



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 16-19 January 2024

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 ongoing 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.

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 that no restriction in the involvement of meeting participants for agenda topics was identified.

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 welcomed the new member and thanked the departing alternate for his contributions to the Committee.

The EMA Secretariat announced the names of the Committee members who delegated their vote via proxy and the Committee members who received such proxy.

The Chair announced the start of the Belgian presidency of the Council of the European Union (EU).

1.2. Adoption of agenda

The agenda for 16-19 January 2024 meeting was adopted with amendments.

Topics added:

9.2.2. Coordination Group for Mutual Recognition and Decentralised Procedures – Human (CMDh)

9.4.2. Conect4Children (C4C) multi-stakeholder meeting on paediatric irritability

10.6 Feedback from FDA workshop "ADEPT 8: Drug Dosing in Paediatric Patients with Renal Impairment"

1.3. Adoption of the minutes

The minutes for 12-15 December 2023 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. Upadacitinib - EMEA-001741-PIP10-23

AbbVie Ltd; Treatment of alopecia areata

Day 120 opinion

Dermatology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Paediatric Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive paediatric investigation plan (PIP) opinion for upadacitinib in the paediatric population from 6 to less than 18 years of age in the condition of 'treatment of alopecia areata' was adopted.

The PDCO agreed on a waiver in a subset of children below the age of 6 years 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(s).

The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.2. Orforglipron - EMEA-003299-PIP02-22

Eli Lilly and Company; Treatment of obesity

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Paediatric Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for orforglipron, for the paediatric population from 6 years to less than 18 years of age in the condition treatment of obesity was adopted.

The PDCO agreed on a waiver in the 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 some measures contained in this PIP.

2.1.3. [Sepiapterin - Orphan - EMEA-003027-PIP02-23](#)

PTC Therapeutics International; Treatment of hyperphenylalaninaemia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Paediatric Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from birth to less than 18 years of age, in the condition of treatment of hyperphenylalaninaemia was adopted.

The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.4. [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 120 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 on a positive opinion for a paediatric investigation plan (PIP) for synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues in the condition of familial chylomicronaemia syndrome (FCS). The PIP includes one quality study, two clinical studies, two modelling and simulation studies, an extrapolation plan and a deferral and a waiver below the age of 2 years 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(s).

2.1.5. [Dupilumab - EMEA-001501-PIP12-23](#)

Treatment of eosinophilic gastritis / gastroenteritis

Day 120 opinion

Gastroenterology-Hepatology

Note: Withdrawal request received on 17 January 2024

2.1.6. [Ianalumab - EMEA-002338-PIP04-23](#)

Novartis Europharm Limited; Treatment of autoimmune haemolytic anaemia

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive paediatric investigation plan (PIP) opinion for ianalumab in the paediatric population from 5 to less than 18 years of age in the condition of treatment of autoimmune haemolytic anaemia 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 subset(s).

The PDCO granted a deferral for the completion of this PIP.

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

Sanofi Winthrop Industrie; Treatment of graft versus host disease (GVHD)

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The applicant addressed the remaining issues raised by the Paediatric Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for patients from 1 year to less than 18 years of age in the condition of treatment of GVHD was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of lack of significant therapeutic benefits.

The PDCO granted a deferral for one or more measures contained in the PIP.

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

Amgen Europe B.V.; Treatment of B-lymphoblastic leukaemia/lymphoma

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the January 2024 plenary meeting, a paediatric investigation plan (PIP) for blinatumomab for the treatment of B-lymphoblastic leukaemia/lymphoma.

The PDCO confirmed all conclusions reached at Day 90 and took into consideration the information the applicant provided between Day 30 and Day 60. Based on the assessment of this application, a positive opinion was adopted on a PIP for children from 28 days to less than 18 years of age, with a deferral for the treatment of B-lymphoblastic leukaemia/lymphoma and a waiver for a subset of children from birth to less than 28 days of age on the grounds that the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2.1.9. Clobetasol propionate - EMEA-003458-PIP01-23

Laboratorios Salvat, S.A.; Treatment of ocular inflammations and manifestations associated with ocular surgery

Day 120 opinion

Ophthalmology

Summary of Committee discussion:

The PDCO discussed at Day 120 an application for a paediatric investigation plan (PIP), and a deferral for clobetasol propionate for the treatment of ocular inflammations and manifestations associated with ocular surgery. The Paediatric Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion was adopted on a PIP with a deferral for clobetasol propionate for the treatment of ocular inflammations and manifestations associated with ocular surgery.

2.1.10. Tinalrebant - Orphan - EMEA-003225-PIP01-22

Belite Bio, Inc; Treatment of Stargardt disease

Day 120 opinion

Ophthalmology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from 3 years to less than 18 years of age, in the condition of treatment of Stargardt disease was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 3 years of age on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit or fulfil a therapeutic need of the specified paediatric subset(s). The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.11. 2-(3,5-dichloro-1-methyl-indazol-4-yl)-1-[(1S,3R)-3-(hydroxymethyl)-5-(1-hydroxy-1-methyl-ethyl)-1-methyl-3,4-dihydro-1H-isoquinolin-2-yl]ethanone monohydrate - EMEA-003515-PIP01-23

UCB Pharma S.A.; Treatment of Parkinson's disease

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 2-(3,5-dichloro-1-methyl-indazol-4-yl)-1-[(1S,3R)-3-

(hydroxymethyl)-5-(1-hydroxy-1-methyl-ethyl)-1-methyl-3,4-dihydro-1H-isoquinolin-2-yl]ethanone monohydrate for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of Parkinson's disease. The waiver is based on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

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.

2.1.12. IgG-like T cell engager binding to DLL3 and CD3 - EMEA-003516-PIP01-23

Boehringer Ingelheim International GmbH; Treatment of neuroendocrine carcinoma (excluding neuroblastoma) / Treatment of small cell lung carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the January 2024 plenary meeting, an application for a product-related full waiver for IgG-like T cell engager against DLL3 and CD3 for treatment of neuroendocrine carcinoma (excluding neuroblastoma) and for treatment of small cell lung carcinoma.

Based on the assessment of this application and further discussions at the Paediatric Committee including the applicant's clarifications, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for IgG-like T cell engager binding to DLL3 and CD3 for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of neuroendocrine carcinoma (excluding neuroblastoma) on ground of lack of significant therapeutic benefit due to lack of study feasibility, and in treatment of small cell lung carcinoma on the ground that the disease does not occur in 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. The PDCO identified neuroblastoma as an unmet need but also encouraged the applicant to explore other potential paediatric oncology indications in which the target (DLL3) might be of relevance. 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.

2.1.13. Interleukin-1 beta, human / interferon gamma / granulocyte colony-stimulating factor / interleukin-2 / tumour necrosis factor-alpha - EMEA-003523-PIP01-23

CEL-SCI Corporation; Treatment of head and neck epithelial malignant neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the January 2024 plenary meeting, a product-specific waiver request for interleukin-1 beta, human / interferon gamma / granulocyte colony-stimulating factor / interleukin-2 / tumour necrosis factor-alpha for the treatment of squamous cell carcinoma of the head and neck (SCCHN) on the grounds that the disease does not occur in the paediatric population and on lack of significant therapeutic benefit. The Paediatric Committee confirmed all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 for this product for the treatment of head and neck epithelial malignant neoplasms on the grounds of lack of significant therapeutic benefit because clinical studies would not be feasible.

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. Isatuximab - EMEA-002205-PIP02-23

Sanofi Winthrop Industrie; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

Day 60 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Paediatric 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 solution for injection and subcutaneous use) for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of malignant neoplasms of the haematopoietic and lymphoid tissue on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. This was based on the results of Study 2 of PIP01 for the pharmaceutical form 'concentrate for solution for infusion' for 'intravenous use' and the understanding, based on the available data, that no relationship was observed between the expression of CD38 and the response to the product. 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. Volrustomig - EMEA-003423-PIP02-23

AstraZeneca AB; Treatment of head and neck epithelial malignant neoplasms

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 volrustomig for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of head and neck epithelial malignant neoplasms. The waiver is based 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. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.16. Zanzalintinib - EMEA-003522-PIP01-23

Exelixis, Inc.; Treatment of colorectal cancer / Treatment of renal cell carcinoma / Treatment of head and neck squamous cell carcinoma

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 conditions 'treatment of colorectal cancer' 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), and 'treatment of renal cell carcinoma' and 'treatment of head and neck epithelial malignant neoplasms' based on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

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.

2.1.17. Zenocutuzumab - EMEA-003519-PIP01-23

Merus N.V.; Treatment of pancreatic cancer / Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the January 2024 plenary meeting, an application for a product-related full waiver for zenocutuzumab for treatment of lung cancer and treatment

of pancreatic cancer.

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 zenocutuzumab for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of pancreatic cancer, treatment of lung cancer on grounds that these diseases occur only in adult populations.

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. The PDCO encouraged the applicant to explore the use of the product in paediatric oncology indications where the molecular target may be of relevance. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.18. [Motugivatrep - EMEA-003520-PIP01-23](#)

SENJU PHARMACEUTICAL CO., LTD.; Treatment of dry eye disease

Day 60 opinion

Ophthalmology

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 motugivatrep for all subsets of the paediatric population (0 to 18 years of age) in the condition of dry eye disease.

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. [Borrelia outer surface protein A \(OspA\) serotypes \(ST1-6\) lipidated, fusion protein vaccine - EMEA-003130-PIP02-23](#)

Pfizer Europe MA EEIG; Prevention of Lyme disease

Day 90 opinion

Vaccines

Summary of Committee discussion:

The PDCO reviewed and discussed the responses to the request for modification and agreed that all the outstanding issues have now been resolved. A positive opinion agreeing to the modified paediatric investigation plan for *Borrelia* outer surface protein A (OspA) serotypes (ST1-6) lipidated, fusion protein vaccine (VLA15 - PF-07307405) in the condition of prevention of Lyme disease has been adopted. A deferral of some of the agreed measures as well as a waiver for the paediatric population from birth to less than 1 year of age on the

grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible have also been granted.

2.1.20. [Anti-alpha-synuclein recombinant humanised monoclonal antibody - EMEA-003541-PIP01-23](#)

Treatment of Parkinson's disease

Day 30 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 30, during the January 2024 plenary meeting the application for a product related full waiver for anti-alpha-synuclein recombinant humanised monoclonal antibody for treatment of treatment of Parkinson's disease.

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 anti-alpha-synuclein recombinant humanised monoclonal antibody for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of Parkinson's disease on grounds that the specific medicinal product is likely to be ineffective.

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.

2.2. **Opinions on Compliance Check**

2.2.1. [Pegfilgrastim - EMEA-C-002671-PIP02-20](#)

Accord Healthcare S.L.U.; Treatment of chemotherapy-induced neutropenia / Prevention of chemotherapy-induced febrile neutropenia

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO adopted on 17 January 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0206/2021) of 10 May 2021.

2.2.2. [Casirivimab - EMEA-C-002964-PIP01-21-M02](#)

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the completed study(ies) and took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-002964-PIP01-21

The PDCO adopted on 19 January 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0300/2022) of 10 August 2022.

2.2.3. Imdevimab – EMEA-C-002965-PIP01-21-M02

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the completed study(ies) and took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-002965-PIP01-21

The PDCO adopted on 19 January 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0300/2022) of 10 August 2022.

2.2.4. Talimogene laherparepvec - EMEA-C-001251-PIP01-11-M06

Amgen Europe B.V.; Treatment of melanoma

Day 60 opinion

Oncology

Summary of Committee discussion:

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

- EMEA-C1-001251-PIP01-11

The PDCO adopted on 19 January 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0275/2023) of 14 July 2023.

2.2.5. Selexipag - EMEA-C-000997-PIP01-10-M07

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 opinion

Other

Summary of Committee discussion:

The PDCO adopted a positive opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0417/2023) of 25 October 2023.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Azilsartan medoxomil - EMEA-000237-PIP01-08-M12

Takeda Development Centre Europe Ltd; Treatment of hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the January 2024 plenary meeting, a request for modification for azilsartan medoxomil for the treatment of hypertension.

The applicant requested to modify the clinical plan for children from 2 years to less than 6 years of age. The requested changes will result in completing the paediatric investigation plan (PIP) with a delay of approximately 5 years and a half.

The PDCO recommended that the applicant liaises as soon as possible with the CHMP to obtain without delay a licence for paediatric patients, since data from patients from 6 years to less than 18 years of age are already available.

Based on the review of the rationale submitted by the application for modifying the agreed PIP, the PDCO agreed that the requested modifications could be accepted. The Paediatric Committee, therefore, adopted a positive opinion.

2.3.2. Difelikefalin - EMEA-002565-PIP02-19-M01

Vifor Fresenius Medical Care Renal Pharma France; Treatment of chronic kidney disease associated pruritus

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), deletion of open-label extension phase of clinical study, 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/0172/2020 of 13 May 2020).

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

2.3.3. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M06

AstraZeneca AB; Treatment of hyperkalaemia

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 (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification, the applicant requested to modify the inclusion criteria for patients 1 month and less and to delay the date of completion of Study 4.

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0477/2021 of 3 December 2021).

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

2.3.4. Eluxadoline - EMEA-001579-PIP01-13-M06

AbbVie Limited; Treatment of diarrhoea-predominant irritable bowel syndrome

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), 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/0128/2022 of 13 April 2022).

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

2.3.5. Mepolizumab - EMEA-000069-PIP01-07-M08

GSK Trading Services Limited; Treatment of hypereosinophilic syndrome

Day 60 opinion

Haematology-Hemostaseology

Note: Withdrawal request received on 18 January 2024

2.3.6. Recombinant human A disintegrin and metalloprotease with thrombospondin type-1 motifs 13 (rADAMTS13) - Orphan - EMEA-001160-PIP01-11-M04

Takeda Pharmaceuticals International AG; Treatment of thrombotic thrombocytopenic purpura

Day 60 opinion

Haematology-Hemostaseology

Note: Withdrawal request received on 17 January 2024

2.3.7. Upadacitinib - EMEA-001741-PIP04-17-M05

AbbVie Ltd; Treatment of atopic dermatitis

Day 60 opinion

Immunology-Rheumatology-Transplantation / Dermatology

Summary of Committee discussion:

Following a teleconference, held with the PDCO evaluation team after Day 30, the applicant addressed most of the remaining issues raised by the Paediatric Committee.

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), 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/0166/2021 of 14 April 2021).

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

2.3.8. Oritavancin (diphosphate) - EMEA-001270-PIP01-12-M07

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 Paediatric Committee. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), 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/0104/2023 of 13 April 2023).

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

2.3.9. Acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) - Orphan - EMEA-002796-PIP01-20-M02

IntraBio Ltd.; Treatment of Niemann-Pick disease type C

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 (PIP), the PDCO considered that most of proposed changes could be accepted. One additional change was made on PDCO's own motion to adapt the wording of the modelling and simulation study as consequence of PIP modifications proposed by the applicant.

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as

set in the Agency's latest decision (P/0146/2023 of 21 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. [Fordadistrogene movaparvovec - Orphan - EMEA-002741-PIP01-20-M02](#)

Pfizer Europe MA EEIG; 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 (PIP), 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/0062/2023 of 24 February 2023).

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

2.3.11. [Givinostat - Orphan - EMEA-000551-PIP04-21-M03](#)

Italfarmaco S.p.A.; 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 (PIP) and the answers provided after Day 30, 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/0240/2023 of 14 June 2023).

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

2.3.12. [Abemaciclib - EMEA-002342-PIP01-18-M04](#)

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 January 2024 plenary meeting, a request for modification for abemaciclib for the treatment of Ewing's sarcoma and confirmed all conclusions reached at Day 30.

Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed change 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/0312/2023 of 9 August 2023).

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

[2.3.13. Abemaciclib - EMEA-002342-PIP02-18-M03](#)

Eli Lilly and Company Limited; Treatment of glioma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the January 2024 plenary meeting, a request for modification for abemaciclib for the treatment of glioma and confirmed all conclusions reached at Day 30.

Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed change 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/0313/2023 of 9 August 2023).

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

[2.3.14. Dinutuximab beta - Orphan - EMEA-001314-PIP01-12-M02](#)

Recordati Netherlands B.V.; Treatment of neuroblastoma

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 (PIP), 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/0093/2022 of 11 March 2022).

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

[2.3.15. Midostaurin - Orphan - EMEA-000780-PIP01-09-M07](#)

Novartis Europharm Limited; Treatment of acute myeloid leukaemia / Treatment of mast cell leukaemia / Treatment of malignant mastocytosis

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO took note of the clarifications provided by the applicant after Day 30. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), 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/0086/2021 of 19 March 2021).

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

2.3.16. Atropine sulfate - EMEA-002744-PIP01-19-M01

Nevakar Inc.; Treatment of myopia

Day 60 opinion

Ophthalmology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), 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/0229/2021 of 8 June 2021).

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

2.3.17. Molgramostim - Orphan - EMEA-002282-PIP01-17-M02

Savara Aps; Treatment of pulmonary alveolar proteinosis

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 (PIP), 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/0509/2021 of 3 December 2021).

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

2.3.18. Nedosiran - Orphan - EMEA-002493-PIP01-18-M06

Novo Nordisk A/S; Treatment of primary hyperoxaluria

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that some of the proposed changes (e.g. timeline 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/0007/2023 of 10 February 2023).

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

2.3.19. Purified rabies virus, WISTAR PM/WI 38-1503-3M strain (inactivated) - EMEA-002234-PIP01-17-M02

Sanofi Pasteur; Prevention of rabies 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 (PIP), 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/0319/2020 of 12 August 2020).

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

2.3.20. Recombinant SARS-CoV-2 spike protein - EMEA-002915-PIP01-20-M03

Sanofi Pasteur; 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 (PIP) into a waiver, 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/0046/2022 of 14 February 2022). The PIP was transformed into a waiver based on lack of significant therapeutic benefit.

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

2.3.21. Chikungunya virus virus-like particle vaccine / aluminum hydroxide - EMEA-002656-PIP01-19-M01

Bavarian Nordic A/S; Prevention of chikungunya disease

Day 60 opinion

Vaccines / Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), 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/0159/2021 of 16 April 2021).

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

2.3.22. Satralizumab - Orphan - EMEA-001625-PIP02-21-M03

Roche Registration GmbH; Treatment of generalised myasthenia gravis

Day 30 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed the modification request during the January 2024 plenary meeting. Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), 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/0114/2023 of 13 April 2023).

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

2.3.23. Daridorexant - EMEA-002121-PIP03-19-M02

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of insomnia

Day 30 opinion

Psychiatry

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), 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/0116/2022 of 13 April 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

2.4.1. Ritlecitinib - EMEA-002451-PIP01-18-M02

Pfizer Europe MA EEIG; Treatment of alopecia areata

Day 30 opinion

Dermatology

Summary of Committee discussion:

The re-examination consisted of requesting the deletion of the inclusion criteria of Study 4 (B7981027) in patients from 6 years to less than 12 years of age requiring psychological counselling and psychological impairment based on several grounds.

Based on the review of the grounds for re-examination the PDCO concluded that the proposed changes on the inclusion criteria were acceptable.

The PDCO therefore adopted a favourable opinion.

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. Obecabtagene autoleucel - EMEA-C1-003171-PIP01-21

Autolus GmbH; Treatment of acute lymphoblastic leukaemia

Day 30 letter

Oncology

2.7.2. Anti-neonatal Fc receptor human monoclonal antibody - EMEA-C1-002559-PIP02-19

Janssen-Cilag International NV; Treatment of myasthenia gravis

Day 30 letter

Neurology

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

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Iodine (¹³¹I) apamistamab - Orphan - EMEA-003395-PIP02-23

Immedica Pharma AB; Treatment in allogenic stem cell transplantation

Day 90 discussion

Immunology-Rheumatology-Transplantation / Oncology

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

Treatment of lower respiratory tract and lung infections

Day 90 discussion

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

3.1.4. EMEA-003477-PIP01-23

Treatment of appetite and general nutrition disorders

Day 90 discussion

Nutrition

3.1.5. Disitamab vedotin - EMEA-003443-PIP02-23

Treatment of HER2 expressing tumours / Treatment of solid tumours

Day 90 discussion

Oncology

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

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

Day 90 discussion

Other

3.1.7. Garetosmab - Orphan - EMEA-002736-PIP02-23

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

Day 90 discussion

Other

3.1.8. Losmapimod - Orphan - EMEA-003448-PIP01-23

Fulcrum Therapeutics, Inc.; Treatment of facioscapulohumeral muscular dystrophy

Day 90 discussion

Other

3.1.9. Cannabidiol - Orphan - EMEA-003176-PIP02-22

Zynerba Pharmaceuticals Inc; Treatment of fragile X syndrome (FXS)

Day 90 discussion

Psychiatry

3.1.10. mRNA encoding CMV gB / mRNA encoding the gH protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL131A protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL130 protein in the CMV glycoprotein complex pentamer / mRNA encoding the gL protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL128 protein in the CMV glycoprotein complex pentamer - EMEA-003405-PIP01-23

Prevention of cytomegalovirus infection

Day 90 discussion

Vaccines

3.1.11. Orforglipron - EMEA-003299-PIP01-22

Treatment of type 2 diabetes mellitus

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.12. Belimumab - EMEA-000520-PIP03-23

Treatment of systemic sclerosis

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.13. EMEA-003394-PIP01-23

Treatment of Duchenne/Becker muscular dystrophy

Day 90 discussion

Other / Neurology

3.1.14. Pegtibatase - Orphan - EMEA-003518-PIP01-23

Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.15. [Nomacopan - Orphan - EMEA-003517-PIP01-23](#)

Akari Malta Ltd; Treatment in haematopoietic stem cell transplantation

Day 60 discussion

Haematology-Hemostaseology

3.1.16. [Ensitrelvir - EMEA-003192-PIP02-23](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Infectious Diseases

3.1.17. [Lenacapavir - EMEA-002740-PIP02-23](#)

Prevention of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Infectious Diseases

3.1.18. [Iptacopan - EMEA-002705-PIP06-23](#)

Treatment of generalised myasthenia gravis (gMG)

Day 60 discussion

Neurology

3.1.19. [Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003524-PIP01-23](#)

Sensorion SA; Treatment of otoferlin gene-mediated hearing loss

Day 60 discussion

Other

3.1.20. [Mirdametinib - Orphan - EMEA-003525-PIP01-23](#)

Springworks Therapeutics Ireland Limited; Treatment of neurofibromatosis type 1 - plexiform neurofibroma

Day 60 discussion

Other

3.1.21. Vosoritide - EMEA-002033-PIP02-23

Treatment of hypochondroplasia

Day 60 discussion

Other

3.1.22. Recombinant varicella zoster virus glycoprotein E adjuvanted – EMEA-003526-PIP01-23

Prevention of herpes zoster

Day 60 discussion

Vaccines

3.1.23. Single-stranded 5' capped mRNA encoding the Has of the influenza virus and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein – EMEA-003521-PIP01-23

Prevention of influenza and coronavirus disease 2019 (COVID-19)

Day 60 discussion

Vaccines

3.1.24. Ramipril / nebivolol – EMEA-003530-PIP01-23

Treatment of hypertension / Treatment of heart failure / Treatment of coronary artery disease / Treatment of hypertension with coexisting heart failure / Treatment of hypertension with coexisting coronary artery disease

Day 30 discussion

Cardiovascular Diseases

3.1.25. Human IgG1 monoclonal antibody targeting amyloid transthyretin - EMEA-003548-PIP01-23

Treatment of transthyretin amyloidosis (ATTR)

Day 30 discussion

Cardiovascular Diseases / Neurology

3.1.26. Urea / propylene glycol - EMEA-003542-PIP01-23

Treatment of dry skin

Day 30 discussion

Dermatology

3.1.27. EMEA-003531-PIP01-23

Treatment of hyperphenylalaninemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.28. Dapagliflozin / sitagliptin - EMEA-003534-PIP01-23

Treatment of type II diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.29. Human HSD17B1 enzyme inhibitor - EMEA-003537-PIP01-23

Treatment of endometriosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.30. Efinopegdutide - EMEA-003549-PIP01-23

Treatment of non-alcoholic steatohepatitis

Day 30 discussion

Gastroenterology-Hepatology

3.1.31. 2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid (fipaxalparant) - Orphan - EMEA-003539-PIP01-23

Horizon Therapeutics Ireland DAC; Treatment of progressive fibrosing interstitial lung disease

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.32. Empasiprubart - EMEA-003528-PIP01-23

Treatment of multifocal motor neuropathy

Day 30 discussion

Neurology

3.1.33. Glenzocimab - EMEA-003536-PIP01-23

Treatment of acute ischemic stroke

Day 30 discussion

Neurology

3.1.34. [Suvecaltamide - EMEA-003248-PIP02-23](#)

Treatment of moderate to severe tremor in patients with Parkinson's disease

Day 30 discussion

Neurology

3.1.35. [Unasnemab - EMEA-003529-PIP01-23](#)

Treatment of spinal cord injury

Day 30 discussion

Neurology

3.1.36. [Ursodoxicoltaurine / sodium phenylbutyrate - Orphan - EMEA-002876-PIP02-23](#)

Amylyx Pharmaceuticals EMEA B.V.; Treatment of progressive supranuclear palsy

Day 30 discussion

Neurology

3.1.37. [Autologous T-cells expressing a chimeric antigenic receptor against G protein coupled receptor family C group 5 member D \(GPRC5D\) - EMEA-003543-PIP01-23](#)

Treatment of multiple myeloma

Day 30 discussion

Oncology

3.1.38. [Budigalimab - EMEA-003532-PIP01-23](#)

Treatment of solid tumours

Day 30 discussion

Oncology

3.1.39. [Livmoniplimab - EMEA-003533-PIP01-23](#)

Treatment of solid tumours

Day 30 discussion

Oncology

3.1.40. Zongertinib - EMEA-003546-PIP01-23

Treatment of non-small cell lung cancer

Day 30 discussion

Oncology

3.1.41. Fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer - EMEA-003538-PIP01-23

Treatment of Duchenne muscular dystrophy

Day 30 discussion

Other

3.1.42. Retatrutide - EMEA-003258-PIP02-23

Treatment of obesity

Day 30 discussion

Other

3.1.43. Vixarelimab - EMEA-003540-PIP01-23

Treatment of idiopathic pulmonary fibrosis

Day 30 discussion

Pneumology - Allergology

3.1.44. Efgartigimod alfa - EMEA-002597-PIP10-23

Treatment of thyroid eye disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.45. Ensitrelvir - EMEA-003192-PIP03-23

Post exposure prophylaxis of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.1.46. Palonosetron / netupitant – EMEA-001198-PIP04-23

Prevention of chemotherapy-induced nausea and vomiting

Day 30 discussion

Other

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. Avacopan - EMEA-C5-002023-PIP01-16-M07

Amgen Europe B.V.; Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.2. Letemovir - EMEA-C-001631-PIP01-14-M05

Merck Sharp & Dohme B.V.; Prevention of cytomegalovirus infection

Day 30 discussion

Infectious Diseases

3.2.3. Tedizolid phosphate - EMEA-C2-001379-PIP01-12-M08

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

Day 30 discussion

Infectious Diseases

3.2.4. Hydrocortisone - EMEA-C-002305-PIP01-17-M01

Laboratoire AGUETTANT; Prevention of bronchopulmonary dysplasia

Day 30 discussion

Neonatology - Paediatric Intensive Care

3.2.5. Blinatumomab - EMEA-C-000574-PIP02-12-M04

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

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Chloroprocaine (hydrochloride) - EMEA-000639-PIP03-16-M03

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

Day 30 discussion

Anaesthesiology

3.3.2. Aficamten - EMEA-002958-PIP01-21-M01

Cytokinetics, Inc.; Treatment of hypertrophic cardiomyopathy

Day 30 discussion

Cardiovascular Diseases

3.3.3. Milvexian - EMEA-003220-PIP01-22-M01

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

Day 30 discussion

Cardiovascular Diseases

3.3.4. Ruxolitinib phosphate - EMEA-002618-PIP02-20-M01

Incyte Biosciences Distribution B.V.; Treatment of vitiligo

Day 30 discussion

Dermatology

3.3.5. Glucagon analogue linked to a human immunoglobulin Fc fragment - Orphan - EMEA-003170-PIP01-21-M02

Hanmi Pharm. Co., Ltd.; Treatment of congenital hyperinsulinism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19-M03

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

Day 30 discussion

Haematology-Hemostaseology

3.3.7. Filgotinib - EMEA-001619-PIP04-17-M03

Galapagos NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis, and juvenile idiopathic arthritis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.8. BNT162b2 / tozinameran / famtozinameran / riltozinameran / raxtozinameran - EMEA-002861-PIP02-20-M07

BioNTech Manufacturing GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.9. Cedazuridine / decitabine - EMEA-003071-PIP01-21-M01

Otsuka Pharmaceutical Netherlands B.V.; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.3.10. Vamikibart - EMEA-003215-PIP01-22-M01

Roche Registration GmbH; Treatment of macular oedema

Day 30 discussion

Ophthalmology

3.3.11. Afamelanotide – Orphan – EMEA-000737-PIP02-11-M03

Clinuvel Europe Limited; Treatment of erythropoietic protoporphyria

Day 30 discussion

Other

3.3.12. In vitro expanded autologous human articular chondrocytes - EMEA-001823-PIP01-15-M03

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 30 discussion

Other

3.3.13. Seltorexant - EMEA-002746-PIP01-20-M03

Janssen-Cilag International NV; Treatment of major depressive disorder

Day 30 discussion

Psychiatry

3.3.14. L-carnitine / glucose / calcium chloride dihydrate / magnesium chloride hexahydrate / sodium lactate / sodium chloride - EMEA-003049-PIP01-21-M01

Iperboreal Pharma Srl; Treatment of patients in need of peritoneal dialysis

Day 30 discussion

Uro-nephrology

3.3.15. Mirabegron - EMEA-000597-PIP03-15-M06

Astellas Pharma Europe B.V.; Treatment of neurogenic detrusor overactivity

Day 30 discussion

Uro-nephrology

3.3.16. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitidis* group B protein 961c / recombinant *Neisseria meningitidis* group B protein 287- 953 / meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitidis* group B protein 936-741 / outer membrane vesicles (OMV) from *N. meningitidis* strain NZ 98/254 - EMEA-001260-PIP01-11-M03

GlaxoSmithKline Biologicals SA; Prevention of meningococcal meningitis

Day 30 discussion

Vaccines

3.3.17. 1-[[[4-(4-fluoro-2-methyl-1H-indol-5-yloxy)-6-methoxyquinolin-7-yl]oxy]methyl]cyclopropanamine-dihydrochloride - Orphan - EMEA-002486-PIP04-21-M01

Advenchen Laboratories, LLC; Treatment of Ewing sarcomas / Treatment of soft tissue sarcomas

Day 30 discussion

Oncology

3.3.18. 12 Grass pollen extract and cultivated rye pollen extract - EMEA-000813-PIP01-09-M01

Allergy Therapeutics (UK) Ltd.; Treatment of allergic rhinitis/rhino-conjunctivitis

Day 30 discussion

Pneumology - Allergology

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 22 January 2024 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

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 Chair thanked Dominique Ploin for his contribution as an alternate for France.

The Chair announced that Greta Budukeviciute is the new member for Lithuania, replacing Dovile Zacharkiene.

9.1.2. Vote by Proxy

None

9.1.3. Strategic Review and Learning Meeting (SRLM)

No item

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 December 2023, was presented to the PDCO members.

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

9.2.2. Coordination Group for Mutual Recognition and Decentralised Procedures – Human (CMDh)

Art. 29/13 referrals for discussion at CMDh

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. Paediatric Formulation Operational Expert Group (PFOEG, former Formulation Working Group)

PDCO member: Brian Aylward (*ad interim*)

Summary of Committee discussion:

The Chair of the PFOEG identified the products which will require PFOEG evaluation and discussion.

9.3.3. PDCO - Paediatric Formulation Operational Expert Group (PFOEG) joint session

PDCO Chair: Brian Aylward; PDCO members: Dina Apele-Freimane, Siri Wang, Louisa Braun Exner and Jana Lass

Summary of Committee discussion:

PFOEG updated the Committee on the progress of their work on the revision of the formulation section in the neonatal guideline. Final text is planned for adoption by the February 2024 meeting.

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

No item

9.3.5. Upcoming Innovation Task Force (ITF) meetings

Summary of Committee discussion:

Two upcoming meetings were presented to the Committee for information.

9.3.6. PDCO - Modelling and Simulation Operational Expert Group (MSOEG)

Draft summary slide for PDCO-MSOEG BINGO

PDCO members: Sara Vennberg and David Khan; Expert: Kristin Karlsson

Summary of Committee discussion:

Proposal to add headings to the PDCO BINGO document for paediatric investigation plans (PIPs) referred to the MSOEG was presented to the Committee. This would aid in asking the correct questions to the MSOEG. Proposal for a pilot was supported by the Committee. Topic leads will continue the work and implementation of the pilot. Training for coordinators could be arranged if needed.

9.4. Cooperation within the EU regulatory network

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

No item

9.4.2. Conect4Children (C4C) multi-stakeholder meeting on paediatric irritability

Summary of Committee discussion:

The Committee noted the upcoming C4C multi-stakeholder meeting on paediatric irritability which will take place on 18-19 March 2024 in Nice, France.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The January 2024 agenda and December 2023 ad hoc meeting 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

No item

10. Any other business

10.1. Feedback from oncology break out session – framework discussions supporting regulatory decision-making

Summary of Committee discussion:

The PDCO was invited to contribute to ongoing discussions related to framework considerations supporting regulatory decision making which move from a product focus towards a population focus.

10.2. Onboarding of Paediatrics processes on IRIS

Summary of Committee discussion:

The Committee was informed about plans for including paediatric procedures on the IRIS platform.

10.3. Training on Proxy votes

Summary of Committee discussion:

The PDCO Secretariat organised a training session on Proxy votes. The presentation with instructions and templates were shared with the Committee.

10.4. Report from paediatric clinical trial breakout session, ACT EU-methodology workshop

PDCO members: Dina Apele-Freimane and Anette Solli Karlsen

Summary of Committee discussion:

The ACT-EU methodology workshop was held on 23 November 2023. A break-out session on paediatric clinical trials was included in the workshop.

A summary of the discussion in the break-out session and the suggested ways forward were provided.

Following that, the PDCO discussed the issue of lack of harmonisation in how NCA/EC interpret CTR Article 32. PDCO members provided examples on clinical trials for which EU member states or EC have requested changes to clinical trials, in conflict with requirements

in the agreed PIP. This issue will be addressed by the PDCO - Clinical Trials Coordination Group (CTCG) interaction subgroup and tabled for discussion with the CTCG.

10.5. EU Network Training Centre (EU NTC): Paediatric curriculum follow-up

PDCO members: Sylvie Benchetrit, Sara Vennberg and Francesca Rocchi

Summary of Committee discussion:

The Paediatric curriculum was updated over 5 years ago. Therefore, there was a need to review and update it as required. The update will focus firstly on scientific sections, while the regulatory sections will be revised following regulatory upcoming discussions. New topics will be added, based on guidelines-recommendations, and on specific paediatric topics such as maturation and developmental processes on organs and systems. Updated/new paediatric-related courses (such as extrapolation) will be added. A call was launched for Committee members to support the update of the Paediatric curriculum. Further details around how Committee members can contribute will be provided at the PDCO February 2024 meeting.

10.6. Feedback from FDA workshop "ADEPT 8: Drug Dosing in Paediatric Patients with Renal Impairment"

PDCO member: Dimitar Roussinov

Summary of Committee discussion:

The Committee noted the feedback from the FDA workshop held on 30 November – 1 December 2023.

11. Breakout sessions

11.1. Neonatology

Summary of Committee discussion:

The neonatal PDCO group continued discussions on the revision of the neonatal guideline.

11.2. Vaccines

Summary of Committee discussion:

The breakout session was cancelled.

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 16-19 January 2024 meeting PDCO meeting which was held in-person.

An asterisk (*) after the role, in the second column, signals that the member/alternate attended remotely. Additional experts participated in part of the meeting, either in person or remotely.

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	
Agnes Gyurasics	Alternate	Austria	No interests declared	
Marleen Renard	Member*	Belgium	No restrictions applicable to this meeting	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Maria Eleni Avraamidou	Alternate	Cyprus	No interests declared	
Tereza Bazantova	Member	Czechia	No interests declared	
Pavlina Chladova	Alternate	Czechia	No interests declared	
Louisa Braun Exner	Member	Denmark	No interests declared	
Jana Lass	Member	Estonia	No interests declared	
Liisa Saare	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	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Moutaki	Alternate*	Greece	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Adrienn Horváth	Member*	Hungary	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Cinzia Ciceroni	Alternate*	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
Olivier Moes	Alternate*	Luxembourg	No interests declared	
Greta Budukeviciute	Member*	Lithuania	No interests declared	
Herbert Lenicker	Alternate*	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No restrictions applicable to this meeting	
Maaïke 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 Sztanyi	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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
			meeting	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jose Ignacio Malagon	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Tomasz Grybek	Member	Patients' Organisation Representative	No interests declared	
Jaroslav Sterba	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Viviana Giannuzzi	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Patricia Felgueiras Seabra Durao	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Victoria Romero Pazos	Alternate	Patients' Organisation Representative	No interests declared	
Celine Chu	Expert	France	No interests declared	
María Estela Moreno Martín	Expert	Spain	No interests declared	
Sandra Schmidt	Expert	Germany	No interests declared	
Susanne Kaul	Expert	Germany	No interests declared	
Birgit Ahrens	Expert	Germany	No interests declared	
Kristin Karlsson	Expert	Sweden	No restrictions applicable to this meeting	
Ann Marie Tötterman	Expert	Finland	No interests declared	
Isabelle	Expert	Belgium	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Delneuvillle				
Flora Musuamba Tshinanu	Expert	Belgium	No restrictions applicable to this meeting	
Sara Arenas-Lopez	Expert	Spain	No restrictions applicable to this meeting	
Daniela Reins	Expert	Germany	No interests declared	
Danila Renzo	Expert	Italy	No interests declared	
Emma Cassar Buontempo	Expert	Malta	No interests declared	
Rutger de Vries	Expert	Netherlands	No interests declared	
Beata Szabady	Expert	Hungary	No interests declared	
Gudrun Fridgeirsdottir	Expert	Norway	No interests declared	
Rut Anna-Lena Axelson	Expert	Sweden	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.

For a list of acronyms and abbreviations, see:

[Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA's regulatory activities](#)

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/