7 December 2020
EMA/PDCO/622400/2020
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 8-11 December 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

8 -11 December 2020, 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).

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1. Introductions

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

The Chairperson opened the meeting by welcoming all participants. Due to the current coronavirus (COVID-19) outbreak, and the associated EMA Business Continuity Plan (BCP), the meeting was held remotely.

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.

Due to restricted involvement, the Chair Koenraad Norga was replaced by the Vice-Chair Sabine Scherer for the discussion on agenda topic 2.3.32, 3.1.14, 3.1.31, 3.1.50, 3.1.54, 3.3.36.

1.2. Adoption of agenda

PDCO agenda for 8-11 December 2020

The agenda of the PDCO meeting 8-11 December 2020 was adopted.

1.3. Adoption of the minutes

PDCO minutes for 10-13 November 2020

The minutes of the PDCO meeting 10-13 November 2020 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. 3-((1R,3s,5S)-3-((7-((5-methyl-1H-pyrazol-3-yl)amino)-1,6-naphthyridin-5-yl)amino)-8-azabicyclo[3.2.1]octan-8-yl)propanenitrile (TD-1473) - EMEA-002757-PIP01-19

Theravance Biopharma Ireland Limited; Ulcerative colitis

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO agreed on a positive opinion for this PIP for TD-1473 for the treatment of ulcerative colitis in children from 2 years of age. The paediatric programme is deferred. A waiver was granted for a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

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

Roche Registration GmbH; Systemic lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed in December 2020 the responses of the applicant to the issues raised by the PDCO at D90.
Therefore, the PDCO agreed a PIP for obinutuzuamb in the condition of treatment systemic lupus erythematosus with a waiver for a subset of the paediatric population and a deferral.

2.1.3. Lenacapavir - EMEA-002740-PIP01-19

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO in December 2020 noted that the points raised at D90 had been satisfactorily addressed.
Therefore, the PDCO agreed a PIP for lenacapavir in the condition of treatment of human immunodeficiency virus (HIV-1) infection with a deferral in a subset of the paediatric population.

2.1.4. Retinol (Vitamin A) - Orphan - EMEA-002790-PIP01-20

orphanix GmbH; Prevention of bronchopulmonary dysplasia (BPD)

Day 120 opinion

Neonatology - Paediatric Intensive Care

Summary of committee discussion:

The Committee noted that the applicant agreed to lower the waiver cut-off from 32 down to 30 weeks GA, which was found agreeable. In addition, 2 secondary endpoints were proposed by the applicant for assessment of retinol activity and added to the Opinion.
In conclusion, the PDCO adopted a positive opinion for retinol for the prevention of bronchopulmonary dysplasia with a waiver for preterm newborn infants from 30+0 to 36+6 gestational age (GA) on the ground of lack of significant therapeutic benefit over existing treatments and for term newborn infants onwards on the ground that the condition does not occur in the specified paediatric subsets.

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

Orphazyme A/S; Treatment of amyotrophic lateral sclerosis

Day 120 opinion

Neurology

Summary of committee discussion:

The Paediatric Committee adopted a positive opinion for arimoclomol (citrate) in the condition treatment of amyotrophic lateral sclerosis, including a deferral and waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2.1.6. Autologous tumour-infiltrating lymphocytes - EMEA-002776-PIP01-20

Iovance Biotherapeutics, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the proposed plan taking into account the clarifications provided by the applicant after the D90 discussion, their comments on the draft opinion and the input received by the PDCO.
In conclusion, the PDCO recommends granting a paediatric investigation plan with a deferral for autologous tumour-infiltrating lymphocytes for the paediatric population for the condition 'treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)', and with a waiver for a subset of the paediatric population on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

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

Amgen Europe BV; Treatment of acute lymphoblastic leukemia / Treatment of paediatric

patients aged 1 month or older and young adult patients up to 21 years of age with bone marrow relapse of T-cell acute lymphoblastic leukemia (ALL) treated with at least one prior therapy or B-cell ALL treated with prior targeted immune therapy, with or without extramedullary disease.

Day 120 opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, the PDCO adopted a positive opinion for the PIP for carfilzomib for the subset of a paediatric population , in the condition of treatment of acute lymphoblastic leukemia.
The PDCO agreed on a waiver in a subset of children on the grounds of lack of significant therapeutic benefit over existing treatments.
The PDCO granted a deferral for the completion of this PIP.

2.1.8. KH176: (S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidin-3-yl)chroman-2-carboxamide hydrochloride - Orphan - EMEA-002113-PIP01-16

Khondrion BV; Treatment of mitochondrial respiratory chain/oxidative phosphorylation defects / Treatment of mitochondrial respiratory chain/oxidative phosphorylation defects

Day 120 opinion

Other

Summary of committee discussion:

The Applicant addressed the remaining issues after Day 90.
The PDCO adopted a positive opinion.

2.1.9. Dexmedetomidine (hydrochloride) - EMEA-002758-PIP01-19

BioXcel Therapeutics, Inc.; Treatment of acute agitation in bipolar disorder / Treatment of acute agitation in schizophrenia

Day 120 opinion

Psychiatry

Summary of committee discussion:

The applicant's responses were noted by the PDCO.
Based on the assessment of this application, the PDCO agreed on a PIP for dexmedetomidine in the conditions, treatment of schizophrenia, and treatment of bipolar disorder, as well as on a deferral, and a waiver in a subset of children on the grounds that the conditions for which the specific medicinal product is intended do not occur in the specified paediatric subset(s).

2.1.10. Esketamine (hydrochloride) - EMEA-002772-PIP01-20

Celon Pharma S.A.; Bipolar depression, Major depressive disorder / Treatment-resistant bipolar depression / Treatment-resistant depression in the course of major depressive

disorder

Day 120 opinion

Psychiatry

Summary of committee discussion:

The applicant's responses to the Day 90 comments were noted by the PDCO. Consequently, all outstanding issues were resolved, and, in line with previous discussions, the PDCO agreed on a PIP for esketamine for the conditions, treatment of bipolar depression, and treatment of major depressive disorder, as well as on a deferral, and a waiver: for bipolar depression in a subset of children on the grounds that the condition does not occur in the specified paediatric subset, and – on its own motion - in a subset of children on the grounds that the specific medicinal product is likely to be unsafe in the specified subset; as well as for major depressive disorder in a subset of children on the grounds that the condition does not occur in the specified paediatric subset, and in a subset of children on the grounds that the specific medicinal product is likely to be unsafe in the specified subset.

2.1.11. Allopurinol / verinurad - EMEA-002754-PIP01-19

AstraZeneca AB; Chronic kidney disease / Treatment of chronic kidney disease in children and adolescents (6 to <18 years old) with hyperuricaemia and albuminuria

Day 120 opinion

Uro-nephrology

Summary of committee discussion:

The committee endorsed its views expressed on Day 90.
The PDCO grants a waiver in a subset of children.
Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO adopted a positive opinion for allopurinol / verinurad in the condition of "treatment of chronic kidney disease".

2.1.12. Sparsentan - Orphan - EMEA-001984-PIP02-20

Travere Therapeutics Ireland Ltd.; Treatment of focal segmental glomerular sclerosis (FSGS)

Day 120 opinion

Uro-nephrology

Summary of committee discussion:

Based on the assessment of this application, the PDCO adopted a positive PIP Opinion during its plenary on 11 December 2020, for sparsentan for a subset of children , in the condition of treatment of focal segmental glomerular sclerosis (FSGS).
The PDCO granted a waiver for a subset of children on the grounds that the specific medicinal product is likely to be unsafe.
The PDCO granted also a deferral for the completion of the PIP.

2.1.13. Sparsentan - EMEA-001984-PIP03-20

Travere Therapeutics Ireland Ltd.; Treatment of IgA nephropathy (IgAN)

Day 120 opinion

Uro-nephrology

Summary of committee discussion:

Based on the assessment of this application, the PDCO adopted a positive PIP Opinion during its plenary on 11 December 2020, for sparsentan for a subset of children , in the condition of treatment of immunoglobulin A nephropathy (IgAN).
The PDCO granted a waiver for a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.
The PDCO granted also a deferral for the completion of the PIP.

2.1.14. Rosuvastatin (calcium) / acetylsalicylic acid - EMEA-002891-PIP01-20

Neopharmed Gentili S.p.A.; Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

In line with the Day 30 assessment, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Rosuvastatin calcium / Acetylsalicylic acid for all subsets of the paediatric population (0 to 18 years of age) in the condition of prevention of cardiovascular events on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.15. Heparin sodium - EMEA-002885-PIP01-20

YES Pharmaceutical Development Services GmbH; Prevention and treatment of thromboembolic events

Day 60 opinion

Cardiovascular Diseases / Haematology-Hemostaseology

Summary of committee discussion:

Based on the final assessment of this application and discussions at the Paediatric Committee discussion the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Heparin Sodium Solution for Injection/Infusion containing unfractionated heparin (UFH) for all subsets of the paediatric population (0 to less than 18 years of age) covering the condition of prevention and treatment of thromboembolic events.
The PDCO agrees as proposed by the applicant to grant a full product specific on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.16. Reldesemtiv - Orphan - EMEA-002868-PIP01-20

Cytokinetics, Inc.; Amyotrophic lateral sclerosis (ALS)

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO's view expressed at Day 30 was re-discussed.
The Committee highlighted again the high need for treatment of amyotrophic lateral sclerosis (ALS) in children.
In conclusion, the PDCO agreed to grant a product specific waiver for reldesemtiv for the treatment of amyotrophic lateral sclerosis on the ground of lack of significant therapeutic benefit as clinical trials are not feasible.

2.1.17. UCB0107 - EMEA-002884-PIP01-20

UCB Pharma S.A.; Progressive supranuclear palsy

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the final assessment of this application and further discussions at the Paediatric Committee discussion the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for recombinant humanised monoclonal immunoglobulin G4 (UCB0107) for all subsets of the paediatric population (0 to less than 18 years of age) in the condition of Treatment of progressive supranuclear palsy.
The PDCO agrees as proposed by the applicant to grant a full product specific waiver with the justification that the disease or condition for which the specific medicinal product is intended occurs only in adult populations.

2.1.18. 18-(p-[131I]-iodophenyl)octadecyl phosphocholine - EMEA-002745-PIP02-20

Cellectar Biosciences, Inc.; Treatment of lymphoplasmacytic lymphoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion made at D30. 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 18-(p-[131I]-iodophenyl)octadecyl phosphocholine for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of lymphoplasmacytic lymphoma based on the ground that the disease does not occur in the paediatric subsets.
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. Catumaxomab - EMEA-002879-PIP01-20

Lindis Biotech GmbH; Treatment of malignant ascites

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion made at D30. 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 catumaxomab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of malignant ascites based on the ground of lack of significant therapeutic benefit because studies 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.

2.1.20. Lorlatinib - EMEA-002669-PIP02-20

Pfizer Europa MA EEIG; Treatment of lung malignant neoplasms

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion made at D30. 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 lorlatinib for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of lung cancer based on the ground that the disease does not occur in the paediatric subsets.
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. The PDCO identified the condition of treatment of neuroblastoma as an unmet 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. Ublituximab - Orphan - EMEA-002889-PIP01-20

CambPharma Solutions (CY) Ltd; Treatment of chronic lymphocytic leukemia (CLL)

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion made at D30. 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 ublituximab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mature B cell malignancies, 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.

2.1.22. Umbralisib tosylate - EMEA-002890-PIP01-20

CambPharma Solutions (CY) Ltd; Treatment of chronic lymphocytic leukemia (CLL)

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion made at D30. 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 umbralisib tosylate for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mature B cell malignancies, 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.

2.1.23. Paracetamol / nefopam (hydrochloride) - EMEA-002877-PIP01-20

Aptys Pharmaceuticals; Treatment of acute pain / Symptomatic short-term treatment of moderate to severe somatic acute pain

Day 60 opinion

Pain

Summary of committee discussion:

Based on the assessment of this application the Paediatric Committee agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for nefopam (hydrochloride) / paracetamol for all subsets of the paediatric population (0 to 18 years of

age) in the condition of treatment of acute pain.

2.1.24. Eliapixant - EMEA-002882-PIP01-20

Bayer AG; Treatment of refractory and/or unexplained chronic cough (RUCC)

Day 60 opinion

Pneumology - Allergology

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 eliapixant for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of refractory and/or unexplained chronic cough" on the grounds that this condition does not occur in the paediatric population.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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.

2.1.25. Alpha1-Proteinase Inhibitor (Human) - EMEA-002888-PIP01-20

Baxalta Innovations GmbH; Treatment of emphysema secondary to alpha 1-proteinase inhibitor deficiency

Day 60 opinion

Pneumology - Allergology / Haematology-Hemostaseology

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 Alpha1-Proteinase Inhibitor (Human) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of emphysema secondary to alpha 1-proteinase inhibitor deficiency" on the grounds that the disease does not occur in the paediatric population.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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.

2.1.26. Acetylcysteine / Ibuprofen (as ibuprofen sodium dihydrate) - EMEA-002561-PIP02-20

E-Pharma Trento S.p.A.; Upper respiratory tract signs and symptoms

Day 60 opinion

Pneumology - Allergology / Oto-rhino-laryngology

Summary of committee discussion:

Based on the assessment of this application the Paediatric Committee agrees with the applicant's request for a waiver.
The PDCO recommends granting a waiver for acetylcysteine / ibuprofen (sodium dihydrate) for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of upper respiratory tract infections.

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. Riociguat - EMEA-C-000718-PIP01-09-M06

Bayer AG; Treatment of pulmonary hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C2-000718-PIP01-09-M02
- EMEA-C3-000718-PIP01-09-M05
- (EMEA-C1-000718-PIP01-09-M01 – withdrawn)

The PDCO adopted on 11 December 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/0289/2016) of 4 November 2016.

2.2.2. Idursulfase - EMEA-C-000294-PIP02-12-M01

Shire Human Genetic Therapies AB; Treatment of mucopolysaccharidosis II (Hunter syndrome)

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO adopted on 11 December an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0008/2017) of 31 January 2017.

2.2.3. Elbasvir / grazoprevir - EMEA-C-001604-PIP01-13-M03

Merck Sharp & Dohme B.V.; Treatment of chronic hepatitis C

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

In line with the Day 30 discussion, the PDCO adopted during its December 2020 plenary an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision P/0255/2017 of 04 September 2017.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Remimazolam (as besylate) - EMEA-001880-PIP02-19-M01

PAION Deutschland GmbH; Sedation / General anesthesia / Sedation of mechanically ventilated patients / Procedural sedation

Day 60 opinion

Anaesthesiology

Post-meeting note: The application was withdrawn on the 10/12/2020.

2.3.2. Denosumab - EMEA-000145-PIP02-12-M03

Amgen Europe B.V.; Treatment of osteoporosis / Treatment of osteogenesis imperfecta / Treatment of glucocorticoid induced osteoporosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0102/2020 of 20 March 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Darvadstrocel - Orphan - EMEA-001561-PIP01-13-M02

Takeda Pharma A/S; Perianal fistula

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/0207/2020 of 16 June 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Obeticholic Acid - Orphan - EMEA-001304-PIP02-13-M05

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

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant's responses to the D30 issues were considered acceptable.
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/0204/2019 of 12 June 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Tofacitinib - EMEA-000576-PIP03-12-M05

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant's response was considered acceptable.
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/0380/2020 of 9 September 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Betibeglogene autotemcel - Orphan - EMEA-001665-PIP01-14-M04

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:

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/0020/2020 of 06/01/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Luspatercept - Orphan - EMEA-001521-PIP01-13-M05

Celgene Europe B.V.; Treatment of beta-thalassaemia / Treatment of anaemia in patients with beta-thalassemia intermedia and major

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the December 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 specified above 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/0024/2020 of 6 January 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Risankizumab - EMEA-001776-PIP02-17-M01

AbbVie Ltd; Chronic idiopathic arthritis

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO confirmed the outcome of the Day 30 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/0343/2017 of 23 November 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Tofacitinib citrate - EMEA-000576-PIP01-09-M13

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis) / Juvenile idiopathic arthritis

Day 60 opinion

Immunology-Rheumatology-Transplantation

Post-meeting note: The applicant withdrew the application on the 09/12/2020, prior to adoption of the opinion.

2.3.10. 3-({5-chloro-1-[3-(methylsulfonyl)propyl]-1Hindol-2-yl}methyl)-1-(2,2,2-trifluoroethyl)-1,3-dihydro-2H-imidazo[4,5-C]pyridin-2-one) - EMEA-001838-PIP01-15-M03

Janssen-Cilag International NV; Treatment of respiratory tract disease caused by human respiratory syncytial virus (RSV) / Treatment of respiratory tract disease caused by human RSV

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The committee discussed the applicant's clarifications and considers them acceptable.
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/0081/2019 of 22 March 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Aztreonam / Avibactam - EMEA-002283-PIP01-17-M01

Pfizer Europe MA EEIG; Treatment of infections caused by aerobic Gram-negative bacteria / Treatment of infections caused by aerobic Gram-negative bacteria in patients with limited therapeutic options

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0022/2020 of 06 January 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Baloxavir marboxil - EMEA-002440-PIP01-18-M01

Roche Registration GmbH; Prevention of Influenza / Treatment of Influenza / Treatment of influenza type A/B in otherwise healthy and high risk patients / Prevention (post-exposure prophylaxis) of influenza type A/B / Reduction of transmission of influenza type A/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/0300/2019 of 02/09/2019).

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

2.3.13. Ceftazidime / avibactam - EMEA-001313-PIP01-12-M10

Pfizer Europe MA EEIG; Treatment of bacterial infections / For the treatment of complicated urinary tract infections / For the treatment of complicated intraabdominal infections / For the treatment of pneumonia / For the treatment of infections due to aerobic Gram-negative organisms

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/0340/2018 of 8 November 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Cobicistat - EMEA-000969-PIP01-10-M05

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus type-1 (HIV-1) infection / Treatment of human immunodeficiency virus type-1 (HIV-1) infection - pharmacoenhancer for use in combination with antiretroviral agents

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

In December 2020 the PDCO discusses the responses received from the applicant after D30.

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0060/2017 of 17/3/2017).

2.3.15. Cobicistat / darunavir - EMEA-001280-PIP01-12-M03

Janssen-Cilag International NV; Treatment of HIV-1 infection / Treatment of HIV-1 infection in paediatric patients from 3 to less than 18 years

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO re-discussed the proposed modification taking into account the responses provided by the Applicant after D30.
In summary, the changes of Study 2 and Study 8 were considered acceptable whereas no change in Study 6 and in the studies timelines was agreed.
In conclusion, the PDCO adopted a favourable opinion on the modification of the agreed PIP

as set in the Agency's latest decision (P/0006/2019 of 3 January 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Tenofovir Alafenamide / Emtricitabine - EMEA-001577-PIP02-14-M04

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection / for the treatment of HIV-1 infection

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO noted the responses of applicant to the PDCO D30.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0171/2018 of 15/6/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Tenofovir alafenamide / Emtricitabine / Bictegravir - EMEA-001766-PIP01-15-M03

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus [HIV] disease resulting in other conditions / treatment of adults and paediatrics aged less than 2 years weighing more than 4 kg infected with human immunodeficiency virus-1 (HIV-1) without any known mutations associated with resistance to the individual components

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO discussed the response received after D30.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0334/2020 of 24/8/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. Cannabidiol - Orphan - EMEA-001964-PIP01-16-M03

GW Pharma (International) B.V.; Lennox Gastaut Syndrome / Tuberous Sclerosis Complex / infantile spasms / Dravet Syndrome / treatment of seizures

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0350/2020 of 09 September 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Lasmiditan - EMEA-002166-PIP01-17-M05

Eli Lilly and Company Limited; Migraine with and without aura

Day 60 opinion

Neurology

Summary of committee discussion:

PDCO discussion:
The PDCO discussed the thorough replies provided by the applicant to the comments raised at D30.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0299/2020 of 12/8/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Ofatumumab - EMEA-002397-PIP01-18-M01

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

Day 60 opinion

Neurology

Summary of committee discussion:

The Committee discussed the clarification provided by the applicant. This was found agreeable by the PDCO.
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.

2.3.21. Pitolisant - Orphan - EMEA-001176-PIP01-11-M06

BIOPROJET PHARMA; Narcolepsy / Narcolepsy with or without cataplexy

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 therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0298/2020 of 12/08/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.22. Siponimod hemifumarate - EMEA-000716-PIP01-09-M03

Novartis Europharm Ltd; Treatment of Multiple Sclerosis / Treatment of children/adolescent patients (10-18 years old) with relapsing forms of multiple sclerosis

Day 60 opinion

Neurology

Summary of committee discussion:

The Committee discussed the clarification provided by the applicant. This was found agreeable by the PDCO.
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/0098/2017 of 11 April 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.23. Brentuximab vedotin - Orphan - EMEA-000980-PIP01-10-M07

Takeda Pharma A/S; Treatment of Hodgkin lymphoma / Treatment of paediatric patients with newly diagnosed relapse or refractory Hodgkin lymphoma (from 5 years of age)

Day 60 opinion

Oncology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, including additional information submitted after the committee's day 30 discussion, 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/0243/2020 of 22 June 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.24. Crizotinib - EMEA-001493-PIP03-18-M01

Pfizer Europe MA EEIG; Inflammatory myofibroblastic tumour (IMT), Anaplastic large cell lymphoma (ALCL) / Treatment of paediatric patients that are able to swallow capsules (age range: 6 years to less than 18 years of age) with relapsed/refractory systemic ALK-positive ALCL / Treatment of paediatric patients that are able to swallow capsules (age range: 6 years to less than 18 years of age) with unresectable or relapsed/refractory ALK-positive IMT / Treatment of paediatric patients with relapsed/refractory systemic ALK-positive ALCL, Treatment of paediatric patients with unresectable or relapsed/refractory ALK-positive IMT

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application, taking into consideration the additional information received by the applicant.
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/0399/2019 of 04/12/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.25. Eribulin - EMEA-001261-PIP01-11-M06

Eisai GmbH; Soft Tissue Sarcoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the December 2020 plenary meeting.
The PDCO confirmed all the conclusions reached at Day 30.
Taking into consideration the conclusions reached at Day 30 and at Day 60 and 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/0264/2018 of 15 August 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.26. Fosdenopterin - Orphan - EMEA-001491-PIP01-13-M01

Origin Biosciences, Inc.; treatment of molybdenum cofactor deficiency type A

Day 60 opinion

Other

Summary of committee discussion:

During its plenary on 11 December 2020, the PDCO discussed the applicants responses to the day 30 issues for the PIP for fosdenopterin (synthetic pterin derivative called cyclic pyranopterin monophosphate (cPMP) expected to increase molybdopterin (MPT) synthesis and restore Sulfite Oxidase (SO) activity) for the treatment of molybdenum cofactor deficiency type A (MoCo Type A).
In short, all outstanding issues could be satisfactorily addressed by the applicant and a positive Opinion on this modification request has been adopted by the PDCO.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0071/2014 of 21/03/2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.27. Human thrombin (component 2) / Human fibrinogen (component 1) - EMEA-001598-PIP01-13-M03

Instituto Grifols, S.A.; Treatment of haemorrhage resulting from a surgical procedure / Supportive treatment in surgery where standard surgical techniques are insufficient for improvement of haemostasis, and as a suture support in vascular surgery

Day 60 opinion

Other

Summary of committee discussion:

The Committee discussed the clarification provided by the applicant with regards to the recruitment issues in the paediatric study subject to this modification. The clarification was found agreeable by the PDCO.
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/0270/2016 of 7 October 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.28. Human Thrombin / Human Fibrinogen - EMEA-001149-PIP01-11-M06

Omrix Biopharmaceuticals N.V.; Treatment of haemorrhage resulting from a surgical procedure / Treatment of cerebrospinal fluid leakage resulting from a surgical procedure / supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis / suture line sealing in dura mater closure / supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis

Day 60 opinion

Other

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion made 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.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0339/2019 of 10 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.29. In vitro expanded autologous human articular chondrocytes - EMEA-001823-PIP01-15-M02

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 60 opinion

Other

Summary of committee discussion:

The PDCO confirmed all the conclusions reached at Day 30.
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/0074/2019 of 22 March 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.30. In vitro expanded autologous human articular chondrocytes - EMEA-002217-PIP01-17-M01

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 60 opinion

Other

Post meeting note: The application was withdrawn on the 10/12/2020.

2.3.31. Vortioxetine - EMEA-000455-PIP02-10-M07

H. Lundbeck A/S; Major Depressive Disorder

Day 60 opinion

Psychiatry

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/0271/2020 of 15 July 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.32. Daprodustat - EMEA-001452-PIP01-13-M03

GlaxoSmithKline Trading Services Limited; Treatment of anaemia associated with chronic kidney disease

Day 60 opinion

Uro-nephrology / 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/0025/2018 of 30 January 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.33. Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Yamagata lineage) / Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Victoria lineage) / Split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain - EMEA-002359-PIP01-18-M02

Sanofi Pasteur; Prevention of influenza infection

Day 60 opinion

Vaccines

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0064/2010 of 20 February 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.4. Opinions on Re-examinations

2.4.1. 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 30 opinion

Neurology

Summary of committee discussion:

In December 2020 the PDCO discussed a request of re-examination of the PDCO Opinion for the adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926) for Treatment of Duchenne Muscular Dystrophy.

The applicant presented their position at the meeting together with the scientific justification supporting a change of two points of the agreed opinion.

The PDCO considered that at this point in time there should be no change in the Opinion.

In summary the PDCO agreed to maintain the Opinion on this PIP adopted on 16 October 2020.

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of opinions

2.6.1. BNT162 (mRNA S protein) - EMEA-002861-PIP02-20

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

Day 30 Opinion

Vaccines

Post meeting note: Adopted via written procedure on 26 November 2020

2.6.2. CX-024414 - mRNA that encodes for the pre-fusion stabilized Spike glycoprotein of 2019-novel Coronavirus - EMEA-002893-PIP01-20

Moderna Therapeutics Inc; Prevention of COVID-19 / Active immunisation against SARS-CoV-2

Day 30 Opinion

Vaccines

Post meeting note: Adopted via written procedure on 26 November 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. Migalastat (hydrochloride) - EMEA-C1-001194-PIP01-11-M04

Amicus Therapeutics Europe Limited; Treatment of Fabry disease

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.2. Copanlisib - EMEA-C1-001757-PIP02-15-M02

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

Day 30 letter

Oncology

2.7.3. Ruxolitinib (phosphate) - EMEA-C1-000901-PIP03-16-M01

Novartis Europharm Limited; Treatment of acute Graft versus Host Disease (aGvHD)

Day 30 letter

Oncology

2.7.4. Ivacaftor / tezacaftor / elexacaftor - EMEA-C3-002324-PIP01-17-M01

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 letter

Other

2.7.5. Daridorexant (hydrochloride) - EMEA-C1-002121-PIP03-19

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of insomnia

Day 30 discussion

Psychiatry

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. EMEA-002329-PIP02-20

Treatment of dermatitis and eczema / Treatment of chronic hand eczema, Treatment of atopic dermatitis

Day 90 discussion

Dermatology

3.1.2. Crinecerfont; 2-Thiazolamine, 4-(2-chloro-4-methoxy-5-methylphenyl)-N-[(1S)-2-cyclopropyl-1-(3-fluoro-4-methylphenyl)ethyl]-5-methyl-N-2-propyn-1-yl; - Orphan - EMEA-002700-PIP01-19

Neurocrine Therapeutics Ltd; Treatment of congenital adrenal hyperplasia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. EMEA-002773-PIP01-20

Treatment of non-alcoholic steatohepatitis (NASH)

Day 90 discussion

Gastroenterology-Hepatology

3.1.4. Crovalimab - EMEA-002709-PIP01-19

paroxysmal nocturnal hemoglobinuria (PNH

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Rozibafusp alfa - EMEA-002815-PIP01-20

Systemic lupus erythematosus

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.6. Secukinumab - EMEA-000380-PIP06-19

Treatment of lupus nephritis

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Tacrolimus - EMEA-001642-PIP02-20

Solid organ transplant rejection / 1. Prophylaxis of transplant rejection in liver, kidney or heart allograft recipients (children aged from birth to less than 18 years) / Treatment of allograft rejection resistant to treatment with other immunosuppressive medical products in children aged from birth to less than 18 years

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.8. Telitacicept - EMEA-002824-PIP01-20

Treatment of systemic lupus erythematosus

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.9. Bimekizumab - EMEA-002189-PIP04-20

Treatment of hidradenitis suppurativa / Treatment of moderate to severe hidradenitis suppurativa in adolescents from 12 years of age

Day 90 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.10. Rimegepant - EMEA-002812-PIP02-20

Acute treatment of migraine

Day 90 discussion

Neurology

3.1.11. Imetelstat - Orphan - EMEA-001910-PIP03-20

Geron Corporation; Treatment of acute myeloid leukemia (AML), Treatment of myelodysplastic syndromes (MDS), including juvenile myelomonocytic leukemia (JMML) / Treatment of pediatric patients with relapsed or refractory AML or MDS, including JMML, from 28 days to less than 18 years of age

Day 90 discussion

Oncology

3.1.12. Linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV5-hRKp.RPGR) - Orphan - EMEA-002827-PIP01-20

MeiraGTx UK II Ltd; Retinitis pigmentosa / RPGR mutation-associated X-linked retinitis pigmentosa

Day 90 discussion

Ophthalmology

3.1.13. Seltorexant - EMEA-002746-PIP01-20

Major Depressive Disorder (MDD)

Day 90 discussion

Psychiatry

3.1.14. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002821-PIP01-20

Prevention of medically assessed RSV- associated lower respiratory tract illness through maternal immunization / Active immunization of pregnant women during the second and third trimester of pregnancy to prevent medically assessed respiratory syncytial virus (RSV) -associated lower respiratory tract illness (LRTI) in infants by transfer of maternal antibodies

Day 90 discussion

Vaccines

3.1.15. Human, recombinant, non-fucosylated IgG1k monoclonal antibody (KHK4083) targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20

Atopic dermatitis / Treatment of moderate to severe atopic dermatitis

Day 60 discussion

Dermatology

3.1.16. Semaglutide - EMEA-001441-PIP05-20

Treatment of non-alcoholic fatty liver disease (NAFLD)

Day 60 discussion

Gastroenterology-Hepatology

3.1.17. Fenebrutinib - EMEA-002349-PIP03-20

Treatment of multiple sclerosis / Treatment of relapsing multiple sclerosis in patients 10 years of age to less than 18 years of age

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.18. Trimodulin - EMEA-002883-PIP01-20

Treatment of bacterial infections / Treatment of community-acquired sepsis / Treatment of neonatal sepsis / Treatment of severe community-acquired pneumonia / Treatment of community-acquired septic shock / Treatment of community-acquired severe sepsis

Day 60 discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Pneumology - Allergology

3.1.19. Zidebactam / cefepime - EMEA-002892-PIP01-20

Treatment of complicated urinary tract infections (cUTI)

Day 60 discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Uro-nephrology

3.1.20. Ravulizumab - EMEA-001943-PIP03-20

Acetylcholine receptor-antibody positive generalized myasthenia gravis / Treatment of acetylcholine receptor-antibody positive generalized myasthenia gravis

Day 60 discussion

Neurology

3.1.21. Allogeneic anti-CD19 CAR T cells produced using CRISPR/Cas9 to disrupt the T cell receptor alpha constant (TRAC) and β2-microglobulin (B2M) genomic loci and a recombinant adeno-associated viral vector to deliver donor template for insertion of the anti-CD19 CAR expression cassette into the TRAC locus - EMEA-002881-PIP01-20

B cell lymphoblastic leukemia/lymphoma, Mature B cell neoplasms / Treatment of relapsed/refractory B cell ALL / Treatment of relapsed/refractory B cell NHL

Day 60 discussion

Oncology

3.1.22. 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 - Orphan - EMEA-001862-PIP03-20

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms / Treatment of paediatric and adolescent subjects with relapsed or refractory B-cell non-Hodgkin lymphoma (NHL)

Day 60 discussion

Oncology

3.1.23. Ribitol - EMEA-002887-PIP01-20

Treatment of Limb-Girdle Muscular Dystrophy / Treatment of paediatric patients aged 5 years and older with a genetically confirmed diagnosis of Limb-Girdle Muscular Dystrophy type 2i (LGMD2I)/ LGMD R9-fukutin related

Day 60 discussion

Other

3.1.24. COVID-19 Vaccine - EMEA-002862-PIP01-20

COVID-19 / For the prevention of COVID-19

Day 60 discussion

Vaccines

3.1.25. Hydrochlorothiazide / Amlodipine / Ramipril - EMEA-002906-PIP01-20

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.26. Olpasiran - EMEA-002910-PIP01-20

Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

3.1.27. Canakinumab - EMEA-000060-PIP09-20

Schnitzler Syndrome

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.28. Otilimab - EMEA-001882-PIP03-20

Treatment of COVID-19

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.29. Secukinumab - EMEA-000380-PIP07-20

Thyroid disease

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.30. Anti-C1s Humanized IgG4 Monoclonal Antibody - EMEA-002903-PIP01-20

Immune Thrombocytopenia Purpura

Day 30 discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology / Neurology

3.1.31. Allogeneic multi-virus specific T lymphocytes targeting BK Virus, cytomegalovirus, human herpes virus-6, Epstein Barr virus and adenovirus. - Orphan - EMEA-002908-PIP01-20

AlloVir International DAC; Treatment of viral diseases in haematopoietic stem cell transplantation / Treatment of virus-associated haemorrhagic cystitis in allogeneic haematopoietic stem cell transplantation recipients / Pre-emptive treatment of Cytomegalovirus disease in allogeneic haematopoietic stem cell transplantation recipients / Pre-emptive treatment of Adenovirus disease in allogeneic haematopoietic stem cell transplantation recipients

Day 30 discussion

Infectious Diseases

3.1.32. Fully human neutralizing immunoglobulin G-1 kappa monoclonal antibody directed against a conserved epitope on the SARS CoV1 and 2 spike protein - EMEA-002899-PIP01-20

Treatment of coronavirus disease 2019 (COVID-19) / For the treatment of severe or critical COVID-19 in hospitalised children from 32 weeks gestational age (GA) to less than 18 years of age

Day 30 discussion

Infectious Diseases

3.1.33. Potassium Bitartrate / Citric Acid / L-Lactic Acid - EMEA-002917-PIP01-20

Prevention of urogenital Chlamydia trachomatis (CT) infection and Neisseria gonorrhoeae (GC) infection in females

Day 30 discussion

Infectious Diseases

3.1.34. Edaravone - Orphan - EMEA-002897-PIP01-20

Mitsubishi Tanabe Pharma GmbH; Amyotrophic lateral sclerosis

Day 30 discussion

Neurology

3.1.35. Eptinezumab - EMEA-002243-PIP02-20

Episodic cluster headache / Prophylaxis

Day 30 discussion

Neurology

3.1.36. Omaveloxolone - Orphan - EMEA-002487-PIP01-18

Reata Pharmaceuticals Inc.; Treatment of Friedreich's ataxia

Day 30 discussion

Neurology

3.1.37. Ublituximab - EMEA-002889-PIP02-20

Relapsing forms of Multiple Sclerosis (RMS)

Day 30 discussion

Neurology

3.1.38. Vatiquinone - Orphan - EMEA-001238-PIP02-20

PTC Therapeutics International Limited; Treatment of mitochondrial epilepsy

Day 30 discussion

Neurology

3.1.39. Alpelisib - EMEA-002016-PIP04-20

ovarian cancer

Day 30 discussion

Oncology

3.1.40. Autologous CD4+ and CD8+ T cells genetically modified with a lentiviral vector encoding a B cell maturation antigen-specific chimeric antigen receptor - EMEA-002909-PIP01-20

Treatment of mature B cell neoplasms

Day 30 discussion

Oncology

3.1.41. Epcoritamab - EMEA-002907-PIP01-20

Treatment of mature B-cell lymphoma / Treatment of paediatric patients with relapsed/refractory aggressive mature B-cell lymphoma

Day 30 discussion

Oncology

3.1.42. EMEA-002895-PIP01-20

Treatment of macular oedema due to central or tributary (branch) retinal vein occlusion / Treatment of retinopathy of prematurity / Treatment of diabetic retinopathy / Treatment of choroidal neovascularisation

Day 30 discussion

Ophthalmology

3.1.43. Isopropyl alcohol / Povidone-iodine - EMEA-002902-PIP01-20

Prevention of infection prior to invasive procedures

Day 30 discussion

Other

3.1.44. Iptacopan - EMEA-002705-PIP04-20

Atypical haemolytic uremic syndrome

Day 30 discussion

Other / Haematology-Hemostaseology

3.1.45. Dupilumab - EMEA-001501-PIP08-20

Chronic Rhinosinusitis without Nasal Polyposis / Chronic Rhinosinusitis without Nasal Polyposis

Day 30 discussion

Oto-rhino-laryngology

3.1.46. Brensocatib - EMEA-002905-PIP01-20

Non-cystic fibrosis bronchiectasis (NCFBE) / Treatment of NCFBE for reducing exacerbations

Day 30 discussion

Pneumology - Allergology

3.1.47. Recombinant human pentraxin-2 - Orphan - EMEA-002878-PIP02-20

Roche Registration GmbH; Idiopathic pulmonary fibrosis

Day 30 discussion

Pneumology - Allergology

3.1.48. Single chain urokinase plasminogen activator (scuPA) - Orphan - EMEA-002896-PIP01-20

Lung Therapeutics, Inc.; Treatment of infectious pleural effusion / Treatment of infected, non-draining pleural effusions including complicated parapneumonic pleural effusion (CPE), empyema and other forms of pleural space infection

Day 30 discussion

Pneumology - Allergology

3.1.49. Thienopyrimidine Derivative - EMEA-002901-PIP01-20

Treatment of fibrosing ILDs / Treatment of chronic fibrosing interstitial lung diseases (ILD)

Day 30 discussion

Pneumology - Allergology

3.1.50. Bardoxolone methyl - Orphan - EMEA-002488-PIP01-18

Reata Pharmaceuticals Inc.; Treatment of Alport syndrome

Day 30 discussion

Uro-nephrology

3.1.51. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002904-PIP01-20

Prevention of lower respiratory tract disease caused by respiratory syncytial virus / Active immunization in the prevention of respiratory syncytial virus (RSV)-associated lower respiratory tract disease (LRTD) in subjects aged 2 to 18 years who are at high risk of RSV-associated LRTD

Day 30 discussion

Vaccines

3.1.52. EMEA-002880-PIP01-20

Active immunisation for the prevention of COVID-19

Day 30 discussion

Vaccines / Infectious Diseases

3.1.53. Baricitinib - EMEA-001220-PIP07-20

Treatment of COVID-19

Day 30 discussion

Vaccines / Infectious Diseases

3.2. Discussions on Compliance Check

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

3.2.1. Dapagliflozin - EMEA-C-000694-PIP01-09-M08

Astrazeneca AB; Treatment of type 2 diabetes

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Dulaglutide - EMEA-C2-000783-PIP01-09-M05

Eli Lilly & Company Limited; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Oseltamivir phosphate - EMEA-C-000365-PIP01-08-M11

Roche Registration GmbH; Treatment and prevention of influenza

Day 30 discussion

Infectious Diseases

3.2.4. Cytarabine (liposomal combination) / Daunorubicin (liposomal combination) - EMEA-C2-001858-PIP02-16-M03

Jazz Pharmaceuticals Ireland Limited; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Lamivudine (3TC) / Abacavir (ABC) / Dolutegravir (DTG) - EMEA-001219-PIP01-11-M05

ViiV Healthcare UK Limited; Treatment human immunodeficiency virus (HIV-1) infection in paediatric population

Day 30 discussion

3.3.2. Enalapril maleate - EMEA-001706-PIP01-14-M03

Proveca Pharma Limited; Treatment of heart failure

Day 30 discussion

Cardiovascular Diseases

3.3.3. 6-cyclopropaneamido-4-{[2-methoxy-3-(1-methyl-1H-1,2,4 triazol-3-yl)phenyl]amino}-N-(2H3)methylpyridazine-3-carboxamide - EMEA-002350-PIP01-18-M01

Bristol-Myers Squibb International Corporation; Treatment of psoriasis / Treatment of moderate to severe plaque psoriasis in paediatric patients 6 years of age and older

Day 30 discussion

Dermatology

3.3.4. Tildrakizumab - EMEA-001451-PIP01-13-M01

Almirall, S.A; Psoriasis / Treatment of moderate to severe plaque psoriasis in patients from 6 to less than 18 years of age who are candidates for systemic therapy

Day 30 discussion

Dermatology

3.3.5. Empagliflozin - EMEA-000828-PIP01-09-M08

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Evinacumab - EMEA-002298-PIP01-17-M02

Regeneron Ireland DAC; Treatment of elevated cholesterol / Treatment of homozygous familial hypercholesterolemia (HoFH)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Exenatide - EMEA-000689-PIP01-09-M11

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 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.8. Linagliptin - EMEA-000498-PIP01-08-M09

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.9. Ozanimod hydrochloride - EMEA-001710-PIP04-17-M02

Celgene Europe B.V.; Treatment of Crohn's disease / Treatment of moderate to severe active Crohn's disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.10. Ustekinumab - EMEA-000311-PIP04-13-M04

Janssen-Cilag International NV; Crohn's Disease / Treatment of Crohn's Disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.11. Ustekinumab - EMEA-000311-PIP05-17-M02

Janssen-Cilag International NV; Ulcerative Colitis / Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.12. Roxadustat - EMEA-001557-PIP01-13-M05

Astellas Pharma Europe B.V.; treatment of anaemia due to chronic disorders

Day 30 discussion

Haematology-Hemostaseology

3.3.13. Sarilumab - EMEA-001045-PIP01-10-M02

sanofi-aventis recherche & développement; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.14. Cabotegravir - EMEA-001418-PIP02-15-M01

ViiV Healthcare UK Limited; Prevention of human immunodeficiency virus (HIV-1) infection / In combination with safer sex practices for pre-exposure prophylaxis to reduce the risk of HIV-1 acquisition in sexually active adolescents at high risk, from 12 to less than 18 years of age

Day 30 discussion

Infectious Diseases

3.3.15. Islatravir / Doravirine - EMEA-002707-PIP01-19-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection.

Day 30 discussion

Infectious Diseases

3.3.16. Remdesivir - EMEA-002826-PIP01-20-M01

Gilead Sciences International Ltd.; Coronavirus disease 2019 (COVID-19) / for the treatment of paediatric patients aged from birth to < 18 years (weighing < 40kg) with coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.17. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M01

argenx BV; Generalised myasthenia gravis

Day 30 discussion

Neurology

3.3.18. Eladocagene exuparvovec - Orphan - EMEA-002435-PIP01-18-M02

PTC Therapeutic International Limited; Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency / Treatment of Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency

Day 30 discussion

Neurology

3.3.19. Bintrafusp alfa - Orphan - EMEA-002586-PIP01-19-M01

Merck Europe B.V.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 0 to <18 years of age with solid malignant tumours

Day 30 discussion

Oncology

3.3.20. Cobimetinib - EMEA-001425-PIP01-13-M05

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) with Ras, Raf or MEK pathway activation / Treatment of children with a paediatric solid malignant tumour with known or expected Ras, Raf or MEK pathway activation, at first relapse or refractory to initial treatment

Day 30 discussion

Oncology

3.3.21. Durvalumab - EMEA-002028-PIP01-16-M02

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

Day 30 discussion

Oncology

3.3.22. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M05

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

Burkitt and Burkitt-like lymphoma

Day 30 discussion

Oncology

3.3.23. Idecabtagene vicleucel - Orphan - EMEA-002369-PIP01-18-M02

Celgene Europe B.V.; Treatment of mature B-cell neoplasms / Treatment of paediatric patients with relapsed or refractory BCMA+ B-cell non-Hodgkin lymphoma

Day 30 discussion

Oncology

3.3.24. Larotrectinib - EMEA-001971-PIP02-16-M03

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with advanced solid tumours harbouring an NTRK fusion

Day 30 discussion

Oncology

3.3.25. Larotrectinib - EMEA-001971-PIP03-18-M01

Bayer AG; Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients from birth to less than 18 years of age with a primary CNS tumour harbouring an NTRK fusion

Day 30 discussion

Oncology

3.3.26. Midostaurin - Orphan - EMEA-000780-PIP01-09-M06

Novartis Europharm Limited; C92.0 Acute myeloid leukaemia, C94.3 Mast cell leukaemia, C96.2 Malignant mastocytosis / Treatment of paediatric patients with FLT3 mutated AML, newly diagnosed

Day 30 discussion

Oncology

3.3.27. Nivolumab / relatlimab - EMEA-002727-PIP01-19-M01

Bristol-Myers Squibb International Corporation; Treatment of melanoma / Relatlimab/nivolumab fixed dose combination for treatment of unresectable or metastatic melanoma in patients from 12 to 18 years

Day 30 discussion

Oncology

3.3.28. Palbociclib - EMEA-002146-PIP01-17-M02

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

Day 30 discussion

Oncology

3.3.29. Tremelimumab - EMEA-002029-PIP01-16-M02

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

Day 30 discussion

Oncology

3.3.30. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M03

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukemia / Treatment of patients from 6 months to less than 18 years of age with relapsed or refractory FLT3 / ITD positive acute myeloid leukaemia or newly-diagnosed FLT3 / ITD positive acute myeloid leukaemia

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.3.31. Vamorolone - Orphan - EMEA-001794-PIP02-16-M03

ReveraGen BioPharma Ltd; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Other

3.3.32. Ponesimod - EMEA-000798-PIP01-09-M03

Janssen-Cilag International NV; Multiple sclerosis

Day 30 discussion

Other / Neurology

3.3.33. Bupivacaine - EMEA-000877-PIP03-17-M02

Pacira Ltd; Postsurgical analgesia

Day 30 discussion

Pain

3.3.34. Glycopyrronium bromide / Formoterol fumarate dihydrate / Beclometasone dipropionate - EMEA-001875-PIP02-18-M03

Chiesi Farmaceutici S.p.A.; Treatment of asthma / Regular treatment of asthma in patients not controlled with medium-high doses of inhaled corticosteroids and long-acting beta2-agonists

Day 30 discussion

Pneumology - Allergology

3.3.35. Interferon beta-1a - Orphan - EMEA-002238-PIP01-17-M01

Faron Pharmaceuticals Ltd.; Treatment of Acute Respiratory Distress Syndrome (ARDS)

Day 30 discussion

Pneumology - Allergology

3.3.36. Vilanterol / fluticasone furoate - EMEA-000431-PIP01-08-M12

Glaxo Group Limited; Mixed Asthma / Treatment of asthma where use of a combination product (long acting beta agonist and inhaled corticosteroid) is appropriate

Day 30 discussion

Pneumology - Allergology

3.3.37. Agomelatine - EMEA-001181-PIP01-11-M06

Les Laboratoires Servier; Major depressive episodes

Day 30 discussion

Psychiatry

3.3.38. Cariprazine (hydrochloride) - EMEA-001652-PIP01-14-M03

Gedeon Richter Plc.; Schizophrenia

Day 30 discussion

Psychiatry

3.3.39. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues (DCR-PHXC) - Orphan - EMEA-002493-PIP01-18-M02

Dicerna Ireland Limited; Primary hyperoxaluria

Day 30 discussion

Uro-nephrology

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 4 January 2021 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 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

No items

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

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

No items

8. Annual reports on deferrals

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

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 November 2020 by CHMP. These include Xarelto (rivaroxaban) and Tivicay (dolutegravir).

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

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 FWG 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:

No item

9.5. Cooperation with International Regulators

No item

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

No item

9.7. PDCO work plan

9.7.1. PDCO Work Plan 2021

PDCO Chair: Koenraad Norga;

Summary of committee discussion:

The draft PDCO work plan for 2021 was endorsed by the committee.

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. Progress report on EMA/EC paediatric action plan

Summary of committee discussion:

The committee was informed that a progress report on the EMA/EC joint action plan on paediatrics would be published by mid-December 2020.

10.3. EMA Business Pipeline activity and Horizon scanning

Summary of committee discussion:

The pipeline activity was presented for information only.

10.4. Big Data Training Signpost

Summary of committee discussion:

The committee was informed about the publication of the Big Data Training Signpost which is a collection of external training courses on Big Data skills that can benefit the Network.

11. Breakout sessions

11.1. Neonatology

Summary of committee discussion:

Neonatal issues related to PIPs were discussed.

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 8-11 December 2020 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	When chairing the meeting: To be replaced for discussions, final deliberations and voting When not chairing the meeting: No participation in final deliberations and voting	2.3.32. Daprodustat - EMEA-001452-PIP01-13-M03 3.1.14. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002821-PIP01-20 3.1.31. Fully human neutralizing immunoglobulin G-1 kappa monoclonal antibody directed against a conserved epitope on the SARS CoV1 and 2 spike protein - EMEA-002899-PIP01-20 3.1.50. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002904-PIP01-20 3.1.54. Otilimab - EMEA-001882-PIP03-20 3.3.36. Vilanterol / fluticasone furoate - EMEA-000431-PIP01-08-M12
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in final deliberations and voting on: No participation in discussion, final deliberations and voting on:	3.3.22. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M05 2.7.4. Ruxolitinib (phosphate) - EMEA-C1-000901-PIP03-16-M01
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar	Member	Bulgaria	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Roussinov			applicable to this meeting	
Georgios Savva	Member	Cyprus	No interests declared	
Lucie Kravackova	Member	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	
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	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
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	Member	Sweden	No interests declared	
Sara Vennberg	Alternate	Sweden	No interests declared	
Johannes	Alternate	Healthcare	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Taminiau		Professionals' Representative		
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 participation in final deliberations and voting on:	3.3.39. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues (DCR-PHXC) - Orphan - EMEA-002493-PIP01-18-M02 2.3.21. Pitolisant - Orphan - EMEA-001176-PIP01-11-M06
Jaroslav Sterba	Member	Patients' Organisation Representative	No interests declared	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Alternate	Patients' Organisation Representative	No interests declared	
Michal Odermarsky	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
María Estela Moreno Martín	Expert - in person*	Spain - AGEMED/AEMPS	No interests declared	
Lutz Wiesner	Expert - via telephone*	Germany - BfArM	No interests declared	
Emmely de Vries	Expert - via telephone*	Netherlands - CBG/MEB	No interests declared	
Victor Mangas Sanjuan	Expert - via telephone*	Spain - AGEMED/AEMPS	No interests declared	
Frederike Lentz	Expert - via telephone*	BfArM	No interests declared	
Louisa Braun Exner	Expert - via telephone*	Denmark - DKMA	No interests declared	
Roderick Houwen	Expert - via telephone*	Netherlands - CBG/MEB	No restrictions applicable to this meeting	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Maja Sommerfelt Grønvold	Expert - via telephone*	Norway - NOMA	No interests declared	
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/