

Lysosomal Diseases New Zealand – Annual Report

This report covers the financial year July 2009 – June 2010, and includes some commentary up to the date of our meeting in February 2011.

Overview: 2010 was again a difficult year for fundraising. We have found LDNZ is having difficulty fitting into the new criteria set out by several of our traditional funders and we have lost several grants usually obtained in past years. However a surplus from the Adelaide conference did provide us with a good return and this helped save the situation for us.

Arrangements for the International MPS and related diseases symposium which was held in Adelaide were a major task during 2010. I want to commend the efforts of Jenny Noble who put in a very substantial effort to ensure the success of this meeting, and Wendy Boon of the Australian MPS Society who contributed significantly.

Throughout 2010 we continued to advocate for Access to Medicines for our diseases. This is proving to be quite a challenge for us, but in December 2010 two applications were submitted through Exceptional Circumstances for Pompe and Hunter syndrome. We had high hopes that we would see some progress through these two applications. At the time of our trustees annual meeting we were waiting anxiously for a response from Pharmac.

Finances: Raising funds via charitable grants has become very difficult for LDNZ. We have normally received grants from Lottery Grants board, J R McKenzie Trust, Todd Foundation, Pub Charity and other such groups. However most of these funders have less money to spend and some have changed their funding criteria, meaning we are receiving considerably less from these traditional sources. Without the wonderful fundraisers that we have held in the past we would be in a very difficult situation.

Income for the financial year ended 30th June 2010 was \$99,803 with expenditure of \$124,008 giving us a deficit of \$24,205. However we were able to maintain financial viability thanks to the Charity dinners and Charity golf functions that have been held over the last few years as this has left us with funds in the bank of \$89,658 at the end of June 2010. The surplus from the Adelaide conference came in after the end of the financial year and will help maintain our activities into the future, however even with those additional funds, the Trust has identified the need for fundraising a minimum of \$40,000 to keep us going for the next two years.

If you would like to have a closer look at our accounts please ask Jenny to send you a copy, or you can go to the Charities Commission website www.charities.govt.nz, search the register using the word Lysosomal, and this will bring up links to all recorded information about LDNZ, including links to audited accounts.

New Families: Over the last 12 month a total of 10 new families have been identified and although we would prefer to not to see new families diagnosed with these diseases we do welcome all them all to our Lysosomal community. The disease groups for new families are: Batten 2, Fabry 2, Gaucher 1, MLD 1, Pompe 1, Tay Sachs 1, Krabbe 1, Sanfilippo 1

Publicity Materials: We spent the last year giving LDNZ a fresh new look and would like to acknowledge the grant given to us by Genzyme to help make this all happen. We now have a beautiful new A4 flyer with new information that we hope will be very helpful for new families. We have taken this flyer to several professional meetings where many copies have been snapped up by those present.

10 year report: During the last 3 months of 2010 we started work on our 10 year report. It has been like a trip down memory lane, looking back at all the things we have done as an organisation over 10 years. We had hoped to have this project completed prior to Christmas 2010, but we have been waiting for government decisions on the funding of specialised medicines and the review on

Exceptional Circumstances which is being done by Pharmac. With the delay in both of these areas we have put the report on hold, as we are keen to include detail of progress on this issue in the report. It is, after all, the most pressing issue we face. We are hopeful that we will have this completed before Christmas 2011.

Advocacy within the health system: We have continued to keep the pressure on both Pharmac and the Minister of Health, in regards to the funding of Enzyme Replacement Therapies. The Minister did not make his expected decision on the funding of specialised medicines at the end of 2010, instead he asked Pharmac to review the EC application process as a possible way of improving access to these medicines.

We are very concerned about the consultation document put out by Pharmac and believe in its current form we will see great difficulty getting applications for ERT accepted. We have made a formal submission to Pharmac and joined with a number of other groups in making representations to Pharmac and to government about this issue. We expect this issue will take up a lot of our time in the coming year.

Our work with the National Health Board has progressed well and late in 2010 they confirmed that Genetic services and Metabolic services will be national services that are centrally planned and funded. Work is continuing to get these new services set up by 1 July 2011. LDNZ expects this will result over time in better clinical care services for families, faster diagnosis, and thus better outcomes for all of us. There will still be a need for advocacy for our families as we do not expect all the problems regarding referrals from District Health Boards, transition to adult services, and so on, to be resolved overnight. But the new services will have specific responsibilities that have not been clear in the past. We are optimistic that improvements will occur.

Natural History Study for Glycoprotein Storage Diseases. The Scholarship won by Jenny Noble from AMP in 2008 was used during 2009/10 to include 15 patients from New Zealand and Australia into a Natural History Study for Glycoprotein Storage diseases. This was an exciting opportunity for our families to be involved in this International Study and another good excuse to bring this group of families together again. We continue to support this project and will be working with Dr Cathey in 2012 as she extends the study to include the 9 diseases that ISMRD (The International for Glycoprotein Storage Diseases) supports.

Sanfilippo Research: In Jan 2010 Jenny and John were invited to Massey University to meet with Bob Jolly to see some of the results from his research project that LDNZ supported during 2009. Bob has been looking at how they can get Enzyme replacement therapy into the brain via intrathecal injection into the cerebral-spinal fluid, using the Huntaway dogs that naturally have this disease. He was able to show that they can get some enzyme into the brain, and was able to show that positioning of the dog during treatment was an important factor in the flow of enzyme into brain cavities. Bob presented his work in Adelaide at the International MPS family meeting. We will continue to follow his work as he moves onto considering Gene Therapy for Sanfilippo disease.

Trust Accountability: The trustees met in February 2011 for their Annual Meeting and to plan activities for the year ahead. Activities based on that plan have been carried out mainly by Jenny with regular reporting to the chair of the Trust, with additional issues being referred to the trustees by e-mail for decisions. This continues the pattern from past years where we meet face-to-face once a year for a full day of review and planning, and deal with all matters in between times by phone and email.

Keeping our books in order is very ably supported by Tim Hannagan through his accounting firm and this help is much appreciated, especially as the volume and complexity of transactions has grown in recent years.

My grateful thanks to trustees Dianne Webster, Dave Palmer, and Philip McKinstry for their support throughout this time, and in particular to Jenny for the significant commitment she makes to ensure the success of LDNZ's day-to-day operations.

John Forman
Chairperson
LNDZ