Canterbury Medical Research Foundation

Final report for Project Grant 01/2019

Grant recipient

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Grant details

GRANT TYPE	Project Grant	FUNDING ROUND	2019 Major Project Grant
GRANT REFERENCE	01/2019	GRANT AMOUNT	$100,000

Final report

1. Report for the Scientific Assessing Committee

View an attachment by double clicking the icon to the left of the file name. Icons are not displayed and attachments are not accessible when this PDF is viewed in a web browser; you must open it in PDF reader software.

CMRF 2019 Final report.pdf
660.6 KiB

2. Brief summary

Neuronal ceroid lipofuscinoses (NCL; Batten disease) are a group of inherited neurodegenerative diseases of childhood with a worldwide incidence of 1:12,500 live births. Affected children lose their vision, suffer seizures and experience physical and cognitive decline. There is currently no cure. Lincoln University has unique flocks of sheep with a naturally occurring CLN5 variant of Batten disease. Clinical and neuropathological disease progression is well defined in these sheep which have proven to be excellent models of the human condition. Corrective gene therapy we delivered to the brains of CLN5 affected sheep showed exciting results, protecting against stereotypical brain atrophy and clinical decline, as monitored by in vivo non-invasive methods and verified post mortem. Sheep that received brain-directed treatment still went blind, but findings from a subsequent pilot study of eye-directed gene therapy were encouraging. Gene therapy to one eye of affected sheep provided long term protection from loss of retinal cell activity compared to the untreated eye. In this project we administered combined brain- and eye-directed gene therapy in both pre- and post-symptomatic CLN5 sheep in an effort to prevent both neurological disease and retinal degeneration simultaneously. Sheep that received this combined treatment consistently scored high on the ovine Batten disease clinical rating scale, maintained stable brain volumes, and retained vision in their treated eye. We are now in the final stages of bringing this therapy for CLN5 Batten disease into human clinical trials.

3. Photographs

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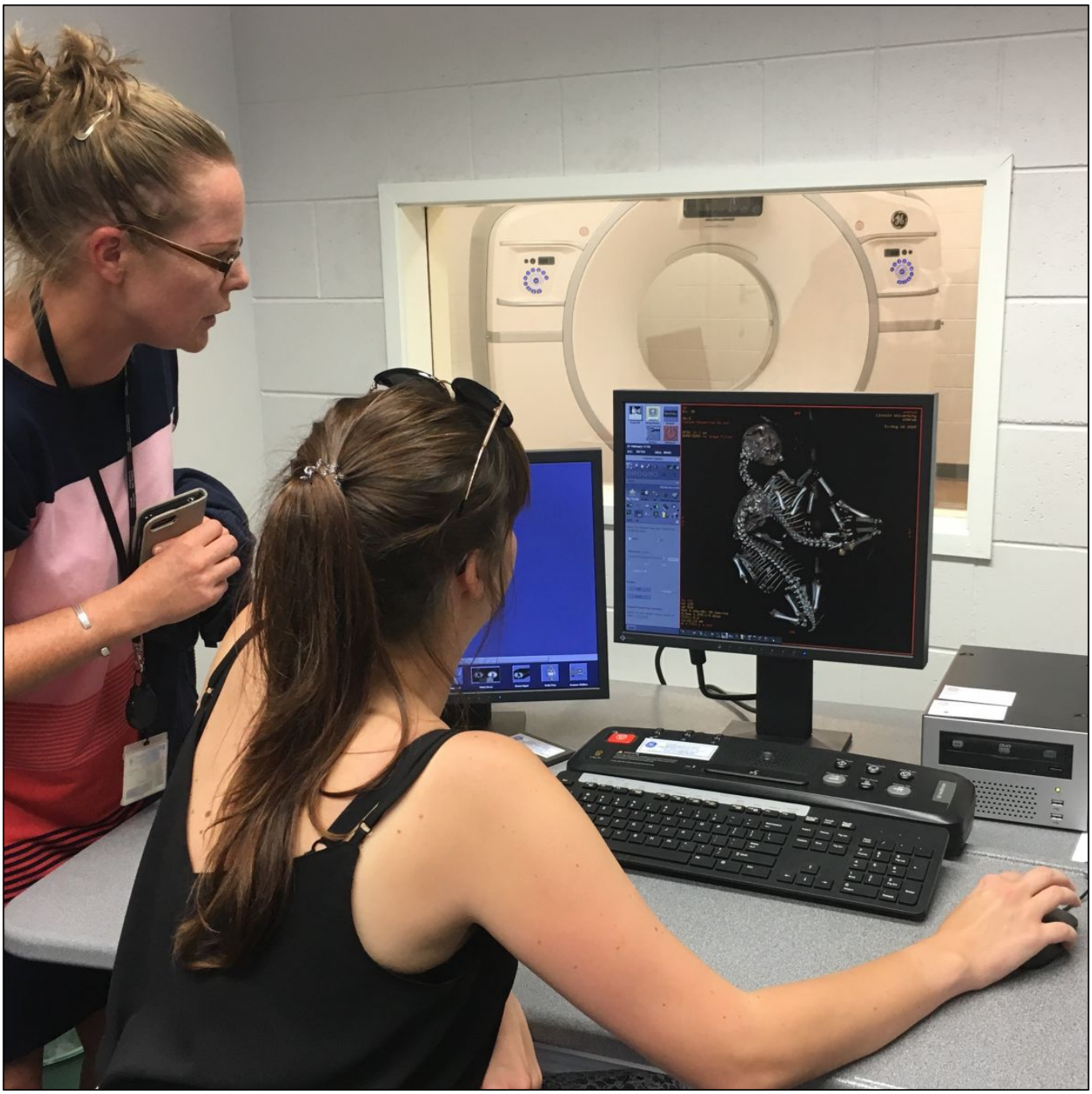
2019-09-23 20.42.19.jpg
136.3 KiB

Nadia and Sam-CT Scanner.JPG
1.3 MiB



Research team.jpg
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5. Feedback

Publication

Date

01/12/2020