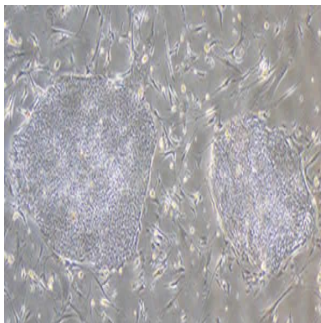

Dantrolene May Treat Wolfram Syndrome



According to a new research report from the Washington University School of Medicine, dantrolene, a commonly prescribed muscle relaxant, may be an effective treatment for a rare but devastating form of diabetes known as Wolfram syndrome. The results have been published in the Proceedings of the National Academy of Sciences (PNAS) Online Early Edition.

Dantrolene was able to prevent the destruction of insulin-producing beta cells in animal models of Wolfram syndrome and in cells that were taken from patients who have the illness. One in 500,000 people worldwide are affected by Wolfram syndrome and many patients with this disease die by the age of 40.

With Wolfram syndrome, very young children develop type 1 diabetes and need insulin injections several times a day. The syndrome also results in hearing loss, vision problems and difficulty with balance. The primary cause of the disease is elevated levels of an enzyme called calpain 2. The enzyme causes the death of brain cells and insulin-producing cells.

Dantrolene was shown to block the calpain 2 enzyme and prevent brain cell death in both animal and cell models. Dantrolene is already approved by the FDA, and therefore clinical trials in patients suffering from Wolfram syndrome may get underway relatively quickly.

The research team investigated the effects of dantrolene on stem cells from Wolfram syndrome patients and their close relatives including parents and siblings. The stem cells were grown from skin cells and the researchers treated these stem cells with growth factors so that they would differentiate into neurons and insulin producing cells. The researchers observed that cells that came from Wolfram patients produced higher levels of calpain 2. They treated the cells with dantrolene and found that the levels of the enzyme dropped and the cells stopped dying.

According to Fumihiko Urano, MD, PhD, the Samuel E. Schechter Professor of Medicine and the senior investigator, "We also found that dantrolene was not toxic to cells grown from the skin samples donated by patients' relatives. The drug interfered with cell death in cells from Wolfram patients but did not harm cells that came from parents and siblings."

It is hoped that dantrolene may be effective against type 1 and type 2 diabetes. The research team is also planning to look at the effects of the drug on other tissues including the eye.

Source: Washington University School of Medicine

Image Credit: Urano Lab

Published on : Wed, 26 Nov 2014