June Newsletter 2018 **Charity Commission Number CC24962**

Access to Medicines in New Zealand - An Update

The past 12 months have been very full-on in this campaign, and there have been highs and lows too. A good deal of time in 2017 was spent with submissions to the Health select committee regarding Samantha Lenik's petition on funding of orphan drugs, and following this up to seek political commitments during the general election campaign. Positive policies from several parties drew great optimism when those parties formed a government late last year, but the resulting coalition agreement did not match the pre-election promises, and there was a decision that Pharmac would keep responsibility for orphan drug funding. This was a big blow to us, but a round of meetings earlier this year with select committee members and the Health Minister, left us optimistic that a solution to our problem of access to medicines, was still a live issue. We have been invited to work on a proposal with Ministry of Health officials, and we are pleased to note that the Minister is taking a close interest in this work.

Read More

Conference Planning is well on its way. We are very pleased to announce the

members of our Scientific Committee. Keep watching our website. We hope to have it live by the end of June.

Scientific committee and advisers

Chair. Professor David Palmer, Lincoln University, New Zealand. (Lysosomal structure and function, Batten disease, large animal models)

Deputy Chair. Dr Nadia Mitchell, Lincoln and Otago Universities, New Zealand. (Batten disease in sheep, gene therapy, longitudinal *in vivo* monitoring of therapy efficacy via neuroimaging, neurological and behavioral studies).

Members

- **Professor Hans Aerts**, Leiden, Netherlands
- **Dr Alessandra d'Azzo**, St. Jude Children's Research Hospital, Memphis, TN USA. (Lysosome/autophagy biogenesis; mechanisms of pathogenesis in animal models of glycoprotein and glycosphingolipid storage diseases; ERT and gene therapy for lysosomal diseases; lysosomes in AD, fibrosis and cancer)
- **Professor Brian Bigger**, University of Manchester, UK. (gene therapy in lysosomal storage diseases with a current emphasis on MPS II (Hunter) and IIIA, B and C (San Filippo))
- **Professor Jon Cooper,** Washington University Medical School, St Louis MO, USA. (Neuroanatomical and neurochemical studies of neurodegeneration in various lysosomal disorders via studies of animal models and therapies)
- **Professor Antony Fairbanks**, University of Canterbury, New Zealand (Enhancing carbohydrate binding tags on lysosomal proteins and the chemical biology of endo-beta-N-acetylglucosidaminidase (ENGase) enzymes to facilitate the synthesis of a range of defined glycopeptides and glycoproteins)
- **Dr Steven Gray,** University of Texas Southwestern Medical Center, TX, USA. (AAV gene therapy, bench-to-bedside translation, CLN1, CLN2, CLN5, CLN6, CLN7 and other lysosomal diseases)
- **Dr Ivanhoe Leung**, University of Auckland, New Zealand – (Biochemistry, enzyme replacement therapy, Batten disease).

- **Dr Kim Hemsley**, South Australia Health and Medical Research Institute, Adelaide, Australia
- **Dr Katsumi Higaki**, Research Center for Bioscience and Technology, Tottori University, Honshu, Japan. (Neiman-Pick C, Gauchers disease, chaperones, glycosidases)
- **Dr Jim McGill**, Lady Cilento Children's Hospital, Brisbane, Australia
- **Dr Andrew Munkacsi**, Victoria University of Wellington, New Zealand
- ***Professor Konrad Sandhoff**, Bonn, Germany. (Lysosome biology and biochemistry: Regulation of lysosomal processes by membrane lipids)*
- **Dr David Sleat**, Rutgers University, Piscataway, NJ, USA. (Lysosomal proteomics and enzyme replacement therapy)
- **Professor Steven Walkley**, Albert Einstein Medical Center, New York, USA (Pathobiology of neuronal dysfunction and therapy development for lysosomal diseases)

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