20 August 2019
EMA/PDCO/4352091/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the written procedure 20-23 August 2019

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 20-23 August 2019. See 20-23 August 2019 PDCO minutes (to be published post 17-20 September 2019 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 20-23 August 2019

1.3. Adoption of the minutes

PDCO minutes for 23-26 July 2019.

2. Opinions

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

2.1. Opinions on Products

2.1.1. Aprocitentan - EMEA-001978-PIP02-19

Hypertension

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. EMEA-002608-PIP01-19

Prostate cancer - diagnosis

Day 60 Opinion

Action: For adoption

Diagnostic / Oncology

2.1.3. Human fibrinogen - EMEA-001931-PIP02-19

Treatment of acquired fibrinogen deficiency

Day 60 Opinion

Action: For adoption

Haematology-Hemostaseology

2.1.4. EMEA-002424-PIP02-19

Lewy body dementia

Day 60 Opinion

Action: For adoption

Neurology

2.1.5. EMEA-002585-PIP01-19

Multiple myeloma

Day 60 Opinion

Action: For adoption

Oncology

2.1.6. Timolol / bimatoprost - EMEA-002583-PIP01-19

Ocular hypertension / Primary open-angle glaucoma

Day 60 Opinion

Action: For adoption

Ophthalmology

2.1.7. Emiplacel - EMEA-002539-PIP02-19

Treatment of muscle injury

Day 60 Opinion

Action: For adoption

Other

2.2. Opinions on Compliance Check

The following compliance checks have been put up for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance

2.2.1. Perampanel - EMEA-C6-000467-PIP01-08-M11

Eisai Europe Ltd; Treatment of treatment-resistant epilepsies

Day 60 letter

Action: For adoption

Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

No items

2.4. Opinions on Re-examinations

No items

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of opinions

No items

2.7. Partial Compliance Checks completed by EMA

For the following partial compliance checks, no need to refer them to PDCO Committee for discussion, were identified by the PME coordinator and PDCO Rapporteur. The PDCO has been informed in writing.

No items

2.7.1. Sofosbuvir / velpatasvir - EMEA-C1-001646-PIP01-14-M02

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C

Day 1 letter

Action: For information

Infectious Diseases

2.7.2. Ivacaftor / tezacaftor - EMEA-C2-001640-PIP01-14-M05

Vertex Pharmaceutical (Europe) Limited; Treatment of cystic fibrosis

Day 1 letter

Action: For information

Other

2.7.3. Ponatinib (as hydrochloride) - EMEA-C1-001186-PIP01-11-M02

Incyte Biosciences Distribution B.V.; Treatment of chronic myeloid leukaemia

Day 1 letter

Action: For information

Oncology

2.7.4. Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human arylsulfatase A (ARSA) cDNA sequence - EMEA-C3-001765-PIP02-15-M02

Orchard Therapeutics (Europe) Limited; Treatment of metachromatic leukodystrophy (MLD)

Day 1 letter

Action: For information

Other

2.7.5. Recombinant Influenza Hemagglutinin-strain B (Victoria lineage) / Recombinant Influenza Hemagglutinin-strain A (H1N1 subtype) / Recombinant Influenza Hemagglutinin-strain B (Yamagata lineage) / Recombinant Influenza Hemagglutinin-strain A (H3N2 subtype) - EMEA-C2-002418-PIP01-18-M01

Sanofi Pasteur; Prevention of influenza infection

Day 1 letter

Action: For information

Vaccines

3. Discussion of applications

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

3.1. Discussions on Products D90-D60-D30

3.1.1. Avatrombopag maleate - EMEA-001136-PIP02-19

Chemotherapy-induced thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in patients receiving myelosuppressive chemotherapy for solid tumours

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.2. Marzeptacog alfa (activated) - Orphan - EMEA-002270-PIP01-19

Voisin Consulting S.A.R.L; Treatment of haemophilia B

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.3. Marzeptacog alfa (activated) - EMEA-002270-PIP02-19

Treatment of haemophilia A

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. Allogeneic, non-expanded, umbilical cord blood-derived, haematopoietic mature myeloid and lymphoid cells (NF[1]) / Allogeneic, ex vivo expanded, umbilical cord blood-derived, haematopoietic CD34+progenitor cells (CF[2]) - Orphan - EMEA-001913-PIP02-18

Gamida Cell Ltd; Treatment in haematopoietic stem cell transplantation / Haematopoietic reconstitution of patients who are medically indicated for allogeneic haematopoietic stem cell transplantation

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.5. C1-esterase inhibitor human - Orphan - EMEA-002316-PIP03-19

CSL Behring GmbH; Treatment of antibody mediated rejection (AMR) in kidney

[1] Non cultured fraction
[2] Cultured fraction

transplantation

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.6. EMEA-002566-PIP01-19

Treatment of multiple sclerosis / Treatment of secondary progressive multiple sclerosis / Treatment of primary progressive multiple sclerosis / Treatment of relapsing remitting multiple sclerosis

Day 60 discussion

Action: For discussion

Neurology

3.1.7. Arimoclomol citrate - Orphan - EMEA-001748-PIP03-19

Orphazyme A/S; Treatment of amyloid lateral sclerosis

Day 60 discussion

Action: For discussion

Neurology

3.1.8. Cannabidiol - EMEA-001964-PIP02-19

Treatment of Rett syndrome

Day 60 discussion

Action: For discussion

Neurology

3.1.9. Dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19

EryDel S.p.A; Treatment of ataxia telangiectasia (AT) / Treatment of neurological symptoms in patients with AT

Day 60 discussion

Action: For discussion

Neurology

3.1.10. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19

argenx BVBA; Treatment of myasthenia gravis

Day 60 discussion

Action: For discussion

Neurology

3.1.11. Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain - Orphan - EMEA-002314-PIP01-17

DNAtrix, Inc.; High-Grade Glioma / Treatment of unresectable high-grade glioma in first recurrence, and diffuse intrinsic pontine glioma after failure of radiotherapy

Day 60 discussion

Action: For discussion

Oncology

3.1.12. Bintrafusp alfa - Orphan - EMEA-002586-PIP01-19

Merck Europe B.V.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 3 to <18 years of age with solid malignant tumors

Day 60 discussion

Action: For discussion

Oncology

3.1.13. Emixustat - EMEA-002581-PIP01-19

Stargardt disease

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.14. Timrepigene emparvovec - Orphan - EMEA-002430-PIP01-18

Nightstar Europa Limited; Treatment of choroideremia

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.15. Anti-neonatal Fc receptor human monoclonal antibody - EMEA-002559-PIP02-19

Myasthenia gravis

Day 60 discussion

Action: For discussion

Other

3.1.16. Adeno-associated viral vector serotype 8 containing the human MTM1 gene - Orphan - EMEA-002571-PIP01-19

Audentes Therapeutics, Inc.; X-linked myotubular myopathy (XLMTM)

Day 60 discussion

Action: For discussion

Other

3.1.17. 2-[[2-ethyl-6-[4-[2-(3-hydroxyazetidin-1-yl)-2-oxoethyl]piperazin-1-yl]-8-methylimidazo[1,2-a]pyridin-3-yl](methyl)amino]-4-(4-fluorophenyl)-1,3-thiazole-5-carbonitrile - Orphan - EMEA-002333-PIP02-19

Galapagos NV; Treatment of idiopathic pulmonary fibrosis / Treatment of interstitial lung disease with fibrosis in children

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.18. EMEA-002612-PIP01-19

Prevention of pulmonary dysfunction / Prevention of cardiopulmonary bypass (CPB) induced postoperative pulmonary dysfunction (PPD)

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.19. Benralizumab - EMEA-001214-PIP03-19

Treatment of vasculitides / Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.20. EMEA-002121-PIP03-19

Treatment of insomnia / Treatment of insomnia in children with comorbid

neurodevelopmental and psychiatric disorders

Day 60 discussion

Action: For discussion

Psychiatry

3.1.21. Dasotraline hydrocholride - EMEA-002590-PIP01-19

Binge eating disorder / Moderate to severe binge eating disorder

Day 60 discussion

Action: For discussion

Psychiatry

3.1.22. Ecopipam hydrochloride - EMEA-002564-PIP01-19

Tourette syndrome

Day 60 discussion

Action: For discussion

Psychiatry

3.1.23. EMEA-002589-PIP01-19

Schizophrenia / Treatment of schizophrenia

Day 60 discussion

Action: For discussion

Psychiatry

3.2. Discussions on Compliance Check

The following compliance checks have been put up for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance

3.2.1. Dabigatran etexilate mesilate - EMEA-C-000081-PIP01-07-M11

Boehringer Ingelheim International GmbH; Treatment of thromboembolic events

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.2.2. Sodium thiosulfate - EMEA-C-002147-PIP02-17

Fennec Pharmaceuticals, Inc.; Prevention of platinum-induced ototoxic hearing loss

Day 30 discussion

Action: For discussion

Oncology / Oto-rhino-laryngology

3.2.3. N. meningitidis serogroup A polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup W polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup C polysaccharide conjugated to tetanus toxoid - EMEA-C1-001930-PIP01-16-M01

Sanofi Pasteur; Prevention of meningococcal disease

Day 30 discussion

Action: For discussion

Vaccines

3.2.4. Caplacizumab- EMEA-C-001157-PIP01-11-M02

ABLYNX NV; Treatment of thrombotic thrombocytopenic purpura / Treatment of acquired thrombotic thrombocytopenic purpura

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

No items

4. Nominations

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

4.1. List of letters of intent received for submission of applications with start of procedure 17 September 2019 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver.

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

5.1. New Scientific Advice

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information

No items

5.2. Ongoing Scientific Advice

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information

No items

5.3. Final Scientific Advice (Reports and Scientific Advice letters)

Information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information

No items

6. Discussion on the applicability of class waivers

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Discussions on the applicability of class waiver for products

No items

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver

7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver

No items

8. Annual reports on deferrals

Note: The annual reports on deferrals to be noted by the members of the PDCO are flagged in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

No items

9.2. Coordination with EMA Scientific Committees or CMDh-v

No items

9.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

No items

9.4. Cooperation within the EU regulatory network

No items

9.5. Cooperation with International Regulators

No items

9.6. Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee

No items

9.7. PDCO work plan

No items

9.8. Planning and reporting

No items

10. Any other business

No items

11. Breakout sessions

No items

12. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Paediatric investigation plan (PIP) (section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs)
A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, when it is safe to do so, to support the authorisation of a medicine for children. Pharmaceutical companies submit proposals for PIPs to the European Medicines Agency's Paediatric Committee (PDCO). This Committee is responsible for agreeing or refusing the plan.

Compliance checks (section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check)
A compliance check may be necessary before any application for marketing authorisation (even for an adult indication) can be considered valid, if there was no deferral for at least one of the studies agreed in the PIP, or after the due date of initiation or completion of a study/measure. The same applies to some regulatory applications for authorised products, as described above.

Modification of an Agreed Paediatric Investigation Plan (section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP)
The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.
In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

Class waiver (section 6 Discussion on the applicability of class waiver)
As some diseases do not affect children (for example Parkinson's disease), the development of medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived**.** For more information on the classes of diseases subject to waivers, see class waivers.

Annual reports on deferrals (section 8)
If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/