6 November 2020
EMA/PDCO/555707/2020
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 13-16 October 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

13 October 2020, 14:00- 19:00, Virtual meeting

14 October 2020, 08:30- 19:00, Virtual meeting

15 October 2020, 08:30- 19:00, Virtual meeting

16 October 2020, 08:30- 13:00, Virtual meeting

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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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. 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. 17 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

1.2. Adoption of agenda

PDCO agenda for 13-16 October 2020

The agenda of the PDCO meeting 13-16 October 2020 was adopted.

1.3. Adoption of the minutes

PDCO minutes for 1-4 September 2020

The minutes of the PDCO meeting 1-4 September 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. Recombinant human acid alpha-glucosidase - Orphan - EMEA-002447-PIP01-18

Amicus Therapeutics Europe Limited; Treatment of glycogen storage disease Type II (Pompe's disease)

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The applicant addressed the remaining issue in a response after Day 90. The PDCO agreed to the Applicant's reasoning.
The PDCO adopted a positive opinion, including a deferral.

2.1.2. Bispecific antibody binding to clotting factor IX and X - EMEA-002762-PIP02-20

Novo Nordisk A/S; Treatment of haemophilia A / Routine prophylaxis to prevent or reduce frequency of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) with or without factor VIII inhibitors

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee during its October 2020 plenary, the PDCO agreed on a PIP as well as the applicant's request for a waiver.

2.1.3. Garadacimab - EMEA-002726-PIP01-19

CSL Behring GmbH; Hereditary angioedema attacks (HAE)

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO adopted a positive Opinion on the PIP for Garadacimab (CSL312) for the routine prevention of HAE attacks. Garadacimab is a monoclonal antibody and inhibits activated Factor XII (FXIIa).

2.1.4. Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded (MC0518) - Orphan - EMEA-002706-PIP01-19

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of acute graft-versus-host disease

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed in October 2020 the responses of the applicant to the issues raised at D90. Therefore, the PDCO agreed a PIP with a waiver for a subset of the paediatric population and a deferral.

2.1.5. Alpha1-proteinase inhibitor (human) - EMEA-001312-PIP03-19

CSL Behring GmbH; Treatment of acute graft-versus-host disease (GVHD)

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The committee adopted a positive opinion and agreed on a PIP for the treatment of acute graft-versus-host disease in children. A waiver was agreed for children below a certain age based on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2.1.6. Bimekizumab - EMEA-002189-PIP03-19

UCB Biopharma SRL; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis) / Treatment of JIA (enthesitis-related arthritis [ERA] and juvenile psoriatic arthritis [JPsA]) in patients from ≥2 years to <18 years of age

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

All outstanding issues could be satisfactorily addressed between Day 90 and Day 120. The PDCO agreed on a PIP in the condition "treatment of chronic idiopathic arthritis". A waiver was granted in a subset of children due to lack of significant therapeutic benefit.

2.1.7. Cotadutide - EMEA-002712-PIP01-19

AstraZeneca AB; Treatment of non-cirrhotic non-alcoholic steatohepatitis (NASH) with fibrosis in children and adolescents

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

During its plenary on 16 October 2020, the PDCO adopted a positive Opinion for the PIP for cotadutide for the treatment of (non-cirrhotic) non-alcoholic steatohepatitis (NASH) with fibrosis.
A waiver was agreed in the paediatric population from birth to less than 8 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO granted a deferral for the paediatric development.

2.1.8. Dapirolizumab pegol - EMEA-002702-PIP01-19

UCB Biopharma SRL; Treatment of systemic lupus erythematosus (SLE) / Treatment of children and adolescents ≥5 years to <18 years of age with active SLE despite standard

therapy

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

Between Day 90 and Day 120 the applicant provided clarifications, and also addressed the PDCO's concerns in the draft PIP opinion satisfactorily.
The PDCO agreed on a waiver for a subset of children based on lack of significant therapeutic benefit of the medicinal product in the treatment of systemic lupus erythematosus in this age group.

2.1.9. Vonoprazan - EMEA-002703-PIP01-19

Phathom Pharmaceuticals, Inc.; Treatment of reflux oesophagitis, Treatment of *Helicobacter pylor*i infection / Eradication of *H. pylori* concurrently given with appropriate antibiotic therapy, Treatment of erosive reflux oesophagitis, Maintenance of healed erosive reflux oesophagitis

Day 120 opinion

Infectious Diseases / Gastroenterology-Hepatology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO adopted a positive opinion for the proposed product in children from birth to less than 18 years for the condition treatment of gastroesophageal reflux disease including a deferral. A waiver for the entire paediatric population from birth to less than 18 years for the condition treatment of *Helicobacter pylori* infection on the grounds lack of significant benefit was also adopted.

2.1.10. Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926) - Orphan - EMEA-002741-PIP01-20

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 120 opinion

Neurology

Summary of committee discussion:

The PDCO discussed the responses provided by the applicant after D90 and considered them overall satisfactory. The PDCO agreed a PIP for Treatment of Duchenne Muscular Dystrophy for Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926) with a waiver for the paediatric population below 2 years of age and a deferral with a request for further interaction during development to refine the subsequent stages with updated data considering this innovative compound in the children.

2.1.11. Lanadelumab - Orphan - EMEA-001864-PIP03-19

Shire Pharmaceuticals Ireland Limited (a Takeda company); Prevention of attacks of Idiopathic non-histaminergic angioedema (INHA) / Prevention of attacks of idiopathic

non-histaminergic angioedema (INHA)

Day 120 opinion

Other

Summary of committee discussion:

During its plenary on 16 October 2020, the PDCO adopted a positive Opinion on the PIP for lanadelumab for the prevention of attacks of Idiopathic non-histaminergic angioedema (INHA).

2.1.12. Adrenaline (epinephrine) - EMEA-002749-PIP01-19

ARS Pharmaceuticals IRL, Limited; Treatment of allergic reactions / The emergency treatment of allergic reactions, including anaphylaxis.

Day 120 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO adopted a positive opinion, including a waiver for children from birth to less than 1 year and a deferral for studies in the PIP.

2.1.13. BI 425809 - EMEA-002653-PIP01-19

Boehringer Ingelheim International GmbH; Treatment of Schizophrenia / Treatment of cognitive impairment associated with schizophrenia in patients 13 to <18 years of age

Day 120 opinion

Psychiatry

Summary of committee discussion:

The PDCO recommended granting a paediatric investigation plan for BI 425809 for the treatment of schizophrenia with a deferral and a waiver for an age subset based on the ground that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset.

2.1.14. Ezetimibe / Atorvastatin - EMEA-002852-PIP01-20

Sandoz B.V.; Prevention of cardiovascular events, Treatment of hypercholesterolemia / The combination of Atorvastatin and Ezetimibe is indicated for the treatment of hypercholesterolemia as substitution therapy in adult patients adequately controlled with the individual substances given concurrently at the same dose level as in the fixed dose combination, but as separate products., Atorvastatin/Ezetimibe Sandoz is indicated as substitution therapy to reduce the risk of cardiovascular events in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS), for adults receiving atorvastatin and ezetimibe concurrently at the same dose level

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 Atorvastatin / Ezetimibe for all subsets of the paediatric population (0 to 18 years of age) in the conditions of "Prevention of cardiovascular events" and "Treatment of hypercholesterolemia" 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.

2.1.15. Perindopril arginine / Indapamide / Amlodipine besilate - EMEA-002849-PIP01-20

Teva B.V.; Hypertension

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 Perindopril arginine / Amlodipine besilate / Indapamide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of 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.

2.1.16. Chimeric fibril-reactive IgG1k monoclonal antibody 11-1F4 - Orphan - EMEA-002791-PIP01-20

Real Regulatory Limited; AL Amyloidosis / Treatment of AL Amyloidosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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 chimeric fibril-reactive IgG1k monoclonal antibody 11-1F4 for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of systemic light chain amyloidosis.

2.1.17. Fasudil hydrochloride - EMEA-002841-PIP01-20

Aneuryst (Ireland) Limited; non-traumatic subarachnoid haemorrhage

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee taking into account the additional clarifications by the Applicant, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for fasudil hydrochloride for all subsets of the paediatric population (0 to 18 years of age) in the condition of "treatment of non-traumatic subarachnoid haemorrhage" on the grounds of lack of significant benefit as separate paediatric clinical trials are not feasible.
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.

2.1.18. Allogeneic BCMA-directed chimeric antigen receptor T Cell - EMEA-002834-PIP01-20

CRISPR Therapeutics AG; Multiple myeloma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the October 2020 plenary meeting. The PDCO confirmed all the conclusions that emerged at Day 30 and adopted a positive opinion on a product specific waiver for allogeneic B-cell maturation antigen (BCMA)-directed chimeric antigen receptor T cell for all subsets of the paediatric population (from birth to less than18 years of age) in the condition of treatment of multiple myeloma on the grounds that the condition only occurs in adult populations.
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.

2.1.19. Lurbinectedin - Orphan - EMEA-002846-PIP01-20

Pharma Mar, S.A.; Treatment of small cell lung cancer

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the October 2020 plenary meeting.

The PDCO confirmed all the conclusions that emerged at Day 30 and adopted a positive opinion on a product specific waiver for lurbinectedin for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of small cell lung cancer on the grounds that condition only occurs in adult populations.
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.

2.1.20. Serplulimab - EMEA-002859-PIP01-20

Shanghai Henlius Biotech, Inc.; Lung cancer (SCLC and NSCLC)

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the October 2020 plenary meeting.
The PDCO confirmed all the conclusions that emerged at Day 30 and adopted a positive opinion on a product specific waiver for serplulimab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung cancer (small cell and non-small cell lung cancer) on the grounds that condition only occurs in adult populations.
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.

2.1.21. 2-(6-azaspiro[2.5]octan-6-yl)-N-[2-(4,4-difluoropiperidin-1-yl)-6-methylpyrimidin-4-yl]- 4-[(2-hydroxyethanesulfonyl)amino]benzamide hydrochloride - EMEA-002843-PIP01-20

Amgen Europe BV; Treatment of ovarian cancer

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the October 2020 plenary meeting.
The PDCO confirmed all the conclusions that emerged at Day 30 and adopted a positive opinion on a product specific waiver for 2-(6-azaspiro[2.5]octan-6-yl)-N-[2-(4,4-difluoropiperidin-1-yl)-6-methylpyrimidin-4-yl]-4-[(2-hydroxyethanesulfonyl)amino]benzamide hydrochloride for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of ovarian cancer on the grounds that condition only occurs in adult populations.
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.

2.1.22. 2-(6-azaspiro[2.5]octan-6-yl)-N-[2-(4,4-difluoropiperidin-1-yl)-6-methylpyrimidin-4-yl]- 4-[(2-hydroxyethanesulfonyl)amino]benzamide hydrochloride - EMEA-002843-PIP02-20

Amgen Europe BV; Treatment of breast cancer

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the October 2020 plenary meeting. The PDCO confirmed all the conclusions that emerged at Day 30 and adopted a positive opinion on a product specific waiver for 2-(6-azaspiro[2.5]octan-6-yl)-N-[2-(4,4-difluoropiperidin-1-yl)-6-methylpyrimidin-4-yl]-4-[(2-hydroxyethanesulfonyl)amino]benzamide hydrochloride for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of breast cancer on the grounds that condition only occurs in adult populations.
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.

2.1.23. (1R,2R,3S,4S,5R,6S)-cyclohexane-1,2,3,4,5,6-hexayl-hexakis (dihydrogen phosphate) - Orphan - EMEA-002854-PIP01-20

Sanifit Therapeutics S.A.; Treatment of calciphylaxis

Day 60 opinion

Other

Summary of committee discussion:

The PDCO's view expressed at Day 30 was re-discussed and endorsed.
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 (1R,2R,3S,4S,5R,6S)-cyclohexane-1,2,3,4,5,6-hexayl-hexakis (dihydrogen phosphate) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of calciphylaxis.

2.1.24. Dapagliflozin - EMEA-000694-PIP05-20

AstraZeneca AB; COVID-19, virus identified, COVID-19, virus not identified

Day 60 opinion

Other

Summary of committee discussion:

During its plenary on 16 October 2020, the PDCO adopted a positive Opinion on the applicant's proposal for a full waiver.
An Opinion on a full product specific waiver has been adopted in children from birth to less than 18 years of age for dapagliflozin in the condition treatment of coronavirus disease 2019 (COVID-19) on the ground of lack of significant therapeutic benefit as clinical studies(s) are not feasible.

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. Sofosbuvir / Voxilaprevir / Velpatasvir - EMEA-C-001822-PIP01-15-M01

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO adopted on 16 October 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0006/2020) of 6 July 2020.

2.2.2. Odevixibat - EMEA-C1-002054-PIP01-16-M02

Albireo AB; Treatment of Progressive familial intrahepatic cholestasis (PFIC)

Day 30 letter

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO discussed the compliance check request. The PDCO concluded positively on this partial compliance check.

2.2.3. Regorafenib - EMEA-C2-001178-PIP01-11-M05

Bayer AG; Treatment of all conditions contained in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 30 letter

Oncology

Summary of committee discussion:

The PDCO discussed the completed PIP study(ies) 3, 4 and 5 taking into account feedback provided by the applicant after the D30 discussion and considered that these are compliant with the latest Agency's Decision P/0141/2020 of 17 April 2020.

The PDCO finalised this partially completed compliance procedure on 16/10/2020.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Bilastine - EMEA-000347-PIP02-16-M02

Faes Farma S.A.; Treatment of allergic conjunctivitis

Day 60 opinion

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/0408/2019 of 04 December 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Tralokinumab - EMEA-001900-PIP02-17-M04

LEO Pharma A/S; Treatment of 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 therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0145/2020 of 15 April 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Canagliflozin (1s)-1,5-anhydro-1-[3-[[5-(4-fluorophenyl)-2-thienyl]methyl]-4-methylphenyl]-D-glucitol hemihydrate - EMEA-001030-PIP01-10-M08

Janssen-Cilag International NV; Type 2 Diabetes Mellitus / Treatment of Type 2 Diabetes Mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

During its plenary on 16 October 2020, the PDCO adopted a positive Opinion on the modification request for the PIP for the SGLT-2 transporter inhibitor canagliflozin for the treatment of type 2 diabetes mellitus.
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/0205/2017 of 09/08/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Drospirenone / Estetrol - EMEA-001332-PIP01-12-M04

Estetra SPRL; Prevention of pregnancy

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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/0256/2020 issued on 15 July 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Lixisenatide - EMEA-000916-PIP01-10-M07

sanofi-aventis R&D; Type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

During its plenary on 16 October 2020, the PDCO adopted a positive Opinion on the requested modification to transform the PIP into a full waiver, for the GLP-1 receptor agonist lixisenatide for the treatment of type 2 diabetes mellitus.
The product specific waiver has basically been extended to a subset of the paediatric population for the treatment of type 2 diabetes mellitus , on the grounds of lack of significant therapeutic benefit over existing treatments.
A product specific waiver in a subset of the paediatric population for the treatment of type 2 diabetes mellitus has been adopted already during the initial PIP agreement for this product, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset (Agency decision P/18/2011 from 24/01/2011, and subsequent modifications thereof).
In conclusion, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0380/2018 of 07/12/2018).
The new PDCO Opinion supersedes the previous PDCO Opinion.

2.3.6. Olipudase alfa - Orphan - EMEA-001600-PIP01-13-M02

Genzyme Europe B.V.; Treatment of Niemann-Pick disease

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO's view expressed at Day 30 were endorsed and 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/0258/2018 of 15/08/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Recombinant parathyroid hormone: rhPTH (1-84) - Orphan - EMEA-001526-PIP01-13-M04

Shire Pharmaceuticals Ireland Limited; Hypoparathyroidism / Treatment of hypoparathyroidism

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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.

2.3.8. Potassium chloride / Sodium chloride / Ascorbic acid / sodium sulfate / Sodium ascorbate / Polyethylene Glycol 3350 - EMEA-001705-PIP02-15-M03

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 60 opinion

Gastroenterology-Hepatology

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/0196/2019 of 20/05/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Teduglutide - Orphan - EMEA-000482-PIP01-08-M06

Shire Pharmaceuticals Ireland Limited; Diagnosis - Other and unspecified post surgical non absorption - Syndrome Short Bowel / Treatment of Short Bowel Syndrome

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 therefore adopted a favourable Opinion on the modification of the agreed PIP as set

in the Agency's latest decision (P/0410/2019 issued on 6 December 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Vonicog alfa - EMEA-001164-PIP01-11-M04

Baxalta Innovations GmbH; Von Willebrand disease / Prevention and treatment of bleeding episodes and for surgical and invasive procedures in paediatric patients (less than 18 years of age) with von Willebrand disease

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the responses received from the applicant.
A positive opinion was adopted at D60.

2.3.11. Apremilast - EMEA-000715-PIP03-11-M06

Amgen Europe B.V.; Treatment of psoriasis

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 therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0163/2018 issued on 15 June 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Ustekinumab - EMEA-000311-PIP03-11-M06

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis)/ Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed the proposed modification taking into account the clarifications provided by the applicant on the pending issues identified at D30, their comments on the draft opinion. All pending issues were considered solved.
In conclusion, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0109/2017 of 11/04/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Tazobactam / ceftolozane - EMEA-001142-PIP01-11-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of urinary tract infections, Treatment of intra-abdominal infections

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 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/0154/2019 of 17/04/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Tedizolid - EMEA-001379-PIP01-12-M05

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

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO accepted some of the modifications proposed by the applicant and issued a positive opinion.

2.3.15. Tenofovir (disoproxil fumarate) - EMEA-000533-PIP01-08-M09

Gilead Sciences International Limited; Treatment of chronic viral hepatitis B

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 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/0121/2020 issued on 20 March 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Tenofovir Alafenamide / Emtricitabine / Elvitegravir / Cobicistat - EMEA-001460-PIP01-13-M05

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection / For the treatment of HIV-1 infection in paediatric patients from 6 years to less than 18 years.

Day 60 opinion

Infectious Diseases

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/0202/2020 of 27/5/2020)
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16-M03

Zogenix International Ltd; Dravet syndrome / The adjunctive treatment of seizures in paediatric patients at least 1 year of age with Dravet syndrome

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.

2.3.18. Humanised anti-IL-6 receptor (IL-6R) monoclonal antibody (satralizumab) - Orphan - EMEA-001625-PIP01-14-M06

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion made at D30. The Committee also noted the additional clarifications provided by the applicant on the requested changes to the timelines, which could be followed.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0204/2020 of 16 June 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Nivolumab - EMEA-001407-PIP01-12-M03

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 60 opinion

Oncology

Summary of committee discussion:

The PDCO's views expressed at D30 were endorsed and 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/0026/2020 of 09/01/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Nivolumab - EMEA-001407-PIP02-15-M04

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 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the request for modification taking into account the clarifications provided by the applicant after D30.
The PDCO considered that all 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 (of P/0027/2020 09 January 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.21. Veliparib - EMEA-000499-PIP02-10-M01

AbbVie Ltd; Treatment of high-grade glioma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the October plenary meeting.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be acceptable.
Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/212/2011 of 2 September 2011).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M10

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

Day 60 opinion

Other

Summary of committee discussion:

The PDCO re-discussed this application taking into consideration the outstanding issues identified at D30.
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.

2.3.23. Palovarotene - Orphan - EMEA-001662-PIP01-14-M04

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 60 opinion

Other

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the October 2020 plenary meeting.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be acceptable.
Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0144/2020 of 15 April 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Birch pollen extract (Betula verrucosa) - EMEA-001879-PIP01-15-M02

ALK-Abelló A/S; / Treatment of tree pollen allergic rhinitis / rhino-conjunctivitis

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The committee's views expressed on day 30 were endorsed and re-discussed, taking into account the additional clarifications, which were considered agreeable.
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/0030/2018 of 30 January 2018)
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.25. Live attenuated, chimeric dengue virus, serotype 4 / Live, attenuated, chimeric dengue virus, serotype 1 / Live, attenuated, chimeric dengue virus, serotype 3 / Live, attenuated dengue virus, serotype 2 - EMEA-001888-PIP01-15-M01

Takeda Vaccines, Inc.; Prevention of dengue fever

Day 60 opinion

Vaccines

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan and the additional clarifications received after D30, 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/0180/2017 of 3/7/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.26. Inebilizumab - Orphan - EMEA-001911-PIP01-15-M03

Viela Bio, Inc; Neuromyelitis optica spectrum disorders

Day 30 opinion

Neurology

Summary of committee discussion:

The PDCO adopted a positive opinion at Day 30.

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

2.6.1. Vedolizumab - EMEA-000645-PIP01-09-M07

Takeda Pharma A/S; Crohn's Disease / Ulcerative colitis

Day 30 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

Procedure adopted via written procedure 25 September 2020.

2.7. Partial Compliance Checks completed by EMA

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

2.7.1. Cemiplimab - EMEA-C1-002007-PIP02-17

Regeneron Ireland DAC.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 30 letter

2.7.2. Pneumococcal polyssacharide serotype 5, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 19A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 33F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 9V, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 8, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 19F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 23F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 10A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 7F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 15B, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 18C, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 6B, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 6A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 3, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 4, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 11A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 1, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 12F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 22F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 14, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate - EMEA-C1-002330-PIP01-18

Pfizer Europe MA EEIG; Disease caused by *Streptococcus pneumoniae*

Day 60 letter

Vaccines

2.7.3. Tecovirimat monohydrate - EMEA-C1-001205-PIP02-19

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

Day 30 letter

Infectious Diseases

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. Venglustat - Orphan - EMEA-001716-PIP05-20

Genzyme Europe B.V.; Treatment of autosomal dominant polycystic kidney disease/ Indicated for long term treatment to slow the progression of cysts development in paediatric patients from 12 years to <18 years old with autosomal dominant polycystic kidney disease

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

3.1.2. Etrasimod L-arginine - EMEA-002713-PIP01-19

Treatment of ulcerative colitis / Treatment of moderately or severely active ulcerative colitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.3. Etranacogene dezaparvovec - Orphan - EMEA-002722-PIP01-19

uniQure biopharma B.V.; Treatment of Haemophilia B

Day 90 discussion

Haematology-Hemostaseology

3.1.4. Olinciguat - EMEA-002759-PIP01-19

Treatment of Sickle Cell Disease (SCD)

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Voxelotor - Orphan - EMEA-002356-PIP02-20

Synteract GmbH; Sickle cell disease

Day 90 discussion

Haematology-Hemostaseology

3.1.6. EMEA-002742-PIP01-19

Immunocompromised patients due to having received a solid organ transplant or recent Hematopoietic Stem Cell Transplantation or chemotherapy are high risk of hospitalized patients who are infected with Parainfluenza viral pneumonia and there is no authorised medicinal product for Parainfluenza infection

Day 90 discussion

Infectious Diseases

3.1.7. EMEA-002755-PIP01-19

Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 90 discussion

Infectious Diseases

3.1.8. Sulbactam / durlobactam - EMEA-002807-PIP01-20

Treatment of infections due to organisms of the *Acinetobacter baumannii*-calcoaceticus complex / Treatment of infections due to *Acinetobacter baumannii*-calcoaceticus complex in patients with limited treatment options

Day 90 discussion

Infectious Diseases

3.1.9. EMEA-002635-PIP01-19

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

Day 90 discussion

Oncology

3.1.10. EMEA-002763-PIP01-20

Paediatric low grade glioma / Relapsed or refractory paediatric low grade glioma in adolescents and children 6 months of age and older

Day 90 discussion

Oncology

3.1.11. Tabelecleucel - Orphan - EMEA-002025-PIP04-19

Atara Biotherapeutics, Inc.; Treatment of Epstein-Barr virus associated post-transplant lymphoproliferative disorder / Treatment of allogeneic haematopoietic cell transplant patients with Epstein-Barr virus associated post-transplant lymphoproliferative disease who have received one prior therapy, Treatment of solid organ transplant patients with Epstein-Barr

virus associated post-transplant lymphoproliferative disease who have received one prior therapy

Day 90 discussion

Oncology

3.1.12. EMEA-002814-PIP01-20

Invasive disease caused by *Neisseria meningitidis* group A, B, C, W and Y from 2 months of age.

Day 90 discussion

Vaccines

3.1.13. Ralinepag - Orphan - EMEA-002432-PIP02-20

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension / Treatment of pulmonary arterial hypertension WHO Group I to improve exercise capacity and to delay clinical worsening

Day 60 discussion

Cardiovascular Diseases

3.1.14. Sotatercept - EMEA-002756-PIP01-19

Pulmonary arterial hypertension

Day 60 discussion

Cardiovascular Diseases

3.1.15. Indometacin / Levonorgestrel - EMEA-002820-PIP01-20

Contraceptive management

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.16. Pioglitazone hydrochloride / Spironolactone / Metformin hydrochloride - EMEA-002187-PIP01-17

Treatment of polycystic ovary syndrome (PCOS) / Treatment of adolescent polycystic ovary syndrome (PCOS) in post-menarche adolescents <18 yrs and young adult women ≥18 yrs and <24.0 yrs

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.17. EMEA-002845-PIP01-20

Treatment of mucopolysaccharidosis II (Hunter syndrome) / Treatment of mucopolysaccharidosis II (MPS II, Hunter syndrome)

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. Maralixibat Chloride - EMEA-001475-PIP04-20

Biliary atresia (BA) / Treatment of Biliary atresia

Day 60 discussion

Gastroenterology-Hepatology

3.1.19. Recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8 - Orphan - EMEA-002856-PIP01-20

Allakos Inc; Treatment of eosinophilic gastrointestinal inflammatory disorders / Treatment of eosinophilic esophagitis / Treatment of eosinophilic gastritis and/or eosinophilic gastroenteritis

Day 60 discussion

Gastroenterology-Hepatology

3.1.20. Pegfilgrastim - EMEA-002671-PIP02-20

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 peadiatric patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 60 discussion

Haematology-Hemostaseology

3.1.21. EMEA-002350-PIP03-20

Treatment of systemic lupus erythematosus (SLE) / Treatment of lupus nephritis (LN) despite receiving SoC, Treatment of systemic lupus erythematosus (SLE) despite receiving standard of care (SoC)

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.22. Iscalimab - EMEA-002842-PIP01-20

Prophylaxis against transplant rejection / Prophylaxis of graft rejection in paediatric renal transplant patients 2 years of age and older

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.23. Rimegepant - EMEA-002812-PIP02-20

Acute treatment of migraine

Day 60 discussion

Neurology

3.1.24. Viltolarsen - Orphan - EMEA-002853-PIP01-20

NS Pharma, Inc.; Treatment of Duchenne muscular dystrophy / Treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

Day 60 discussion

Neurology

3.1.25. Talazoparib - EMEA-002066-PIP01-20

Treatment of Ewing sarcoma

Day 60 discussion

Oncology

3.1.26. Flotetuzumab - EMEA-002855-PIP01-20

Acute myeloid leukemia (AML)

Day 60 discussion

Oncology / Haematology-Hemostaseology

3.1.27. Atropine Sulfate - EMEA-002858-PIP01-20

Prevention of myopic progression

Day 60 discussion

Ophthalmology

3.1.28. Udonitrectag lysine - Orphan - EMEA-002848-PIP01-20

Recordati Rare Diseases; neurotrophic keratitis

Day 60 discussion

Ophthalmology

3.1.29. Garetosmab - Orphan - EMEA-002736-PIP01-19

Regeneron Ireland DAC; Treatment of Fibrodysplasia Ossificans Progressiva

Day 60 discussion

Other

3.1.30. Ravulizumab - EMEA-001943-PIP02-20

Treatment in haematopoietic stem cell transplantation

Day 60 discussion

Uro-nephrology / Haematology-Hemostaseology

3.1.31. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20

Prevention of disease caused by *Streptococcus pneumoniae*

Day 60 discussion

Vaccines

3.1.32. EMEA-002870-PIP01-20

Induction of general anesthesia in adults and children

Day 30 discussion

Anaesthesiology

3.1.33. Bisoprolol / ramipril - EMEA-002860-PIP01-20

Essential hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.34. Dapagliflozin - EMEA-000694-PIP06-20

Prevention of hospitalisation for heart failure and cardiovascular death in adults who have had a myocardial infarction

Day 30 discussion

Cardiovascular Diseases

3.1.35. Ziltivekimab - EMEA-002840-PIP01-20

Prevention of cardiovascular events in patients with atherosclerosis

Day 30 discussion

Cardiovascular Diseases

3.1.36. Allogeneic skin-derived ABCB5-positive mesenchymal stem cells - Orphan - EMEA-002875-PIP01-20

RHEACELL GmbH & Co. KG; Treatment of epidermolysis bullosa / Treatment of recessive dystrophic epidermolysis bullosa and junctional epidermolysis bullosa

Day 30 discussion

Dermatology

3.1.37. EMEA-002597-PIP03-20

Treatment of pemphigus

Day 30 discussion

Dermatology

3.1.38. Infigratinib - EMEA-002594-PIP02-20

Treatment of achondroplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.39. Calmangfodipir - EMEA-002865-PIP01-20

Paracetamol overdose in adolescents (more than 12 years) for increased risk (late presenters) to develop hepatoxicity

Day 30 discussion

Gastroenterology-Hepatology

3.1.40. Lanifibranor - EMEA-002872-PIP01-20

NASH / Treatment of NASH

Day 30 discussion

Gastroenterology-Hepatology

3.1.41. Odevixibat - Orphan - EMEA-002054-PIP03-20

Albireo AB; Alagille syndrome

Day 30 discussion

Gastroenterology-Hepatology

3.1.42. Vedolizumab - EMEA-000645-PIP04-20

Pouchitis (in patients who underwent proctocolectomy and ileal-pouch anal anastomosis for ulcerative colitis)

Day 30 discussion

Gastroenterology-Hepatology

3.1.43. EMEA-002863-PIP01-20

Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Haematology-Hemostaseology

3.1.44. Obinutuzumab - Orphan - EMEA-001207-PIP03-20

Roche Registration GmbH; Treatment of membranous glomerulonephritis also known as membranous nephropathy (MN)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.45. EMEA-002866-PIP01-20

Treatment of idiopathic Parkinson's disease (PD)

Day 30 discussion

Neurology

3.1.46. Tauroursodeoxycholic acid / Sodium phenylbutyrate - Orphan - EMEA-002876-PIP01-20

Drug Development and Regulation SL; Treatment of Amyotrophic Lateral Sclerosis

Day 30 discussion

Neurology

3.1.47. Autologous CD4+ and CD8+ T cells transduced with a lentiviral vector containing an affinity-enhanced T-cell receptor targeting the MAGE-A4 antigen - Orphan - EMEA-002867-PIP01-20

Adaptimmune Ltd; Soft Tissue Sarcoma / Myxoid/ Round Cell Liposarcoma / Synovial Sarcoma

Day 30 discussion

Oncology

3.1.48. Delolimogene mupadenorepvec - Orphan - EMEA-002864-PIP01-20

Lokon Pharma AB; Colorectal cancer / Ovarian cancer / Pancreatic cancer / Malignant melanoma / Biliary cancer

Day 30 discussion

Oncology

3.1.49. Magrolimab - Orphan - EMEA-002819-PIP01-20

Gilead Sciences International Ltd; Treatment of acute myeloid leukaemia, Treatment of myelodysplastic syndromes (including juvenile myelomonocytic leukaemia)

Day 30 discussion

Oncology

3.1.50. Pralsetinib - EMEA-002575-PIP02-20

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of pediatric patients from 12 years to less than 18 years of age with RET-altered solid tumors who require systemic therapy and have no satisfactory alternative treatment options

Day 30 discussion

Oncology

3.1.51. Tipifarnib - EMEA-002871-PIP01-20

Treatment of HRAS mutant head and neck squamous cell carcinoma (Malignant Neoplasms of Head, Face, and Neck)

Day 30 discussion

Oncology

3.1.52. Doconexent (Docosahexaenoic acid, DHA) - Orphan - EMEA-002808-PIP01-20

Natac Pharma S.L.; Retinitis Pigmentosa

Day 30 discussion

Ophthalmology

3.1.53. (2S,4S)-2-(4-Carboxyphenyl)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-1-ium chloride—water (1/1) - Orphan - EMEA-002705-PIP03-20

Novartis Europharm Limited; Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Other / Haematology-Hemostaseology

3.1.54. Autologous selected renal cells - EMEA-002844-PIP01-20

Treatment of chronic kidney disease

Day 30 discussion

Uro-nephrology

3.1.55. EMEA-002873-PIP01-20

Active immunisation for the prevention of disease caused by chikungunya virus

Day 30 discussion

Vaccines

3.1.56. EMEA-002862-PIP01-20

Prevention of COVID-19

Day 30 discussion

Article 7; PIP and Deferral and Waiver

Vaccines

3.1.57. EMEA-002861-PIP02-20

SARS-CoV-2 (COVID-19) infection prevention

Day 30 discussion

Article 7; PIP and Deferral and Waiver

Vaccines

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

Grifols Therapeutics LLC; Treatment of primary immunodeficiency

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Captopril - EMEA-001544-PIP01-13-M02

Proveca Pharma Limited; Heart failure / Treatment of heart failure in children from birth to 18 years

Day 30 discussion

Cardiovascular Diseases

3.3.2. Landiolol (hydrochloride) - EMEA-001150-PIP02-13-M03

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrythmias

Day 30 discussion

Cardiovascular Diseases

3.3.3. Fluciclovine (18F) - Orphan - EMEA-001644-PIP02-14-M02

Blue Earth Diagnostics Ireland Ltd; Diagnosis of amino acid metabolism in solid malignant tumours / Diagnosis of primary and recurrent brain tumours

Day 30 discussion

Diagnostic / Oncology

3.3.4. Cotadutide - EMEA-002287-PIP01-17-M01

AstraZeneca AB; Treatment of Type 2 Diabetes Mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Ladarixin - EMEA-002642-PIP01-19-M02

Dompé farmaceutici S.p.A; Treatment of type 1 diabetes / Treatment of new-onset type 1 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Testosterone - EMEA-001529-PIP02-14-M03

Acerus Biopharma Inc.; Male hypogonadism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Naloxegol (as naloxegol oxalate) - EMEA-001146-PIP01-11-M06

Kyowa Kirin Pharmaceutical Development Limited; Treatment of opioid-induced constipation

Day 30 discussion

Gastroenterology-Hepatology

3.3.8. Avatrombopag maleate - EMEA-001136-PIP02-19-M01

Dova Pharmaceuticals Ireland Limited; Treatment of chemotherapy-induced thrombocytopenia/ Treatment of chemotherapy-induced thrombocytopenia in paediatric patients from 28 days to less than 18 years of age receiving myelosuppressive chemotherapy for treatment of solid tumours

Day 30 discussion

Haematology-Hemostaseology

3.3.9. Eltrombopag - EMEA-000170-PIP03-13-M04

Novartis Europharm Limited; Bone Marrow Depression and Hypoplastic Anaemia / Treatment of cytopenias in paediatric patients with severe aplastic anaemia who are not receiving hematopoietic stem cell transplant

Day 30 discussion

Haematology-Hemostaseology

3.3.10. Aztreonam - Orphan - EMEA-000827-PIP01-09-M05

Gilead Sciences International Ltd.; Treatment of *Pseudomonas aeruginosa* pulmonary infection / colonisation in patients with cystic fibrosis

Day 30 discussion

Infectious Diseases

3.3.11. Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of HIV-1 infection / in combination with other ARV medicinal products for the treatment of HIV-1 infected adults and children from 3 years of age without known mutations associated with resistance to atazanavir.

Day 30 discussion

Infectious Diseases

3.3.12. Eculizumab - Orphan - EMEA-000876-PIP03-14-M05

Alexion Europe SAS; Neuromyelitis optica spectrum disorders / Treatment of relapsing neuromyelitis optica spectrum disorders (NMOSD) in the paediatric population

Day 30 discussion

Neurology

3.3.13. Peramivir - EMEA-001856-PIP02-16-M01

Biocryst Ireland Limited; Treatment of influenza

Day 30 discussion

Infectious Diseases

The procedure was withdrawn on the 13/10/20 before the discussion took place.

3.3.14. Ocrelizumab - EMEA-000310-PIP03-10-M04

Roche Registration GmbH; Treatment of Multiple Sclerosis / Treatment of relapsing remitting multiple sclerosis

Day 30 discussion

Neurology

3.3.15. Avelumab - EMEA-001849-PIP02-15-M03

Merck Healthcare GmbH; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumors, haematopoetic and lymphoid tissue neoplams) / Treatment of malignant neoplasms of lymphoid tissue / Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients from 2 years to less than 18 years old with a relapsed or refractory solid tumour or with a solid tumour as part of the first line treatment

Day 30 discussion

Oncology

3.3.16. Brigatinib - EMEA-002296-PIP01-17-M02

Takeda Pharm A/S; Treatment of non-small cell lung cancer (NSCLC)/ treatment of inflammatory myofibroblastic tumors (IMT), Treatment of anaplastic large cell lymphoma (ALCL) / 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 30 discussion

Oncology

3.3.17. Burosumab - Orphan - EMEA-001659-PIP01-15-M05

Kyowa Kirin Holdings B.V.; X-linked Hypophosphataemia / Treatment of X-linked Hypophosphataemia

Day 30 discussion

Other

3.3.18. Ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M10

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

Day 30 discussion

Other

3.3.19. Levofloxacin hemihydrate - EMEA-001211-PIP01-11-M02

Chiesi Farmaceutici S.p.A.; Treatment of cystic fibrosis

Day 30 discussion

Pneumology - Allergology

3.3.20. Calcifediol - EMEA-002093-PIP02-17-M01

Vifor Fresenius Medical Care Renal Pharma France; endocrine, nutritional and metabolic diseases, and immunity disorders

Day 30 discussion

Uro-nephrology

3.3.21. Cholera vaccine, recombinant, live, oral (strain CVD 103-HgR) - EMEA-001490-PIP01-13-M02

Emergent Netherlands B.V.; Cholera disease caused by *Vibrio cholerae* serogroup O1 / Prevention of disease caused by *Vibrio cholerae* serogroup O1

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 13 October 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

No item

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.

PDCO representatives at SAWP (elected by the PDCO) are Dr Karl-Heinz Huemer, Dr Sara Galluzzo, as PDCO / SAWP members and Dr Johanna Wernsperger, Dr Dina Apele-Freimane as their PDCO / SAWP Alternates respectively.

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. Ensifentrine - EMEA-08-2020

Verona Pharma plc; 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 of chronic obstructive pulmonary disease

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: asthma and cystic fibrosis.

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 item

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

Nora Kriauzaitė has been appointed as member of the PDCO by the European Commission to represent health professionals.

Marleen Renard has been appointed as member of the PDCO from Belgium.

Eva Agurell has changed membership from alternate to member of the PDCO from Sweden.

Roel Bolt has resigned as member of the PDCO.

Melinda Sobor has resigned as alternate of the PDCO.

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 six medicinal products with recommended paediatric indications adopted in September 2020 by CHMP. These include Flucelvax Tetra (influenza vaccine) Fycompa (perampanel), Kalydeco (ivacaftor), Symkevi

(tezacaftor/ivacaftor), Velphoro (sucroferric oxyhydroxide) and Zavicefta (ceftazidime/avibactam).

The list of procedures with paediatric indications to be evaluated by the CHMP, starting in July 2020, was presented to the PDCO members.

9.2.2. Interactions between CAT and PDCO

Outcome of the brainstorming meeting of 17 September 2020

Summary of committee discussion:

The Committee was informed about the outcome of the brainstorming meeting that took place to discuss improvement of interactions between CAT and PDCO.

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 (NcWG) identified the products which will require 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.

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA)

Summary of committee discussion:

The committee received an update on the 2020 annual meeting of the members and Coordinating Group of the European network of paediatric research at the EMA (Enpr-EMA). Moreover, PDCO members were reminded of the possibility to reach out to Enpr-EMA member networks to seek input to general questions on paediatric conditions in the context of PIP assessments. The committee was encouraged to make use of the pool of expertise available in Enpr-EMA.

9.5. Cooperation with International Regulators

9.5.1. FDA cluster teleconference

Summary of committee discussion:

The Committee was informed about the discussions at the Paediatric Cluster teleconference.

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

9.6.1. Multi-regional clinical trials (MRCT) centre project on global paediatric trials

Summary of committee discussion:

The PDCO was updated about the MRCT project on promoting global clinical research in children.

9.7. PDCO work plan

No item

9.8. Planning and reporting

No item

10. Any other business

10.1. Covid-19 update

Summary of committee discussion:

The PDCO was updated on the most relevant aspects of therapeutics and vaccines for COVID-19.

10.2. Disclosure of confidential information - refresher training

Summary of committee discussion:

The committee was reminded on the main principles from the policy on handling of competing interests of scientific committees' members and alternates.

Key aspects for handling confidential information were mentioned and reference was made to the process to be followed in case of unintended disclosure of confidential information.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The group discussed ongoing and planned activities related to paediatric oncology.

11.1.2. Neonatology

Summary of committee discussion:

Discussion on the upcoming workshop of the International Neonatal Consortium.

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 13-16 October 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	No restrictions applicable to this meeting	
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Georgios Savva	Member	Cyprus	No interests declared	
Lucie Kravackova	Member	Czech Republic	No interests declared	
Petra Dominikova	Alternate	Czech Republic	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Ann Marie Totterman	Member	Finland	No interests declared	
Sylvie Benchetrit	Member	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member (Vice-Chair)	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Mountaki	Alternate	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-Freimane	Member	Latvia	No restrictions applicable to this meeting	
Sigita Burokiene	Member	Lithuania	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Herbert Lenicker	Alternate	Malta	No interests declared	
Marleen Renard	Member	Netherlands	No participation in final deliberations and voting on the following products:	3.3.16. Avelumab - EMEA-001849-PIP02-15 -M03
Maaike van Dartel	Alternate	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	
Eva Agurell	Alternate	Sweden	No interests declared	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jaroslav Sterba	Member	Patients' Organisation Representative	No interests declared	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Alternate	Patients' Organisation	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
		Representative		
María Estela Moreno Martín*	Expert via Adobe	AEMPS	No interests declared	
Lutz Wiesner*	Expert via Adobe	BfArM	No interests declared	
Emmely de Vries*	Expert via Adobe	Netherlands	No interests declared	
Michal Odermarsky*	Expert via Adobe	EC	No restrictions applicable to meetings	
Daniela Philadelphy	SAWP Coordinator	Austria	No interests declared	
Philipp Janesch	SAWP Coordinator	Austria	No interests declared	
Andreas Kirisits	SAWP Coordinator	Austria	No interests declared	
Rune Kjeken	SAWP Coordinator	Norway	No restrictions applicable to meetings	
Ole Weis Bjerrum	SAWP Coordinator	DKMA	No restrictions applicable to meetings	
Anja Schiel	SAWP Coordinator	NOMA	No interests declared	
	Observer	Health Canada		
	Observer	Health Canada		
	EC Representative	European Commission		
A representative from the European Commission attended the meeting				
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/