29 February 2024
EMA/PDCO/531147/2023 Corr.1*
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 07-10 November 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

Health and safety information

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

Disclaimers

Some of the information contained in this set of minutes is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Of note, this set of minutes is a working document primarily designed for PDCO members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

* Evaluation of Declaration of Interest (DoI) outcome amended - Agenda point 12 (page 46)

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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 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 welcomed the new member and alternate.

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

1.2. Adoption of agenda

The agenda for 07-10 November 2023 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 10-13 October 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. 3-(4-acetamidophenyl)-2-(S)-methoxypropionic acid, or (S)-3-(4-acetamidophenyl)-2-methoxypropanoic acid - EMEA-002674-PIP01-19

Nogra Pharma Limited; Treatment of acne vulgaris

Day 120 opinion

Dermatology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for the paediatric population from 9 years to less than 18 years of age, in the condition treatment of acne vulgaris was adopted. The PDCO agreed on a waiver in children from birth to less than 9 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

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

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

Day 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 endorsed the proposed paediatric investigation plan for modified messenger ribonucleic acid encoding human propionyl-coenzyme A carboxylase alpha and beta subunits encapsulated into lipid nanoparticles for the condition of treatment of propionic acidaemia for all subsets of the paediatric population. Granting a deferral for one or more measures contained in the paediatric investigation plan has been recommended. A positive opinion has been adopted.

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

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

Day 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 concluded that the modified paediatric investigational plan (PIP) is acceptable. The PDCO adopted a PIP for treatment of methylmalonic acidaemia.

A deferral has been granted for one or more measures contained in the PIP.

2.1.4. AstraZeneca anti-SARS-CoV-2 monoclonal antibody (AZD3152) - EMEA-003350-PIP01-23

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

Day 120 opinion

Immunology-Rheumatology-Transplantation

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 the paediatric population from birth to less than 18 years of age, in the condition of prevention of coronavirus disease 2019 (COVID-19) was adopted.
The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.5. Inebilizumab - EMEA-001911-PIP03-23

Horizon Therapeutics Ireland Designated Activity Company (DAC); Treatment of immunoglobulin G4-related disease

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from 2 years to less than 18 years of age in the condition of treatment of immunoglobulin G4-related disease was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of lack of significant therapeutic benefit. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.6. Broadly neutralising anti-HIV human monoclonal antibody (VH3810109)- EMEA-003392-PIP01-23

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) Infection

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

In November 2023, 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 2 years to less than 18 years of age, in the condition of treatment of HIV-1 infection was adopted. The PDCO agreed on a waiver for the paediatric population from birth to less than 2 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 thePIP.

2.1.7. Vemircopan - Orphan - EMEA-002863-PIP02-23

Alexion Europe SAS; Treatment of generalised myasthenia gravis

Day 120 opinion

Neurology

Note: Withdrawal request received on 9 November 2023

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

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

Day 120 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO adopted on its own motion a waiver for the entire paediatric population (from birth to less than 18 years of age) in the condition 'treatment of acute myeloid leukaemia (AML)', based 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 in the paediatric population. This was based on the consideration that the studies that may be carried in paediatric patients with AML harbouring isocitrate dehydrogenase (IDH) mutations are not expected to generate the necessary data to establish a benefit/risk of the product in paediatric patients with AML.
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 gliomas as a disease with an unmet need that may benefit from the treatment with olutasidenib. 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.

2.1.9. Tamibarotene - Orphan - EMEA-003329-PIP02-22

Syros Pharmaceutical (Ireland) Limited; Treatment of acute myeloid leukaemia / Treatment of myelodysplastic syndromes

Day 120 opinion

Oncology

Note: Withdrawal request received on 1 November 2023

2.1.10. Cedazuridine / decitabine - EMEA-003071-PIP02-23

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

Day 120 opinion

Oncology / Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for patients from 3 months to less than 18 years of age, in the condition of treatment of myelodysplastic syndromes, including juvenile myelomonocytic leukaemia was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of lack of significant therapeutic benefit. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.11. Taldefgrobep alfa - Orphan - EMEA-003386-PIP01-22

Biohaven Bioscience Ireland Limited; Treatment of spinal muscular atrophy

Day 120 opinion

Other

Summary of Committee discussion:

The PDCO discussed at Day 120, during the November 2023 plenary meeting, an application for a paediatric investigation plan, waiver and deferral for taldefgrobep alfa for treatment of spinal muscular atrophy.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.
The PDCO adopted a positive opinion on a paediatric investigation plan.

2.1.12. Complement factor B antisense oligonucleotide (RO7434656) - EMEA-003396-PIP01-23

Roche Registration GmbH; Treatment of primary IgA nephropathy

Day 120 opinion

Uro-nephrology

Summary of Committee discussion:

Prior to Day 120, the applicant addressed all outstanding issues satisfactorily. The PDCO adopted a positive opinion for a paediatric investigation plan (PIP) for complement factor B antisense oligonucleotide (RO7434656) for the treatment of primary immunoglobulin A nephropathy (IgAN) in paediatric patients from 2 years of age. A waiver was agreed for the paediatric population from birth to less than 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The PIP includes one clinical study (single-arm safety, tolerability, pharmacokinetics, pharmacodynamics, and activity study), a modelling and simulation study and an extrapolation plan. The completion of the PIP is deferred. However, the clinical study

must be initiated prior to submission of the application for a standard marketing authorisation for the treatment of adult IgAN patients.

2.1.13. EMEA-003487-PIP01-23

Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Note: Withdrawal request received on 19 October 2023

2.1.14. Elinzanetant - EMEA-003500-PIP01-23

Bayer AG; Treatment of vasomotor symptoms caused by endocrine therapy related to breast cancer

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for elinzanetant for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of vasomotor symptoms caused by endocrine therapy related to breast cancer, on the grounds that the condition for which this product is intended does not occur in the specified paediatric subset.
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. Humanised IgG1 kappa monoclonal antibody directed against IGF-1R - EMEA-003499-PIP01-23

Viridian Therapeutics, Inc.; Treatment of thyroid eye disease

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO confirmed the outcome of Day 30 discussion and based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant´s request for a waiver. The PDCO recommended granting a waiver for humanised IgG1 kappa monoclonal antibody directed against IGF-1R (VRDN-001) for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition of treatment of thyroid eye disease on the grounds that the specific medicinal product is likely to be unsafe in the paediatric population from birth to adolescence before growth is complete, and

on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments in the paediatric population from adolescents whose growth is complete to less than 18 years.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.16. Sitagliptin / dapagliflozin - EMEA-003486-PIP01-23

KRKA, d.d., Novo mesto; Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO confirmed the outcome of Day 30 discussion and based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant´s request for a waiver. The PDCO recommended granting a waiver for sitagliptin / dapagliflozin for the paediatric population from birth to less than 10 years of age for the condition of treatment of type 2 diabetes mellitus on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset and a waiver for sitagliptin / dapagliflozin for the paediatric population from 10 to less than 18 years of age for the condition of treatment of type 2 diabetes mellitus on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.17. Diflunisal - Orphan - EMEA-003490-PIP01-23

AO Pharma AB; Treatment of transthyretin amyloidosis

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 diflunisal for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of transthyretin amyloidosis, 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.

2.1.18. EMEA-003495-PIP01-23

Treatment of tenosynovial giant cell tumours

Day 60 opinion

Oncology

Note: Withdrawal request received on 8 November 2023

2.1.19. Oregovomab - EMEA-003497-PIP01-23

CanariaBio Inc.; Treatment of primary peritoneal cancer / Treatment of ovarian cancer / Treatment of fallopian tube cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the November 2023 plenary meeting, an application for a waiver for oregovomab for all subsets of the paediatric population in the condition of treatment of ovarian cancer.
Between Day 30 and Day 60 of the procedure, that applicant submitted an amended application form in which, in line with the PDCO Day 30 comments, treatment of fallopian tube and primary peritoneal cancers were also included.
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 oregovomab for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of ovarian cancer, treatment of fallopian tube cancer and treatment of primary peritoneal cancer.
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.

2.1.20. Rilvegostomig - EMEA-003501-PIP01-23

AstraZeneca AB; Treatment of biliary tract cancer / Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for rilvegostomig for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung cancer and treatment of biliary tract cancer. Since the agreed waiver ground is disease not occurring, the possibility to apply this to all pharmaceutical forms and all routes of

administrations was agreed to by the applicant.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.21. Lotilaner - EMEA-003488-PIP01-23

Tarsus Pharmaceuticals, Inc.; Treatment of *Demodex* blepharitis

Day 60 opinion

Ophthalmology / Infectious Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for lotilaner for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition treatment of *Demodex* blepharitis.

2.1.22. DNA plasmid encoding HPV type 18 consensus E6 and E7 proteins (pGX3002 - bizalimogene ralaplasmid) / DNA plasmid encoding HPV type 16 consensus E6 and E7 proteins (pGX3001-mavilimogene ralaplasmid) - EMEA-002022-PIP02-23

Inovio Pharmaceuticals Inc.; Treatment of squamous intraepithelial lesions of the anus caused by HPV types 16 and 18

Day 60 opinion

Vaccines / Infectious Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for DNA plasmid encoding HPV type 16 consensus E6 and E7 proteins (pGX3001 - mavilimogene ralaplasmid) / DNA plasmid encoding HPV type 18 consensus E6 and E7 proteins (pGX3002 - bizalimogene ralaplasmid) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of squamous intraepithelial lesions of the anus caused by HPV types 16 and 18. The waiver is based 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 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. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.23. Baxdrostat - EMEA-003507-PIP01-23

AstraZeneca AB; Treatment of hypertension

Day 30 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 baxdrostat for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of hypertension.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.24. Retifanlimab - Orphan - EMEA-002798-PIP04-23

Incyte Biosciences Distribution B.V.; Treatment of lung cancer

Day 30 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 30, during the November 2023 plenary meeting, an application for a waiver for oregovomab for all subsets of the paediatric population in the condition of treatment of ovarian cancer.
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 retifanlimab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of lung cancer.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.25. Allogeneic cultured postnatal thymus-derived tissue - Orphan - EMEA-003496-PIP01-23

Enzyvant Therapeutics Ireland Limited; Treatment of congenital athymia

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, a positive opinion was adopted by the PDCO for the paediatric investigation plan

(PIP) for allogeneic cultured postnatal thymus-derived tissue for paediatric patients from birth to less than 18 years of age, in the condition of treatment of congenital athymia. A deferral was granted for the completion of this PIP.

2.1.26. Govorestat - Orphan - EMEA-003365-PIP02-23

Applied Therapeutics, Inc; Treatment of galactosaemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

Summary of Committee discussion:

Based on the assessment of this application, additional clarifications by the applicant, and further discussions at the Paediatric Committee, a positive opinion was adopted by the PDCO for the paediatric investigation plan (PIP) for govorestat for paediatric patients from birth to less than 18 years of age, in the condition of treatment of galactosaemia. A deferral was granted for the completion of this PIP.

2.2. Opinions on Compliance Check

2.2.1. Dupilumab - EMEA-C1-001501-PIP07-20-M01

Sanofi Winthrop Industrie; Treatment of chronic spontaneous urticaria

Day 60 letter

Dermatology

Summary of Committee discussion:

The applicant provided a clear explanation of the dose rationale. The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0297/2023) of 11 August 2023.
The PDCO finalised this partially completed compliance procedure on 10 November 2023.

2.2.2. Doxribtimine / doxecitine - EMEA-C1-003210-PIP01-22

UCB Pharma S.A.; Treatment of thymidine kinase 2 deficiency

Day 60 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The clarification provided by the applicant is endorsed and therefore Study 4 can also be considered compliant.
The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0287/2022) of 11 August 2022.
The PDCO finalised this partially completed compliance procedure on 10 November 2023.

2.2.3. Avibactam / ceftazidime - EMEA-C-001313-PIP01-12-M13

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

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

- EMEA-C1-001313-PIP01-12-M03
- EMEA-C2-001313-PIP01-12-M08

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0551/2022) of 4 January 2023.

2.2.4. Eliglustat - EMEA-C-000461-PIP02-11-M05

Sanofi B.V.; Treatment of Gaucher disease type 1 and type 3

Day 60 opinion

Other

Summary of Committee discussion:

The PDCO re-discussed at Day 60 this final compliance check for eliglustat during the November 2023 plenary meeting.
The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-000461-PIP02-11
- EMEA-C2-000461-PIP02-11-M05

and adopted on 10 November 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0440/2022) of 28 October 2022.

2.2.5. Isoflurane - EMEA-C-002320-PIP01-17-M03

Sedana Medical AB; Sedation of mechanically ventilated patients

Day 30 opinion

Neonatology - Paediatric Intensive Care

Summary of Committee discussion:

The PDCO adopted on 10 November 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0031/2023) of 31 January 2023.

2.2.6. Palovarotene - EMEA-C-001662-PIP01-14-M05

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 30 opinion

Other

Summary of Committee discussion:

The PDCO discussed at Day 30 this final compliance check for palovarotene during the November 2023 plenary meeting.
The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-001662-PIP01-14-M02
- EMEA-C2-001662-PIP01-14-M04

and adopted on 10 November 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0562/2021) of 31 December 2021.

2.2.7. Apremilast - EMEA-C-000715-PIP03-11-M06

Amgen Europe B.V.; Treatment of psoriasis

Day 30 opinion

Immunology-Rheumatology-Transplantation / Dermatology

Summary of Committee discussion:

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

- EMEA-C1-000715-PIP03-11-M01
- EMEA-C2-000715-PIP03-11-M06

The PDCO adopted on 10 November 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0469/2020) of 1 December 2020.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Birch bark extract - EMEA-001299-PIP01-12-M02

Amryt Pharmaceuticals DAC; Treatment of skin injuries

Day 60 opinion

Dermatology

Note: Withdrawal request received on 3 November 2023

2.3.2. Delgocitinib - EMEA-002329-PIP02-20-M03

LEO Pharma A/S; Treatment of chronic hand eczema

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/0136/2023 of 14 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Glycopyrronium bromide - EMEA-002383-PIP01-18-M03

Dr. August Wolff GmbH & Co. KG - Arzneimittel; Treatment of hyperhidrosis

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 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/0467/2022 of 17 November 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Interleukin-23 receptor antagonist peptide - EMEA-003301-PIP01-22-M01

Janssen-Cilag International NV; Treatment of psoriasis

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 the proposed changes on the weight cut-off for the study population for Studies 3 and 4 could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0323/2023 of 11 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Pfizer Europe MA EEIG; Treatment of alopecia areata

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 most of the proposed changes to some measures for Study 4 (B7981027) and 5 (B7981028) 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/0446/2022 of 28 October 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Venglustat - Orphan - EMEA-001716-PIP04-19-M01

Sanofi B.V.; Treatment of GM2 gangliosidosis / Treatment of GM1 gangliosidosis / Treatment of galactosialidosis / Treatment of sialidosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the November 2023 plenary meeting, a request for modification for venglustat for the treatment of GM2 gangliosidosis, treatment of GM1 gangliosidosis, treatment of galactosialidosis and treatment of sialidosis.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that some of 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/0457/2020 of 4 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Filgotinib - EMEA-001619-PIP03-16-M02

Galapagos NV; Treatment of ulcerative colitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification the applicant requested to:

1. Withdraw the condition Crohn's disease.
2. Request an extension of the waiver to children from 2 to <8 years of age.
3. Revise the ulcerative colitis clinical paediatric development.
4. Delay of the PIP completion date.

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0386/2020 of 25 September 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Mirikizumab - EMEA-002208-PIP01-17-M03

Eli Lilly and Company; Treatment of ulcerative colitis / Treatment of Crohn's disease

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification elements of Studies 4, 5 and 7 were updated.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0088/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Potassium chloride / sodium chloride / ascorbic acid / sodium sulfate / sodium ascorbate / polyethylene glycol 3350 - EMEA-001705-PIP02-15-M05

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 60 opinion

Gastroenterology-Hepatology

Note: Withdrawal request received on 10 November 2023

2.3.10. Zinc gluconate / alisitol / retinyl palmitate - Orphan - EMEA-002198-PIP01-21-M01

Vanessa Research Spain S.L.; Treatment of microvillus inclusion disease

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification procedure, the applicant requested to delay the completion of all studies in the PIP.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0171/2022 of 13 May 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Iron as ferric maltol - EMEA-001195-PIP01-11-M07

Norgine BV; Treatment of iron deficiency

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (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/0316/2021 of 11 August 2021).

2.3.12. Baricitinib - EMEA-001220-PIP01-11-M09

Eli Lilly and Company Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis 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 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/0202/2023 of 13 June 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Cendakimab - EMEA-002640-PIP01-19-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of eosinophilic esophagitis

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/0375/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19-M03

Sanofi B.V.; Treatment of immune thrombocytopenia

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:
In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (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/0262/2022 of 18 July 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Fully human IgG1 RB-1 YTE anti-RSV F monoclonal antibody - EMEA-002755-PIP01-19-M02

Merck Sharp & Dohme (Europe), Inc.; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 60 opinion

Infectious Diseases

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 the proposed changes in the number of patients and changes in the analysis and timelines for the Studies 1, 2, 3, 4, 5 and 7, including an interim analysis and a request for a deferral aimed to permit an earlier submission of the MAA 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/0554/2021 of 31 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Vaborbactam / meropenem - EMEA-001731-PIP01-14-M04

Menarini International Operations Luxembourg S.A.; Treatment of gram-negative bacterial infections

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/0260/2021 of 9 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Inebilizumab - EMEA-001911-PIP01-15-M05

Horizon Therapeutics Ireland DAC; Treatment of neuromyelitis optica spectrum disorders

Day 60 opinion

Neurology

Summary of Committee discussion:

The applicant addressed the remaining issues raised by the Committee at Day 30. Based on the review of the rationale submitted by the 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/0213/2022 of 10 June 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Pridopidine (hydrochloride) - Orphan - EMEA-003174-PIP01-21-M01

Prilenia Therapeutics B.V.; Treatment of Huntington disease (HD)

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 delay of the Study 3 start and delay of the planned PIP modification were both accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0546/2022 of 30 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Vamorolone - Orphan - EMEA-001794-PIP02-16-M06

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

Day 60 opinion

Other

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 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/0295/2022 of 11 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M04

Heron Therapeutics B.V.; Treatment of acute postoperative pain

Day 60 opinion

Pain

Note: Withdrawal request received on 9 November 2023

2.3.21. Mirabegron - EMEA-000597-PIP02-10-M10

Astellas Pharma Europe B.V.; Treatment of idiopathic overactive bladder

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

In line with the arguments outlined at Day 30, the PDCO refused the proposed modification and granted a full waiver for the treatment of idiopathic overactive bladder for the paediatric population from birth to less than 18 years on its own motion (based on lack of significant therapeutic benefit as clinical studies are not feasible).

2.3.22. Pneumococcal polysaccharide serotype 35B – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 31 – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 24F – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 23B – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 23A – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 16F – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 15C – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 15A – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 20A – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 17F – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 12F – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 11A – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 10A – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 9N – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 8 – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjuguate / Pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjuguate - EMEA-003155-PIP01-21-M01

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by *Streptococcus pneumoniae*

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of 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/0485/2022 of 2 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Recombinant respiratory syncytial virus pre-fusion F protein, adjuvanted with AS01E - EMEA-002904-PIP01-20-M01

GlaxoSmithKline Biologicals S.A.; Prevention of lower respiratory tract disease caused by respiratory syncytial virus

Day 60 opinion

Vaccines

Summary of Committee discussion:

The PDCO discussed the additional information provided by the applicant and consider the additional justification provided acceptable. 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/0456/2021 of 29 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Etrasimod - EMEA-002713-PIP02-21-M01

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. The modification request pertained to a delay to the completion of Studies 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/0346/2022 of 10 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.25. Satralizumab - Orphan - EMEA-001625-PIP01-14-M07

Roche Registration GmbH; Treatment of neuromyelitis optica

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 postponement of the completion of two studies, due to recruitment difficulties, 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/0477/2020 of 1 December 2020). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

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

2.7.1. Tezacaftor / ivacaftor / elexacaftor – EMEA-C5-002324-PIP01-17-M05

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 letter

Other

2.7.2. Fidanacogene elaparvovec – EMEA-C2-002362-PIP02-19-M02

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

Day 30 letter

Haematology-Hemostaseology

2.7.3. Nirogacestat hydrobromide – EMEA-C1-002971-PIP01-21

SpringWorks Therapeutics Ireland Limited; Treatment of soft tissue sarcoma

Day 30 letter

Oncology

2.7.4. Nemolizumab – EMEA-C1-001624-PIP01-14-M06

Galderma International S.A.S.; Treatment of atopic dermatitis

Day 30 letter

Dermatology

2.7.5. Bimezikumab – EMEA-C1-002189-PIP03-19

Manuel Iniesta; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 letter

Immunology-Rheumatology-Transplantation

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. Obicetrapib - EMEA-003438-PIP02-23

Treatment of elevated cholesterol

Day 90 discussion

Cardiovascular Diseases

3.1.2. Remibrutinib - EMEA-002582-PIP03-23

Treatment of chronic inducible urticaria

Day 90 discussion

Dermatology

3.1.3. Frexalimab - EMEA-002945-PIP03-23

Treatment of type 1 diabetes mellitus

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Semaglutide - EMEA-003402-PIP01-23

Treatment of non-alcoholic steatohepatitis (NASH)

Day 90 discussion

Gastroenterology-Hepatology

3.1.5. Tarperprumig - Orphan - EMEA-003432-PIP01-23

Alexion Europe SAS; Treatment of sickle cell disease (SCD)

Day 90 discussion

Haematology-Hemostaseology

3.1.6. EMEA-003271-PIP02-22

Treatment of epilepsy syndromes / Treatment of primary generalised tonic-clonic seizures

Day 90 discussion

Neurology

3.1.7. Dordaviprone - Orphan - EMEA-003389-PIP01-23

Chimerix IRL Limited; Treatment of glioma

Day 90 discussion

Oncology

3.1.8. Trotabresib - EMEA-003361-PIP01-22

Treatment of malignant neoplasms of the central nervous system

Day 90 discussion

Oncology

3.1.9. Apitegromab - Orphan - EMEA-002951-PIP02-21

Scholar Rock, Inc.; Treatment of spinal muscular atrophy

Day 90 discussion

Other / Neurology

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

Treatment of anaemias due to chronic kidney disorders

Day 90 discussion

Uro-nephrology

3.1.11. Zigakibart - Orphan - EMEA-003300-PIP01-22

Chinook Therapeutics, Inc.; Treatment of primary IgA nephropathy

Day 90 discussion

Uro-nephrology

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

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Vaccines / Infectious Diseases

3.1.13. Seralutinib - Orphan - EMEA-002972-PIP02-23

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 60 discussion

Cardiovascular Diseases

3.1.14. Mitapivat - Orphan - EMEA-002684-PIP03-23

Agios Netherlands B.V.; Treatment of sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

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

Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.16. *Betula pendula* pollen allergoid, mannan-conjugated, polymerised - EMEA-003492-PIP01-23

Treatment of allergic rhinitis/rhino-conjunctivitis / Treatment of birch pollen allergic rhinitis/rhino-conjunctivitis

Day 60 discussion

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 9 November 2023

3.1.17. *Dermatophagoides pteronyssinus* allergoid, mannan-conjugated, polymerised / *Dermatophagoides farinae* allergoid, mannan-conjugated, polymerised - EMEA-003493-PIP01-23

Treatment of allergic rhinitis/rhino-conjunctivitis / Treatment of house dust mite allergic rhinitis/rhino-conjunctivitis

Day 60 discussion

Immunology-Rheumatology-Transplantation

Note: *Withdrawal request received on 9 November 2023*

3.1.18. *Phleum pratense* pollen allergoid, mannan-conjugated, polymerised / *Dactylis glomerata* pollen allergoid, mannan-conjugated, polymerised - EMEA-003491-PIP01-23

Treatment of grass pollen allergic rhinitis/rhino-conjunctivitis / Treatment of allergic rhinitis/rhino-conjunctivitis

Day 60 discussion

Immunology-Rheumatology-Transplantation

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

Treatment of type 1 interferonopathies

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.20. EMEA-003489-PIP01-23

Treatment of relapsed and refractory solid malignant tumours / Treatment of relapsed and refractory malignant neoplasms of the hematopoietic and lymphoid tissue / Treatment of malignant neoplasms of the hematopoietic and lymphoid tissue

Day 60 discussion

Oncology

3.1.21. Live attenuated respiratory syncytial virus (RSV) - EMEA-003277-PIP02-23

Prevention of respiratory syncytial virus (RSV) disease

Day 60 discussion

Vaccines

3.1.22. Human papillomavirus type 58 L1 protein / human papillomavirus type 52 L1 protein / human papillomavirus type 45 L1 protein / human papillomavirus type 33 L1 protein / human papillomavirus type 31 L1 protein / human papillomavirus type 18 L1 protein / human papillomavirus type 16 L1 protein / human papillomavirus type 11 L1 protein / human papillomavirus type 6 L1 protein - EMEA-003209-PIP02-23

Prevention of infection by human papillomavirus (HPV)

Day 60 discussion

Vaccines / Infectious Diseases

3.1.23. Ezetimibe / obicetrapib - EMEA-003514-PIP01-23

Treatment of mixed hyperlipidaemia

Day 30 discussion

Cardiovascular Diseases

3.1.24. Ezetimibe / obicetrapib - EMEA-003514-PIP02-23

Treatment of elevated cholesterol

Day 30 discussion

Cardiovascular Diseases

3.1.25. Ruxolitinib (phosphate) - EMEA-002618-PIP04-23

Treatment of *Prurigo nodularis*

Day 30 discussion

Dermatology

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

Sanofi B.V.; Treatment of Fabry disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.27. EMEA-003513-PIP01-23

Treatment of coeliac disease

Day 30 discussion

Gastroenterology-Hepatology

3.1.28. Mezagitamab - EMEA-003502-PIP01-23

Treatment of immune thrombocytopenia (ITP)

Day 30 discussion

Haematology-Hemostaseology

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

Prevention of hereditary angioedema attacks

Day 30 discussion

Haematology-Hemostaseology

3.1.30. EMEA-003503-PIP01-23

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.31. Nipocalimab - Orphan - EMEA-002559-PIP09-23

Janssen-Cilag International NV; Prevention of foetal and neonatal alloimmune thrombocytopenia

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.32. Zunsemetinib - EMEA-003511-PIP01-23

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.33. Humanised IgG1K monoclonal antibody against interferon beta - Orphan - EMEA-003089-PIP02-23

Pfizer Europe MA EEIG; Treatment of idiopathic inflammatory myopathy (ICD11 4A41)

Day 30 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.34. Contezolid - EMEA-003508-PIP01-23

Acute bacterial skin and skin structure infection (ABSSSI) / Moderate to severe diabetic foot infection (DFI) without concomitant osteomyelitis

Day 30 discussion

Infectious Diseases

3.1.35. Contezolid acefosamil - EMEA-003509-PIP01-23

Acute bacterial skin and skin structure infection (ABSSSI) / Moderate to severe diabetic foot infection (DFI) without concomitant osteomyelitis

Day 30 discussion

Infectious Diseases

3.1.36. Cladribine - EMEA-000383-PIP04-23

Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.1.37. Sonrotoclax - EMEA-003489-PIP02-23

Treatment of malignant solid tumours

Day 30 discussion

Oncology

3.1.38. Tifcemalimab - EMEA-003512-PIP01-23

Treatment of all conditions in the category of malignant neoplasms (except central nervous system, lymphoid and haematopoietic malignancies)

Day 30 discussion

Oncology

3.1.39. Tinengotinib - EMEA-003504-PIP01-23

Treatment of cholangiocarcinoma

Day 30 discussion

Oncology

3.1.40. Faricimab - EMEA-002817-PIP05-23

Treatment of choroidal neovascularisation secondary to pathologic myopia

Day 30 discussion

Ophthalmology

3.1.41. Multivalent, recombinant, N-terminal surface protein vaccine, containing the alpha-like proteins Rib, AlpC, Alp1, Alp 2/3 antigens of *Streptococcus agalactiae* - EMEA-003505-PIP01-23

Prevention of group B streptococcal invasive disease in infants through maternal immunisation

Day 30 discussion

Vaccines

3.1.42. Doruxapapogenum ralaplasmidum (pGX3024) - Orphan - EMEA-003506-PIP01-23

Inovio, Inc.; Treatment of papilloma viral infections

Day 30 discussion

Vaccines / Infectious Diseases

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. Belimumab - EMEA-C-000520-PIP02-13-M04

Glaxo Group Limited; Treatment of systemic lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.2. Bosutinib - EMEA-C-000727-PIP01-09-M07

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 30 discussion

Oncology

3.2.3. Isatuximab - EMEA-C-002205-PIP01-17-M04

Sanofi Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Tocilizumab - EMEA-000309-PIP07-21-M01

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.2. Etripamil - EMEA-002303-PIP01-17-M04

Milestone Pharmaceuticals, Inc.; Treatment of supraventricular arrhythmia

Day 30 discussion

Cardiovascular Diseases

3.3.3. Deucravacitinib - EMEA-002350-PIP01-18-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of psoriasis

Day 30 discussion

Dermatology

3.3.4. Tralokinumab - EMEA-001900-PIP02-17-M08

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.5. Regadenoson - EMEA-000410-PIP01-08-M06

GE Healthcare AS; Diagnosis of myocardial perfusion disturbances

Day 30 discussion

Diagnostic / Cardiovascular Diseases

3.3.6. Adeno-associated viral vector serotype 8 containing the human glucose-6-phosphatase gene (DTX401, pariglasgene brecaparvovec) - Orphan - EMEA-002734-PIP01-19-M01

Ultragenyx Germany GmbH; Treatment of glycogen storage disease type Ia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Tolvaptan - EMEA-001231-PIP02-13-M11

Otsuka Pharmaceutical Netherlands B.V.; Treatment of polycystic kidney disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

3.3.8. Crizanlizumab - Orphan - EMEA-002141-PIP01-17-M04

Novartis Europharm Limited; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.3.9. Mozafancogene autotemcel - Orphan - EMEA-002578-PIP01-19-M01

Rocket Pharmaceuticals, Inc; Treatment of Fanconi anaemia subtype A

Day 30 discussion

Haematology-Hemostaseology

3.3.10. Apremilast - EMEA-000715-PIP02-11-M07

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.11. Upadacitinib - EMEA-001741-PIP01-14-M07

AbbVie Ltd; Treatment of chronic idiopathic arthritis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.12. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M06

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 30 discussion

Infectious Diseases

3.3.13. Ivosidenib - Orphan - EMEA-002247-PIP03-17-M01

Les Laboratoires Servier; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology

3.3.14. Obecabtagene autoleucel - Orphan - EMEA-003171-PIP01-21-M01

Autolus GmbH; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Oncology

Note: Withdrawal request received on 9 November 2023

3.3.15. Odronextamab - Orphan - EMEA-003149-PIP01-21-M02

Regeneron Ireland DAC; Treatment of mature B cell malignancies

Day 30 discussion

Oncology

3.3.16. Bupivacaine - EMEA-000877-PIP03-17-M05

Pacira Ireland Ltd; Postsurgical analgesia

Day 30 discussion

Pain

3.3.17. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21-M01

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 30 discussion

Uro-nephrology

3.3.18. Dengue tetravalent vaccine (live, attenuated) - EMEA-001888-PIP01-15-M02

Takeda Vaccines, Inc.; Prevention of dengue fever

Day 30 discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 20 November 2023 for Nomination of Rapporteur and Peer reviewer

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

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

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. Palazestrant- EMEA-11-2023

Olema Pharmaceuticals Inc.; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant neoplasms / Treatment of advanced and/or metastatic hormone receptor (HR)-positive (HR+), human epidermal growth factor 2-neu (HER2)-negative (HER2−) breast cancer following CDK4/6 inhibitor therapy

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: none identified at this stage.

7. Discussion on the inclusion of 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 welcomed Liisa Saare, as the new alternate for Estonia.

The Chair announced that Jana Lass is the new member for Estonia replacing Irja Lutsar.

9.1.2. Vote by Proxy

None

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

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

Summary of Committee discussion:

The PDCO received an update on the SRLM meeting held in Madrid during the Spanish Presidency. ES member and alternate expressed their appreciation to the many participants for their important contributions to the discussion on all the topics addressed during the meeting. Presentations will be made available to PDCO.

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 October 2023, was presented to the PDCO members.

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

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

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

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

9.3.2. Formulation Working Group

PDCO member: Brian Aylward (*ad interim*)

Summary of Committee discussion:

The Chair of the Formulation Working Group (FWG) identified the products which will require FWG evaluation and discussion.

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

Summary of Committee discussion:

The draft Agenda of PCWP/HCPWP and all eligible meetings on 14 and 15 November 2023 was shared with the PDCO members for information.

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Summary of Committee discussion:

The presentation on the upcoming Innovation Task Force (ITF) meeting was shared with the PDCO members for information.

9.4. Cooperation within the EU regulatory network

No item

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

9.7.1. Draft PDCO Work Plan for 2024

PDCO Chair: Brian Aylward

Summary of Committee discussion:

The draft PDCO work plan was presented to the Committee and topics from members were proposed.

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

No item

10. Any other business

10.1. Quick tour of the clinical trial approval process under Clinical Trial Regulation (CTR)

PDCO member: Anette Solli Karlsen

Summary of Committee discussion:

Features of the clinical trial regulation were presented, including implementation and transition period, the Clinical trial information system (CTIS), requirements for the clinical trial application (CTA) under CTR, how the clinical trial application is assessed under CTR and different member state roles. An introduction to how reference member state selections are selected were provided.
Following the presentation, the implication of the implementation of the CTR were discussed, including the intended simplification of the application and approval process, the prolonged application time and how the implementation and the requirement to the application under CTR has affected the ability of non-commercial sponsors to conduct clinical trials in EU.

10.2. Feedback from 12th Paediatric Oncology Strategy Forum

PDCO member: Sylvie Benchetrit

Summary of Committee discussion:

The PDCO was informed about the Forum's discussions and conclusions.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The group discussed emerging issues of ongoing oncological procedures.

11.2. Neonatology

Summary of Committee discussion:

Discussion of topics related to the revision of the neonatal guideline.

11.3. HIV

Summary of Committee discussion:

The group discussed recent interactions with WHO on paediatric development in the HIV therapeutic area.

11.4. 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 07-10 November 2023 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 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	
Anne Paavola	Alternate*	Finland	No interests declared	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
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 participation in discussion, final deliberations and voting on:	3.3.12. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M06

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
Olivier Moes	Alternate*	Luxembourg	No interests declared	
Herbert Lenicker	Alternate*	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No restrictions applicable to this meeting	
Maaike Van Dartel	Alternate	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No restrictions applicable to this meeting	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Peter Szitanyi	Alternate	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member*	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Sara Vennberg	Member	Sweden	No interests declared	
David Khan	Alternate	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	2.3.15. Fully human IgG1 RB-1 YTE anti-RSV F monoclonal antibody - EMEA-002755-PIP01-19-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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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	
Maria Luttgen	Expert	Sweden	No restrictions applicable to this meeting	
Emmely de Vries	Expert	Netherlands	No interests declared	
Olga Kholmanskikh	Expert	Belgium	No interests declared	
Meeting run with support from relevant EMA staff				

Experts were evaluated against the agenda topics or activities they participated in.

13. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Paediatric investigation plan (PIP) (*section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs*)
A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, when it is safe to do so, to support the authorisation of a medicine for children. Pharmaceutical companies submit proposals for PIPs to the European Medicines Agency's Paediatric Committee (PDCO). This Committee is responsible for agreeing or refusing the plan.

Compliance checks (*section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check*)
A compliance check may be necessary before any application for marketing authorisation (even for an adult indication) can be considered valid, if there was no deferral for at least one of the studies agreed in the PIP, or after the due date of initiation or completion of a study/measure. The same applies to some regulatory applications for authorised products, as described above.

Modification of an Agreed Paediatric Investigation Plan (*section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP*)
The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.
In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

Class waiver (*section 6 Discussion on the applicability of class waiver*)
As some diseases do not affect children (for example Parkinson's disease), the development of medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived**.** For more information on the classes of diseases subject to waivers, see class waivers.

Annual reports on deferrals (*section 8*)
If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

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