**MEDIA RELEASE**

# Government approves lifesaving treatment

## Australian Pompe's Association celebrates the approval of treatment for all Australians with Pompe disease

**27 July 2015:** The Australian Pompe's Association (APA) welcomes the news that the Minister for Health Sussan Ley has approved treatment for Australians with ultra-rare Pompe disease.

As of the 1st September 2015, the listing of Myozyme (alglucosidase alfa) on the Life Saving Drugs Program will be extended to adults impacted by debilitating Pompe disease. Until now, only children have been given subsidised access to the high cost therapy.

"Today is a day of celebration. We are incredibly grateful to the Minister for Health and her Department in making the life changing decision to make treatment available for this devastating disease," said Raymond Saich, President, Australian Pompe's Association.

"While there are only a small number of Australians living with Pompe disease they are mothers, fathers, sisters, brothers, sons and daughters. Pompe disease causes people's muscles to break down, robbing them of their mobility and will eventually lead to respiratory failure. Treatment can halt the progression of the disease – allowing us to live our lives to the full as active members of the community."

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. Myozyme replaces this missing enzyme, and its listing on the LSDP recognises the role it plays in keeping patients well for longer.

Just 36 Australians are currently known to have the condition with patients living in every state and territory in Australia. Eight are children and 28 are adults. Some adults have been relying on a charitable access program that was established in 2006 and closed to new patients in 2012.

There are eight adults who currently have no treatment at all. The listing from 1 September brings them enormous hope for the future.

The APA commends the government on its decision to make this life saving, lifelong treatment available to all Australians.

**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 36 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.