A.22	Lanreotide and octreotide – gigantism and acromegaly – EML
Draft recommendation	☐ Recommended ☒ Not recommended Justification: The conditions indicated for the proposed medicine are extremely rare and for many cases, surgery is the first treatment option. The high cost of the treatment might not be accessible as well to countries with limited resources where the barrier to an experience neurosurgeon exists.
Does the proposed medicine address a relevant public health need?	☒ Yes ☐ No ☐ Not applicable Comments: The conditions indicated for the proposed medicine are extremely rare. However, the proposed medicine is considered the mainstay of therapy when the first option (surgery) is not possible.
Does adequate evidence exist for the efficacy/effectiveness of the medicine for the proposed indication? (this may be evidence included in the application, and/or additional evidence identified during the review process)	☒ Yes ☐ No ☐ Not applicable Comments: Mainstay of therapy when surgery or radiotherapy is contraindicated or not curative. No large-scale studies evaluating various therapeutic approaches to the treatment of GH excess in pediatric patients are available. SRLs (Octreotide, Lanreotide) injections, typically administered on a monthly basis, suppress growth hormone production in about 70 percent of patients. Significant tumor shrinkage occurs in 30 to 50 percent of patients. Injections often improve symptoms of soft tissue swelling, headache, joint pains and sleep apnea.
Does adequate evidence exist for the safety/harms associated with the proposed medicine? (this may be evidence included in the application, and/or additional evidence identified during the review process)	☒ Yes ☐ No ☐ Not applicable Comments: No large-scale studies evaluating various therapeutic approaches to the treatment of GH excess in pediatric patients are available. Used safely in children with both sporadic and syndromic gigantism for extended periods of time alone and in combination with dopamine analogues (bromocriptine and cabergoline).[1]

[1] Shah AS, Wilson DP. Genetic Disorders Causing Hypertriglyceridemia in Children and Adolescents. [Updated 2023 Feb 22]. In: Feingold KR, Anawalt B, Blackman MR, et al., editors. Endotext [Internet]. South Dartmouth (MA): MDText.com, Inc.; 2000-

Are there any adverse effects of concern, or that may require special monitoring?	☒ Yes ☐ No ☐ Not applicable Comments: Major side effect of all the somatostatin analogues is an increased risk of biliary sludge and gallstones after sustained use, necessitating periodic ultrasound examinations in patients treated long-term.[1]
Are there any special requirements for the safe, effective and appropriate use of the medicines? (e.g. laboratory diagnostic and/or monitoring tests, specialized training for health providers, etc)	☒ Yes ☐ No ☐ Not applicable Comments: The medical management is most often performed by endocrinologists. Periodic monitoring of GH and IGF-1 levels for dose adjustment.
Are there any issues regarding cost, cost-effectiveness, affordability and/or access for the medicine in different settings?	☒ Yes ☐ No ☐ Not applicable Comments: The cost is high; no generic is available, and the treatment is lifelong.
Are there any issues regarding the registration of the medicine by national regulatory authorities? (e.g. accelerated approval, lack of regulatory approval, off-label indication)	☐ Yes ☒ No ☐ Not applicable Comments: Approved by FDA, EMA, Australian Govt, Health Canada and Japan PMDA. Lanreotide is included in the EML of 14 countries[2] while octreotide is in the EML of 40+ countries (including some LMICs).
Is the proposed medicine recommended for use in a current WHO guideline? (refer to: https://www.who.int/publications/who-guidelines)	☐ Yes ☐ No ☒ Not applicable Comments: No WHO guideline exist for the condition.

[2] https://global.essentialmeds.org/dashboard/medicines/1026