



Can gene therapy treat CLN5 Batten disease?

Following her recent trip to New Zealand, Ana Assis, Research Assistant for this project, tells us about her experiences and how the work is progressing. The Principal Investigator for the project is Professor Jon Cooper.

Can you tell us what the overall aim of the project is?

CLN5 disease is a late-infantile variant of Batten disease, and this project was developed to test how effective gene therapy might be in a sheep model of the disease.

Gene therapy is based on the injection of a virus in the sheep brain, which replaces the faulty gene with its corresponding healthy copy in the sheep DNA. The cells can then produce the CLN5 protein that would otherwise be missing, which causes the symptoms associated with this disease.

What did you learn from your time at Lincoln University, New Zealand?

For the first part of my project, I travelled to New Zealand to spend two months collaborating with Professor David Palmer's lab. During my stay I witnessed the behavioural tests used to assess the CLN5 sheep's motor abilities, involving a maze made out of metal gates set up on a field on the University farm.

I learnt valuable techniques to help me analyse what has been going on in brains of the treated sheep that will enable me to test whether the therapy has been successful and/or if there may be any harmful long-term effects.

What is a typical day in the lab at Kings College for you?

I am now back in London, where I will complete the analysis of the results. I usually get to the office in the morning and after catching up on emails I get to the laboratory, where

I either stain tissue using histology techniques or I spend some time in the microscope room taking pictures of brain sections for analysis. There is a very good work environment at our Institute (the Maurice Wohl Clinical Neuroscience Institute) and people from different groups are always happy to help each other out, so I consider myself very lucky!

Always a challenging question, but when could we expect the first results from your analysis?

This will take some time – the rest of the one-year project – but by early next year I am optimistic that I will have some interesting results to share.

Outside of the lab, what do you like to do?

I enjoy going for the occasional jog in the park and expanding my cooking skills whilst bringing friends together around a table. Being Portuguese and living in London, I generally try to soak up any ray of sun that breaches the clouds!

And your plans for the future?

When this project is finished I would like to keep pursuing the topic of neurodegenerative diseases by starting a PhD on this topic. We'll see what the future brings!



Ana B. Assis

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