11 December 2019
EMA/PDCO/615413/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes for the meeting on 12-15 November 2019

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in these 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 these 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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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 (See 12.).

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. No new or additional interests or restrictions were declared.

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.

Due to restricted involvement, the Chair Koenraad Norga deputised chairing the meeting to the Vice-Chair, Sabine Scherer, for the following procedures: EMEA-000520-PIP02-13-M03, EMEA-C-000139-PIP01-07-M03, EMEA-002515-PIP01-18, EMEA-002153-PIP01-17-M01.

1.2. Adoption of agenda

The agenda of the 12-15 November 2019 PDCO meeting was adopted and was published on the EMA website.

1.3. Adoption of the minutes

The minutes of the 15-18 October 2019 PDCO meeting were adopted and will be published on the EMA website.

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. Aztreonam / avibactam - EMEA-002283-PIP01-17

Pfizer Europe MA EEIG; Treatment of infections caused by aerobic gram-negative bacteria

Day 120 Opinion

Infectious Diseases

Summary of committee discussion:

The PDCO adopted a favourable opinion on the paediatric investigation plan for aztreonam/avibactam for the treatment of infections caused by aerobic gram-negative bacteria for all the subsets of the paediatric population. Study initiation dates were not deferred. Deferrals for all the other measures contained in the paediatric investigation plan were agreed by the PDCO.

2.1.2. Abemaciclib - EMEA-002342-PIP02-18

Eli Lilly and Company Limited; High grade glioma (HGG) / Neuroblastoma (NBL) / Treatment of relapsed or refractory neuroblastoma in combination with irinotecan and temozolomide in paediatric patients / Treatment of newly diagnosed high grade glioma in combination with temozolomide in paediatric patients

Day 120 Opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 120 during the November 2019 plenary meeting.
The Committee took into consideration all the previous discussions and the clarifications provided by the applicant and concluded that all issues have been resolved.
Therefore, the PDCO adopted a positive Opinion at Day 120 for abemaciclib for all subsets of the paediatric population (from birth to less than 18 years of age) in the conditions of treatment of glioma and treatment neuroblastoma.

2.1.3. Chloroprocaine (hydrochloride) - EMEA-000639-PIP04-19

Sintetica GmbH; Epidural block (extension of epidural anaesthesia in unplanned caesarean section)

Day 60 Opinion

Anaesthesiology

Summary of committee discussion:

The PDCO adopted a negative Opinion on this full waiver request for the local anaesthetic chloroprocaine (hydrochloride), solution for injection (epidural use) for epidural block (extension of epidural anaestesia in unplanned Cesarean section).

2.1.4. Chloroprocaine (hydrochloride) - EMEA-000639-PIP05-19

Sintetica GmbH; Ocular surface anaesthesia

Day 60 Opinion

Anaesthesiology

Summary of committee discussion:

The PDCO discussed the applicant's responses to the PDCO questions from day 30 of the procedure for the full waiver proposal for this local anaesthetic chloroprocaine (hydrochloride) eye gel for use during or after ophthalmologic procedures in children.

The PDCO adopted a negative Opinion on the full waiver request on 15th November 2019.

2.1.5. Ethanol - EMEA-002672-PIP01-19

Ablative Solutions Inc.; Treatment of primary hypertension

Day 60 Opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO recommends granting a waiver for Ethanol for all subsets of the paediatric population (from birth to 18 years of age) in the condition of "treatment of uncontrolled primary hypertension" on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.
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.

2.1.6. Masitinib (mesylate) - Orphan - EMEA-001266-PIP04-19

AB Science; Treatment of amyotrophic lateral sclerosis

Day 60 Opinion

Neurology

Summary of committee discussion:

The PDCO recommends granting a product-specific waiver for masitinib mesylate in all subsets of the paediatric population for the treatment of amyotrophic lateral sclerosis on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to the specified paediatric subset(s).
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. Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti CD19 CD28/CD3-zeta chimeric antigen receptor and cultured - EMEA-001862-PIP02-19

Kite Pharma EU B.V.; Treatment of mantle cell lymphoma

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO agreed a product-specific waiver for this product on the grounds that the disease or condition for which the medicinal product is intended occurs only in the adult population. The PDCO recommends granting a waiver for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mantle cell lymphoma.
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. Iberdomide - EMEA-002636-PIP01-19

Celgene Europe B.V.; Treatment of mature B-cell neoplasms

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO recommends granting a waiver for iberdomide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mature B-cell neoplasms based on the ground of lack of significant therapeutic benefit.
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. Sacituzumab govitecan - EMEA-002645-PIP01-19

Immunomedics GmbH; Refractory/relapsed triple-negative breast cancer (TNBC)

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO recommends granting a waiver for sacituzumab govitecan for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of breast cancer based on the ground that the disease does not occur in children.

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. Bispecific T-cell engager antibody with a single-chain fragment crystallizable moiety that binds to B cell maturation antigen surface receptor on tumour cells and the cluster of differentiation 3 receptor on T-cells - EMEA-002606-PIP02-19

Amgen Europe BV; Treatment of multiple myeloma

Day 60 Opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO recommends granting a waiver for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of multiple myeloma based on the ground that the disease does not occur.

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.

2.2. 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. Valoctocogene roxaparvovec - EMEA-C1-002427-PIP01-18

BioMarin International Ltd.; Treatment of congenital haemophilia A

Day 60 Opinion

Haematology-Hemostaseology

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.

2.2.2. Baloxavir marboxil - EMEA-C1-002440-PIP01-18

Roche Registration GmbH; Treatment of influenza

Day 60 Opinion

Infectious Diseases

Summary of committee discussion:

The studies were considered compliant.

2.2.3. Lasmiditan - EMEA-C1-002166-PIP01-17-M02

Eli Lilly and Company Limited; Treatment of migraine headache

Day 60 Opinion

Neurology

Summary of committee discussion:

The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0291/2019 of 14/8/2019).
The PDCO finalised this partially completed compliance procedure on 15/11/2019.

2.2.4. Fostemsavir - EMEA-C1-001687-PIP01-14-M03

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 Opinion

Infectious Diseases

Summary of committee discussion:

The PDCO noted the positive outcome of this compliance check.

2.2.5. Lacosamide - EMEA-C1-000402-PIP03-17-M03

UCB Pharma S.A.; Treatment of generalised epilepsy and epileptic syndromes

Day 30 Opinion

Neurology

Summary of committee discussion:
The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision.
The PDCO finalised this partially completed compliance procedure on 15/11/2019.

2.2.6. Ad26.ZEBOV (recombinant, replication-incompetent) - EMEA-C1-002307-PIP01-17

Janssen-Cilag International NV; Prevention of Ebola virus disease

Day 30 Opinion

Vaccines

Summary of committee discussion:

The study submitted was considered compliant.

The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0059/2019) of 28/02/2019.

The PDCO finalised this partially completed compliance procedure on 15/11/2019.

2.2.7. MVA-BN-Filo (recombinant, non-replicating) - EMEA-C1-002308-PIP01-17

Janssen-Cilag International NV; Prevention of Ebola virus disease / Active immunization for prevention of disease caused by Ebola virus (Zaire ebolavirus species) in individuals ⩾1 year of age

Day 30 Opinion

Vaccines – Infectious disease

Summary of committee discussion:

The study submitted was considered compliant.

2.2.8. Vosoritide - EMEA-C2-002033-PIP01-16

BioMarin International Limited; Treatment of achondroplasia

Day 30 Opinion

Other

Summary of committee discussion:

The PDCO discussed the completed study and considered it compliant with the latest Agency's Decision.

2.2.9. Ragweed pollen extract (ambrosia artemisiifolia) - EMEA-C-001881-PIP01-15

ALK Abelló A/S; Treatment of allergic rhinitis / Rhino-conjunctivitis

Day 60 Opinion

Pneumology – Allergology

Summary of committee discussion:

The PDCO adopted on 15 November 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/0266/2016 of 5 October 2016.

2.2.10. Potassium hydrogen carbonate / Potassium citrate monohydrated - EMEA-C-001357-PIP01-12-M02

ADVICENNE; Treatment of renal tubular acidosis

Day 60 Opinion

Uro-nephrology

Summary of committee discussion:

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision.

2.2.11. Outer membrane vesicles (OMV) from *Neisseria meningitidis* serogroup B strain NZ98/254 measured as amount of total protein containing the PorA P1.4 / recombinant *Neisseria meningitidis* serogroup B NadA protein / recombinant *Neisseria meningitidis* serogroup B fHBP fusion protein / recombinant *Neisseria meningitidis* serogroup B NHBA fusion protein - EMEA-C-000139-PIP01-07-M03

GSK Vaccines S.r.l.; Prevention of meningococcal meningitis

Day 30 Opinion

Vaccines

Summary of committee discussion:

The PDCO adopted on 15 November 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/0334/2019) of 11 September 2019.

2.2.12. Nonacog beta pegol (glycopegylated recombinant coagulation factor IX) - EMEA-C-000731-PIP01-09-M03

Novo Nordisk A/S; Treatment of factor IX deficiency

Day 30 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO adopted on 15 November 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/0139/2019 of 17/04/2019.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Edoxaban (tosylate) - EMEA-000788-PIP02-11-M09

Daiichi Sankyo Europe GmbH; Prevention of arterial thromboembolism / Prevention of venous thromboembolism / Treatment of venous thromboembolism / Prevention of arterial thromboembolism in paediatric cardiac patients at risk of thrombotic events / Acute treatment & secondary prevention of symptomatic recurrent venous thrombotic events (VTE) in paediatric patients at risk

Day 60 Opinion

Cardiovascular Diseases / Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0368/2018 of 7/12/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Small molecule Janus Kinase -1 inhibitor (abrocitinib) - EMEA-002312-PIP01-17-M01

Pfizer Europe MA EEIG; Treatment of moderate to severe atopic dermatitis

Day 60 Opinion

Dermatology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0391/2018 of 7 December 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Exenatide - EMEA-000689-PIP01-09-M09

AstraZeneca AB; Non-insulin dependent diabetes mellitus (excluding treatment with thiazolidinediones) / Non-insulin dependent diabetes mellitus (treatment including thiazolidinediones) / Non-insulin dependent diabetes mellitus - in combination with insulin (with or without oral antidiabetics) / Treatment of type 2 diabetes mellitus

Day 60 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO discussed the applicant's responses on 15 November 2019.

The PDCO adopted a positive PIP Opinion for the pivotal paediatric study, as set in the Agency's latest decision (P/0297/2018 of 12/09/2018).

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

2.3.4. Tolvaptan - EMEA-001231-PIP02-13-M07

Otsuka Pharmaceutical Netherlands B.V.; Polycystic kidney disease (PKD) /Treatment of progression of autosomal dominant polycystic kidney disease (ADPKD)

Day 60 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0378/2018 of 07/12/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Ozanimod hydrochloride - EMEA-001710-PIP03-17-M01

Celgene Europe B.V.; Treatment of ulcerative colitis / Treatment of moderate to severely active ulcerative colitis

Day 60 Opinion

Gastroenterology-Hepatology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0153/2019 of 17 April 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human betaA-T87Q-globin gene - Orphan - EMEA-001665-PIP01-14-M03

bluebird bio (Netherlands) B.V.; Treatment of β-thalassaemia / Treatment of beta-thalassaemia major and severe intermedia

Day 60 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the applicant's comments and counter proposals on the draft PIP Opinion for the modification procedure during its plenary on 15 November 2019.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP, in line with above and as set in the Agency's latest decision (P/0067/2018 of 16/03/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Human Cell Line recombinant human factor VIII (human-cl rhFVIII) / human coagulation factor VIII (rDNA) - EMEA-001024-PIP01-10-M02

Octapharma Pharmazeutika Produktionsges.m.b.H; D66: Hereditary factor VIII deficiency, haemophilia A / Haemophilia A

Day 60 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the application 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/0214/2012 of 28/09/2012).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Luspatercept - Orphan - EMEA-001521-PIP01-13-M04

Celgene Europe B.V.; Anaemias due to chronic disorders / Treatment of anaemia in patients with beta-thalassemia intermedia and major

Day 60 Opinion

Haematology-Hemostaseology

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0130/2019 of 17 April 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Belimumab - EMEA-000520-PIP02-13-M03

Glaxo Group Limited; Systemic lupus erythematosus / Treatment of systemic lupus erythematosus

Day 60 Opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0313/2018 of 12/09/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Voxilaprevir / velpatasvir / sofosbuvir - EMEA-001822-PIP01-15-M01

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C / Treatment of chronic hepatitis C in adolescents and children 12 years of age and older

Day 60 Opinion

Infectious Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO thus considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0121/2016 of 29 April 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Daclizumab - EMEA-001349-PIP01-12-M03

Biogen Idec Ltd; Multiple sclerosis / Treatment of relapsing remitting forms of multiple sclerosis

Day 60 Opinion

Neurology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0147/2014 of 13 June 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Avapritinib - Orphan - EMEA-002358-PIP02-18-M01

Blueprint Medicines (Netherlands) B.V.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 2 to less than 18 years of age with relapsed/refractory solid tumour harbouring mutations in either KIT or PDGFR-alpha.

Day 60 Opinion

Oncology

Summary of committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0026/2019 of 22 February 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Brigatinib - EMEA-002296-PIP01-17-M01

Takeda Pharm A/S; Inflammatory myofibroblastic tumors (IMT) / Non-small cell lung cancer (NSCLC) / Anaplastic large cell lymphoma (ALCL) / Treatment of anaplastic lymphoma kinase (ALK) positive advanced non-small cell lung cancer (NSCLC) / Treatment of paediatric patients ≥1 years of age with ALK+ unresectable or recurrent IMT / Treatment in combination with standard chemotherapy in paediatric patients ≥1 years of age with newly diagnosed ALK+ ALCL at high risk for recurrence

Day 60 Opinion

Oncology

Summary of committee discussion:

The clarifications provided by the applicant for the completion of the study were considered acceptable.
All pending issues were considered solved.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0350/2018 of 15 November 2018).

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

2.3.14. Lumacaftor / ivacaftor - EMEA-001582-PIP01-13-M09

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

Day 60 Opinion

Other

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 only part of the proposed changes could be accepted and adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0407/2018 of 19 December 2018).

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

2.3.15. Dupilumab - EMEA-001501-PIP02-13-M04

sanofi-aventis recherche & développement; Treatment of asthma

Day 60 Opinion

Pneumology – Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the application 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/0304/2018 of 12 September 2018).

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

2.3.16. Tezepelumab - EMEA-001613-PIP01-14-M04

AstraZeneca AB; Treatment of asthma / Tezepelumab is indicated as add-on maintenance treatment of patients with severe asthma aged 5 years and older

Day 60 Opinion

Pneumology – Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0263/2019 of 19 July 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Agomelatine - EMEA-001181-PIP01-11-M05

Les Laboratoires Servier; Treatment of major depressive episodes

Day 60 Opinion

Psychiatry

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.

The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0191/2016 of 15/07/2016).

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

2.3.18. Lumasiran sodium - Orphan - EMEA-002079-PIP01-16-M01

Alnylam UK Limited; Treatment of primary hyperoxaluria type 1

Day 60 Opinion

Uro-nephrology

Summary of committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0373/2017 of 13/10/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

The following partial compliance checks have concluded positively without PDCO discussion. The Committee has been informed in writing.

2.7.1. Acalabrutinib - EMEA-C1-001796-PIP03-16-M01

Acerta Pharma B.V.; Indicated for the treatment of adult patients with chronic lymphocytic

leukaemia (CLL)/small lymphocytic lymphoma (SLL) / Treatment of children from 1 year to less than 18 years of age with newly-diagnosed and relapsed/refractory mature B-cell lymphoma, diffuse large B-cell lymphoma or Burkitt lymphoma or primary mediastinal lymphoma

Day 1 letter

Oncology

2.7.2. Tralokinumab - EMEA-C1-001900-PIP02-17-M02

LEO Pharma A/S; Treatment of atopic dermatitis

Day 1 letter

information

Dermatology

2.8. Revision of PDCO Opinions

No items

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. Asciminib hydrochloride - EMEA-002347-PIP01-18

Treatment of Philadelphia positive chronic myelogenous leukemia in chronic phase

Day 90 discussion

3.1.2. EMEA-002568-PIP01-19

Psoriasis / Treatment of moderate to severe chronic plaque-type psoriasis who are candidates for systemic therapy

Day 90 discussion

Dermatology

3.1.3. Dupilumab - EMEA-001501-PIP04-19

Treatment of eosinophilic esophagitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.4. Norursodeoxycholic acid - Orphan - EMEA-002485-PIP01-18

Dr. Falk Pharma GmbH; Primary sclerosing cholangitis (PSC)

Day 90 discussion

Gastroenterology-Hepatology

3.1.5. EMEA-002501-PIP01-18

Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital FVIII deficiency)

Day 90 discussion

Haematology-Hemostaseology

3.1.6. Anti-CD7 mAb conjugated to ricin toxin A chain / anti-CD3 mAb conjugated to ricin toxin A chain - Orphan - EMEA-002087-PIP01-16

Xenikos BV; Steroid refractory acute graft versus host disease

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Mecasermin rinfabate - Orphan - EMEA-000534-PIP03-17

Premacure AB, a member of the Shire group of companies; Chronic lung disease of prematurity

Day 90 discussion

Neonatology - Paediatric Intensive Care

3.1.8. Atogepant - EMEA-002530-PIP01-18

G43 Migraine / Prophylaxis of migraine

Day 90 discussion

Pain

3.1.9. (R)-1-(3-(aminomethyl) phenyl)-N-(5-((3 cyanophenyl)(cyclopropylmethylamino)methyl)-2-fluorophenyl)-3-(trifluoromethyl)-1H-pyrazole-5-carboxamide dihydrochloride - Orphan - EMEA-002449-PIP02-18

BioCryst UK; Treatment of hereditary angioedema (HAE)/ Treatment of HAE attacks /Prevention of HAE attacks

Day 90 discussion

Pneumology – Allergology

3.1.10. EMEA-002484-PIP01-18

Asthma / Use as an add-on controller medication in the treatment of adults, adolescents and children (>1 year of age) with inadequately controlled asthma

Day 90 discussion

Pneumology – Allergology

3.1.11. EMEA-002515-PIP01-18

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

Day 90 discussion

Pneumology – Allergology

3.1.12. Budesonide - Orphan - EMEA-002500-PIP01-18

Calliditas Therapeutics AB; Primary IgA nephropathy

Day 90 discussion

Uro-nephrology

3.1.13. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19

Alexion Europe S.A.S.; Wilson disease

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.14. Pegfilgrastim - EMEA-002671-PIP01-19

Treatment of chemotherapy-induced neutropenia and prevention of chemotherapy-induced febrile neutropenia / Reduction in the duration of neutropenia and the incidence of febrile neutropenia in paediatric patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 60 discussion

Haematology-Hemostaseology

3.1.15. Taniborbactam / cefepime - EMEA-002576-PIP01-19

Treatment of bacterial infections / Treatment of complicated urinary tract infections (cUTI) / Treatment of hospital acquired and ventilator acquired pneumonia (HAP/VAP) / Treatment of complicated intra-abdominal infections (CIAI)

Day 60 discussion

Infectious Diseases

3.1.16. Rozanolixizumab - Orphan - EMEA-002681-PIP01-19

UCB Pharma S.A.; Treatment of myasthenia gravis

Day 60 discussion

Neurology

3.1.17. EMEA-002635-PIP01-19

Treatment of advanced or metastatic malignancies harbouring anaplastic lymphoma kinase ALK, ROS1, or NTRK1-3 alterations

Day 60 discussion

Oncology

3.1.18. Cyclophosphamide - EMEA-002644-PIP01-19

Treatment of malignant disease / Cyclophosphamide is a cytotoxic drug for the treatment of malignant disease in children. As a single agent, it has successfully produced an objective remission in a wide range of malignant conditions / Cyclophosphamide is also frequently used in combination with other cytotoxic drugs, radiotherapy or surgery

Day 60 discussion

Oncology

3.1.19. Imatinib - EMEA-002643-PIP01-19

Treatment of newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy / Treatment of chronic myelogenous leukaemia: Philadelphia chromosome (Ph1) positive with crisis of blast cells / Paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment / Paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy or in accelerated phase or blast crisis / Paediatric patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy

Day 60 discussion

Oncology

3.1.20. Lenvatinib - EMEA-001119-PIP03-19

Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue

Day 60 discussion

Oncology

3.1.21. EMEA-002656-PIP01-19

Chikungunya disease

Day 60 discussion

Vaccines

3.1.22. EMEA-002657-PIP01-19

Visualisation of prostate-specific membrane antigen in adenocarcinoma of the prostate

Day 30 discussion

Diagnostic

3.1.23. EMEA-002682-PIP01-19

Acromegaly and gigantism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. Rebisufligene etisparvovec - Orphan - EMEA-002206-PIP02-19

Abeona Therapeutics Inc.; Treatment of mucopolysaccharidosis IIIA (ICD-10 E76.2) / Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome) in children from 6 months to less than 18 years of age

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. Naltrexone - EMEA-002670-PIP01-19

Treatment of Crohn's disease / Treatment of moderate to severe active Crohn's disease as an adjuvant therapy in paediatric patients (from 6 years of age)

Day 30 discussion

Gastroenterology-Hepatology

3.1.26. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19

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

Day 30 discussion

Haematology-Hemostaseology

3.1.27. Evobrutinib - EMEA-002284-PIP02-19

Treatment of systemic lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.28. Ritonavir / darunavir - EMEA-002537-PIP02-19

Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.1.29. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19

SIGA Technologies, Inc.; Orthopoxvirus disease (smallpox, monkeypox, cowpox, and vaccinia complications)

Day 30 discussion

Infectious Diseases

3.1.30. Glibenclamide - EMEA-002651-PIP01-19

Large hemispheric infarction

Day 30 discussion

Neurology

3.1.31. Hyoscine / physostigmine - EMEA-002678-PIP01-19

Poisoning by nervous system stimulants

Day 30 discussion

Neurology

3.1.32. EMEA-002679-PIP01-19

Prostate cancer

Day 30 discussion

Oncology

3.1.33. EMEA-002650-PIP01-19

Multiple myeloma

Day 30 discussion

Oncology

3.1.34. Loncastuximab tesirine - EMEA-002665-PIP01-19

Treatment of diffuse large B-cell lymphoma (DLBCL)

Day 30 discussion

Oncology

3.1.35. 1-[(3S)-3-{4-amino-3-[(3,5-dimethoxyphenyl)ethynyl]-1H-pyrazolo[3,4-d]pyrimidin-1-yl}pyrrolidin-1-yl]-2-propen-1-one - Orphan - EMEA-002647-PIP01-19

Taiho Pharma Europe Lt; Biliary tract cancer

Day 30 discussion

Oncology

3.1.36. Ripasudil - EMEA-002676-PIP01-19

Treatment of corneal dystrophy

Day 30 discussion

Ophthalmology

3.1.37. EMEA-002658-PIP01-19

Treatment of uveal melanoma

Day 30 discussion

Ophthalmology / Oncology

3.1.38. 1-(2,2-diphenyltetrahydrofuran-3-yl)-n,n-dimethylmethanamine hydrochloride - Orphan - EMEA-002688-PIP01-19

Anavex Germany GmbH; Rett syndrome

Day 30 discussion

Other

3.1.39. Benzocaine - EMEA-002654-PIP02-19

Sore throat

Day 30 discussion

Oto-rhino-laryngology

3.1.40. Fasinumab - EMEA-002059-PIP02-19

Chronic pain / Chronic musculoskeletal pain / Chronic non-musculoskeletal pain / Treatment of chronic cancer pain in a palliative care setting / Treatment of moderate to severe chronic pain associated with osteoarthritis (OA) of the knee or hip in patients who achieve an inadequate response to or are intolerant to currently available analgesics (Adults only)

Day 30 discussion

Pain

3.1.41. Sodium alginate oligosaccharide - Orphan - EMEA-002321-PIP01-17

AlgiPharma AS; Symptomatic treatment of cystic fibrosis

Day 30 discussion

Pneumology – Allergology

3.1.42. Levocetirizine / montelukast - EMEA-002646-PIP01-19

Allergic rhinitis

Day 30 discussion

Pneumology - Allergology / Oto-rhino-laryngology

3.1.43. EMEA-002653-PIP01-19

Treatment of schizophrenia / Treatment of cognitive impairment associated with schizophrenia in patients 13 to <18 years of age

Day 30 discussion

Psychiatry

3.1.44. Canakinumab - EMEA-000060-PIP08-19

Treatment of lung carcinoma

Day 30 discussion

Oncology

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. Selumetinib - EMEA-C1-001585-PIP01-13-M03

AstraZeneca AB; Treatment of neurofibromatosis type 1

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Zoledronic acid - EMEA-000057-PIP01-07-M07

Novartis Europharm Limited; Osteoporosis / Glucocorticoid-induced osteoporosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.2. Albutrepenonacog alfa - Orphan - EMEA-001107-PIP01-10-M04

CSL Behring GmbH; Haemophilia B / Treatment of hereditary factor IX deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3.3. Alemtuzumab - EMEA-001072-PIP01-10-M03

Genzyme Europe B.V.; Multiple sclerosis / For paediatric patients with relapsing remitting multiple sclerosis (RRMS) with active disease on prior disease modifying treatment (DMT) defined by clinical or imaging features

Day 30 discussion

Neurology

3.3.4. Cannabidiol - Orphan - EMEA-001964-PIP01-16-M01

GW Pharma (International) B.V.; Lennox Gastaut syndrome / Dravet syndrome / Tuberous sclerosis complex / InfantilesSpasms / Treatment of seizures

Day 30 discussion

Neurology

3.3.5. Lenvatinib - EMEA-001119-PIP02-12-M06

Eisai GmbH; 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 30 discussion

Oncology

3.3.6. Lipegfilgrastim - EMEA-001019-PIP01-10-M05

UAB ”Sicor Biotech“; Treatment of chemotherapy-induced neutropenia / Prevention of chemotherapy-induced febrile neutropenia

Day 30 discussion

Oncology

3.3.7. Nivolumab - EMEA-001407-PIP01-12-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old / Treatment of a paediatric malignant solid tumour in paediatric patients from 6 months to less than 18 years old

Day 30 discussion

Oncology

3.3.8. Nivolumab - EMEA-001407-PIP02-15-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue / Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients with a relapsed or refractory non-Hodgkin lymphoma in the age group from 6 months to less than 18 years old / Treatment of paediatric patients from 6 months to less than 18 years of age with a recurrent or progressive high-grade glioma / Treatment of paediatric patients with a relapsed or refractory Hodgkin lymphoma in the age group from 5 years to less than 18 years

Day 30 discussion

Oncology

3.3.9. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M03

Helsinn Birex Pharmaceuticals Limited; Prevention of chemotherapy-induced nausea and vomiting

Day 30 discussion

Other

3.3.10. Vamorolone - Orphan - EMEA-001794-PIP02-16-M02

ReveraGen BioPharma Ltd.; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Other

3.3.11. Vosoritide - Orphan - EMEA-002033-PIP01-16-M01

BioMarin International Limited; Treatment of achondroplasia

Day 30 discussion

Other

3.3.12. Bupivacaine - EMEA-000877-PIP03-17-M01

Pacira Ltd; Postsurgical analgesia

Day 30 discussion

Pain

3.3.13. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-001715-PIP01-14-M03

Seqirus Netherlands B.V.; Influenza / Prevention of influenza

Day 30 discussion

Vaccines

3.3.14. (RS)-baclofen/ natrexone HCl /D-sorbitol - EMEA-002164-PIP01-17-M02

Pharnext S.A.; Treatment of Charcot-Marie-Tooth type 1A in symptomatic paediatric patients / Treatment of Charcot-Marie-Tooth disease type 1A in adults and paediatric patients

Day 30 discussion

Neurology

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 6 January 2020 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

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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.

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

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

6.1.1. Brimonidine - EMEA-15-2019

Allergan Pharmaceuticals International Limited; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema/ Treatment of geographic atrophy secondary to age-related macular degeneration

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.

6.1.2. Acetylcysteine - EMEA-17-2019

Zambon S.p.A.; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft-versus-host disease after [bone-marrow] transplantation/ maintenance treatment in moderate COPD in adult patients

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

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

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 2 medicinal products with recommended paediatric indications adopted in October 2019. These included Baqsimi (glucagon) and Toujeo (insulin glargine).

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

9.2.2. Committee for Advanced Therapies - Update on recent CAT discussions on paediatric developments of gene therapies in haemophilia

Summary of Committee discussion:

The PDCO members were informed about the latest discussions with CAT on paediatric developments of gene therapies in haemophilia.

9.2.3. Pharmacovigilance Risk Assessment Committee (PRAC)

Summary of Committee discussion:

The PDCO noted the questions adopted at the PRAC.

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. Extrapolation: extrapolation guidance template

Summary of Committee discussion:

A guidance template on how to structure the discussion on the use of extrapolation to support paediatric development was agreed by the committee.

9.4. Cooperation within the EU regulatory network

9.4.1. Draft recommendations from the joint EMA-HMA Big Data Taskforce

Summary of Committee discussion:

The Committee was debriefed on the joint EMA-HMA Big data task force.

9.5. Cooperation with International Regulators

9.5.1. Report from the Paediatric Cluster Teleconference

Summary of Committee discussion:

The Committee was informed on the dates for the cluster teleconferences for 2020.

9.5.2. ICH E11A – Clinical Trials in Paediatric Population - Briefing ahead of face-to-face to meeting in Singapore

Summary of Committee discussion:

The Committee was briefed on the ICH E11A meeting in Singapore.

9.5.3. Report from the pan-European Paediatric Formulary (PaedForm) project - EDQM

Summary of Committee discussion:

A European Directorate for the Quality of Medicines & HealthCare (EDQM) presented to the PDCO an overview of the activities surrounding the Paediatric Formulary and the anticipated next developments.

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

9.7.1. PDCO Work Plan 2020

PDCO Chair: Koenraad Norga;

Summary of Committee discussion:

The committee discussed topics to be selected for the PDCO work plan 2020.

9.8. Planning and reporting

No items

10. Any other business

10.1.1. Organisational changes

Summary of Committee discussion:

The Committee was updated on the organisational changes of the EMA.

10.1.2. Procedural improvement of PIP compliance checks

Summary of Committee discussion:

Some specific aspects of the SOP/H/3456 "Compliance check of an agreed paediatric investigation plan" have been discussed and endorsed by the PDCO.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of Committee discussion:

The Oncology breakout session discussed upcoming activities relative to paediatric oncology, and ongoing oncology PIPs.

11.1.2. Neonatology

Summary of Committee discussion:

The Neonatology breakout session discussed ongoing PIPs and Scientific Advices involving neonates.

11.1.3. Inventory

Summary of Committee discussion:

The Inventory breakout session discussed ongoing PIPs and Scientific Advices involving paediatric unmet needs.

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 DD Month YEAR meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium		- 2.3.9. Belimumab - EMEA-000520-PIP02-13-M03; - 3.1.11. Human immunoglobulin G2 isotype antibody to IL-33R - EMEA-002515-PIP01-18; - 3.2.7. Outer membrane vesicles (OMV) from neisseria meningitidis serogroup B (...) - EMEA-C-000139-PIP01-07-M03; - 3.3.14. Vilanterol trifenatate / umeclidinium bromide / fluticasone furoate - EMEA-002153-PIP01-17-M01;
Karl-Heinz Huemer	Member	Austria	No interests declared	N/A
Karen Van Malderen	Alternate	Belgium	No interests declared	N/A
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	None
Georgios Savva	Member	Cyprus	No interests declared	N/A
Kirstine Moll Harboe	Member	Denmark	No interests declared	N/A
Jana Lass	Alternate	Estonia	No interests declared	N/A
Ann Marie Totterman	Member	Finland	No interests declared	N/A
Pia Annunen	Alternate	Finland	No participation in final deliberations and voting on:	- 3.3.7. Nivolumab - EMEA-001407-PIP01-12-M02; - 3.3.8. Nivolumab - EMEA-001407-PIP02-15-M03
Sylvie	Member	France	No interests declared	N/A

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Benchetrit				
Dominique Ploin	Alternate	France	No interests declared	N/A
Sabine Scherer	Member	Germany	No interests declared	N/A
Yuansheng Sun	Alternate	Germany	No interests declared	N/A
Eleni Katsomiti	Member	Greece	No interests declared	N/A
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	N/A
Brian Aylward	Member	Ireland	No interests declared	N/A
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	None
Sigita Burokiene	Member	Lithuania	No interests declared	N/A
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	N/A
Herbert Lenicker	Alternate	Malta	No interests declared	N/A
Roel Bolt	Member	Netherlands	No interests declared	N/A
Maaike van Dartel	Alternate	Netherlands	No interests declared	N/A
Siri Wang	Member	Norway	No interests declared	N/A
Anette Solli Karlsen	Alternate	Norway	No interests declared	N/A
Marek Migdal	Member	Poland	No interests declared	N/A
Helena Fonseca	Member	Portugal	No interests declared	N/A
Hugo Tavares	Alternate	Portugal	No interests declared	N/A
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	N/A
Peter Sisovsky	Member	Slovakia	No interests declared	N/A
Peter Szitanyi	Alternate	Slovakia	No interests declared	N/A
Stefan Grosek	Member	Slovenia	No interests declared	N/A
Fernando de Andrés Trelles	Member	Spain	No interests declared	N/A
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	N/A
Ninna Gullberg	Member	Sweden	No interests declared	N/A
Eva Agurell	Alternate	Sweden	No interests declared	N/A
Angeliki Siapkara	Member	United Kingdom	No interests declared	N/A
Fernando	Member	Healthcare	No restrictions	None

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Cabanas		Professionals' Representative	applicable to this meeting	
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	N/A
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	3.3.17 Lumasiran sodium - Orphan - EMEA-002079-PIP01-16-M01
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	N/A
Doina Plesca	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	None
Günter Karl-Heinz Auerswald	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	None
Paola Baiardi	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	None
Michal Odermarsky	Member	Patients' Organisation Representative	No participation in final deliberations and voting on:	None
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	N/A
Dirk Leutner	Expert - in person*	EDQM	No interests declared	N/A
Maria Estela Moreno Martin	Expert - in person*	ES Expert	No interests declared	
Kristin Karlsson	Expert - via telephone*	MSWP Chair	No participation in discussion, final deliberations and voting on:	2.3.1. Edoxaban (tosylate) - EMEA-000788-PIP02-11-M09
Johanna Lähteenvuo	Expert - via telephone*		No interests declared	
Michel Kooijman	Expert - via telephone*		No interests declared	
Anette Stark	Expert - via telephone*		No interests declared	
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/