GIG CYMRU NHS WALES

Pwyllgor Gwasanaethau Iechyd Arbenigol Cymru (PGIAC)

Welsh Health Specialised Services Committee (WHSSC)

Specialised Services Policy Position PP217

Volanesorsen for treating familial chylomicronaemia syndrome

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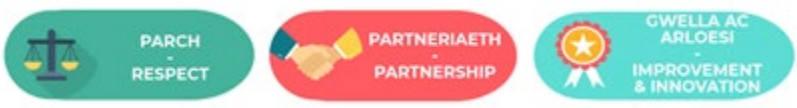

Document information	
Document purpose	Policy Position
Document name	Volanesorsen for treating familial chylomicronaemia syndrome
Author	Welsh Health Specialised Services Committee
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Commissioning Team	Women and Children
Target audience	Chief Executives, Medical Directors, Directors of Finance, Pharmacists and Inherited Metabolic Disease Consultants.
Description	NHS Wales propose to routinely commission this specialised service in accordance with the criteria described in this policy
Document No	PP217

Contents

Policy Statement

Welsh Health Specialised Services Committee (WHSSC) will commission Volanesorsen for treating familial chylomicronaemia syndrome in accordance with the criteria outlined in this document.

In creating this document WHSSC has reviewed the relevant guidance issued by National Institute of Health and Care Excellence[1] (NICE) and has concluded that Volanesorsen 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] Overview | Volanesorsen for treating familial chylomicronaemia syndrome | Guidance | NICE

1. Introduction

This Policy Position has been developed for the planning and delivery of Volanesorsen for treating familial chylomicronaemia syndrome for people resident in Wales. This service will only be commissioned by the Welsh Health Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

1.1 Plain language summary

Familial chylomicronaemia syndrome (FCS) is a rare genetic metabolic disorder of lipid metabolism caused by homozygous mutations in the lipoprotein lipase gene. It is characterised by high levels of triglycerides in the plasma and a build-up of chylomicrons (the lipoprotein particles responsible for transporting dietary fat from the intestine to the rest of the body). Symptoms include repeated episodes of severe abdominal pain, unpredictable and recurrent episodes of acute pancreatitis, liver and spleen enlargement, and fatigue. Acute pancreatitis is a life-threatening condition for which intensive care may be needed. Repeated attacks of acute pancreatitis may lead to chronic pancreatitis. Diabetes can develop as a result of pancreatitis and often makes FCS more difficult to manage.

1.2 Aims and Objectives

This Policy Position aims to define the commissioning position of WHSSC on the use of Volanesorsen for treating familial chylomicronaemia syndrome.

The objectives of this policy are to:

- ensure commissioning for the use of Volanesorsen is evidence based
- ensure equitable access to Volanesorsen
- define criteria for people with Volanesorsen to access treatment
- improve outcomes for people with Familial Chylomicronaemia Syndrome.

1.3 Epidemiology

The prevalence of FCS is estimated to be 1 to 2 per million people, which equates to about 55 to 110 people in England. At the time of the evidence submission, there were thought to be around 80 to 100 people with FCS eligible for treatment with Volanesorsen in the UK[2].

1.4 Current Treatment

Current treatment options for people with FCS are limited. To keep plasma triglyceride levels low, management consists of severely restricting dietary fat intake (usually to between 10 g and 20 g daily, about a quarter of the normal daily intake suggested for an adult) and consuming no alcohol.

[2] https://www.nice.org.uk/guidance/gid-hst10015/documents/final-evaluation-determination-document

People with the condition may take several drugs to control pain and other symptoms of FCS, including corticosteroids, analgesics, anxiolytics, antidepressants, diabetes treatments and antithrombotic drugs. People on a fat-restricted diet need supplements of essential fatty acids (linoleic and alpha linolenic acids) and fat-soluble vitamins (vitamins A, D, E and K). In addition, treatments for hypercholesterolaemia (such as fibrates, nicotinic acids and statins) may be prescribed but are of limited value. The strict dietary regimen is highly restrictive and often challenging for people with the condition and their families. Also, people often still have high triglyceride levels even when the diet is closely followed.

1.5 Proposed Treatment

Volanesorsen is an antisense oligonucleotide inhibitor of apolipoprotein C-III (apoC-III) production. ApoC-III inhibits the metabolism of triglycerides via the actions of both the lipoprotein lipase and lipoprotein lipase-independent pathways. It selectively binds to apoC-III mRNA to prevent the production of the apoC-III protein, so increasing metabolism of triglycerides.

Volanesorsen is an adjunct to diet in adult patients with genetically confirmed familial chylomicronemia syndrome (FCS) and at high risk for pancreatitis, in whom response to diet and triglyceride-lowering therapy has been inadequate'. Volanesorsen is administered by subcutaneous injection.

The recommended starting dosage, as described in the summary of product characteristics, is 285 mg once weekly for 3 months, followed by down titration to a maintenance dosing schedule of once every 2 weeks.

1.6 What NHS Wales has decided

WHSSC has carefully reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE)[3] who have concluded that the use of Volanesorsen should be made available within the criteria set out in section 2.1.

[3] Overview | Volanesorsen for treating familial chylomicronaemia syndrome | Guidance | NICE

2. Criteria for Commissioning

The Welsh Health Specialised Services Committee approve funding of Volanesorsen for Welsh adult patients with familial chylomicronaemia syndrome in-line with the criteria identified in the policy.

2.1 Inclusion Criteria

Volanesorsen[4] is recommended, within its marketing authorisation, as an option for treating familial chylomicronaemia syndrome in adults with genetically confirmed familial chylomicronaemia syndrome who are at high risk of pancreatitis, and when response to diet and triglyceride-lowering therapy has been inadequate. It is recommended only if the company provides volanesorsen according to the commercial arrangement.[5]

2.2 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.

2.3 Patient Pathway (Annex ii)

Following confirmation of familial chylomicronaemia syndrome a patient will be referred to:

- Metabolic Medicine Service
  Directorate of Haematology, Immunology and Metabolic Medicine
  University Hospital of Wales
  Heath Park Way
  Cardiff
  CF14 4XW

- Wrexham Maelor Hospital
  Croesnewydd Road
  Wrexham
  LL13 7TD

[4] https://www.nice.org.uk/guidance/hst13/chapter/1-Recommendations
[5] Commercial arrangement: There is a simple discount patient access scheme for Volanesorsen. Contact, Mail.ul@sobi.com for details.

2.4 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

2.5 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.

2.6 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 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:

- **National Institute of Health and Care Excellence (NICE) guidance**
  - https://www.nice.org.uk/guidance/gid-hst10015/documents/final-evaluation-determination-document , Final Evaluation Document, 18th of September 2020.

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

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.