BACKGROUND INFORMATION ON THE PROCEDURE

1. Submission of the dossier

The applicant Swedish Orphan International AB submitted on 06 June 2003 an application for Marketing Authorisation to the European Medicines Agency (EMEA) through the centralised procedure for Orfadin, which was designated as an orphan medicinal product.

The Rapporteur and Co-Rapporteur appointed by the CHMP were:
Rapporteur: Prof. Giuseppe Nisticó Co-Rapporteur: Dr Jens Ersbøll

Orphan Drugs:

An Orphan Drug status was granted on 29 December 2000 (EU/3/00/012) to Swedish Orphan International AB for use of nitisinone (Orfadin) in the following indication: Treatment of hereditary tyrosinemia type 1. The calculated prevalence of this condition was 0.1 per 10,000 EU population.

Scientific Advice:

The applicant did not seek scientific advice at the CHMP.

Licensing status:

Orfadin has been given a Marketing Authorisation in United States on 18 January 2002.

2 Steps taken for the assessment of the product

- The application was received by the EMEA on 06 June 2003.
- The procedure started on 21 July 2003.
- The Rapporteur's first Assessment Report was circulated to all CHMP members on 30 September 2003. The Co-Rapporteur's first Assessment Report was circulated to all CHMP members on 30 September 2003.
- During the meeting on 18-20 November 2003, 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 20 November 2003.
- The applicant submitted the responses to the CHMP consolidated List of Questions on 13 May 2004.
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the List of Questions to all CHMP members on 25 June 2004.
- During the CHMP meeting on 27-29 July 2004, the CHMP agreed on a list of outstanding issues to be addressed in writing by the applicant.
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the List of Outstanding Issues on 01 October 2004.
- The Rapporteurs circulated the revised Joint Assessment Report on 05 November 2004.
- During the meeting on 15-18 November 2004, 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 Orfadin on 18 November 2004. The applicant provided the letter of undertaking on the specific obligations and follow-up measures to be fulfilled post-authorisation on 18 November 2004.
- The CHMP opinions were forwarded, in all official languages of the European Union, to the European Commission, which adopted the corresponding Decisions on 21 February 2005.