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Scientists Identify Drugs for Batten Disease

-- Currently, no effective drug is available for Batten diseases, a group of devastating disorders in children. FDA-approved lipid-lowering drugs Lopid and Tricor, in combination with vitamin A, may help patients with late infantile Batten disease. --

CHICAGO, IL, September 20, 2012 **/24-7PressRelease/** -- Lopid and Tricor, FDA-approved lipid-lowering medications, may prevent the progression of late infantile Batten disease. Batten diseases are a group of devastating neurodegenerative disorders that begin in childhood. In case of the late infantile Batten disease, death usually occurs between the ages of 8 and 14 depending on the rate of disease progression. Currently, no drug is available to halt the disease progression.

Neurological researchers at the Rush University Medical Center (Chicago) have found that gemfibrozil and fenofibrate, known as Lopid and Tricor, respectively in US Pharmacies, successfully increase the amount and function of a protein that is either low or missing in late infantile Batten disease. Results of the study were published in the Journal of Biological Chemistry, September 18, 2012.

"Understanding mechanism of the disease is important to developing effective drugs that protect the brain and stop the progression of Batten," said Kalipada Pahan, the communicating author of the study. He is a professor of Neurological Sciences, Biochemistry and Pharmacology and the Floyd Davis Endowed Chair of Neurology at the Rush University Medical Center. Other researchers at the Rush University involved in this study were Arunava Ghosh and Grant T. Corbett.

Although due to gene mutation, one protein called tripeptidyl tripeptidase I (TPP-I) is absent in late infantile Batten disease, a few copies of normal gene remain in affected patients. "Therefore, one approach for treatment could be to enhance the levels of normal TPP-I protein", said Dr. Pahan.

The authors have shown that in addition to Lopid and Tricor, retinoic acid (vitamin A) also increases the TPP-I protein in brain cells. "Therefore, vitamin A supplementation could be beneficial for patients with late infantile Batten disease," Dr. Pahan said.

Sometimes a combination of low-dose medications works better than larger doses of one single drug. "Interestingly, the combination of vitamin A and Lopid at very low doses greatly stimulates this protein," Dr. Pahan said.

This research was supported by a generous donation from Noah's Hope Foundation and a grant from National Institutes of Health to Dr. Pahan. "Now we need to translate this finding to the clinic and test if the combination of vitamin A and Lopid or Tricor delays disease progression and prolongs life span in affected children. If these results are replicated in patients, it would open up a promising avenue of treatment of this devastating disease," Dr. Pahan said.

Batten disease is rare and occurs in an estimated 2 to 4 out of every 100,000 births in the United States. It is inherited in an autosomal recessive pattern. The mutation causes the buildup of pigments called lipofuscins in the brain, leading to neuronal death. Eventually, children with Batten disease become blind, bedridden, and demented. Batten Disease is a life limiting disease; life expectancy varies depending on the type or variation. Death usually occurs in middle childhood between the ages of 5 and 14 years, depending on the speed of disease progression.

Website: http://www.pahanlab.com

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Press Release Contact Information:

Kalipada Pahan
Pahanlab.com
Professor
1735 West Harrison St
Chicago, IL
USA 60612
Voice: 312-563-3592
Fax: 312-563-3571
E-Mail: Email us Here
Website: Visit Our Website