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‘Radical shift required to save lives’

A normally developing two-year-old starts to have problems walking, within a year has lost the ability to speak or move, and is not expected to live past age five – these are the effects of an inherited nervous system disorder known as late infantile MLD.

Gene therapy provides the science and know-how to potentially change the course of this devastating disease, but according to GlaxoSmithKline’s Doctor Russell Jones, the scientific advancements are being held back by logistical challenges.

“In order to deliver the emerging medicine on a large scale a radical shift in business practices is required,” he said.

This is a challenging and complex endeavour.

Not only are there very few places in the world with the capability to make the medicine, but Dr Jones said there are further complications.

“The current shelf-life for the manufactured cells is only a few hours,” he said. “This effectively means that the manufacturing has to be done adjacent to the hospital.

“Also, it is critical the patient gets their own specific cells returned to them; hence the tracking and integrity of materials is critical.”

Despite the significant hurdles, Dr Jones’s team is making progress towards helping those living with the nervous system disorder.

“We are in the clinical phase of development and have one manufacturing location in Milan where the clinical studies are being conducted,” he said. “This is a new area of medicine and we hope to be pioneers in bringing this type of product to market and expanding the use more widely around the world.”

Dr Jones will share some of the learning from his experience and also seek new ideas as part of his attendance and presentation at the 10th Annual International Electromaterials Science Symposium, running from February 11 to 13 at the ARC Centre of Excellence for Electromaterials Science (ACES), University of Wollongong.

ACES Director Professor Gordon Wallace applauded GSK’s pioneering efforts.

“At the frontiers of science it is unlikely that traditional business models will enable effective translation,” he said.

“This recent development from GSK shows how agile we must remain on all fronts.

“It is critical that scientists at the cutting edge of science couple with those at the leading edge of business innovation to ensure maximum impact.”

About the International Electromaterials Science Symposium

This symposium brings together leading researchers engaged in ground-breaking materials science. Applications that will be presented include solar and hydro-energy generation, printing 3D structures, building ultra-strong electrolyte gels and muscle regeneration through electrical stimulation of cells.

About the ARC Centre of Excellence for Electromaterials Science (ACES)

The ARC Centre of Excellence for Electromaterials Science (ACES) is a global leader in advanced materials and integrated device development. Encompassing researchers, clinicians and industry partners worldwide, ACES is uniquely positioned to translate materials research into innovative next-generation solutions for clean energy and medical bionics.

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