GIG CYMRU NHS WALES

Pwyllgor Gwasanaethau Iechyd Arbenigol Cymru (PGIAC)

Welsh Health Specialised Services Committee (WHSSC)

Specialised Services Policy Position Statement PP262

Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 for children aged under 16 years

November 2023

Version 1.0

PARCH - RESPECT

PARTNERIAETH - PARTNERSHIP

GWELLA AC ARLOESI - IMPROVEMENT & INNOVATION

Document information	
Document purpose	For information and action
Publication date	November 2023
Commissioning Team	Women and Children
Target audience	**For information** Chief Executives, Medical Directors, Directors of Finance, Directors of Planning, All Wales Medical Genetic Service
	For action Chief Pharmacists, Clinical Leads, Paediatric Consultant Neurologists, Paediatric neurology Nurse Specialists, Paediatric Nurse Specialists, Consultant paediatricians, Director of Nursing, Specialist Head of Finance and Commissioning, Health Board Commissioning Managers, Planning managers
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
Document No	PP262
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Contents

Policy Statement

Welsh Health Specialised Services Committee (WHSSC) will commission Cerliponase alfa for people aged under 16 years with neural ceroid lipofuscinosis type 2 in accordance with the criteria outlined in this document.

In creating this document WHSSC has reviewed the relevant guidance issued by the National Institute of Health and Care Excellence (NICE)[1] and has concluded that cerliponase alfa for neuronal ceroid lipofuscinosis type 2 should be made available.

Welsh Language

WHSSC 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, WHSSC 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

WHSSC 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 WHSSC commitment.

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.

[1] Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 NICE HST12, November 2019

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, or Local Authority.

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 cerliponase alfa for treating children under 16 years of age with neuronal ceroid lipofuscinosis type 2. 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 Background

Neuronal ceroid lipofuscinosis type 2 (CLN2) is a rare genetic disease caused by the lack of an enzyme called tripeptidyl peptidase 1 (TPP1). It is one form of neuronal ceroid lipofuscinosis, also known as Batten disease. CLN2 is inherited as an autosomal recessive disorder, which means that both chromosome copies carry mutations in the CLN2 gene, and both parents are unaffected carriers. A deficiency of TPP1 results in abnormal storage of proteins and lipids in neurons and other cells. Accumulation of these proteins and lipids prevents the cells from functioning as they should.

CLN2 progresses rapidly and predictably from presentation in late infancy to death by early adolescence. Symptoms in children with CLN2 appear in the second year of life and can then progress rapidly with a decline in speech, the onset of seizures, loss of mobility, involuntary muscle spasms and, later on, visual impairment leading to blindness. Ultimately, the child will become totally dependent on family and carers for all their needs. Life expectancy is around 8 years to early adolescence.

Cerliponase alfa (Brineura, BioMarin) is an enzyme replacement therapy consisting of a recombinant form of human tripeptidyl peptidase 1. It is expected to restore deficient tripeptidyl peptidase 1 (TPP1) activity in the brain caused by the genetic mutation.

The exact prevalence and incidence of CLN2 is unknown. It is estimated that, in the UK, around 3 to 6 children are diagnosed each year and currently around 30 to 50 children are living with the condition[1].

1.2 Equality Impact 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 subject to an Equality Impact Assessment in line with guidance contained in CPL-026[2].

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 EQIA was also carried out by NICE during the evaluation of cerliponase alfa. For further details, please refer to the NICE website at: https://www.nice.org.uk/guidance/hst12

[2] https://whssc.nhs.wales/publications/corporate-policies-and-procedures/corp-026-eqia-policy/

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[3], alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

- Cerliponase alfa is recommended as an option for treating neuronal ceroid lipofuscinosis type 2, also known as tripeptidyl peptidase 1 deficiency.

It is recommended only if the conditions in the Managed Access Agreement (MAA) are followed.

2.2 Exclusion Criteria

- People aged 16 years and older

Commissioning responsibility of cerliponase alfa for people aged 16 years and older lies with Health Boards in Wales.

2.3 Stopping Criteria

Stopping criteria are detailed in the MAA.

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.

[3] Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 NICE HST12, November 2019

2.6 Transition arrangements

Transition arrangements should be in line with Transition from children's to adults' services for young people using health or social care services NICE guidance NG43 and the Welsh Government Transition and Handover Guidance.

Transition involves a process of preparation for young people and their families for their transition to adulthood and their transition to adult services. This preparation should start from early adolescence 12-13 year olds. The exact timing of this will ideally be dependent on the wishes of the young person but will need to comply with local resources and arrangements.

The transition process should be a flexible and collaborative process involving the young person and their family as appropriate and the service.

The way this process is managed will vary on an individual case basis with multidisciplinary input often required and patient and family choice taken into account together with individual health board and environmental circumstances factored in.

2.7 Designated Providers

Patients should be referred for assessment of eligibility for treatment to a paediatric neurology/paediatric metabolic medicine team at the following centres:

Children's Hospital for wales
University hospital of wales
Health Park Way
Cardiff
CF14 4XW

Alderhey Children's Hospital
East Prescot Road
Liverpool
L14 5AB

Birmingham Children's hospital
Steelhouse Lane
Birmingham
B4 6NH

Treatment cannot commence until the patient and/or their parent or guardian has signed the MAA.

2.8 Blueteq and reimbursement

Cerliponase alfa will only be funded for patients registered via the Blueteq system and where an appropriately constructed MDT (including neurology, metabolic medicine and neurosurgery for device insertion) has approved its use within highly specialised paediatric metabolic medicine/neurology centres.

Where the patient meets the criteria in this policy and the referral is received by an agreed centre, a Blueteq form should be completed for approval. For further information on accessing and completing the Blueteq form please contact WHSSC using the following e-mail address: WHSSC.blueteq@wales.nhs.uk

If a non-contracted provider wishes to treat a patient that meets the criteria they should contact WHSSC (e-mail: WHSSC.IPC@Wales.nhs.uk). They will be asked to demonstrate they have an appropriate MDT in place.

Cerliponase alfa is administered into the cerebrospinal fluid by infusion via a surgically implanted intracerebroventricular access device (reservoir and catheter). It must only be given in a healthcare setting by a trained healthcare professional knowledgeable in intracerebroventricular infusion administration. The recommended dose is 300 mg cerliponase alfa once every other week, but lower doses are recommended in patients under 2 years[4].

The list price of cerliponase alfa is £20,107 per 300-mg pack (excluding VAT), consisting of 2×150-mg vials. The recommended dosage for those over 2 years old is 300 mg every other week (at an annual cost of £522,782 per person). The company has a commercial access agreement (CAA) which makes cerliponase alfa available to the NHS with a discount. The size of the discount is commercial in confidence. Health Boards in Wales should refer to the AWTTC vault for further information on the CAA price.

2.9 Action to be taken

- Health Boards and WHSSC are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the technology will be commissioned.

- WHSSC are to ensure that all providers are purchasing cerliponase alfa at the agreed discounted price.

- Providers are to ensure the need to approve cerliponase alfa at the appropriate MDT and are registering use on the Blueteq system, and

[4] Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 NICE HST12, November 2019

the treatment will only be funded where the Blueteq minimum dataset is fully and accurately populated.

- Providers are to determine estimated patient numbers and the current dose of any patient(s) who will transfer from any company compassionate use scheme or EAMS.

- The Provider should work to written quality standards and provide monitoring information to WHSSC 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 WHSSC.

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 All Wales IPFR Panel will then consider the request.

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

Annex i Codes

Please enter all relevant codes (OPCS/ICD)

Code Category	Code	Description
ICD-11	5C56.1	Neuronal ceroid lipofuscinosis