9 February 2012
EMA/COMP/81554/2012
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

7-8 February 2012

The Committee for Orphan Medicinal Products held its 131st plenary meeting on 7-8 February.

Orphan medicinal product designation

The COMP adopted 7 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 11 November 2011 with an active review time of 90 days:

- Dipalmitoylphosphatidylcholine, 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, sodium salt, synthetic surfactant protein C analogue and synthetic surfactant protein B analogue for treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age; Chiesi Farmaceutici S.P.A.
- Genistein sodium salt dihydrate for treatment of mucopolysaccharidosis type III (Sanfilippo syndrome); Axcentua Pharmaceuticals AB.
- Melatonin for treatment of perinatal asphyxia; Dr Nicola J. Robertson.

For the following medicines the review began on 12 December 2011 with an active review time of 59 days:

- Adenovirus-associated viral vector serotype 2 containing the human *RPE65* gene for treatment of Leber's congenital amaurosis; Alan Boyd Consultants Ltd.
- Antisense oligonucleotide targeted to the *SMN2* gene for treatment of 5q spinal muscular atrophy; Isis USA Ltd.
- Linsitinib for treatment of adrenal cortical carcinoma; Astellas Pharma Europe B.V.
- Sodium thiosulfate for treatment of calciphylaxis; Aptiv Solutions (UK) Limited.

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

Public summaries of opinions will be available on the Agency's website following adoption of the respective decisions on orphan designation by the European Commission.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 5 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to adoption of the opinion.

Oral hearings

6 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 applications for orphan medicinal product designation were withdrawn.

Detailed information on the orphan designation procedure

An overview of orphan designation procedures since 2000 is provided in Annex 1.

No orphan designation[1] decisions have been given by the European Commission since the last COMP meeting.

Applications for marketing authorisation for orphan medicinal products

Details on the opinions for marketing authorisation for orphan medicinal products adopted by the Committee for Medicinal Products for Human Use (CHMP) can be found in the CHMP monthly report on the Agency's website.

Article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council

In line with its responsibility to review whether or not a designated orphan medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation, the COMP adopted 1 opinion recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal products:

- Signifor (pasireotide) for treatment of Cushing's disease; Novartis Europharm Limited.

Upcoming meetings

- The 132nd meeting of the COMP will be held on 7-8 March 2012.

Other matters

The main topics addressed during the meeting related to:

- 4 protocol assistance letters were discussed.

[1] Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products http://ec.europa.eu/enterprise/sectors/pharmaceuticals/documents/community-register/html/index_en.htm

Note

This monthly report, together with other information on the work of the European Medicines Agency, can be found on the Agency's website: www.ema.europa.eu

Contact our press officer

Monika Benstetter or Sabine Haubenreisser

Tel. +44 (0)20 7418 8427

E-mail: press@ema.europa.eu

Annex 1

Overview for orphan medicinal product designation procedure since 2000

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	Designations granted by Commission
2012	17	30	23 (77%)	7 (23%)	0 (0%)	11
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107
2010	174	176	123 (70%)	51 (29%)	2[2] (1%)	128
2009	164	137	113 (82%)	23 (17%)	1 (1%)	106
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88
2004	108	101	75 (74%)	22 (22%)	4 (4%)	72
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55
2002	80	76	43 (57%)	30 (39%)	3 (4%)	49
2001	83	92	64 (70%)	27 (29%)	1 (1%)	64
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14
Total	**1417**	**1354**	**984 (73%)**	**352 (26%)**	**18 (1%)**	**945**

[2] One more opinion was re-adopted in 2010 following the appeal to a negative opinion from 2009