BACKGROUND INFORMATION ON THE PROCEDURE

1. Submission of the dossier

The applicant Genzyme Europe B.V. submitted on 1 December 2004 an application for Marketing Authorisation to the European Medicines Agency (EMEA) through the centralised procedure. The active ingredient, recombinant human acid alpha glucosidase was designated as an orphan medicinal product EU/3/01/018 on 14 February 2001 for the following indication: treatment of Glycogen Storage Disease type II (Pompe's disease). The calculated prevalence of this condition was 0.137 per 10.000 EU population.

Scientific Advice:
The applicant did not seek Scientific Advice at the CHMP.

Licensing status:
The product was not licensed in any country at the time of submission of the application.

The Rapporteur and Co-Rapporteur appointed by the CHMP were:
Rapporteur: B. Flamion Co-Rapporteur: E. Abadie

EMEA Product Team Leader: Outi Maki-Ikola

2. Steps taken for the assessment of the product

- The application was received by the EMEA on 1 December 2004.
- The procedure started on 20 December 2004.
- The Rapporteur's first Assessment Report was circulated to all CHMP members on 22 March 2005. The Co-Rapporteur's first Assessment Report was circulated to all CHMP members on 15 March 2005.
- During the meeting on 18 – 21 April 2005, the CHMP agreed on the consolidated List of Questions to be sent to the applicant. The final consolidated List of Questions was sent to the applicant on 22 April 2005.
- The applicant submitted the responses to the CHMP consolidated List of Questions on 8 September 2005.
- The summary report of the inspection carried out at the manufacturing site Allston MA, USA and Framingham MA, USA between 11-15 July 2005 was issued on 31 August 2005.
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the List of Questions to all CHMP members on 4 November 2005.
- During the CHMP meeting on 14-17 November 2005, the CHMP agreed on a list of outstanding issues to be addressed in writing and in an oral explanation by the applicant.
- The applicant submitted written explanations on the Outstanding issues on 21 November 2005.
- The Rapporteurs circulated the Joint Assessment Report on applicant's written explanations on the CHMP List of Outstanding Issues to the CHMP on 6 December 2006.
- During the CHMP meeting on 12-15 December 2005, outstanding issues were addressed by the applicant during an oral explanation before the CHMP. The applicant submitted additional written explanations on the Outstanding issues on 5 January 2006, upon request by the CHMP.
- The Rapporteurs circulated on 16 January 2006 the Joint Assessment Report on further data submitted to the CHMP following oral explanation.
- During the meeting on 23-26 January 2006, the CHMP, in the light of the overall data submitted and the scientific discussion within the Committee, issued a positive opinion for granting a Marketing Authorisation to Myozyme. The applicant provided the letter of undertaking on the follow-up measures to be fulfilled post-authorisation on 26 January 2006.
- The CHMP opinions were forwarded, in all official languages of the European Union, to the European Commission, which adopted the corresponding Decisions on 29 March 2006.

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