



Q&A – Research into Huntington’s Disease

The information below is to help explain current research by Living Cell Technologies and the implications for people affected by Huntington’s Disease (HD). It is in response to the media announcement on preclinical results released August 2005.

What did the trial involve?

The trial uses living natural brain cells that produce a range of therapeutic proteins. The cells are coated in a gel produced from seaweed. The gel coating stops the cells from being rejected by the body’s immune system. The resulting product is called NtCell.

The gel biocapsules are loaded into a syringe and injected into the region of the brain affected by Huntington’s, known as the striatum. Primates were used in this study. Once the protection of NtCell was in place, Quinolinic acid was injected to mimic the effects of HD. The trial was a month long.

Where were the cells from?

The cells used were from specially-bred pigs in New Zealand. The pigs are quite unique and don’t have any viruses or diseases that affect the safety of the product. They are kept in very clean facilities, under strict regulatory and ethical guidelines.

What were the results?

The results show that primates treated with the biocapsules have few damaged cells, where as untreated primates had large lesions and damaged brains, as actually occurs in humans with HD. Results indicate that the injected cells produce a cocktail of therapeutic proteins (polypeptides), that act to protect and repair the brain from the damage caused by HD. These injected cells continue to produce the proteins for an extended period of time. In initial studies, cells appear to survive for up to 6 months, further studies defining the actual longevity of the cells are ongoing.

Where to from here?

These results are for late-stage pre-clinical studies. This is usually the last stage of development before starting human clinical studies. At present, the company is submitting an application to the relevant regulatory bodies for permission to conduct a clinical

trial. We anticipate that approval will be received in 2006 for this trial.

If human trials are approved, what will they involve?

At present, the trial would involve the injection of a small volume of the capsules into the brain of patients who already experience severe symptoms of the disease. This trial would initially treat approximately 5 patients to test for safety of the procedure.

What would the timeframe be?

It is hoped that if the initial human trial shows a positive result, the product will be fast-tracked to enable a larger patient trial much faster than is normal. This could result in an approval of a product within 2-3 years.

Where will the clinical trials be done?

The clinical studies will be done in the United States. The US has published clear guidelines covering the use of animal cells to treat humans (called xenotransplantation).

Will trials be done in Australia?

At present, the Australian regulatory body has a hold on any human trials involving animal cells (called xenotransplantation). The company believes that there is now sufficient information available to show that there is not an elevated risk in using these cells. A flyer on xenotransplantation with some of this information is available on the company website at: <http://www.lctglobal.com/resources.php>.

The company will continue to talk with the regulators to see if Australian human clinical trials will be possible in the future.

About the company:

The company is called ‘Living Cell Technologies’ and is based in Australia, New Zealand and the United States. The company uses healthy living cells to treat Huntington’s disease, stroke, diabetes and haemophilia.

Further information, research papers, fact sheets and company details are available on the website at www.lctglobal.com