



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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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

June 2018

The Committee for Orphan Medicinal Products held its 201<sup>st</sup> plenary meeting on 19-21 June 2018.

### 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:

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

- Combination of carboplatin and sodium valproate for treatment of glioma, Dr Ulrich Granzer;
- Ex-vivo fused autologous human bone marrow-derived mesenchymal stem cell with allogenic human myoblast for treatment of Duchenne muscular dystrophy, Dystrogen Therapeutics S.A.;
- Synthetic antisense oligonucleotide directed against human dystrophin pre-mRNA for treatment of Duchenne muscular dystrophy, Wave life Sciences Ireland Limited;
- Tamibarotene for treatment of acute myeloid leukaemia, Lakeside Regulatory Consulting Services Ltd.

2. Opinions adopted at the first COMP discussion:

- 2'-O-(2-methoxyethyl) antisense oligonucleotide targeting microtubule-associated protein tau pre-mRNA for treatment of behavioural variant frontotemporal dementia, Ionis USA Ltd;
- Adenovirus associated viral vector serotype 2/8 containing the human *CNGA3* gene for treatment of achromatopsia, MeiraGTx UK II Limited;
- Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded, medac Gesellschaft für klinische Spezialpräparate mbH (WEDEL);



- Givinostat for treatment of Becker muscular dystrophy, Italfarmaco S.p.A.;
- Liposomal mannose-1-phosphate for treatment of phosphomannomutase-2 congenital disorder of glycosylation, Glycomine SARL;
- N-acetylgalactosamine-conjugated synthetic double-stranded oligomer specific to serpin family A member 1 gene for treatment of congenital alpha-1 antitrypsin deficiency, Pharma Gateway AB;
- Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 for treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 deficiency, Inozyme Pharma Ireland Ltd;
- Selumetinib for treatment of neurofibromatosis type 1, AstraZeneca AB;
- Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues for treatment of primary hyperoxaluria, Dicerna EU Limited;
- Tetracosactide for treatment of Duchenne muscular dystrophy, Mallinckrodt Specialty Pharmaceuticals Ireland Limited.

3. Opinion following appeal procedures:

None

Public summaries of opinions will be available on the [EMA website](#) following adoption of the respective decisions on orphan designation<sup>1</sup> by the European Commission. Please also refer to the Community Register of orphan medicinal products for human use.

## Negative opinion

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

None

2. Opinion following appeal procedures:

None

## 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

4 oral hearings took place.

## Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

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<sup>1</sup> 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)

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. Opinions adopted at time of CHMP opinion:

None

2. Opinion 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 202<sup>nd</sup> meeting of the COMP will be held on 17-19 July 2018.

### Note

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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](http://www.ema.europa.eu)

### Contact details of our press officer

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Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: [press@ema.europa.eu](mailto: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 <sup>2</sup>	Negative COMP opinions	EC designations	Orphan medicinal products <sup>3</sup> authorised	Orphan designations included in authorised therapeutic indication <sup>4</sup>
2018	99	131	83 (63%)	46 (35%)	2 (2%)	73	8	8
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

<sup>2</sup> Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000

<sup>3</sup> The number of orphan medicinal products authorised includes the products for which the market exclusivity has expired.

<sup>4</sup> 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
<b>Total</b>	<b>3074</b>	<b>2916</b>	<b>2054 (70%)</b>	<b>835 (29%)</b>	<b>27 (1%)</b>	<b>2025</b>	<b>150</b>	<b>165</b>

## 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
Edaravone	Treatment of amyotrophic lateral sclerosis	Mitsubishi Tanabe Pharma Europe Ltd	EU/3/15/1510