European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
JULY 2009 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its 103rd plenary meeting on 7-8 July 2009. The Committee welcomed the new appointed members; Ms. Lesley Greene, Ms. Birthe Byskov Holm and Ms Pauline Evers nominated by the European Commission to represent patients' organizations, Dr David Lyons nominated by the European Commission based on a recommendation from the Agency and Prof. Dainis Krieviņš as the new COMP member from Latvia.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 18 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the EMEA review began on 14 April 2009 with an active review time of 86 days.

- **Allogeneic *ex vivo* expanded umbilical cord blood cells** for treatment of myelodysplastic syndrome, from Teva Pharma GmbH
- **Allogeneic *ex vivo* expanded umbilical cord blood cells** for treatment of chronic myeloid leukaemia, from Teva Pharma GmbH
- **Low molecular weight dextran sulfate** for prevention of graft rejection during pancreatic islet transplantation, from TikoMed AB
- **Human tumour necrosis factor alfa-derived peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys** for treatment of acute lung injury, from Apeptico Forschung und Entwicklung GmbH
- **Human C1-inhibitor** for treatment of angioedema caused by C1 inhibitor deficiency, from ViroPharma SPRL

For the following medicines the EMEA review began on 5 June 2009 with an active review time of 34 days.

- **(-)-*trans*-3-(5,6-dihydro-4H-pyrrolo [3,2,1-ij] quinolin-1yl)-4(1H-indo-3-yl) pyrrolidine-2, 5-dione** for treatment of soft tissue sarcoma, from Gregory Fryer Associates Ltd
- **(S)-ethyl 2-amino-3-(4-(2-amino-6((R)-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate** for treatment of carcinoid tumours, from Lexicon Celtic Ltd
- **26 base single stranded phosphodiester DNA oligonucleotide** for treatment of acute myeloid leukaemia, from Antisoma Research Ltd
- **Adeno-associated viral vector containing modified U1 snRNA** for treatment of Duchenne muscular dystrophy, from Amsterdam Molecular Therapeutics BV
- **Eicosapentaenoic acid** for treatment of familial adenomatous polyposis, from SLA Pharma (UK)

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 74 18 86 13
E-mail: mail@emea.europa.eu http://www.emea.europa.eu

- **Expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue** for treatment of anal fistula, from Cellerix S.A
- **Pasireotide** for treatment of Cushing's disease, from Novartis Europharm Limited
- **Pasireotide** for treatment of acromegaly, from Novartis Europharm Limited
- **Pomalidomide** for treatment of multiple myeloma, from Celgene Europe Limited
- **Recombinant antibody construct against human CD30 and CD16A** for treatment of Hodgkin lymphoma, from Affimed Therapeutics AG
- **Sequence modified human recombinant factor VIIa** for treatment of hemophilia B, from Bayer Schering Pharma AG
- **Sequence modified human recombinant factor VIIa** for treatment of hemophilia A, from Bayer Schering Pharma AG
- **Recombinant human serum amyloid protein** for prevention of scarring post glaucoma filtration surgery, from RegPak BioPharma Consulting

Public summaries of opinion will be available on the EMEA website which the Agency updates 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 seven lists of questions on initial applications. These applications will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

Oral hearings

Four oral hearings took place.

Withdrawal of application for orphan medicinal product designation

The COMP noted that three of the 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**.

The list of medicinal products for which decisions on orphan designation[1] have been given by the European Commission since the last COMP plenary meeting is provided in **Annex 2**.

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 EMEA 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] 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/pharmaceuticals/index_en.htm)

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

- **Cayston** (Aztreonam lysinate inhalation use), from Gilead Sciences International Ltd, for the treatment of gram negative bacterial lung infection in cystic fibrosis

UPCOMING MEETINGS FOLLOWING THE JULY 2009 COMP PLENARY MEETING

- The next meeting of the COMP will be held on 1-2 September 2009.

OTHER MATTERS

The main topics addressed during the July 2009 COMP meeting related to:

- The appointment of Prof. Dainis Krieviņš as the new COMP member from Latvia.
- The appointment of Ms. Lesley Greene , Ms. Birthe Byskov Holm and Ms Pauline Evers nominated by the European Commission to represent patients' organizations, Dr David Lyons nominated by the European Commission based on a recommendation from the Agency .
- Discussion with industry representatives on the experiences and challenges in the development of products for orphan indications.

NOTE: This Monthly Report and other documents may be found on the internet at the following location: http://www.emea.europa.eu

For further information, please contact:
Martin Harvey Allchurch, EMEA press officer
Tel. (+44-20) 74 18 84 27
E-mail: press@emea.europa.eu

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
2009	80	66	9	1	52
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

MEDICINAL PRODUCTS GRANTED A COMMUNITY DESIGNATION AS ORPHAN MEDICINAL PRODUCT SINCE THE JUNE 2009 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	Chimeric-anti-interleukin-6 monoclonal antibody
Sponsor	Centocor B.V.
Orphan Indication	Treatment of multiple myeloma
COMP Opinion date	05/05/09
Orphan Designation date	12/06/09

Active substance	Desipramine chlorhydrate
Sponsor	Targeon SAS
Orphan Indication	Treatment of Rett syndrome
COMP Opinion date	05/05/09
Orphan Designation date	12/06/09

Active substance	Murine monoclonal antibody to GD2
Sponsor	United Therapeutics Europe Ltd
Orphan Indication	Treatment of neuroblastoma
COMP Opinion date	05/05/09
Orphan Designation date	12/06/09

Active substance	Octreotide chloride (lipid depot solution)
Sponsor	Camurus AB
Orphan Indication	Treatment of acromegaly
COMP Opinion date	05/05/09
Orphan Designation date	12/06/09

Active substance	Talampanel
Sponsor	Teva Pharma GmbH
Orphan Indication	Treatment of amyotrophic lateral sclerosis
COMP Opinion date	05/05/09
Orphan Designation date	12/06/09