Specialised Services Commissioning Policy: CP55

Drug Treatment for Lysosomal Storage Disorders

Document information	
Document purpose	Policy
Document name	Drug Treatment for Lysosomal Storage Disorders
Author	Welsh Health Specialised Services Committee
Published date	March 2013
Last Updated	September 2017
WHSSC Executive Lead:	Director of Planning
Target audience	Chief Executives, Medical Directors, Directors of Finance, Chief Pharmacists, LSD Clinicians
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
Document No:	CP55

Document History

Revision History

Version No.	Revision date	Summary of Changes	Updated to version no.:
1.0	06/08/07	Version 1.0 Addition of annexes; new and revised clinical guidelines	
1.0	02/11/07	Version 1.0 Addition of NCG ERT/LSD Group	
2.0	15/09/11	Following strategy development through option appraisal, policy position undated to only drugs approved by the All-Wales Medicines Strategy Group can be prescribed.	2.1
2.1	July 2012	Transferred to new template minor changes made.	2.2
2.2	March 2013	Ratified through Chair's Action on behalf of Management Group	3.0
3.0	April 2017	Addition of Migalastat as treatment option for Fabry disease. Removed reference to CP56 Service specification and changed the document footer to CP55.	3.1
3.1	August 2017	Added Eliglustat as treatment option for Gaucher disease. Plain language summary added. National guidance amended.	4.0
Date of next revision		**Review date September 2020**	

Policy Statement

<table>
<tr><td>Background</td><td>Lysosomal Storage Disorders are a class of metabolic diseases, which are caused by a deficiency in the activity of a specific enzyme, which impairs the ability of the cell to carry out its normal degradation process. The disorders which can be treated with drug therapy are summarised in the table below.

<table>
<tr><th>Disease</th><th>Drugs</th></tr>
<tr><td>Gaucher disease I and III</td><td>Cerezyme,
Velaglucerase,
Miglustat
Eliglustat</td></tr>
<tr><td>Anderson-Fabry Disease</td><td>Replagel, Fabrazyme, Migalastat*</td></tr>
<tr><td>Pompe disease</td><td>Myozyme</td></tr>
<tr><td>MPS I (Hurler/Hurler-Scheie)</td><td>Aldurazyme</td></tr>
<tr><td>MPS II (Hunter syndrome)</td><td>Elaprase</td></tr>
<tr><td>MPS IV
Maroteaux-Lamy syndrome
Niemann-Pick syndrome</td><td>Naglazyme
Miglustat</td></tr>
</table>
**Migalastat for patients over 16 years of age*

The conditions are extremely rare and the drugs meet the European Union definition of orphan drugs.</td></tr>
<tr><td>Summary of Access Criteria</td><td>This Policy does not apply retrospectively to patients already on drug treatment.

WHSSC funds drug therapy for new patients with Lysosomal Storage Disorders only for those drugs that have been approved by the All Wales Medicines Strategy Group (AWMSG), and the National Institute for Health and Care Excellence (NICE).

The criteria for drug treatment are as follows:
• a confirmed genetic or enzymatic diagnosis of the relevant Lysosomal Storage Disorder;</td></tr>
</table>

	• a documented view from the responsible Consultant that the patient is likely to benefit from drug treatment. • the drug is licensed and the patient's condition meets the condition of the license; • the drug is approved by the AWMSG and NICE • the relevant gatekeeper at Cardiff and Vale University Health Board has given approval for treatment to start. All of the criteria must be met in order to start drug treatment.
Responsibilities	Referrers should: • Inform the patient that this treatment is not routinely funded outside the criteria in this policy; and • Refer via the agreed pathway. Clinician considering treatment should: • Discuss all the alternative treatment with the patient; • Advise the patient of any side effect and risks of the potential treatment; • Inform the patient that treatment is not routinely funded outside of the criteria in the policy; and • Confirm that there is contractual agreement with WHSSC for the treatment. • In all other circumstances submit an IPFR request. The role of the nominated gatekeeper is to maintain an overview of all Welsh patients on drug treatment and to approve drug treatment for new patients under WHSSC's Referral Management process.

Table of Contents

1. Aim

1.1 Introduction

The document has been developed as the policy for the drug treatment of Lysosomal Storage Disorders for patients resident in Wales.

The purpose of this document is to:

- Clearly set out the circumstances under which patients will be able to access drug treatment for Lysosomal Storage Disorders;
- Clarify the referral process;
- Indicate which organisations are able to provide drug treatment for Lysosomal Storage Disorders for Welsh patients;
- Outline the criteria that must be met in order for drug treatment to be prescribed.

1.2 Plain language summary

Lysosomal Storage Disorders are a class of metabolic diseases, which are caused by a deficiency in the activity of a specific enzyme, which impairs the ability of the cell to carry out its normal degradation process. This leads to an abnormal build-up of various toxic materials in the cells. Depending on the disorder, this may have multi-system effects.

This policy describes the treatments that are funded within NHS Wales for patients with Lysosomal Storage Disorders, the clinical criteria that must be met, the referral pathways and which hospitals can provide these treatments.

1.3 Relationship with other Policies and Service Specifications

This document should be read in conjunction with the following document:

- All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR).

2. Scope

2.1 Definition

The diseases which can be treated with drug therapy are summarised in the table below. Of the drugs listed, all are enzyme replacement therapies with the exception of Miglustat, which is a substrate reduction therapy.

Disease	**Drugs**
Gaucher disease I and III	Cerezyme, Velaglucerase, Miglustat, Eliglustat
Anderson-Fabry Disease	Replagel, Fabrazyme, Migalastat*
Pompe disease	Myozyme
MPS I (Hurler/Hurler-Scheie)	Aldurazyme
MPS II (Hunter syndrome)	Elaprase
MPS IV Maroteaux-Lamy syndrome Niemann-Pick syndrome	Naglazyme Miglustat

**Migalastat for patients over 16 years of age*

2.2 Aims and objectives

This policy aims to describe the policy for the drug treatment of Lysosomal Storage Disorders for patients resident in Wales.

The objectives are to:

- Clearly set out the circumstances under which patients will be able to access drug treatment for Lysosomal Storage Disorders;
- Clarify the referral process;
- Indicate which organisations are able to provide drug treatment for Lysosomal Storage Disorders for Welsh patients;
- Outline the criteria that must be met in order for drug treatment to be prescribed.

2.3 Codes

ICD-10 Codes

LSDs (Mucopolysaccharidoses Pompe)	
LSDs (Hurler)	E76.0 Type 1
LSDs (Hunter)	E76.1 Type 11
LSDs (Morquio)	E76.2 also Types 111, 1V and V11
Sphingolipidoses (Gaucher)	E75.2
Sphingolipidoses (Anderson-Fabry)	E75.2
Sphingolipidoses (Niemann-Pick)	E75.2

3. Access Criteria

3.1 Clinical Indications – general principles

The diagnosis of the Lysosomal Storage Disorder should be based upon the full assessment of clinical signs and symptoms and a documented deficiency of the relevant enzyme.

- DNA mutational analysis may also be undertaken where appropriate.

3.2 Criteria for Treatment

The criteria for drug treatment are as follows:

- A confirmed genetic or enzymatic diagnosis of the relevant Lysosomal Storage Disorder; and
- A documented view from the responsible Consultant that the patient is likely to benefit from drug treatment, based on the published national guidance e.g. NICE (www.nice.org.uk), AWMSG (www.awmsg.org)
- The drug is licensed and the patient's condition meets the condition of the license
- The drug is approved by the All-Wales Medicines Strategy Group
- The relevant gatekeeper at Cardiff and the Vale has given approval for treatment to start

All of the criteria must be met in order to start drug treatment.

3.3 Referral Pathway (Annex i)

All new patients should be referred to Cardiff and Vale University Health Board.

The nominated gatekeeping Consultants are:

- Dr Duncan Cole, Consultant in Adult Metabolic Medicine
- Dr Graham Shortland, Consultant in Paediatric Metabolic Medicine

All onward referrals to other centres should be via the centre in Cardiff. Shared care arrangements will be made for patients from North Wales with the NCG designated centres in Manchester and Salford. The Consultants at Cardiff and Vale UHB may also refer onwards to other NCG designated centres if clinically required.

3.4 Exclusions

The exclusion criteria are:

- The drug is not approved by the All-Wales Medicines Strategy Group (this includes drugs which have not been considered or are subject to Statements of Advice)
- The presence of a illness or disease where survival is unlikely to be improved by these drug treatments
- The patient is pregnant or lactating
- The patient will not, or cannot comply with the treatment regime

Patients must be counselled and advised that if their condition deteriorates whilst on optimum drug therapy, the drug treatment will be stopped. (Deterioration should be measured as per the National Guidelines).

3.5 Exceptions

If the patient does not meet the criteria for treatment, but the referring clinician believes that there are exceptional grounds for treatment, an Individual Patient Funding Request (IPFR) can be made to WHSSC under the *All Wales Policy for Making Decisions on Individual Patient Funding Requests (IPFR).*

If the patient wishes to be referred to a provider out of the agreed pathway and the referring clinician believes that there are exceptional grounds for treatment at an alternative provider, an Individual Patient Funding Request (IPFR) can be made to WHSSC under the *All Wales Policy for Making Decisions on Individual Patient Funding Requests (IPFR).*

Guidance on the IPFR process is available at:
www.whssc.wales.nhs.uk

3.6 Responsibilities

Referrers should:

- Inform the patient that this treatment is not routinely funded outside the criteria in this policy; and
- Refer via the agreed pathway

Clinician considering treatment should:

- Discuss all the alternative treatment with the patient;
- Advise the patient of any side effect and risks of the potential treatment;
- Inform the patient that treatment is not routinely funded outside of the criteria in the policy; and

- Confirm that there is contractual agreement with WHSSC for the treatment.

The role of the Consultant responsible for the patient is to:

- Confirm the diagnosis
- Assess whether the patient could benefit from drug treatment according to the published national guidance e.g. NICE (www.nice.org.uk), AWMSG (www.awmsg.org)
- Check that the drug is licensed and approved by the All-Wales Medicines Strategy Group
- Ensure that the relevant gatekeeper at Cardiff and the Vale UHB has given approval for treatment to start
- If the above criteria are met and the patient is started on treatment the role of the Consultant responsible for the patients is to:
- Follow-up the patient as clinically required
- Manage the patient according to the National Guidelines*
- Undertaken an annual review of each patient
- Assess whether the patient continues to benefit from drug treatment
- Titrate the drug dosages according to the patient's presentation
- Counsel patients that if their condition deteriorates, drug treatment will be stopped
- To stop drug treatment if the patient's condition deteriorates

In all other circumstances submit an IPFR request.

4. Putting Things Right: Raising a Concern

Whilst every effort has been made to ensure that decisions made under this policy are robust and appropriate for the patient group, it is acknowledged that there may be occasions when the patient or their representative are not happy with decisions made or the treatment provided. The patient or their representative should be guided by the clinician, or the member of NHS staff with whom the concern is raised, to the appropriate arrangements for management of their concern:

- When a patient or their representative is unhappy with the decision that the patient does not meet the criteria for treatment further information can be provided demonstrating exceptionality. The request will then be considered by the All Wales IPFR Panel.
- If the patient or their representative is not happy with the decision of the All Wales IPFR Panel the patient and/or their representative has a right to ask for this decision to be reviewed. The grounds for the review, which are detailed in the All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR), must be clearly stated. The review should be undertaken, by the patient's Local Health Board;
- When a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for NHS Putting Things Right. For services provided outside NHS Wales the patient or their representative should be guided to the NHS Trust Concerns Procedure, with a copy of the concern being sent to WHSSC.

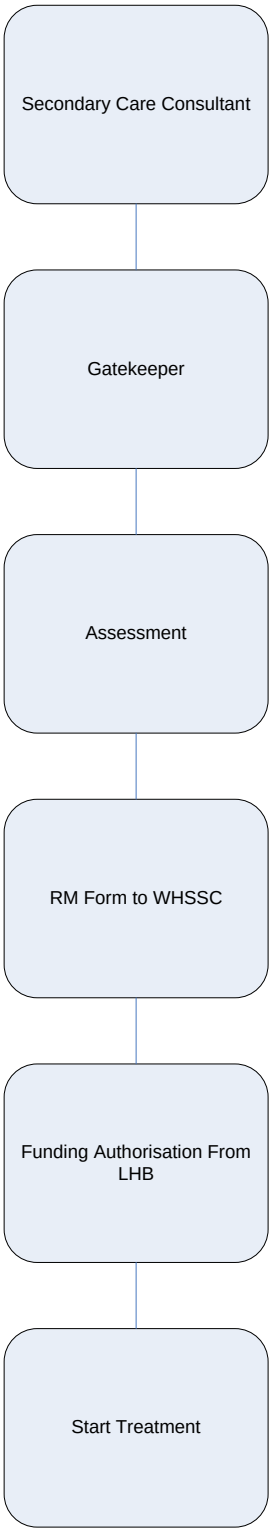
5. Equality Impact and Assessment

The Equality Impact Assessment (EQIA) process has been developed to help promote fair and equal treatment in the delivery of health services. It aims to enable Welsh Health Specialised Services Committee to identify and eliminate detrimental treatment caused by the adverse impact of health service policies upon groups and individuals for reasons of race, gender re-assignment, disability, sex, sexual orientation, age, religion and belief, marriage and civil partnership, pregnancy and maternity and language (Welsh).

This policy has been subjected to an Equality Impact Assessment. The Assessment demonstrates the policy is robust and there is no potential for discrimination or adverse impact. All opportunities to promote equality have been taken.

Annex (i) Referral Pathway

Adult Referral Pathway



Annex (ii) Checklist

CP55 Lysosomal Storage Disorders

The following checklist should be completed and retained as evidence of policy compliance by the receiving centre. It is expected that this evidence will be provided at the point of invoicing by the receiving centre.

i) Where the patient meets the criteria **AND** the procedure is included in the contract **AND** the referral is received by an agreed centre, the form should be completed by the receiving centre for audit purposes.
ii) The patient meets the criteria **AND** is received at an agreed centre, but the procedure is not included in the contract. The checklist must be completed and submitted to WHSSC for prior approval to treatment.
iii) The patient meets the criteria but wishes to be referred to a non contracted provider. An Individual Patient Funding Request (IPFR) Form must be completed and submitted to WHSSC for consideration.
iv) The patient does not meet criteria, but there is evidence of exceptionality. An Individual Patient Funding Request (IPFR) Form must be completed and submitted to WHSSC for consideration for treatment.