Highly Specialised Technologies (HST) criteria checklist

Setmelanotide for obesity and hyperphagia in Bardet-Biedl syndrome [ID3947]

### Introduction

The NICE HST criteria checklist is to highlight where a technology meets/partially meets or does not meet the criteria for routing to the HST programme. Its purpose is to show the details of why a technology may not be appropriate for HST evaluation, but also where it has been identified as suitable. For more information, please see [section 7 of NICE health technology evaluation topic selection: the manual](https://www.nice.org.uk/process/pmg37/chapter/highly-specialised-technologies)

### Key – Please use the colour key to advise if the technology meets the criteria

|  |  |
| --- | --- |
| Met | There is clear and strong evidence that the criterion is met |
| Unclear | There is some evidence, or the evidence available is unclear that the criterion is met. |
| Not met | There is no evidence or limited evidence that the criterion is met. |

### MA wording: XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX

| **Number** | **Criterion** | **Description of how the technology meets the criteria** | **Does the technology meet the criteria?** |
| --- | --- | --- | --- |
|  | The condition is very rare defined by 1:50,000 in England | * Bardet-Biedl syndrome (BBS) results from a genetic mutation. Whilst exact prevalence data is unknown it is estimated and published that BBS has a prevalence of ~1 in 100,000 ([Bardet-Biedl Syndrome – BBSUK](https://bbsuk.org.uk/about-bbs-uk/bardet-biedl-syndrome/)). Other references refer to a prevalence of 1 in 140,000 to 1 in 160,000 ([Bardet-Biedl syndrome: MedlinePlus Genetics](https://medlineplus.gov/genetics/condition/bardet-biedl-syndrome/#inheritance)). * BBS UK registry notes 533 BBS patients in England. * As many as 90% of people with BBS have obesity and less will be eligible for the treatment (see criteria 2 for eligibility criteria). Only 2 centres for adults and 2 centres for children. | Met |
|  | Normally no more than 300 people in England are eligible for the technology in its licensed indication and no more than 500 across all its indications | * Updated information following workshop and January Topic Selection Oversight Panel (TSOP): * From the 4 clinics for BBS and BBS UK the number of patients with BBS in England is approximately 533 patients. Based on assumptions by the company that only 72-92% with obesity and 20% excluded because they would not have identifiable pathogenic variants, and narrowed further because license will only apply to those over 6 years of age (95% of patients) and 20% would be unsuitable because of chronic renal failure, the number of BBS patients eligible for setmelanotide is 234-298. * Across all indications: * Setmelanotide is currently being appraised as an HST for treating obesity caused by LEPR or POMC deficiency (ID3764) which has possibly 12-22 patients eligible for treatment. * Eligible patients less than the threshold of 300 for this indication, and eligible patients less than the threshold of 500 across all indications. | Met |
|  | The very rare condition significantly shortens life or severely impairs its quality | * This very rare condition significantly shortens life or severely impairs its quality. 3 or 4 primary clinical features: * Rod-cone dystrophy * Polydactyly * Obesity * Learning disabilities * Hypogonadism in males * Renal anomaliesAdd evidence and information about how the technology meets the criteria.   Hyperphagia   * Hyperphagia is an overwhelming, heightened, and relentless hunger, mimicking feelings of starvation, a longer time to reach satiety and shorter duration of satiety, severe preoccupation with food, persistent and potentially extreme food-seeking behaviours (e.g. night eating, stealing food, and eating non-food items) and distress or inappropriate behavioural response if denied food. * Hyperphagia negatively impacts quality of life – preoccupation with food dominates life and affects concentration, productivity and education and leads to obesity and a range of subsequent conditions impacting morbidity and mortality.   Obesity and high BMI   * Hyperphagia leads to excess energy intake and lifetime weight gain. * The association between high BMI and mortality has been well documented, compared with individuals of healthy weight (BMI 18·5–24·9 kg/m2), life expectancy from age 40 years was 4·2 years shorter in obese (BMI ≥30·0 kg/m2) men and 3·5 years shorter in obese women. * A high BMI also has a significant impact on morbidity; some examples include: long term morbidities such as CV disease and cancer; children can also develop sleep apnoea and they may have lung development and musculoskeletal problems both of which are disabling. In addition to the physical impact there is significant psychological impact on patients. * It is also important to note that the impact of obesity on metabolic syndrome is compounded in patients with BBS by an impaired renal function. Likewise the impact of obesity on motor performance and risk to physical health is compounded in patients with BBS by visual impairment. * Clinical feedback reports that the hyperphagia is debilitating both inside and outside the home and made more complex if sight loss and learning difficulties also co-exist. The obesity can further complicate existing renal dysfunction and the conditions have variable impact on different people. | Met |
|  | There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options. | * No current treatment options for hyperphagia and obesity in BBS. * There is no data supporting the use of orlistat or liraglutide in BBS patients or in any patient suffering from Rare Genetic Diseases of Obesity (RGDO). * Bariatric surgery is not recommended for RGDO patients and does not address the genetic impairment and resulting insatiable hunger and excess eating; it is also unsuitable for patients with cognitive impairment so these are not appropriate comparators. | Met |