24 June 2022
EMA/PDCO/561127/2022
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 17-20 May 2022

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

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.

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

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.

1.2. Adoption of agenda

The agenda for 17-20 May 2022 meeting was adopted with amendment.

1.3. Adoption of the minutes

The minutes for 19-22 April 2022 meeting were adopted and will be published on the EMA website.

2. Opinions

Some information related to this section cannot be disclosed at the present time as it is deemed to contain commercially confidential information.

2.1. Opinions on Products

2.1.1. Semaglutide / cagrilintide - EMEA-003059-PIP01-21

Novo Nordisk A/S; Treatment of obesity

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children and adolescents from 8 years to less than 18 years, in the condition treatment of obesity was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 8 years on the grounds of lack of significant therapeutic benefit over existing treatments. The PDCO granted a deferral for the completion of this PIP.

2.1.2. EMEA-003090-PIP01-21

Treatment of hereditary angioedema

Day 120 opinion

Haematology-Hemostaseology

Note: Withdrawal request received on 16 May 2022

2.1.3. Bepirovirsen - EMEA-003082-PIP01-21

GlaxoSmithKline Trading Services Limited; Treatment of chronic hepatitis B infection

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agreed on a PIP for bepirovirsen for children from 2 years to less than 18 years of age in the condition of treatment of chronic hepatitis B infection. The PIP includes a waiver below 2 years of age on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset.

2.1.4. Lonafarnib - Orphan - EMEA-002516-PIP02-21

EigerBio Europe Limited; Treatment of hepatitis D virus infection

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the responses and the new proposal of the second quality study and agreed to grant a positive opinion on the PIP.
Based on the assessment of this application the PDCO adopted a positive opinion on a PIP for lonafarnib from 3 years to less than 18 years of age for the treatment of hepatitis D

virus infection. A deferral for the completion of the PIP was granted as well as a waiver for the paediatric population from birth to less than 3 years of age on the grounds that clinical studies with lonafarnib cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need in this age group.

2.1.5. Mixture of 2 synthetic double-stranded N-acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus - EMEA-002694-PIP02-21

Janssen-Cilag International NV; Treatment of chronic hepatitis D virus infection

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP in children from 2 years to less than 18 years of age in the condition of 'treatment of chronic hepatitis D virus infection' was adopted.
The PDCO agreed on a waiver in a subset of children on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subsets.
The PDCO granted a deferral for the completion of this PIP.

2.1.6. Alprazolam - EMEA-003043-PIP01-21

UCB Pharma SA.; Treatment of epileptic seizures

Day 120 opinion

Neurology

Summary of Committee discussion:

During the May 2022 plenary meeting, the PDCO adopted a positive opinion for an application for a paediatric investigation plan with a deferral and a waiver for the specific product containing alprazolam for inhalation (using Staccato device) for treatment of epileptic seizures.
Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO agreed with the applicant's request for a partial waiver. The PDCO recommended granting a waiver for the specific product containing alprazolam for inhalation (using Staccato device) for some subsets of the paediatric population (from birth to less than 12 years of age) in the condition of treatment of epileptic seizures.

2.1.7. Cannabidiol - EMEA-001964-PIP03-21

GW Pharma (International) B.V; Treatment of epilepsy with myoclonic atonic seizures

Day 120 opinion

Neurology

Summary of Committee discussion:

During the May 2022 plenary meeting PDCO confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.
The PDCO adopted a positive opinion for an application for a paediatric investigation plan for cannabidiol for treatment of epilepsy with myoclonic atonic seizures (EMAS). A waiver for children from birth to less than 1 year of age was granted on the grounds that EMAS does not occur in this subset.

2.1.8. Autologous tumour-infiltrating lymphocytes (TILs) isolated from a patient's cancer tissue and expanded ex vivo - EMEA-003072-PIP01-21

Instil Bio, Inc.; Treatment of melanoma

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 120, during the May 2022 plenary meeting, a PIP application for autologous tumour-infiltrating lymphocytes (TILs) isolated from a patient's cancer tissue and expanded ex vivo for the treatment of melanoma.
The PDCO agreed with all the conclusions reached at Day 90 and adopted a positive opinion on a paediatric investigation plan for the treatment of melanoma, with a deferral and a waiver for children less than 12 years of age on the grounds that the condition for which the specific medicinal product is intended does not occur in the paediatric population.

2.1.9. Autologous CD3+CD4+CD25+CD127-FoxP3+ polyclonal regulatory T cells ex vivo expanded - EMEA-002737-PIP01-19

PolTreg SA; Treatment of type 1 diabetes mellitus (T1DM)

Day 120 opinion

Other / Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for autologous CD3+CD4+CD25+CD127-FoxP3+ polyclonal regulatory T cells ex vivo expanded for patients 3 years of age and/or more than 20 kg of bodyweight to less than 18 years of age in the condition of treatment of type 1 diabetes mellitus was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 3 years of age and/or less than 20 kg of bodyweight on the grounds of lack of safety.

2.1.10. Benralizumab - EMEA-001214-PIP09-21

AstraZeneca AB; Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 120 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion on a PIP in the condition of 'treatment of eosinophilic granulomatosis with polyangiitis' was adopted.
The PDCO agreed on a waiver in a subset of children from birth to less than 6 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The Committee granted a deferral for completion of this PIP.

2.1.11. Sibeprenlimab - Orphan - EMEA-003085-PIP01-21

Otsuka Pharmaceutical Netherlands B.V.; Treatment of primary immunoglobulin A nephropathy

Day 120 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from 2 years to less than 18 years of age, in the condition of "treatment of primary immunoglobulin A nephropathy" was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The PDCO granted a deferral for the completion of this PIP.

2.1.12. Respiratory syncytial virus stabilised prefusion F subunit vaccine (RSVpreF, PF-06928316) - EMEA-002795-PIP02-21

Pfizer Europe MA EEIG; Prevention of lower respiratory tract disease caused by respiratory syncytial virus

Day 120 opinion

Vaccines

Summary of Committee discussion:

Based on the assessment of this application and further discussions the PDCO agreed a PIP for respiratory syncytial virus stabilised prefusion F subunit vaccine (RSVpreF, PF-06928316) for children from 2 years to less than 18 years of age in the condition of prevention of RSV-associated lower respiratory tract illness. The PIP contains two clinical studies. A waiver was granted for children from birth to less than 2 years of age, based on the grounds that the vaccine is likely to be unsafe in this age group, due to the potential for disease enhancement.

2.1.13. Autologous bone marrow-derived mononuclear cell enriched white blood cells - EMEA-003193-PIP01-22

Ixaka Iberia SLU; Treatment of chronic limb-threatening ischemia

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for autologous bone marrow-derived mononuclear cell enriched white blood cells for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of chronic limb-threatening ischemia.
The PDCO emphasised 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.

2.1.14. Botulinum toxin type E - EMEA-003190-PIP01-22

Allergan Pharmaceuticals Ireland; Treatment of skin wrinkling

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition of treatment of skin wrinkling on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.
The PDCO emphasised 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. Derivative of 6-[2-(pyridin-2-yl)phenoxy]methyl}-1,2,3,4-tetrahydroisoquinoline - EMEA-003002-PIP02-22

Treatment of clinically significant portal hypertension (CSPH)

Day 60 opinion

Gastroenterology-Hepatology

Note: Withdrawal request received on 19 May 2022

2.1.16. ^{224}Radium adsorbed in calcium carbonate microparticles - EMEA-003199-PIP01-22

Oncoinvent AS; Treatment of peritoneal carcinomatosis

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of peritoneal carcinomatosis 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).
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.17. Lurbinectedin - Orphan - EMEA-002846-PIP02-22

Pharma Mar, S.A.; Treatment of malignant mesothelioma

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for lurbinectedin for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of malignant mesothelioma on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in children.
The PDCO recommended to grant the waiver for all pharmaceutical forms and all routes of administration to which the applicant agreed.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.18. Parsaclisib (as hydrochloride) - Orphan - EMEA-002696-PIP03-22

Incyte Biosciences Distribution B.V.; Treatment of myelofibrosis

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for parsaclisib (as hydrochloride) for all subsets of the

paediatric population (0 to 18 years of age) in the condition of treatment of myelofibrosis. The PDCO emphasised 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. Peptide KLBPVQLWV / Peptide SMPPPGTRV / Peptide YLQLVFGIEV / Peptide RLLQETELV / Peptide YLSGADLNL / Peptide LLTFWNPPV / Peptide IMIGHLVGV / Peptide KVAEIVHFL / Peptide KVFGSLAFV / Pan HLA DR-binding epitope D-Ala-Lys-Cha-Val-Ala-Ala-Trp-Thr-Leu-Lys-Ala-Ala-D-Ala (OSE2101) - EMEA-003181-PIP01-22

OSE Immunotherapeutics; Treatment of lung cancer (small cell and non-small cell lung cancer)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the May 2022 plenary meeting, an application for a product specific waiver for Peptide KLBPVQLWV / Peptide SMPPPGTRV / Peptide YLQLVFGIEV / Peptide RLLQETELV / Peptide YLSGADLNL / Peptide LLTFWNPPV / Peptide IMIGHLVGV / Peptide KVAEIVHFL / Peptide KVFGSLAFV / Pan HLA DR-binding epitope D-Ala-Lys-Cha-Val-Ala-Ala-Trp-Thr-Leu-Lys-Ala-Ala-D-Ala (OSE2101) for the treatment of lung cancer on the grounds that the disease does not occur in the paediatric population.
The PDCO agreed with all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 on a product specific waiver for this product 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 the disease does not occur in the paediatric population.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.20. Trabectedin - Orphan - EMEA-000610-PIP02-22

Pharma Mar, S.A.; Treatment of soft tissue sarcoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion from D30. Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for trabectedin for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of soft tissue sarcoma. Since the agreed waiver ground is that the product is likely to be ineffective in the paediatric population, the possibility to apply this to all pharmaceutical forms and all routes

of administrations was agreed to by the applicant.
The PDCO emphasised 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. Botulinum toxin type A - EMEA-003202-PIP01-22

Galderma International S.A.S.; Treatment of skin wrinkling

Day 60 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for botulinum toxin type A for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of skin wrinkling". The PDCO recommended to extend the waiver to all pharmaceutical forms and routes of administration to which the applicant agreed.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.2. Opinions on Compliance Check

2.2.1. Ritlecitinib - EMEA-C1-002451-PIP01-18

Pfizer Europe MA EEIG; Treatment of alopecia areata

Day 60 letter

Dermatology

Summary of Committee discussion:

The PDCO discussed the completed studies as well as the additional information received after Day 30 and considered that these are compliant with the latest Agency's Decision (P/0147/2021) of 14 April 2021.

2.2.2. Pitolisant - EMEA-C-001176-PIP01-11-M06

BIOPROJET PHARMA; Treatment of narcolepsy

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed the completed Study 4 and considered that it is compliant with the

latest Agency's Decision (P/0057/2021) of 27 January 2021. Furthermore, the PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-001176-PIP01-11-M01
- EMEA-C2-001176-PIP01-11-M03

The PDCO adopted on 20/05/2022 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0057/2021) of 27 January 2021.

2.2.3. Sacubitril / valsartan - EMEA-C-000316-PIP02-11-M05

Novartis Europharm Ltd; Treatment of heart failure

Day 30 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO discussed the completed Studies 6 and 7 and considered that these are compliant with the latest Agency's Decision (P/0327/2021) of 13 August 2021. Moreover, the PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-000316-PIP02-11-M01
- EMEA-C2-000316-PIP02-11-M05

The PDCO adopted on 20 May 2022 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0327/2021) of 13 August 2021.

2.2.4. Methoxy polyethylene glycol-epoetin beta - EMEA-C-000172-PIP01-07-M03

Roche Registration GmbH; Treatment of symptomatic anaemia associated with chronic kidney disease

Day 30 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0317/2017) of 31 October 2017.

2.2.5. Cabotegravir - EMEA-C-001418-PIP02-15-M03

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

Day 30 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed at Day 30, during the May 2022 plenary meeting, an application for a full compliance check for cabotegravir, for the prevention of human immunodeficiency virus

(HIV-1) infection.
The Committee discussed the completed studies and considered that these are compliant with the latest Agency's Decision. The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0216/2016) of 12 August 2016.

2.2.6. Lisdexamfetamine dimesylate - EMEA-C-000553-PIP01-09-M05

Shire Pharmaceutical Contracts Ltd; Treatment of attention deficit hyperactivity disorder

Day 30 opinion

Psychiatry

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:
- EMEA-C1-000553-PIP01-09, EMEA-C2-000553-PIP01-09-M02, EMEA-C4-000553-PIP01-09-M03

The PDCO adopted on 20 May 2022 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0097/2022) of 17 March 2022.
The PDCO considers that the following studies could be considered significant:
Study 8 (SPD489-404).

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Lebrikizumab - EMEA-002536-PIP01-18-M02

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Note: Withdrawal request received on 19 May 2022

2.3.2. Garadacimab - Orphan - EMEA-002726-PIP01-19-M02

CSL Behring GmbH; Prevention of hereditary angioedema attacks

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the May 2022 plenary meeting, this request for modification for garadacimab for the treatment of hereditary angioedema attacks.
The applicant requested changes in some elements of three clinical studies.
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/0274/2021 of 8 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Vadadustat - EMEA-001944-PIP01-16-M04

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of anaemia due to chronic disorders

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the May 2022 plenary meeting, this request for modification for vadadustat for the treatment of anaemia due to chronic disorders.
The applicant requested to delay the initiation date of four studies.
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/0024/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Avacopan - Orphan - EMEA-002023-PIP01-16-M06

ChemoCentryx Ireland Ltd.; Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

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/0181/2021 of 10 May 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Liposomal ciclosporin A (L-CsA) - Orphan - EMEA-002344-PIP02-18-M01

Zambon S.p.A.; Treatment of bronchiolitis obliterans syndrome (BOS)

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/0312/2019 of 11 September 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Oritavancin (diphosphate) - EMEA-001270-PIP01-12-M05

Menarini International Operations Luxembourg S.A.; Treatment of acute bacterial skin and skin structure infections

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

In the written response the applicant addressed the remaining issues raised by the Committee. The PDCO considered that some 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/0498/2021 of 3 December 2021.

2.3.7. Remdesivir - EMEA-002826-PIP01-20-M03

Gilead Sciences International Ltd.; Treatment of coronavirus disease 2019 (COVID-19)

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/0338/2021 of 9 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Cannabidiol - Orphan - EMEA-001964-PIP01-16-M04

GW Pharma (International) B.V.; Treatment of seizures associated with Dravet syndrome / Treatment of seizures associated with Lennox-Gastaut syndrome / Treatment of seizures associated with tuberous sclerosis complex

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/0033/2021 of 29 January 2021). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Delandistrogene moxeparvovec - Orphan - EMEA-002677-PIP01-19-M02

Roche Registration GmbH; Treatment of Duchenne muscular dystrophy

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/0052/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Lacosamide - EMEA-000402-PIP03-17-M06

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

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/0349/2021 of 20 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Daratumumab - Orphan - EMEA-002152-PIP01-17-M03

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B cell neoplasms)

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, 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/0421/2020 of 22 October 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Ixazomib - Orphan - EMEA-001410-PIP02-17-M04

Takeda Pharma A/S; Treatment of lymphoid malignancies (excluding multiple myeloma) / Treatment of multiple myeloma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from the D30 discussion.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that some of the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0351/2020 of 9 September 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Mometasone (furoate) / indacaterol (acetate) - EMEA-001217-PIP01-11-M08

Novartis Europharm Limited; Treatment of asthma

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0155/2021 of 16 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Dexmedetomidine (hydrochloride) - EMEA-002758-PIP01-19-M02

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

Day 60 opinion

Psychiatry

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0002/2022 of 7 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Daprodustat - EMEA-001452-PIP01-13-M04

GlaxoSmithKline Trading Services Limited; Treatment of anaemia due to chronic disorders

Day 60 opinion

Uro-nephrology / Haematology-Hemostaseology

Summary of Committee discussion:

In the written response before the PDCO's Day 60 discussion, the applicant addressed the remaining issues raised by the Committee.
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/0047/2021 of 27 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed) - EMEA-002330-PIP01-18-M02

Pfizer Europe MA EEIG; Prevention of disease caused by S*treptococcus pneumoniae*

Day 60 opinion

Vaccines

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee.
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/0380/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Hepatitis B (rDNA) surface antigen adjuvanted - EMEA-001127-PIP02-11-M02

Dynavax GmbH; Prevention of hepatitis B virus infection

Day 60 opinion

Vaccines

Summary of Committee discussion:

The PDCO has discussed this request for modification at D60, having taken into account the response from the applicant to the D30 minutes.

Based on the fact that the agreed PIP is restricted to primary series vaccination of naïve children, and HBV vaccines (monovalent or as combination vaccine) have been introduced into the expanded programme of immunization in naïve children in the past decades, the PDCO agreed with the applicant about infeasibility of the studies included in the PIP and

therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0304/2021 of 11 August 2021), extending the waiver to all subsets of the paediatric population.

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

2.3.18. *Neisseria meningitidis* serogroup W polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup Y polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup C polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M04

Sanofi Pasteur; Prevention of invasive meningococcal disease

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, 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/0285/2021 of 29 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Nirsevimab - EMEA-001784-PIP01-15-M04

AstraZeneca AB; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

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, the PDCO considered that proposed changes to timelines of Study 4 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/0296/2021 of 11 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. NVX-CoV2373 - EMEA-002941-PIP01-20-M02

Novavax CZ, a.s.; Prevention of coronavirus disease 2019 (COVID-19)

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 responses provided between Day 30 and Day 60, 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/0483/2021 of 3 December 2021).

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

2.3.21. 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-M05

Sanofi Pasteur; Prevention of influenza infection

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, 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/0042/2022 of 10 February 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Upadacitinib - EMEA-001741-PIP03-16-M02

AbbVie Ltd; Treatment of Crohn's disease

Day 30 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/0214/2020 of 17 June 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

The following partial compliance checks have been identified by the PME coordinator and PDCO rapporteur as not needing to be referred to the PDCO for discussion. The PDCO has been informed in writing.

2.7.1. Dupilumab - EMEA-C2-001501-PIP04-19-M01

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 30 letter

Gastroenterology-Hepatology

2.7.2. Chloroprocaine hydrochloride - EMEA-C1-000639-PIP03-16-M01

Sintetica GmbH; Peripheral nerve block (local anaesthesia by perineural injection)

Day 30 letter

Anaesthesiology

2.7.3. Formoterol fumarate dihydrate / glycopyrronium bromide / budesonide - EMEA-C1-002063-PIP01-16-M01

AstraZeneca AB; Treatment of asthma

Day 30 letter

Pneumology – Allergology

2.7.4. PEGylated-fibroblast growth factor 21 (BMS-986036) - EMEA-C2-002448-PIP01-18-M02

Bristol-Myers Squibb International Corporation; Treatment of non-alcoholic steatohepatitis (NASH)

Day 30 letter

Gastroenterology-Hepatology

2.7.5. Ivacaftor / lumacaftor - EMEA-C7-001582-PIP01-13-M10

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 letter

Other

2.7.6. Giroctocogene fitelparvovec - EMEA-C1-002724-PIP01-19-M02

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 30 letter

Haematology-Hemostaseology

3. Discussion of applications

Some information related to this section cannot be disclosed at the present time as it is deemed to contain commercially confidential information.

3.1. Discussions on Products D90-D60-D30

3.1.1. Oxytocin - Orphan - EMEA-003148-PIP01-21

OT4B; Treatment of Prader-Willi syndrome

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Venglustat - Orphan - EMEA-001716-PIP06-21

Genzyme Europe B.V.; Treatment of Fabry disease

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Efruxifermin - EMEA-003114-PIP01-21

Treatment of non-alcoholic fatty liver disease including non-alcoholic steatohepatitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.4. Etrasimod L-arginine - EMEA-002713-PIP02-21

Treatment of Crohn's disease

Day 90 discussion

Gastroenterology-Hepatology

3.1.5. Ritlecitinib - EMEA-002451-PIP02-21

Treatment of ulcerative colitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.6. Deucravacitinib - EMEA-002350-PIP04-21

Treatment of ulcerative colitis

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Gliadin protease - EMEA-003116-PIP01-21

Treatment of coeliac disease

Day 90 discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.1.8. Remibrutinib - EMEA-002582-PIP02-21

Treatment of multiple sclerosis

Day 90 discussion

Neurology

3.1.9. (R)-tetrahydrofuran-3-yl 4-(6-(5-(4-ethoxy-1-isopropylpiperidin-4-yl) pyridin-2-yl)pyrrolo[1,2-b]pyridazin-4-yl)piperazine-1-carboxylate sesquisuccinate - Orphan - EMEA-003133-PIP01-21

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 90 discussion

Other

3.1.10. Azelastine hydrochloride / mometasone furoate - EMEA-003122-PIP01-21

Treatment of seasonal allergic rhinitis

Day 90 discussion

Oto-rhino-laryngology

3.1.11. A 2'-O-(2'-methoxyethyl) modified antisense oligonucleotide targeting prekallikrein (PKK) mRNA - EMEA-003112-PIP01-21

Prevention of hereditary angioedema

Day 90 discussion

Pneumology - Allergology / Haematology-Hemostaseology

3.1.12. EMEA-003098-PIP01-21

Treatment of proteinuric chronic kidney disease

Day 90 discussion

Uro-nephrology

3.1.13. Lademirsen - Orphan - EMEA-003064-PIP01-21

Genzyme Europe B.V.; Treatment of Alport syndrome

Day 90 discussion

Uro-nephrology

3.1.14. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 90 discussion

Uro-nephrology

3.1.15. Vibegron - EMEA-001415-PIP02-21

Treatment of myoneurogenic bladder disorders

Day 90 discussion

Uro-nephrology

3.1.16. RSV F protein - EMEA-003094-PIP02-21

Prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV)

Day 90 discussion

Vaccines / Infectious Diseases

3.1.17. EMEA-003196-PIP01-22

Treatment of ulcerative colitis / Treatment of Crohn’s disease

Day 60 discussion

Gastroenterology-Hepatology

3.1.18. 6-[(3S,4S)-4-methyl-1-(pyrimidin-2-ylmethyl)pyrrolidin-3-yl]-3-tetrahydropyran-4-yl-7H-imidazo[1,5-a]pyrazin-8-one - Orphan - EMEA-002991-PIP02-22

Imara Inc.; Treatment of sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

Note: Withdrawal request received on 26 April 2022

3.1.19. Depemokimab - EMEA-003051-PIP05-22

Treatment of hypereosinophilic syndrome (HES)

Day 60 discussion

Haematology-Hemostaseology

3.1.20. Vidofludimus - EMEA-003195-PIP01-22

Treatment of multiple sclerosis

Day 60 discussion

Immunology-Rheumatology-Transplantation / Neurology

Note: Withdrawal request received on 19 May 2022

3.1.21. Exenatide acetate - Orphan - EMEA-003183-PIP02-22

Invex Therapeutics Ltd; Treatment of idiopathic intracranial hypertension

Day 60 discussion

Neurology

3.1.22. Troriluzole - Orphan - EMEA-003084-PIP03-22

Biohaven Pharmaceutical Ireland DAC; Treatment of hereditary spinocerebellar ataxia

Day 60 discussion

Neurology

3.1.23. Infigratinib - Orphan - EMEA-002594-PIP04-22

Helsinn Birex Pharmaceuticals Ltd.; Treatment of paediatric low-grade gliomas (LGG)

Day 60 discussion

Oncology

3.1.24. Efavaleukin alfa - EMEA-003156-PIP02-22

Treatment of ulcerative colitis

Day 60 discussion

Other

3.1.25. Depemokimab - EMEA-003051-PIP04-22

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 60 discussion

Pneumology - Allergology

3.1.26. Stiripentol - Orphan - EMEA-003200-PIP01-22

Biocodex SA; Treatment of primary hyperoxaluria

Day 60 discussion

Uro-nephrology

3.1.27. Acetylsalicylic acid / rosuvastatin - EMEA-003206-PIP01-22

Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

3.1.28. Ezetimibe / atorvastatin - EMEA-003205-PIP01-22

Prevention of cardiovascular events / Treatment of hypercholesterolaemia

Day 30 discussion

Cardiovascular Diseases

3.1.29. Milvexian - EMEA-003220-PIP01-22

Prevention of thromboembolism

Day 30 discussion

Cardiovascular Diseases

3.1.30. Zilebesiran - EMEA-003218-PIP01-22

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.31. EMEA-003223-PIP01-22

Treatment of palmoplantar pustulosis

Day 30 discussion

Dermatology

3.1.32. Nipocalimab - Orphan - EMEA-002559-PIP05-22

Janssen-Cilag International NV; Treatment of bullous pemphigoid

Day 30 discussion

Dermatology

3.1.33. Perflubutane - EMEA-003037-PIP02-22

Diagnostic evaluation of focal hepatic lesions

Day 30 discussion

Diagnostic / Oncology

3.1.34. Danuglipron - EMEA-002944-PIP02-22

Treatment of obesity / Treatment of chronic weight management

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. Doxribtimine / doxecitine - Orphan - EMEA-003210-PIP01-22

Zogenix ROI Limited; Treatment of thymidine kinase 2 deficiency

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.36. Insulin human (rDNA) - EMEA-003194-PIP02-22

Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.37. Inclaclumab - EMEA-003219-PIP01-22

Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.1.38. Synthetic peptide of 20 amino acids comprising a T cell epitope from pro-insulin - EMEA-002379-PIP01-22

Treatment of type 1 diabetes mellitus

Day 30 discussion

Immunology-Rheumatology-Transplantation / Endocrinology-Gynaecology-Fertility-Metabolism / Vaccines

3.1.39. EMEA-003222-PIP01-22

Treatment of SCN8A developmental and epileptic encephalopathy

Day 30 discussion

Neurology

3.1.40. Gold (Au) - EMEA-003211-PIP01-22

Treatment of amyotrophic lateral sclerosis (ALS)

Day 30 discussion

Neurology

3.1.41. Satralizumab - Orphan - EMEA-001625-PIP04-22

Roche Registration GmbH; Treatment of autoimmune encephalitis

Day 30 discussion

Neurology

3.1.42. Autologous CD4+ and CD8+ T cells transduced with lentiviral vector encoding a chimeric antigen receptor (CAR) directed against CD19 and preserving the T cell phenotype of the leukapheresis starting material - EMEA-003212-PIP01-22

Treatment of mature B cell neoplasms

Day 30 discussion

Oncology

3.1.43. Fianlimab - EMEA-003207-PIP01-22

Treatment of melanoma

Day 30 discussion

Oncology

3.1.44. Modakafusp alfa - EMEA-003217-PIP01-22

Treatment of multiple myeloma

Day 30 discussion

Oncology

3.1.45. Humanised IgG2 monoclonal antibody against interleukin-6 - EMEA-003215-PIP01-22

Treatment of macular oedema

Day 30 discussion

Ophthalmology

3.1.46. Humanised monoclonal antibody of IgG1 subtype targeting NRP1 - EMEA-003214-PIP01-22

Treatment of diabetic retinopathy

Day 30 discussion

Ophthalmology

3.1.47. Magnesium lactate dihydrate / tramadol - EMEA-003216-PIP01-22

Management of chronic pain in adults with osteoarthritis of the hip and/or knee

Day 30 discussion

Other

3.1.48. Pregabalin - EMEA-003221-PIP01-22

Treatment of postherpetic neuralgia / Treatment of neuropathic pain associated with diabetic peripheral neuropathy

Day 30 discussion

Pain / Neurology

3.1.49. Benzylamine derivative of benzofuran - EMEA-002974-PIP02-22

Treatment of C3 glomerulopathy

Day 30 discussion

Uro-nephrology

3.2. Discussions on Compliance Check

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

3.2.1. Apixaban - EMEA-C-000183-PIP01-08-M08

Bristol-Myers Squibb / Pfizer EEIG; Prevention of arterial thromboembolism

Day 30 discussion

Cardiovascular Diseases

3.2.2. Pegzilarginase - EMEA-C1-001925-PIP02-19

Immedica Pharma AB; Treatment of hyperargininaemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Obinutuzumab - Orphan - EMEA-001207-PIP02-19-M01

Roche Registration GmbH; Treatment of systemic lupus erythematosus

Day 30 discussion

Antineoplastic and immunomodulating agents

3.3.2. Chloroprocaine hydrochloride - EMEA-000639-PIP03-16-M02

Sintetica GmbH; Peripheral nerve block (local anaesthesia by perineural injection)

Day 30 discussion

Anaesthesiology

3.3.3. Bempedoic acid - EMEA-001872-PIP01-15-M02

Esperion Therapeutics, Inc.; Treatment of elevated cholesterol

Day 30 discussion

Cardiovascular Diseases

3.3.4. Etripamil - EMEA-002303-PIP01-17-M03

Milestone Pharmaceuticals, Inc.; Treatment of supraventricular tachycardia

Day 30 discussion

Cardiovascular Diseases

3.3.5. Omecamtiv mecarbil - EMEA-001696-PIP01-14-M02

Cytokinetics, Inc.; Treatment of heart failure

Day 30 discussion

Cardiovascular Diseases

3.3.6. Bimekizumab - EMEA-002189-PIP01-17-M03

UCB Biopharma SRL; Treatment of psoriasis

Day 30 discussion

Dermatology

3.3.7. Deucravacitinib - EMEA-002350-PIP01-18-M02

Bristol-Myers Squibb International Corporation; Treatment of psoriasis

Day 30 discussion

Dermatology

3.3.8. Fluciclovine (18F) - Orphan - EMEA-001644-PIP02-14-M03

Blue Earth Diagnostics Ireland Ltd; Diagnosis of amino acid metabolism in solid malignant tumours

Day 30 discussion

Diagnostic / Oncology

3.3.9. Pegunigalsidase alfa - Orphan - EMEA-001828-PIP01-15-M03

Chiesi Farmaceutici S.p.A; Treatment of Fabry disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.10. Cilofexor - Orphan - EMEA-002554-PIP02-19-M01

Gilead Sciences International Ltd.; Treatment of primary sclerosing cholangitis (DB96.2)

Day 30 discussion

Gastroenterology-Hepatology

3.3.11. Maralixibat chloride - Orphan - EMEA-001475-PIP02-13-M02

Mirum Pharmaceuticals; Treatment of Alagille syndrome

Day 30 discussion

Gastroenterology-Hepatology

3.3.12. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19-M02

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

Day 30 discussion

Haematology-Hemostaseology

3.3.13. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19-M02

Principia Biopharma, Inc.; Treatment of immune thrombocytopenia

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.14. Avibactam / ceftazidime - EMEA-001313-PIP01-12-M12

Pfizer Europe MA EEIG; Treatment of urinary tract infections / Treatment of intra-abdominal infections / Treatment of pneumonia / Treatment of infections due to aerobic gram-negative organisms

Day 30 discussion

Infectious Diseases

3.3.15. Bulevirtide - Orphan - EMEA-002399-PIP01-18-M01

Gilead Sciences International Ltd.; Treatment of chronic hepatitis D infection

Day 30 discussion

Infectious Diseases

3.3.16. Casirivimab - EMEA-002964-PIP01-21-M02

Roche Registration GmbH; Treatment of coronavirus disease 2019 (COVID-19) in hospitalised patients / Prevention of coronavirus disease 2019 (COVID-19) / Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.17. Enmetazobactam - EMEA-002240-PIP02-17-M01

Allecra Therapeutics GmbH; Treatment of urinary tract infections

Day 30 discussion

Infectious Diseases

3.3.18. Imdevimab - EMEA-002965-PIP01-21-M02

Roche Registration GmbH; Treatment of coronavirus disease 2019 (COVID-19) / Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.19. Pretomanid - Orphan - EMEA-002115-PIP01-17-M05

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

Day 30 discussion

Infectious Diseases

3.3.20. Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M02

Mundipharma Corporation (Ireland) Limited; Treatment of invasive candidiasis

Day 30 discussion

Infectious Diseases

3.3.21. Ritonavir / (1R,2S,5S)-N-{(1S)-1-cyano-2-[(3S)-2-oxopyrrolidin-3-yl]ethyl}-6,6-dimethyl-3-[3-methyl-N-(trifluoroacetyl)-L-valyl]-3-azabicyclo[3.1.0]hexane-2-carboxamide - EMEA-003081-PIP01-21-M01

Pfizer Europe MA EEIG; Treatment of coronavirus disease 2019 (COVID-19) / Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.22. Eptinezumab - EMEA-002243-PIP01-17-M03

H. Lundbeck A/S; Prevention of migraine headaches

Day 30 discussion

Neurology

3.3.23. Lasmiditan - EMEA-002166-PIP01-17-M06

Eli Lilly and Company Limited; Treatment of migraine with and without aura

Day 30 discussion

Neurology

3.3.24. Ravulizumab - EMEA-001943-PIP03-20-M01

Alexion Europe SAS; Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.3.25. Rimegepant - EMEA-002812-PIP02-20-M01

Biohaven Pharmaceutical Ireland DAC; Treatment of migraine headaches

Day 30 discussion

Neurology

3.3.26. Setmelanotide - Orphan - EMEA-002209-PIP01-17-M03

Rhythm Pharmaceuticals, Inc; Treatment of appetite and general nutrition disorders

Day 30 discussion

Nutrition

3.3.27. Bosutinib - EMEA-000727-PIP01-09-M06

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 30 discussion

Oncology

3.3.28. Larotrectinib - EMEA-001971-PIP03-18-M02

Bayer AG; Treatment of malignant neoplasms of the central nervous system

Day 30 discussion

Oncology

3.3.29. Lenvatinib - EMEA-001119-PIP02-12-M08

Eisai GmbH; Treatment of follicular carcinoma / Treatment of osteosarcoma / Treatment of papillary thyroid carcinoma

Day 30 discussion

Oncology

3.3.30. Andexanet alfa - EMEA-001902-PIP01-15-M06

AstraZeneca AB; Prevention of factor Xa inhibitor associated haemorrhage / Treatment of factor Xa inhibitor associated haemorrhage

Day 30 discussion

Other

3.3.31. Setrusumab - Orphan - EMEA-002169-PIP01-17-M01

Mereo Biopharma 3 Ltd; Treatment of osteogenesis imperfecta

Day 30 discussion

Other

3.3.32. Vamorolone - Orphan - EMEA-001794-PIP02-16-M05

ReveraGen BioPharma Ltd; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Other

3.3.33. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M03

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

Other / Pneumology - Allergology

3.3.34. Dermatophagoides farinae / dermatophagoides pteronyssinus - EMEA-001258-PIP01-11-M08

ALK-Abelló A/S; Treatment of asthma / Treatment of allergic rhinitis

Day 30 discussion

Pneumology - Allergology

3.3.35. Brexpiprazole - EMEA-001185-PIP01-11-M08

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of schizophrenia

Day 30 discussion

Psychiatry

3.3.36. Vortioxetine - EMEA-000455-PIP02-10-M09

H. Lundbeck A/S; Treatment of major depressive disorder

Day 30 discussion

Psychiatry

3.3.37. COVID-19 Vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M03

AstraZeneca AB; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 23 May 2022 for Nomination of Rapporteur and Peer reviewer

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

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

5.1. New Scientific Advice

No item

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

No item

5.3. General update on SAWP/PDCO interactions

5.3.1. General update on SAWP/PDCO interactions

Summary of Committee discussion:

A presentation was provided to the PDCO members as a reminder of the current process for PDCO input on scientific advices.

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

No item

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

9.1.1. PDCO membership

The PDCO Chair welcomed Kristin Karlsson representing Sweden as the new Member following the departure of Eva Agurell.

9.1.2. Vote by Proxy

No item

9.1.3. Mandate of PDCO Chairperson - call for nominations

Summary of Committee discussion:

The PDCO noted the information about the Chair and Vice-Chair elections planned to take place at the July and September 2022 plenary, respectively.

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 list of PIP-related CHMP procedures starting in April 2022, was presented to the PDCO members.

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

The Vice-Chair of the Non-clinical Working Party (NcWP) identified the products which will require NcWP 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.

9.3.3. Patients and Consumers Working Party (PCWP)/Healthcare Professionals Working Party (HCPWP)

Summary of Committee discussion:

The Meeting Summary PCWP-HCPWP meeting 2-3 March 2022, draft Agenda - PCWP-HCPWP Joint meeting - June 2022 and draft PCWP-HCPWP Work plan 2022-2025 were presented for information.

9.4. Cooperation within the EU regulatory network

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

No item

9.5. Cooperation with International Regulators

9.5.1. Report from the Paediatric Cluster Teleconference

Summary of Committee discussion:

The April and May 2022 minutes of the cluster were shared with the PDCO members for information.

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

No item

9.8. Planning and reporting

No item

10. Any other business

10.1. ALADDIN - Educational program on regulatory science in paediatric oncology

Summary of Committee discussion:

The PDCO was informed about the multi stakeholder educational program on regulatory science to which EMA contributes as associate partner, organised by ACCELERATE and funded by the European Commission.

10.2. COVID-19 update

Summary of Committee discussion:

The PDCO was updated on COVID-19 aspects relevant to paediatrics.

10.3. Exparel liposomal - EMEA/H/C/004586/II/0005 PDCO consultation from CHMP

Summary of Committee discussion:

A product-specific question from CHMP was presented to PDCO and the Committee appointed a small group of members to prepare a draft response.

10.4. Feedback from FDA paediatric oncology subcommittee of Oncologic Drugs Advisory Committee (ODAC)

PDCO member: Siri Wang, Karen Van Malderen

Summary of Committee discussion:

The PDCO was provided feedback from the paediatric oncology subcommittee of ODAC to which EMA and PDCO contributed.

10.5. New EMA Online Booking Tool

Summary of Committee discussion:

A presentation was provided to the PDCO members on the new EMA online booking tool.

11. Breakout sessions

11.1. Internal PDCO Operations

Summary of Committee discussion:

The PDCO discussed matters relating to internal operations.

11.2. Paediatric oncology

Summary of Committee discussion:

The breakout session was cancelled.

11.3. Neonatology

Summary of Committee discussion:

Discussion on accelerating access to current and novel antiretroviral drugs in neonates and product related matters.

11.4. Vaccines

Summary of Committee discussion:

The PDCO discussed relevant principles of COVID vaccines development in the paediatric population.

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 17-20 May 2022 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 interests declared	
Karl-Heinz Huemer	Member	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in final deliberations and voting on:	3.2.1. Apixaban - EMEA-C-000183-PIP01-08-M08
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Zena Gunther	Member	Cyprus	No interests declared	
Tereza Bazantova	Alternate	Czechia	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Louisa Braun Exner	Alternate	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Anne Paavola	Alternate	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	
Ágnes Gyurasics	Member (CHMP alternate)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No participation in discussion, final deliberations and voting on:	2.3.19. Nirsevimab - EMEA-001784-PIP01-15-M04
Dovile Zacharkiene	Member	Lithuania	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No restrictions applicable to this meeting	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No restrictions applicable to this meeting	
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 restrictions applicable to this meeting	
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	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	2.3.19. Nirsevimab - EMEA-001784-PIP01-15-M04
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:	2.3.19. Nirsevimab - EMEA-001784-PIP01-15-M04 3.3.37. COVID-19 Vaccine (ChAdOx1-S [recombinant]) - EMEA-

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
				002862-PIP01-20-M03
Jaroslav Sterba	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
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 participation in final deliberations and voting on:	3.2.2. Sacubitril / valsartan - EMEA-C-000316-PIP02-11-M05
María Estela Moreno Martín	Expert - via telephone*	Spain	No interests declared	
Celine Chu	Expert - via telephone*	France	No interests declared	
Kristin Karlsson	Expert - via telephone*	Sweden	No restrictions applicable to this meeting	
David Khan	Expert - via telephone*	Sweden	No interests declared	
Elita Poplavska	Expert - via telephone*	Latvia	No interests declared	
Margareta Bego	Expert - via telephone*	Croatia	No interests declared	
Ineta Popena	Expert - via telephone*	Latvia	No interests declared	
Janis Kurlovics	Expert - via telephone*	Latvia	No interests declared	
Emilie Birch Kristensen	Expert - via telephone*	Denmark	No restrictions applicable to this meeting	
Meeting run with support from relevant EMA staff				
* Experts were 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/