

Patient Initial: .....

ID Number: F-.....

## **Lifesaving Drugs Program**

# **Application Form**

## **For**

## **Subsidised Enzyme Replacement**

## **Therapy for** **Fabry Disease**



**Australian Government**

**Department of Health and Ageing**

Patient Initial: .....

ID Number: F-.....

**PATIENT'S HISTORY WITH REGARD TO THE PROGRESSION OF FABRY DISEASE TO THIS INQUIRY FOR ENZYME REPLACEMENT THERAPY**

Was the Original Diagnosis of Fabry Disease made by you ?                      Yes/No

If No – How did you become involved in this patient's treatment?

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If Yes – On what date did you first see this patient for treatment of Fabry Disease?

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What factors in the progression of this patient's disease made you decide that enzyme replacement therapy is possibly now required?

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If any aspect of the disease – eg pain - has become worse, please indicate where this occurs in the body, the severity and the time span over which the deterioration has occurred:

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OR

I do not consider that this patient is severe enough to need enzyme replacement treatment at this time, but I would like to have the patient assessed and registered for possible treatment in the future.

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**APPLICATION FORM FOR SUBSIDISED ENZYME  
REPLACEMENT THERAPY FOR  
FABRY DISEASE**

**Prescribing Doctor**

Surname: \_\_\_\_\_

Given name: \_\_\_\_\_

Prescriber number: \_\_\_\_\_

Postal address: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

Phone: (\_\_\_\_) \_\_\_\_\_ Fax: (\_\_\_\_) \_\_\_\_\_

Email: \_\_\_\_\_

**Patient Details**

Surname: \_\_\_\_\_

Given Name: \_\_\_\_\_

Medicare number: \_\_\_\_\_

DOB: \_\_\_\_\_ Sex: \_\_\_\_\_

Address \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

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Has the patient received enzyme replacement before? **YES / NO**

**If yes** Date treatment commenced: \_\_\_\_\_

Brand of product: \_\_\_\_\_

Dose: \_\_\_\_\_mg/kg Fortnightly dose: \_\_\_\_\_

Is the patient currently registered with a Fabry registry? **YES /NO**

**If Yes**, which one? Fabry registry (Genzyme)

FIRE (TKT)

**Place of Administration of Algalasidase if Approved**

Designated Centre \_\_\_\_\_

Infusion site: \_\_\_\_\_

Pharmacist: \_\_\_\_\_

Address \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

Phone: (\_\_\_\_)\_\_\_\_\_ Fax: (\_\_\_\_)\_\_\_\_\_

Brand of Product requested: \_\_\_\_\_

I agree to provide full patient details in accordance with the Guidelines for Eligibility to receive Algalasidase through the Lifesaving Drugs Program.

Prescriber's signature: \_\_\_\_\_

Date: \_\_\_\_\_

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### DIAGNOSTIC DATA

Test	Result	Date	Laboratory Name
Leukocyte Alpha-GAL activity (nmol/hr/mg protein)			
Plasma alpha-Gal activity (nmol/hr/mg protein)			
Genotype			
Blood type			

### LABORATORY DATA

	Special Investigations:	Initial Data (If available)		Current Data (Complete all sections)	
		Date d/m/y	Result	Date d/m/y	Result
<b>Haematology</b>	Haemoglobin		G/L		G/L
	Platelets		10 <sup>9</sup> /L		10 <sup>9</sup> /L
	White Cell Count		10 <sup>9</sup> /L		10 <sup>9</sup> /L
<b>Biochemistry</b>	Total bilirubin		µmol/L		µmol/L
	Alkaline Phosphatase		u/L		u/L
	GGT		u/L		u/L
	ALT		u/L		u/L
	Cholesterol		mmol/L		mmol/L
	Triglyceride		mmol/L		mmol/L
	Plasma Urea		mmol/L		mmol/L
	Plasma Creatinine		mmol/L		mmol/L
	Albumin excretion rate		G/L		G/L
	Urine Alb:Cr ratio				
	Protein:Cr ratio				
	Proteinuria		mg/24hr		mg/24hr
	GFR		mL/min/1.73m <sup>2</sup>		mL/min/1.73m <sup>2</sup>
<b>Others</b>	Plasma GL-3 Antibody testing				

Height: \_\_\_\_\_ (cm)

Weight: \_\_\_\_\_ (kg)

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Calculated weight for BMI of 27: \_\_\_\_\_ (kg)

Systolic: \_\_\_\_\_ Diastolic: \_\_\_\_\_

**CARDIOVASCULAR**

Cardiologist name: \_\_\_\_\_

**Hypertension:** Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

**Echo:**

Date: \_\_\_\_\_ Ventricular wall thickness: \_\_\_\_\_ mm

Other: \_\_\_\_\_

\_\_\_\_\_  
\_\_\_\_\_

**ECG:**

Date: \_\_\_\_\_ Normal: \_\_\_\_\_ Abnormal: \_\_\_\_\_

PR interval: \_\_\_\_\_ mm

PQ interval (if available): \_\_\_\_\_

Other arrhythmia: \_\_\_\_\_

\_\_\_\_\_  
\_\_\_\_\_

**Stress test:**

Date: \_\_\_\_\_ Normal: \_\_\_\_\_ Abnormal: \_\_\_\_\_

Findings: \_\_\_\_\_

\_\_\_\_\_  
\_\_\_\_\_

**Cardiac MRI:** Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

Findings: \_\_\_\_\_

\_\_\_\_\_  
\_\_\_\_\_

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**Cardiac Biopsy:** Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

Findings: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

**Electrophysiological Study:** Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

Findings: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

**History of acute myocardial infarction:**

Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

Investigations / Interventions: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

**Other cardiac event?:** Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

Findings: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

**CEREBROVASCULAR**

MRI date: \_\_\_\_\_ Location: \_\_\_\_\_

Findings: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

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## NEUROLOGY

Clinical examination: Sensory (Please tick)

Sensation	Normal	Abnormal	Sensory level (if abnormal)
Light touch			
Temperature			
Vibration			
Joint position sense			
Sweating			
Heat/cold tolerance			
Pain sensation			

Other sensory abnormalities: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

Motor examination/coordination

Test	Normal	Abnormal
Heel/toe:		
Romberg:		

Other gait/motor abnormalities: \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

**Has this patient had a CVA :** Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

**If yes, Measure of disability:** \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

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**PAIN :** Yes: \_\_\_\_\_ No: \_\_\_\_\_

**If yes,** Chronic: \_\_\_\_\_ Episodic: \_\_\_\_\_

Pain score BPI-9: \_\_\_\_\_

**VERTIGO :** Yes: \_\_\_\_\_ No: \_\_\_\_\_

**If yes,** Chronic: \_\_\_\_\_ Episodic: \_\_\_\_\_

**RENAL**

Dialysis : Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

Renal transplant : Yes: \_\_\_\_\_ No: \_\_\_\_\_ Date: \_\_\_\_\_

**OTHER**

**LUNG FUNCTION TESTS**

		Date
FEV1		
FVC		
VC		
FEF 50		
FEF 25-75		

**HEARING TEST:**

Date: \_\_\_\_\_ Normal: \_\_\_\_\_ Abnormal: \_\_\_\_\_

Abnormalities : Left ear: \_\_\_\_\_

Right ear: \_\_\_\_\_

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QOL SCORE SF-36: \_\_\_\_\_

Date: \_\_\_\_\_

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**OPHTHALMIC EVALUATION:**

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**GASTROINTESTINAL**

Abdominal pain:                      Yes: \_\_\_\_\_ No: \_\_\_\_\_

Diarrhoea:                              Yes: \_\_\_\_\_ No: \_\_\_\_\_

Details (include frequency): \_\_\_\_\_

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