ELIGIBILITY CRITERIA FOR TALIGLUCERASE ALFA (ELELYSO)

Contents

SA1880 Initial application taliglucerase alfa (Elelyso)

Please send applications to:	
Facsimile	(04) 460 4995
Further Contact Details:	
Address	Gaucher Panel Co-ordinator PHARMAC P O Box 10-254 WELLINGTON
Phone	(04) 460 4990
Email	gaucherpanel@pharmac.govt.nz

Patient Details			
Last Name			
First Name/s			
NHI No			
Date of birth			
Gender	Male: ☐	Female: ☐	
DHB of domicile			

Applying Practitioner			
Speciality		NZMC Registration Number:	
Last Name			
First Name			
Contact address			
Phone			
Fax			
Email Address			
Cell phone			

Patient physical examination
Date of examination
Weight (kg)
Height (cm) (if child, please attach height chart)

Access criteria

Must be ALL of the following (1- 4)

☐	1	The patient has a diagnosis of symptomatic type 1 or type 3 Gaucher disease confirmed by the demonstration of specific deficiency of glucocerebrosidase in leukocytes or cultured skin fibroblasts, and genotypic analysis; and
☐	2	The patient does not have another life-threatening or severe disease where the prognosis is unlikely to be influenced by taliglucerase alfa or might be reasonably expected to compromise a response to therapy with taliglucerase alfa; and
☐	3	Taliglucerase alfa is to be administered at a dose no greater than 30 unit/kg every other week rounded to the nearest whole vial (200 units), unless otherwise agreed by PHARMAC; and
☐	4	Supporting clinical information including test reports, MRI whole body STIR, haematological data, and other relevant investigations, are submitted to the Gaucher Panel for assessment; and

Have you included the following? Please indicate attachments.

☐	*test reports*
☐	*MRI whole body STIR*
☐	*haematological data*
☐	*other relevant investigations. Specify:*

Plus ANY of the following (5.1- 5.5)

☐	5.1	Patient has haematological complications such as haemoglobin less than 95 g/l, symptomatic anaemia, thrombocytopenia; at least two episodes of severely symptomatic splenic infarcts confirmed with imagery; or massive symptomatic splenomegaly; OR

Date of blood test	*Hb*	*Platelets*	*AST*	*ALP*	*INR*	*APPT*	*Acid Phos.*

Date	*Spleen size Clinical: cm below costal margin*		*Radiology: spleen volume (specify type of imaging used)*	

OR

☐	5.2	Patient has skeletal complications such as acute bone crisis requiring hospitalisation or major pain management strategies; radiological MRI evidence of incipient destruction of any major joint (e.g. hips or shoulder); spontaneous fractures or vertebral collapse; chronic bone pain not controlled by other pharmaceuticals; OR

Please send imaging, including reports, of hips, femur, lumbo-sacral spine and other bones clinically affected, as per MRI guidelines

OR

☐	5.3	Patient has significant liver dysfunction or hepatomegaly attributable to Gaucher disease; OR

Date	*Liver size* *Clinical: cm below costal margin*	*Radiology: liver volume* *(specify type of imaging used)*

OR

☐	5.4	Patient has reduced vital capacity from clinically significant or progressive pulmonary disease due to Gaucher disease; OR

Date	*Pulse rate / min*	*Blood pressure (mm/Hg)*	*Echo (RV Pressure)*	*Lung function DLCO. Radiology*

OR

☐	5.5	Patient is a child and has experienced growth failure with significant decrease in percentile linear growth over a 6-12 month period

Other comments (include any neurological symptoms) (attach additional information if more space is needed):

Applicant's Declaration

I confirm that the above and attached details are correct and that in signing this form I understand that I may be audited. I recognise the requirements for monitoring and managing treatment with taliglucerase alfa.

Complete reports are attached, including test reports, MRI whole body STIR, haematological data and other relevant investigations.

Signature: Date:

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SA1880 Renewal application taliglucerase alfa (Elelyso)

Please send applications to:	
Facsimile	(04) 460 4995
Further Contact Details:	
Address	Gaucher Panel Co-ordinator PHARMAC P O Box 10-254 WELLINGTON
Phone	(04) 460 4990
Email	gaucherpanel@pharmac.govt.nz

Patient Details	
Last Name	
First Name/s	
NHI No	
Date of birth	
Gender	Male: ☐ Female: ☐
DHB of domicile	

Applying Practitioner		
Speciality		NZMC Registration Number:
Last Name		
First Name		
Contact address		
Phone		
Fax		
Email Address		
Cell phone		

Patient physical examination
Date of examination:
Weight (kg):
Height (cm) (if child, please attach height chart):

Current dose taliglucerase (max 30 units/kg every other week)

Transition comments (attach additional information if more space is needed)

Has patient transitioned from imiglucerase to taliglucerase alfa since December 2018? When? Dose? Any issues with patient acceptance, timing, tolerance, access to treatment?

Renewal criteria

Must be ALL of 1-7

☐	1	Patient has demonstrated a symptomatic improvement or no deterioration in the main symptom for which therapy was initiated; and
☐	2	Patient has demonstrated a clinically objective improvement or no deterioration in haemoglobin levels, platelet counts and liver and spleen size; and
☐	3	Radiological (MRI) signs of bone activity performed at two years since initiation of treatment, and three yearly thereafter, demonstrate no deterioration shown by the MRI, compared with MRI taken immediately prior to commencement of therapy or adjusted dose; and
☐	4	Patient has not had severe infusion-related adverse reactions which were not preventable by appropriate pre-medication and/or adjustment of infusion rates; and
☐	5	Patient has not developed another medical condition that might reasonably be expected to compromise a response to ERT; and
☐	6	Patient is compliant with regular treatment and taliglucerase alfa is to be administered at a dose no greater than 30 unit/kg every other week rounded to the nearest whole vial (200 units), unless otherwise agreed by PHARMAC; and
☐	7	Supporting clinical information including test reports, MRI whole body STIR, haematological data, and other relevant investigations are submitted to the Gaucher Panel for assessment as required.

	Have you included the following? Please indicate attachments.
☐	*test reports*
☐	*MRI whole body STIR*
☐	*haematological data*
☐	*other relevant investigations. Specify:*

Applicant's Declaration

I confirm that the above and attached details are correct and that in signing this form I understand that I may be audited. I recognise the requirements for monitoring and managing treatment with taliglucerase alfa.

Complete reports are attached, including test reports, MRI whole body STIR, haematological data and other relevant investigations.

Signature: Date:

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