HSCB Service Notification for the managed entry of new medicines and technologies

Health and Social Care Board

1.	**Treatment & Condition** NICE Technology Appraisal 443 (TA443): Obeticholic acid for treating Primary Biliary Cholangitis
2.	**Associated appraisal body *&* Summary of ruling** NICE Technology Appraisal guidance (TA443), April 2017. **1.1** Obeticholic acid is recommended, within its marketing authorisation, as an option for treating primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid for people whose disease has responded inadequately to ursodeoxycholic acid or as monotherapy for people who cannot tolerate ursodeoxycholic acid. Obeticholic acid is recommended only if the company provides it with the discount agreed in the patient access scheme. **1.2** Assess the response to obeticholic acid after 12 months. Only continue if there is evidence of clinical benefit. Prior to the introduction of obeticholic acid, ursodeoxycholic acid was the only disease-modifying treatment available for PBC. Treatment with ursodeoxycholic acid was recommended for all patients diagnosed with PBC to restore their liver function to as close to normal as possible. For patients who had inadequate response to or could not tolerate ursodeoxycholic acid the only option was best supportive care and consideration of liver transplant. Patients whose disease responded inadequately to ursodeoxycholic acid were likely to progress rapidly and die from the disease within 5 to 7 years. Obeticholic acid will now be an option for those who have responded inadequately to or cannot tolerate ursodeoxycholic acid.
3.	**Number of people in Northern Ireland expected to take up service/therapy** *(including new cases per year)* The following estimates were calculated using the NICE Resource Impact Template and Resource Impact Report that accompanies TA 443, available at https://www.nice.org.uk/guidance/ta443/resources. Note that the NICE estimates for proportions have been used in absence of any available evidence of variance from these figures for Northern Ireland. It is estimated that of the 1,417,588 adults in Northern Ireland *(NISRA 2015 mid-year population estimate):*

- 567 have Primary Biliary Cholangitis (diagnosed or undiagnosed)
- 295 have been diagnosed with Primary Biliary Cholangitis
- 271 will be receiving treatment
  - 68 (25%) of these will have an inadequate response to ursodeoxycholic acid
  - 20 (7.5%) of these cannot tolerate ursodeoxycholic acid

In summary, it is estimated that around 88 (68 + 20) people will be eligible for treatment with obeticholic acid each year.

From year 5 it is estimated that 52 people will have treatment with obeticholic acid each year, once uptake has reached 59%.

	2017/18	2018/19	2019/20	2020/21	2121/22
Number of people eligible for treatment	88	88	88	88	88
% uptake	9%	30%	41%	50%	59%
Number of people treated	8	27	36	44	52

The NICE resource impact template makes the following assumptions:

- 25% (midpoint of 20%-30%) of people will have inadequate response to ursodeoxycholic acid
- 7.5% (midpoint of 5%-10%) of people will not tolerate ursodeoxycholic acid
- People who respond to ursodeoxycholic acid are excluded from the template because they are unaffected by the recommendations
- Best supportive care has no cost in the resource impact template because it is assumed that all people have this.

Advice from local clinicians is that uptake of this treatment in Northern Ireland will be lower than NICE estimates.

4. **Patient Access Scheme Availability**

(<u>Yes</u>/No)

The company has agreed a patient access scheme with the Department of Health. This scheme provides a simple discount to the list price of obeticholic acid, with the discount applied at the point of purchase or invoice. The level of the discount is commercial in confidence. The Department of Health considered that this patient access scheme does not constitute an excessive administrative burden on the NHS.

5. **Costs** *(before PAS if applicable)*

5.1 **Drug cost per patient per annum (for new and prevalent cases)**

Obeticholic acid is given orally as follows: 5mg once daily starting dose, increasing to 10mg once daily after 6 months, based on tolerability, to achieve optimal response.

	The list price for 5mg or 10mg is £2,384.04per 30-tablet pack. In year 1, and subsequent years, the cost per patient = £2,384.04 x 12 = £28,608.48
5.2	**Total Drug Costs Per Annum** See table below
5.3	**Infrastructure costs Per annum** No infrastructure costs are anticipated in association with implementation of this TA.
6.	**Expected implementation period** There is no impediment to implementation of this guidance.
7.	**Commissioning arrangements** HSCB will commission this treatment in accordance with BASL guidelines (http://www.basl.org.uk/uploads/Advice%20for%20second%20line%20therapy%20for%20PBC.pdf). Given the uncertainty in terms of the numbers commencing treatment the drug will be commissioned on a cost per case basis. This will allow patient numbers to be identified and the level of uptake monitored.
8.	**Monitoring arrangements** The cost per case applications will be reviewed by SSCT on a quarterly basis to allow patients numbers to be identified and the level of uptake to be monitored.
9.	**DoH (NI) Legislative/Policy Caveats** This advice does not override or replace the individual responsibility of health professionals to make appropriate decisions in the circumstances of their individual patients, in consultation with the patient and/or guardian or carer. This would, for example, include situations where individual patients have other conditions or complications that need to be taken into account in determining whether the NICE guidance is fully appropriate in their case.

	2017/18	2018/19	2019/20	2020/21	2021/22
% uptake	9%	30%	41%	50%	59%
Number of people treated	8	26	36	44	52
Cost (before PAS discount)	228,868	743,820	1,029,905	1,258,773	1,487,352