

SickKids Corporate Ventures office licenses new compound that may treat lysosomal storage and neurological disorders

The Hospital for Sick Children (SickKids) has licensed its rights to a new compound to Neuraltus Pharmaceuticals in order to further research and development, and move the technology into clinical trials. The compound, which was developed in collaboration with Neuraltus, may be useful in treating certain lysosomal storage disorders and neurological disorders.

Neuraltus approached Dr. Clifford Lingwood, Senior Scientist in the Molecular Structure & Function program at the SickKids Research Institute when they noticed synergies between work they were doing and a publication he had authored. After a year and a half of collaboration between the two groups, enough data was generated to patent the pharmaceutical technology and focus on the development of a novel compound for the treatment of lysosomal storage disorders.

“In a healthy human, cell structures called lysosomes process and breakdown unwanted substances inside the cell,” said Lingwood. “Individuals suffering from a lysosomal storage disorder have a build up of the unwanted substances within the cells.” This can lead to any of approximately 40 known lysosomal storage disorders with a wide variety of symptoms, including developmental delays, muscular disorders, seizures, deafness and blindness, and can end in death. Neuraltus and Lingwood will continue to perform research in hopes of developing the compound as a treatment for a number of these disorders.

The compound, collaboratively developed by Lingwood and Neuraltus, affects lysosomal storage disorders by reducing the rate at which a molecule called a glycolipid is created within the body. Overproduction of glycolipids can interfere with how the cells grow and mature, how the cells adhere to each other and their ability to prevent tumours from forming. This can lead to serious disorders like Tay-Sachs, Gaucher's, and Fabry's diseases.

After an initial patent application had been filed, the compound was also found to have an additional affect on neurodegenerative disorders, and researchers at the Parkinson's Institute in Sunnyvale, CA joined the collaboration. Neurodegenerative disorders such as Parkinson's disease damage or destroy cells in the brain and spinal cord, and can impede movement and interfere with memory and brain function. A patent application has also been filed for this new use for the compound.

The license for SickKids's patent rights to the technology was developed and negotiated by SickKids' Corporate Ventures office and is effective as of February 1. SickKids will continue to collaborate with Neuraltus and the Parkinson's Institute to further develop the compound.