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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
APRIL 2009 PLENARY MEETING
MONTHLY REPORT

100 MEETINGS WORKING FOR ORPHAN MEDICINAL PRODUCTS IN THE EU

The Committee for Orphan Medicinal Products (COMP) held its hundredth plenary meeting on 1-2 April 2009. After 100 meetings the dedicated work of the Committee has resulted in the adoption of more than 640 positive opinions out of more than 900 applications for orphan designation reviewed. The success rate of the applications is approximately 70% and the short evaluation time is 60 days for about 70% of the applications. These data illustrates the commitment of the Committee to working for the designation of medicines for rare diseases to stimulate their research and development. The Committee has received many applications for innovative products and has designated, amongst other, fusion proteins, monoclonal antibodies, cell and gene therapy products, tissue engineered products and oligonucleotides to be developed for the treatment of rare diseases.

End of the 3rd mandate for three COMP members

The Committee thanked warmly Prof. Magdaléna Kuželová (appointed member from Slovakia), Dr Greg Markey (appointed member from the United Kingdom) and Mr Yann Le Cam (appointed representative of Patient Organisation) who will leave the COMP, for their successful contribution to the work of the Committee.

The departure of Mr Yann Le Cam marks a milestone in the history of the Committee. Mr Le Cam was appointed as one of the first patient representatives by the European Commission in 2000. This was the first time patient representatives were represented as full members in an EMEA Scientific Committee. The result of this participation has been extremely fruitful. This initiated a collaboration which has brought benefits both to patients and regulators and has extended patients participation in other Committees. Both parties built mutual respect, worked on communication and learned from each others' needs and goals. Some of the initiatives that the COMP supported, such as the publication of the public summary of opinions on orphan designations or the emphasis on global collaboration in the field of rare diseases were initiated and supported by this partnership. Mr Le Cam exemplifies the enthusiasm and commitment that a patient representatives offer, in an altruistic manner, which benefit and advance public health in the European Union.

Presentation on the review of the ICD lcassification

The Committee welcomed Dr Ségolène Aymé from Orphanet and chair of the Rare Diseases Task Force and of the WHO Topic Advisory Group (TAG) for Rare Diseases. Dr Aymé was invited to give a presentation in the context of the revision of the ICD classification. Dr Aymé presented the advances that the TAG has already achieved and the future tasks of the group. The group is working on the introduction of new codes for the classification of rare diseases, which could have an unprecedented impact for those conditions, as they will be come visible in health information systems. The TAG is proposing thousands of new codes for rare diseases. The group is also reviewing the current classification system in order to make it more adaptable to the multisystemic reality of many rare diseases and revisiting the previous criteria for classification. The 'ICD-10 plus' version of the ICD classification, which will include already some of the new proposals for rare diseases, is made

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progressively accessible to the public for consultation on the WHO website. The alpha draft of the ICD-11 classification is expected for 2010 and the final version for 2013.

In the margins of the Committee meeting, collaboration and discussion continues between the FDA and the EMEA for the simplification of administrative processes for Orphan Drug Designation for the two jurisdictions.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 9 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 5 January 2009 with an active review time of 88 days.

- **2',3',5'-tri-O-acetyluridine,** from Wellstat Therapeutics EU Limited, for treatment of 5-fluorouracil overdose.
- **Humanised IgG_4 monoclonal antibody to the human toll-like receptor type 2,** from Opsona Therapeutics, for prevention of the ischemia/reperfusion injury associated with solid organ transplantation.
- **Dexamethasone phosphate (iontophoretic solution, ocular use),** from Voisin Consulting S.A.R.L., for treatment of corneal graft rejection.
- **S-[2,3-bispalmitoyloxy-(2*R*)-propyl]-cysteinyl-GNNDESNISFKEK,** from Mbiotec GmbH, for treatment of pancreatic cancer.

For the following medicines the EMEA review began on 6 February 2009 with an active review time of 56 days.

- **4,6,8-trihydroxy-10-(3,7,11-trimethyldodeca-2,6,10-trienyl)-5,10-dihydrodibenzo[*b,e*][1,4] diazepin-11-one ,** from Albany Regulatory Consulting Ltd, for treatment of glioma.
- **Alicaforsen,** from Atlantic Healthcare Limited, for treatment of pouchitis.
- **L-asparaginase encapsulated in erythrocytes,** from Erytech Pharma S.A., for treatment of pancreatic cancer.
- **Pegylated recombinant human factor IX,** from Novo Nordisk A/S, for treatment of haemophilia B.
- **Treprostinil diethanolamine,** from United Therapeutics Europe Ltd, for treatment of systemic sclerosis.

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.

All public summaries of opinion for already designated orphan designated medicinal products can be found on the EMEA website, using search options after the orphan condition of interest or after the name of the active substance.

OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

The COMP adopted one list of question on an initial application. This application will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

Oral hearings

Four oral hearings took place.

Withdrawals of application for orphan medicinal product designation

The COMP noted that one 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**.

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 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 3**.

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 one opinion recommending to the European Commission the removal of the following orphan medicinal product from the Community registry of orphan medicinal products:

- **Purified inactivated Japanese encephalitis SA14-4-2 virus vaccine (Ixiaro),** from Intercell AG, Austria, for the prevention of Japanese encephalitis.

UPCOMING MEETINGS FOLLOWING THE APRIL 2009 COMP PLENARY MEETING

- The 101th meeting of the COMP will be held on 5 May 2009.

OTHER MATTERS

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

- Discussion on the Informal COMP Meeting held on 9-10 March 2009 in Prague.
- Discussion on the COMP Work Programme 2009-2012
- Discussion on the ICD classification with the chair of the WHO Topic Advisory Group for Rare Diseases
- Discussion on the Pharmaceutical Forum-Delivering for Patients meeting held on 25 March 2009 in Brussels
- One Protocol Assistance letter was adopted for an orphan medicinal product.

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

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[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)

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	39	29	5	-	25
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 MARCH 2009 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	(6R)-4, 5, 6, 7-tetrahydro-N6-propyl-2, 6-benzothiazolediamine dihydrochloride monohydrate
Sponsor	Knopp Neurosciences Sub Ltd
Orphan Indication	Treatment of amyotrophic lateral sclerosis
COMP Opinion date	07/01/2009
Orphan Designation date	27/02/2009

Active substance	Allogeneic ex vivo expanded umbilical cord blood cells
Sponsor	Teva Pharma GmbH
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	07/01/2009
Orphan Designation date	27/02/2009

Active substance	Allogeneic ex vivo expanded umbilical cord blood cells
Sponsor	Teva Pharma GmbH
Orphan Indication	Treatment of acute lymphoblastic leukaemia
COMP Opinion date	07/01/2009
Orphan Designation date	27/02/2009

Active substance	N-terminal haxaglutamine-tagged recombinant N-acetylgalactosamine-6-sulfate sulfatase
Sponsor	Prof. Dr. Arndt Rolfs
Orphan Indication	Treatment of mucopolysaccharidosis, type IVA (morquio A Syndrome)
COMP Opinion date	07/01/2009
Orphan Designation date	27/02/2009

Active substance	Mifepristone
Sponsor	EXELGYN
Orphan Indication	Treatment of hypercortisolism (Cushing's syndrome)
COMP Opinion date	07/01/2009
Orphan Designation date	27/02/2009

Active substance	Tobramycin (inhalation use)
Sponsor	PARI Pharma GmbH

Orphan Indication	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis
COMP Opinion date	07/01/2009
Orphan Designation date	27/02/2009

Active substance	Exon 44 specific phosphorothioate oligonucleotide
Sponsor	Prosensa Therapeutics B.V.
Orphan Indication	Treatment of Duchenne muscular dystrophy
COMP Opinion date	05/11/2008
Orphan Designation date	27/02/2009

Active substance	Exon 51specific phosphorothioate oligonucleotide
Sponsor	Prosensa Therapeutics B.V.
Orphan Indication	Treatment of Duchenne muscular dystrophy
COMP Opinion date	05/11/2008
Orphan Designation date	27/02/2009

Active substance	2,2-dimethylbutyric acid, sodium salt
Sponsor	Isabelle Ramirez
Orphan Indication	Treatment of beta-thalassaemia intermedia and major
COMP Opinion date	07/01/2009
Orphan Designation date	27/02/2009

DESIGNATED ORPHAN MEDICINAL PRODUCTS THAT HAVE BEEN SUBJECT OF A NEW COMMUNITY MARKETING AUTHORISATION APPLICATION UNDER THE CENTRALISED PROCEDURE SINCE THE MARCH 2009 COMP MONTHLY REPORT

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Ethyl eicosopentaenoate	Ethyl Eicosopent soft Geltain Capsules	Amarin Neuroscience Ltd. UK	EU/3/00/013	treatment of Huntington's disease
Pazopanib hydrochloride	Patorma	Glaxo Group Limited UK	EU/3/06/382	treatment of renal cell carcinoma