

BACKGROUND INFORMATION ON THE PROCEDURE

1. Submission of the dossier

The applicant OTL Pharma S.A. submitted on 7 September 2005 an application for Marketing Authorisation to the European Medicines Agency (EMA) through the centralised procedure for Siklos, which was designated as an orphan medicinal product (EU/3/03/154) on 9 July 2003. Siklos was designated as an orphan medicinal product in the following indication: Treatment of sickle cell syndrome. The calculated prevalence of this condition was not more than 0.5 in 10,000 persons.

The applicant applied for the following indication: "Prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in paediatric and adult patients suffering from symptomatic Sickle Cell Syndrome".

Protocol Assistance:

The applicant received Protocol Assistance from the CHMP on 22 October 2003. The Protocol Assistance pertained to quality and clinical aspects of the dossier.

Licensing status:

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 the evaluation teams were:

Rapporteur:	P. Neels	Co-Rapporteur:	N. Drakoulis
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2 Steps taken for the assessment of the product

- The application was received by the EMA on 07 September 2005.
- The procedure started on 26 October 2005.
- The Rapporteur's first Assessment Report was circulated to all CHMP members on 05 January 2006. The Co-Rapporteur's first Assessment Report was circulated to all CHMP members on 05 January 2006.
- During the meeting on 23 February 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 23 February 2006.
- The applicant submitted the responses to the CHMP consolidated List of Questions on 8 August 2006.
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the CHMP List of Questions to all CHMP members on 26 September 2006 and 27 September 2006.
- Following the transfer of the Orphan Medicinal Product Designation from OTL Pharma to Addmedica SAS on 17 October 2006, the applicant was also changed to Addmedica SAS.
- During the CHMP meeting on 18 October 2006, the CHMP agreed on a first list of outstanding issues to be addressed in writing and/or in an oral explanation by the applicant.
- During the CHMP meeting on 16 November 2006, the CHMP agreed on a second list of outstanding issues to be addressed in writing and/or in an oral explanation by the applicant, and questions for the Quality Working Party.
- The applicant submitted the written responses to the first and second CHMP list of outstanding issues on 17 November 2006 and on 24 November 2006.
- During the Quality Working Party (QWP) meeting on 28 November 2006, the QWP discussed the outstanding quality issues, the applicant presented his views on these questions and the QWP adopted a report to the CHMP.
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the first and second list of outstanding issues provided by the applicant on 5 December 2006 and on 12 December 2006.

- During the CHMP meeting on 14 December 2006, the CHMP agreed on a third list of outstanding issues to be addressed in writing and/or in an oral explanation by the applicant.
- The applicant submitted the written responses to the third CHMP list of outstanding issues on 11 April 2007.
- The Rapporteurs circulated the Joint Assessment Report on the applicant's responses to the third list of outstanding issues provided by the applicant on 17 April 2007 and on 23 April 2007.
- During the meeting in April 2007, 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 Siklos on 26 April 2007. The applicant provided the letter of undertaking on the follow-up measures to be fulfilled post-authorisation on 25 April 2007.