29 May 2019
EMA/PDCO/239430/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes of the meeting on 23-26 April 2019

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

23 April 2019, 14:00- 19:30, room 2D

24 April 2019, 08:30- 19:30, room 2D

25 April 2019, 08:30- 19:30, room 2D

26 April 2019, 08:30- 13:00, room 2D

Disclaimers

Some of the information contained in this set of minutes 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).

Of note, this set of minutes is a working document primarily designed for PDCO members and the work the Committee undertakes.

Further information with relevant explanatory notes can be found at the end of this document.

Note on access to documents

Some documents mentioned in the minutes 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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Table of contents

1. Introductions

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

In accordance with the Agency's policy on handling of declarations of interests of scientific Committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and based on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some meeting participants in upcoming discussions as included in the pre-meeting list of participants and restrictions.

Participants in this meeting were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure and as included in the list of participants. All decisions taken at this meeting were made in the presence of a quorum of members (i.e. 23 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

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 23-26 April 2019. See 23-26 April 2019 PDCO minutes (to be published post 27-29 May 2019 PDCO meeting).

1.2. Adoption of agenda

The agenda for 23-26 April 2019 meeting was adopted and published on the EMA website.

1.3. Adoption of the minutes

The minutes of 26-29 March 2019 meeting were adopted and will be published on the EMA website.

2. Opinions

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. Adeno-associated viral vector serotype 5 containing a B domain deleted variant of human coagulation factor VIII gene - Orphan - EMEA-002427-PIP01-18

BioMarin International Limited; Treatment of patients with haemophilia A

Day 120 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

A positive Opinion was adopted at Day 120.
In conclusion, the PDCO recommends granting a paediatric investigation plan for children from birth to less than 18 years of age and a deferral for the treatment of congenital haemophilia A.

2.1.2. Dexamethasone - EMEA-002423-PIP01-18

Ocular Therapeutix, Inc.; ICD10 H59.9 Post-procedural disorder of eye and adnexa

Day 120 Opinion

Ophthalmology

Summary of committee discussion:

The PDCO concluded to grant a product specific waiver on own motion based on lack of significant therapeutic benefit over existing treatments.
Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees to granting the Applicant a waiver on own motion. The PDCO recommends granting a waiver for dexamethasone for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Treatment of postoperative pain and inflammation associated with ophthalmic surgery'.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.3. lentiviral vector containing the human ABCA4 gene for treatment of Stargardt's disease - Orphan - EMEA-002407-PIP01-18

Sanofi-Aventis Recherche & Développement; Treatment of inherited retinal disorders

Day 120 Opinion

Ophthalmology

Summary of committee discussion:

A positive Opinion was adopted on Day 120.

2.1.4. Ramipril / bisoprolol - EMEA-002531-PIP01-18

Midas Pharma GmbH; Treatment of chronic (systolic) heart failure (ICD10: I50.22) / Treatment of coronary artery disease (ICD10: I25-1) / Treatment of essential hypertension (ICD10: I10) / Treatment of hypertension with stable coronary artery disease and those with stable chronic heart failure with reduced systolic left ventricular function in adult patients adequately controlled with bisoprolol and ramipril given concurrently at the same dose level (substitution indication)

Day 60 Opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for ramipril / bisoprolol for all subsets of the paediatric population (0 to 18 years of age) in the conditions of treatment of hypertension, treatment of heart failure, and treatment of coronary artery disease on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.5. Sutimlimab - Orphan - EMEA-002542-PIP01-18

Bioverativ Inc; Treatment of primary cold agglutinin disease

Day 60 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the application and the additional information from the Applicant, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for sutimlimab for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Treatment of primary cold agglutinin disease'. The grounds are that the disease does not occur in the paediatric population.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the Applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.6. Abemaciclib - EMEA-002342-PIP03-18

Eli Lilly and Company Limited; Treatment of breast cancer

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the April 2019 plenary and confirmed all conclusions reached at Day 30.
Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for abemaciclib for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of Breast Cancer.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the Applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.7. Capivasertib - EMEA-002551-PIP01-18

AstraZeneca AB; Prostate cancer / breast cancer

Day 60 Opinion

Oncology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for capivasertib for all subsets of the paediatric population (0 to 18 years of age) in the conditions of 'treatment of prostate cancer' and 'treatment of breast cancer'.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the Applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.8. Tisotumab vedotin - EMEA-002522-PIP01-18

Genmab A/S; Treatment of cervical cancer

Day 60 Opinion

Oncology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for tisotumab vedotin for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Treatment of cervical cancer'.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the Applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.9. Glu-NH-CO-NH-Lys-(Ahx)-[N,N9-bis[2-hydroxy-5-(carboxyethyl) benzyl]ethylenediamine-N,N9-diacetic acid - EMEA-002503-PIP01-18

Trasis S.A.; Biochemical recurrence of prostate cancer

Day 60 Opinion

Oncology / Uro-nephrology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Visualisation of prostate specific membrane antigen in

prostate cancer'.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the Applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.10. Emiplacel - EMEA-002539-PIP01-18

Pluristem Ltd.; Treatment of peripheral ischaemia

Day 60 Opinion

Other

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for emiplacel for all subsets of the paediatric population (0 to less than 18 years of age) in the condition of 'treatment of peripheral ischaemia'. The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the Applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.11. 2-(2-(3-Butoxy-phenyl)-ethylamino)-N,N-dimethyl-acetamide hydrochloride - EMEA-002519-PIP02-18

Newron Pharmaceuticals SpA; Treatment of schizophrenia

Day 60 Opinion

Psychiatry

Summary of committee discussion:

During its April 2019 plenary meeting, the PDCO adopted a negative Opinion for this full product specific waiver request for evenamide for the treatment of schizophrenia.

2.1.12. Genetically modified replication-incompetent herpes simplex virus-1 expressing collagen VII - Orphan - EMEA-002472-PIP02-19

Krystal Biotech, Inc.; Dystrophic epidermolysis bullosa

Day 60 Opinion

Dermatology

Summary of committee discussion:

In conclusion, the Committee confirmed adoption of a negative Opinion at Day 60.

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. Emicizumab - EMEA-C-001839-PIP01-15

Roche Registration GmbH; Treatment of hereditary Factor VIII deficiency

Day 60 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO took note of the outcome of the preceding partial compliance check procedure:
• partial compliance procedure number EMEA-C1-001839-PIP01-15
The PDCO adopted on 26 April 2019 an Opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0196/2016) of 15 July 2016.

2.2.2. Turoctocog alfa - EMEA-C-000428-PIP01-08-M03

Novo Nordisk A/S; Treatment of hereditary Factor VIII deficiency

Day 60 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed this procedure at Day 60 during the April 2019 plenary meeting.
The PDCO took note of outcomes of preceding partial compliance check procedures:
• EMEA-C1-000428-PIP01-08-M01
The PDCO adopted on 26 April 2019 an Opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0207/2016) of 12 August 2016.

2.2.3. Split influenza virus, inactivated containing antigen equivalent to A/ California/7/2009(H1N1)-like strain (A/California/7/2009), adjuvanted - EMEA-C-000669-PIP01-09-M02

Sanofi Pasteur SA; Influenza

Day 60 Opinion

Vaccines

Summary of committee discussion:

All studies are compliant. A positive Opinion on the compliance check was adopted.

2.2.4. Anidulafungin - EMEA-C-000469-PIP01-08-M07

Pfizer Limited; Treatment of invasive candidiasis

Day 30 Opinion

Infectious Diseases

Summary of committee discussion:

The PDCO adopted on 26 April 2019 an Opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision P/0053/2017 of 17 March 2017.

2.2.5. Beclometasone dipropionate / formoterol fumarate dihydrate - EMEA-C-000548-PIP01-09-M08

Chiesi Farmaceutici S.p.A.; Maintenance therapy of asthma where use of a combination product (inhaled corticosteroid and long-acting beta2-agonist) is appropriate: patients not adequately controlled with inhaled corticosteroids and 'as needed' inhaled short-acting beta2-agonist or patients already adequately controlled on both inhaled corticosteroids and long-acting beta2-agonists in adults, as well as in children aged 5 to 11 years and in adolescents aged 12 to 17 years

Day 30 Opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:
- EMEA-C1-000548-PIP01-09-M02 (withdrawn)
- EMEA-C2-000548-PIP01-09-M03
- EMEA-C3-000548-PIP01-09-M04
- EMEA-C4-000548-PIP01-09-M05
- EMEA-C5-000548-PIP01-09-M06

The PDCO adopted on 26 April 2019 an Opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision P/0159/2018 of 15 June 2018.

2.2.6. Sofosbuvir / ledipasvir - EMEA-C-001411-PIP01-12-M04

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C

Day 30 Opinion

Infectious Diseases

Summary of committee discussion:

The PDCO adopted on 26 April 2019 an Opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0063/2017) of 16 March 2017.

2.2.7. Sofosbuvir - EMEA-C-001276-PIP01-12-M02

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C

Day 30 Opinion

Infectious Diseases

Summary of committee discussion:

The PDCO adopted on 26 April 2019 an Opinion confirming the compliance of all studies

in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0172/2018) of 15 June 2018.

2.2.8. N.meningitidis serogroup A polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup W135 polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup C polysaccharide conjugated to tetanus toxoid - EMEA-C-000429-PIP01-08-M04

Pfizer Europe MA EEIG; Prevention of meningococcal disease

Day 30 Opinion

Vaccines

Summary of committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:
• EMEA-C1-000429-PIP01-08
• EMEA-C2-000429-PIP01-08-M04
The PDCO adopted on 26 April 2019 an Opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0089/2015) of 8 May 2015).
The PDCO considers that the following studies completed at the time of initial PDCO review could be considered significant:
• Study 1
• Study 2
• Study 3
• Study 5

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Semaglutide - EMEA-001441-PIP02-15-M02

Novo Nordisk; Type 2 diabetes mellitus

Day 60 Opinion

sEndocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0206/2017 of 09/08/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Obeticholic Acid - Orphan - EMEA-001304-PIP02-13-M04

Intercept Pharma Ltd.; Primary biliary cholangitis (PBC) / Biliary atresia

Day 60 Opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The Applicant's responses to the Day 30 issues were considered acceptable. Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0213/2017 of 9 August 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Macrogol 3350 / sodium ascorbate / sodium sulfate / ascorbic acid / sodium chloride potassium chloride - EMEA-001705-PIP02-15-M02

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 60 Opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The Applicant provided further information regarding the product expected compatibility with feeding tubes, which were reviewed by members of the PDCO's Formulation Working Group (FWG).
Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0315/2018 of 12/09/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Rilpivirine (hydrochloride) - EMEA-000317-PIP01-08-M11

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type 1 (HIV-1) infection / Rilpivirine is indicated, in combination with other antiretroviral (ARV) medicinal products, for the treatment of human immunodeficiency virus type 1 (HIV-1) infection in ARV-naïve paediatric patients from 2 to less than 18 years with a baseline viral load below 100,000 HIV-1 RNA copies/mL.

Day 60 Opinion

Infectious Diseases

Summary of committee discussion:

In conclusion, based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0322/2018 of 12 September 2018).

The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Perampanel - EMEA-000467-PIP01-08-M11

Eisai Europe Limited; Treatment of treatment-resistant epilepsies / Adjunctive therapy in patients with other paediatric epilepsies - Adjunctive therapy in patients with refractory partial onset seizures including secondarily generalised seizures

Day 60 Opinion

Neurology

Summary of committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, namely the responses to the questions raised during the Day 30 discussion, which include a proposal for an extrapolation study, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0308/2018 of 12 September 2018).

2.3.6. Atezolizumab - EMEA-001638-PIP01-14-M02

Roche Registration GmbH; 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 birth to less than 18 years old with a PD-L1 positive paediatric solid tumour as part of the first line treatment

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO's views expressed at Day 30 were endorsed and based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
Furthermore, it was agreed to take the opportunity of this modification to align the description of the pharmaceutical form reported in the latest agreed decision with that one authorised (i.e. to replace solution for infusion with concentrate for solution for infusion).
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0220/2015 of 02/10/2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Lisocabtagene maraleucel - autologous CD4+ and CD8+ T cells Expressing a CD19-Specific Chimeric Antigen Receptor - Orphan - EMEA-001995-PIP01-16-M02

Celgene Europe B.V.; Treatment of B-lymphoblastic leukemia/lymphoma / Treatment of mature B-cell neoplasms / Treatment of paediatric patients with CD19+ relapsed or refractory B-cell acute lymphoblastic leukaemia / Treatment of paediatric patients with

CD19+ relapsed or refractory diffuse-large B-cell lymphoma, Burkitt lymphoma or primary mediastinal large B-cell lymphoma

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO discussed this procedure at Day 60 during the April 2019 plenary meeting. Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0119/2018 of 11 April 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Daratumumab - Orphan - EMEA-002152-PIP01-17-M01

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with a lymphoid malignancy (except mature B-cell neoplasms)

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0180/2018 of 19 June 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M04

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasm / Treatment of children from 1 year to less than 18 years of age with newly-diagnosed and relapsed/refractory mature B-cell lymphoma, that is, diffuse large B-cell lymphoma or Burkitt and Burkitt-like lymphoma

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO discussed the proposed modification. In conclusion, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0398/2017 of 19 December 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Isatuximab - Orphan - EMEA-002205-PIP01-17-M01

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of relapsed, refractory and newly-diagnosed acute lymphoblastic leukemia in combination with standard treatment in paediatric patients from 28 days to less than 18 years of age / Treatment of relapsed, refractory and newly-diagnosed acute myeloid leukemia in combination with standard treatment in paediatric patients from 28 days to less than 18 years of age

Day 60 Opinion

Oncology

Summary of committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0156/2018 of 15 June 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Lenvatinib - EMEA-001119-PIP02-12-M05

Eisai Europe Ltd; Treatment of papillary thyroid carcinoma / Treatment of osteosarcoma, Treatment of follicular thyroid carcinoma / Treatment of refractory or relapsed osteosarcoma in children and adolescents / Treatment of progressive, radioiodine-refractory differentiated thyroid cancer in children and adolescents

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO discussed the proposed modification. In conclusion, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0389/2018 of 07 December 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.
Note: Regarding the already authorised indication in adults for the treatment of hepatocellular carcinoma and for the treatment of renal cell carcinoma that were covered at the time of the submission of the marketing authorisation applications (MAAs) for those indications by the Decision CW/1/2011 granting the class-waiver, the Applicant is reminded that any future application falling within the scope of Article 8 of Regulation (EC) No 1901/2006 will require that all already approved and applied for indications (as well as pharmaceutical forms and routes of administration) are covered by a PIP or waiver.

2.3.12. Palbociclib - EMEA-002146-PIP01-17-M01

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma / Treatment of refractory or recurrent Ewing sarcoma

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO discussed the proposed modification for palbociclib. In conclusion, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0209/2018 of 17 July 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

Allergy Therapeutics (UK) Ltd; Allergic rhinitis and acute atopic conjunctivitis due to house dust mites / allergic rhinitis / allergic conjunctivitis

Day 60 Opinion

Pneumology - Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/205/2010 of 27 October 2010). The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Birch, Hazel and Alder Pollen Extract - EMEA-000808-PIP01-09-M01

Allergy Therapeutics (UK) Ltd; J.30.1 Allergic rhinitis due to pollen H10.1 Acute atopic conjunctivitis / Allergic rhinitis / Allergic conjunctivitis

Day 60 Opinion

Pneumology - Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/204/2010 of 27 October 2010). The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Ravulizumab - Orphan - EMEA-001943-PIP01-16-M02

Alexion Europe SAS; Atypical haemolytic uremic syndrome / Treatment of atypical haemolytic uremic syndrome

Day 60 Opinion

Uro-nephrology / Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed

paediatric investigation plan, the PDCO considered that some of the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0261/2018 of 15/08/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.4. Opinions on Re-examinations

2.4.1. Neisseria meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup W-135 polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup C polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M01

Sanofi Pasteur; Prevention of meningococcal disease

Day 30 Opinion

Vaccines

Summary of committee discussion:

The PDCO at its April 2019 meeting agreed on the re-examination proposal of the Applicant of the Opinion previously agreed in February 2019 based on the grounds provided that a misunderstanding had occurred in the number of patients to be recruited in Study 3.

The final Opinion was adopted on 26 April 2019.

2.4.2. Ofatumumab - EMEA-002397-PIP01-18

Novartis Europharm Limited; Treatment of multiple sclerosis / Treatment of relapsing remitting multiple sclerosis

Opinion

Neurology

Summary of committee discussion:

An oral explanation took place during the April PDCO plenary.

Based on the submitted re-examination grounds the PDCO decided to revise their Opinion in some points.

The final PDCO Opinion was adopted on 26 April 2019.

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.

2.7.1. Mometasone (furoate) / indacaterol (acetate) - EMEA-C2-001217-PIP01-11-M05

Novartis Europharm Limited.; Treatment of asthma

Day 30 letter

Pneumology - Allergology

Summary of committee discussion:

The partial compliance check has been finalised with a confirmation of compliance of all those studies contained in the agreed paediatric investigation plan that were subject of this procedure.

3. Discussion of applications

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. Emricasan - EMEA-002457-PIP01-18

Non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis (F2-F4) in patients aged 8 to less than 18 years old

Day 90 discussion

Gastroenterology-Hepatology

Non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis (F2/F3) in paediatric patients from 8 to less than 18 years of age

Day 90 discussion

Gastroenterology-Hepatology

3.1.3. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene - Orphan - EMEA-001665-PIP02-17

bluebird bio France; Sickle Cell Disease

Day 90 discussion

Haematology-Hemostaseology

3.1.4. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP01-18

Pfizer Europe MA EEIG; Treatment of congenital factor IX deficiency (haemophilia B) / Prophylaxis of haemophilia B (congenital factor IX deficiency)

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes - Orphan - EMEA-002025-PIP02-16

Atara Biotherapeutics, Inc.; Treatment of Epstein-Barr virus associated post-transplant lymphoproliferative disorder / Treatment of solid organ transplant (SOT) patients with Epstein-Barr virus associated post transplant lymphoproliferative disease (EBV+ PTLD), who have failed prior therapy with rituximab / Treatment of allogeneic haematopoietic cell transplant (alloHCT) patients with Epstein-Barr virus associated post transplant lymphoproliferative disease (EBV+ PTLD) who have failed prior therapy with rituximab

Day 90 discussion

Oncology

3.1.6. Rapastinel - EMEA-002357-PIP01-18

Major depressive disorder

Day 90 discussion

Psychiatry

3.1.7. EMEA-002310-PIP02-17

Treatment of C3 glomerulopathy

Day 90 discussion

Uro-nephrology

3.1.8. Human monoclonal IgG2 antibody against tissue factor pathway inhibitor - Orphan - EMEA-002498-PIP01-18

Bayer AG; Treatment of haemophilia A / Treatment of haemophilia B

Day 60 discussion

Haematology-Hemostaseology

3.1.9. Iclaprim mesylate - EMEA-002391-PIP02-19

Infection with gram-positive bacteria

Day 60 discussion

Infectious Diseases

3.1.10. 1-[4-bromo-5-[1-ethyl-7-(methylamino)-2-oxo-1,2-dihydro-1,6-naphthyridin-3-yl]-2-fluorophenyl]-3-phenylurea - Orphan - EMEA-002526-PIP02-19

Deciphera Pharmaceuticals LLC; Treatment of gastrointestinal stromal tumours

Day 60 discussion

Oncology

3.1.11. Trilaciclib - EMEA-002534-PIP02-19

Prevention of chemotherapy induced myelosuppression

Day 60 discussion

Oncology

3.1.12. Human immunoglobulin G2 isotype antibody to IL-33R - EMEA-002515-PIP01-18

Treatment of asthma / Add-on therapy for the maintenance treatment for moderate-severe asthma

Day 60 discussion

Pneumology - Allergology

3.1.13. Bisoprolol fumarate / ramipril - EMEA-002560-PIP01-19

Adults: treatment Essential Hypertension / Adults: treatment heart failure

Day 30 discussion

Cardiovascular Diseases

3.1.14. Ezetimibe / rosuvastatin calcium - EMEA-002541-PIP01-18

Elevated cholesterol

Day 30 discussion

Cardiovascular Diseases

3.1.15. Heparin sodium - EMEA-002557-PIP01-19

Prevention of thromboembolic events

Day 30 discussion

Cardiovascular Diseases

3.1.16. EMEA-002327-PIP02-19

Treatment and prevention of oral mucositis

Day 30 discussion

Dermatology

Atopic dermatitis

Day 30 discussion

Dermatology

3.1.18. EMEA-002552-PIP01-19

Treatment of non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Gastroenterology-Hepatology

3.1.19. Hematopoietic stem cells modified with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene - Orphan - EMEA-002562-PIP01-19

Rocket Pharmaceuticals, Inc.; Severe leukocyte adhesion deficiency type I (LAD-I)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.20. Obinutuzumab - Orphan - EMEA-001207-PIP02-19

Roche Registration GmbH; Systemic lupus erythemathosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.21. Ritonavir / darunavir - EMEA-002537-PIP01-18

Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.1.22. EMEA-002563-PIP01-19

Treatment of focal epilepsy

Day 30 discussion

Neurology

3.1.23. EMEA-002318-PIP03-19

Treatment of malignant melanoma

Day 30 discussion

Oncology

3.1.24. 6-(2-hydroxy-2-methylpropoxy)-4-(6-(6-((6-methoxypyridin-3-yl)methyl)-3,6-diazabicyclo[3.1.1]heptan-3-yl)pyridin-3-yl)pyrazolo[1,5-a]pyridine-3-carbonitrile - Orphan - EMEA-002544-PIP01-18

Loxo Oncology, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from ≥6 months to <18 years of age with RET-altered, locally advanced or metastatic, solid tumours or primary central nervous system (CNS) tumours

Day 30 discussion

Oncology

3.1.25. Belantamab mafodotin - Orphan - EMEA-002468-PIP03-19

GlaxoSmithKline Trading Services; Treatment of mature B-Cell neoplasms / Treatment for adult patients with BCMA-expressing mature B-cell neoplasms

Day 30 discussion

Oncology

3.1.26. Momelotinib - Orphan - EMEA-001656-PIP02-19

Sierra Oncology Inc.; Treatment of primary myelofibrosis

Day 30 discussion

Oncology

3.1.27. Moxetumomab pasudotox - Orphan - EMEA-002525-PIP01-18

AstraZeneca AB; Chronic lymphocytic leukaemias

Day 30 discussion

Oncology

3.1.28. Carfilzomib - Orphan - EMEA-001806-PIP04-19

Amgen Europe BV; Treatment of acute lymphoblastic leukemia (ALL) / Treatment of pediatric patients aged 1 year or older and young adult patients up to 21 years of age with bone marrow relapse of T-cell ALL treated with at least 1 prior therapy or B-cell ALL treated with at least 2 prior therapies, with or without extramedullary disease

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.29. Atropine sulphate - EMEA-002538-PIP01-18

Treatment of myopia

Day 30 discussion

Ophthalmology

3.1.30. Brimonidine tartrate - EMEA-002558-PIP01-19

Conjunctival hyperaemia due to minor eye irritation

Day 30 discussion

Ophthalmology

3.1.31. Lonafarnib - Orphan - EMEA-002516-PIP01-18

Eiger BioPharmaceuticals Europe Limited; progeroid laminopathies, Hutchinson-Gilford Progeria Syndrome (HGPS)

Day 30 discussion

Other

3.1.32. Bempedoic acid - EMEA-001872-PIP02-19

Treatment of mixed dyslipidaemia

Day 30 discussion

Other / Cardiovascular Diseases

3.1.33. Ezetimibe / bempedoic acid - EMEA-002200-PIP02-19

Treatment of mixed dyslipidaemia

Day 30 discussion

Other / Cardiovascular Diseases

3.1.34. Dexamethasone sodium phosphate / levofloxacin - EMEA-002375-PIP02-18

Acute Otitis Externa / Treatment of acute otitis externa

Day 30 discussion

Oto-rhino-laryngology

3.1.35. Dexamethasone sodium phosphate / levofloxacin - EMEA-002375-PIP03-18

Acute Otitis Media with spontaneous tympanic membrane perforation / Treatment of acute otitis media with spontaneous tympanic membrane perforation

Day 30 discussion

Oto-rhino-laryngology

3.1.36. Selonsertib - EMEA-001868-PIP04-18

Chronic Kidney Disease / Treatment of patients with progressive chronic kidney disease (CKD) resulting from congenital anomalies of the kidney and urinary track (CAKUT) aged 3 to less than 18 years

Day 30 discussion

Uro-nephrology

3.1.37. Bordetella pertussis antigen: pertactin / bordetella pertussis antigen: filamentous haemagglutinin / bordetella pertussis antigen: pertussis toxoid / tetanus toxoid / diphtheria toxoid - EMEA-002343-PIP01-18

ICD10: A36 (diphtheria), ICD10: A37 (whooping cough), ICD10: A35 (other tetanus) / Active booster immunization

Day 30 discussion

Vaccines / Infectious Diseases

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. Filgotinib - EMEA-C1-001619-PIP04-17-M01

Gilead Sciences International Ltd.; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis, and juvenile idiopathic arthritis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.2. Lefamulin - EMEA-C1-002075-PIP01-16-M01

Nabriva Therapeutics AG; Treatment of community-acquired pneumonia

Day 30 discussion

Infectious Diseases

3.2.3. 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-C1-002418-PIP01-18

Sanofi Pasteur; Prevention of influenza infection

Day 30 discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Baricitinib - EMEA-001220-PIP03-16-M01

Eli Lilly and Company Limited; Treatment of atopic dermatitis / Treatment of patients with moderate to severe atopic dermatitis

Day 30 discussion

Dermatology

3.3.2. Terbinafine hydrochloride - EMEA-001259-PIP02-13-M02

Polichem, S.A.; Treatment of onychomycosis

Day 30 discussion

Dermatology

3.3.3. Testosterone - EMEA-001529-PIP02-14-M02

Acerus Biopharma Inc.; Male hypogonadism / Treatment of male hypogonadism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Human normal immunoglobulin for subcutaneous administration - EMEA-001853-PIP01-15-M02

Grifols Therapeutics LLC; Treatment for primary immunodeficiency

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.5. Tocilizumab - EMEA-000309-PIP04-17-M02

Roche Registration GmbH; Cytokine release syndrome / Treatment of chimeric antigen receptor (CAR) T cell-induced severe or life-threatening cytokine release syndrome (CRS) in adults and paediatric patients 2 years of age and older

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.6. Eravacycline - EMEA-001555-PIP01-13-M03

Tetraphase Pharmaceuticals, Inc.; Complicated intra-abdominal infection

Day 30 discussion

Infectious Diseases

3.3.7. Posaconazole - EMEA-000468-PIP02-12-M05

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections / Treatment of invasive fungal infections / For treatment of invasive fungal infections in the following paediatric patients: Invasive aspergillosis in patients with disease that is refractroy to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products; Treatment of Invasive Aspergillosis / Patients receiving remission-induction chemotherapy for acute myelogenous leukemia (AML) or myelodysplastic syndromes (MDS) expected to result in prolonged neutropenia and who are at high risk of developing invasive fungal infections / Hematopoietic stem cell transplant (HSCT)

recipients who are undergoing high-dose immunosuppressive therapy for graft versus host disease and who are at high risk of developing invasive fungal infections

Day 30 discussion

Infectious Diseases

3.3.8. Tedizolid phospate - EMEA-001379-PIP01-12-M04

Merck Sharp & Dohme (Europe) Inc.; Treatment of acute bacterial skin and skin structure infections

Day 30 discussion

Infectious Diseases

3.3.9. Balovaptan - EMEA-001918-PIP01-15-M02

Roche Registration GmbH; ICD1O F84: Treatment of autism spectrum disorder / Treatment of core social and communication deficits in people with autism spectrum disorder aged 2 years or older

Day 30 discussion

Neurology

3.3.10. Eculizumab - Orphan - EMEA-000876-PIP03-14-M03

Alexion Europe SAS; Neuromyelitis optica spectrum disorders / Treatment of paediatric patients with relapsing neuromyelitis optica spectrum disorders

Day 30 discussion

Neurology

3.3.11. Galcanezumab - EMEA-001860-PIP03-16-M03

Eli Lilly and Company Limited; Prevention of migraine headaches

Day 30 discussion

Neurology

3.3.12. Humanised anti-IL-6 receptor (IL-6R) monoclonal antibody - Orphan - EMEA-001625-PIP01-14-M03

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 30 discussion

Neurology

3.3.13. Afatinib - EMEA-001596-PIP02-17-M01

Boehringer Ingelheim International GmbH; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and

lymphoid tissue neoplasms) / Treatment of malignant neoplasms of the central nervous system

Day 30 discussion

Oncology

3.3.14. Durvalumab - EMEA-002028-PIP01-16-M01

AstraZeneca AB; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lyphoid tissue) / Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy / Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumour

Day 30 discussion

Oncology

3.3.15. Tremelimumab - EMEA-002029-PIP01-16-M01

AstraZeneca AB; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lyphoid tissue) / Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy / Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumour

Day 30 discussion

Oncology

3.3.16. Venetoclax - Orphan - EMEA-002018-PIP02-16-M02

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms / Treatment of solid tumour malignant neoplasms / Treatment of a specific paediatric hematopoietic and lymphoid malignant neoplasm or of a solid malignant neoplasm, in patients from 1 month to 18 years of age

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.3.17. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M08

MediWound Germany GmbH; Treatment of burns of external body surfaces / Removal of eschar in deep partial and/or full thickness burns

Day 30 discussion

Other

3.3.18. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M04

Shire Pharmaceuticals Ireland Limited (an indirect wholly owned subsidiary of Shire plc); Hereditary angioedema / Treatment of hereditary angioedema

Day 30 discussion

Other

3.3.19. Fentanyl hydrochloride - EMEA-001509-PIP01-13-M02

Incline Therapeutics Europe Ltd. (a wholly owned subsidiary of The Medicines Company); Treatment of acute pain

Day 30 discussion

Pain

3.3.20. Ivacaftor / tezacaftor - Orphan - EMEA-001640-PIP01-14-M05

Vertex Pharmaceuticals (Europe) Ltd.; Cystic Fibrosis / Treatment of cystic fibrosis

Day 30 discussion

Pneumology - Allergology

3.3.21. Esketamine hydrochloride - EMEA-001428-PIP03-15-M01

Janssen-Cilag International NV; Major Depressive Disorder (MDD)

Day 30 discussion

Psychiatry

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by Streptococcus pneumoniae / Active immunisation for the prevention of invasive disease, pneumonia and acute otitis media caused by Streptococcus pneumoniae in infants, children and adolescents from 6 weeks to less than 18 years of age

Day 30 discussion

Vaccines

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 25 June 2019 for Nomination of Rapporteur and Peer reviewer

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

No items

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

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.

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.

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.

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

6.1.1. 2-[3,5-bis(trifluoromethyl)phenyl]-N-{4-(4-fluoro-2-methylphenyl)-6-[(7S,9aS)-7-(hydroxymethyl)hexahydropyrazino[2,1-c][1,4]oxazin-8(1H)-yl]-3-pyridinyl}-N,2-dimethylpropanamide- EMEA-03-2019

KaNDy Therapeutics Limited; All classes of medicinal products for treatment of climacteric symptoms associated with decreased oestrogen levels, as occurring at menopause / Treatment of menopausal-related symptoms

Summary of committee discussion:

The applicability of the class waiver as referred to in the Agency's Decision CW/0001/2015 to the planned therapeutic indication was confirmed.

Other potential paediatric interests of this medicine suggested by PDCO: None identified at this stage.

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

The members of the PDCO took note of the products listed in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. Update on PDCO member(s)/alternate(s) mandate status

No items

9.2. Coordination with EMA Scientific Committees or CMDh-v

9.2.1. Committee for Medicinal Products for Human Use (CHMP)

Summary of committee discussion:

The PDCO members were informed about the final CHMP Opinions on medicinal products with recommended paediatric indications adopted in March 2019. These included Zynteglo (autologous cd34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin bb305 lentiviral vector encoding the beta-a-t87q-globin gene) and Mozobil (plerixafor).

The list of procedures with paediatric indications to be evaluated by the CHMP, starting in March 2019, was presented to the PDCO members.

9.2.2. Coordination group for mutual recognition and decentralised procedures – Human (CMDh) – Paediatric subgroup

Summary of committee discussion:

During the April 2019 PDCO meeting, a joint session with the CMDh Working Party on Paediatric Regulation was held to initiate collaboration between the PDCO and the CMDh Working Party on Paediatric Regulation mainly for the PDCO to provide scientific expertise when needed in the context of article 45 worksharing procedures.

9.2.3. Concept paper on the need for revision of the guideline on the investigation of medicinal products in the term and preterm neonate - comments received during public consultation

Summary of committee discussion:

The Committee was made aware of the response to the comments received during the public consultation on the "Concept paper on the need for revision of the guideline on the investigation of medicinal products in the term and preterm neonate" prepared by the Neonatal break-out session. Members were invited to send comments before the next Plenary Meeting.

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Summary of committee discussion:

The Chair of the Non-clinical Working Group identified the products which will require Non-clinical Working Group (NCWG) evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

The Chair of the Formulation Working Group (FWG) identified the products which will require Formulation Working Group evaluation and discussion.

9.3.3. Healthcare Professionals Working Party (HCPWP) and Patients and Consumers Working Party (PCWP) - Nomination of PDCO representative(s)

Summary of committee discussion:

The EMA Secretariat launched a call at PDCO for nominating representative(s) to the PCWP and HCPWP for the mandate 2019-2022. The PDCO endorsed the nominations of the PDCO delegate appointed by the EC representing patients' organisations: Dimitrios Athanasiou as well as PDCO delegate appointed by the EC representing healthcare professionals: Johannes Taminiau. One PDCO representative of the patients' organisations together with a PDCO representative of the healthcare professionals will attend PCWP meetings. The same applies for HCPWP meetings.

9.3.4. Healthcare Professionals Working Party (HCPWP) and Patients and Consumers Working Party (PCWP) - Proposal to increase the current membership of the PCWP and HCPWP from 20 to 22 organisation members each

Summary of committee discussion:

The proposal of EMA Secretariat to increase the current membership of the PCWP and HCPWP from 20 to 22 organisation members each was endorsed by the PDCO.

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA) – draft letter to Innovative Therapies for Children with Cancer (ITCC) and Accelerate on foster age inclusive research

Summary of committee discussion:

The PDCO adopted a letter, addressed to ITCC and Accelerate, supporting age inclusive research in paediatric oncology whenever scientifically justified.

9.4.2. Multi-stakeholder Meeting on Allergen Immuno-therapy (AIT) for Children – minutes from the meeting

Summary of committee discussion:

The final summary of the meeting including comments from various meeting participants were adopted by the PDCO.

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

9.6.1. Cardiac Safety Research Consortium Think Tank: Non-vitamin K antagonist oral anticoagulants (NOAC) Use in the Pediatric Population: Defining the Path Forward - ACC Heart House - Washington, DC – Feedback

PDCO: Dirk Mentzer (Chair)

Summary of committee discussion:

The Committee was provided with feedback from the conference, gathering representatives from Industry, Regulators and Academia. Unmet needs and the positioning of standard of care and new anticoagulants were discussed, as well as regulatory requirements for trial design. A whitepaper summarizing the conference outcomes shall be published by the Cardiac Safety Research Consortium.

9.7. PDCO work plan

No items

9.8. Planning and reporting

9.8.1. Marketing authorisation applications (MAA) forecast for 2019 – planning update dated Q1 2019

Summary of committee discussion:

The EMA Business Analysis & Forecasting presented to PDCO for information a quarterly updated report on marketing authorisation applications planned for submission (the business 'pipeline').

9.8.2. Strategic Review and Learning Meeting (SRLM) under the Romanian Presidency to be held in Malta on 13-14 June 2019

PDCO members: Dana Gabriela Marin, John Joseph Borg, Herbert Lenicker

Summary of committee discussion:

The EMA Secretariat presented a draft Agenda for the Strategic Review and Learning Meeting (SRLM) that will be held on 13-14 June 2019 in Malta under the Romanian Presidency.

10. Any other business

10.1.1. Policy on handling competing interests for Scientific Committees' members and experts

Summary of committee discussion:

The EMA Secretariat reminded the Committee of the principles on handling competing interests for Scientific Committees' members and experts as detailed in the corresponding EMA Policy on handling competing interests for Scientific Committees' members and experts (EMA/626261/2014, Rev. 1. In particular, the presentation detailed the types of interests to be declared in the electronic declaration of interests (eDOI) by a member or an expert, the corresponding restrictions in EMA activities if an interest is declared, the requirement to inform the EMA of any intention to become an employee in a pharmaceutical company and the breach of trust procedure in case an interest is not declared intentionally or through gross negligence.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The break-out session was cancelled.

11.1.2. Neonatology

Summary of committee discussion:

The break-out session finalised the response to the comments received during the public consultation on the "Concept paper on the need for revision of the guideline on the investigation of medicinal products in the term and preterm neonate" in preparation for its final adoption.

11.1.3. Inventory

Summary of committee discussion:

The inventory group continued discussion on the assessment of unmet needs in the context of the procedures discussed during the PDCO plenary meetings.

The Chair thanked all participants and closed the meeting.

12. List of participants

List of participants including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 23-26 April 2019 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dirk Mentzer	Chair	Germany	No interests declared	
Karl-Heinz Huemer	Member	Austria	No interests declared	
Koenraad Norga	Member (Vice-Chair)	Belgium	When chairing the meeting: To be replaced for discussions, final deliberations and voting When not chairing the meeting: No participation in final deliberations and voting	3.1.12. Human immunoglobulin G2 isotype antibody to IL-33R - EMEA-002515-PIP01-18 (GSK) 3.1.25. Belantamab mafodotin - Orphan - EMEA-002468-PIP03-19(GSK)
Karen Van Malderen (via TC)	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Tereza Bazantova	Member	Czech Republic	No interests declared	
Kirstine Moll Harboe	Member	Denmark	No interests declared	
Ann Marie Totterman (via TC)	Member	Finland	No interests declared	
Pia Annunen	Alternate	Finland	No restrictions applicable to this meeting	
Sylvie Benchetrit	Member	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-	Member	Latvia	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Freimane			applicable to this meeting	
Sigita Burokiene	Member	Lithuania	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Maaike van Dartel	Member	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No interests declared	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Peter Szitanyi	Alternate	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Ninna Gullberg	Member	Sweden	No interests declared	
Eva Agurell	Alternate	Sweden	No interests declared	
Angeliki Siapkara	Member	United Kingdom	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No interests declared	
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	
Catherine Cornu (via TC)	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Günter Karl-	Member	Patients'	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Heinz Auerswald		Organisation Representative	applicable to this meeting	
Paola Baiardi	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Michal Odermarsky	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Roel Bolt	Expert - in person*		No interests declared	
Martina Schussler-Lenz	Expert - in person*	CAT Chair	No interests declared	
Prof Wout Lamers	Expert - in person*	(Amsterdam UMC)		
Sarah Branch	Expert - via telephone*	United Kingdom - MHRA		
Johanna Lähteenvuo	Expert - via telephone*	Finland - FIMEA		
Karri Penttilä	Expert - via telephone*	Finland - FIMEA		
Meeting run with support from relevant EMA staff				

* Experts were only evaluated against the agenda topics or activities they participated in

13. 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/