Engagement Report

Topic details

Title of policy or policy statement:	Lanreotide as a monotherapy for the treatment of symptomatic non-localised autosomal dominant polycystic liver disease (adults)
Programme of Care:	Internal medicine
Clinical Reference Group:	Hepatobiliary and pancreas
URN:	2422

1. Summary

This report summarises the feedback NHS England received from engagement during the development of this policy proposition, and how this feedback has been considered.

2. Background

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.

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.

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.

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, medicine that mimics a natural hormone called somatostatin. This attaches to specific receptors on cells and block 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.

3. Engagement

Following stakeholder testing feedback, the Programme of Care has considered that the proposition offers a clear and positive impact on patient treatment, by potentially making a new treatment available which widens the range of treatment options without disrupting current care or limiting patient choice, and therefore further public consultation was not required. This decision has been assured by the Patient Public Voice Advisory Group.

NHS England has a duty under Section 13Q of the NHS Act 2006 (as amended) to 'make arrangements' to involve the public in commissioning. Full guidance is available in the Statement of Arrangements and Guidance on Patient and Public Participation in Commissioning. In addition, NHS England has a legal duty to promote equality under the Equality Act (2010) and reduce health inequalities under the Health and Social Care Act (2012).

The policy proposition underwent a two-week stakeholder testing between the 15th and 29th October 2025 with registered stakeholders from the following Clinical Reference Group:

- Hepatobiliary and Pancreas CRG

Respondents were asked the following consultation questions:

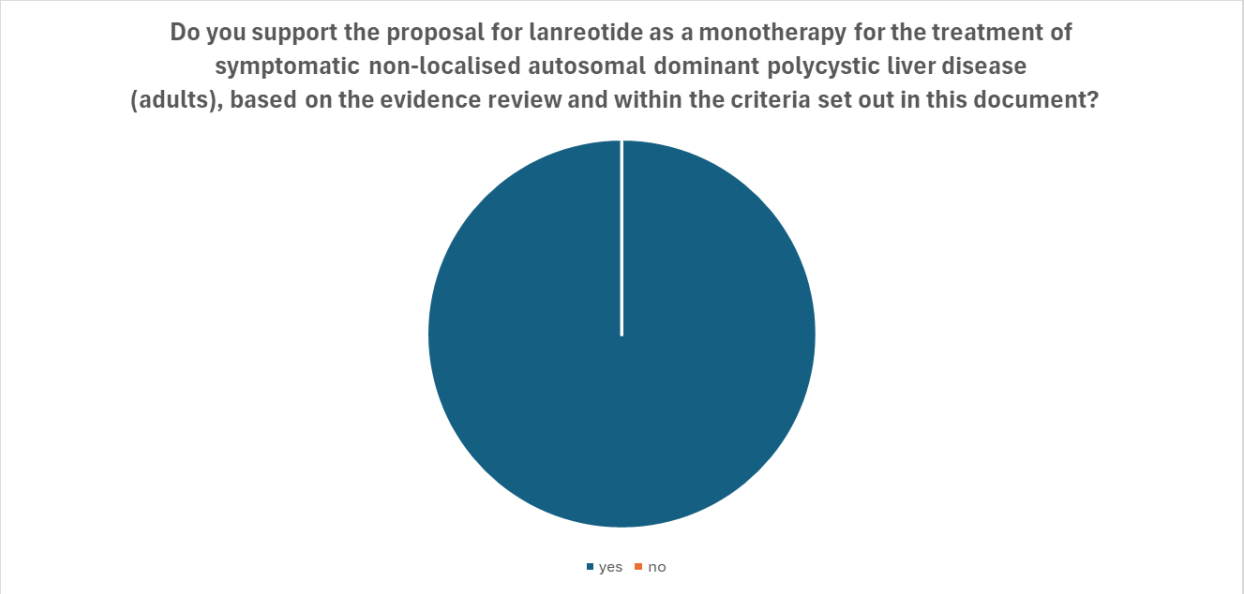
- Do you support the proposition for lanreotide for PLD to be available through routine commissioning based on the evidence review and within the criteria set out in this document?
- Do you believe that there are any potential positive and/or negative impacts on patient care as a result of making this treatment option available?
- Do you believe that there is any additional information that we should have considered in the evidence review?
- Do you support the Equality and Health Inequalities Impact Assessment (EHIA)?
- Does the Patient Impact Assessment (PIA) present a true reflection of the patient and carers lived experience of this condition?
- Do you have any further comments on the policy proposal?
- Do you have any potential conflict of interest relating to this document or service area?

4. Engagement Results

Number stakeholders responded: 4

- 2 clinicians
- 1 patient
- 1 pharmacist

All respondents were supportive of the policy proposition.



5. How has feedback been considered?

Responses to engagement have been reviewed by the Policy Working Group and the Internal Medicine PoC. The following themes were raised during engagement:

Keys themes in feedback	NHS England Response
Relevant Evidence	
When queried regarding the evidence, two individuals presented Aerts et al. (2019) and Chrispijn et al. (2011) as further evidence.	During the rapid literature review, van Aerts et al. (2019) was identified as an eligible paper and has been incorporated into the evidence review. This was considered by the PWG when developing the policy proposal. The rapid literature review considers papers within the last 10 year period. Due to Chrispijn et al. being published in 2011, it would be out of scope of the evidence review. Therefore this would not be considered by the PWG when developing the policy proposition.
One respondent shared their clinical experience of using octreotide being positive.	The use of octreotide in the context of non-localised autosomal dominant polycystic liver disease is out of scope of this policy proposition.
Impact Assessment	

One respondent did not agree that small magnitude of reduced cyst volume is unlikely to be clinically meaningful.	The Patient Impact Assessment captures the impact of the medical condition for which the proposed treatment is indicated. The evidence review noted that the reduced cyst volume is not likely to be clinically meaningful. The evidence review used the default minimal clinically important difference (MCID) for continuous outcomes to reach this conclusion. Though the PWG acknowledged that these improvements are not significant when compared to standard care, they chose to proceed with the policy proposition as it provides a further option for those who have already exhausted these approaches.
Potential impact on equality and health inequalities	
All respondents agreed with the Equality and Health Inequalities Impact Assessment (EHIA)	No further action.
Changes to criteria	
One respondent recommended caution in patients with history of hepatic cyst infection	Due to the evidence presented, an exclusionary criteria of 'active infection' including cysts has been included in the policy proposal. However, particularly due to the subjectivity of diagnosis, the PWG would not recommend those with a history of hepatic cyst infections to be excluded.

The responses should answer all the themes reported in section 4 and cover the outcome of reviews of any additional evidence highlighted during engagement

6. Has anything been changed in the policy proposition as a result of the stakeholder testing and consultation?

Nil.

7. Are there any remaining concerns outstanding following the consultation that have not been resolved in the final policy proposition?

Nil.