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Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

November 2013

The Committee for Orphan Medicinal Products held its 150th plenary meeting on 5-6 November 2013.

Orphan medicinal product designation

Positive opinions

The COMP adopted 13 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission (EC).

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- **Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma** for treatment of glioma; ERC Belgium
- **Ibrutinib** for treatment of follicular lymphoma; Janssen-Cilag International N.V.
- ***Lactobacillus acidophilus* and *Bifidobacterium bifidum*** for prevention of necrotising enterocolitis; Laboratorio Farmaceutico S.I.T. s.r.l.
- **Recombinant human parathyroid hormone** for treatment of hypoparathyroidism; NPS Phama UK Ltd.
- **(4R,5R)-1-[[4-[[4-[3,3-dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy]methyl]phenyl]methyl]-4-aza-1-azoniabicyclo[2.2.2]octane chloride** for treatment of primary biliary cirrhosis; Lumena Pharma UK Limited
- **(4R,5R)-1-[[4-[[4-[3,3-dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy]methyl]phenyl]methyl]-4-aza-1-azoniabicyclo[2.2.2]octane chloride** for treatment of Alagille syndrome; Lumena Pharma UK Limited

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- **(4R,5R)-1-[[4-[[4-[3,3-dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy]methyl]phenyl]methyl]-4-aza-1-azoniabicyclo[2.2.2]octane chloride** for treatment of primary sclerosing cholangitis, Lumena Pharma UK Limited
- **(4R,5R)-1-[[4-[[4-[3,3-dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy]methyl]phenyl]methyl]-4-aza-1-azoniabicyclo[2.2.2]octane chloride** for treatment of progressive familial intrahepatic cholestasis; Lumena Pharma UK Limited.

2. Opinions adopted at the first COMP discussion:

- **Fenfluramine hydrochloride** for treatment of Dravet's[1] syndrome; Brabant Pharma Limited
- **Humanised monoclonal modified IgG4 antibody with bispecific structure targeting factors IX, IXa, X and Xa** for treatment of haemophilia A, Chugai Pharma Europe Ltd
- **Nitric oxide** for treatment of cystic fibrosis; Novoteris
- **Poly[2-[(4-{[1-carboxy-2-(hexadecylcarbamoyl)ethyl]sulfanyl}-2,3-bis({2-[((2S)-2-(2-{[(2R)-2-carbamoyl-(2-{[(2S)-1-ethoxy-3-(3-hydroxy-4oxo-1,4-dihydropyridin-1-yl)-1-oxopropan-2-yl]carbamoyl}ethyl]sulfanyl}-3-{[(2S)-1-ethoxy-3-(3-hydroxy-4-oxo-1,4-dihydropyridin-1-yl)-1-oxopropan-2-yl]carbamoyl}propanamido)-3-(3-hydroxy-4-oxo-1,4-dihydropyridin-1-yl)propanoyl ethyl ester))-methoxy]acetyl}oxy)butyl)sulfanyl]-3-(hexadecylcarbamoyl)propanoic acid]-PEG1500-ester]** for treatment of dengue[2]; Coté Orphan Consulting UK Limited
- **Recombinant human type I pancreatic elastase** for prevention of arteriovenous access dysfunction in haemodialysis patients; Proteon Therapeutics Limited.

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation[3] by the European Commission.

Lists of questions

The COMP adopted 17 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to the adoption of an opinion.

Oral hearings

9 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 applications for orphan medicinal product designations were withdrawn.

Detailed information on the orphan designation procedures

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

[1] Corrected indication
[2] Corrected indication
[3] Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products

No orphan designation decisions have been given by the European Commission since the last COMP meeting.

Applications for marketing authorisation for orphan medicinal products

No orphan medicinal products have been subject of a new European Union marketing authorisation application through the centralised procedure since the last COMP meeting.

Details on the authorised orphan medicinal products can be found on the EMA website.

Article 5(12) (b) 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 an opinion recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal products:

- **Opsumit** (Macitentan) for treatment of pulmonary arterial hypertension; Actelion Registration Ltd. (EU/3/11/909)

Other matters

The main topics addressed during the meeting related to:

- 3 protocol assistance (PA) requests were discussed and 3 PA letters were adopted.

Upcoming meetings

- The 151st meeting of the COMP will be held on 10-12 December 2013.

Note

This monthly report, together with other information on the work of the European Medicines Agency, can be found on the EMA 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	EC designations	Orphan medicinal products[4] authorised	Orphan designations included in authorised therapeutic indication
2013	181	175	123 (70%)	51 (29%)	1 (1%)	111	7	8
2012	197	192	139 (72%)	52 (27%)	1 (1%)	148	10	12
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4	4
2009	164	136	113 (83%)	23 (17%)	0[5] (0%)	106	9	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6	7
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9	11
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6	6
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55	5	5
2002	80	75	43 (57%)	30 (40%)	2[6] (3%)	49	4	4
2001	83	90	62[4] (70%)	27 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0	0
Total	**1778**	**1687**	**1221 (72%)**	**448 (27%)**	**18 (1%)**	**1194**	**85**	**91**

[4] Number of authorised orphan medicinal products may cover more than one orphan designation
[5] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[6] Following a quality assurance exercise it was identified that this figure needed correction