8 September 2023
EMA/PDCO/352213/2023
Human Medicines Division

Paediatric Committee (PDCO)

Draft Minutes for the meeting on 18-21 July 2023

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 Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

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

Note on access to documents

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

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1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

The Chair opened the meeting by welcoming all participants. The meeting was held 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 welcomed the new member and thanked the departing member for his contributions to the Committee.

1.2. Adoption of agenda

The agenda for 18-21 July meeting was adopted with amendments.

1.3. Adoption of the minutes

The minutes for 20-23 June 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. Dexmedetomidine - EMEA-003283-PIP01-22

Cessatech A/S; Sedation

Day 120 opinion

Anaesthesiology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the July 2023 plenary meeting, an application for a paediatric investigation plan for dexmedetomidine for sedation.
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.

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

Timber Pharmaceuticals LLC; Treatment of congenital ichthyosis

Day 120 opinion

Dermatology

Note: Withdrawal request received on 14 July 2023

2.1.3. Upadacitinib - EMEA-001741-PIP07-22

AbbVie Ltd; Treatment of vitiligo

Day 120 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 the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from 6 years to less than 18 years of age, in the condition of treatment of vitiligo was adopted. The PDCO agreed on a waiver in a subset of children 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(s). The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

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

Sanofi B.V.; Treatment of achondroplasia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

During its plenary in July 2023, the PDCO discussed at Day 120 the paediatric investigation plan with a deferral for humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 (SAR442501) in the treatment of achondroplasia.

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.

2.1.5. Recombinant human tissue nonspecific alkaline phosphatase (TNSALP) fragment crystallizable (Fc) deca aspartate fusion protein (ALXN1850) - EMEA-003343-PIP01-22

Alexion Europe SAS; Treatment of hypophosphatasia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Between Day 90 and Day 120, the applicant revised the draft PIP opinion and agreed with the PDCO's request for a study in children below 2 years of age to evaluate pharmacokinetics and pharmacodynamics. The Committee adopted a positive opinion on this PIP for ALXN1850 (solution for subcutaneous injection) for the treatment of hypophosphatasia, covering the whole paediatric age group from birth to less than 18 years of age. The PIP includes one non-clinical study. The clinical development consists of 4 paediatric clinical studies, which are part of an extrapolation plan of efficacy data from adults to the paediatric population. Moreover, the PIP includes a modelling and simulation study to support dose selection. A deferral has been granted for one or more studies contained in the PIP.

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

Applied Therapeutics, Inc; Treatment of galactosaemia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Ophthalmology / Neurology

Note: Withdrawal request received on 20 July 2023

2.1.7. Derivative of 6-[2-(pyridin-2-yl)phenoxy]methyl}-1,2,3,4-tetrahydroisoquinoline - EMEA-003002-PIP03-22

Boehringer Ingelheim International GmbH; Treatment of portal hypertension

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 PIP for the proposed medicine for children from 6 years to less than 18 years of age, in the condition of treatment of portal hypertension was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 6 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for

one or more measures contained in the paediatric investigation plan.

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

Roche Registration GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO adopted a positive opinion on Day 120 for obinutuzumab for treatment of glomerulonephritis and nephrotic syndrome for paediatric patients from 2 years to less than 18 years of age with a deferral for the completion of the study contained in the PIP.
A waiver was granted for paediatric population from birth to less than 2 years of age on the grounds that the specific medicinal product is likely to be unsafe.

2.1.9. Funobactam - EMEA-003326-PIP01-22

Evopoint Biosciences USA, Inc.; Treatment of infections caused by gram-negative organisms (in combination with imipenem and cilastatin)

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO adopted a positive opinion for this PIP for funobactam (XNW4107) for the treatment of infections caused by gram-negative organisms (in combination with imipenem and cilastatin) in paediatric patients from birth to less than 18 years of age. The PIP includes a quality-related study (development of an intravenous age-appropriate dosage form), a non-clinical toxicity study, an open-label single-dose pharmacokinetics, safety and tolerability study as well as a multiple-dose, active-controlled safety, pharmacokinetics and efficacy study. Moreover, the PIP includes a modelling and simulation study. The two clinical studies and the modelling and simulation study are part of an extrapolation plan. A deferral for one or more measures contained in the paediatric investigation plan has been granted.

2.1.10. Trimodulin - EMEA-002883-PIP02-22

Biotest AG; Treatment of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Neonatology - Paediatric Intensive Care / Infectious 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 PIP for the proposed medicine for the paediatric population from birth to less than 18 years of age in the condition of treatment of

coronavirus disease 2019 (COVID-19) was adopted. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

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

Calico Life Sciences LLC; Treatment of vanishing white matter disease

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the July 2023 plenary meeting, an application for a paediatric investigation plan with a deferral for sodium({(2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2-yl}oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) (ABBV-CLS-7262) for treatment of vanishing white matter disease.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.
The PDCO adopted a positive opinion on a paediatric investigation plan with a deferral.

2.1.12. Vesleteplirsen - EMEA-003305-PIP01-22

Sarepta Therapeutics Ireland; Treatment of Duchenne muscular dystrophy

Day 120 opinion

Neurology

Summary of Committee discussion:

In July 2023 the PDCO noted that the applicant had addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for vesleteplirsen, in the condition of treatment of Duchenne muscular dystrophy was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of the paediatric population from birth to less than 6 months of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.13. Retatrutide - EMEA-003258-PIP01-22

Treatment of obesity

Day 120 opinion

Other

Note: Withdrawal request received on 11 July 2023

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

Humacyte Global, Inc. (previously known as Humacyte, Inc.); Treatment of vascular injuries

Day 120 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for an acellular tubular graft composed of human collagen types I and III and other extracellular matrix proteins, including fibronectin and vitronectin, for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of vascular injuries, 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.

2.1.15. Dexpramipexole dihydrochloride monohydrate - EMEA-003328-PIP01-22

Areteia Therapeutics; Treatment of asthma

Day 120 opinion

Pneumology - Allergology

Summary of Committee discussion:

In the written response the applicant addressed the remaining issues raised by the Committee. The planned oral explanation was cancelled.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion on a PIP for dexpramipexole in children from 1 year to less than 18 years of age for treatment of asthma was adopted by the PDCO.
The Paediatric Committee granted a deferral for completion of the paediatric clinical trial and modelling & simulation studies. A waiver was granted in children below 1 year of age on the grounds that clinical studies with dexpramipexole cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset.

2.1.16. Substituted 3,7-dihydro-1H-purine-2,6-dione - EMEA-003319-PIP02-22

Boehringer Ingelheim International GmbH; Treatment of major depressive disorder (MDD)

Day 120 opinion

Psychiatry

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for the paediatric population from 12 years to less than 18 years of age, in the condition treatment of major depressive disorder was adopted by consensus.
The PDCO agreed on a waiver from birth to less than 7 years of age 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 from 7 years 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.
The PDCO granted a deferral for the completion of this PIP.

2.1.17. Substituted 3,7-dihydro-1H-purine-2,6-dione - EMEA-003319-PIP03-22

Boehringer Ingelheim International GmbH; Treatment of post-traumatic stress disorder (PTSD)

Day 120 opinion

Psychiatry

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for the paediatric population from 12 years to less than 18 years of age, in the condition of treatment of posttraumatic stress disorder was adopted by consensus.
The PDCO agreed on a waiver from birth to less than 2 years of age 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 from 2 years to less than 7 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.
The PDCO granted a deferral for the completion of this PIP.

2.1.18. Obicetrapib - EMEA-003438-PIP01-23

NewAmsterdam Pharma BV; Treatment of mixed dyslipidaemia

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 obicetrapib for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of mixed dyslipidaemia.
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.

2.1.19. Ramipril / nebivolol (hydrochloride) - EMEA-003430-PIP01-23

Menarini Ricerche S.p.A.; Treatment of hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

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

2.1.20. Pemafibrate - EMEA-001573-PIP03-23

Kowa Pharmaceutical Europe AG; Treatment of primary biliary cholangitis

Day 60 opinion

Gastroenterology-Hepatology

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 the proposed product for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of primary biliary cholangitis on the grounds that the disease occurs only in adult populations.

2.1.21. Abemaciclib - EMEA-002342-PIP05-23

Eli Lilly and Company Limited; Treatment of prostate cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2023 plenary meeting, a request for a product-specific waiver for abemaciclib for the treatment of prostate cancer on the grounds that this cancer only occurs in adults.
The PDCO confirmed all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 for abemaciclib for the treatment of prostate cancer on the grounds that this disease occurs only in adult populations.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all

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

2.1.22. Binimetinib - EMEA-001454-PIP06-23

Pierre Fabre Médicament; 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 binimetinib for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung cancer, based on the ground that the diseases do not occur in children. Since the agreed waiver ground is disease not occurring, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.23. Encorafenib - EMEA-001588-PIP04-23

Pierre Fabre Médicament; 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, PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for encorafenib for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung cancer, based on the ground that the diseases do not occur in children. Since the agreed waiver ground is disease not occurring, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

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

Astellas Pharma Europe B.V.; Treatment of head and neck epithelial malignant neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver.
The PDCO recommended granting a waiver for enfortumab vedotin for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of head and neck epithelial malignant neoplasms, based on the ground of lack of significant therapeutic benefit as studies are not feasible.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.25. Ivonescimab - EMEA-003378-PIP01-23

Summit Therapeutics Sub Inc.; 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 ivonescimab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung cancer, based on the ground that the diseases do not occur in children.
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.
The PDCO stressed the potential of this product, based on its mechanism of action, being able to offer significant therapeutic benefit in areas of high unmet medical needs and encouraged the applicant to further explore development opportunities, including the use of methodologies to define relevance of the targets, e.g. by means of additional pre-clinical work.

2.1.26. Lerociclib (dihydrochloride) - EMEA-003436-PIP01-23

EQRx International, Inc.; Treatment of endometrial carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2023 plenary meeting, a request for a product-specific waiver for lerociclib (dihydrochloride) for the treatment of endometrial cancer on the grounds that the condition does not occur in children.
The PDCO confirmed all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 for lerociclib (dihydrochloride) for the 'treatment of endometrial carcinoma' on the grounds that this disease occurs only in adult populations.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.27. Modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens (V940, mRNA-4157) - EMEA-003434-PIP01-23

Merck Sharp & Dohme (Europe) Inc.; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition of treatment of lung cancer on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

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

Treatment in allogeneic haematopoietic stem cell transplantation

Day 60 opinion

Oncology / Haematology-Hemostaseology

Note: Withdrawal request received on 13 July 2023

2.1.29. Clotrimazole - EMEA-003431-PIP01-23

Laboratorios Salvat, S.A.; Treatment of otitis externa

Day 60 opinion

Oto-rhino-laryngology

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 clotrimazole for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'treatment of otitis externa' on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2.1.30. 3-[2-(Dimethylamino)ethyl]-1H-indol-4-yl dihydrogen phosphate - EMEA-002990-PIP02-23

COMPASS Pathfinder Limited; Treatment of major depressive disorder

Day 60 opinion

Psychiatry

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 (3[2(-dimethylamino)ethyl]-1H-indol-4-yl dihydrogen phosphate) for the paediatric population from birth to less than 7 years of age for the condition of treatment of major depressive disorder 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 the paediatric population from 7 years to less than 18 years of age for the condition treatment of major depressive disorder on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.31. Ravulizumab (ALXN1210) - EMEA-001943-PIP06-23

Alexion Europe SAS; Prevention of kidney injury in high-risk patients with chronic kidney disease undergoing cardiopulmonary bypass

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for ravulizumab for all subsets of the paediatric population from birth to less than 18 years of age for the condition of 'prevention of kidney injury in high-risk patients with chronic kidney disease undergoing cardiopulmonary bypass' on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2.1.32. 5-{4-[(7-ethyl-6-oxo-5,6-dihydro-1,5-naphthyridin-3-yl)methyl]-1-piperazinyl}-N-methyl-2-pyridinecarboxamide - EMEA-003447-PIP01-23

AstraZeneca AB; Treatment of prostate cancer / Treatment of breast cancer

Day 30 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 5-{4-[(7-ethyl-6-oxo-5,6-dihydro-1,5-naphthyridin-3-yl)methyl]-1-piperazinyl}-N-methyl-2-pyridinecarboxamide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of breast cancer and treatment prostate 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.

2.1.33. Teprotumumab - EMEA-001973-PIP02-23

Horizon Therapeutics Ireland Designated Activity Company; Treatment of thyroid eye disease

Day 30 opinion

Ophthalmology

Summary of Committee discussion:

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

2.1.34. Concentrate of proteolytic enzymes enriched in bromelain - Orphan - EMEA-000142-PIP03-23

MediWound Germany GmbH; Treatment of venous leg ulcers / Treatment of diabetic foot ulcers

Day 30 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for concentrate of proteolytic enzymes enriched in bromelain for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of diabetic foot ulcers and treatment of venous leg ulcers.
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.

2.1.35. Rosuvastatin / ezetimibe - EMEA-003444-PIP01-23

Prevention of cardiovascular events

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

2.2. Opinions on Compliance Check

2.2.1. Birch bark extract - EMEA-C-001299-PIP03-17-M02

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 30 opinion

Dermatology

Summary of Committee discussion:

The PDCO adopted on 21 July 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/0243/2023) of 16 June 2023.

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

AstraZeneca AB; Treatment of type 2 diabetes

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

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

- EMEA-C1-000200-PIP01-08-M03

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/0478/2022) of 2 December 2022.

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

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

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

- EMEA-C1-001501-PIP04-19-M01
- EMEA-C2-001501-PIP04-19-M01

The PDCO adopted on 21 July 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/0541/2022) of 30 December 2022.

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

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

Day 60 opinion

Ophthalmology

Summary of Committee discussion:

The PDCO discussed the additional information provided by the applicant and concluded that compliance for Study 1 could be confirmed.
The PDCO adopted on 21 July 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/0066/2022) of 11 March 2022.

2.2.5. Isavuconazonium (sulfate) - EMEA-C-001301-PIP02-12-M04

Basilea Pharmaceutica Deutschland GmbH; Treatment of invasive aspergillosis

Day 30 opinion

Infectious Diseases

Summary of Committee discussion:

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

- EMEA-C1-001301-PIP02-12-M03

The PDCO adopted on 21 July 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/0479/2021) of 3 December 2021.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

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

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

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, 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/0199/2020 of 20 May 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

CUTISS AG; Treatment of burns

Day 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, the PDCO considered that the proposed changes (such as timeline changes and minor changes to sample size and inclusion criteria) 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/0364/2020 of 9 September 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

UCB Biopharma SRL; Treatment of psoriasis

Day 60 opinion

Dermatology

Note: Withdrawal request received on 19 July 2023

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

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

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Within this modification, the applicant requested to delete Study 2 as no adolescents were recruited into the adult/adolescent study and proposed some changes to the study design of Study 3 which is a safety and efficacy study in children from 2 years to less than 18 years of age with primary sclerosing cholangitis without or with features of auto-immune hepatitis (AIH). A delay of completion of the PIP was also agreed.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0050/2020 of 29 January 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

AbbVie Ltd; Treatment of Crohn's disease

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

The modification request concerned elements of Study 3 which is an open-label study in paediatric patients with Crohn's disease (CD) to evaluate the efficacy, safety, and pharmacokinetics (PK) of risankizumab.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0230/2018 of 3 August 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Within this modification the applicant requested to change the date of completion of Studies 3, 4, 5 and 6. The date of completion of the PIP is brought forward by 1 year.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0401/2022 of 9 September 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

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, the PDCO considered that the proposed changes could be accepted. Several details of Study 3 were modified and a new study (Study 6, a compassionate use program) was added to this PIP.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0371/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

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 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/0403/2020 of 22 October 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

CSL Behring GmbH; Treatment of haemophilia B

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, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0380/2022 of 9 September 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Ravulizumab - Orphan - EMEA-002077-PIP01-16-M06

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

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, 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/0191/2022 of 10 June 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

In the written response the applicant addressed most of the remaining issues raised by the Committee.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that some of the proposed changes could be accepted. A waiver was granted for 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 as clinical studies are not feasible.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0266/2022 of 27 July 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes (eligibility criteria, wording of endpoints of the 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/0435/2022 of 28 October 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Roche Registration GmbH; Treatment of influenza / Prevention of influenza

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0098/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that 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/0067/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Merck Sharp & Dohme (Europe), Inc.; Treatment of acute bacterial skin and skin structure 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, 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/0112/2023 of 13 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Gilead Sciences International Ltd.; Treatment of chronic viral hepatitis B

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Within this modification, the applicant requested to delay the completion of Studies 2 (Development of an age appropriate oral non-tablet formulation) and 4 (Double-blind, placebo-controlled, pharmacokinetic, efficacy, safety, tolerability and antiviral activity study in children from 2 years to less than 18 years of age).
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0269/2021 of 9 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Teva GmbH; Prevention of migraine headaches

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0411/2019 of 4 December 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

UCB Pharma S.A; Treatment of myasthenia gravis

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that some of the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0410/2022 of 29 September 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 60 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0279/2022 of 10 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 30, during the July 2023 plenary meeting, a request for modification for brexucabtagene autoleucel for the treatment of acute lymphoblastic leukaemia.
The PDCO confirmed all the conclusions reached at Day 30.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be acceptable. Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0142/2020 of 18 April 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0237/2021 of 14 June 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

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

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. Study 3 and 4 were modified and two new studies (6 and 7) were added to the PIP. Also, a modification was made in the Study 5 (Modelling and Simulation).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0125/2023 of 18 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Ravulizumab - Orphan - EMEA-001943-PIP01-16-M08

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes (timeline changes) could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0188/2022 of 10 June 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0152/2023 of 24 April 2023).

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

2.3.25. Repotrectinib - EMEA-002635-PIP02-21-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic neoplasms)

Day 30 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0150/2023 of 26 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. Semaglutide - EMEA-001441-PIP07-21-M01

Novo Nordisk A/S; Treatment of obesity

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

This is a regulatory modification to link this PIP decision to other PIP decisions for this active substance. No changes to the scientific part of the PIP are proposed.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0481/2022 of 2 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.27. Guselkumab - EMEA-001523-PIP02-14-M03

Janssen-Cilag International NV; Treatment of psoriasis

Day 30 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The agreed PIP includes the following studies:
Study 1: Development of an age-appropriate paediatric presentation, a single use VarioJect manual injector (pre-filled pen).
Study 2: Active-controlled, double blind study to evaluate the PK, safety and efficacy of guselkumab in paediatric patients with plaque psoriasis (CNTO1959PSO3011).
Study 3: Extrapolation to select the paediatric study dose regimen.
Study 4: Extrapolation/Interpolation for exposure-response analysis.
The applicant within this modification procedure is requesting to delay the completion of Studies 1, 2 and 4.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0129/2018 of 6 April 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Iclepertin - EMEA-002653-PIP01-19-M01

Boehringer Ingelheim International GmbH; Treatment of schizophrenia

Day 30 opinion

Psychiatry

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0473/2020 of 1 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.29. Botaretigene sparoparvovec - Orphan - EMEA-002827-PIP01-20-M02

Janssen-Cilag International NV; Treatment of retinitis pigmentosa

Day 30 opinion

Ophthalmology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0115/2023 of 13 April 2023). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.30. Vedolizumab - EMEA-000645-PIP04-20-M02

Takeda Pharma A/S; Treatment of pouchitis

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, 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/0508/2022 of 2 December 2022). 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. Erenumab - EMEA-C3-001664-PIP02-15-M05

Novartis Europharm Limited; Prevention of migraine headaches

Day 30 letter

Pain / Neurology

2.7.2. Odronextamab - EMEA-C1-003149-PIP01-21-M01

Regeneron Ireland DAC; Treatment of mature B-cell malignancies

Day 30 letter

Oncology

2.7.3. Iptacopan - EMEA-C1-002705-PIP01-19-M01

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Day 30 letter

Other / Uro-nephrology

2.7.4. Brivaracetam - EMEA-C1-000332-PIP02-17-M04

UCB Pharma S.A.; Treatment of neonatal seizures

Day 30 letter

Neurology

2.7.5. Cariprazine hydrochloride - EMEA-C1-001652-PIP01-14-M05

Gedeon Richter Plc.; Treatment of schizophrenia

Day 30 letter

Psychiatry

2.7.6. Marstacimab - EMEA-C2-002285-PIP02-19-M02

Pfizer Europe MA EEIG; Treatment of congenital haemophilia A / Treatment of congenital haemophilia B

Day 30 letter

Haematology-Hemostaseology

2.7.7. Garadacimab - EMEA-C1-002726-PIP01-19-M03

CSL Behring GmbH; Prevention of hereditary angioedema attacks

Day 30 letter

Immunology-Rheumatology-Transplantation

2.7.8. Cabotegravir / cabotegravir sodium - EMEA-C1-001418-PIP01-13-M05

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

Day 30 letter

Infectious Diseases

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. A self-complementary adeno-associated virus [AAV] serotype 8 virus particle encoding the human ornithine transcarbamylase [OTC] gene sequence - Orphan - EMEA-002830-PIP01-20

Ultragenyx Germany GmbH; Treatment of ornithine transcarbamylase deficiency

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. A nonreplicating, recombinant adeno-associated virus (AAV) serotype 9 (AAV9) gene transfer vector that contains a modified human ATP7B coding sequence - Orphan - EMEA-003131-PIP01-21

Ultragenyx Germany GmbH; Treatment of Wilson disease

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Fazirsiran - Orphan - EMEA-003355-PIP01-22

Takeda Pharma A/S; Treatment of alpha-1 antitrypsin deficiency-associated liver disease

Day 90 discussion

Gastroenterology-Hepatology

3.1.4. Inhibitor of receptor-interacting protein kinase 1 - EMEA-003356-PIP01-22

Treatment of ulcerative colitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.5. Tezepelumab - EMEA-001613-PIP03-21

Treatment of eosinophilic esophagitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.6. EMEA-003090-PIP02-22

Treatment of hereditary angioedema

Day 90 discussion

Haematology-Hemostaseology

3.1.7. Ciraparantag - EMEA-003321-PIP01-22

Treatment of FXa inhibitor-associated haemorrhage / Prevention of FXa inhibitor-associated haemorrhage

Day 90 discussion

Haematology-Hemostaseology

3.1.8. Luspatercept - Orphan - EMEA-001521-PIP03-22

Bristol-Myers Squibb Pharma EEIG; Treatment of alpha-thalassaemia

Day 90 discussion

Haematology-Hemostaseology

3.1.9. Enpatoran - EMEA-003342-PIP02-22

Treatment of systemic lupus erythematosus

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.10. Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate - Orphan - EMEA-003025-PIP03-23

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

Day 90 discussion

Immunology-Rheumatology-Transplantation / Oncology / Haematology-Hemostaseology

3.1.11. Ensitrelvir - EMEA-003192-PIP01-22

Treatment of coronavirus disease (COVID-19)

Day 90 discussion

Infectious Diseases

3.1.12. Ruzotolimod - EMEA-003363-PIP01-22

Treatment of chronic hepatitis B

Day 90 discussion

Infectious Diseases

3.1.13. Xalnesiran - EMEA-003362-PIP01-22

Treatment of chronic hepatitis B

Day 90 discussion

Infectious Diseases

3.1.14. Rozanolixizumab - EMEA-002681-PIP03-21

Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 90 discussion

Neurology

3.1.15. Nemvaleukin alfa - EMEA-003357-PIP01-22

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

Day 90 discussion

Oncology

3.1.16. Pembrolizumab / vibostolimab - EMEA-003063-PIP03-22

Treatment of melanoma

Day 90 discussion

Oncology

3.1.17. Uproleselan - Orphan - EMEA-003307-PIP01-22

GlycoMimetics, Inc.; Treatment of acute myeloid leukaemia

Day 90 discussion

Oncology

3.1.18. Pabinafusp alfa - Orphan - EMEA-003033-PIP02-22

JCR Pharmaceuticals Co., Ltd.; Treatment of mucopolysaccharidosis II (Hunter´s syndrome)

Day 90 discussion

Other

3.1.19. EMEA-003347-PIP01-22

Treatment of glomerulonephritis and nephrotic syndrome

Day 90 discussion

Uro-nephrology

3.1.20. Inaxaplin - Orphan - EMEA-003368-PIP01-22

Vertex Pharmaceuticals (Ireland) Limited; Treatment of APOL1-mediated kidney disease

Day 90 discussion

Uro-nephrology

3.1.21. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / outer membrane vesicles (OMV) from *N. meningitidis* / recombinant *Neisseria meningitidis* group B fHbp 2-3-1.13NB fusion protein / recombinant *Neisseria meningitis* group B protein 961c / recombinant *Neisseria meningitis* group B protein 287-953 / recombinant *Neisseria meningitis* group B protein 936-741 - EMEA-003359-PIP01-22

Prevention of meningococcal disease

Day 90 discussion

Vaccines

3.1.22. *Neisseria meningitidis* serogroup B protein-based active substance / recombinant *Neisseria meningitidis* serogroup B protein 3 / recombinant *Neisseria meningitidis* serogroup B protein 2 / recombinant *Neisseria meningitidis* serogroup B protein 1 / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-003379-PIP01-22

Prevention of meningococcal disease

Day 90 discussion

Vaccines

3.1.23. Obicetrapib - EMEA-003438-PIP02-23

Treatment of elevated cholesterol

Day 60 discussion

Cardiovascular Diseases

3.1.24. 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 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.27. Dupilumab - EMEA-001501-PIP12-23

Treatment of eosinophilic gastroenteritis

Day 60 discussion

Gastroenterology-Hepatology

3.1.28. Tarperprumig - EMEA-003432-PIP01-23

Treatment of patients with sickle cell disease (SCD)

Day 60 discussion

Haematology-Hemostaseology

3.1.29. Amiodarone - EMEA-003428-PIP01-23

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

Day 60 discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

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

Marinus Pharmaceuticals Inc.; Treatment of tuberous sclerosis complex

Day 60 discussion

Neurology

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

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

Day 60 discussion

Neurology

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

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

Day 60 discussion

Other

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

Treatment of anaemias due to chronic kidney disorders

Day 60 discussion

Uro-nephrology

3.1.34. Amlodipine / rosuvastatin - EMEA-003446-PIP01-23

Treatment of angina and dyslipidaemia / Treatment of essential hypertension in patients who are estimated to have a high risk for a first cardiovascular event / Treatment of concomitant hypertension and dyslipidaemia

Day 30 discussion

Cardiovascular Diseases

3.1.35. Modified, recombinant version of the human myeloid-derived growth factor - EMEA-003449-PIP01-23

Treatment of myocardial infarction

Day 30 discussion

Cardiovascular Diseases

3.1.36. Oral inhibitor of PCSK9 - EMEA-003453-PIP01-23

Treatment of hypercholesterolemia

Day 30 discussion

Cardiovascular Diseases

3.1.37. Upadacitinib - EMEA-001741-PIP10-23

Treatment of alopecia areata

Day 30 discussion

Dermatology

3.1.38. GIPR antagonist/GLP-1R agonist - EMEA-003439-PIP02-23

Treatment of obesity

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.39. Plecanatide - EMEA-003441-PIP01-23

Treatment of irritable bowel syndrome with constipation / Treatment of chronic idiopatic constipation

Day 30 discussion

Gastroenterology-Hepatology

3.1.40. EMEA-003456-PIP01-23

Treatment of myelofibrosis

Day 30 discussion

Haematology-Hemostaseology

3.1.41. Hydroxycarbamide - EMEA-003388-PIP01-23

Prevention of sickle cell disease crises

Day 30 discussion

Haematology-Hemostaseology

3.1.42. Ianalumab - EMEA-002338-PIP04-23

Treatment of autoimmune haemolytic anaemia

Day 30 discussion

Haematology-Hemostaseology

3.1.43. Reparixin - Orphan - EMEA-001693-PIP06-23

Dompé farmaceutici S.p.A.; Treatment of infectious pneumonia acquired in the community, excluding coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.1.44. Ganaxolone - EMEA-002341-PIP03-23

Treatment of refractory status epilepticus

Day 30 discussion

Neurology

3.1.45. Recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA - Orphan - EMEA-003459-PIP01-23

Myrtelle, Inc.; Treatment of Canavan disease

Day 30 discussion

Neurology

3.1.46. EMEA-003455-PIP01-23

Treatment of lung cancer

Day 30 discussion

Oncology

3.1.47. Autologous patient-derived CD4+ and CD8+ T cells expressing a chimeric antigen receptor specific for claudin 6 - EMEA-003377-PIP01-23

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

Day 30 discussion

Oncology

3.1.48. Belrestotug - EMEA-003452-PIP01-23

Treatment of lung cancer

Day 30 discussion

Oncology

3.1.49. Bezuclastinib - EMEA-003445-PIP01-23

Treatment of mastocytosis

Day 30 discussion

Oncology

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

Treatment of HER2 expressing tumours / Treatment of solid tumours

Day 30 discussion

Oncology

3.1.51. Humanised IgG1 monoclonal antibody against TROP2, conjugated to a topoisomerase I inhibitor belotecan-derivative - EMEA-003461-PIP01-23

Treatment of lung cancer / Treatment of ovarian cancer / Treatment of cervical cancer / Treatment of gastric cancer / Treatment of breast cancer / Treatment of endometrial cancer

Day 30 discussion

Oncology

3.1.52. Liposome-formulated messenger ribonucleic-acid vaccine encoding the chimeric antigen-receptor target antigen claudin 6 - EMEA-003464-PIP01-23

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

Day 30 discussion

Oncology

3.1.53. Obecabtagene autoleucel - Orphan - EMEA-003171-PIP02-23

Autolus GmbH; Treatment of non-Hodgkin lymphoma

Day 30 discussion

Oncology

3.1.54. Tiragolumab - EMEA-002721-PIP04-23

Treatment of hepatocellular carcinoma

Day 30 discussion

Oncology

3.1.55. Unesbulin - Orphan - EMEA-003297-PIP02-23

PTC Therapeutics International; Treatment of soft tissue sarcoma

Day 30 discussion

Oncology

3.1.56. Valemetostat tosilate - Orphan - EMEA-003256-PIP02-23

Daiichi Sankyo Europe GmbH; Treatment of mature T-cell neoplasms

Day 30 discussion

Oncology

3.1.57. Zanidatamab - Orphan - EMEA-003450-PIP01-23

Jazz Pharmaceuticals Ireland Limited; Treatment of biliary tract cancer (BTC)

Day 30 discussion

Oncology

3.1.58. Allogeneic faecal microbiota, pooled - Orphan - EMEA-003435-PIP02-23

MaaT Pharma; Treatment of graft-versus-host disease

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.59. Clobetasol - EMEA-003458-PIP01-23

Treatment of inflammation and pain associated with ocular surgery

Day 30 discussion

Ophthalmology

3.1.60. Laruparetigene zovaparvovec - Orphan - EMEA-003457-PIP01-23

FGK Representative Service GMBH; Treatment of X-linked retinitis pigmentosa (XLRP)

Day 30 discussion

Ophthalmology

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

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

Day 30 discussion

Other

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

Fulcrum Therapeutics, Inc.; Treatment of facioscapulohumeral muscular dystrophy

Day 30 discussion

Other

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

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

Day 30 discussion

Other

3.1.64. Sisunatovir - EMEA-002529-PIP02-23

Treatment of respiratory tract disease caused by respiratory syncytial virus (RSV) infection

Day 30 discussion

Other

3.1.65. Imlifidase - EMEA-002183-PIP02-23

Treatment of patients with Duchenne muscular dystrophy (DMD) and pre-existing antibodies to the AAV vector to enable gene therapy

Day 30 discussion

Other / Uro-nephrology

3.1.66. Mometasone - EMEA-003454-PIP01-23

Treatment of chronic rhinosinusitis (CRS)

Day 30 discussion

Oto-rhino-laryngology

3.1.67. EMEA-003451-PIP01-23

Treatment of bronchiectasis

Day 30 discussion

Pneumology - Allergology

3.1.68. *Borrelia* outer surface protein A (OspA) serotypes (ST1-6) lipidated, fusion protein vaccine - EMEA-003130-PIP02-23

Prevention of Lyme disease

Day 30 discussion

Vaccines

3.1.69. Pertussis filamentous haemagglutinin (FHA) / Genetically detoxified pertussis toxin (PTgen) - EMEA-003442-PIP01-23

Prevention of pertussis disease

Day 30 discussion

Vaccines

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. Abacavir (ABC) / lamivudine (3TC) / dolutegravir (DTG) - EMEA-C-001219-PIP01-11-M06

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

Day 30 discussion

Infectious Diseases

3.2.2. Leriglitazone - EMEA-C-002106-PIP01-16-M02

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 30 discussion

Neurology

3.2.3. Molgramostim - EMEA-C1-002282-PIP01-17-M01

Savara ApS; Treatment of pulmonary alveolar proteinosis

Day 30 discussion

Pneumology - Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Dupilumab - EMEA-001501-PIP02-13-M08

Sanofi Winthrop Industrie; Treatment of asthma

Day 30 discussion

Dermatology

3.3.2. Fitusiran - Orphan - EMEA-001855-PIP01-15-M05

Genzyme Europe B.V.; Treatment of congenital haemophilia A / Treatment of congenital haemophilia B

Day 30 discussion

Haematology-Hemostaseology

3.3.3. Vonicog alfa - EMEA-001164-PIP01-11-M07

Baxalta Innovations GmBH; Treatment of Von Willebrand disease

Day 30 discussion

Haematology-Hemostaseology

3.3.4. BNT162b2 / tozinameran / famtozinameran / riltozinameran - EMEA-002861-PIP02-20-M06

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

Day 30 discussion

Infectious Diseases

3.3.5. Eravacycline - EMEA-001555-PIP01-13-M05

PAION Deutschland GmbH; Treatment of complicated intra-abdominal infection

Day 30 discussion

Infectious Diseases

3.3.6. Remdesivir - EMEA-002826-PIP01-20-M04

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

Day 30 discussion

Infectious Diseases

3.3.7. Eptinezumab - EMEA-002243-PIP01-17-M04

H. Lundbeck A/S; Prevention of migraine headaches

Day 30 discussion

Neurology

3.3.8. Nipocalimab (Anti-neonatal Fc receptor human monoclonal antibody) - EMEA-002559-PIP02-19-M01

Janssen-Cilag International NV; Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.3.9. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M05

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 30 discussion

Neurology

3.3.10. Peginterferon beta-1a - EMEA-001129-PIP01-11-M06

Biogen Idec Ltd; Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.3.11. Carfilzomib - Orphan - EMEA-001806-PIP04-19-M02

Amgen Europe B.V; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Oncology

3.3.12. Cemiplimab - EMEA-002007-PIP02-17-M03

Regeneron Ireland DAC; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 30 discussion

Oncology

3.3.13. Tovorafenib - Orphan - EMEA-002763-PIP01-20-M01

Day One Biopharmaceuticals, Inc; Treatment of paediatric low-grade glioma

Day 30 discussion

Oncology

3.3.14. Iptacopan - Orphan - EMEA-002705-PIP02-19-M01

Novartis Europharm Limited; Treatment of IgA nephropathy

Day 30 discussion

Other

3.3.15. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M05

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

Other

3.3.16. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M05

Helsinn Birex Pharmaceuticals Limited; Prevention of chemotherapy-induced nausea and vomiting

Day 30 discussion

Other

3.3.17. Selexipag - EMEA-000997-PIP01-10-M07

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 discussion

Other

3.3.18. Evenamide - EMEA-002519-PIP03-21-M01

Newron Pharmaceuticals S.p.A.; Treatment of schizophrenia

Day 30 discussion

Psychiatry

3.3.19. NVX-CoV2373 - EMEA-002941-PIP01-20-M04

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

Day 30 discussion

Vaccines

3.3.20. Recombinant COVID-19 subunit nanoparticle - EMEA-003115-PIP01-21-M02

SK Chemicals GmBH; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 14 August 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)

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

6.1.1. N-phenyl-diazino-pyrimidin-amine derivative - EMEA-07-2023

Boehringer Ingelheim; The class of pyrimidine- and pyrimidine analogue-containing medicinal products for treatment of lung malignant neoplasms / Treatment of non-small cell lung cancer

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 not confirmed.

This is based on the consideration that this medicinal product, being a tyrosine kinase inhibitor that inhibits ERBB2/HER2, does not belong to the medicinal class of pyrimidine- and pyrimidine analogue-containing medicinal products for treatment of lung malignant neoplasms.

Other potential paediatric interests of this medicine suggested by PDCO: none identified at this stage.

6.1.2. Pegylated interferon alfa-2a (Pegasys) - EMEA-06-2023

zr pharma& GmbH; The class of immunomodulatory cytokine medicinal products for myeloproliferative neoplasms / Treatment of polycythaemia vera and treatment of essential thrombocythaemia

Summary of Committee discussion:

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

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

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver

7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver

7.1.1. Bempedoic acid – EMEA-001872-PIP01-15-M02; Bempedoic acid / ezetimibe - EMEA-002200-PIP01-17

Daiichi Sankyo Europe GmbH; Treatment of elevated cholesterol

Proposed indication: Cardiovascular risk reduction

Summary of Committee discussion:

The PDCO has concluded that the proposed indication falls under the agreed condition.

8. Annual reports on deferrals

The members of the PDCO took note of the products listed in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

The Chair thanked Dimitrios Athanasiou for his contribution as a member representing patients' organisations.

The Chair announced that Louisa Braun Exner is the new member for Denmark, replacing Nanna Borup Johansen.

9.1.2. Vote by Proxy

None

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

Summary of Committee discussion:

The Committee was updated about the next strategic review and learning meeting in Madrid.

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 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 July 2023, was provided by a CHMP / PDCO member.

The results of survey on the ongoing pilot were presented to the PDCO members. Based on the positive survey results the Committee concluded that this activity should continue monthly. The CHMP feedback should be obtained as well.

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 ínterim*)

Summary of Committee discussion:

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

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

No item

9.4. Cooperation within the EU regulatory network

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

No item

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The July 2023 agenda of the cluster was shared with the PDCO members for information.

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

No item

10. Any other business

10.1. Paediatric health threats update

Summary of Committee discussion:

The paediatric health threats update was cancelled.

10.2. PDCO requirement for modifying allergen PIPs

Summary of Committee discussion:

The response to the Bundesverband der Pharmazeutischen Industrie has been finalised.

10.3. Request for feedback on recommendation for inclusion of adolescents in adult trials from CTCG

PDCO member: Anette Solli Karlsen

Summary of Committee discussion:

The Committee discussed the topic of inclusion of adolescents in adult trials, based on a request coming from CTCG.

11. Breakout sessions

11.1. Internal PDCO Operations

Summary of Committee discussion:

Topics relating to PDCO internal operation were discussed.

11.2. Neonatology

Summary of Committee discussion:

Topics related to the revision of the Neonatal Guideline were discussed.

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 18-21 July 2023 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Brian Aylward	Chair	Ireland	No interests declared	
Johanna Wernsperger	Member	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in final deliberations and voting on:	2.3.1. Apixaban - EMEA-000183-PIP02-12-M04
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Zena Gunther	Member	Cyprus	No interests declared	
Tereza Bazantova	Alternate	Czechia	No interests declared	
Louisa Braun Exner	Member	Denmark	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Pauliina Lehtolainen-Dalkilic	Member	Finland	No interests declared	
Anne Paavola	Alternate	Finland	No interests declared	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
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	
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Olivier Moes	Alternate	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No restrictions applicable to this meeting	
Maaike van Dartel	Alternate	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No restrictions applicable to this meeting	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Peter Szitanyi	Alternate	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Sara Vennberg	Member	Sweden	No interests declared	
David Khan	Alternate	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jose Ignacio Malagon Calle	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jaroslav Sterba	Member	Patients' Organisation Representative	No participation in final deliberations and voting on:	2.3.20. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M03

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Member	Patients' Organisation Representative	No interests declared	
Celine Chu	Expert - via telephone*	France	No interests declared	
María Estela Moreno Martín	Expert - via telephone*	Spain	No interests declared	
Peter Van Meer	Expert - via telephone*	Netherlands	No interests declared	
Michiel van den Heuvel	Expert - via telephone*	Netherlands	No interests declared	
Caroline Auriche	Expert - via telephone*	France	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/