MEDIA RELEASE

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RARE DISEASE RESEARCH COLLABORATIVE AT GRIFFITH AND UQ TAKES FLIGHT AS INTERNATIONAL VISITORS ARRIVE TO TAKE PART IN AUSTRALIA

Internationally renowned researchers from the Griffith University and the University of Queensland have developed a collaborative research program to find a drug treatment for a currently incurable ultra-rare genetic disease SPG 56.

With research well underway, the success of this project is now attracting international patients, who are traveling to Australia to participate in the research.

Hereditary Spastic Paraplegia Type 56 (SPG56) is a degenerative brain disease that begins in childhood and continuously worsens throughout life. Every 3 weeks a child in Australia is born with an SPG mutation, of which there are over 90 types.

Research began in Australia when then 1 year old Tallulah Moon Whitrod was diagnosed with “currently incurable” SPG56. Determined to find a treatment to save their daughter, parents Chris and Golden Whitrod started the foundation Genetic Cures for Kids Inc to propel research forward, and their first mission is Our Moon’s Mission: to cure SPG56.

2017 Australian of the Year, Emeritus Professor Alan Mackay-Sim from the Griffith Institute of Drug Discovery (GRIDD), who recently discovered a drug that could potentially treat a different spastic paraplegia, SPG4, said he was optimistic about finding a drug to treat SPG56 as well.

“The Whitrods and Genetic Cures for Kids are funding a two-part collaboration with Professor Ernst Wolvetang at UQ, where we use Tallulah’s stem cells to help us with our research” Prof Mackay-Sim said.

“Now that other SPG56 patients are traveling to Australia to participate in medical procedures to harvest further samples, we will improve our chances of finding a drug because we’ll have multiple stem cell samples to study ‘in vitro’.”

“This is the successful approach we used to find a drug for Hereditary Spastic Paraplegia Type 4 (SPG4), which we are advancing to clinical trials in patients. If we can use this method to find a drug for SPG56, we could extend it to other kids around the world who've got a similar mutation. We will validate and extend our drug discovery using technologies with Professor Wolvetang.”

Professor Ernst Wolvetang from Australian Institute of Bioengineering and Nanotechnology (UQ), grows organoids known as “mini-brains” to study brain diseases like SPG56 and to test drugs to prove effectiveness. He said research progress was on target and the international samples would improve research outcomes.

"Thanks to Genetic Cures for Kids, blood samples from other children around the world living with SPG56 enables us to create patient specific brain organoids that will inform us how SPG56 mutations affect brain development and function," Prof Wolvetang said.

"This great progress means we will soon test a variety of therapeutic approaches on the international and Australian samples to look for a treatment for this 'currently incurable' disease, and puts us on track to meet the urgent need for drugs that could change the course of the illness of children with SPG56, rather than simply treat symptoms," he said.

Co-founder of Genetic Cures for Kids Inc, Golden Whitrod, said she was pleased the foundation was now helping international visitors to participate in the research through funding vital procedures that could only take place in Australia.

"We are so thrilled that other families can now get involved in the great research happening here in Australia, and that our foundation can give hope to families like ours, whether in Australia or overseas," Golden said.

"The long-term success of our research is reliant on great researchers like these, but to keep them working we rely on philanthropy: in kind contributions, partnerships and sustained funding opportunities," Golden said.

Prof. Mackay said vital research into rare diseases could still attract government attention as the project progresses and continues to succeed.

"Right now, philanthropic funds are essential to get this work going, but if it gets going well, it can lead to other competitive sources like government funds," Prof. Mackay-Sim said.

Our Moon's Mission is committed to finding a cure for SPG56 through dedicated, results-driven research.

You can watch the film that captures Our Moon's Mission and Genetic Cures for Kids story here.

To donate head to www.ourmoonsmission.org/donate/

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