

Murdoch Childrens Research Institute enters licensing agreement with US company Jupiter Orphan Therapeutics

Murdoch Childrens Research Institute, Australia's largest child health research institute, today announced that they have entered into a global development and license agreement with US company, Jupiter Orphan Therapeutics, Inc. (JOT).

The agreement will see the parties jointly develop an appropriate delivery system and conduct clinical trials with the purpose of getting a product approved for the treatment of Friedreich's Ataxia (FA).

Friedreich's Ataxia is an inherited life-shortening disease that causes progressive damage to the nervous system resulting in those affected becoming increasingly unsteady until they require the use of a wheelchair. There are currently no proven treatments.

Murdoch Childrens' Professor Martin Delatycki is the head of the largest FA clinic in the southern hemisphere, and his intellectual input and design of clinical trials is central to the agreement.

The product, JOT101, will be a novel proprietary formulation developed by JOT utilising the active ingredient of resveratrol - a natural compound present in grapes and red wine.

Initial studies done by Murdoch Childrens show that this agent has great promise – it was recently found that resveratrol increases a protein called frataxin, which is deficient in FA in cells and mice. An open-label clinical trial in FA patients found that there was improvement of neurological symptoms but there were also adverse side effects that limited dosing, meaning a more appropriate formulation and delivery system must be devised.

“There are no treatments that can slow the inexorable progression of this devastating disease. If we can show that JOT101, an improved formulation of resveratrol, is safe, well-tolerated and slows the progression or even reverses some of the symptoms, this would be a significant advancement for people with Friedreich's ataxia” says Prof Delatycki.

“Our collaboration with Jupiter Orphan Therapeutics gives us the greatest chance of achieving this.”

“The Friedrich’s Ataxia Research Alliance (FARA) has supported much of the pre-clinical and clinical research that Prof Delatycki and his team at Murdoch Childrens has advanced on resveratrol. We are excited that Jupiter has a new formulation, JOT101, so that we can overcome some of the existing roadblocks and fully evaluate the potential efficacy of resveratrol”, says Jennifer Farmer, Executive Director of FARA.

Jupiter Orphan Therapeutics, Inc. was recently formed to address rare diseases caused by, or related to, single gene deficiencies.

JOT co-founders Christer Rosén, CEO, and Claes Wahlestedt, MD PhD, jointly stated: “We are very pleased and excited about working together with Prof Delatycki and the Murdoch Childrens’ team with their vast experience in this area. With the addition of JOT’s scientific, clinical, regulatory and formulation expertise we believe that we can, together with Murdoch Childrens, within a relatively short time frame identify a breakthrough treatment for this very serious disease.”

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