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

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PRESS RELEASE

19th Meeting of the Committee for Orphan Medicinal Products

The nineteenth meeting of the Committee for Orphan Medicinal Products (COMP) was held on 17-18 December 2001.

During the meeting, Prof. Jørn Olsen from the Danish Epidemiology Science Centre, a key member of the COMP's ad-hoc group of experts on epidemiology, made a presentation on measuring the frequency of rare diseases.

Four positive opinions on the designation of orphan medicinal products, were adopted by the Committee, for the following conditions:

- Familial Adenomatous Polyposis (FAP)
- Glioma
- High-grade dysplasia in Barrett's Esophagus
- *Pseudomonas aeruginosa* lung infection (including colonisation) in cystic fibrosis

These opinions, will now be forwarded to the European Commission for the decision making process.

One negative opinion on orphan medicinal product designation was adopted. This opinion will be forwarded to the sponsor, who may submit detailed grounds for appeal within 90 days of receipt of the opinion.

Three oral explanations took place during the meeting. The COMP noted that two applications for orphan medicinal product designation were withdrawn by sponsors.

The European Commission granted eight decisions on orphan designation[1] since the last COMP meeting on 20-21 November 2001, see Annex I. The status of orphan designation procedures, as of 18 December 2001, 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Intent to file notified	*Applications submitted*	*Applications withdrawn*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*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	155	29	88	2[2]	78

The next COMP meeting will be held on 22-23 January 2002. This will be followed, on 24 January 2002, by an EMEA Workshop with Health Professionals and Academia on Orphan Medicinal Products.

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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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, two appeals are ongoing. Two applications have been subsequently withdrawn and one has resulted in a final positive opinion following appeal.

Medicinal products Designated as Orphan Medicinal Products in December 2001

Active substance	Stiripentol
Sponsor	Laboratoires BIOCODEX
Orphan Indication	Treatment of severe myoclonic epilepsy in infancy
Opinion receipt date	17/9/01
Date of Commission Decision	5/12/01

Active substance	Apomorphine (oromucosal use)
Sponsor	Orion Corporation
Orphan Indication	Treatment of off-periods in Parkinson´s disease not responding to other oral treatment
Opinion receipt date	17/9/01
Date of Commission Decision	5/12/01

Active substance	Recombinant human monoclonal antibody to hsp90
Sponsor	NeuTec Pharma plc
Orphan Indication	Treatment of invasive fungal infections
Opinion receipt date	17/9/01
Date of Commission Decision	5/12/01

Active substance	Halofuginone Hydrobromide
Sponsor	PPD Global Ltd
Orphan Indication	Treatment of Systemic Sclerosis
Opinion receipt date	5/11/01
Date of Commission Decision	11/12/01

Active substance	Octovalent *Pseudomonas aeruginosa* O-polysaccharide-toxin A conjugate vaccine
Sponsor	Orphan Europe
Orphan Indication	Prevention of *Pseudomonas aeruginosa* infections in patients with cystic fibrosis
Opinion receipt date	5/11/01
Date of Commission Decision	11/12/01

Active substance	Denileukin diftitox
Sponsor	Ligand Pharmaceuticals UK Limited
Orphan Indication	Treatment of cutaneous T-cell lymphoma
Opinion receipt date	5/11/01
Date of Commission Decision	11/12/01

Active substance	[gly^2] Recombinant human glucagon-like peptide
Sponsor	PRA International
Orphan Indication	Treatment of Short Bowel Syndrome
Opinion receipt date	5/11/01
Date of Commission Decision	11/12/01

Active substance	Iduronate-2-sulfatase
Sponsor	TKT UK Ltd.
Orphan Indication	Treatment of Mucopolysaccharidosis, type II (Hunter Syndrome).
Opinion receipt date	5/11/01
Date of Commission Decision	11/12/01