21 July 2023
EMA/PDCO/308544/2023
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 20-23 June 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

Health and safety information

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

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 Chair opened the meeting by welcoming all participants. The meeting was held in-person with some members connected 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 on the topics in the agenda of the meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda topics.

Participants were asked to declare any changes, omissions or errors to their declared interests concerning the matters for discussion. No new or additional competing interests were declared. Restrictions applicable to this meeting are captured in the List of participants included in the minutes.

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. All decisions taken at this meeting were made in the presence of a quorum of members. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The Chair thanked the departing members for their contributions to the Committee.

1.2. Adoption of agenda

The agenda for 20-23 June 2023 meeting was adopted with amendments.

1.3. Adoption of the minutes

The minutes for 23-26 May 2023 meeting were adopted with amendments 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. Furosemide - EMEA-003316-PIP01-22

Proveca Pharma Limited; Treatment of fluid retention

Day 120 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, a positive opinion for the PIP for the proposed medicine for children from birth to less than 18 years of age for the treatment of fluid retention was adopted.

2.1.2. Milvexian - EMEA-003220-PIP01-22

Janssen-Cilag International N.V.; Prevention of thromboembolism in patients with cardiovascular diseases

Day 120 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, a positive opinion for the PIP for the proposed medicine for children from 28 days to less than 18 years of age for the prevention of thromboembolism in patients with cardiovascular diseases was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 28 days of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.3. Interleukin-23 receptor antagonist peptide - EMEA-003301-PIP01-22

Janssen-Cilag International NV; Treatment of psoriasis

Day 120 opinion

Dermatology

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 paediatric patients from 6 years to 18 years of age, in the condition of treatment of psoriasis was adopted by the PDCO.
The PDCO agreed on a waiver in paediatric population from birth to less than 6 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.4. Povorcitinib - EMEA-003313-PIP01-22

Incyte Biosciences Distribution B.V.; Treatment of hidradenitis suppurativa

Day 120 opinion

Dermatology

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 the paediatric population from 12 years to less than 18 years, in the condition of treatment of hidradenitis suppurativa was adopted. The PDCO agreed on a waiver in children from birth to less than 12 years 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 granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.5. Ritlecitinib - EMEA-002451-PIP03-22

Pfizer Europe MAA EEIG; Treatment of vitiligo

Day 120 opinion

Dermatology

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 6 years to less than 18 years, in the condition of treatment of vitiligo was adopted. The PDCO agreed on a waiver in 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 over existing treatments. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.6. Encaleret - Orphan - EMEA-003348-PIP01-22

Calcilytix Therapeutics, Inc a BridgeBio Company; Treatment of hypoparathyroidism

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The applicant addressed the remaining issues raised by the Committee at Day 90.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion on a PIP for encaleret in the condition of treatment of hypoparathyroidism in the paediatric population from birth to less than 18 years of age was adopted. The PDCO granted a deferral for completion of this PIP.

2.1.7. Dirloctocogene samoparvovec - Orphan - EMEA-003290-PIP01-22

Spark Therapeutics Ireland Limited; Treatment of haemophilia A

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric

Committee, the PDCO issued a positive opinion on this paediatric investigational plan for dirloctocogene samoparvovec in the condition of treatment of moderate or severe haemophilia A without Factor VIII inhibitors.

2.1.8. Mocravimod - Orphan - EMEA-003304-PIP01-22

Priothera SAS; Treatment in haematopoietic stem cell transplantation in patients with acute myeloid leukaemia

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed at Day 120, during the June 2023 plenary meeting a paediatric investigation plan for mocravimod for the treatment in haematopoietic stem cell transplantation.
The PDCO confirmed all conclusions reached at Day 90 and noted that the applicant provided further information between Day 90 and Day 120.
Based on the assessment of this application and the additional information provided by the applicant, the PDCO adopted a positive opinion on a paediatric investigation plan for children from 12 years to less than 18 years, with a deferral for the treatment in haematopoietic stem cell transplantation (HSCT) in patients with acute myeloid leukaemia and a waiver for a subset of children from birth to less than 12 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.9. Recombinant human arylsulfatase A - Orphan - EMEA-002050-PIP02-22

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of metachromatic leukodystrophy

Day 120 opinion

Neurology

Note: Withdrawal request received on 13 June 2023

2.1.10. XEN1101 - EMEA-003271-PIP01-22

Xenon Pharmaceuticals Inc.; Treatment of focal onset seizures

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the June 2023 plenary meeting, an application for a paediatric investigation plan with a deferral and a waiver for XEN1101 for treatment of focal onset seizures.
The Committee 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 on a paediatric investigation plan with a deferral, but refused to grant a waiver for neonates (from birth to less than 1 month of age) on the grounds that the specific medicinal product is likely to be ineffective or unsafe in the paediatric population. The arguments provided by the applicant with regards to the possibility of development of an intravenous (IV) formulation were questioned and more data to support this claim would be expected to justify a waiver request.

2.1.11. Enomimeran / gindameran / ontasameran / vibosameran (BNT111) - EMEA-003274-PIP01-22

BioNTech SE; Treatment of melanoma

Day 120 opinion

Oncology

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 treatment of anti-programmed death 1/programmed death ligand 1 (PD-1/PD-L1)-refractory/relapsed, unresectable Stage III or IV cutaneous melanoma, in the condition of treatment of melanoma was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 12 years of age, on the grounds that the disease does not occur.

2.1.12. Dersimelagon - EMEA-002850-PIP03-22

Mitsubishi Tanabe Pharma GmbH; Treatment of systemic sclerosis

Day 120 opinion

Other

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 paediatric patients from 5 years to 18 years of age, in the condition of treatment of systemic sclerosis was adopted by the PDCO.
The PDCO agreed on a waiver in paediatric population from birth to less than 5 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 PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.13. mRNA encoding modified human ornithine transcarbamylase – Orphan – EMEA-003315-PIP01-22

Arcturus Therapeutics Europe B.V.; Treatment of ornithine transcarbamylase deficiency

Day 120 opinion

Other

Summary of Committee discussion:

The PDCO discussed at Day 120, during the June 2023 plenary meeting, an application for a paediatric investigation plan with a deferral for mRNA encoding modified human ornithine transcarbamylase in the treatment of ornithine transcarbamylase deficiency (OTD).
The Committee 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 on a paediatric investigation plan with a deferral.

2.1.14. Pamrevlumab - EMEA-002979-PIP04-22

FibroGen, Inc; Treatment of interstitial lung diseases with fibrosis

Day 120 opinion

Pneumology - Allergology

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 for the proposed medicine for the paediatric population from 28 days to less than 18 years of age, in the condition of treatment of interstitial lung diseases with fibrosis was adopted. The PDCO agreed on a waiver in a subset of children 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 granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.15. Repagermanium - Orphan - EMEA-003154-PIP01-21

Dimerix Bioscience Pty Ltd; Treatment of focal segmental glomerulosclerosis

Day 120 opinion

Uro-nephrology

Summary of Committee discussion:

The PDCO issued a positive opinion and agreed on a PIP for repagermanium capsules for the treatment of focal segmental glomerulosclerosis in children from 1 year of age. A waiver was granted for the paediatric population from birth to less than 1 year of age based on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The PIP includes a quality-related measure for the development of an age-appropriate capsule formulation, a double-blind, placebo-controlled trial in adolescents from 12 years of age (and adults) as well as an open-label, single arm trial in children from 1 year to less than 12 years of age. Moreover, the plan includes a population pharmacokinetics modelling study as well as a pharmacodynamics modelling study to support the extrapolation of efficacy from older patients to children.

2.1.16. Influenza recombinant H7 haemagglutinin - EMEA-003314-PIP01-22

Sanofi Pasteur; Prevention of influenza infection

Day 120 opinion

Vaccines

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO issued a positive opinion for influenza recombinant H7 haemagglutinin paediatric investigation plan for in the condition of prevention of influenza infection in subjects from birth to less than 18 years of age.

The PDCO agreed on a deferral in children from birth to less than 6 months of age. No waiver was agreed for this product.

2.1.17. Single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A and B (mRNA-1010) - EMEA-003346-PIP01-22

Moderna Biotech Spain S.L.; Prevention of influenza disease

Day 120 opinion

Vaccines

Summary of Committee discussion:
Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed on a PIP for single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A and B for all subsets of the paediatric population (from 6 weeks of age to less than 18 years of age) in the condition of prevention of influenza disease. The PIP contains a waiver in children below 6 weeks of age and a deferral.

2.1.18. MVA-BN-RSV (construct MVA-mBN294B) - EMEA-003185-PIP01-22

Bavarian Nordic A/S; Prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV)

Day 120 opinion

Vaccines / Infectious 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 PIP for MVA-BN-RSV (construct MVA-mBN294B) for the paediatric population from 2 months of age to less than 18 years of age) in the condition prevention of lower respiratory tract disease (LRTD) caused by RSV. The PIP contains a waiver for children below 2 months of age and a deferral.

2.1.19. Semaglutide - EMEA-001441-PIP08-23

Novo Nordisk A/S; Prevention of cardiovascular events in patients with atherosclerosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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 semaglutide for all subsets of the paediatric population (0 to 18 years of age) in the condition of prevention of cardiovascular events in patients with atherosclerosis, on the grounds that the condition for which the specific medicinal product is intended occurs only in adult population.

2.1.20. Cagrilintide / semaglutide - EMEA-003059-PIP02-23

Novo Nordisk A/S; Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO confirmed the outcome of Day 30 discussion and 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 cagrilintide / semaglutide for the paediatric population from birth to less than 10 years of age for the condition of treatment of type 2 diabetes mellitus on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset and a waiver for cagrilintide / semaglutide for the paediatric population from 10 to less than 18 years of age for the condition of treatment of type 2 diabetes mellitus on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
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.

2.1.21. Rilzabrutinib - Orphan - EMEA-002438-PIP04-23

Sanofi B.V.; Treatment of autoimmune haemolytic anaemia

Day 60 opinion

Haematology-Hemostaseology

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 autoimmune haemolytic anaemia on the grounds of lack of significant therapeutic benefit.
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.

2.1.22. Sotuletinib - Orphan - EMEA-003415-PIP01-23

Novartis Europharm Limited; Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Neurology

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 sotuletinib for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of amyotrophic lateral sclerosis (ALS).
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.

2.1.23. Atezolizumab / tiragolumab - EMEA-003418-PIP01-23

Roche Registration GmbH; Treatment of lung carcinoma (non-small cell carcinoma)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the June 2023 plenary meeting, an application for a waiver for atezolizumab / tiragolumab for treatment of lung carcinoma (non-small cell carcinoma).
Based on the assessment of this application the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for atezolizumab / tiragolumab for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of lung carcinoma (non-small cell carcinoma) 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. 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.

2.1.24. Autologous dendritic cells pulsed with allogeneic tumour cell lysate - Orphan - EMEA-002381-PIP02-23

Amphera BV; Treatment of pancreatic cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the June 2023 plenary meeting, a request for a product-specific waiver for autologous dendritic cells pulsed with allogeneic tumour cell lysate for the treatment of pancreatic cancer on the basis that this product would lack significant therapeutic benefit for children with pancreatic cancer, since clinical studies would not be feasible.
The PDCO confirmed all conclusions reached at Day 30 and adopted a positive opinion at Day 60 for autologous dendritic cells pulsed with allogeneic tumour cell lysate for the treatment of pancreatic cancer on the grounds that this disease occurs only in adult populations.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.
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.

2.1.25. Domvanalimab - EMEA-003429-PIP01-23

Gilead Sciences International Ltd.; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from the Day 30 discussion.
Based on the assessment of this application and further discussions at the Paediatric Committee, PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for domvanalimab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung cancer, based on the ground that the diseases do not occur in children. Since the agreed waiver ground is disease not occurring, 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.

2.1.26. Domvanalimab - EMEA-003429-PIP02-23

Gilead Sciences International Ltd.; Treatment of gastric and gastroesophageal junction adenocarcinoma / Treatment of oesophageal carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the June 2023 plenary meeting, a request for a product-specific waiver for domvanalimab for the treatment of gastric, gastroesophageal junction, and oesophageal adenocarcinoma on the grounds that the disease does not occur in the paediatric population.
The PDCO confirmed all conclusions reached at Day 30 and adopted a positive opinion at Day 60 for domvanalimab for the treatment of gastric and gastroesophageal junction adenocarcinoma and for the treatment of oesophageal carcinoma on the grounds that this disease occurs only in adult populations.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.
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.

2.1.27. Tazemetostat - Orphan - EMEA-003055-PIP02-23

Ipsen Pharma; Treatment of follicular lymphoma

Day 60 opinion

Oncology

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 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 follicular lymphoma 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 applicant agreed with extending the waiver to all pharmaceutical forms and all routes of administration.

2.1.28. Zimberelimab - EMEA-003427-PIP01-23

Gilead Sciences International Ltd.; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for zimberelimab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung cancer, based on the ground that the diseases do not occur in children. Since the agreed waiver ground is disease not occurring, 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.

2.1.29. Zimberelimab - EMEA-003427-PIP02-23

Gilead Sciences International Ltd.; Treatment of gastric and gastroesophageal junction adenocarcinoma / Treatment of oesophageal carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the June 2023 plenary meeting, a product-specific waiver for zimberelimab for the treatment of gastric, gastroesophageal junction, and oesophageal adenocarcinoma on the grounds that the disease does not occur in the paediatric population.
The PDCO confirmed all conclusions reached at Day 30 and adopted a positive opinion at Day 60 for zimberelimab for the treatment of gastric and gastroesophageal junction adenocarcinoma and for the treatment of oesophageal carcinoma on the grounds that this disease occurs only in adult populations.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.
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.

2.1.30. Satralizumab - Orphan - EMEA-001625-PIP05-23

Roche Registration GmbH; Treatment of thyroid eye disease

Day 60 opinion

Ophthalmology / Neurology

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 satralizumab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of thyroid eye disease (TED) on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments in all age paediatric subsets.
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.

2.1.31. Sodium lactate / xylitol / magnesium chloride hexahydrate / calcium chloride dihydrate / sodium chloride / L-carnitine / D-glucose - EMEA-003417-PIP01-23

Treatment of patients in need of peritoneal dialysis

Day 60 opinion

Uro-nephrology

Note: Withdrawal request received on 12 June 2023

2.2. Opinions on Compliance Check

2.2.1. Clascoterone - EMEA-C-003330-PIP01-22

Cassiopea S.p.A; Treatment of acne vulgaris

Day 60 opinion

Dermatology

Summary of Committee discussion:

The PDCO adopted on 23 June 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0076/2023) of 10 March 2023.

2.2.2. Marstacimab - EMEA-C1-002285-PIP02-19-M02

Pfizer Europe MA EEIG; Treatment of congenital haemophilia A

Day 60 letter

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO discussed the completed study and considered that it is compliant with the latest Agency's Decision (P/0443/2022) of 28 October 2022.

The PDCO finalised this partially completed compliance procedure on 23 June 2023.

2.2.3. Rilpivirine (hydrochloride) - EMEA-C-000317-PIP01-08-M13

Janssen-Cilag International NV; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures: EMEA-C1-000317-PIP01-08-M01, EMEA-C2-000317-PIP01-08-M07.
The PDCO adopted an opinion (narrowly) confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0101/2022) of 17 March 2022.

2.2.4. Teriflunomide - EMEA-C-001094-PIP01-10-M04

Sanofi Aventis Groupe; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

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

- EMEA-C1-001094-PIP01-10
- EMEA-C2-001094-PIP01-10-M04

The PDCO adopted on 23 June 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0165/2017) of 3 July 2017.

2.2.5. Imetelstat - EMEA-C1-001910-PIP03-20-M01

Geron Corporation; Treatment of myelodysplastic syndromes (MDS), including juvenile myelomonocytic leukaemia (JMML)

Day 60 letter

Oncology

Summary of Committee discussion:

The PDCO discussed the completed study(ies) and considered that these are compliant with the latest Agency's Decision (P/0517/2022) of 30 December 2022.
The PDCO finalised this partially completed compliance procedure on 23 June 2023.

2.2.6. Peanut (*Arachis hypogaea*) allergen powder (previously known as peanut flour) - EMEA-C-001734-PIP01-14-M06

Aimmune Therapeutics Ireland Ltd; Treatment of peanut allergy

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

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

- EMEA-C1-001734-PIP01-14-M04
- EMEA-C2-001734-PIP01-14-M04

The PDCO adopted on 23 June 2023 an opinion confirming the compliance of all studies in

the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0103/2023) of 25 March 2023.

2.2.7. Spesolimab - EMEA-C-002475-PIP02-19-M02

Boehringer Ingelheim International GmbH; Treatment of generalised pustular psoriasis / Prevention of generalised pustular psoriasis

Day 30 opinion

Dermatology

Summary of Committee discussion:

The PDCO adopted on 23 June 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0049/2022) of 18 February 2022.

2.2.8. Elosulfase alfa - EMEA-C-000973-PIP01-10-M04

BioMarin International Limited; Treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome)

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO discussed at Day 30, during the June 2023 plenary meeting, a request for full compliance check for elosulfase alpha for the treatment of mucopolysaccharidosis type IVA (Morquio A syndrome).
The PDCO took note of outcomes of the preceding partial compliance check procedures:

- EMEA-C1-000973-PIP01-10-M01
- EMEA-C2-000973-PIP01-10-M03

and adopted on 26 June 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0170/2023) of 15 May 2023.

2.2.9. Lanthanum carbonate hydrate - EMEA-C-000637-PIP02-10-M07

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of hyperphosphataemia

Day 30 opinion

Uro-nephrology

Summary of Committee discussion:

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0507/2022) of 2 December 2022.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22-M01

Krystal Biotech, Inc.; Treatment of dystrophic epidermolysis bullosa

Day 60 opinion

Dermatology

Summary of Committee discussion:

The applicant addressed the remaining issues raised by the Committee at Day 30.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that some of the proposed changes could be accepted. A deferral for one or more measures in the paediatric investigation plan was granted whereas the waiver request for some of the subsets of the paediatric population was refused for not meeting the grounds detailed in Article 11(1).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0464/2022 of 28 October 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Dupilumab - EMEA-001501-PIP07-20-M01

Sanofi Winthrop Industrie; Treatment of chronic spontaneous urticaria

Day 60 opinion

Dermatology

Summary of Committee discussion:

The applicant addressed most of the remaining issues raised by the Committee at Day 30.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that some 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/0153/2021 of 16 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Inclisiran - EMEA-002214-PIP01-17-M02

Novartis Europharm Ltd.; Treatment of elevated cholesterol

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0495/2022 of 2 December 2022).

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

2.3.4. Obeticholic acid - Orphan - EMEA-001304-PIP02-13-M07

Advanz Pharma Limited; Treatment of primary biliary cirrhosis / Treatment of biliary atresia

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 modification concerned an update to the design of Study 9 (randomised, placebo-controlled study to evaluate the efficacy, safety, tolerability, pharmacokinetics, and pharmacodynamics of obeticholic acid in children from birth to less than 18 years with biliary atresia, post-hepatoportoenterostomy).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0041/2021 of 27 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. (S)-2-(5-((3-ethoxypyridin-2-yl)oxy)pyridin-3-yl)-N-(tetrahydrofuran 3 yl) pyrimidine-5-carboxamide (PF-06865571) - EMEA-002773-PIP01-20-M01

Pfizer Europe MA EEIG; Treatment of non-alcoholic steatohepatitis

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. This request for modification concerned a delay to the entire development programme.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0102/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Iron as ferric maltol - EMEA-001195-PIP01-11-M06

Norgine BV; Treatment of iron deficiency anaemia (IDA)

Day 60 opinion

Haematology-Hemostaseology

Note: Withdrawal request received on 16 June 2023

2.3.7. Ibrutinib - EMEA-001397-PIP04-17-M02

Janssen-Cilag International NV; Treatment of chronic graft-versus-host disease

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 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/0421/2019 of 6 December 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Tofacitinib citrate - EMEA-000576-PIP01-09-M15

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

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 to the statistical analysis for Study 7, changes on the design, objectives, methodology and data to be used in Study 11 and updates to Studies 10 and 13 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/0102/2023 of 24 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Delandistrogene moxeparvovec - Orphan - EMEA-002677-PIP01-19-M03

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 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/0230/2022 of 8 July 2022).

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

2.3.10. Erenumab - EMEA-001664-PIP02-15-M06

Novartis Europharm Limited; Prevention of migraine headaches

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 and the responses provided to the issues raised in May 2023, 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/0475/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Lacosamide - EMEA-000402-PIP03-17-M07

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

Day 60 opinion

Neurology

Note: Withdrawal request received on 23 June 2023

2.3.12. Abemaciclib - EMEA-002342-PIP01-18-M03

Eli Lilly and Company Limited; Treatment of Ewing's sarcoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the June 2023 plenary meeting, a request for modification for abemaciclib for the treatment of Ewing's sarcoma. The applicant requested to modify one of the secondary endpoints in a clinical study.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be acceptable. Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0440/2021 of 29 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Abemaciclib - EMEA-002342-PIP02-18-M02

Eli Lilly and Company Limited; Treatment of glioma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the June 2023 plenary meeting, a request for modification for abemaciclib for the treatment of glioma and for the treatment of neuroblastoma. The applicant requested to delete the part of the PIP for the treatment of neuroblastoma and to include it in another PIP. In addition, the applicant proposed changes to one of the clinical studies.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be acceptable. Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0439/2021 of 29 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Avapritinib - Orphan - EMEA-002358-PIP02-18-M03

Blueprint Medicines (Netherlands) B.V.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 opinion

Oncology

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 applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0016/2022 of 31 January 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M06

Ipsen Pharma; Treatment of malignant solid tumours

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/0357/2022 of 11 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Olaparib - EMEA-002269-PIP01-17-M02

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

Day 60 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the 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/0250/2020 of 15 July 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Quizartinib - Orphan - EMEA-001821-PIP01-15-M07

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology

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 applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0091/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Bupropion (hydrochloride) / naltrexone (hydrochloride) - EMEA-001373-PIP01-12-M05

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 60 opinion

Other

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 to timelines, duration, primary endpoint and definition of the population for analysis of Study 5 could be accepted, along with a delay in the completion of Studies 4, 6 and 7, and in the PIP completion date.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0390/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Tirzepatide - EMEA-002360-PIP02-22-M01

Eli Lilly and Company; Treatment of obesity

Day 60 opinion

Other

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 modification concerned updates to Study 2 (randomised, double-blind, parallel-arm, multicentre, placebo controlled trial to assess the efficacy, safety, and pharmacokinetics (PK) of tirzepatide as an adjunct to lifestyle intervention in children and adolescents aged from 12 years to less than 18 years with overweight and obesity).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0033/2023 of 31 January 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Ketamine / sufentanil - EMEA-001739-PIP02-16-M02

Cessatech A/S; Treatment of acute pain

Day 60 opinion

Pain

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 on the completion dates of Study 4 (PDC 01-0205), Study 6 (PDC 01-0202), Study 7 (PDC 01-0207) and Study 8 (PDC 01-0208) and overall completion date of the PIP could be accepted.
The modification also involved a change in the agreed measures for Study 6 (PDC 01-0202) regarding the sample size and secondary endpoints.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0177/2022 of 13 May 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Durvalumab - EMEA-002028-PIP01-16-M04

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue)

Day 30 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/0366/2022 of 9 September 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Tremelimumab - EMEA-002029-PIP01-16-M04

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue)

Day 30 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/0366/2022 of 9 September 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Sarilumab - EMEA-001045-PIP01-10-M03

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

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, which concerned timelines and wordings of secondary endpoints, 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/0114/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Mexiletine (hydrochloride) - Orphan - EMEA-002012-PIP01-16-M04

Lupin Europe GmbH; Treatment of myotonic disorders

Day 30 opinion

Other

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/0365/2021 of 8 September 2021).
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. Alprazolam - EMEA-C1-003043-PIP01-21

UCB Pharma SA; Treatment of epileptic seizures

Day 30 letter

Neurology

2.7.2. Resmetirom - EMEA-C1-003087-PIP01-21

Madrigal Pharmaceuticals EU Limited; Treatment of non-alcoholic steatohepatitis

Day 30 letter

Gastroenterology-Hepatology

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. Dexmedetomidine - EMEA-003283-PIP01-22

Sedation

Day 90 discussion

Anaesthesiology

3.1.2. Barzolvolimab - EMEA-003327-PIP01-22

Treatment of chronic spontaneous urticaria

Day 90 discussion

Dermatology

Note: Withdrawal request received on 10 July 2023

3.1.3. Isotretinoin - Orphan - EMEA-003303-PIP01-22

Timber Pharmaceuticals LLC; Treatment of congenital ichthyosis

Day 90 discussion

Dermatology

3.1.4. Upadacitinib - EMEA-001741-PIP07-22

Treatment of vitiligo

Day 90 discussion

Dermatology

3.1.5. Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 - Orphan - EMEA-003253-PIP01-22

Sanofi B.V.; Treatment of achondroplasia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.6. Recombinant human tissue nonspecific alkaline phosphatase (TNSALP) fragment crystallizable (Fc) deca aspartate fusion protein - EMEA-003343-PIP01-22

Treatment of hypophosphatasia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. Govorestat - Orphan - EMEA-003365-PIP01-22

Applied Therapeutics, Inc; Treatment of galactosaemia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Ophthalmology / Neurology

3.1.8. EMEA-003002-PIP03-22

Treatment of portal hypertension

Day 90 discussion

Gastroenterology-Hepatology

3.1.9. Obinutuzumab - Orphan - EMEA-001207-PIP05-22

Roche Registration GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.10. EMEA-003326-PIP01-22

Treatment of infections caused by gram-negative organisms / Complicated urinary tract infections (cUTI) / Hospital associated pneumonia or ventilator associated pneumonia

Day 90 discussion

Infectious Diseases

3.1.11. Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP02-22

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases

3.1.12. Sodium ({(2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2-yl}oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) - Orphan - EMEA-003344-PIP01-22

Calico Life Sciences LLC; Treatment of vanishing white matter disease

Day 90 discussion

Neurology

3.1.13. Vesleteplirsen - EMEA-003305-PIP01-22

Treatment of Duchenne muscular dystrophy

Day 90 discussion

Neurology

3.1.14. Retatrutide - EMEA-003258-PIP01-22

Treatment of obesity

Day 90 discussion

Other

3.1.15. An acellular tubular graft composed of human collagen types I and III and other extracellular matrix proteins, including fibronectin and vitronectin - EMEA-003345-PIP01-22

Treatment of vascular injuries

Day 90 discussion

Other

3.1.16. Dexpramipexole - EMEA-003328-PIP01-22

Treatment of asthma

Day 90 discussion

Pneumology - Allergology

3.1.17. EMEA-003319-PIP02-22

Treatment of major depressive disorder (MDD)

Day 90 discussion

Psychiatry

3.1.18. EMEA-003319-PIP03-22

Treatment of post-traumatic stress disorder (PTSD)

Day 90 discussion

Psychiatry

3.1.19. Remibrutinib - EMEA-002582-PIP03-23

Treatment of chronic inducible urticaria

Day 60 discussion

Dermatology

3.1.20. Spesolimab - EMEA-002475-PIP04-23

Treatment of hidradenitis suppurativa

Day 60 discussion

Dermatology

3.1.21. EMEA-003414-PIP01-23

Treatment of type 2 diabetes mellitus

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.22. EMEA-003414-PIP02-23

Treatment of obesity

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.23. Frexalimab - EMEA-002945-PIP03-23

Treatment of type 1 diabetes mellitus

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. Mavodelpar - Orphan - EMEA-003331-PIP02-23

Reneo Pharmaceuticals Inc; Treatment of primary mitochondrial disorders

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. Modified messenger ribonucleic acid encoding human propionyl-coenzyme a carboxylase alpha and beta subunits encapsulated into lipid nanoparticles - Orphan - EMEA-003419-PIP01-23

Moderna Biotech Spain, S.L.; Treatment of propionic acidaemia

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.26. Sepiapterin - Orphan - EMEA-003027-PIP02-23

PTC Therapeutics International; Treatment of hyperphenylalaninaemia

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.27. Cobitolimod - Orphan - EMEA-001589-PIP02-23

InDex Pharmaceuticals AB; Treatment of ulcerative colitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.28. Autologous CD34+ cells transduced with a lentiviral vector containing a codon-optimised RPS19 gene - Orphan - EMEA-003074-PIP02-23

Apriligen LLC; Treatment of Diamond-Blackfan anaemia

Day 60 discussion

Haematology-Hemostaseology

3.1.29. Belumosudil - Orphan - EMEA-003425-PIP01-23

Sanofi Winthrop Industrie; Treatment of transplant complications

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.30. Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP03-23

Treatment of lower respiratory tract and lung infections

Day 60 discussion

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

3.1.31. Abemaciclib - EMEA-002342-PIP04-23

Treatment of neuroblastoma

Day 60 discussion

Oncology

3.1.32. Blinatumomab - Orphan - EMEA-000574-PIP03-23

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia

Day 60 discussion

Oncology

3.1.33. Olutasidenib - Orphan - EMEA-003421-PIP01-23

Rigel Pharmaceuticals B.V.; Treatment of acute myeloid leukaemia

Day 60 discussion

Oncology

3.1.34. Volrustomig - EMEA-003423-PIP01-23

Treatment of lung cancer / Treatment of renal cell carcinoma / Treatment of cervical carcinoma / Treatment of mesothelioma

Day 60 discussion

Oncology

3.1.35. Cedazuridine / decitabine - Orphan - EMEA-003071-PIP02-23

Otsuka Pharmaceutical Netherlands B.V.; Treatment of myelodysplastic syndromes

Day 60 discussion

Oncology / Haematology-Hemostaseology

3.1.36. Iptacopan - EMEA-002705-PIP05-23

Treatment of immune-complex mediated membranoproliferative glomerulonephritis (IC-MPGN)

Day 60 discussion

Other

3.1.37. Autologous cell product composed of CD34+ enriched haematopoietic stem cells (HSCs) that have been genetically modified to express the functional β-glucocerebrosidase (GCase) protein - Orphan - EMEA-003424-PIP01-23

AVROBIO, Inc.; Treatment of Gaucher disease type 1 / Treatment of Gaucher disease type 2 / Treatment of Gaucher disease type 3

Day 60 discussion

Other / Pain / Endocrinology-Gynaecology-Fertility-Metabolism / Pneumology - Allergology / Gastroenterology-Hepatology / Haematology-Hemostaseology / Neurology

3.1.38. mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 - EMEA-003426-PIP01-23

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Vaccines / Infectious Diseases

3.1.39. Obicetrapib - EMEA-003438-PIP01-23

Treatment of mixed dyslipidaemia

Day 30 discussion

Cardiovascular Diseases

3.1.40. Obicetrapib - EMEA-003438-PIP02-23

Treatment of elevated cholesterol

Day 30 discussion

Cardiovascular Diseases

3.1.41. Ramipril / nebivolol - EMEA-003430-PIP01-23

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.42. Modified mRNA encoding human methylmalonyl-coenzyme A mutase containing a polymorphism at position 671 - Orphan - EMEA-003437-PIP01-23

Moderna Biotech Spain, S.L.; Treatment of methylmalonic acidaemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.43. Navepegritide - Orphan - EMEA-002689-PIP02-23

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.44. Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-003420-PIP01-23

Arrowhead Pharmaceuticals, Inc.; Treatment of familial chylomicronemia syndrome (FCS)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.45. Dupilumab - EMEA-001501-PIP12-23

Treatment of eosinophilic gastroenteritis

Day 30 discussion

Gastroenterology-Hepatology

3.1.46. Pemafibrate - EMEA-001573-PIP03-23

Treatment of primary biliary cholangitis

Day 30 discussion

Gastroenterology-Hepatology

3.1.47. Tarperprumig - EMEA-003432-PIP01-23

Treatment of patients with sickle cell disease (SCD)

Day 30 discussion

Haematology-Hemostaseology

3.1.48. Amiodarone - EMEA-003428-PIP01-23

Treatment of supraventricular tachyarrhythmias in children from birth to less than 18 years of age

Day 30 discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

3.1.49. Ganaxolone - Orphan - EMEA-002341-PIP02-23

Marinus Pharmaceuticals Inc.; Treatment of tuberous sclerosis complex

Day 30 discussion

Neurology

3.1.50. Tralesinidase alfa - Orphan - EMEA-003433-PIP01-23

Labcorp Clinical Development Limited; Treatment of mucopolysaccharidosis type III B (Sanfilippo B syndrome)

Day 30 discussion

Neurology

3.1.51. Abemaciclib - EMEA-002342-PIP05-23

Treatment of prostate cancer

Day 30 discussion

Oncology

3.1.52. Binimetinib - EMEA-001454-PIP06-23

Treatment of lung cancer

Day 30 discussion

Oncology

3.1.53. Encorafenib - EMEA-001588-PIP04-23

Treatment of non-small cell lung cancer

Day 30 discussion

Oncology

3.1.54. Enfortomab vedotin - EMEA-002299-PIP02-23

Treatment of head and neck epithelial malignant neoplasms

Day 30 discussion

Oncology

3.1.55. Ivonescimab - EMEA-003378-PIP01-23

Treatment of lung cancer

Day 30 discussion

Oncology

3.1.56. Lerociclib - EMEA-003436-PIP01-23

Treatment of endometrial cancer

Day 30 discussion

Oncology

3.1.57. Modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens - EMEA-003434-PIP01-23

Treatment of melanoma / Treatment of lung cancer

Day 30 discussion

Oncology

3.1.58. Allogeneic faecal microbiota, pooled - EMEA-003435-PIP01-23

Treatment in allogeneic haematopoietic stem cell transplantation

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.59. Alpelisib - Orphan - EMEA-002016-PIP05-23

Novartis Europharm Limited; Treatment of lymphatic malformations associated with a PIK3CA mutation

Day 30 discussion

Other

3.1.60. Clotrimazole - EMEA-003431-PIP01-23

Treatment of fungal otitis externa (otomycosis)

Day 30 discussion

Oto-rhino-laryngology

3.1.61. EMEA-002990-PIP02-23

Treatment of major depressive disorder

Day 30 discussion

Psychiatry

3.1.62. Ferric citrate coordination complex (FCCC) - EMEA-001213-PIP03-23

Treatment of anaemias due to chronic kidney disorders

Day 30 discussion

Uro-nephrology

3.1.63. Ravulizumab - EMEA-001943-PIP06-23

Prevention of death, kidney replacement therapy, or sustained reduction in kidney function in high-risk adult patients with stage 3 or 4 chronic kidney disease undergoing cardiopulmonary bypass for coronary artery bypass graft, valve replacement or repair, or combined procedure

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. Saxagliptin - EMEA-C-000200-PIP01-08-M10

AstraZeneca AB; Treatment of type 2 diabetes

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Dupilumab - EMEA-C-001501-PIP04-19-M02

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 30 discussion

Gastroenterology-Hepatology

3.2.3. Ex vivo expanded autologous human corneal epithelial cells containing stem cells - EMEA-C-001082-PIP02-11-M03

Holostem Terapie Avanzate S.r.l.; Treatment of limbal stem cell deficiency due to ocular burns

Day 30 discussion

Ophthalmology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Apixaban - EMEA-000183-PIP02-12-M04

Bristol-Myers Squibb / Pfizer EEIG; Treatment of venous thromboembolism

Day 30 discussion

Cardiovascular Diseases

3.3.2. Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19-M01

CUTISS AG; Treatment of burns

Day 30 discussion

Dermatology

3.3.3. Bimekizumab - EMEA-002189-PIP01-17-M04

UCB Biopharma SRL; Treatment of psoriasis

Day 30 discussion

Dermatology

3.3.4. Norucholic acid - Orphan - EMEA-002485-PIP01-18-M01

Dr. Falk Pharma GmbH; Treatment of autoimmune sclerosing cholangitis / Treatment of primary sclerosing cholangitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.5. Risankizumab - EMEA-001776-PIP03-17-M01

AbbVie Ltd; Treatment of Crohn's disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.6. Ustekinumab - EMEA-000311-PIP04-13-M06

Janssen-Cilag International NV; Treatment of Crohn's disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.7. Concizumab - Orphan - EMEA-002326-PIP04-20-M01

Novo Nordisk A/S; Treatment of congenital haemophilia A and B

Day 30 discussion

Haematology-Hemostaseology

3.3.8. Danicopan - Orphan - EMEA-002310-PIP01-17-M01

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Haematology-Hemostaseology

3.3.9. Etranacogene dezaparvovec - Orphan - EMEA-002722-PIP01-19-M03

CSL Behring GmbH; Treatment of haemophilia B

Day 30 discussion

Haematology-Hemostaseology

3.3.10. Ravulizumab (ALXN1210) - Orphan - EMEA-002077-PIP01-16-M06

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Haematology-Hemostaseology

3.3.11. Avacopan - Orphan - EMEA-002023-PIP01-16-M07

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.12. Guselkumab - EMEA-001523-PIP03-18-M03

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.13. Baloxavir marboxil - EMEA-002440-PIP01-18-M05

Roche Registration GmbH; Treatment of influenza / Prevention of influenza

Day 30 discussion

Infectious Diseases

3.3.14. Fostemsavir (tromethamine) - EMEA-001687-PIP01-14-M07

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

Day 30 discussion

Infectious Diseases

3.3.15. Tedizolid phosphate - EMEA-001379-PIP01-12-M08

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

Day 30 discussion

Infectious Diseases

3.3.16. Tenofovir alafenamide - EMEA-001584-PIP01-13-M07

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B

Day 30 discussion

Infectious Diseases

3.3.17. Fremanezumab - EMEA-001877-PIP01-15-M04

Teva GmbH; Prevention of migraine headaches

Day 30 discussion

Neurology

3.3.18. Rozanolixizumab - Orphan - EMEA-002681-PIP01-19-M02

UCB Pharma S.A; Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.3.19. Bosutinib - EMEA-000727-PIP01-09-M07

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 30 discussion

Oncology

3.3.20. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M03

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Oncology

3.3.21. Nivolumab - EMEA-001407-PIP02-15-M06

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of the central nervous system / Treatment of malignant neoplasms of lymphoid tissue

Day 30 discussion

Oncology

3.3.22. Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 - Orphan - EMEA-003232-PIP01-22-M01

Inozyme Pharma Ireland Limited; Treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency

Day 30 discussion

Other / Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.3.23. Ravulizumab (ALXN1210) - Orphan - EMEA-001943-PIP01-16-M08

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 30 discussion

Uro-nephrology

3.3.24. Davesomeran / imelasomeran / elasomeran - EMEA-002893-PIP01-20-M04

Moderna Biotech Spain, S.L.; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Vaccines

3.3.25. Recombinant SARS-CoV-2 spike protein - EMEA-002915-PIP01-20-M02

Sanofi Pasteur; 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 10 July 2023 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

No item

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

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

6.1.1. Allogeneic adipose-derived mesenchymal stem cells - EMEA-04-2023

Magellan Stem Cells Pty Ltd; All classes of medicinal products for treatment of primary and secondary osteoarthrosis / Treatment of osteoarthritis of the knee and hip

Summary of Committee discussion:

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

Other potential paediatric interests of this medicine suggested by PDCO: genetic bone disorders, other forms of arthritis with inflammation such juvenile idiopathic arthritis.

6.1.2. Nanrilkefusp alfa - EMEA-05-2023

SOTIO Biotech AG; The class of immunomodulatory cytokine medicinal products for treatment of skin malignant neoplasms / Treatment of skin squamous-cell carcinoma (alone or in combination with pembrolizumab)

Summary of Committee discussion:

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

Other potential paediatric interests of this medicine suggested by PDCO: paediatric tumours as identified in Pearson et al., 2020 (https://doi.org/10.1016/j.ejca.2019.12.029).

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 Chair thanked Karl-Heinz Huemer as a member of Austria, Irja Lutsar as a member for Estonia and Nanna Borup Johansen as a member of Denmark for their contribution.

The Chair announced that Johanna Wernsperger is the new member for Austria, replacing Karl-Heinz Huemer.

9.1.2. Vote by Proxy

None

9.1.3. Strategic Review and Learning Meeting (SRLM) – Madrid, Spain 17-18 October 2023

PDCO member: Fernando de Andrés Trelles, Maria Jesús Fernández Cortizo

Summary of Committee discussion:

PDCO members were informed about the date of the next strategic review and learning meeting in Madrid.

9.1.4. Strategic Review and Learning Meeting (SRLM) – Uppsala, Sweden 7-8 June 2023

PDCO member: Sara Vennberg

Summary of Committee discussion:

Thanks were extended to all those who attended the SRLM in Uppsala in person or virtually. Presentations were made available for the Committee members. All speakers confirmed their willingness to receive further questions directly. Minutes of the SRLM meeting were discussed and shared with the PDCO members.

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 May 2023, was presented to the PDCO

members.

Feedback on the ongoing CHMP procedures was provided to the Committee by the nominated PDCO experts.

An overview of discussions on PIP-related procedures, held by the CHMP in June 2023, was provided by a CHMP / PDCO member.

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 (*ad interim*)

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)

No item

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Summary of Committee discussion:

Two ITF cases 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. Paediatric Cluster Teleconference

Summary of Committee discussion:

The May 2023 minutes and June 2023 agenda and 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

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q2/2023 Update of the Business Pipeline report for the human scientific committees

Summary of Committee discussion:

The business pipeline report for Q2/2023 was provided for information.

10. Any other business

10.1. DARWIN EU® update

Summary of Committee discussion:

An update on DARWIN EU was provided. The list of currently ongoing and shortlisted studies was presented. Next steps include the scaling up of studies conducted via DARWIN EU and the onboarding of data partners.

10.2. Update on the Reflection Paper on Acute Kidney Injury (AKI)

PDCO members: Dimitar Roussinov, Sylvie Benchetrit

Summary of Committee discussion:

The draft reflection paper on acute kidney injury and its section on paediatric considerations were presented and discussed.

10.3. Updated scientific document and key elements form

Summary of Committee discussion:

The updated scientific document and key elements forms were presented to the Committee.

10.4. PDCO requirement for modifying allergen PIPs

Summary of Committee discussion:

A response to the Bundesverband der Pharmazeutischen Industrie (BPI) was presented to the PDCO members.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The group was informed about the U.S. Food and Drug Administration Paediatric Oncology Subcommittee of the Oncologic Drugs Advisory Committee Meeting on dosage optimisation of new drug and biological products for paediatric patients with cancer.

11.2. Neonatology

Summary of Committee discussion:

The PDCO group discussed topics for the revision of the neonatal guideline.

11.3. HIV

Summary of Committee discussion:

The PDCO discussed some HIV related procedures in advance to the plenary discussion.

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 20-23 June 2023 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Brian Aylward	Chair	Ireland	No interests declared	
Johanna Wernsperger	Member	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in final deliberations and voting on:	2.3.7. Ibrutinib - EMEA-001397-PIP04-17-M02 3.3.1. Apixaban - EMEA-000183-PIP02-12-M04
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	
Maria Eleni Avraamidou	Alternate	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	
Jana Lass	Alternate	Estonia	No interests declared	
Pauliina Lehtolainen-Dalkilic	Member	Finland	No interests declared	
Anne Paavola	Alternate	Finland	No interests declared	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Adrienn Horváth	Member	Hungary	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Cinzia Ciceroni	Alternate	Italy	No interests declared	
Dina Apele-	Member	Latvia	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Freimane			applicable to this meeting	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
Olivier Moes	Alternate	Luxembourg	No interests declared	
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	
Sara Vennberg	Member	Sweden	No interests declared	
David Khan	Alternate	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jose Ignacio Malagon	Alternate	Healthcare Professionals'	No restrictions applicable to this	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Calle		Representative	meeting	
Jaroslav Sterba	Member	Patients' Organisation Representative	No participation in final deliberations and voting on:	3.3.21. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M03
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Member	Patients' Organisation Representative	No interests declared	
Celine Chu	Expert - via telephone*	France	No interests declared	
María Estela Moreno Martín	Expert - via telephone*	Spain	No interests declared	
Hrefna Gudmundsdottir	Expert - via telephone*	Iceland	No interests declared	
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/