Reference number: 2422
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Clinical commissioning policy:

Lanreotide as a monotherapy for the treatment of symptomatic non-localised autosomal dominant polycystic liver disease (adults)

Summary

The off-label use of lanreotide is recommended to be available as a routine commissioning treatment option for autosomal dominant polycystic liver disease (PLD) within the criteria set out in this document.

The policy is restricted to adults aged ≥18 years old[1] in line with the findings of the evidence review.

Committee discussion

Please see Clinical Panel reports for full details of Clinical Panel's discussion.

The Clinical Priorities Advisory Group committee papers can be accessed on the NHS England website.

What we have decided

NHS England has carefully reviewed the evidence to treat autosomal dominant polycystic liver disease with lanreotide. We have concluded that there is enough evidence to make the treatment available at this time.

The evidence review which informs this commissioning position can be accessed on the NHS England website.

Plain language summary

Polycystic liver disease (PLD) is a rare genetic disease that exists in two forms, in isolation as autosomal dominant polycystic liver disease (ADPLD) or as part of autosomal dominant polycystic kidney disease (ADPKD). It is characterised by the formation of multiple cysts that cause progressive liver enlargement.

[1] Post-pubescent children can access under the Commissioning Medicines for Children Policy. Pre-pubescent children are not covered by this policy given the unavailability of safety data in this age group and children not being included in the drug's marketing authorisation.

About polycystic liver disease

Abnormal liver function and liver failure is rare in PLD. Most people with PLD have few to no symptoms and therefore do not require any treatment. However, for those otherwise defined as experiencing symptomatic PLD, symptoms can be broadly classified into two categories: mass effect resulting from the enlarged liver; and those which are complications of the cysts (such as infection, torsion, rupture, and haemorrhage). Mass effect can result in symptoms including abdominal distention, early satiety, postprandial fullness, gastro-oesophageal reflux, malnutrition, dyspnoea, hepatic venous-outflow obstruction (Budd-Chiari syndrome), inferior vena cava syndrome, portal-vein compression and bile-duct compression.

About current treatments

Current treatment of PLD depends on symptomatic burden as well as cyst distribution and size. Ideally following cross-sectional imaging and specialist multi-disciplinary team (MDT) consideration, patients with a dominant cyst may be offered aspiration sclerotherapy of the cyst, fenestration of the cyst(s) or partial liver resection. In severe cases, or for those with untreatable complications or no treatment options, a liver transplant may be offered. Injectable lanreotide would aim to reduce total liver volume (TLV) growth rate and reduce symptom burden associated with PLD.

About lanreotide

Inside cells, a molecule called adenosine 3',5'-cyclic monophosphate (cAMP) plays an important role in controlling activities like multiplying cells and fluid production. In PLD, this pathway can become overactive, leading to the growth of fluid-filled liver cysts. Lanreotide, known as a somatostatin analogue, is a medicine that mimics a natural hormone called somatostatin. This attaches to specific receptors on cells and blocks the production of cAMP. This helps reduce the amount of fluid in liver cysts.

Injectable lanreotide, as a single therapy, is proposed as an alternative first-line treatment option for adult patients with symptomatic non-localised autosomal dominant PLD where individuals decline, are unsuitable for, or show incomplete response to non-surgical treatments to the cysts (aspiration sclerotherapy), the creation of an opening (fenestration) or surgical resection. Lanreotide is licensed for the treatment of excessive growth hormone secretion (acromegaly), certain neuroendocrine tumours and their related symptoms. The use of this medicine in PLD is off-label.

Epidemiology and needs assessment

Though limited by a lack of a comprehensive established registry with national coverage, literature suggests that PLD has an estimated prevalence of 10 per 100,000 people, equating to around 5,000 people in England. However, many people with PLD do not have significant symptoms and only around 5% would have the combination of both diffuse non-dominant cystic distribution and significant symptoms that would warrant lanreotide. This would estimate approximately 250 people would have the characteristics suggesting benefit from lanreotide.

Due to the action of oestrogen in the development of cysts, women are disproportionately affected by PLD by a ratio of 6 to 1, experiencing greater symptoms and more severe disease (Masyuk et al, 2017).

Implementation

NHS England will routinely commission lanreotide within the patient pathway for individuals meeting all of the following inclusion criteria, and none of the exclusion criteria.

Inclusion criteria

Adult patients (aged 18 years and above) should be considered for treatment with lanreotide if they meet **all** the following criteria:

- Non-localised PLD resulting from ADPKD or ADPLD
- Who are symptomatic
- Who decline, are unsuitable for, or show incomplete response to the following:
    - Aspiration sclerotherapy
    - Fenestration
    - Surgical resection
- Who have exhausted all current standard care
- It is determined by the multidisciplinary team (MDT)[2] to gain benefit from the use of lanreotide.

Exclusion criteria

Patients are excluded if they meet **any** of the following exclusion criteria:

- Isolated polycystic kidney disease with an absence of liver involvement
- Known hypersensitivity to lanreotide or any of the excipients
- Active infection including, but not limited to, renal or liver cysts or urinary tract infections
- Symptomatic gallstones
- Active cancer
- Systolic/diastolic blood pressure > 180/110 mmHg
- Decompensated liver cirrhosis
- Diagnosis of any other liver or kidney cystic diseases

[2] It is recommended that the structure be similar to that of a hepatopancreaticobiliary (HPB) MDT. For consideration of treatment for post-pubescent children, this should include at least two consultants from the relevant subspecialty with active and credible expertise in the relevant field, including at least one consultant paediatrician. The paediatric MDT should include a paediatric pharmacist and other professional groups appropriate to the disease area.

- Patients for whom the treating clinician have assessed and identified one or more of the following conditions:
    - Who are pregnant, lactating, or potentially childbearing women without adequate contraception
    - Urinary tract lithiasis
    - Undergone kidney **or** liver transplantation
    - Advanced stage 4 or 5 chronic kidney disease
    - Diabetes mellitus that is not readily managed with anti-diabetic medication
    - Actively using systemic exogenous oestrogens

    and

    - Where the MDT considers such a condition or other individual clinical circumstances would limit the ability of the patient to gain benefit over risk from lanreotide.

Starting criteria

Treatment with lanreotide should be initiated and agreed following discussion at an MDT meeting. Treatment with lanreotide should be supervised by physician(s) with experience in the treatment of PLD.

The benefits and risk of treatment with lanreotide must be discussed and agreed with the patient through shared decision making prior to initiating treatment.

A baseline validated disease-specific questionnaire[3] score, standardised quality of life (QoL) questionnaire[4] score, heart rate, blood glucose, HbA1c, renal and liver function test should be taken. If not carried out as part of the diagnosis of PLD, liver cross-sectional imaging with volumetry should be completed.

Stopping criteria

Treatment will be stopped in patients who meet **any** of the following criteria at 1 year or earlier:

- Failure in symptom control defined as a lack of improvement in a validated disease-specific questionnaire[3] score
- A lack of improvement in the patient's QoL defined as a lack of improvement in a standardised QoL questionnaire[4] score
- Unacceptable side effects
- Patient or carer decision to stop treatment

[3] This can include the PLD Questionnaire (PLD-Q) or the PLD complaint-specific assessment (POLCA) score.
[4] This can include EQ-5D or Short Form-36 (SF-36).

- Patients for whom the treating clinician have assessed and identified one or more of the following conditions:
    - A rise in liver volume >5% above baseline[5]
    - Become pregnant
    - Developed advanced stage 4 or 5 chronic kidney disease
    - Development of diabetes mellitus that is not readily managed with anti-diabetic medication
    - Requiring the use of systemic exogenous oestrogens
    - Proceeding to renal **or** liver transplantation

    and

    - Where the MDT considers such a change in individual clinical circumstances would limit the ability of the patient to gain benefit over risk from lanreotide.

Monitoring

Patients must be monitored for symptoms and have their renal and liver function checked at regular intervals.

A formal medical review and MDT discussion to determine whether the treatment should continue, should take place by one year following the initiation of treatment. The formal medical review should include clinical assessment, liver cross-sectional imaging with volumetry, heart rate, blood glucose, HbA1c, renal and liver function test. Additional monitoring requirements for lanreotide should be met, as listed in the Summary of Product Characteristics (SmPC).

Dosing

The use of lanreotide for the treatment of PLD is off-label.

Dose reduction and/or dose interruption may be required based on individual safety and/or tolerability. This should be determined by the treating clinician.

Lanreotide may be available for home administration depending on local arrangements and patient requirements. If the patient is able to self-administer or can otherwise be given by family, carers and/or nurses, the relevant individuals must be taught how to store, administer and dispose appropriately.

The recommended initial dosage of long-acting lanreotide 90 milligrams (mg) to a maximum of 120mg subcutaneously every 28 days.

The dosage could be reduced down to 90 mg[6] if the estimated glomerular filtration rate (eGFR) <30 mL/min/1.73m2 or if there are significant side effects.

[5] Based on clinical consensus of available evidence.
[6] This is based on the evidenced dosing from the evidence review however is not a requirement set out in the SmPC.

Patient pathway

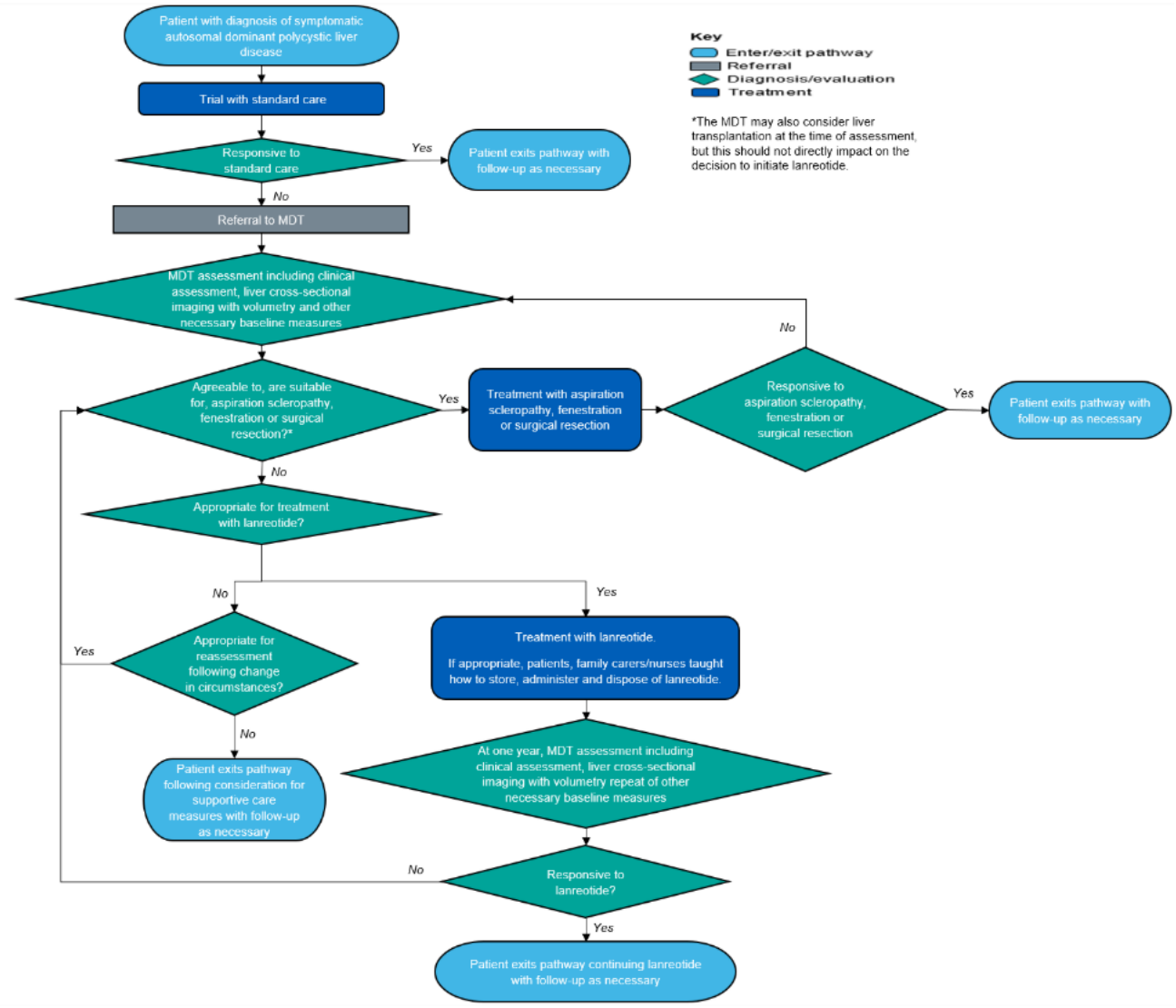

Figure 1 - Patient pathway for symptomatic severe autosomal dominant PLD

Governance arrangements

Service specification: Hepatobiliary and pancreas (adult)

Any provider organisation treating patients with this intervention will be required to assure itself that the internal governance arrangements have been completed before the medicine is prescribed. These arrangements may be through the Trust's Drugs and Therapeutics committee (or similar) and NHS England may ask for assurance of this process. Lanreotide must only be used for treatment in specialised centres or in collaboration with specialised centres under the supervision of a multi-disciplinary team.

Provider organisations must register all patients using prior approval software and ensure monitoring arrangements are in place to demonstrate compliance against the criteria as outlined.

Mechanism for funding

Lanreotide is categorised as high-cost drugs to be reimbursed via the fixed 'block' element of the contract.

Audit requirements

Trusts will be required to ensure that processes are in place to track both decision to treat and evidence of effectiveness, e.g. POLCA score. Use of nationally approved software systems and prior approval forms to track and audit use of lanreotide by clinicians is to be mandated, in order to ensure it is administered according to the Criteria for Commissioning.

Specific audit reports on the use of lanreotide and specific outcomes in this patient group will be requested by the commissioner. Participation in research studies is encouraged.

Policy review date

This document will be reviewed when information is received which indicates that the policy requires revision. If a review is needed due to a new evidence base then a new Preliminary Policy Proposal needs to be submitted by contacting england.CET@nhs.net.

Our policies provide access on the basis that the prices of therapies will be at or below the prices and commercial terms submitted for consideration at the time evaluated. NHS England reserves the right to review policies where the supplier of an intervention is no longer willing to supply the treatment to the NHS at or below this price and to review policies where the supplier is unable or unwilling to match price reductions in alternative therapies.

Equality statement

Promoting equality and addressing health inequalities are at the heart of NHS England's values. Throughout the development of the policies and processes cited in this document, we have:

- Given due regard to the need to eliminate discrimination, harassment and victimisation, to advance equality of opportunity, and to foster good relations between people who share a relevant protected characteristic (as cited under the Equality Act 2010) and those who do not share it; and

- Given regard to the need to reduce inequalities between patients in access to, and outcomes from healthcare services and to ensure services are provided in an integrated way where this might reduce health inequalities.

Definitions

Aspiration sclerotherapy	A minor procedure involving inserting a needle and removing fluid from the cyst followed by a temporary direct injection of sterile alcohol into the cyst cavities.

Autosomal dominant	Autosomal dominant is a pattern of inheritance characteristic of some genetic disorders. "Autosomal" is a type of gene and "dominant" means that a single copy of the mutated gene (from one parent) is enough to cause the disorder.
Cell proliferation	The processes that result in an increase in the number of cells.
Dyspnoea	Otherwise known as shortness of breath. It is the sensation that the individual can't get enough air into their lungs.
Fenestration	A surgical procedure that involves the removal of the free wall of a hepatic cystic allowing for any secreted fluid subsequently produced by the cyst lining to be absorbed by the abdomen.
Haemorrhage	Used to describe blood loss
Hepatic	Relating to the liver
Hepatic venous-outflow obstruction	An impediment to hepatic venous outflow anywhere from the small hepatic veins to the cavoatrial junction.
Liver resection	Surgical procedure to remove part of the liver.
Postprandial	During or relating to the period after a meal.
Somatostatin analogues	A manufactured version of somatostatin. It slows down the production of hormones.
Torsion	Action of twisting or the state of being twisted.

References

Masyuk TV, Masyuk AI, LaRusso NF. Therapeutic Targets in Polycystic Liver Disease. Curr Drug Targets. 2017;18(8):950-957. doi: 10.2174/1389450116666150427161743. PMID: 25915482; PMCID: PMC5777530.