22 May 2017
EMA/COMP/265114/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

May 2017

The Committee for Orphan Medicinal Products held its 189th plenary meeting on 10-12 May 2017.

Orphan medicinal product designation

Positive opinion(s)

The COMP adopted 10 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

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

- Decitabine and tetrahydrouridine for treatment of sickle cell disease, Ulrich Muehlner;
- Ibutamoren mesilate for treatment of growth hormone deficiency, Richardson Associates Regulatory Affairs Ltd;
- Recombinant human factor IX protein modified with three point mutations for treatment of haemophilia B, Voisin Consulting S.A.R.L.;
- Sildenafil for treatment of congenital diaphragmatic hernia, Avivia Beheer BV;
- Sirolimus for treatment of tuberous sclerosis, Vale Pharmaceuticals Limited;
- Synthetic glucagon analogue modified to contain 7 amino acid substitutions for treatment of congenital hyperinsulinism, Zealand Pharma A/S;
- Tripotassium citrate monohydrate and potassium hydrogen carbonate for treatment of distal renal tubular acidosis, Advicenne Pharma SA.

2. Opinions adopted at the first COMP discussion:

- Asp-Arg-Val-Tyr-Ile-His-Pro for treatment of epidermolysis bullosa, Envigo Pharma Consulting Limited;

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

- Avacopan for treatment of C3 glomerulopathy, ChemoCentryx Limited;
- Pentamer formyl thiophene acetic acid for treatment of Creutzfeldt-Jakob disease, NeuroScios GmbH.

3. Opinions following appeal procedures

None

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation[1] by the European Commission. Please also refer to the Community Register of orphan medicinal products for human use.

Negative opinion(s)

The COMP did not adopt any negative opinions recommending the refusal of orphan medicinal product designations to the European Commission (EC).

Lists of questions

The COMP adopted 10 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

8 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 8 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

Detailed information on the orphan designation procedures

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

The list of medicinal products for which decisions on orphan designation have been granted by the European Commission since the last COMP meeting is provided in Annex 2.

Re-assessment of orphan designation at time of marketing authorisation

(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 3 opinions recommending to the European Commission that the following orphan medicinal product be kept in the Community Register of orphan medicinal products for human use:

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

1. Opinion(s) adopted at time of CHMP opinion

- Besponsa (inotuzumab ozogamicin) for treatment of B-cell acute lymphoblastic leukaemia, Pfizer Limited (EU/3/13/1127);
- Brineura (cerliponase alfa) for treatment of neuronal ceroid lipofuscinosis type 2, BioMarin International Limited (EU/3/13/1118). The opinion was adopted by written procedure after the April meeting;
- Spinraza (nusinersen) for treatment of 5q spinal muscular atrophy, Biogen Idec Ltd (EU/3/12/976). The opinion was adopted by written procedure after the April meeting.

2. Opinion(s) following appeal procedures

None

Details of the designated orphan medicinal products that have been subject of a new European Union (EU) marketing authorisation application since the last COMP monthly report are provided in Annex 3.

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

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 190th meeting of the COMP will be held on 13-15 June 2017.

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

Contact details of our press officer

Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: press@ema.europa.eu

Annex 1

Overview for orphan medicinal product designation procedure since 2000

Please also refer to the Community Register of orphan medicinal products for human use.

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn[2]	Final negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication[4]
2017	58	105	61 (58%)	43 (41%)	1 (1%)	63	5	5
2016	330	304	220 (72%)	82 (27%)	2 (1%)	209	14	14
2015	258	272	177 (65%)	94 (35%)	1 (1%)	190	14	21
2014	329	259	196 (76%)	62 (24%)	2 (1%)	187	15	16
2013	201	197	136 (69%)	60 (30%)	1 (1%)	136	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 (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%)	37 (40%)	1 (1%)	55	5	5
2002	80	75	43 (57%)	32 (42%)	2 (3%)	49	4	4

[2] Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000
[3] The number of orphan medicinal products authorised includes the products for which the market exclusivity has expired.
[4] The market authorisation of an orphan medicinal product may cover more than one orphan designation.

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	EC designations	Orphan medicinal products authorised	Orphan designations included in authorised therapeutic indication
2001	83	90	62 (70%)	26 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	3 (10%)	0 (0%)	14	0	0
Total	**2773**	**2644**	**1888 (71%)**	**732 (28%)**	**24 (1%)**	**1868**	**133**	**147**

Annex 2

Designations granted by the European Commission following COMP opinion on the fulfilment of the orphan designation criteria since last COMP plenary meeting

Please also refer to the Community Register of orphan medicinal product for human use.

The list includes designation decisions that were revised following the amendment of an existing designated condition (identified by * when applicable)

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Autologous adult bone marrow-derived non-expanded CD133+ haematopoietic stem cells	Treatment of Asherman's syndrome	Igenomix, S.L.	15 March 2017	20 April 2017
Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains	Treatment of multiple myeloma	Bluebird bio France	15 March 2017	20 April 2017
Emeramide	Prevention of mercury toxicity	NBMI Science Limited	15 March 2017	20 April 2017
Estetrol	Treatment of neonatal encephalopathy	Mithra Pharmaceuticals S.A.	15 March 2017	20 April 2017
Human normal immunoglobulin	Treatment in solid organ transplantation	Hôpital Foch	15 March 2017	20 April 2017
Modified messenger ribonucleic acid encoding human ornithine transcarbamylase enzyme encapsulated into lipid nanoparticles	Treatment of ornithine transcarbamylase deficiency	PhaseRx Ireland, Ltd	15 March 2017	20 April 2017
N-[(1R)-1-phenylethyl]-6-{1H-pyrazolo[3,4-d]pyrimidin-4-yl}quinazolin-2-amine	Treatment of fragile X syndrome	Sentinel Oncology Limited	15 March 2017	20 April 2017
Rituximab	Treatment in solid organ	Hôpital Foch	15 March 2017	20 April 2017

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
	transplantation			
(S)-8-{2-amino-6-[1-(5-chloro-biphenyl-2-yl)-(R)-2,2,2-trifluoro-ethoxy]-pyrimidin-4-yl}-2,8-diaza-spiro[4.5]decane-3-carboxylic acid ethyl ester	Treatment of pulmonary arterial hypertension	Biological Consulting Europe Ltd	15 March 2017	20 April 2017
Thymidine and deoxycytidine	Treatment of mitochondrial DNA depletion syndrome, myopathic form	Vall d'Hebron Institute of Research	15 March 2017	20 April 2017

Annex 3

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

Please also refer to the Community Register of orphan medicinal products for human use.

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Brentuximab vedotin	Treatment of cutaneous T-cell lymphoma	Takeda Pharma A/S	EU/3/11/939
Letermovir	Prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk	Merck Sharp & Dohme Limited	EU/3/11/849
Recombinant human beta-glucuronidase	Treatment of mucopolysaccharidosis type VII (Sly syndrome)	Ultragenyx Germany GmbH	EU/3/12/973