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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

5-6 May 2010

The Committee for Orphan Medicinal Products held its 112th plenary meeting on 5-6 May 2010.

On 3-4 May the Agency celebrated the 10th anniversary of the implementation of the European Orphan Regulation. The meeting had participants from the European Parliament, the European Commission, international and European regulatory agencies, members of the Committee for Orphan Medicines (COMP), patient groups, health professionals, and pharmaceutical industry. The participants reviewed the impact of ten years of orphan medicines legislation and discussed future opportunities and challenges for orphan medicinal products. A press release from the conference will be published very shortly on the Agency website and some of the presentations will be made available.

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 all positive opinions the review began on 5 March 2010 with an active review time of 62 days:

- **(3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt** for treatment of ovarian cancer, Merck Sharp & Dohme Limited –
- **3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid** for treatment of cystic fibrosis, Voisin Consulting S.A.R.L.
- **Bosutinib** for treatment of chronic myeloid leukaemia, Wyeth Europa Limited
- **Dexamethasone (intravitreal implant)** for treatment of non-infectious uveitis affecting the posterior segment of the eye, Allergan Pharmaceuticals Ireland
- **Everolimus** for treatment of tuberous sclerosis, Novartis Europharm Limited
- **Midostaurin** for treatment of mastocytosis, Novartis Europharm Limited

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- **Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt** for treatment of ovarian cancer, Æterna Zentaris GmbH

Negative opinions

The COMP adopted 1 negative opinion recommending the refusal of the orphan medicinal product designation for the following medicine:

- Molgramostim for treatment of cystic fibrosis, Drugrecure Aps. The review began on 10 August 2009.

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 6 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

3 oral hearings took place.

Appeal

Currently no appeal procedures are ongoing.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that three 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 new decisions on orphan designation[1] have been given by the European Commission since the last COMP meeting.

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new community marketing authorisation application through the centralised procedure since the last COMP plenary meeting are normally provided together with this press release. Since the lat Committee meeting no new procedures for marketing authorisation for orphan designated products have started.

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.

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

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

No opinions were discussed under this procedure.

Upcoming meetings

- The 113th meeting of the COMP will be held on 1 to 3 June 2010.

Other matters

The main topics addressed during the meeting related to:

- The Committee adopted the topics to be included in the agenda of the upcoming informal COMP meeting to be held on 16-17 June 2010 in Barcelona.
- The Committee was informed about the Policy on scientific publication and representation for European Medicines Agency's scientific committees and their members.
- A discussion took place on the proposal for a job description of patient representatives in Agency committees. The members of the Committee will provide comments.
- Due to the high number of applications received the Committee will held a three day meeting in June.

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

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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
2010	54	66 (100%)	41 (62%)	23 (35%)	2 (3%)	29
2009	164	137 (100%)	113 (82%)	23 (17%)	1 (1%)	106
2008	119	118 (100%)	86 (73%)	31 (26%)	1 (1%)	73
2007	125	117 (100%)	97 (83%)	19 (16%)	1 (1%)	98
2006	104	103 (100%)	81 (79%)	20 (19%)	2 (2%)	80
2005	118	118 (100%)	88 (75%)	30 (25%)	0 (0%)	88
2004	108	101 (100%)	75 (74%)	22 (22%)	4 (4%)	72
2003	87	96 (100%)	54 (56%)	41 (43%)	1 (1%)	55
2002	80	76 (100%)	43 (57%)	30 (39%)	3 (4%)	49
2001	83	92 (100%)	64 (70%)	27 (29%)	1 (1%)	64
2000	72	32 (100%)	26 (81%)	6 (19%)	0 (0%)	14
Total[2]	**1114**	**1056 (100%)**	**768 (73%)**	**272 (26%)**	**16 (2%)**	**728**

[2] Revised final numbers