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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

April 2016

The Committee for Orphan Medicinal Products held its 177th plenary meeting on 19-21 April 2016.

Orphan medicinal product designation

Positive opinions

The COMP adopted 14 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:

- Arimoclomol citrate for treatment of inclusion body myositis, Orphazyme ApS;
- Fc- and CDR-modified humanised monoclonal antibody against C5 for treatment of paroxysmal nocturnal haemoglobinuria, Alexion Europe SAS.

2. Opinions adopted at the first COMP discussion:

- (R)-6-(2-fluorophenyl)-N-(3-(2-((2-methoxyethyl)amino)ethyl)phenyl)-5,6-dihydrobenzo[h]quinazolin-2-amine dihydrochloride for treatment of biliary tract cancer, Coté Orphan Consulting UK Limited;
- 4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide for treatment of retinitis pigmentosa, Shire Pharmaceuticals Ireland Limited;
- Autologous CD34+ cells transduced with lentiviral vector encoding the human beta globin gene for treatment of beta thalassaemia intermedia and major, Fondazione Telethon;
- H-Phe-Ser-Arg-Tyr-Ala-Arg-OH-acetate for treatment of amyotrophic lateral sclerosis, QRC Consultants Ltd;
- Pentosan polysulfate sodium for treatment of interstitial cystitis, Nextrasearch di Gasparetto Adolfo & C., Sas;

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- Polyethylene glycol-modified human recombinant truncated cystathionine beta-synthase for treatment of homocystinuria, Alan Boyd Consultants Ltd;
- Recombinant adeno-associated viral vector containing the human *RPGR* gene for treatment of retinitis pigmentosa caused by mutations in the *RPGR* gene, TMC Pharma Services Ltd;
- Rimiducid for treatment of graft-versus-host disease, QRC Consultants Ltd;
- Rovalpituzumab tesirine for treatment of small cell lung cancer, Aceso Biologics Consulting Ltd;
- Sodium nitrite and ethylenediaminetetraacetic acid for treatment of cystic fibrosis, Arch Bio Ireland Ltd;
- Temsirolimus for treatment of adrenoleukodystrophy, Centro de Investigación Biomédica en Red (CIBER);
- Vemurafenib for treatment of Langerhans' cell histiocytosis, Groupe d'étude des histiocytoses.

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.

Lists of questions

The COMP adopted 15 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

5 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 3 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.

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

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 products be kept in the EU registry of orphan medicinal product:

- Darzalex (daratumumab) for treatment of plasma cell myeloma, Janssen-Cilag International N.V. (EU/3/13/1153);
- Galafold (migalastat) for treatment of Fabry disease, Amicus Therapeutics UK Ltd (EU/3/06/368);
- Strimvelis (autologous cd34+ enriched cell fraction that contains cd34+ cells transduced with retroviral vector that encodes for the human ada cdna sequence) for treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency, GlaxoSmithKline Trading Services (EU/3/05/313).

The COMP adopted 1 opinion after appeal recommending to the European Commission that the following orphan medicinal product be removed from the EU registry of orphan medicinal product:

- Empliciti (elotuzumab) for treatment of multiple myeloma, Bristol-Myers Squibb (EU/3/12/1037).

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 178th meeting of the COMP will be held on 17-19 May 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

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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[2]	Final negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication
2016	83	85	63 (74%)	22 (26%)	0	40	2	2
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
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	**2468**	**2320**	**1670 (72%)**	**629 (27%)**	**21 (1%)**	**1636**	**116**	**130**

[2] Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000
[3] 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 March 2016 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 to a new European Union marketing authorisation application under the centralised procedure since the March 2016 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Olaratumab	Treatment of soft tissue sarcoma	Eli Lilly Nederland B.V.	EU/3/15/1447
Paclitaxel	Treatment of ovarian cancer	Oasmia Pharmaceutical AB	EU/3/06/422
Pentosan polysulfate sodium	Treatment of interstitial cystitis	Bene-Arzneimittel GmbH	EU/3/14/1411

Annex 4

COMP opinions on amendment of existing orphan drug designations since March 2016 COMP monthly report

Active substance	Initial orphan indication	Amended orphan indication	Sponsor/applicant	EU designation number
(S)-ethyl 2-amino-3-(4-(2-amino-6((R)-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate	Treatment of carcinoid tumours	Treatment of carcinoid syndrome	Ipsen Pharma	