The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

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PRESS RELEASE

21st Meeting of the Committee for Orphan Medicinal Products

The twenty-first meeting of the Committee for Orphan Medicinal Products (COMP) was held on 26 February 2002.

Mr. Andersen, European Organisation for Rare Disorders (EURORDIS), updated the Committee on the PARD I project, titled "Orphan Medicinal Products to the service of patients affected by rare diseases". The Committee congratulated EURORDIS on the success of this project, which has achieved a common understanding among patients groups for rare diseases in Europe and established a platform for addressing issues regarding orphan medicinal products.

Dr. Hoppu (COMP Member representing Finland) and Dr. Saint-Raymond (EMEA) presented the EU paediatric initiatives to the Committee. It was noted that 66% of the COMP opinions in 2001 concerned medical conditions that affect children.

The Committee discussed four applications for orphan designation. The sponsors of these applications will be asked to provide the Committee with further information prior to adopting opinions at its March meeting.

The COMP noted that one application for orphan medicinal product designation was withdrawn by a sponsor.

The European Commission granted eight positive decisions on orphan designation[1] since the last COMP meeting on 22-23 January 2002, see Annex I. The first negative decision on orphan medicinal product designation was adopted by the European Commission on 1 February 2002. It has not been established that the condition which was the subject of the negative decision, B-cell non-Hodgkin's lymphoma (NHL), affects not more than 5 in 10,000 persons in the Community and as such the designation criteria have not been meet.

The status of orphan designation procedures, as of 26 February 2002, is summarised in the table below:

Intent to file notified	*Applications submitted*	*Applications withdrawn*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*
46	166	34	98	1[2]	86

The next COMP meeting will be held on 25-26 March 2002.

1 Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products (http://pharmacos.eudra.org/F2/register/index.htm)

2 Of the five negative opinions adopted to date, one appeal is ongoing. Two applications have been subsequently withdrawn, one has resulted in a final negative decision and one has resulted in a final positive opinion following appeal.

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NOTE: This Press Release, together with other information about the work of the EMEA, may be found on the internet at the following location: http://www.emea.eu.int/

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Medicinal products Designated as Orphan Medicinal Products in February 2002

Active substance	Fumagillin
Sponsor	Sanofi-Synthelabo
Orphan Indication	Treatment of diarrhoea associated with intestinal microsporidial infection
Opinion receipt date	27/11/01
Date of Commission Decision	4/2/02

Active substance	Human engineered monoclonal antibody specific for Transforming Growth Factor β1
Sponsor	Genzyme Europe BV
Orphan Indication	Treatment of Systemic Sclerosis
Opinion receipt date	27/11/01
Date of Commission Decision	4/2/02

Active substance	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol and palmitic acid
Sponsor	Discovery Laboratories Inc
Orphan Indication	Treatment of Acute Lung Injury
Opinion receipt date	27/11/01
Date of Commission Decision	4/2/02

Active substance	2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine
Sponsor	ILEX Services Limited
Orphan Indication	Treatment of acute lymphoblastic leukaemia
Opinion receipt date	27/11/01
Date of Commission Decision	5/2/02

Active substance	Adenovirus-mediated *Herpes Simplex* Virus-thymidine kinase gene
Sponsor	Ark Therapeutics Limited
Orphan Indication	Treatment of high-grade glioma with subsequent use of ganciclovir sodium
Opinion receipt date	27/11/01
Date of Commission Decision	6/2/02

Active substance	Azacitidine
Sponsor	Pharmion Limited
Orphan Indication	Treatment of myelodysplastic syndromes
Opinion receipt date	27/11/01
Date of Commission Decision	6/2/02

Active substance	Eflornithine hydrochloride
Sponsor	ILEX Services Limited
Orphan Indication	Treatment of Familial Adenomatous Polyposis (FAP)
Opinion receipt date	7/1/02
Date of Commission Decision	19/2/02

Active substance	Colistimethate sodium
Sponsor	Pharmax Limited
Orphan Indication	Treatment of *Pseudomonas aeruginosa* lung infection (including colonisation) in cystic fibrosis
Opinion receipt date	7/1/02
Date of Commission Decision	19/2/02