BACKGROUND INFORMATION ON THE PROCEDURE

1. Submission of the dossier

The applicant Shire Human Genetic Therapies AB submitted on 1 December 2005 an application for Marketing Authorisation to the European Medicines Agency (EMEA) for Elaprase, through the centralised procedure falling within the Article 3(1) and point 1 of Annex of Regulation (EC) No 726/2004.

The legal basis for this application refers to Article 8.3 of Directive 2001/83/EC, as amended - complete and independent application.

Elaprase was designated as an orphan medicinal product EU/3/01/078 on 11 December 2001 in the following indication: treatment of Mucopolysaccharidosis, type II (Hunter syndrome). The calculated prevalence of this condition was 0.02 per 100,000 EU population.

The applicant applied for the following indication: Long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II).

The applicant received Protocol Assistance from the CHMP on 20 February 2003 and 29 July 2004. The Protocol Assistance pertained to the clinical development programme and issues concerning quality and pre-clinical development of the dossier.

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 and their evaluation teams were:

Rapporteur: Ian Hudson (UK) Co-Rapporteur: Frits Lekkerker (NL)

Steps taken for the assessment of the product

- The application was received by the EMEA on 1 December 2005.
- The procedure started on 28 December 2005.
- The Rapporteur's first Assessment Report was circulated to all CHMP members on 17 March 2006. The Co-Rapporteur's first Assessment Report was circulated to all CHMP members on 20 March 2006. In accordance with Article 6(3) of Regulation (RC) No 726/2004, the Rapporteur and Co-Rapporteur declared that they had completed their assessment report in less than 80 days.
- During the meeting on 24-27 April 2006, 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 27 April 2006.
- The applicant submitted the responses to the CHMP consolidated List of Questions on 19 May 2006.
- The summary reports of the inspections carried out at the following sites:
 - Baxter Pharmaceutical Solutions LLC P.O. Box 3068. 927 South Curry Pike -Bloomington, IN between 30 January 2006 and 03 February 2006 was issued on 30 August 2006.
 - Shire (TK3) 205 Alewife Brook Parkway Cambridge, MA USA, Shire (TK8 facility) - 300 Main Street - Cambridge – USA and Shire (TK9 facility) - 33 Brighton Street - Belmont, MA – USA; between the 16–17 August 2006, was issued on 12 September 2006.
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the List of Questions to all CHMP members on 7 July 2006.
- During the CHMP meeting on 24-27 July, the CHMP agreed on a list of outstanding issues to be addressed in writing by the applicant.
- The applicant submitted the responses to the list of outstanding issues on 31 August 2006.

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- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the list of outstanding issues to all CHMP members on 29 September 2006.
- During the meeting on 16-18 October 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 under exceptional circumstances to Elaprase on 18 October 2006. The applicant provided the letter of undertaking on the specific obligations and follow-up measures to be fulfilled post-authorisation on 17 October 2006.
- The CHMP opinions were forwarded, in all official languages of the European Union, to the European Commission, which adopted the corresponding Decisions on 8 January 2007.

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