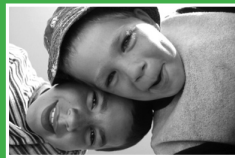


# The Linking Hand

Across Australia and New Zealand

May 2011



## Our Motto

A life is not measured by the number of days, months  
or years that it is lived,  
but by the effect that life has on other people who touch it.  
It is not measured by the length of its days or the height  
of its achievements,  
but by the breadth of its influence.

Anonymous

## Inside this issue

From the editor	1 page
Presidents report	3 page
Advocacy report	5 page
City to Surf 2010	7 page
Sydney Sausage Sizzle	8 page
Rare Disease Symposium Report	9-13 page
Society News	14 page
In the News	15-17 page

*I have a Disability  
yes thats true, but all  
that really means is  
I may have to take  
a slightly different  
path than you.*

**-Robert M. Hensel-**

# Mission Statement

To value, nurture and support those affected directly or indirectly by Mucopolysaccharide and related diseases

## Aims

To act as a support group for our members through the provision of local family support networks, the publication of quarterly newsletters, the holding of biennial National Conferences and by providing relevant information;

To promote a partnership between families and professionals, both locally and internationally; and to

Promote community awareness of MPS and its impact on families and carers.

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## From the Editor

Welcome to the May issue of the Linking Hand. Easter has already come and gone but not all the chocolate (in our house anyway). Hopefully you all had time to relax and enjoy it. I must say our family are all already looking forward to the next long weekend in June.

In this issue you find all the usual sections however we have made a slight change to the cover. Instead of categorising by season we are now going by month.

Barbara Rendell recently took time to attend the "Rare Diseases Symposium 2011 : Awakening Australia to Rare Diseases" that took place in W.A. from 17-20 April 2011. Barbara has contributed an extensive report for the Linking Hand that I think you will all enjoy reading.

An issue that has been widely publicised and will potentially be important to us all is The National Disability Insurance Scheme. This issue is in the news regularly so included in this issue is some general information on the scheme. Further reading can of course be undertaken at the suggested websites.

Once again I'd like to take the opportunity to encourage anyone interested in volunteering to please contact the Society and do so. At present the Society is functioning with only the assistance of a handful of volunteers. It would be great to have some new faces and some new hands to help out. I know it wasn't that long ago that the last conference was held in Adelaide but time slips away so quickly and if we want to have another conference in 2012 it is getting around to that time when things need to start being investigated etc. etc. Nearly all of what the Committee members do is done from

our own homes so please think about volunteering!

Unfortunately we haven't a Family Story for this issue. The Family Storys' are always a favourite with our readers so if any of you would like to contribute a story please feel free to write as little or as much as you'd like on your family experience. Photos are also a great addition! Please send your story or any other contributions to ;

[keryn@mpssociety.org.au](mailto:keryn@mpssociety.org.au)

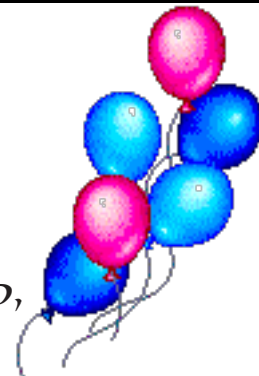
Thank you again to the Linking Hand regular contributors David Oliver for his President Report, Susan Connery for her Sydney Family Day (Sausage Sizzle) that always proves to be such a resounding success and Christina Oliver for once again reporting on the City to Surf experience.

Until Next time

*Keryn Rule*



# Birthday List



Birthday congratulations for those children and MPS Adults who celebrated their Birthdays in December 2010, January & February 2011.

## December 2010

John Wojnar	MPS IV Morquio	25 years
Bowen Rule	MPS II Hunter	12 years
Cooper Dare (Cornford)	MPS III Sanfillippo	6 years

## January 2011

Jenni Antoun	MPS III Sanfillippo	22 years
Rebecca Milne	Mannosidosis	40 years
Jospeh Bourke	MPS III Sanfillippo	32 years
Allison Dennis	ML Mucopolidosis	28 years
Caleb Faria	MPS II Hunter	2 years
Jenny Waite	MPS III Sanfillippo	2 years

## February 2011

Justin Hannan	MPS I	31 years
Jordan James	MPS III Sanfillippo	16 years
Tabitha Ellem	MPS I	9 years
Amelia Callow	MPS IV Morquio	8 years
Kaitie Francis	MPS I Hurler	6 years
Chelsy Jones	MPS IV Morquio	3 years

Dear Members,  
Please let David Oliver know if your child has not been included in the Birthday wishes list, or if there is an error in the above list.  
[president@mpssociety.org.au](mailto:president@mpssociety.org.au)  
Regards, David Oliver





## President's Report

Welcome to the Linking Hand for 2011. I hope that all our readers had a good break over the Christmas and New Year period, and 2011 is already shaping up to be a busy year for us all.

In the last six months we have been contacted by a number of newly diagnosed families who are contacting the Society looking for assistance in understanding the details and implications of their child's diagnosis. Almost every person who reads this newsletter will undoubtedly remember, in vivid detail, their own diagnosis, or that of a family member, and the emotions and reactions that they experienced at that time. I still consider that our response to these calls is one of the most critical roles the Society fulfils. We are the voice that can let the family know that in this time of their worst despair, that they are not alone, and that there are others who are walking this path with them. We work very hard to ensure that our new families receive the information they need at this critical time, and to help them to make contact, and receive support from other families in this time of need. I would like to thank all of those families who have responded to, and been able to help to support a new family.

While on the subject of contact with other families, I have just returned from another very successful Sydney Family Day hosted by Sue and Bruce Connery. These are always a good opportunity to catch up with friends and to meet with our new families, who may previously have only been a voice on a phone, or an email. While the weather was not kind, the rain could not dampen the bonds that exists between our families, and I would like to

thank Bruce & Sue for their hospitality for yet another great day.

The Society maintains a number of interactions outside of our immediate MPS sphere. We have been a long supporter of AGSA (Association of Genetic Support Australasia), the umbrella group for genetic disorders. We regularly attend meetings of support groups hosted by AGSA. These meetings are a forum for reviewing matters relevant to our needs, co-ordinates plans for actions where required, and maintain links with other support groups. Current activities with AGSA include participation in Rare Diseases Day, which in Australia has been supported by the Steve Waugh Foundation. Also we are participating in a campaign seeking to encourage the Federal Government to broaden the "A Better Start" program, in order to ensure that early intervention funding and services are made available to those affected by MPS and other genetic disorders. It is important that our Society's voice is heard outside of our immediate area, and particularly where government policy does not deliver the support that we need.

Also on the Society's agenda is the campaign for a National Disability Insurance Scheme (NDIS). This is an ongoing campaign which the Society, and many members have been actively engaged with in recent years. I would expect that most of our members would be aware of this campaign already. We are now in receipt of a Draft Report by the Productivity Commission, and following a review, will prepare a submission if this is required.

## *P*resident's Report *continued ...*

The Draft Report can be downloaded at <http://www.pc.gov.au/projects/inquiry/disability-support> and I would encourage everybody to obtain a copy. This is one area of government that we should be particularly interested in as it will directly affect how the essential services we need for our children and families are provided in the future.

Your Committee of Management has been working hard on a number of fronts, particularly as our numbers have been reduced through the retirement of Nicole Millis and Greg Moran. I would like to extend our congratulations to Nicole and her husband, David, who are expecting their second child and we wish them well. As a result we are looking for some new members to join the Committee of Management and participate in the running of the Society. While there are some particular roles we would like to fill, we also welcome those who would like to participate and contribute to the general running of the Society.

In particular we are seeking a Treasurer to take care of the finances of the Society, and we would also like to develop a role of following up contact from new families to ensure they are receiving the support they require. Other projects which we are looking to commence include the updating and reissue of our syndrome booklets, and the development of a better means of contact for our families in general.

I would like to thank all the Committee for their continued contributions and support, and look forward to another year of development and growth for the Society.

I hope that you enjoy this edition of the Linking Hand, and that I will have an opportunity to talk or meet with you soon.

Best Wishes,

*David Oliver*





# Advocacy, Research and Therapies Report

## MPS I - Aldurazyme

Aldurazyme is now available in Australia for patients who meet the government criteria for eligibility for treatment. These guidelines have recently been released and are available at <http://www.health.gov.au/internet/main/publishing.nsf/Content/health-pbs-general-supply-criteria>

Clinical trials are to be undertaken overseas (US & Finland) for intrathecal ERT of Aldurazyme as a means of possibly enabling treatment of central nervous system (CNS) disease arising from MPS I. The outcomes of the trials are initially to determine the safety of such a practice, and secondly, any improvement in spinal cord compression by the introduction of Aldurazyme. More details can be found at the following clinical trials website <http://www.clinicaltrials.gov/ct2/show/NCT00215527?term=MPS+I&rank=12>

## MPS II - Elaprase

Funding for Elaprase in Australia was announced in August 2008. Some patients have commenced treatment, while some are still undertaking baseline testing prior to commencement. Some patients that have applied have been rejected on the basis of CNS involvement which places them outside the approval guidelines. The Society is taking up this matter with the government and would like to hear from any patients or families who are in this situation. The government guidelines can be found at <http://www.health.gov.au/internet/main/publishing.nsf/Content/health-pbs-general-supply-othersupply>

A clinical trial is due to begin, June 2009, of Idursulfase (Intrathecal) Administration via an Intrathecal Drug Delivery Device in pediatric patients with Hunter Syndrome who have central nervous system involvement and are receiving treatment with Elparase. The estimated study completion date is December 2010. Further information can be found at the clinical trials website <http://clinicaltrials.gov>

## MPS III - updated 08/09/2010

Shire's Sanfilippo A syndrome program. Shire are conducting two concurrent studies. The first is a natural history surrogate endpoint trial, or SET as it is often referred to at Shire. SET is being conducted in the U.S. at the University of Minnesota. The second is a Phase I / II clinical trial to evaluate an investigational enzyme replacement therapy (ERT) administered directly into the cerebrospinal fluid. The Phase I / II clinical study is being conducted at two sites: Emma Children's Hospital, Academic Medical Center in The Netherlands by Dr. Frits Wijberg, and the Manchester St. Mary's Hospital in the UK by Drs. Simon Jones and Ed Wraith.

Additional information about both current studies, including eligibility criteria, is available at: [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT01155778 and NCT01047306).

An extension study listed on the Netherlands Trial Register to determine efficacy of genestein as a food supplement in patients with Sanfilippo Syndrome planned start date 12 Jul 2010.

Further information can be obtained from; <http://www.trialregister.nl/>

# Advocacy, Research and Therapies *continued ...*

## MPS IV

We have been advised that the clinical trials proposed by Vivendy have been abandoned, and that BioMarin are now the only company proceeding to Phase I trials for the treatment of MPS IVA. It is our understanding that these trials will be held in the UK and the US. The phase 1a study will provide patient data on the disease, including endurance and respiratory function, and the phase 1b study will evaluate safety and optimum dose of enzyme replacement therapy.

GALNS for MPS IVA: BioMarin initiated the Phase I/II trial in mid-April 2009 and completed enrollment in mid-July. The Phase I/II study is an open-label, within-patient dose escalation trial followed by a treatment continuation phase. The company expects to report initial results in the first half of 2010. Assuming positive results from the Phase I/II study, BioMarin expects to initiate a pivotal Phase III study in the second half of 2010.

## MPS VI - Naglazyme

Funding for Naglazyme commenced in November 2008. Patients should be enrolled or enrolling. Please contact the Society if you require any further information. No results have been published yet on a further trial of intra-articular enzyme injections into hip joints, although it appears the results are not conclusive.

## Mucopolipidosis (MLII and MLIII)

The 2007 Natural History study of MLII and MLIII patients provided evidence that the MLII and MLIII mutations were both occurring on the same Alpha Beta sub units (previously thought of as occurring separately). Further research is now experimenting with using a Aldurazyme (developed for MPS I), perhaps in combination with other drugs, to bring about changes in the cell lines. The outcome of some of this research was reviewed in a 2 day meeting prior to the 2008 Conference, and some agreement was reached on possible future directions.

## Fucosidosis

This branch of the MPS disorders is not well known to most members, with only a single case occurring in Australia recently. However, Prof John Hopwood in Adelaide has commenced a collaboration with Dr Sarah Cathey at the Greenwood Genetic Centre (South Carolina, USA) and Prof Michael Beck (Mainz, Germany) to enable a better understanding of the progression and effects of Fucosidosis. The study will include a gene mutation analysis, and measures of storage levels from skin and urine samples.





## City to Surf Report 2010

On a cool clear Sydney morning on 8 August 2010 80,000 Sydneysiders gathered for the annual City to Surf fun run.

Amongst the crowd were some dedicated MPS members and friends who ran one of the drink stations for the event. For the fourth year in a row, friends of the MPS Society assembled in town at 6am to fill hundreds of cups of water, and give directions to numerous lost runners all in the name of a good cause. The organisers of the event (Fairfax Media) make a donation to our charity based on the number of volunteers we provide, which this year amounted to a donation of over \$400 to the Society.

I would like to thank Fairfax Media for their continued support. I would also like to thank the Connery and Lawson families for their assistance and giving up their Sunday morning.

*Christina Oliver*



**L to R** – Bowen Oliver, Christina Oliver, Graeme Lawson, Georgia Lawson, Peter Oliver, Bruce Connery, Sue Connery, Laurelle Lawson

# Sydney Sausage Sizzle 2011

Sunday 27th March was our first Sausage Sizzle for 2011. There is no way I can say that the weather was kind to us this time! Rain drove us under cover several times during the afternoon. The best part was that the weather didn't deter people. We had 31 adults and children come and it was lovely to meet the Gauci family - Anne Marie, Craig, Sam [MPSIIIA] and Tommy.

Judging by the noise level there was no shortage of conversation and thanks to Stephanie Brown, all the children seemed to have a lot of fun.

Hopefully those who couldn't come this time will be able to join us at our next Sausage Sizzle later in the year.

*Susan Connery*



2 people missing from the photograph are Ben Betteridge and Lesley Waite.

**L-R Back row** ; Isabel Glasson, Bruce Connery, David Oliver, Justine Betteridge, Phillip Brown, Sandra Brown, Christina Oliver.

**Next row L-R** ; Craig Gauci, Aida Antoun, Ros McKay, Alanna Antoun, Deirdre Teague, Gared O'Neill, Susan Connery.

**Next Row L-R** ; Vanita Connery, Jordan Smith, Jenni Antoun, Tom Betteridge, Jessie Neil, Cindy Lorenz.

**Front Row L-R** ; Jacinta Selby, Charlotte Selby, Tommy Gauci, Maddie Selby, Hannah Selby, Amy Betteridge, Stephanie Brown, Sam Gauci and Anne Marie Gauci.



# Rare Diseases Symposium 2011

Hi everyone, my name is Barbara Rendell, I live in Oakford, just outside Perth, in WA. My son Joseph, who is 19 years old has MPSI. Recently David Oliver contacted the WA families regarding attending a conference that was going to be held in Fremantle WA. The conference was called the "Rare Diseases Symposium 2011 Awakening Australia to Rare Diseases" from 17-20 April 2011. The wonderful and always generous Genzyme and Murdoch University WA and the WA Government were the gold sponsors.

This was a first for us because we have always had to travel to the Eastern States for any conferences in the past.

The conference started at 9am and finished at 5pm on each of the three days so I wasn't sure if I could attend all the sessions because of Joe's schedule. Luckily he was owed some hours from his Alternative to Employment providers and I was able to increase his hours to allow me to attend for the entire time by having Joe picked up an hour earlier each day and dropped off to me each afternoon at the conference venue.

David Oliver had received word that the conference registration could be refunded to me by the organizers (The office of Population Health Genomics and the Australian Paediatric Surveillance Unit) so my only contribution would be time and a bit of petrol!

On the Sunday evening prior to the start of the conference we were invited to a reception to welcome the delegates and launch awareness of the Adopt a Politician scheme. The reception was at the Fremantle Esplanade Hotel. Unfortunately the location

within the hotel and the route to it was not exactly wheelchair friendly, through tiny doors and across very uneven cobbles etc, but the evening was well patronized and there was plenty to eat and drink on offer. There were a few pollios in attendance, each of whom had been "adopted" and the usual speeches about how important it is for policy makers to be aware of the difficulties facing families affected by rare diseases. I only hope the politicians remember these evenings when it really matters!

## **The Symposium Objectives:**

To develop a National Plan for Australia for Rare Diseases using the Europlan and other international models as a framework.

## **Main issues covered:**

- Development of a National Plan for a rare diseases
- Patient empowerment
- Patient care, support and management
- Research and translation into active therapies
- International and national networks
- Networks, partnerships and collaboration

Day one started with a welcome from Dr Noel Nannup, who is a Noonga Elder. He welcomed in the good spirits to watch over the delegates during the conference, as a traditional owner of the area of the conference venue.

The morning session was about developing the Rare diseases plan using the Europlan model as a starting point. Dr Domenica Taruscio from Italy spoke about her role in the formation of Europlan and about the Italian National Centre for Rare Diseases.

## Rare Diseases Symposium 2011 continued ...

The plenary session was followed by workshops divided into three sections for medical, patients/carers, and scientists. The workshops main area of discussion was why do we need a National plan, experiences within the health system and challenges for rare diseases within the system.

After lunch the plenary session continued with session 2: Patient Empowerment. Many people will be familiar with Mr John Forman who is the Executive Director of NZORD the New Zealand Organisation for Rare Disorders. John had been at all of the MPS conferences I have attended. John spoke of his desire for collaboration and networking of rare disease groups to give them more voice and reduce isolation. Mary Dunkle spoke next, she is Vice President of Communications for NORD (National Organization for Rare Disorders USA). NORD was established in 1983 (oops we are a bit behind there aren't we) and produced the enactment of the Orphan Drug Act 1983 which many of us are thankful for as it has helped fast track some of the Life Saving Drugs our children now receive. NORD has 150 disease specific groups under its heading, and works with the groups in the following ways:

- Support
- Advocacy
- Public policy
- Healthcare reform
- Medical foods
- Off label treatment
- Education for medical professions and public
- Assisting patients to receive treatment when funding is difficult
- Research by providing seed money grants
- Having medical advisors across the US

NORD are working towards joint with the organization EURODIS ([www.eurodis.org](http://www.eurodis.org)) and eventually worldwide collaboration.

EURODIS is the Voice of Rare Disease Patients in Europe but can be accessed by anyone on the above website and I recommend all parents to give it a try. It carries a wealth of information about your child's disease current research and treatment worldwide. It represents more than 469 rare diseases groups throughout 45 countries. It's also worth telling your local GP or specialist about the website so they can be better informed about what's happening out in the big wide world, as it is so hard for individuals to know what's happening everywhere else, and hard for GP's and even specialists to keep up with rapidly changing events when they may only see one or two of us during the year.

Dr Rhonda Galbally spoke about the National Disability Insurance Scheme ([www.everyaustraliancounts.com.au](http://www.everyaustraliancounts.com.au)). This scheme will provide a lifetime approach to the care of every person with a disability from whatever source. The scheme will be funded by a "levy" on the Australian taxpayers but will provide an equitable access to funding for care and treatment for every one that requires it.

Dr Segolene Ayme spoke about the website [www.orpha.net](http://www.orpha.net) or ORPHANET this provides a portal for rare diseases and orphan drugs. This is a very similar website to EURODIS but still worth a look. Dr Ayme invited Australia to join Orphanet.

Day one ended with a second workshop session entitled Patient Empowerment : what improvements can be made in Australia to aid patient / family empowerment and does



## Rare Diseases Symposium 2011 continued ...

Australia need a national umbrella organization.

### Day 2 Tuesday

Plenary session 3 was about Patient Care, support and management in Australia. A number of clinicians gave talks about patient care but the most moving story was from Maria Heaton a mum of two lovely children with a rare disease who gave her personal account of her trials and tribulations battling her way through the medical system over the years, the loss of her beautiful daughter and her continuing battle for care for her son, she described the negative attitude of some members of the medical profession which is something that we all probably have experienced and have in common despite our different diseases. Dr Jack Goldblatt, a geneticist from WA, spoke about the clinical management of rare disorders. Unfortunately I felt the problems appeared to revolve around the high cost of treating rare disorders and not around the need for the sufferers to receive it. This is so often the case, especially if the patient has intellectual issues, and "are not worth it".

Ms Susan Peden from the Disability Services Commission reminded us that Australia is a signatory to the United Nations Convention on the rights of persons with a disability. The first and foremost right is the RIGHT TO LIFE, sometimes I feel that the government of the day forget they sign up to these things! The commission is forming a 10 year National disability strategy for social, economic, and community inclusion. The six key areas of focus will be;

- Inclusive and accessible communities
- Rights protection and justice and legislation
- Economic security
- Personal and community support
- Learning and skills

- Health and wellbeing to ensure that people with a disability attain the highest possible health and wellbeing outcomes throughout their lives.

A WA GP, Dr Chris Fox, spoke of the important role GP's should play in the overall care. He said that 40% of patients are initially misdiagnosed as access to a one stop website and GP education are lacking. A GP can play a strong role in patient care by knowing the entire family situation and understanding the impact on all members and to take a "gatekeeper role" forwarding patients to specialist services and most importantly LISTENING! He also noted that it would be advantageous for there to be a good information flow between specialists and support providers.

Workshop 3 was mixed breakout groups to discuss Patient care and management in Australia : What improvements can be made and what should a National plan focus on in this area.

After lunch the Plenary session 4 was about research and translation of the research into treatment. We heard from a young man, Hayden Stevens who has Muscular Dystrophy. Hayden is 33 years old and has not let his disability beat him, he values his independence and is currently building a profile as a public speaker, telling of his battles with his disease and his love for his family. He believes that as individuals all rare disease sufferers have to fight each other for funding and treatment dollars, but if we band together we can fight together and carry much more weight!

Hayden thinks research concentrates too

# Rare Diseases Symposium 2011 *continued ...*

much on “a cure” for a disease but he says that he doesn’t want to be cured but he does want to live, and enjoy the most productive and comfortable life he can manage with the disability.

Dr Christopher Beroud is an assistant Professor of Molecular Genetics France. He told how the geographical spread of patients makes collaboration difficult and increases the need for a National and Global registry. The registry would require the use of multiple languages to ensure it’s easy access for all nations, and regular data collection and easy data access. The registry would evolve over time as new diseases and new therapies and discoveries come to light.

Dr Monique Ryan and Dr Yvonne Zurynski spoke about clinical trial and research in the Australian context.

Workshop 4 with mixed breakout groups discussed Research and Translation: What improvements can be made in Australia.

The Symposium Dinner was held on the Tuesday evening and gave people a good opportunity to network and socialize.

## **Day three Wednesday**

Plenary Session 5: networks, partnerships, collaborations.

Ms Lesley Murphy began the session, her son has muscular dystrophy, and gave her personal perspective of a person living with a rare disease. Lesley is the support manager of Muscular Dystrophy WA. Although her area of expertise is MD, her help and support is not limited by disease type. Her organization is available to support any person / family whatever their disease or need.

Next was Ms Sharon Terry, President and CEO of Genetic Alliance in the USA. Ms Terry serves on many Governmental advisory committees and is the forefront of consumer participation in genetics research services and policies, as she has two children affected by PXE. This has inspired her career path and she is an inspiration to all parents facing such difficult challenges.

Dr Kathryn North, University of Sydney officially launched the “collaborative approach to rare disease” which will hopefully kick start a National Plan.

Workshop session 5, breakout groups What improvements can be made in Australia to build better networks, partnerships and collaborations.

After lunch there was a further workshop session : Staying Awake to Rare Diseases: How to maintain the momentum for a National Plan on Rare Diseases. Followed by Plenary session 6 which basically was a summing up of all the topics discussed over the course of the conference, followed by an open forum discussion.

For me the conference was a good opportunity to meet new allies, make good contacts and catch up with some old friends. Many of you may know David Lewis and Charles Adderley formally of Genzyme Australia and now with their own consultancy company Cedar Glen. They were of great support to me when I was fighting to get Joe ERT, and it is always a pleasure to catch up with them, they are wonderful supporters of the MPS families.

Overall the conference was quite inspiring but I did feel that much of the speakers content was somewhat repetitive.



## Rare Diseases Symposium 2011 *continued ...*

It would be wonderful to have a national approach to the treatment of rare diseases, and one thing I did note is the inequality of funding and support from State to State. A lot of the talks were from people who's area of expertise appeared to be the Muscular Dystrophy group of diseases and I felt that other groups were very poorly represented. I think some of the blame for that has to lie with the organizers of the symposium as it was not advertised widely enough, and certainly I would have not known about it at all if it wasn't for David Oliver contacting me despite it being in my home State! Many of the delegates said they had the same experience, that they found out about the

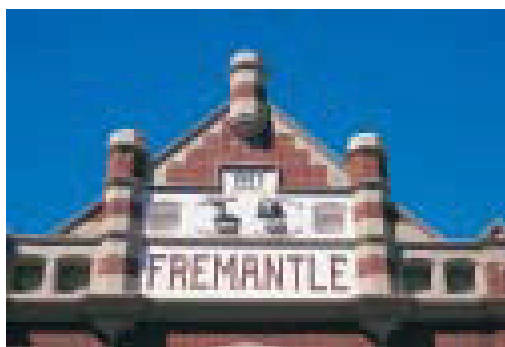
conference at the last minute through a "friend of a friend" kind of thing! It would have been nice to hear from a few more families with their personal experiences as I think often they hit home harder than professional talks.

I was glad of the opportunity to attend and thanks to David Oliver for alerting us.

*Barbara Rendell*  
Oakford WA

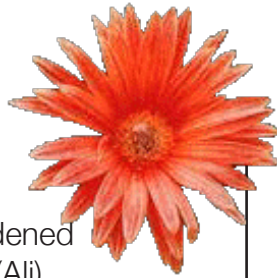


**2011**  
**Rare Diseases *symposium***  
**AWAKENING AUSTRALIA TO RARE DISEASES:**  
Global perspectives on establishing a coordinated approach to a national plan  
Esplanade Hotel, Fremantle Western Australia 17 - 20 April 2011



# Society News

## With Sympathy



The MPS Society was very saddened to hear of the passing of Alison (Ali) Kathleen King on Friday 1st April. She passed away quietly at the Tweed Heads Hospital after a short illness. Ali was 31 years old.

We extend our deepest condolences to all her family and friends.



## Better Start for Children with Disability (Better Start) initiative

From 1 July 2011, eligible children diagnosed with Down syndrome, cerebral palsy, Fragile X syndrome, or a moderate or greater vision or hearing impairment, including deafblindness, will have access to funding and new Medicare items under the Better Start for Children with Disability (Better Start) initiative.

The initiative includes the following components:

- Funding for early intervention services and treatments for eligible children under the age of six
- Medicare items for the development of a treatment and management plan for eligible children under the age of 13
- Medicare items for up to four allied health diagnostic services and for 20 relevant allied health services (in total) for eligible children up to the age of 15 (provided

the treatment and management plan is in place before the age of 13).

For further information about the Better Start initiative visit <http://www.fahcsia.gov.au/> or contact the Better Start Helpline on 1800 989 530. This helpline operates from 8:30am – 5:00pm Monday to Fridays (excluding Public Holidays).

Enquiries can also be emailed to; [Better.Start@fahcsia.gov.au](mailto:Better.Start@fahcsia.gov.au)

## National Disability Awards nominations open

Nominations are now open for the National Disability Awards 2011. These annual awards, now in their fifth year, celebrate and acknowledge the achievements and contributions that individuals or groups with disability make to our community.

The 2011 National Disability Award categories are aligned with the National Disability Strategy priority areas, a 10 year strategy to improve the lives of people with disability, their families and carers.

Nominations for the 2011 National Disability Awards opened on Wednesday 4 May 2011 and close on Thursday 30 June 2011.

To view nomination guidelines or to download a nomination form visit <http://www.idpwd.com.au/> or call 1800 440 385 to order hard copies.

The National Disability Awards are part of the Australian Government's celebration of International Day of People with Disability.



## In the News

# Genzyme Recognizes International Rare Disease Day by Launching New Patient Advocacy Grant Program

Genzyme Corporation recognized International Rare Disease Day with the launch of a new program, the Genzyme Patient Advocacy Leadership Awards (PAL Awards). A global grant program, the Genzyme PAL Awards will support non-profit organizations that work on behalf of patients living with lysosomal storage disorders (LSDs), a group of rare, inherited disorders that cause progressive and debilitating health problems. A total of \$50,000 will be awarded through a competitive application process to organizations that seek funding for new initiatives that support the LSD patient community.

“For 30 years, Genzyme has been supporting patients through pioneering new treatments, free drug programs and through ongoing partnerships with patient organizations that serve the LSD patient community,” said Genzyme’s Director of Patient Advocacy, Jamie Manganello. “International Rare Disease Day is a great moment to launch the PAL Awards to commemorate the progress that has been made in partnership with the patient community, and as a reminder of our commitment to the work that is still ahead for us on behalf of patients and families affected by rare diseases.”

Organizations may apply for a Genzyme PAL Award for programs that support disease awareness, advocacy on behalf of patient communities, patient education and patient care, support and communication networks. This grant program is supplemental to Genzyme’s existing grants program and will not replace the contributions made locally each year to support advocacy groups. Proposals will be reviewed by an external review committee. Applications must be received by June 15, 2011, and the award recipients will be announced by July 31, 2011. For more information on the Genzyme PAL Awards program, or to apply for a grant, please visit [www.genzymeadvocacyawards.com](http://www.genzymeadvocacyawards.com).

February 28, 2011 marks the fourth International Rare Disease Day, created by the patient organization EURORDIS and involving rare disease organizations globally. Genzyme is proud to have been a partner each year for International Rare Disease Day, and has organized events at Genzyme locations around this year’s theme, “Rare but Equal.” These events include presentations by patients with rare diseases at Genzyme offices, participation in a public event at the Massachusetts State House and today’s launch of the Genzyme PAL Awards.



## In the News

### A New System for Disability Care and Support

An entirely new model for providing supports and services for people with a disability is needed, according to a draft report released by the Productivity Commission. The draft report — Disability Care and Support — identifies the current disability support system as underfunded, unfair, fragmented, and inefficient. It gives people with a disability little choice and no certainty that they will get the support they need.

The Commission is proposing two schemes to address the flaws, with a rollout to commence in 2013-14. The biggest scheme, the National Disability Insurance Scheme, would be like Medicare in that all Australians would know that they or their families would get long-term care and support if they acquired a significant disability. A second much smaller scheme would cover people's lifetime care and support needs if they acquired a catastrophic injury from any accident. It would be based on widening and strengthening existing state and territory schemes.

Patricia Scott, the presiding commissioner for the inquiry, said 'Every day nearly 100 people acquire a significant disability. This will have life long impacts on them and their family. Under the proposed new schemes, people would not wait years for suitable wheelchairs or only get two showers a week. Our preliminary estimate is that the additional cost of the big scheme would be around \$6 billion per annum.'

The report says that reform is necessary and

the current system is not sustainable without significant additional resources. Associate Commissioner, John Walsh said 'We have a 'death spiral' in the current system, with ageing carers unable to cope, giving up their adult children to expensive taxpayer-funded care, leading to reduced respite support, and putting more strain on the remaining carers. Not providing adequate support now requires increased dollars later.'

The report says Australia should move to a system in which people with a disability and their carers have a lot more choice. They could decide what service providers to use and some could cash out their support packages to organise their supports much more flexibly.

The Commission proposes a new body — the National Disability Insurance Agency — to oversee the main scheme. The Australian and State and Territory Governments would appoint its board, but the agency would run the scheme independently, using clear criteria for entry to the scheme, tight controls to ensure that spending is based on reasonable need, and a focus on cost-effectively achieving much better economic and social outcomes for people.

Interested parties and individuals are encouraged to provide feedback on the Commission's draft proposals either by submission or attending its public hearings in April. The final report will be delivered to the Government in July 2011.

Further information on the draft report can be found at the following website; <http://www.pc.gov.au/projects/inquiry/disability-support/draft>





## In the News

# Ten Reasons why we need an National Disability Scheme (NDS)

1. The support system for people with a disability, their families and carers is in crisis. If you, or someone you love, is born with a disability or acquires one later in life, you all run the risk of falling through a huge hole in Australia's safety net.
2. People with a disability and their families and carers want to participate in the social, economic, and cultural life of the nation. But there are many barriers to their full inclusion.
3. Lack of support and services means families are primarily responsible for meeting the needs of their family member with a disability. Many families are struggling with high rates of physical, emotional and financial stress.
4. The current situation is inequitable – people receive different levels of support depending on how, when and where their disability was acquired.
5. An economic crisis is looming. The number of people with a disability is increasing and the number of people willing and able to provide unpaid care is falling. This means the cost to government is expected to escalate dramatically in the future.
6. A National Disability Insurance Scheme would provide people with a disability and their families and carers with the regular care, support, therapy and equipment they need. As a Medicare-type scheme, it would provide a secure and consistent pool of funds for these services and support.
7. It would be fair, efficient and effective. It would focus on early intervention and delivering those supports which produce the best long term outcomes. It would maximise opportunities for independence, participation and productivity.
8. It would be individualised and person-centred. Support would be based on the choices of person with a disability and their family.
9. The scheme would reframe support as investment rather than charity. Timely interventions, appropriate aids and equipment, training and development would become investment in individual capacity rather than welfare. The scheme would therefore lead to more positive results for people with a disability, their families and carers as well as being fiscally responsible.
10. All Australians would benefit from this scheme because disability can affect anyone, anytime. Everyone will benefit from building a more inclusive, more diverse community.

**Sourced** from Every Australian Counts:  
[http://everyaustraliancounts.com.au/about/ten\\_reasons\\_why\\_we\\_need\\_an\\_ndis/](http://everyaustraliancounts.com.au/about/ten_reasons_why_we_need_an_ndis/)





Make-A-Wish grants the cherished Wishes of children with life-threatening medical conditions. A Wish is filled with magic, hope and joy. The entire Wish process - from the planning, to the anticipation, to the Wish itself, and the memories that it creates - provides an exciting and welcome diversion from daily challenges, medical treatments and hospital admissions. A Wish is a positive experience for the entire family, brightening the day with laughter and smiles.

Wishes come in many shapes and sizes; they're as diverse as a child's imagination. The magic begins when a child utters the words "I Wish..."

I wish to BE"...a policeman or a ballerina for a day  
I wish to HAVE"... a laptop computer or dirt bike  
I wish to MEET"... a favourite rock star or celebrity

"I wish to GO"...on a holiday to a favourite destination (overseas Wishes are available to children 10 and over)

Children and teenagers aged from three up to 18 years with a life-threatening medical condition are able to apply for a wish. It is a fun time in which the whole family can take part. Children under the age of three may apply for a 'Wish Hamper' package which is a selection of wonderful age appropriate toys.

To find out more about Make-A-Wish visit [www.makeawish.org.au](http://www.makeawish.org.au) or telephone 1800 032 260.