GIG CYMRU NHS WALES

Pwyllgor Gwasanaethau Iechyd Arbenigol Cymru (PGIAC)

Welsh Health Specialised Services Committee (WHSSC)

Specialised Services

Policy Position PP155

Pasireotide for Cushings' Disease

July 2019

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Document information	
Document purpose	Policy Position
Document name	Pasireotide for Cushings' Disease
Author	Welsh Health Specialised Services Committee
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Commissioning Team	Neurological and Complex Conditions
Target audience	Chief Executives, Medical Directors, Directors of Finance, Chief Pharmacists
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
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Contents

Policy Statement

Welsh Health Specialised Services Committee (WHSSC) will commission Pasireotide for people with Cushings' Disease in accordance with the criteria outlined in this document.

In creating this policy WHSSC has reviewed the relevant guidance issued by NHS England and has concluded that Pasireotide for Cushings' disease should be made available.

Disclaimer

WHSSC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this policy position statement.

This policy may not be clinically appropriate for use in all situations and does not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian.

WHSSC disclaims any responsibility for damages arising out of the use or non-use of this policy position statement.

1. Introduction

This Policy Position statement has been developed for the planning and delivery of Pasireotide for Cushings' disease for people resident in Wales. This service is only commissioned by the Welsh Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

1.1 Plain language summary

Cushing's disease is caused by a tumour of the 'pituitary gland'.

- This means that the pituitary gland makes too much of a hormone called 'adrenocorticotropic hormone' (ACTH).
- ACTH then causes the 'adrenal gland' to make too much of another hormone called 'cortisol'.
- Cortisol is known as the 'stress hormone' and is involved in lots of processes in the body, including control of blood sugar and immune responses.

Cushing's disease is rare - affecting only one or two people in every million, per year. However, it can lead to a number of medical problems and shorten life expectancy. Without treatment, around half of people with Cushing's disease would not live for longer than 5 years. The main causes of death are heart disease and stroke.

1.2 Aims and Objectives

This policy position proposal aims to define the commissioning position of WHSSC on the use of Pasireotide for Cushings' disease.

The objectives of this policy are to:

- ensure commissioning for the use of Pasireotide for Cushings' disease is evidence based
- ensure equitable access to Pasireotide for Cushings' disease
- define criteria for people with Cushings' disease to access treatment
- improve outcomes for people with Cushings' disease

1.3 Epidemiology

Cushing's disease is a rare condition with an incidence of 1-2 per million population per year. For some patients (20-40%) primary pituitary surgery is not curative and further treatment is required. Medical therapy is used in order to manage the condition whilst waiting for curative treatment to become possible or effective.

Cushing's disease is a heterogeneous disorder requiring a multi-disciplinary and individualised approach to patient management. Generally, the treatment of choice for Cushing's disease is curative surgery with selective pituitary resection. Second-line treatments include more radical surgery, pituitary radiation therapy, medical therapy, and bilateral adrenalectomy.

Because of the significant morbidity of Cushing's disease, early diagnosis and prompt therapy is important.

1.4 Current Treatment

The first treatment for Cushing's disease is usually an operation to remove the tumour. However, in around 20% to 40% of people, this does not cure the illness. In these people, other treatments will be needed.

This may include:

- another operation on the pituitary gland
- radio-therapy
- an operation on the adrenal gland.

Medicines are often used as part of treatment to help control the illness. However, these treatments are not a cure and cannot be used for a long time as they may cause side effects.

Medicines that might be used include:

- those that stop cortisol being made
- those that stop the adrenal gland from releasing cortisol
- those that stop cortisol working in the same way in the body
- those that stop the pituitary gland from releasing ACTH.

1.5 New Treatment

Pasireotide diaspartate is a medicine that stops the pituitary gland from releasing ACTH. It is put forward as an alternative treatment that might benefit patients if:

- they are not able to tolerate the usual medicines
- their symptoms are not well controlled by the usual medicines.

1.6 What NHS Wales has decided

WHSSC has carefully reviewed the relevant guidance issued by NHS England. We have concluded that there is enough evidence to fund the use of Pasireotide for Cushings' disease within the criteria set out in section 2.1.

2. Criteria for Commissioning

The Welsh Health Specialised Services Committee approve funding of Pasireotide diaspartate: an injectable medical therapy for the treatment of Cushings' Disease in-line with the criteria identified in the policy.

2.1 Inclusion Criteria

Pasireotide diaspartate should be used, according to its licensed dose, for patients with Cushing's disease requiring medical therapy who have not achieved control, or who are unable to tolerate, metyrapone and ketoconazole.

As a number of treatment modalities are available, patients will have their condition managed by a full multi-disciplinary team with access to a dedicated pituitary surgeon, pituitary endocrinologist, laparoscopic adrenal surgeon and pituitary radiotherapist.

The decision to use pasireotide diaspartate must be endorsed by the patient's multi-disciplinary team (with experience in the management of Cushing's disease) with support from other relevant service areas.

Pasireotide diaspartate may only be used where a definitive curative therapy is planned (further surgery, radiotherapy or bilateral adrenalectomy) and should only be used for a defined period (for example, while waiting for radiotherapy treatment to become effective or to stabilise prior to surgery).

In all cases initial therapy will be for a defined period of 2 months. Pasireotide diaspartate therapy may continue if tolerated by the patient and if measures of cortisol production show a 50% fall compared to levels measured before commencing treatment.

Cortisol production must be monitored every 2 months with a trial of withdrawal as cortisol production returns to the normal range.

2.2 Exclusion Criteria

Patients who require medical therapy but have not trialled, and are not contraindicated to, metyrapone and ketoconazole. Patients who are contraindicated to pasireotide diaspartate as per the licence.

2.3 Stopping Criteria

Pasireotide diaspartate will be stopped if treatment is not tolerated by the patient. Pasireotide diaspartate will be stopped if measures of cortisol production do not show an improvement at 2 months and a 50% fall from baseline at 4 months. Pasireotide diaspartate will be withdrawn when definitive therapy becomes effective. This may require a trial period off

pasireotide diaspartate therapy to demonstrate normal or low cortisol production (pasireotide diaspartate may need to be reinstated if unsuccessful).

2.4 Continuation of Treatment

Healthcare professionals are expected to review a patient's health at regular intervals to ensure they are demonstrating an improvement to their health due to the treatment being given.

If no improvement to a patient's health has been recorded then clinical judgement on the continuation of treatment must be made by the treating healthcare professional.

2.5 Acceptance Criteria

The service outlined in this specification is for patients ordinarily resident in Wales, or otherwise the commissioning responsibility of the NHS in Wales. This excludes patients who whilst resident in Wales, are registered with a GP practice in England, but includes patients resident in England who are registered with a GP Practice in Wales.

2.6 Patient Pathway (Annex ii)

Primary treatment for Cushing's disease is pituitary surgery. For some patients (30-40%) this is not curative and radiotherapy to the tumour remnant can result in cure over the following months or years. Medical therapy is most commonly used at this stage in order to manage the condition whilst waiting for radiotherapy to become effective. In all cases where medical therapy is prescribed, pasireotide diaspartate is commissioned as a second line treatment where metyrapone and ketoconazole have not been tolerated or are not clinically effective.

Pasireotide diaspartate should only be used for a defined period for patients who are on a curative pathway - for example patients who are waiting for radiotherapy treatment to become effective or require additional condition management prior to surgery. The license states: after two months, the patient's response to treatment should be evaluated, and the dose adjusted as appropriate or treatment stopped if no benefit is seen.

For some patients who are unable to tolerate medical therapy, adrenalectomy may be considered as a more radical approach to reduce treatment time.

In summary, the treatment pathway for an individual patient can be complex and medical therapy may be used at a number of stages to stabilise the condition however it will not in itself result in a long-term cure.

Patients who do not receive or respond to curative therapy would likely require lifelong palliative support.

A flow diagram of the most common patient pathway is outlined in Annex ii.

2.7 Exceptions

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If the patient wishes to be referred to a provider outside of the agreed pathway, and IPFR should be submitted.

Further information on making IPFR requests can be found at: Welsh Health Specialised Services Committee (WHSSC) | Individual Patient Funding Requests

2.8 Clinical Outcome and Quality Measures

The Provider must work to written quality standards and provide monitoring information to the lead commissioner.

The centre must enable the patient's, carer's and advocate's informed participation and to be able to demonstrate this. Provision should be made for patients with communication difficulties and for children, teenagers and young adults.

2.9 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 effects 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 an IPFR must be submitted.

3. Documents which have informed this policy

The following documents have been used to inform this policy:

- **NHS England policies**
  - Clinical Commissioning Policy: Pasireotide diaspartate: an injectable medical therapy for the treatment of Cushings' Disease, NHS England 16052/P. December 2016

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

- **NHS Wales**
  - All Wales Policy: Making Decisions in Individual Patient Funding requests (IPFR).

4. Date of Review

This document will be reviewed when information is received which indicates that the policy requires revision.

5. Putting Things Right: Raising a Concern

5.1 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.

If 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.

5.2 Individual Patient Funding Request (IPFR)

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent review of the process to be undertaken by the patient's Local Health Board. The ground for the review, which are detailed in the All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR), must be clearly stated

If the patient wishes to be referred to a provider outside of the agreed pathway, an IPFR should be submitted.

Further information on making IPFR requests can be found at: Welsh Health Specialised Services Committee (WHSSC) | Individual Patient Funding Requests

6. 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 Codes

Code Category	Code	Description
HCPCS	C9454	Pasireotide
ICD10	E24	Cushing's Syndrome

Annex ii Patient Pathway