

Health Canada Approves Drug for Treatment of Friedreich's Ataxia

In September 2008, Health Canada authorized (with conditions) the use of CATENA® (idebenone) in Canada. The drug is indicated for the symptomatic management of Friedreich's Ataxia, a rare neuromuscular disease that results in the degeneration of nerve and muscle tissue. This disorder causes loss of muscle control, uncoordinated movements, muscle wasting and thickening of heart walls, which frequently leads to a shortened life span.

Friedreich's Ataxia results from a genetic defect in the gene encoding for *frataxin*. Reduced levels of the frataxin protein ultimately result in impaired energy production in mitochondria, the cells' energy production centers, and elevated oxidative stress. Tissues that have the highest need for energy, in particular nerve and cardiac tissues, are primarily affected by frataxin deficiency resulting in pathological changes in heart muscle anatomy and function and loss of nerve cells.

CATENA® has shown an improvement of neurological performance in patients with Friedreich's Ataxia. It is the first and only approved therapy in Canada to treat this genetic disorder. The drug is thought to improve energy production by body cells. It may also scavenge (mop-up) substances that can cause oxidative stress and damage to cells. Health Canada's authorization of this drug reflects the promising nature of the clinical evidence of efficacy and safety, which must be verified and/or extended with a further study, which is currently underway.

Approximately several hundred Canadians are affected by Friedreich's Ataxia, with a higher density in the Quebec, where the prevalence ranks among the highest concentrations in North America.

For more information, please visit www.hc-sc.gc.ca/dhp-mps/alt_formats/hpfb-dgpsa/pdf/prodpharma/catena_fs_fd_117672-eng.pdf or www.santhera.com.

This information is intended for general education and awareness. Muscular Dystrophy Canada cannot provide medical advice related to individual diagnosis or treatment. Please consult your physician.