15 March 2018
EMA/COMP/145093/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

March 2018

The Committee for Orphan Medicinal Products held its 198th plenary meeting on 13-15 March 2018.

Orphan medicinal product designation

Positive opinions

The COMP adopted 7 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

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

- Autologous dendritic cells pulsed with killed ovarian cancer cells and matured by TLR3 ligand ex vivo for treatment of ovarian cancer, SOTIO a.s;
- Genetically modified replication-incompetent herpes simplex virus-1 expressing collagen VII for treatment of epidermolysis bullosa, IDEA Innovative Drug European Associates Limited;
- Polatuzumab vedotin for treatment of diffuse large B-cell lymphoma, Roche Registration Limited.

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 8 containing the human acid alpha-glucosidase gene for treatment of glycogen storage disease type II (Pompe's disease), Dr Philippe Moullier;
- Adeno-associated viral vector serotype 9 encoding miRNA against human superoxide dismutase 1 for treatment of amyotrophic lateral sclerosis, Stolmár & Partner Patentanwälte PartG mbB;
- Branaplam for treatment of spinal muscular atrophy, Novartis Europharm Limited;
- Burosumab for treatment of phosphaturic mesenchymal tumour, Ultragenyx Germany GmbH.

3. Opinion(s) following appeal procedures:

None

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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)

1. Opinion(s) adopted following the sponsor's response to the COMP list of questions:

None

2. Opinion(s) following appeal procedures:

- Melatonin for treatment of subarachnoid haemorrhage, Therapicon Srl.

Lists of questions

The COMP adopted 9 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 10 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)

When a designated orphan medicinal product receives a positive opinion for marketing authorisation from EMA's Committee for Medicinal Products for Human Use (CHMP), the COMP has the responsibility to review whether or not the medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation.

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

None

2. Opinion(s) following appeal procedures:

None

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

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 199th meeting of the COMP will be held on 17-19 April 2018.

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

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]	Negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication[4]
2018	34	72	42 (58%)	28 (39%)	2 (3%)	34	3	3
2017	260	245	144 (59%)	100 (41%)	2 (1%)	147	14	15
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

[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
2002	80	75	43 (57%)	32 (42%)	2 (3%)	49	4	4
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	**3009**	**2857**	**2013 (70%)**	**817 (29%)**	**27 (1%)**	**1986**	**145**	**160**

Annex 2

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

No new designations were granted by the European Commission since last COMP plenary meeting.

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
Glutamine	Treatment of sickle cell disease	Emmaus Medical Europe Limited	EU/3/12/1011
Trientine dihydrochloride	Treatment of Wilson's Disease	Univar BV	EU/3/03/172