

LYSOSOMAL DISEASES NEW ZEALAND

April Newsletter 2006



2006 is turning out to be a very busy one for LDNZ. This month sees Gina Murray heading to Washington to attend her first ever Tay Sachs meeting, then in May Sharon Noble heads to Sweden for the Batten's Educational Conference and John and Judith Forman will attend the International MPS and Lysosomal Meeting in Venice on behalf of LDNZ. We know Gina and Sharon are going to come home with much information about their particular diseases and John will be able to bring us all up to speed with what else is happening in the World of Lysosomal Diseases. We look forward to reading their reports. Safe travelling everyone.

What's happening in 2006?

- International Tay Sachs Meeting
Washington - April
- Batten's Educational Conference –
Sweden May
- International MPS Meeting –
Venice June/July
- Australian MPS Meeting –
Brisbane Sept/Oct

Sadly we mourn the loss of

Daniel Kumeroa who passed away on the 22nd April. Daniel had Sanfilippo.



Our thoughts and prayers are with Gina Paul and the family at this very difficult time. Daniel will be sadly missed.

A NEW BABY FOR THE PEACOCKS



KIRSTY KENDALL AND JACK WELCOME WITH MUCH LOVE A NEW SON AND BROTHER

For all the girls who want to know all the little details here it is - Alex Sidney Peacock was born on 16th March at 6.50pm weighing 6lb 9oz.

We look forward to meeting this wee man in Palmerston North in May.

"It takes but a moment to change history. It takes love to change a lifetime."

Family Gathering

A series of meetings are being held around New Zealand through out 2006, with the first one held at the end of February in Tauranga. Families from the top of the North down to Rotorua were invited with 7 families gathering for a weekend of great food, good company and a small Lysosomal presentation given by John. Jenny spoke to the families about the fundraising activities taking place in Auckland and Rotorua in Support of Gina and Sharon who are the first two to head overseas to attend conferences.

We also spent some time working out a strategy to get access to the new enzyme replacement therapies that are now available. See below for more detail about this.



Hayden Sarah Ryan Neville

We had the pleasure of meeting Denise Laraman who volunteered to help raise much needed funds for Sharon to attend the Battens conference. Denise will be helping with the huge task of raising funds for LDNZ. She spoke briefly to the families about what she plans to do and both John and I look forward to working with her.

We can report that the comedy night held in Auckland recently raised a total of \$3,200 for Sharon. Mike King was the comedian, who very ably spoke about Battens Diseases and LDNZ. What an honour that have Mike do some research before commencing the show.

The next family gathering is to be held in **Palmerston North over the weekend of 27th – 28th** May Both John and Jenny look forward to catching up with families in the lower half of the North Island.

There are plans to hold two meetings in the South Island with dates still to be set.

Website

Have you checked out our website recently we have finally moved onto the new site sponsored by The New Zealand Organisation for rare Disorders (NZORD).

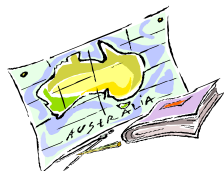
You will see that our format has changed a little. It now looks new and fresh. We would love to see more New Zealand stories on the web. Gina has promised to write her story for us – are there any other families who think they might like to do this. Our web address remains the same www.ldnz.org.nz



Thank you for your support

We are very grateful to the following people who made a donation to LDNZ during 2005.

Catherine Healy, K & C Mills, Tim and Marianne Hannagan, A.E. Dennard, Diane Robertson, Kathleen and Aubrey Walters, Cally and Kevin Martin.



Brisbane MP\$ Meeting

Strengthening Partnerships

This is to be the biggest New Zealand contingent going to Australia yet. We have 8 families who have indicated that they will be attending. Registration forms should have been received by now. If you have not received your conference pack can you please let Jenny know on 07 544-8868. **Could all the families who have indicated that they intend to attend this conference please confirm this with Jenny as we are trying to find some funding to help with the costs of travel.**

With so many Kiwi's attending the Brisbane conference lets have some fun—The Sports night dinner is a real opportunity for us to join forces and have some fun with the Australians. The theme is to dress in your team colours. We all know what our colours are. It would be wonderful to see us all wearing an **ALL BLACK** Shirt. *The challenge is set see you all in Brisbane*

Announcement on a Medicine Strategy for New Zealand is good news for LDNZ families, and for other rare diseases.



An announcement was made just before Easter by Associate Health Minister Peter Dunne of the timetable to develop a medicines strategy for New Zealand. This fulfils a promise in the post-election confidence and supply agreement between United Future and Labour.

We are very pleased to see specific mention in the announcement of a focus on "access to new/innovative/high-cost medicines (especially for niche groups e.g. rare diseases)". A lot of hard work over many years has paid off with the specific inclusion of rare diseases and high cost therapies in the review. The strategy is due for completion in early 2007, which should give us plenty of time for our messages to be clearly heard, yet avoid a long drawn out process.

The best news is that the process will not focus all its attention on Pharmac. While they are the ones who deliver the bad news to many of us, they have a limited budget and cannot approve drugs they don't have money for. The strategy development will look at the bigger picture of access to medicines, quality use of medicines and the rational use of medicines, and will keep away from an exclusive focus on budget management. With that risk avoided we can join in discussion on the strategy with reasonable confidence that all our issues will be thoroughly canvassed.



LDNZ worked closely with the NZ Organisation for Rare Disorders to push for this review, and joined with NZORD in the Access to Medicines Coalition, a collective of 25 support groups which are all seeking a better deal on medicine access. We will be actively involved in this issue over the coming year and will keep you all informed, and we'll welcome your thoughts and comments as we go through this process.

Trying to get a coherent response to new enzyme replacement therapies.

Many of you know that since the development of the first ERT for Gaucher disease in the 1990s there has been a lot of work on developing more therapies for other Lysosomal diseases. New ERTs for Lysosomal diseases that have been approved recently (in either the USA or Europe, or both) include: Aldurazyme for MPS1, Replagal and Fabrazyme for Fabry disease, Naglazyme for MPS6, and Myozyme for Pompe disease.

In addition there is continuing work on developing therapy for Hunter diseases with an expectation that therapy may be available next year, and several more of our diseases have ERT in pre-clinical development.

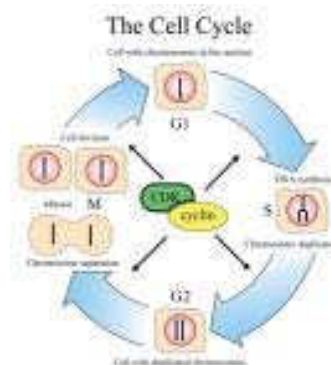
A big frustration in getting access to these therapies for NZ families has been the patchy and uncoordinated approach at several levels. Not all are registered in NZ by the companies, possibly because of small numbers and uncertain funding, leaving application to Pharmac for "exceptional circumstances" approval for individual patients as the main option. But that isolates it to a case by case system which is very difficult to organise any support group activity around, and easier still for Pharmac to reject on what seems at times like very spurious grounds.

"Compassionate use" supply is a system where the company gives the drug free of charge, and while that is great for the individual patients that may receive it this way, it undermines the likelihood of approval being given for access to the ERT for all those who need it, and is very unlikely to benefit more than one or two patients.

Making matters worse, from our perspective, is a variety of applications to Pharmac for different patients from different specialists, when it seems the professionals' approaches are not coordinated centrally. This seems sure to offer an easier option for Pharmac to decline the treatment, and this has consistently happened.

LDNZ has begun discussions with key players to try and see a coordinated approach to licensing through MedSafe, and subsidy applications to Pharmac, plus a coordination of professional efforts through the Metabolic Service. We want to see this occur in a way that keeps our organisation fully aware of steps taken so we can play our part in providing the advocacy and information from a patient and family perspective.

Let's hope that 2006 sees a much better organised approach to enzyme replacement therapies for our patients and quicker access for those who need them.



INOTECH starts Morquio Type A clinical program

Basel, Switzerland, 21 December 2005

Inotech Biotechnologies Ltd. has announced that Saint Louis University, Prof. Shunji Tomatsu, and Inotech are to collaborate and conduct the Morquio Type A clinical trial with Morquio families, Morquio Foundations, and MPS Societies.

The Project has been commenced with the production of enzymes for a pre-clinical study. They hope to start the first clinical trial in early 2007. For more information about this clinical trial please see www.inotech.ch

Feedback / Donation

We plan to try and get a news letter out to you at least 3 times a year. We would value your comments and suggestions for future newsletters.

Please send us your feed back, your request for further information or make a donation to LDNZ .

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Donations over \$5.00 made to Lysosomal Diseases New Zealand are Tax deductible.

What happens to the funds we raise?

- Funding of all administration expenses for our group.
- Supporting families wishing to attend Conferences.
- Advocating for families for disability support, health services and access to therapies.
- Lobbying the Ministry for improvements to diagnosis, screening and care.
- Keeping in touch with researchers and biotech companies on research progress.
- Supporting some research efforts here in New Zealand.
- Keeping you informed of progress with our mission.

FAMILY GATHERING TAURANGA FEBRUARY 2006

