



THE HON SUSSAN LEY MP

Minister for Health
Minister for Sport

MEDIA RELEASE

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Life-saving drug for young Pompe patients

Minister for Health Sussan Ley has approved a life-saving drug that will ensure young Australian Pompe patients receive treatment they previously may not have been able to access.

Ms Ley said she had approved the drug Myozyme® for Government subsidy for children aged two to 18 through the Life Saving Drugs Programme (LSDP).

“Pompe disease is a rare and severe medical condition, affecting only a very small number of Australians every year,” Ms Ley said.

“However, it is a very expensive treatment – costing several hundred thousand dollars – putting it out of reach for many Australians.

“This decision will change the lives of children with late-onset Pompe disease.

“This is only the first step. The Government is committed to continuing to work with the company towards a future application and subsidy for Myozyme® on the PBS for adult late onset Pompe patients.”

The drug has been listed on the LSDP for children with the infantile-onset form of the disease since 2010.

Patients are required to undergo clinical assessment prior to accessing and then an annual assessment throughout their ongoing treatment to ensure the Myozyme® treatment is clinically appropriate and effective.

If patients respond to treatment they continue to receive the subsidised drug, not just in childhood but throughout their adult life.

This approval is the result of considerable effort on behalf of the Australian Pompe Association, the sponsoring company Genzyme and officers of the Health Department to bring together the necessary evidence to support this listing.

The treatment is available now following Ms Ley's approval of the new indication of use for the medicine.

Further details are accessible through the PBS website at www.pbs.gov.au

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