in the study. "But since his treatment at age 3, and in conjunction with therapy, his improvement has been steady and he's now very social and able to interact independently with his peers, he can communicate more clearly with children and adults, and his sensory problems are gone. With support, he entered a mainstream elementary school program this past fall. We are extremely grateful to have participated in this study, and so happy with his continued improvement, now years after his treatment."

"For more than 20 years, ViaCord has actively participated in developments to increase the clinical utility of cord blood stem cells. Over the last few years, cord blood applications have expanded beyond transplant medicine into areas of regenerative medicine like ASD and brain injuries," said Morey Kraus, Chief Scientific Officer, ViaCord. "I've never been more optimistic about the potential of cord blood. It is very gratifying to have collaboration with Drs. Kurtzberg and Dawson at Duke in this ground breaking trial having the potential to transform the lives of thousands of families who have children with ASD."

Further details on the Phase II trial can be found at:
clinicaltrials.gov/ct2/results?term=NCT02847182

ViaCord has preserved the umbilical cord stem cells of more than 350,000 newborns and released stem cells for the treatment of diseases such as acute lymphoblastic leukemia, thalassemia major, and sickle cell disease. It has also released more than 340 units of cord blood for clinical use, including this ASD study. For over 25 years, cord blood stem cells have been used in transplant medicine and today can be used in the treatment of nearly 80 diseases. Please visit www.viacord.com for more information. Follow us on Twitter @ViaCord.

About PerkinElmer
PerkinElmer, Inc., a global leader focused on innovating for a healthier world, provides private cord blood and cord tissue preservation to families through ViaCord, LLC. PerkinElmer reported revenue of approximately $2.1 billion in 2016, has about 9,000 employees serving customers in more than 150 countries, and is a component of the S&P 500 Index. Additional information is available through 1-877-PKI-NYSE, or at www.perkinelmer.com.

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