BACKGROUND INFORMATION ON THE PROCEDURE

1. Submission of the dossier

The applicant Orphan Europe submitted on 5 October 2001 an application for Marketing Authorisation to the European Agency for the Evaluation of Medicinal Products (EMEA) through the centralised procedure for Carbaglu, which was designated as an orphan medicinal product EU/3/00/007 on 18 October 2000.

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

Rapporteur: Prof. Cristina Sampaio Co-Rapporteur: Dr. Pieter Neels

Orphan Drugs:

Carbaglu was designated as an orphan medicinal product on 18 October 2000, in the following indication: Treatment of N-acetylglutamate synthase (NAGS) deficiency.

Scientific Advice:

The applicant 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 22 October 2001.
- The Rapporteur's first Assessment Report was circulated to all CPMP members on 15 January 2002. The Co-Rapporteur's first Assessment Report was circulated to all CPMP members on 17 January 2002.
- During the meeting on 19 – 21 February 2002, the CPMP 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 21 February 2002.
- The applicant submitted the responses to the CPMP consolidated List of Questions on 11 July 2002.
- The joint Rapporteur/Co-Rapporteur Assessment Report on the applicant's responses to the List of Questions was circulated to all CPMP members on 3 September 2002.
- During the CPMP meeting on 17 – 19 September 2002, the CPMP adopted a list of outstanding issues to be addressed by the applicant in writing. The list of outstanding issues was sent to the applicant on 18 September 2002.
- The applicant submitted the responses to the list of outstanding issues on 1 October 2002.
- The applicant provided a letter of undertaking of the specific obligations and follow-up measures to be fulfilled post-authorisation related to quality, safety and efficacy aspects on 15 October 2002.
- During the meeting on 15-17 October 2002, 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 to Carbaglu on 17 October 2002.
- The CPMP opinions were forwarded, in all official languages of the European Union, to the European Commission, which adopted the corresponding Decisions on 24 January 2003.