06 September 2024
EMA/PDCO/364965/2024
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 23-26 July 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

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 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 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 announced the start of the Hungarian presidency of the Council of the European Union (EU).

1.2. Adoption of agenda

The agenda for 23-26 July 2024 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 25-28 June 2024 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. Seralutinib - Orphan - EMEA-002972-PIP02-23

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 120 opinion

Cardiovascular Diseases

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 4 years to less than 18 years of age, in the condition of treatment of pulmonary arterial hypertension was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of lack of significant therapeutic benefit as clinical trials are not feasible. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.2. Pegtibatinase - Orphan - EMEA-003518-PIP01-23

Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 25 July 2024

2.1.3. Efinopegdutide - EMEA-003549-PIP01-23

MSD Europe Belgium SRL; Treatment of metabolic dysfunction-associated steatohepatitis

Day 120 opinion

Gastroenterology-Hepatology

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 8 years to less than 18 years of age, in the condition of treatment of metabolic dysfunction-associated steatohepatitis was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 8 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.4. Recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody - EMEA-003510-PIP01-23

Astria Therapeutics, Inc.; Treatment of hereditary angioedema

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 opinion on a paediatric investigation plan (PIP) for recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody in children from 2 years to less than 18 years of age for 'treatment of hereditary angioedema' was adopted. The PDCO agreed on a waiver in children from birth to less than 2 years on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible. The PDCO granted a deferral for measures contained in the PIP.

2.1.5. Iptacopan - EMEA-002705-PIP06-23

Novartis Europharm Limited; Treatment of myasthenia gravis

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO issued a positive opinion on this paediatric investigation plan (PIP) for iptacopan (capsule, hard, age-appropriate dosage form) for the treatment of myasthenia gravis. A waiver was granted for the paediatric population from birth to less than 2 years of age on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset. The agreed PIP includes the development of an age-appropriate formulation and a juvenile animal study. Regarding the clinical development, a single-arm paediatric clinical study and two modelling and simulation studies are part of an extrapolation plan of efficacy from adults covering the paediatric population from 2 years to less than 18 years of age with generalised myasthenia gravis. The completion of some measures of the PIP is deferred.

2.1.6. 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 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 paediatric investigation plan (PIP) for the proposed medicine for children from 6 months to less than 18 years, in the condition of treatment of otoferlin gene-mediated hearing loss, was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 6 months of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.7. Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23

Glycomine Inc; Treatment of phosphomannomutase 2-congenital disorder of glycosylation

Day 120 opinion

Other

Summary of Committee discussion:

The PDCO discussed at Day 120, during the July 2024 plenary meeting, an application for a stepwise paediatric investigation plan (PIP) (within the sPIP pilot) with a deferral for mannose-1-phosphate for treatment of phosphomannomutase 2-congenital disorder of glycosylation (PMM2-CDG). 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 PIP for all subsets of paediatric population. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.8. Retatrutide - EMEA-003258-PIP02-23

Eli Lilly and Company; Treatment of obesity

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 paediatric investigation plan (PIP) for retatrutide, 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.

2.1.9. Rilonacept - Orphan - EMEA-003571-PIP01-23

Kiniksa Pharmaceuticals (UK), Ltd.; Treatment of idiopathic pericarditis

Day 120 opinion

Other / Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application, the PDCO adopted a positive opinion on Day 120 for rilonacept for the treatment of idiopathic pericarditis in paediatric population from 12 years to less than 18 years of age. A waiver was granted for the paediatric population from birth to less than 12 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2.1.10. Empagliflozin / derivative of 3-phenyl-3H,4H,6H,7H-pyrano[3,4-d]imidazol-4-one (BI 690517) - EMEA-003632-PIP01-24

Boehringer Ingelheim International GmbH; Treatment of heart failure

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for empagliflozin / derivative of 3-phenyl-3H,4H,6H,7H-pyrano[3,4-d]imidazol-4-one (BI 690517) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of heart failure. 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.

2.1.11. Zerlasiran - EMEA-003627-PIP01-24

Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Note: Withdrawal request received on 23 July 2024

2.1.12. Rememulgene arelactibac - EMEA-003638-PIP01-24

Aurealis Oy; Treatment of diabetic foot ulcer / Treatment of venous leg ulcer

Day 60 opinion

Dermatology

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 of 'treatment of venous leg ulcer' and 'treatment of diabetic foot ulcer' on the grounds of lack of significant therapeutic benefit over existing treatments.

2.1.13. Aldesleukin - Orphan - EMEA-001556-PIP02-24

ILTOO Pharma SAS; Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the July 2024 plenary meeting the application for a product specific full waiver for aldesleukin for treatment of amyotrophic lateral sclerosis (ALS). 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 aldesleukin for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of 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.

2.1.14. Anitocabtagene autoleucel - EMEA-003628-PIP01-24

Kite Pharma EU B.V.; Treatment of multiple myeloma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the July 2024 plenary meeting, an application for a full product specific waiver for anitocabtagene autoleucel for treatment of multiple myeloma. 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 anitocabtagene autoleucel for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of multiple myeloma. 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 stressed the potential interest of this therapy in the paediatric setting and further investigation is encouraged. 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. Tafasitamab - Orphan - EMEA-002499-PIP03-24

Incyte Biosciences Distribution B.V; Treatment of mature B-cell neoplasms excluding diffuse large B-cell 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 mature B-cell neoplasms excluding diffuse large B-cell lymphoma 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 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. Rupatadine fumarate / montelukast sodium - EMEA-003629-PIP01-24

Noucor Health, S.A.; Treatment of allergic rhinitis

Day 60 opinion

Oto-rhino-laryngology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO disagreed with the applicant's request for a waiver for all subsets of the paediatric population (from birth to 18 years of age) in the condition of 'treatment of allergic rhinitis'. The Committee, therefore, adopted a negative opinion, refusing the full waiver request for the proposed medicine for all subsets of the paediatric population in the above condition.

2.1.17. Hydroxycarbamide - EMEA-003388-PIP01-23

THERAVIA; Treatment of sickle cell disease

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

In July 2024, the PDCO noted the written response in which 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 paediatric investigation plan (PIP) for the proposed medicine for the paediatric population from 9 months to less than 12 years of age, in the condition of treatment of sickle cell disease was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 9 months of age and from 12 years to less than 18 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.18. Belumosudil - Orphan - EMEA-003425-PIP02-23

Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

In the written response, 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 for the paediatric investigation plan (PIP) for belumosudil for children from 12 years to less than 18 years of age, in the condition of treatment of chronic lung allograft dysfunction 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 specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible. The PDCO granted a deferral for one or more measures contained in the PIP.

2.2. Opinions on Compliance Check

2.2.1. Emtricitabine / tenofovir alafenamide - EMEA-C1-001577-PIP02-14-M05

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

Day 60 letter

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the completed study(ies) and considered that these are compliant with the latest Agency's Decision (P/0402/2022) of 9 September 2022.
The PDCO finalised this partially completed compliance procedure on 26 July 2024.

2.2.2. Palbociclib - EMEA-C-002146-PIP01-17-M04

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO adopted on 26 July 2024 an opinion confirming the compliance of all studies (quality study 1, non-clinical studies 2 and 3 and clinical study 4) in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0117/2022) of 13 April 2022.

2.2.3. Avatrombopag - EMEA-C-001136-PIP01-11-M03

Swedish Orphan Biovitrum (AB); Treatment of idiopathic thrombocytopenic purpura

Day 30 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

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

- EMEA-C1-001136-PIP01-11-M01

The PDCO adopted on 26 July 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/0137/2024) of 6 May 2024.

2.2.4. Eltrombopag - EMEA-C-000170-PIP03-13-M04

Novartis Europharm Limited; Treatment of aplastic anaemia

Day 30 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

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

- EMEA-C1-000170-PIP03-13-M03

The PDCO adopted on 26 July 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/0516/2020) of 22 December 2020.

2.2.5. Ixekizumab - EMEA-C-001050-PIP02-18-M02

Eli Lilly and Company; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis)

Day 30 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO discussed the completed study(ies) and considered that these are compliant with the latest Agency's Decision (P/0049/2023) of 14 July 2023.
The PDCO adopted on 26 July 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/0049/2023) of 14 July 2023.

2.2.6. Efinaconazole - EMEA-C-001627-PIP01-14-M03

Almirall S.A.; Treatment of onychomycosis

Day 30 opinion

Dermatology

Summary of Committee discussion:

The PDCO adopted on 26 July 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/0203/2023) of 13 June 2023.

2.2.7. Influenza vaccine (surface antigen, inactivated, prepared in cell cultures) - EMEA-C-002068-PIP01-16-M05

Seqirus Netherlands B.V.; Prevention of influenza

Day 30 opinion

Vaccines

Summary of Committee discussion:

The PDCO took note of the outcomes of preceding positive partial compliance check procedure EMEA-C2-002068-PIP01-16-M01 (for studies V130_03, V58_31, V58P12, V58P15 and V58P16) and of the positive compliance of studies V130_10 and 7-V130_14.
The PDCO therefore adopted on 26 July 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/0168/2024) of 6 May 2024.

2.2.8. Mirdametinib - EMEA-C1-003525-PIP01-23

SpringWorks Therapeutics Ireland Ltd; Treatment of neurofibromatosis type 1

Day 30 letter

Other

Summary of Committee discussion:

The PDCO discussed the completed studies:
- Study 2 (Open-label, multicentre, single-arm trial to evaluate the safety, pharmacokinetics (PK), and efficacy of mirdametinib in children and adolescents from 2 years to less than 18 years of age (and adults) with an inoperable neurofibromatosis type 1 (NF1)-associated plexiform neurofibroma (PN) causing significant morbidity);
- Study 4 (Population PK model to characterize the PK profile and simulate drug exposure of mirdametinib in paediatric patients from 2 years to less than 18 years of age with neurofibromatosis type 1-associated plexiform).

The PDCO considered that these are compliant with the latest Agency's Decision (P/0223/2024) of 20 June 2024.
The PDCO finalised this partially completed compliance procedure on 26 July 2024.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Fenebrutinib - EMEA-002349-PIP03-20-M01

Roche Registration GmbH; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Note: Withdrawal request received on 26 July 2024

2.3.2. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M10

GlaxoSmithKline (Ireland) Limited; Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular Diseases

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 not be accepted.
The PDCO therefore adopted a negative opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0428/2022 of 28 October 2022). Therefore the key elements remain unchanged.

2.3.3. Spesolimab - EMEA-002475-PIP03-22-M01

Boehringer Ingelheim International GmbH; Treatment of Netherton syndrome

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), 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/0247/2023 of 14 July 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Macimorelin acetate - EMEA-001988-PIP01-16-M02

Atnahs Pharma Netherlands B. V.; Diagnosis of growth hormone deficiency

Day 60 opinion

Diagnostic

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 in the population 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/0076/2020 of 6 April 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M04

Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

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.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0428/2022 of 28 October 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Apremilast - EMEA-000715-PIP02-11-M08

Amgen Europe B.V.; Treatment of juvenile idiopathic arthritis (JIA) / Treatment of juvenile psoriatic arthritis (JPsA)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 25 July 2024

2.3.7. Apremilast - EMEA-000715-PIP05-13-M06

Amgen Europe.B.V; Treatment of Behçet's disease

Day 60 opinion

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 25 July 2024

2.3.8. Deucravacitinib - EMEA-002350-PIP02-20-M01

Bristol-Myers Squibb Pharma 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 (PIP), the PDCO considered that the proposed changes (changes to modelling & simulation study, minor changes to clinical study) 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/0156/2022 of 13 May 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Human plasma derived C1-inhibitor (OCTA-C1-INH) - EMEA-002818-PIP01-20-M01

Octapharma Pharmazeutika Produktionsges. m.b.H; Treatment of hereditary angioedema

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 (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/0225/2021 of 9 June 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Cefiderocol - EMEA-002133-PIP01-17-M04

Shionogi B.V.; Treatment of infections due to aerobic gram-negative bacteria

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes in timelines 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/0441/2022 of 28 October 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M03

Mundipharma GmbH; Treatment of invasive candidiasis

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (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/0342/2022 of 10 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Sotrovimab - EMEA-002899-PIP01-20-M03

GlaxoSmithKline Trading Services Ltd; Treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (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/0235/2023 of 14 June 2023). A waiver was added for the paediatric population below 6 years of age.
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19-M03

SIGA Technologies, Inc.; Treatment of the following viral infections in adults and children with body weight at least 13 kg: smallpox, monkeypox, cowpox / Treatment of complications due to replication of vaccinia virus following vaccination against smallpox in adults and children with body weight at least 13 kg

Day 60 opinion

Infectious Diseases

Note: Withdrawal request received on 26 July 2024

2.3.14. Lasmiditan - EMEA-002166-PIP01-17-M08

Eli Lilly and Company Ltd; Treatment 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 (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/0175/2023 of 15 May 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Rimegepant - EMEA-002812-PIP02-20-M03

Pfizer Europe MA EEIG; Treatment of migraine headaches

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO noted the responses of the applicant and adopted a favourable opinion on the modification of the agreed paediatric investigation plan (PIP) as set in the Agency's latest decision (P/0142/2023 of 14 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M06

Vanda Pharmaceuticals Netherlands B.V.; Treatment of disorders of the sleep-wake

schedule

Day 60 opinion

Neurology

Summary of Committee discussion:

In July 2024 the PDCO noted the responses of the applicant to the issues raised in June 2024.
The PDCO therefore adopted a favourable opinion on the modification of the agreed paediatric investigation plan (PIP) as set in the Agency's latest decision (P/0215/2018 of 17 July 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Vesleteplirsen - EMEA-003305-PIP01-22-M01

Sarepta Therapeutics Ireland; Treatment of Duchenne muscular dystrophy

Day 60 opinion

Neurology

Note: Withdrawal request received on 1 August 2024

2.3.18. Avelumab - EMEA-001849-PIP02-15-M05

Merck Healthcare KGaA; Treatment of malignant neoplasms of lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of malignant neoplasms of the central nervous system

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, including the deletion from the PIP of the expansion phase of Study 7 and deletion of Study 5 in paediatric patients with central nervous system malignant neoplasms, were acceptable.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0546/2021 of 31 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Daratumumab - Orphan - EMEA-002152-PIP01-17-M04

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

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (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/0238/2022 of 8 July 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M03

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2024 plenary meeting, a request for modification for gemtuzumab ozogamicin for the treatment of acute myeloid leukaemia.
The applicant requested to delay completion of a clinical study.
The PDCO confirmed all the conclusions reached at Day 30 and, based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), 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/0014/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21-M01

Miltenyi Biomedicine GmbH; Treatment of mature B-cell neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2024 plenary meeting, a request for modification for zamtocabtagene autoleucel for the treatment of mature B-cell neoplasms.
The applicant requested to remove one of the studies from the paediatric investigation plan (PIP).
The PDCO confirmed all the conclusions reached at Day 30 and assessed additional information provided between Day 30 and Day 60. Based on the review of the documentation submitted by the applicant for modifying the agreed PIP, the Committee 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/0069/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04

Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

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 (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification, the applicant requested to delete Study 2 and update Study 3.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0106/2023 of 14 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Iptacopan - Orphan - EMEA-002705-PIP03-20-M01

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 opinion

Other / Haematology-Hemostaseology

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 (changes to the clinical study design and completion date) 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/0322/2021 of 12 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Ebola Zaire vaccine (rVSVΔG-ZEBOV-GP, live) - EMEA-001786-PIP01-15-M04

Merck Sharp & Dohme (Europe), Inc.; Prevention of Ebola disease

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (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/0495/2023 of 1 December 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.25. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M02

Sanofi Pasteur; Prevention of disease caused by *Streptococcus pneumoniae*

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) for multivalent pneumococcal polysaccharide conjugate to carrier protein (PCV21), 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/0113/2024 of 12 April 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. Recombinant varicella zoster virus glycoprotein E - EMEA-001426-PIP01-13-M03

GlaxoSmithKline Biologicals; Prevention of varicella zoster virus (VZV) reactivation

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/0222/2018 of 17 July 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.27. Leriglitazone - Orphan - EMEA-002106-PIP01-16-M03

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 30 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 relatively minor delay of the PIP 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/0115/2022 of 13 April 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Etrasimod L-arginine - EMEA-002713-PIP02-21-M02

Pfizer Europe MA EEIG; Treatment of Crohn's disease

Day 30 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 (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification the applicant requested an extension to the completion dates of PIP Study 3 and 4.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0528/2023 of 29 December 2023).
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. Avapritinib - EMEA-C2-002358-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 30 letter

Oncology

2.7.2. Danicopan - EMEA-C2-002310-PIP01-17-M01

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 letter

Haematology-Hemostaseology

2.7.3. Mavorixafor - EMEA-C1-002490-PIP01-18

X4 Pharmaceuticals (Austria) GmbH; Treatment of warts, hypogammaglobulinemia, infections and myelokathexis (WHIM) syndrome

Day 30 letter

Immunology-Rheumatology-Transplantation

2.7.4. Cannabidiol - EMEA-C4-001964-PIP01-16-M04

Jazz Pharmaceutical Ireland Ltd.; Treatment of seizures associated with Dravet syndrome

Day 30 letter

Neurology

2.7.5. Metreleptin - EMEA-C3-001701-PIP01-14-M02

Amryt Pharmaceuticals DAC; Treatment of lipodystrophy

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. Bitopertin - Orphan - EMEA-000439-PIP03-23

Disc Medicine B.V.; Treatment of X-linked protoporphyria / Treatment of erythropoietic protoporphyria

Day 90 discussion

Dermatology / Haematology-Hemostaseology

3.1.2. EMEA-003531-PIP01-23

Treatment of hyperphenylalaninemia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Venglustat - Orphan - EMEA-001716-PIP08-23

Sanofi B.V.; Treatment of Fabry disease

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Proline derivative - EMEA-003440-PIP01-23

Treatment of type 1 interferonopathies

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.5. Dazukibart - Orphan - EMEA-003089-PIP02-23

Pfizer Europe MA EEIG; Treatment of idiopathic inflammatory myopathy

Day 90 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.6. 3,3-dimethyl-N-(6-methyl-5-{[2-(1-methyl-1H-pyrazol-4-yl)pyridine-4-yl]oxy}pyridine-2-yl)-2-oxopyrrolidine-1-carboxamide hydrochloride hydrate - Orphan - EMEA-003495-PIP02-24

Abbisko Therapeutics., Co., Ltd.; Treatment of tenosynovial giant cell tumours

Day 90 discussion

Oncology

3.1.7. Idroxioleic acid, sodium - Orphan - EMEA-003565-PIP01-23

Laminar Pharmaceuticals SA; Treatment of malignant glioma in children, including paediatric-type diffuse high-grade glioma

Day 90 discussion

Oncology

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

Prevention of varicella zoster virus reactivation

Day 90 discussion

Vaccines

3.1.9. Adeno-associated virus serotype 9 vector containing the human LAMP2 isoform B transgene - Orphan - EMEA-003639-PIP01-24

Rocket Pharmaceuticals, B.V.; Treatment of Danon disease

Day 60 discussion

Cardiovascular Diseases

3.1.10. EMEA-003633-PIP01-24

Treatment of psoriasis

Day 60 discussion

Dermatology

3.1.11. Dasiglucagon - Orphan - EMEA-002233-PIP03-24

Zealand Pharma A/S; Treatment of congenital hyperinsulinism

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.12. Etavopivat - Orphan - EMEA-002924-PIP03-24

Novo Nordisk A/S; Treatment of thalassaemia

Day 60 discussion

Haematology-Hemostaseology

3.1.13. Sonelokimab - EMEA-002568-PIP03-24

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis, and juvenile idiopathic arthritis)

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.14. Heterologous intestinal microbiota from healthy donors - EMEA-003635-PIP01-24

Treatment of *Clostridioides difficile* infection (CDI)

Day 60 discussion

Infectious Diseases

3.1.15. EMEA-003602-PIP01-24

Treatment of tuberculosis

Day 60 discussion

Infectious Diseases

3.1.16. Adeno-associated viral vector serotype 9 containing the human MECP2 gene - Orphan - EMEA-003634-PIP01-24

Taysha Gene Therapies, Inc.; Treatment of Rett syndrome

Day 60 discussion

Neurology

3.1.17. H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O2)-NH2-DOTA-225-Actinium - Orphan - EMEA-003630-PIP01-24

NovaCurie GmbH; Treatment of low-grade glioma

Day 60 discussion

Neurology

3.1.18. EMEA-003631-PIP01-24

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) whose treatment may include DNA-damaging agents

Day 60 discussion

Oncology

3.1.19. Golcadomide - Orphan - EMEA-003161-PIP02-24

Bristol-Myers Squibb Pharma EEIG; Treatment of mature B-cell neoplasms

Day 60 discussion

Oncology

3.1.20. Esonadogene imvoparvovec - Orphan - EMEA-003626-PIP01-24

Neurophth Therapeutics, Inc.; Treatment of Leber’s hereditary optic neuropathy

Day 60 discussion

Ophthalmology

3.1.21. Tacrolimus - EMEA-003625-PIP01-24

Treatment of vernal keratoconjunctivitis

Day 60 discussion

Ophthalmology

3.1.22. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24

Prevention of lower respiratory tract disease caused by respiratory syncytial virus or human metapneumovirus

Day 60 discussion

Vaccines

3.1.23. mRNA-based vaccine consisting of 3 mRNAs encoding the VP1 capsid protein from NoV genotypes, GII.4, GII.3 and GI.3 - EMEA-003637-PIP01-24

Prevention of norovirus acute gastroenteritis

Day 60 discussion

Vaccines

3.1.24. Zirconium (^{89}Zr) girentuximab senvedoxam - EMEA-002482-PIP01-24

Diagnostic use for indetermined renal masses (IDRMs) detected by conventional imaging

Day 30 discussion

Diagnostic / Oncology / Uro-nephrology

3.1.25. Dexfadrostat phosphate - EMEA-003642-PIP01-24

Treatment of primary aldosteronism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.1.26. EMEA-003652-PIP01-24

Treatment of non-cirrhotic metabolic dysfunction-associated steatohepatitis (MASH) with fibrosis for homozygous carriers of the PNPLA3 rs738409 148M risk allele / cirrhotic metabolic dysfunction-associated steatohepatitis (MASH) for homozygous carriers of the PNPLA3 rs738409 148M risk allele

Day 30 discussion

Gastroenterology-Hepatology

3.1.27. Duvakitug - EMEA-003647-PIP01-24

Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.1.28. Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding the codon-optimised version of PKLR gene - Orphan - EMEA-003650-PIP01-24

Rocket Pharmaceuticals, B.V.; Treatment of pyruvate kinase deficiency (PKD)

Day 30 discussion

Haematology-Hemostaseology

3.1.29. Riliprubart - Orphan - EMEA-002903-PIP04-24

Sanofi B.V.; Treatment of antibody-mediated rejection (AMR) in kidney transplant recipients

Day 30 discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology / Neurology

3.1.30. Lenacapavir sodium - EMEA-002740-PIP03-24

Prevention of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.1.31. Lenacapavir / islatravir - EMEA-003655-PIP01-24

Treatment of human immunodeficiency virus (HIV 1) infection

Day 30 discussion

Infectious Diseases

3.1.32. Ruzasvir / bemnifosbuvir - EMEA-003653-PIP01-24

Treatment of chronic hepatitis C

Day 30 discussion

Infectious Diseases

3.1.33. Zosurabalpin - EMEA-003645-PIP01-24

Treatment of infections due to ABC organisms

Day 30 discussion

Infectious Diseases

3.1.34. Probenecid - EMEA-003654-PIP01-24

Prevention of epilepsy in focal cortical dysplasia

Day 30 discussion

Neurology

3.1.35. Recombinant human progranulin fused to an Fc fragment engineered to contain a human transferrin receptor 1 binding domain - EMEA-003643-PIP01-24

Treatment of frontotemporal dementia

Day 30 discussion

Neurology

3.1.36. Remibrutinib - EMEA-002582-PIP04-24

Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.1.37. ALK inhibitor - EMEA-003648-PIP01-24

Treatment of malignant lung neoplasms

Day 30 discussion

Oncology

3.1.38. Plinabulin - EMEA-003646-PIP01-24

Treatment of stage IIIb and IV non-small cell lung cancer (NSCLC)

Day 30 discussion

Oncology

3.1.39. Zidesamtinib - EMEA-003649-PIP01-24

Treatment of malignant lung neoplasms

Day 30 discussion

Oncology

3.1.40. Zipalertinib - EMEA-003656-PIP01-24

Treatment of non-small cell lung cancer

Day 30 discussion

Oncology

3.1.41. Lunsekimig - EMEA-003644-PIP01-24

Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.1.42. Alogabat - EMEA-003651-PIP01-24

Treatment of autism spectrum disorder (ASD)

Day 30 discussion

Psychiatry

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. Belumosudil mesylate - EMEA-C1-003425-PIP01-23

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.2. Dalbavancin hydrochloride - EMEA-C-000016-PIP01-07-M09

AbbVie Ltd; Treatment of acute bacterial skin and skin structure infections (ABSSSI)

Day 30 discussion

Infectious Diseases

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Dupilumab - EMEA-001501-PIP07-20-M02

Sanofi Winthrop Industrie; Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

3.3.2. Lebrikizumab - EMEA-002536-PIP01-18-M04

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.3. Romosozumab - EMEA-001075-PIP04-15-M07

UCB Pharma S.A.; Treatment of osteoporosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Mepolizumab - EMEA-000069-PIP01-07-M09

GSK Trading Services Limited; Treatment of hypereosinophilic syndrome

Day 30 discussion

Haematology-Hemostaseology

3.3.5. Recombinant human A disintegrin and metalloprotease with thrombospondin type-1 motifs 13 (rADAMTS13) - Orphan - EMEA-001160-PIP01-11-M05

Takeda Pharmaceuticals International AG; Treatment of thrombotic thrombocytopenic purpura

Day 30 discussion

Haematology-Hemostaseology

3.3.6. Belatacept - EMEA-000157-PIP01-07-M06

Bristol-Myers Squibb Pharma EEIG; Prevention of rejection of transplanted kidney

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.7. Upadacitinib - EMEA-001741-PIP01-14-M08

AbbVie Ltd; Treatment of chronic idiopathic arthritis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.8. Ustekinumab - EMEA-000311-PIP03-11-M07

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.9. Ibrexafungerp citrate - Orphan - EMEA-002535-PIP04-21-M02

SCYNEXIS, Inc.; Prevention of recurrent vulvovaginal candidiasis / Treatment of vulvovaginal candidiasis

Day 30 discussion

Infectious Diseases

3.3.10. Posaconazole - EMEA-000468-PIP02-12-M09

MSD Europe Belgium SRL; Treatment of invasive fungal infections / Prevention of invasive fungal infections

Day 30 discussion

Infectious Diseases

3.3.11. Tazobactam / ceftolozane - EMEA-001142-PIP02-16-M02

MSD Europe Belgium SRL; Treatment of pneumonia

Day 30 discussion

Infectious Diseases

3.3.12. Ganaxolone - Orphan - EMEA-002341-PIP01-18-M03

Marinus Pharmaceuticals Inc.; Treatment of cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder

Day 30 discussion

Neurology

3.3.13. Pridopidine (hydrochloride) - Orphan - EMEA-003174-PIP01-21-M02

Prilenia Therapeutics B.V.; Treatment of Huntington disease

Day 30 discussion

Neurology

3.3.14. Vamorolone - Orphan - EMEA-001794-PIP02-16-M09

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Neurology

3.3.15. Avapritinib - Orphan - EMEA-002358-PIP02-18-M04

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

Day 30 discussion

Oncology

3.3.16. Daunorubicin / cytarabine - Orphan - EMEA-001858-PIP02-16-M04

Jazz Pharmaceuticals Ireland Limited; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology

3.3.17. Fidrisertib - Orphan - EMEA-003133-PIP01-21-M01

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva (FOP)

Day 30 discussion

Other

3.3.18. Lomitapide (as lomitapide mesylate) - EMEA-001124-PIP01-10-M06

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia

Day 30 discussion

Other

3.3.19. Brensocatib - EMEA-002905-PIP01-20-M01

Insmed Netherlands B.V.; Treatment of non-cystic fibrosis bronchiectasis

Day 30 discussion

Pneumology - Allergology

3.3.20. Esketamine hydrochloride - EMEA-002772-PIP01-20-M01

Celon Pharma S.A.; Treatment of bipolar depression / Treatment of major depressive disorder

Day 30 discussion

Psychiatry

3.3.21. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21-M03

Swedish Orphan Biovitrum AB (publ); Treatment of glomerulonephritis and nephrotic syndrome

Day 30 discussion

Uro-nephrology

3.3.22. mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 (mRNA-1283) - EMEA-003426-PIP01-23-M02

MODERNA BIOTECH SPAIN, S.L.; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Vaccines

3.3.23. *Neisseria meningitidis* serogroup W polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup Y polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup C polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M06

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 30 discussion

Vaccines

3.3.24. Respiratory syncytial virus vaccine (bivalent, recombinant) - EMEA-002795-PIP02-21-M02

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

Day 30 discussion

Vaccines

Note: Withdrawal request received on 5 August 2024

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 19 August 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

6.1.1. Tezepelumab - EMEA-08-2024

AstraZeneca AB; All classes of medicinal products for the treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft versus-host disease after [bone-marrow] transplantation).

Proposed indication is as an add-on to inhaled maintenance treatment in COPD patients with a history of exacerbations guided by blood eosinophil (EOS) counts.

Summary of Committee discussion:

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

Other potential paediatric interests of this medicine suggested by PDCO: asthma, eosinophilic oesophagitis (EoE), and chronic spontaneous urticaria.

6.1.2. 2'-O-(2-methoxyethyl) antisense oligonucleotide - EMA/PE/0000183128

Biogen Netherlands B.V.; Alzheimer's disease

Summary of Committee discussion:

The inclusion of the indication within the class waived condition was confirmed. The PDCO agreed to update the list of class waivers, last updated 9 years ago.

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

No item

9.1.2. Vote by Proxy

No item

9.1.3. Strategic Review and Learning Meeting (SRLM) – Budapest, Hungary, 10-11 October 2024

Summary of Committee discussion:

The PDCO members were updated on the SRLM under the Hungarian Presidency of the Council of the EU to be held in person on 10-11 October 2024 in Budapest, Hungary.

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 June 2024, 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 July 2024, 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. Paediatric Formulation Operational Expert Group (PFOEG)

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. 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:

The topic has been cancelled as there were no upcoming ITF meetings to be presented.

9.4. Cooperation within the EU regulatory network

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

Summary of Committee discussion:

The Committee was informed about the annual meeting of the European network of paediatric research at EMA (Enpr-EMA), which will take place on 02 October 2024.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

No item

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

No item

9.7. PDCO work plan

Summary of Committee discussion:

The topic has been postponed to September 2024 plenary.

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

No item

10. Any other business

10.1. ICH E11A on Paediatric Extrapolation

Summary of Committee discussion:

The Step 3 document agreed by the ICH Expert Working Group was presented to PDCO for information. The document has previously been adopted by CHMP. The differences between the previous Step 2 document and the new version were highlighted. The process for adoption and implementation were discussed, including the need for relevant case studies as training materials are developed.

10.2. PDCO Agenda and Time schedule in IRIS

Summary of Committee discussion:

The PDCO was presented with a brief live demo of the agenda and time schedule in IRIS and informed on the search function to locate the previous paediatric investigation plan (PIP) numbers of the newly submitted procedures in IRIS.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The Committee discussed clinical factors to be considered when deciding about possible waiver cut-offs for brain tumours.

11.2. Neonatology

Summary of Committee discussion:

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

11.3. Internal PDCO Operations

Summary of Committee discussion:

The PDCO discussed matters concerning internal operations.

11.4. HIV

Summary of Committee discussion:

The PDCO discussed aspects of some procedures belonging to the HIV therapeutic area.

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 23-26 July 2024 PDCO meeting, which was held 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 participation in discussion, final deliberations and voting on:	2.3.18. Avelumab - EMEA-001849-PIP02-15-M05 3.3.10. Posaconazole - EMEA-000468-PIP02-12-M09
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No participation in discussion, final deliberations and voting on:	2.3.22. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04
Miroslav Weiss	Member	Croatia	No interests declared	
Zena Gunther	Member	Cyprus	No interests declared	
Tereza Bazantova	Member	Czechia	No interests declared	
Pavlina Chladová	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	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Anastasia Mountaki	Alternate	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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dina Apele-Freimane	Member	Latvia	No participation in discussion, final deliberations and voting on:	3.1.24. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24 3.3.24. Respiratory syncytial virus vaccine (bivalent, recombinant) - EMEA-002795-PIP02-21-M02
Sima Naujokiene	Alternate	Lithuania	No interests declared	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
John-Joseph Borg	Member	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	
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	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando	Member	Healthcare	No participation in	3.1.24. Human

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Cabanas		Professionals' Representative	discussion, final deliberations and voting on:	metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24 3.3.24. Respiratory syncytial virus vaccine (bivalent, recombinant) - EMEA-002795-PIP02-21-M02
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jose Ignacio Malagon Calle	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	
María Estela Moreno Martín	Expert	Spain	No interests declared	
Friederike Feldmann	Expert	Germany	No interests declared	
Meeting run with support from relevant EMA staff				

Experts' declared interests 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/