MEDIA RELEASE

Children given lifeline

Australian Pompe's Association welcomes approval of treatment for young Australians with life threatening rare disease

17th February 2015: The Australian Pompe's Association (APA) welcomes the news that the Minister for Health Sussan Ley has approved treatment for young Australians with Pompe disease. As of the 1st February 2015, the government will list the only registered treatment for Pompe disease, Myozyme (alglucosidase alfa), on the Life Saving Drugs Program (LSDP) for patients aged 2-18 years.

Christian Rivera was the first to benefit from the decision, receiving an infusion on Thursday 12 February. Christian was diagnosed with Pompe disease when he was just 11 years old, and his family have waited ever since for news that Christian would have access to government subsidised treatment, and they were elated when the day of his first infusion finally arrived.

"Not knowing if this day would ever come has been a nightmare," explains Christian's mother, Rina Sosa. "But we are so happy that it finally is. Christian having guaranteed access to treatment for the rest of his life is something we will always be grateful for. I just feel so sorry for the other patients over 18 who can't get treatment."

In addition to Christian, two young siblings in New South Wales will also have access to treatment via the LSDP.

Pompe disease is a rare, inherited and debilitating disease caused by a mutation in the gene that produces the enzyme responsible for breaking down glycogen in the body.

If not treated, Pompe disease breaks down muscles, causes irreversible damage, puts patients in wheelchairs, and leads to respiratory failure and eventually death. Myozyme replaces this missing enzyme, and its listing on the LSDP recognises the role it plays in keeping patients well for longer.

While the announcement has been a long time coming, the APA commends the government on its decision to make this life saving treatment available to these three children in need.

"Pompe disease has a devastating impact on our bodies and our lives," said Raymond Saich, who is living with Pompe disease and is President of the APA.

"Pompe patients across Australia were delighted to hear that three of the youngest in our ranks, and their families, have been able to start 2015 knowing that their lives will be different."

"This is the first good news we have had since treatment was approved in 2010 for infants aged under two years. Australian and New Zealand are the last developed countries to approve treatment for Pompe disease. The APA would like to thank the Minister for approving treatment for Juvenile Pompe patients and taking this important step forward in approval for all Pompe patients in Australia".

Thirty four Australians are known to be living with Pompe disease. Currently, funding for treatment under the LSDP is only provided to those diagnosed with Pompe disease before the age of 18, which covers seven young Australians. Another 19 people are currently on a compassionate access program, operated by the maker of Myozyme, yet this compassionate access program has been closed to new patients, and those on it don't know how long their lifeline will be in place.

Six Australians are still without any access to any treatment.

- **ENDS** -

Further information, please contact:

- Raymond Saich, President, Australian Pompe's Association - (02) 9629 2842
- Catherine Jenner, Executive Committee, Australian Pompe's Association – 0413 662 015

About Pompe Disease

Pompe disease is a rare, inherited and often fatal disorder caused by the mutation in the gene that produces the enzyme responsible for breaking down glycogen in the body. There are two types of Pompe disease: infantile onset leads to cardiac arrest and/or respiratory failure before patients reach one year of age, and in late onset, Pompe disease patients will experience less rapid but relentless progression of the disease. It is extremely rare and there are only 33 Australians known to be living with the disease.

About the Australian Pompe's Association (APA)

The APA is a support group for patients who have been diagnosed with Pompe disease, their families and friends. The APA is made up of people living with the disease, working together to advocate and raise awareness about Pompe disease to the broader community.