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Contact: Colleen O'Malley
comalley@spectrumscience.com
202-955-6222
Kennedy Krieger Institute

New study shows use of Lorenzo's oil prevents onset of pediatric neurological disorder

Treatment prevents symptoms for majority of young boys with adrenoleukodystrophy, ALD

BALTIMORE, Md. (July 11, 2005) – Use of Lorenzo's Oil in young boys who have been diagnosed with but are not yet showing signs of a pediatric neurological disorder known as X-linked adrenoleukodystrophy (X-ALD) may prevent the disease from developing in the body. According to a study of male children published today in the Archives of Neurology, use of the oil prevented onset of the disease in a majority of cases. For the first time, the study demonstrated the preventive effects of Lorenzo's Oil using rigorous and scientifically-accepted research methods.

The study followed 89 boys ages seven and younger who tested positive for an abnormal gene that identifies X-ALD. Over a period of seven years, researchers treated the boys with daily oral doses of Lorenzo's Oil, a combination of two fats extracted from olive oil and rapeseed oil, and moderately restricted their dietary fat. All patients had a normal brain MRI and showed no neurological signs and symptoms of X-ALD prior to the study. At the completion of the study, nearly three-fourths (74 percent) of the 89 patients showed no signs of disease progression, demonstrating a significant preventive effect. Researchers correlated greater compliance with a lower risk of developing the disease.

"This clinical study clearly demonstrates that the use of Lorenzo's Oil can prevent the onset of the rapidly progressive and devastating form of the brain disease that affects 50 percent of boys with X-ALD," said Hugo Moser, M.D., Director of the Neurogenetics Research Center at the Kennedy Krieger Institute in Baltimore, Md., and lead author of

the study. "This finding is an exciting progression of the research to which my associates and I have dedicated ourselves to help these children."

X-ALD affects 16,000 patients in the United States. The disease causes the breakdown of myelin, a fatty substance that acts as an insulator around nerve fibers. Symptoms appear between four and 10 years of age, and can lead to nerve deterioration, loss of verbal communication, strength and coordination and, eventually, complete breakdown of bodily function.

"The results of this trial offer clinical support for treating the many young boys identified at high-risk for the disease," said Gary Goldstein, M.D., CEO of the Kennedy Krieger Institute. "This advance results from truly collaborative efforts between researchers in the medical community, parents and advocates for these patients."

There is still no cure for X-ALD, and treatment options are limited to hormone therapy and bone marrow transplants. While these transplants can provide important long-term benefits to boys with X-ALD in whom brain involvement is still in the early stages, the procedure carries a significant risk of mortality and morbidity, and is not effective when brain involvement is already severe.

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About Lorenzo's Oil

Lorenzo's Oil was invented by Augusto and Michaela Odone, who began researching X-ALD and advocating for improved research after their son, Lorenzo, was diagnosed with the disease in 1984. The oil, named after the Odone's son, reduces the amount of abnormal fatty acids in the body that cause myelin degeneration. While the oil cannot repair damage already done, it can prevent the disease from progressing into a degenerative state. Though Lorenzo already showed symptoms before he began taking the oil, the treatment stopped the progression of the disease. He celebrated his 27th birthday on May 29, 2005. The story of the Odone's struggle against X-ALD was dramatized in the 1992 Universal Studios motion picture Lorenzo's Oil, starring Nick Nolte and Susan Sarandon.

About Hugo Moser, M.D.

Hugo Moser, M.D. is a research scientist and Director of the Neurogenetics Research Center at the Kennedy Krieger Institute in Baltimore, Maryland. Dr. Moser is also a University Professor of Neurology and Pediatrics at Johns Hopkins University. He was previously President of the Kennedy Krieger Institute.

Dr. Moser has focused his research on genetic disorders that affect the function of the nervous system in children, particularly those that involve a part of the cell referred to as the peroxisome. Of the 15 known peroxisomal disorders that lead to mental retardation and nervous system disabilities, the most common is ALD. Dr. Moser helped to identify the characteristic biochemical abnormalities and the gene mutations that cause each of these disorders. He established methods of early diagnosis, counseling and worldwide programs to evaluate methods of therapy, including diet, pharmacological agents and transplantation.

About the Kennedy Krieger Institute

Internationally recognized for improving the lives of children and adolescents with disorders and injuries of the brain and spinal cord, the Kennedy Krieger Institute in Baltimore, MD serves more than 11,000 children each year through inpatient and day treatment programs, outpatient clinics, home and community services and school-based programs. Kennedy Krieger provides a wide range of services for children with developmental concerns mild to severe, and is home to a team of investigators who are contributing to the understanding of how disorders develop and pioneering new interventions and earlier diagnosis. For more information on Kennedy Krieger Institute, visit http://www.kennedykrieger.org/.