

Progress report

Cystic Fibrosis Geelong Genetic Therapies Innovation Grant

Project: Harnessing the power of magnets: Improved effectiveness of airway gene therapy for the treatment of cystic fibrosis lung disease

Airway gene therapy involves delivering healthy copies of the CF gene to the airway cells to preserve lung health. Lung-directed gene therapy is showing promise in pre-clinical testing, but ineffective therapy delivery remains a challenging barrier to clinical translation. Our proposal will use magnets as a novel tool to guide and hold our gene therapy product in the lungs, as a way to increase gene uptake into the target airway cells.

So far the project is progressing well. In the last 6 months we have achieved the following:

- Animal ethics application written and recently approved by the University of Adelaide Animal Ethics Committee
- Cell culture studies have been performed to find the best conditions for magnet-guided gene therapy delivery - these parameters will be used to directly inform the animal studies that will be conducted in the latter part of this year
- We have submitted an experimental proposal to SPring-8 Synchrotron (Japan) to request synchrotron “beamtime” in November/December this year. We are awaiting approval and notification of our experimental dates.

In the next 6 months we plan to do the following:

- Travel to SPring-8 from July 15-18th to conduct magnetic particle visualisation experiments
 - On this trip we will examine the behaviour of magnetic particles in live rat airways with a focus on assessing the importance of adequate airway hydration as a method of improving the moveability of magnetic particles on the airway surface
- Pending approval, we will travel to SPring-8 in November/December for additional synchrotron experiments that will identify the best combination of airway hydration method and magnet setup
- A study will be conducted in rats to assess the effectiveness of gene therapy using our optimised magnetic guidance system



The CF airway research group (and collaborators) on their visit to Japan to conduct experiments at the SPring-8 synchrotron. From left: A/Prof Martin Donnelley, A/Prof Kaye Morgan, A/Prof David Parsons, Dr Ronan Smith and Dr Ali McCarron