16 October 2017
EMA/COMP/634206/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

03-05 October 2017

The Committee for Orphan Medicinal Products held its 193rd plenary meeting on 03-05 October 2017.

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:

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

- 1,4-diamino-2,3-dicyano-1,4-bis[2-aminophenylthio]butadiene for treatment of non-traumatic subarachnoid haemorrhage, Edvince AB;
- 1-[4-bromo-5-[1-ethyl-7-(methylamino)-2-oxo-1,2-dihydro-1,6-naphthyridin-3-yl]-2-fluorophenyl]-3-phenylurea for treatment of gastrointestinal stromal tumours, Worldwide Clinical Trials Limited;
- C1-esterase-inhibitor human for treatment in solid organ transplantation, CSL Behring GmbH;
- Concizumab for treatment of haemophilia B, Novo Nordisk A/S;
- Diazoxide choline for treatment of Prader-Willi syndrome, Capnia (UK) Ltd;
- N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide for treatment of peripheral T-cell lymphoma, Celleron Therapeutics Limited.

2. Opinions adopted at the first COMP discussion:

- (1'R,6'R)-3-(benzylamine)-6-hydroxy-3'-methyl-4-pentyl-6'-(prop-1-en-2-yl)-[1,1'-bi(cyclohexane)]-2',3,6-triene-2,5-dione for treatment of systemic sclerosis, Quintiles Ireland Limited;
- (R)-troloxamide quinone for treatment of amyotrophic lateral sclerosis, Edison Orphan Pharma BV;

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- 4-amino-1-[(1S,4R,5S)-2-fluoro-4,5-dihydroxy-3-(hydroxymethyl)cyclopent-2-en-1-yl]pyrimidin-2-one for treatment of pancreatic cancer, Quintiles Ireland Limited;
- Antisense oligonucleotide targeting exon 73 in the *COL7A1* gene for treatment of epidermolysis bullosa, ProQR Therapeutics VII BV;
- Recombinant adeno-associated viral vector serotype 9 containing human iduronate-2-sulfatase gene for treatment of mucopolysaccharidosis type II (Hunter's syndrome), REGENXBIO EU Limited;
- Tamoxifen citrate for treatment of Duchenne muscular dystrophy, Duchenne UK;
- Tiratricol for treatment of Allan-Herndon-Dudley syndrome, Medical Need Europe AB.

3. Opinion(s) 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)

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

The COMP adopted 1 negative opinion recommending the refusal of the orphan designation for the following product:

- Melatonin for treatment of subarachnoid haemorrhage, Therapicon Srl. The opinion was adopted by written procedure after the 03-05 October meeting.

2. Opinion(s) following appeal procedures:

None

Lists of questions

The COMP adopted 6 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 4 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.

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

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)

1. Opinion adopted at time of CHMP opinion:

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

- Zejula (niraparib), (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, for treatment of ovarian cancer, Tesaro UK Limited (EU/3/10/760)

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 194th meeting of the COMP will be held on 30-31 October 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

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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]	Final negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication[4]
2017	197	198	118 (60%)	79 (40%)	1 (1%)	106	12	13
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	**2912**	**2737**	**1945 (71%)**	**768 (28%)**	**24 (1%)**	**1911**	**140**	**155**

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
None				

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
Lenvatinib	Treatment of hepatocellular carcinoma	Eisai Ltd	EU/3/15/1460