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# **Volanesorsen for treating familial chylomicronaemia syndrome**

## **Policy Position Statement: PPS217**

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# Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission Volanesorsen for treating familial chylomicronaemia syndrome in accordance with the criteria outlined in this document.

## Welsh Language

NWJCC is committed to treating the English and Welsh languages on the basis of equality, and endeavour to ensure commissioned services meet the requirements of the legislative framework for Welsh Language, including the [Welsh Language Act \(1993\)](#), the [Welsh Language \(Wales\) Measure 2011](#) and the [Welsh Language Standards \(No.7\) Regulations 2018](#).

Where a service is provided in a private facility or in a hospital outside of Wales, the provisions of the Welsh language standards do not directly apply but in recognition of its importance to the patient experience, the referring health board should ensure that wherever possible patients have access to their preferred language.

In order to facilitate this, NWJCC is committed to working closely with providers to ensure that in the absence of a Welsh speaker, written information will be offered, and people have access to either a translator or 'Language-line' if requested. Where possible, links to local teams should be maintained during the period of care.

## Decarbonisation

NWJCC is committed to taking assertive action to reducing the carbon footprint through mindful commissioning activities. Where possible and taking into account each individual patient's needs, services are provided closer to home, including via digital and virtual access, with a delivery chain for service provision and associated capital that reflects the NWJCC commitment.

## Disclaimer

NWJCC assumes that healthcare professionals will use their clinical judgement, knowledge and expertise when deciding whether it is appropriate to apply this document.

This document 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, or Local Authority.

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

# 1. Introduction

This Policy Position Statement 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 NHS Wales Joint Commissioning Committee (NWJCC) and applies to residents of all seven Health Boards in Wales.

In creating this document NWJCC has reviewed the relevant guidance issued by the National Institute of Health and Care Excellence (NICE)<sup>1</sup> and has concluded that Volanesorsen should be made available.

## 1.1 Background

### 1.1.1 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.

### 1.1.2 Aims and Objectives

This Policy Position aims to define the commissioning position of NWJCC 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.

### 1.1.3 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.

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<sup>1</sup> [Volanesorsen for treating familial chylomicronaemia syndrome HST 13 October 2020](#)

#### **1.1.4 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. 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.

#### **1.1.5 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 chylomicronaemia 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 marketing authorisation, is 285 mg once weekly for 3 months, followed by down titration to a maintenance dosing schedule of once every 2 weeks.

#### **1.1.6 What NHS Wales has decided**

NWJCC has carefully reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE)<sup>2</sup> who have concluded that the use of Volanesorsen should be made available within the criteria set out in section 2.1.

## **1.2 Equality Impact Assessment**

The Equality Impact Assessment (EIA) process has been developed to help promote fair and equal treatment in the delivery of health services. It aims to enable NHS Wales Joint

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<sup>2</sup> [Overview | Volanesorsen for treating familial chylomicronaemia syndrome | Guidance | NICE](#)

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Commissioning 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.

An EIA was carried out by NICE during the evaluation of Volanesorsen. For further details, please refer to the NICE website at: [equality-impact-assessment](#)

## 2. Recommendations

The recommendations below represent the views of NICE, arrived at after careful consideration of the evidence available. Health professionals are expected to take into account the relevant NICE guidance<sup>3</sup>, alongside the individual needs, preferences and values of the patient.

### 2.1 Inclusion Criteria

Volanesorsen<sup>4</sup> 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<sup>5</sup>.

### 2.2 Exclusion Criteria

- Hypersensitivity to the active substance or to any of the excipients listed in the marketing authorisation literature
- unexplained thrombocytopenia. Treatment should not be initiated in patients with thrombocytopenia (platelet count  $<140 \times 10^9/L$ ).

### 2.3 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.

### 2.4 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 patient's resident in England who are registered with a GP Practice in Wales.

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<sup>3</sup> [Volanesorsen for treating familial chylomicronaemia syndrome HST13 October 2020](#)

<sup>4</sup> <https://www.nice.org.uk/guidance/hst13/chapter/1-Recommendations>

<sup>5</sup> Commercial arrangement: There is a simple discount patient access scheme for Volanesorsen. Contact, [Mail.ul@sobi.com](mailto:Mail.ul@sobi.com) for details.

## 2.5 Patient Pathway and Designated Providers

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

## 2.6 Mechanism for funding

Volanesorsen will only be funded for patients registered via the Blueteq<sup>®</sup> system and where an appropriately constructed MDT has approved its use within highly specialised centres.

Where the patient meets the criteria in this policy and the referral is received by an agreed centre, a Blueteq<sup>®</sup> form should be completed for approval.

For further information on accessing and completing the Blueteq<sup>®</sup> form please contact NWJCC using the following email address: [NWJCCblueteq@wales.nhs.uk](mailto:NWJCCblueteq@wales.nhs.uk).

If a non-contracted provider wishes to treat a patient that meets the criteria they should contact NWJCC at [NWJCCipc@wales.nhs.uk](mailto:NWJCCipc@wales.nhs.uk). They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that Volanesorsen is prescribed and administered in accordance with its marketing authorisation. The company has a commercial arrangement. This makes volanesorsen available to the NHS with a discount. The size of the discount is commercial in confidence. Health Boards in Wales should refer to the AWTTTC Commercial Medicines Access References Tool (CMART) for further information on the Patient Access Scheme (PAS) price.

If treatment is discontinued, it is the responsibility of the prescribing team to discontinue the Blueteq<sup>®</sup> form.

## 2.7 Clinical Outcome and Quality Measures

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.

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 the NWJCC for the treatment.

In all other circumstances an IPFR must be submitted.

## 2.8 Action to be taken

- Providers and NWJCC are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the treatment will be commissioned.
- Providers are to ensure that all providers are purchasing Volanesorsen at the agreed discounted price.
- Providers are to ensure the need to approve Volanesorsen at the appropriate MDT and are registering use on the Blueteq<sup>®</sup> system, and the treatment will only be funded where the Blueteq<sup>®</sup> minimum dataset is fully and accurately populated.
- The Provider should work to written quality standards and provide monitoring information to NWJCC on request.

## 3. Putting things right

### 3.1 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 NWJCC.

### 3.2 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.

Further information on making IPFR requests can be found at: [Individual Patient Funding Requests](#)

## Contact Us

If you have a question related to this document, you can contact us using one of the methods outlined below.

If you would like this document in an alternative format and/or language, please contact us for assistance.

### **Email:**

NWJCC consultation mailbox – [nwjccconsultation@wales.nhs.uk](mailto:nwjccconsultation@wales.nhs.uk)

### **Telephone:**

General Enquiries – 01443 433112

### **Website:**

[Contact us - NHS Wales Joint Commissioning Committee](#)

### **Writing:**

If you wish to contact the NHS Wales Joint Commissioning Committee, you can write to us at one of our locations below, we welcome correspondence in Welsh or English:

#### **South Wales Offices**

Unit 1, Charnwood Court, Heol Billingsley, Nantgarw, CF15 7QZ

Unit G1 The Willowford, Main Avenue, Treforest Industrial Estate, Pontypridd, CF37 5YL

#### **North Wales Offices**

Unit 3, Media Point - Unit 3, Mold Business Park, Mold, CH7 1XY

Preswylfa, Hendy Road, Mold, CH7 1PZ