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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

July 2017

The Committee for Orphan Medicinal Products held its 191th plenary meeting on 11-13 July 2017.

Orphan medicinal product designation

Positive opinions

The COMP adopted 16 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:

- Asunercept for treatment of myelodysplastic syndromes, Apogenix AG;
- Itraconazole for treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome), Mayne Pharma UK Limited;
- N-{2-[(6-{[(2,6-dichloro-3,5-dimethoxyphenyl)carbamoyl](methyl)amino}pyrimidin-4-yl)amino]-5-(4-ethylpiperazin-1-yl)phenyl}prop-2-enamide for treatment of hepatocellular carcinoma, Eisai Europe Limited;
- Odiparcil for treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome), Inventiva;
- Recombinant fragment of human surfactant protein-D for prevention of bronchopulmonary dysplasia, Trimunocor Ltd.

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype Anc80 containing the truncated human *ATP7B* gene under the control of the human alpha-1 antitrypsin promoter for treatment of Wilson's disease, Vivet Therapeutics SAS;

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

- Antisense oligonucleotide targeting exon 13 in the *USH2A* gene for treatment of retinitis pigmentosa, ProQR Therapeutics IV BV;
- Picropodophyllin for treatment of glioma, Axelar AB;
- Purified pasteurised and freeze-dried cell-wall fragments from *Mycobacterium tuberculosis* strain RUTI for treatment of tuberculosis, Archivel Farma S.L.;
- Recombinant adeno-associated viral vector serotype 5 carrying the gene for the human frataxin protein for treatment of Friedreich's ataxia, Voisin Consulting S.A.R.L.;
- Recombinant truncated N-terminal fragment of human lens epithelium-derived growth factor for treatment of retinitis pigmentosa, Dorian Regulatory Affairs BV;
- *Salmonella typhi* Ty21a strain transfected with a plasmid vector encoding the human vascular endothelial growth factor receptor 2 for treatment of glioma, Vaximm GmbH;
- Sirolimus for treatment of tuberous sclerosis, Best Regulatory Consulting Ltd;
- Sodium 2-hydroxylinoleate for treatment of pancreatic cancer, Ability Pharmaceuticals SL;
- Tacrolimus for treatment of pulmonary arterial hypertension, Vivus B.V.;
- Teicoplanin for treatment of cystic fibrosis, Neupharma S.r.l.

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)

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 13 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 7 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

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

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 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:

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

- Soliris (eculizumab) for treatment of myasthenia gravis, Alexion Europe SAS (EU/3/14/1304). The opinion was adopted by written procedure after the June meeting.

2. Opinion(s) following appeal procedures:

Following the appeal to the COMP opinion of 23 May 2017, the COMP adopted their final opinion recommending to the European Commission that the following orphan medicinal products be removed from the Community Register of orphan medicinal products for human use:

- Cuprior (trientine tetrahydrochloride) for treatment of Wilson's disease, GMP-Orphan SA, (EU/3/15/1471). The opinion was adopted by written procedure after the July meeting.

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 192th meeting of the COMP will be held on 05-07 September 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	135	149	86 (58%)	62 (42%)	1 (1%)	81	9	9
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	**2850**	**2688**	**1913 (71%)**	**751 (28%)**	**24 (1%)**	**1886**	**137**	**151**

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
Asp-Arg-Val-Tyr-Ile-His-Pro	Treatment of epidermolysis bullosa	Envigo Pharma Consulting Limited	12 May 2017	20 June 2016
Avacopan	Treatment of C3 glomerulopathy	ChemoCentryx Limited	12 May 2017	20 June 2016
Decitabine and tetrahydrouridine	Treatment of sickle cell disease	Ulrich Muehlner	12 May 2017	20 June 2016
Ibutamoren mesilate	Treatment of growth hormone deficiency	Richardson Associates Regulatory Affairs Ltd	12 May 2017	20 June 2016
Pentamer formyl thiophene acetic acid	Treatment of Creutzfeldt-Jakob disease	NeuroScios GmbH	12 May 2017	20 June 2016
Recombinant human factor IX protein modified with three point mutations	Treatment of haemophilia B	Voisin Consulting S.A.R.L.	12 May 2017	20 June 2016
Sildenafil	Treatment of congenital diaphragmatic hernia	Avivia Beheer BV	12 May 2017	20 June 2016
Sirolimus	Treatment of tuberous sclerosis	Vale Pharmaceuticals Limited	12 May 2017	20 June 2016
Synthetic glucagon analogue modified to contain 7 amino acid substitutions	Treatment of congenital hyperinsulinism	Zealand Pharma A/S	12 May 2017	20 June 2016
Tripotassium citrate monohydrate and potassium hydrogen carbonate	Treatment of distal renal tubular acidosis	Advicenne Pharma SA	12 May 2017	20 June 2016

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
Budesonide	Treatment of eosinophilic esophagitis	Dr. Falk Pharma GmbH	EU/3/13/1181
Vonicog alfa	Treatment of von Willebrand disease	Baxalta Innovations GmbH	EU/3/10/814