8 April 2011
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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

6-7 April 2011

The Committee for Orphan Medicinal Products held its 122nd plenary meeting on 6-7 April 2011.

During this meeting the Committee adopted three public reports on the recommendation for maintenance of orphan designation at the time of marketing authorisation. With these adoptions the Agency completes adoption of documents for the products for which in 2010 the Committee finalised such procedures. This is in compliance with the objective to increase transparency and awareness of the outcomes of reviews of orphan designation at the time of marketing authorisation. Since May 2010 the Agency has been applying this policy prospectively. The documents are published on the Agency's website, linked to the public summary of opinion on orphan designation and the European public assessment report (EPAR).

The Committee was informed of the upcoming procedures for review of the maintenance of orphan designation. Notably, currently 19 marketing authorisation procedures for orphan designated products are currently under review.

Orphan medicinal product designation

The COMP adopted 4 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 7 February 2011 with an active review time of 60 days:

- **Adeno-associated viral vector serotype 9 containing the human sulfamidase gene** for treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome), Laboratorios del Dr. Esteve, S.A.
- **Allogeneic T cells encoding an exogenous thymidine kinase gene** for treatment of acute lymphoblastic leukaemia, LTKFarma.

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- **Chimeric monoclonal antibody against GD2** for treatment of neuroblastoma, United Therapeutics Europe Ltd.
- **Genetically modified human adenovirus encoding human PH20 hyaluronidase** for treatment of pancreatic cancer, VCN Biosciences S.L.

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 3 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

Two oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 1 application for orphan medicinal product designation was withdrawn.

Detailed information on the orphan designation procedure

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

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new European Union marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 2.

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.

Upcoming meetings

- The 123rd meeting of the COMP will be held on 4-5 May 2011.

Other matters

The main topics addressed during the meeting related to:

- 5 Protocol Assistance letters were adopted.

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
2011	36	43	33 (77%)	10 (23%)	0 (0%)	18
2010	174	176	123 (70%)	51 (29%)	2[1] (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	**1270**	**1209**	**883 (73%)**	**310 (26%)**	**16 (1%)**	**845**

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

Annex 2

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the March 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Eculizumab	Soliris	Alexion Europe SAS	EU/3/09/653	Treatment of atypical haemolytic uremic syndrome
[gly2]-recombinant human glucagon-like peptide	Revestive	Nycomed Danmark APS	EU/3/01/077	Treatment of short bowel syndrome