Zorginstituut Nederland

Migalastat (Galafold®) for the treatment of Fabry's disease

Summary of recommendations by *Zorginstituut Nederland* dated 25 April 2017

Zorginstituut Nederland carried out an assessment of whether, based on the criteria of the Medicines Reimbursement System (GVS), the medicine migalastat (Galafold®) is interchangeable with any other product that is included in the GVS.

In a letter dated 8 August 2016 (CIBG-16-02756), the Minister of Health, Welfare and Sport asked *Zorginstituut Nederland* to assess whether the medicine migalastat (Galafold®) is interchangeable with any other product that is included in the GVS. With the advice of the Scientific Advisory Board (WAR), the Zorginstituut has now completed its assessment.

The manufacturer is asking for inclusion on List 1B of the Medicines Reimbursement system.

Registered indication

Migalastat is available as a hard capsule. Each capsule contains 123 mg migalastat (as hydrochloride).

Migalastat is indicated for the treatment of Fabry's Disease with a treatable α-galactosidase A (GLA) mutation in adults or children aged 16 years and older. The recommended dose schedule is 123 mg migalastat (1 capsule) every other day, at the same time of day.

Conclusion on interchangeability

The results of the pharmacotherapeutic report show that migalastat has a lower therapeutic value in comparison with the standard treatment, enzyme-replacement therapy (ERT), agalsidase α (Replagal®) and β (Fabrazyme®). This opinion is based on the fact that the research data are too limited to be able to conclude with sufficient certainty that the favourable effects of migalastat are comparable with those of ERT. As migalastat is intended as an oral alternative to ERT, its therapeutic value in relation to its favourable and unfavourable effects still requires further research.

As the product has a lower therapeutic value, migalastat is not eligible for inclusion in the GVS.

Conditional inclusion

Since 1 January 2012, the Minister of VWS can decide to include care in the insured package for a specific period even though it does not fulfil the statutory criterion 'established medical science and medical practice', on condition that during this period data are collected on the (cost-) effectiveness of the care. After this period of conditional eligibility, based on these data, it will be possible to determine whether the care can be accepted permanently into the insured package. For more information on the procedures, see the report 'Implementation procedures for conditional inclusion in the insured package'. Based on the primary and secondary criteria for conditional inclusion – insofar as these can be examined based on the data currently available – we examined whether migalastat is a possible candidate for conditional inclusion. Based on the data available on its effectiveness, migalastat does not

seem to be a promising candidate for conditional inclusion. Furthermore, there is also a formal argument, namely that no non-academic treatment centre can participate in the research. Our conclusion is, therefore, that migalastat is not a suitable candidate for conditional inclusion.

Future developments

Zorginstituut Nederland is of course prepared to reconsider the eligibility of migalastat for inclusion in the insured package, if supplementary research data result in scientific publications that the Zorginstituut has not already assessed.

For further information, please contact: JBoer@zinl.nl; warcg@zinl.nl

The original text of this excerpt from advice of Zorginstituut Nederland was in Dutch. Although great care was taken in translating the text from Dutch to English, the translation may nevertheless have resulted in discrepancies. Rights may only be derived on the basis of the Dutch version of Zorginstituut Nederland's advice.
Furthermore, Zorginstituut Nederland points out that only the summary of this report was translated. A proper understanding of all relevant considerations and facts would require familiarity with the Dutch version of this report, including all appendices.