BACKGROUND INFORMATION ON THE PROCEDURE

Submission of the dossier

The applicant Orphan Europe S.A.R.L. submitted on 12 July 2005 an application for Marketing Authorisation to the European Medicines Agency (EMEA) through the centralised procedure for Cystadane, which was designated as an orphan medicinal product EU/3/01/45 on 9 July 2001. Cystadane was designated as an orphan medicinal product in the following indication: treatment of homocystinuria. The calculated prevalence of this condition was 1.65 per 100,000 EU population

The applicant applied for the following indication treatment of homocystinuria.

The legal basis for this application refers to:

Article 8.3 of Directive 2001/83/EC, as amended - complete and independent application

The application submitted is a complete dossier:
composed of administrative information, complete quality data, non-clinical and clinical data based on applicants' own tests and studies and bibliographic literature substituting/supporting certain tests or studies.

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

Licensing status:
Cystadane has been given a Marketing Authorisation in Australia on 01/10/1996, in USA on 25/10/1996, in Canada on 03/07/1998 and in Israel on 13/09/1998.

The Rapporteur and Co-Rapporteur appointed by the CHMP and the evaluation teams were:
Rapporteur: **Dr Harald Enzmann**
Co-Rapporteur: **Dr Ian Hudson**

Steps taken for the assessment of the product

- The application was received by the EMEA on 12 July 2005.
- The procedure started on 17 August 2005.
- The Rapporteur's first Assessment Report was circulated to all CHMP members on 27 October 2005. The Co-Rapporteur's first Assessment Report was circulated to all CHMP members on 27 October 2005.
- During the meeting on 14 December 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 15 December 2005.
- The applicant submitted the responses to the CHMP consolidated List of Questions on 31 August 2006 after extending the deadline for submitting their responses (extension agreed at the CHMP level).
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the List of Questions to all CHMP members on 20 October 2006.
- During the CHMP meeting on 16 November 2006, the CHMP agreed on a List of outstanding issues to be addressed in writing by the applicant.
- The applicant provided responses to the List of outstanding issues on 23 November 2006.
- During the meeting on 14 December 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 Cystadane. The applicant provided the letter of undertaking on the follow-up measures to be fulfilled post-authorisation on 14 December 2006.