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Procedure Management and Committees Support Division

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

December 2015

The Committee for Orphan Medicinal Products held its 173th plenary meeting on 8-10 December 2015.

Orphan medicinal product designation

Positive opinions

The COMP adopted 8 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:

- Live attenuated *Listeria monocytogenes* delta *actA*/delta *inlB* strain expressing human mesothelin for treatment of pancreatic cancer, Medpace Germany GmbH;
- Sodium benzoate for treatment of hyperargininaemia, Syri Limited;
- Sodium benzoate for treatment of arginosuccinic aciduria, Syri Limited;
- Two allogenic irradiated pancreatic tumour cell lines for treatment of pancreatic cancer, Medpace Germany GmbH.

2. Opinions adopted at the first COMP discussion:

- Entolimod for treatment of acute radiation syndrome, TMC Pharma Services Ltd;
- Live attenuated *Listeria monocytogenes* transfected with plasmids encoding the HPV-16E7 protein fused to a truncated fragment of the Lm protein listeriolysin O for treatment of anal cancer, Dr Ulrich Granzer;
- Synthetic double-stranded oligomer specific to the *SERPINA1* gene and containing a cholesterol-conjugated, acyclic nucleobase analogue for treatment of congenital alpha-1 antitrypsin deficiency, Pharma Gateway AB;

[1] Revised numbers in Annex 1

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- (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate for treatment of soft tissue sarcoma, TMC Pharma Services Ltd.

Lists of questions

The COMP adopted 12 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

7 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The COMP noted that 8 applications for orphan medicinal product designation were withdrawn.

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 given by the European Commission since the last COMP 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 European Union (EU) marketing authorisation application through the centralised procedure since the last COMP plenary meeting 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 174st meeting of the COMP will be held on 19-21 January 2016.

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 our press officer

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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[2] authorised	Orphan designations included in authorised therapeutic indication
2015	249	270	177 (66%)	92 (34%)	1 (1%)	174	14	21
2014	329	259	196 (76%)	61 (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%)	41 (43%)	1 (1%)	55	5	5
2002	80	75	43 (57%)	30 (40%)	2 (3%)	49	4	4
2001	83	90	62 (70%)	27 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0	0
Total	**2376**	**2238**	**1607 (72%)**	**610 (27%)**	**21 (1%)**	**1580**	**114**	**128**

[2] Number of authorised orphan medicinal products may cover more than one orphan designation

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the July 2015 COMP monthly report

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 November 2015 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Venetoclax	Treatment of chronic lymphocytic leukaemia	AbbVie Ltd.	EMA/OD/124/12