REPORT 124
Taliglucerase alfa for the treatment of Gaucher disease

Ordinance n° 37/2014 published on 09/29/2014
EXECUTIVE SUMMARY

Technology: Taliglucerase alfa.

Indication: Types 1 and 3 Gaucher disease

Applicant: Departamento de Assistência Farmacêutica e Insumos Estratégicos [Department of Pharmaceutical Assistance and Strategic Inputs] – DAF/SCTIE

Context: Gaucher Disease (GD) is an autosomal recessive disease of the group of lysosomal storage diseases. It is caused by deficiency of the glucocerebrosidase enzyme, which aids in lipid metabolism, and results in glucocerebroside accumulation in macrophages, affecting body organs and tissues. Type 1 (non-neuronopathic form) accounts for 95% of GD cases and has an incidence of 1:10,000 to 1:20,000. Type 3 is the chronic neuronopathic form and affects children and adolescents; the described incidence is lower than 1:100,000.

Question: Is the use of taliglucerase alfa effective and safe in patients with types 1 and 3 Gaucher Disease?

Scientific evidence: The evidence currently available on the efficacy and safety of taliglucerase alfa for the treatment of GD is based on a randomized clinical trial conducted in treatment-naïve patients (age > 18 years) in which two taliglucerase alfa doses were compared during 9 months: 30 U/Kg body weight/infusion (n = 15) and 60 U/Kg body weight/infusion (n = 16). At the end of the study, there were statistically significant reductions in spleen and liver volumes in both treatment groups when compared to the values at the study start. There were no serious adverse events in patients from both treatment groups.

Budget Impact Assessment: Taliglucerase alfa is already made available by SUS. The publication of the Clinical Protocol and Therapeutic Guidelines for Gaucher Disease was in October, 2011; at the time, taliglucerase alfa was not yet registered in Brazil, but, to ensure that patients were not left without treatment due to imiglucerase supply crisis, its use was provided for in exceptional cases. Of the 643 patients with GD treated in SUS in 2013, 39 were treated with taliglucerase alfa. The negotiation for taliglucerase alfa purchase by the Ministry of Health used American dollars in the year 2013 and, therefore, the price paid using Brazilian real was dependent on the exchange rate available at the time of drug acquisition.

Recommendation made by CONITEC: CONITEC, at its 24th ordinary meeting held on April 9 and 10, 2014, recommended incorporating into SUS taliglucerase alfa as Enzyme Replacement Therapy for the treatment of adult patients with types 1 and 3 of Gaucher Disease. For updating the Clinical Protocol and Therapeutic Guidelines for Gaucher Disease, CONITEC also made the following considerations: 1) for new cases of Gaucher Disease, the ERT of first choice for the treatment of adult patients will be taliglucerase alfa, and imiglucerase
should be recommended for the treatment of pediatric patients; 2) for patients with Gaucher Disease who are already using Enzyme Replacement Therapy in SUS, the choice between maintaining or switching the drug will be at the doctor’s discretion, according to patient evolution.

**Public Consultation:** The public consultation was conducted between the period from 04/25/2014 and 05/16/2014. 19 contributions have been received, which were assessed by CONITEC.

**Final Deliberation:** CONITEC members attending the 27th plenary session meeting on 08/06/2014 unanimously deliberated in favor of recommending taliglucerase alfa as Enzyme Replacement Therapy for the treatment of adult patients with Gaucher Disease. For updating the Clinical Protocol and Therapeutic Guidelines for Gaucher Disease, CONITEC also kept the following considerations: 1) for new cases of Gaucher Disease, the ERT of first choice for the treatment of adult patients will be taliglucerase alfa, and imiglucerase should be recommended for the treatment of pediatric patients; 2) for patients with Gaucher Disease who are already using Enzyme Replacement Therapy in SUS, the choice between maintaining or switching the drug will be at the doctor’s discretion, according to patient evolution.