[GUIDANCE IS OUTDATED AND HAS BEEN WITHDRAWN ON 2 JANUARY 2024.]

Somatropin

for the treatment of growth failure in children

Technology Guidance from the MOH Drug Advisory Committee

Guidance Recommendations

The Ministry of Health's Drug Advisory Committee has recommended:

- ✓ Somatropin (recombinant growth hormone) for the treatment of children with short stature due to conditions associated with growth failure.

Somatropin is not recommended for the treatment of idiopathic short stature.

Treatment with somatropin should always be initiated and monitored by a paediatric endocrinologist with specialist expertise in managing growth disorders in children.

Subsidy status

Somatropin (all preparations used at public healthcare institutions) is recommended for inclusion on the Medication Assistance Fund (MAF) in line with the abovementioned condition.

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Factors considered to inform the recommendations for subsidy

Technology evaluation

1.1 The MOH Drug Advisory Committee ("the Committee") considered the evidence presented for the technology evaluation of somatropin for the treatment of growth failure in children. The Agency for Care Effectiveness conducted the evaluation, in consultation with clinical experts from the public healthcare institutions. Published clinical and economic evidence relating to the use of somatropin in children with specific conditions associated with growth failure were considered in line with the indications requested in the application for evaluation (that is, growth hormone deficiency, small for gestational age with poor catch up growth, Turner syndrome, Prader-Willi syndrome and chronic renal failure). The use of somatropin in adults was outside the remit of this evaluation.

1.2 The evidence was used to inform the Committee's deliberations around four core decision-making criteria:

- Clinical need of patients and nature of the condition
- Clinical effectiveness and safety of the technology
- Cost-effectiveness (value for money) – the incremental benefit and cost of the technology compared to existing alternatives
- Estimated annual technology cost and the number of patients likely to benefit from the technology.

1.3 Additional factors, including social and value judgments, may also inform the Committee's subsidy considerations.

Clinical need

2.1 The Committee recognised that somatropin is currently the only pharmacologic option for the treatment of children with short stature due to conditions associated with growth failure.

Clinical effectiveness and safety

3.1 The Committee acknowledged that the use of somatropin in local practice was governed by international clinical guidelines. It agreed that somatropin is clinically effective compared to placebo in increasing height, height standard deviation score (HtSDS) or growth velocity for the conditions under evaluation.

3.2 The Committee acknowledged that Prader Willi syndrome is not a registered indication of somatropin in Singapore despite being included in the licence in most other countries. It noted the expert testimony that confirmed there is currently no suitable treatment alternatives for these patients and somatropin therefore represents established clinical practice.

3.3 The Committee noted the clinical experts' concern that somatropin should not be recommended for subsidy for idiopathic short stature (ISS) because the response to growth hormone is variable in these patients due to their heterogeneous endocrinologic profiles. It was also noted that patients with ISS who respond to growth hormone treatment may have only modest increases in linear growth.

3.4 The Committee expressed concern that the indication "small for gestational age with poor catch-up growth" may be subjectively determined unless diagnosed by experienced paediatric endocrinologists, potentially leading to increased use of somatropin in patients who may not benefit from treatment if it is subsidised. To address this concern, the Committee advised that the listing should stipulate that treatment can only be initiated and monitored by experienced paediatric endocrinologists with expertise in managing growth disorders in children.

Cost effectiveness

4.1 Published economic analyses from overseas show variable results due to study limitations and the lack of robust health-related quality of life data in children. However, the reported incremental cost-effectiveness ratios (ICERs) fall within a cost-effective range for each jurisdiction. In the UK, the ICERs were estimated to be less than £30,000 per quality adjusted life year (QALY) gained for each indication under evaluation. The Committee considered the overseas ICERs represented an acceptable use of healthcare resources when generalised to the local context, especially given the high unmet need that somatropin addresses.

Estimated annual technology cost

5.1 The Committee estimated that around 120 children in Singapore would benefit from Government assistance for somatropin. The cost impact was estimated to be less than $1 million per year in the near term.

Recommendation

6.1 The Committee recommended listing somatropin (all preparations used at public healthcare institutions) on the MAF for children with short stature due to conditions associated with growth failure on the basis of acceptable clinical and cost-effectiveness, and the high clinical need for this treatment in the absence of alternative treatment options.