EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3 February 2010
EMA/COMP/42611/2010 Corr.
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

2-3 February 2010

The Committee for Orphan Medicinal Products held its 109th meeting on 2-3 February 2010. The Committee celebrated the 15th anniversary of the European Medicines Agency, which was inaugurated on 26 January 1995.

In these 15 years the Agency has worked towards the promotion of human and animal health. In the field of rare diseases the Agency and its scientific committees have implemented the Orphan Drug Regulation which has resulted in more than 700 products designated as orphan medicines and more than 60 authorised orphan medicines. During this process a strong collaboration has been established with patient representatives, experts and other stakeholders.

Orphan medicinal product designation

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

For the following medicines the review began on 6 November 2009 with an active review time of 90 days:

- **Glyceryl tri-(4-phenylbutyrate)** treatment of argininosuccinic aciduria, Hyperion Therapeutics Limited.
- **Glyceryl tri-(4-phenylbutyrate)** treatment of citrullinaemia type 1, Hyperion Therapeutics Limited.
- **Glyceryl tri-(4-phenylbutyrate) t**reatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome)[1], Hyperion Therapeutics Limited.
- **Glyceryl tri-(4-phenylbutyrate)** treatment of ornithine carbamoyltransferase deficiency, Hyperion Therapeutics Limited.

[1] Revision of wording of indication.

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
An agency of the European Union

- **Glyceryl tri-(4-phenylbutyrate)** treatment of carbamoyl-phosphate synthase-1 deficiency, Hyperion Therapeutics Limited.
- **Glyceryl tri-(4-phenylbutyrate)** treatment of hyperargininaemia, Hyperion Therapeutics Limited.
- **Glyceryl tri-(4-phenylbutyrate)** treatment of citrullinaemia type 2[2], Hyperion Therapeutics Limited.
- **Pralatrexate** for treatment of cutaneous T-cell lymphoma, Choice Pharma Limited.
- **Raloxifene hydrochloride** for treatment of hereditary haemorrhagic telangiectasia, Consejo Superior de Investigaciones Cientificas (CSIC).

For the following medicines the review began on 4 December 2009 with an active review time of 62 days:

- **2-methoxymethyl-2-hydroxymethyl-1-azabicyclo [2,2,2] octan-3-one** for treatment of acute myeloid leukemia, Aprea AB.
- **Bafetinib** for treatment of chronic myeloid leukaemia, Eudax S.R.L.
- **Entinostat** for treatment of Hodgkin's lymphoma, Nexus Onclology Ltd.
- **Perifosine** for treatment of multiple myeloma, Æterna Zentaris GmbH.

Negative opinion

The COMP adopted, after appeal, one negative opinion recommending the refusal of the orphan medicinal product designation for the following medicine:

- **Gastrin 17C diphtheria toxoid conjugate** for treatment of pancreatic cancer, Aster Biopharmaceuticals Ltd.

Public summaries of opinion 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 five lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to adoption of the opinion.

In a new step towards increased transparency towards sponsors, the Agency has adopted the policy of sending the list of questions integrated in the draft summary report. This should help sponsors to have a better understanding of the origin and justification for the questions raised by the Committee.

Oral hearings

Six oral hearings took place.

[2] Correction of indication.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that five 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.

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 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.

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 one opinion recommending to the European Commission that the following orphan medicinal product be kept in the Community registry of orphan medicinal products:

- **Arzerra (ofatumumab)** for treatment of chronic lymphoid leukaemia, Glaxo Group Limited.

Upcoming meetings

- The 110th meeting of the COMP will be held on 2-3 March 2010.
- The agency will host a public meeting on the 10 years of the Orphan Regulation in Europe. The conference will be held on 3-4 May 2010. The announcement, registration details and preliminary agenda can be found at (http://www.ema.europa.eu/pdfs/conferenceflyers/Conference_announcement3-4May2010.pdf)

Other matters

The main topics addressed during the meeting related to:

- Discussion on the results from the survey of pharmaceutical companies to assess the sponsors' perceptions of the orphan designation procedure. The main findings of the survey were:
    - sponsors give high value to procedure and work of Committee and Agency;
    - there is a need to improve communication with sponsors at time of withdrawal of application and to investigate further reasons for unsatisfactory perception at time of presubmission meeting in a minority of sponsors;
    - due to the relatively low response rate the Agency will consider further actions to obtain more information.

- Discussion on the reflection paper on the further involvement of patients and consumers in the Agency's activities.
- Announcement of next internal Workshop on determination of prevalence of non-endemic infectious diseases in the Community.
- Three Protocol Assistance letters were adopted.
- Third European Rare Disease Day to be held on 28 February 2010 (http://www.rarediseaseday.org).

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 officers

Martin Harvey Allchurch or Monika Benstetter

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	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	Designations granted by Commission
2010	13	17	9	1	-
2009	164	113	23	1	106
2008	119	86	31	1	73
2007	125	97	19	1	98
2006	104	81	20	2	80
2005	118	88	30	0	88
2004	108	75	22	4	72
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14
Total	1073	744	258	15	699

ANNEX 2

Designated orphan medicinal products that have been subject of a new Community marketing authorisation application under the centralised procedure since the January 2010 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Tobramycin (inhalation powder)	TOBI Podhaler	Novartis Europharm Limited-UK	EU/3/03/140	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis