Queensland scientists provide insights into incurable brain disease

22 March 2013

Queensland Institute of Medical Research, Griffith University and University of Queensland scientists researching a degenerative brain disease have developed a new way to understand its progression and safely test potential treatments.

The researchers have managed to generate Ataxia-telangiectasia (A-T) patients ' nasal stem cells in the laboratory.

UQ Centre for Clinical Research Professor Martin Lavin, head of QIMR ' s Radiation Biology and Oncology Laboratory, said the breakthrough meant scientists could study exactly what was happening in a patient ' s brain.

" And we think it means we can make a good contribution to coming up with a drug, to at least slow down the progress of the disease, " Professor Lavin said.

Ataxia-telangiectasia (A-T) is an incurable, degenerative brain disease that leads to severe disabilities, a weakened immune system, and an increased risk of cancer.

Children who have the rare genetic condition are usually wheelchair-bound by their teens and rarely live beyond early adulthood.

Professor Lavin ' s team collaborated with Professor Alan Mackay-Sim from the Eskitis Institute for Drug Discovery at Griffith University to generate the stem cells from the olfactory organ in the nose, which are capable of changing into a wide range of specialised cell types.

These multipotent cells are known as olfactory neurosphere-derived (ONS) cells.

The ONS cells help scientists better understand how the condition develops, and allows them to screen a patient for any new drugs which might slow down the progression of the disease.

" These cells from the nasal passage don ' t need to manipulated, or have viruses or genes added to them, they simply grow in the lab, " Professor Lavin said.

" And it provides us with very individualised information about a patient.

It means we can screen them for any potential treatments, because we have their exact cells which can be converted into brain or other cell types, and they provide a specific guide to how the disease has taken hold in their brain. "

The QIMR team have also developed a rat model for A-T, which mimics the disease ' s effects and are also in the process of making ONS cells from the mutant rats with the Griffith University team.

Future research will involve returning " corrected " ONS back into the rat model ' s brain, to see whether the disease progression can be slowed.

" If those experiments work, then there will be the potential to correct the defects in the patients ' stem cells, and transplant them back into a person ' s brain as neurons.

It ' s just one of the ways we ' re trying to tackle a disease which attacks on so many fronts. "

Brisbane has the only Ataxia-telangiectasia clinic in the southern hemisphere, lead by UQCCR researcher Dr Kate Sinclair and a team of clinicians from UQCCR and the Royal Children ' s Hospital who also contributed to this study.

The research abstract is available to download in the current issue of Human Molecular Genetics.

Professor Lavin ' s research is funded by BrAshA-T Ataxia-Telangiectasia Limited, an organisation set up by the Roebig family to improve treatment and management of A-T patients and foster research into the condition.

It is also supported by a kind donation from an anonymous donor.

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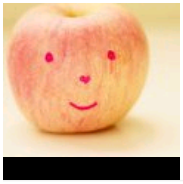
- 
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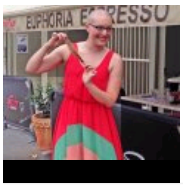
- 
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