MEDIA RELEASE

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‘OUR MOON’S MISSION’ RESEARCHERS LAUNCH COLLABORATIVE INITIATIVE THIS CHRISTMAS INTO RARE GENETIC DISEASE SPG56

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 that is attacking 2-year-old Australian girl, Tallulah Moon Whitrod.

In May 2020, sparkly eyed Tallulah Moon lost her ability to walk, talk or even hold up her head. She was later diagnosed with Hereditary Spastic Paraplegia Type 56 (SPG56), a rare degenerative brain disease that continues to worsen with time.

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.

This December, Tallulah’s parents Golden and Chris Whitrod launched the foundation *Genetic Cures for Kids Inc* to propel genetic research into SPG56 and have raised over $300 000 thanks to generous donors supporting their campaign called ‘Our Moon’s Mission’.

“When Chris and Golden first proposed looking for treatment for Tallulah, my first thought was: ‘It’s possible for us to do that’,” Prof. Mackay-Sim said.

“The Whitrods and Genetic Cures for Kids are funding a two part collaboration with Professor Ernst Wolvetang to use stem cells from Tallulah which we will grow at GRIDD to find out about their biology. We will then look for drugs held in our compound library the largest drug and compound library in the southern hemisphere.”

“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 Tallulah, we could extend it to other kids around the world who've got a similar mutation as she has. 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 expanding to include international samples.

“Thanks to the Whitrods and Genetic Cures for Kids and the funds that they have raised, we’ve been able to transport blood samples from other children around the world living with SPG56, and already reprogrammed the cells into induced pluripotent stem cells that

are now being used to create patient specific brain organoids that should inform us how SPG56 mutations affect brain development and function. We will then be testing a variety of therapeutic approaches on the international samples alongside Tallulah's over the coming year," Prof Wolvetang said.

"The creation of patient specific stem cell-derived brain organoids is progressing as projected, and we are 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.

Tallulah's mother and Co-founder of Genetic Cures for Kids Inc, Golden Whitrod, said the volunteer-led charity had a clear mission: to raise awareness and urgently needed funds to continue dedicated research projects like this and find a cure for SPG56.

"Unfortunately, big pharmaceutical companies aren't interested in funding research for rare diseases, and they also get very little public funding support," Golden said.

"The long-term success of our mission 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 Tallulah's story here.

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

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