



28 March 2001
COMP/90/01

PRESS RELEASE

11th Meeting of the Committee for Orphan Medicinal Products

The Committee for Orphan Medicinal Products (COMP) held its eleventh meeting on 19-20 March 2001.

The Committee welcomed two new COMP members: Dr. Eric Abadie, Vice-Chairperson of the Committee for Proprietary Medicinal Products (CPMP), and Dr. David Lyons, CPMP member for Ireland. Dr. Abadie and Dr. Lyons are replacing Prof. Jean-Michel Alexandre and Dr. Mary Teeling as representatives of the EMEA on the Committee.

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

- Advanced idiopathic Parkinson's disease with severe motor fluctuations and not responding to oral treatment
- Angioedema caused by C1 inhibitor deficiency
- Multiple myeloma

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.

The COMP noted that five applications for orphan medicinal product designation were withdrawn by sponsors. Nine decisions on orphan designation have been granted by the European Commission¹ since the last COMP meeting in February 2001, see Annex I.

The status of orphan designation procedures, as of 20 March 2001, is summarised in the table below:

Year	<i>Intent to file Notified</i>	<i>Applications submitted</i>	<i>Applications withdrawn</i>	<i>Positive COMP Opinions</i>	<i>Negative COMP Opinions</i>	<i>Designations granted by Commission</i>
2000	31	72	3	26	-	14
2001	16	6	6	11	3	12
Total	47	78	9	37	3	26

The Committee appointed co-ordinators and experts for a number of upcoming applications.

An ad-hoc COMP Biotechnology Working Group (COMP-BWG) has been established to provide the COMP with advice on general and product specific biotechnological matters, which are raised in applications for orphan designation. The COMP-BWG, co-chaired by Prof. J-H Trouvin and Dr. H. Seeverens, was convened for the first time on 19 March 2001.

The 1st EMEA Workshop for Patient Organisations on Orphan Medicinal Products was held on 21 March 2001. Thirty-four representatives of patient organisations met with COMP members and EMEA staff. The Committee's achievements to date were discussed as well as proposals for future collaboration with Patients' Organisations. A Press Release from this Workshop (COMP/108/01) will be published on the EMEA's web-site.

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/register/orphreg.htm>)

The next COMP meeting will be held on 9-10 April 2001. The first EMEA Workshop with Industry on Orphan Medicinal Products will be held on 11 April 2001.

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
since the February 2001 Press Release**

Active substance	Recombinant human acid α -glucosidase
Sponsor	Genzyme
Orphan Indication	Treatment of Glycogen Storage Disease type II (Pompe's disease)
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01

Active substance	Bosentan
Sponsor	Actelion Pharmaceuticals France
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01

Active substance	Ibuprofen
Sponsor	Orphan Europe
Orphan Indication	Treatment of patent ductus arteriosus
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01

Active substance	Imatinib mesylate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of chronic myeloid leukaemia
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01

Active substance	Laronidase
Sponsor	Genzyme
Orphan Indication	Treatment of Mucopolysaccharidosis, type I
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01

Active substance	Pegvisomant
Sponsor	Pharmacia and Upjohn
Orphan Indication	Treatment of acromegaly
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01

Active substance	Ribavirin
Sponsor	ICN Pharmaceuticals
Orphan Indication	Treatment of Haemorrhagic Fever with Renal Syndrome
Opinion receipt date	3/1/01

Date of Commission Decision	14/2/01
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Active substance	N-acetylgalactosamine-4-sulfatase
Sponsor	ClinTrials Research Limited
Orphan Indication	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome)
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01

Active substance	L-Lysine-N-acetyl-L-cysteinate
Sponsor	SMB Technology S.A
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	3/1/01
Date of Commission Decision	14/2/01