BACKGROUND INFORMATION ON THE PROCEDURE

1. Submission of the dossier

The company Genzyme Europe B.V. submitted on 4 March 2002 an application for Marketing Authorisation to the European Agency for the Evaluation of Medicinal Products (EMEA) through the centralised procedure for Aldurazyme, which was designated as an orphan medicinal product (EU/3/01/022) on 14 February 2001.

The Rapporteur and Co-Rapporteur appointed by the CPMP and the evaluation teams were:

Rapporteur: Dr I. Hudson Co-Rapporteur: Dr E. Abadie

Orphan Drugs:

Aldurazyme (laronidase) was designated as an orphan medicinal product in the following indication: treatment of Mucopolysaccharidosis, type I.

Scientific Advice:

The company did not seek scientific advice at the CPMP.

Licensing status:

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

2. Steps taken for the assessment of the product

- The procedure started on 26 March 2002.
- The Rapporteur's first Assessment Report was circulated to all CPMP members on 6 June 2002. The Co-Rapporteur's first Assessment Report was circulated to all CPMP members on 10 June 2002.
- During the meeting on 23 – 25 July 2002 the CPMP agreed on the consolidated List of Questions to be sent to the company. The final consolidated List of Questions was sent to the company on 26 July 2002.
- The company submitted the responses to the CPMP consolidated List of Questions on 12 September 2002.
- The summary reports of the inspections carried out at the manufacturing sites were issued on 30 September 2002 (Hollister-Stier Laboratories LLC, Spokane, USA), 13 December 2002 (Genzyme Corp., Framingham, USA) and on 10 January 2003 (BioMarin Pharmaceutical Inc, Novato CA, USA and Genzyme Corporation, Allston, USA).
- The Rapporteur circulated the response Assessment Report on the company's responses to the List of Questions to all CPMP members on 23 October 2002.
- During the meeting on 19 – 21 November 2002 the CPMP adopted a list of outstanding issues to be addressed by the company in writing and if necessary during an oral explanation. The list of outstanding issues was sent to the company on 22 November 2002.
- The company provided written information on these outstanding issues to all CPMP members on 12 December 2002.
- The Rapporteur/Co-Rapporteurs' joint review on the company's responses to the list of outstanding issues was circulated to all CPMP members on 10 January 2003.
- In the margin of the CPMP meeting on 21 - 23 January 2003, an ad-hoc clinical expert meeting took place on 20 January 2003 and a report was adopted by the CPMP on 21 January 2003.

- During the CPMP meeting on 21-23 January 2003, it was decided that in the light of the outcome of the ad hoc expert meeting there was no longer any need for the company to address outstanding issues during an oral hearing before the CPMP.
- During the meeting on 18 – 20 February 2003 the CPMP, 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 Aldurazyme on 20 February 2003.
- The CPMP opinions were forwarded, in all official languages of the European Union, to the European Commission, which adopted the corresponding Decisions on 10 June 2003.