19 April 2024
EMA/PDCO/140329/2024
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 19-22 March 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

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1. Introductions

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

The Chair opened the meeting by welcoming all participants. The meeting was held in-person.

In accordance with the Agency's policy on handling of declarations of interests of scientific Committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and on the topics in the agenda of the meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda topics.

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

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure. All decisions taken at this meeting were made in the presence of a quorum of members. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The Chair thanked the departing member.

1.2. Adoption of agenda

The agenda for 19-22 March 2024 meeting was adopted with amendments.

Topics added:

7.1.1. Tirzepatide - EMEA-002360-PIP02-22-M02

10.3 PIP Focusing on Child-Centric Medicines Developments

1.3. Adoption of the minutes

The minutes for 20-23 February 2024 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. Enlicitide (decanoate) - EMEA-003453-PIP01-23

MSD Europe Belgium; Treatment of hypercholesterolaemia

Day 120 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO adopted a positive paediatric investigation plan (PIP) opinion for enlicitide (decanoate) for the treatment of hypercholesterolemia, with a waiver for the paediatric population from birth to less than 6 years on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments, and a deferral.

2.1.2. Lutikizumab - EMEA-003481-PIP01-23

AbbVie Ltd; Treatment of hidradenitis suppurativa

Day 120 opinion

Dermatology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from 12 years to less than 18 years of age, in the condition treatment of hidradenitis suppurativa was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 12 years of age on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.3. Spesolimab - EMEA-002475-PIP04-23

Boehringer Ingelheim International GmbH; Treatment of hidradenitis suppurativa

Day 120 opinion

Dermatology

Summary of Committee discussion:

Based on the assessment of this application, the PDCO adopted a positive opinion on Day 120 for spesolimab for the treatment of hidradenitis suppurativa in paediatric population after the onset of puberty (Tanner stage 2 or above) to less than 18 years of age in the condition of treatment of hidradenitis suppurativa.
A waiver was granted for the paediatric population prior the onset of puberty (Tanner stage less than 2) on the grounds that the disease or condition for which the specific medicinal

product is intended does not occur in the specified paediatric subset.
The PDCO granted a deferral for the measures contained in this paediatric investigation plan (PIP).

2.1.4. GIPR antagonist/GLP-1R agonist (AMG 133) - EMEA-003439-PIP02-23

Amgen Europe BV; Treatment of obesity

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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 GIPR antagonist/GLP-1R agonist (AMG 133), for the paediatric population from 6 years to less than 18 years of age in the condition treatment of obesity was adopted.
The PDCO agreed on a waiver in the paediatric population from birth to less than 6 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO granted a deferral for some measures contained in this PIP.

2.1.5. Plecanatide - EMEA-003441-PIP01-23

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

Day 120 opinion

Gastroenterology-Hepatology

Note: Withdrawal request received on 12 March 2024

2.1.6. Etavopivat - Orphan - EMEA-002924-PIP02-23

Novo Nordisk A/S; Treatment of sickle cell disease

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from 6 months to less than 18 years of age in the condition of sickle cell disease was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.7. Tozorakimab - EMEA-003360-PIP01-22

AstraZeneca AB; Treatment of acute respiratory failure

Day 120 opinion

Infectious Diseases / Pneumology - Allergology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO agreed on a paediatric investigation plan (PIP) for tozorakimab for all subsets of the paediatric population from birth to less than 18 years of age in the condition of treatment of severe viral lower respiratory tract disease. The PIP contains one clinical study, one non-clinical study, three clinical studies and two modelling studies. A deferral for some of the measures was also granted.

2.1.8. Relatlimab / nivolumab - EMEA-002727-PIP03-23

Bristol-Myers Squibb Pharma EEIG; Treatment of melanoma

Day 120 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and the additional details provided by the applicant via the draft opinion, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for adolescents from 12 years to less than 18 years of age, in the condition of treatment of melanoma, was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

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

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

Day 120 opinion

Ophthalmology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for boys from 5 years to less than 18 years of age, in the condition treatment of X-linked retinitis pigmentosa was adopted. The PDCO agreed on a waiver in the subset of boys from birth to less than 5 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible and in girls from birth to less than 18 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(s). The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.10. Hemopexin, human - Orphan - EMEA-003333-PIP01-22

CSL Behring GmbH; Treatment of sickle cell disease

Day 120 opinion

Other

Summary of Committee discussion:

In March 2024 the PDCO concurred that the applicant had addressed the remaining issues raised by the Committee at Day 90. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the hemopexin for paediatric patients from 6 months to less than 18 years of age, in the condition of treatment of sickle cell disease was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.11. Messenger RNA encoding Cas9, single guide RNA targeting the human KLKB1 gene - Orphan - EMEA-003465-PIP01-23

Intellia Therapeutics, Inc.; Treatment of hereditary angioedema (HAE)

Day 120 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application the PDCO adopted a positive opinion on Day 120 for the proposed medicine for children from 2 years to less than 18 years of age, in the condition treatment of hereditary angioedema. The PDCO agreed on a waiver in a subset of children 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 represent a significant therapeutic benefit as clinical studies are not feasible.
The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.12. Mometasone furoate - EMEA-003454-PIP01-23

Orphix Consulting GmbH; Treatment of chronic rhinosinusitis

Day 120 opinion

Oto-rhino-laryngology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion on paediatric investigation plan (PIP) for mometasone furoate in adolescents 12 years of age and older was agreed in the condition of 'treatment of chronic rhinosinusitis'.
The PDCO granted a waiver in the paediatric population from birth to less than 12 years of

age on the grounds that clinical studies with mometasone furoate cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset. The Committee granted a deferral for one or more measures contained in this PIP.

2.1.13. Tanimilast - EMEA-003393-PIP01-23

Chiesi Farmaceutici S.p.A.; Treatment of asthma

Day 120 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from 6 years to less than 18 years of age, in the condition treatment of asthma 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 PIP.

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

Sanofi Pasteur; Prevention of respiratory syncytial virus (RSV) disease

Day 120 opinion

Vaccines

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed on a positive opinion for live attenuated respiratory syncytial virus (RSV) for all subsets of the paediatric population from birth to less than 18 years of age in the condition of prevention of RSV disease.
The paediatric investigation plan (PIP) contains one non-clinical study and six clinical studies. Some of the studies in the PIP are deferred.

2.1.15. Atorvastatin calcium / fenofibrate - EMEA-003563-PIP01-23

Althera Laboratories Ltd; Treatment of mixed hyperlipidaemia

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 atorvastatin calcium / fenofibrate for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mixed hyperlipidaemia.

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

2.1.16. Ezetimibe / fenofibrate / rosuvastatin calcium - EMEA-003562-PIP01-23

Althera Laboratories Ltd; Treatment of mixed hyperlipidaemia

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

2.1.17. Volixibat potassium - EMEA-003567-PIP01-23

Mirum Pharmaceuticals, Inc.; Treatment of primary biliary cholangitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

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 volixibat potassium for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of primary biliary cholangitis, on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. The PDCO identified primary sclerosing cholangitis as an unmet 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. Acetylcysteine amide - EMEA-003554-PIP01-23

Arctic Therapeutics; Treatment of hereditary cystatin C amyloid angiopathy

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 acetylcysteine amide for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of hereditary cystatin C amyloid angiopathy, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.19. *Clostridium botulinum* neurotoxin type A (150 kD), free from complexing proteins - EMEA-001039-PIP04-23

Merz Pharmaceuticals GmbH; Treatment of essential tremor

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the March 2024 plenary meeting the application for a product specific full waiver for *Clostridium botulinum* neurotoxin type A (150 kD), free from complexing proteins for treatment of essential tremor.
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 *Clostridium botulinum* neurotoxin type A (150 kD), free from complexing proteins for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of essential tremor on grounds of lack of significant therapeutic benefit.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.20. Amivantamab - EMEA-002573-PIP02-23

Janssen-Cilag International N.V.; Treatment of colorectal carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the March 2024 plenary meeting, a request for a product-specific waiver for amivantamab for the treatment of colorectal cancer on the grounds that the disease does not occur in the paediatric population.
The PDCO confirmed all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 for this product for the treatment of colorectal carcinoma on the grounds that the disease does not occur in the paediatric population.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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 applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.21. Camreluzimab - EMEA-003566-PIP01-23

Luzsana Biotechnology Europe AG; Treatment of hepatocellular carcinoma

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 camreluzimab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of hepatocellular carcinoma 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.

2.1.22. Erdafitinib - EMEA-002042-PIP03-23

Janssen-Cilag International N.V; Treatment of urothelial carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO’s views expressed at Day 30 were confirmed. 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 (new formulation, i.e. tablets for intravesical use) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of urothelial carcinoma based, in line with recent discussions at the PDCO for the same

condition, on the grounds that the disease does not occur in the paediatric population.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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. Modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens (V940/mRNA-4157) - EMEA-003434-PIP02-23

MSD Europe Belgium SRL; Treatment of urothelial carcinoma / Treatment of renal cell carcinoma / Treatment of cutaneous squamous cell carcinoma

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 modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens (V940/mRNA-4157) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of urothelial carcinomas and treatment of renal cell carcinoma based on the grounds the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible and treatment of cutaneous squamous cell carcinoma that the disease or condition for which the specific medicinal product is intended does not occur, respectively.
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. N-(2-((2-(dimethylamino)ethyl)(methyl)amino)-5-((4-(1-methyl-1H-indol-3-yl)pyrimidin-2-yl)amino)-6-(2,2,2-trifluoroethoxy)pyridin-3-yl)acrylamide methanesulfonate - EMEA-003485-PIP01-23

ArriVent Biopharma, Inc.; Treatment of non-small cell lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the March 2024 plenary meeting, a product-specific waiver request for N-(2-((2-(dimethylamino)ethyl)(methyl)amino)-5-((4-(1-methyl-1H-indol-3-yl)pyrimidin-2-yl)amino)-6-(2,2,2-trifluoroethoxy)pyridin-3-yl)acrylamide methanesulfonate for the treatment of non-small cell lung cancer on the grounds that the disease does not occur in the paediatric population.
The PDCO assessed the additional information provided by the applicant between Day 30

and Day 60 and confirmed all the conclusions reached at Day 30. The Committee adopted a positive opinion at Day 60 for this product for the treatment of non-small cell lung cancer on the grounds that the disease does not occur in the paediatric population.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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 applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.25. Petosemtamab - EMEA-003557-PIP01-23

Merus N.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 petosemtamab 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, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.26. Raludotatug deruxtecan - EMEA-003569-PIP01-23

Daiichi Sankyo Europe GmbH; Treatment of ovarian cancer / Treatment of primary peritoneal cancer / Treatment of fallopian tube 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 for raludotatug deruxtecan.
The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of fallopian tube cancer on the grounds that the condition does not occur in the paediatric population.
The PDCO recommended granting a waiver for the proposed medicine for all subsets of the

paediatric population (from birth to less than 18 years of age) for the condition treatment of primary peritoneal cancer on the grounds that the condition does not occur in the paediatric population.
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 ovarian cancer on the grounds that the condition does not occur in the paediatric population (all boys from birth to less than 18 years of age and girls from birth to less than 13 years of age), and on the grounds that the specific medicinal product cannot be expected to be of significant therapeutic benefit to the paediatric population (girls from 13 to less than 18 years of age).
The PDCO emphasised that the granting of a waiver for the conditions 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. Riletamotide / tapderimotide / alrefimotide - EMEA-003555-PIP01-23

Ultimovacs ASA; Treatment of mesothelioma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO's views expressed at Day 30 were confirmed.
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 the proposed medicine for all subsets of the paediatric population (from birth to less 18 years of age) for the condition of treatment of mesothelioma on the grounds that the disease does not occur in the paediatric population.
The applicant agreed to extend the waiver 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.

2.1.28. Carbachol / brimonidine tartrate - EMEA-003561-PIP01-23

Visus Therapeutics, Inc.; Treatment of presbyopia

Day 60 opinion

Ophthalmology

Summary of Committee discussion:

In March 2024 the PDCO recommended granting a waiver for the carbachol / brimonidine tartrate for all subsets of the paediatric population (from birth to less than 18 years of age)

for the condition treatment of presbyopia on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).
The PDCO 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.

2.1.29. Human alpha-1-proteinase inhibitor immunoglobulin G fusion protein, recombinant - EMEA-003570-PIP01-23

Inhibrx, Inc.; Treatment of emphysema secondary to congenital deficiency of alpha-1 antitrypsin

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

The PDCO has reviewed the feedback from the applicant on Day 60 and concluded that additional scientific justification for the waiver request was acceptable.
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 human alpha-1-proteinase inhibitor immunoglobulin G fusion protein, recombinant for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of emphysema secondary to congenital deficiency of alpha-1 antitrypsin, on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations. The applicant agreed to extend the scope of the waiver to all pharmaceutical forms, all routes of administration.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.30. Baxdrostat / dapagliflozin (propanediol monohydrate) - EMEA-003559-PIP01-23

AstraZeneca AB; Treatment of chronic kidney disease

Day 60 opinion

Uro-nephrology

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 / dapagliflozin (propanediol monohydrate) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of chronic kidney disease, on the grounds that the specific medicinal product is likely to be unsafe in the paediatric population from birth to less than 2 years of age and does not represent a significant therapeutic benefit over existing treatments in the paediatric population from 2 years to less than 18 years of age.

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

2.1.31. Sargramostim - EMEA-003568-PIP01-23

Partner Therapeutics, Inc.; Treatment of acute radiation syndrome

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed on a positive opinion for a paediatric investigation plan (PIP) for sagramostin in children from birth to less than 18 years of age in the condition treatment of acute radiation syndrome. The PIP contains four non-clinical studies, one clinical study, and one modelling and simulation study. A deferral was granted for the clinical study.

2.1.32. Ezetimibe / pitavastatin calcium - EMEA-003573-PIP01-23

KRKA, d.d., Novo mesto; Treatment of hypercholesterolaemia

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

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 ezetimibe / pitavastatin calcium for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of hypercholesterolaemia 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.

2.1.33. Mirikizumab - EMEA-002208-PIP02-24

Eli Lilly and Company; Treatment of ulcerative colitis

Day 30 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

This is an administrative procedure following the applicant's request to split the paediatric investigation plan (PIP) including both the ulcerative colitis (UC) and Crohn's disease conditions (EMEA-002208-PIP01-17) into separate PIPs. The studies included in the key elements form for this separate PIP for UC are identical to the ones agreed for UC during the latest modification of the combined PIP (EMEA-002208-PIP01-17-M03). Of note, the waiver request in children below 2 years of age should be based on the grounds lack of significant therapeutic benefit over existing treatments, in line with the waiver included in EMEA-002208-PIP01-17 in the condition treatment of ulcerative colitis.
Based on the assessment of this application, a positive opinion for the PIP for the proposed medicine for children from 2 years to less than 18 years of age, in the condition treatment of ulcerative colitis was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 2 years of age on the grounds of lack of significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.34. Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to B-like strain - EMEA-003603-PIP01-24

Sanofi Winthrop Industrie; Prevention of influenza disease

Day 7 opinion

Vaccines

The PDCO adopted the opinion by written procedure on 14 March 2024

2.1.35. Human rabies immune globulin - EMEA-003552-PIP01-23

Kamada Ireland Limited.; Prevention of rabies infection

Day 60 opinion

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 paediatric investigation plan (PIP) for human rabies immune globulin for all subsets of the paediatric population (birth to less than 18 years of age), in the condition of prevention of rabies infection was adopted.

2.2. Opinions on Compliance Check

2.2.1. Tasimelteon - EMEA-C1-001531-PIP01-13-M04

Vanda Pharmaceuticals Netherlands B.V.; Treatment of non-24-hour sleep-wake disorder in the totally blind

Day 60 letter

Neurology

Summary of Committee discussion:

The PDCO discussed the completed study(ies) and considered that only Study 1 and 2 are compliant with the latest Agency's Decision (P/0215/2018) of 17 July 2018. Compliance for Studies 3 and 6 need to be resubmitted after discrepancies and necessary changes are addressed via the modification procedure.
The PDCO finalised this partially completed compliance procedure on 22 March 2024.

2.2.2. Bilastine - EMEA-C-000347-PIP02-16-M05

Faes Farma, S.A.; Treatment of allergic conjunctivitis

Day 60 opinion

Ophthalmology / Pneumology - Allergology

Summary of Committee discussion:

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

- EMEA-C1-000347-PIP02-16-M02

The PDCO adopted on 22 March 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0209/2023) of 14 June 2023.

2.2.3. Influenza virus type B, Victoria lineage / influenza virus type A, H3N2 / influenza virus type A, H1N1 - EMEA-C-003589-PIP01-24

AstraZeneca AB; Prevention of influenza infection

Day 30 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted on 19 March 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0045/2024) of 9 February 2024.

2.2.4. Atropine sulfate - EMEA-C-002744-PIP01-19-M01

Nevakar Inc.; Treatment of myopia

Day 30 opinion

Ophthalmology

Summary of Committee discussion:

The studies are compliant with the latest paediatric investigation plan (PIP) as set out in the above stated Agency's Decision.
The PDCO adopted on 22 March 2024 an opinion confirming the compliance of all studies in the agreed PIP as set out in the latest Agency's Decision (P/0039/2024) of 7 February 2024.

2.2.5. Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to B-like strain - EMEA-C-003603-PIP01-24

Sanofi Winthrop Industrie; Prevention of influenza disease

Day 2 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted on 20 March 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0088/2024) of 15 March 2024.

2.2.6. Birch pollen extract (*Betula verrucosa*) - EMEA-C-001879-PIP01-15-M03

ALK-Abelló A/S; Treatment of allergic rhinitis / rhino-conjunctivitis

Day 30 opinion

Pneumology - Allergology

Summary of Committee discussion:

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

- EMEA-C1-001879-PIP01-15-M01

The PDCO adopted on 22 March 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0207/2021) of 10 May 2021.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Apixaban - EMEA-000183-PIP02-12-M05

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 (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/0338/2023 of 17 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Levonorgestrel - EMEA-002474-PIP02-18-M02

Chemo Research, S.L.; Contraception

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that 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/0447/2022 of 28 October 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Venglustat - Orphan - EMEA-001716-PIP07-22-M01

Sanofi B.V.; Treatment of Gaucher disease type 3 / Treatment of Gaucher disease type 2

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
In March 2024 the PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0489/2023 of 1 December 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Avatrombopag maleate - EMEA-001136-PIP01-11-M03

Swedish Orphan Biovitrum AB; Treatment of idiopathic thrombocytopenic purpura / Treatment of thrombocytopenic purpura secondary to liver disease

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/0010/2023 of 31 January 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Giroctocogene fitelparvovec - Orphan - EMEA-002724-PIP01-19-M03

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 60 opinion

Haematology-Hemostaseology

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/0555/2021 of 4 January 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19-M02

Swedish Orphan Biovitrum AB (publ); 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 (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/0210/2021 of 10 May 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M06

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0412/2023 of 27 October 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP03-20-M02

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the March 2024 plenary meeting, a request for modification for brexucabtagene autoleucel for the treatment of mature B-cell neoplasms. Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed 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/0002/2021 of 5 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Loncastuximab tesirine - EMEA-002665-PIP02-20-M01

Swedish Orphan Biovitrum AB (publ); Treatment of mature B-cell neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the March 2024 plenary meeting, a request for modification for loncastuximab tesirine for the treatment of mature B-cell neoplasms. Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed 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/0400/2021 of 1 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Nivolumab - EMEA-001407-PIP02-15-M07

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

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes 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/0339/2023 of 17 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Olaparib - EMEA-002269-PIP01-17-M03

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

Day 60 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the 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 some proposed changes could be accepted. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision. The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Selumetinib - Orphan - EMEA-001585-PIP01-13-M06

AstraZeneca AB; Treatment of malignant neoplasms of lymphoid tissue / Treatment of malignant neoplasms of the central nervous system

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes 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/0341/2021 of 12 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Berotralstat - EMEA-002449-PIP02-18-M02

BioCryst Ireland Limited; Treatment of hereditary angioedema

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that 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/0353/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Glycopyrronium bromide / formoterol fumarate dihydrate / beclometasone dipropionate - EMEA-001875-PIP02-18-M04

Chiesi Farmaceutici S.p.A.; Treatment of asthma

Day 60 opinion

Pneumology – Allergology

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/0094/2021 of 17 March 2021.
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Mometasone (furoate) / glycopyrronium bromide / indacaterol - EMEA-001812-PIP01-15-M03

Novartis Europharm Limited; Treatment of asthma

Day 60 opinion

Pneumology – Allergology

Note: Withdrawal request received on 22 March 2024

2.3.16. Atrasentan - Orphan - EMEA-001666-PIP02-21-M01

Chinook Therapeutics, Inc.; Treatment of IgA nephropathy

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed 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/0171/2016 of 17 June 2016).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Seqirus Netherlands; Prevention of influenza

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as

set in the Agency's latest decision (P/0492/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Live, attenuated, dengue virus, serotype 4 (DENV4) / live, attenuated, dengue virus, serotype 3 (DENV3) / live, attenuated, chimeric dengue virus, serotype 2 (DENV2) / live, attenuated, dengue virus, serotype 1 (DENV1) - EMEA-002999-PIP01-21-M01

MSD Europe Belgium SRL; Prevention of dengue disease

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0521/2022 of 30 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M01

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

The PDCO's view expressed at Day 30 was re-discussed and endorsed.
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/0373/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Recombinant influenza hemagglutinin-strain B (Yamagata lineage) / recombinant influenza hemagglutinin-strain B (Victoria lineage) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain A (H1N1 subtype) - EMEA-002418-PIP01-18-M03

Sanofi Pasteur; Prevention of influenza infection

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), and the additional justifications provided by the applicant between Day 30 and Day 60, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0100/2022 of 18 March 2022).

2.3.21. Mirikizumab - EMEA-002208-PIP01-17-M04

Eli Lilly and Company; 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 concerns the request to split the PIP containing both Crohn's disease and ulcerative colitis into two separate PIPs. A separate PIP for the condition treatment of ulcerative colitis was submitted in parallel.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0534/2023 of 29 December 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

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

2.7.1. Dupilumab - EMEA-C3-001501-PIP02-13-M08

Sanofi Winthrop Industrie; Treatment of asthma

Day 30 letter

Pneumology – Allergology

2.7.2. Delandistrogene moxeparvovec - EMEA-C1-002677-PIP01-19-M03

Roche Registration GmbH; Treatment of Duchenne Muscular Dystrophy

Day 30 letter

Neurology

2.7.3. Pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 8 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 15C – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 15A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 16F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 23A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 24F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 17F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 10A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 12F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 20A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 31 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 35B – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 9N – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 11A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 23B – diphtheria CRM197 conjugate (V116) - EMEA-C1-003155-PIP01-21-M01

MSD Europe Belgium S.R.L.; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 letter

Vaccines

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. EMEA-003480-PIP01-23

Treatment of psoriasis

Day 90 discussion

Dermatology

3.1.2. EMEA-003478-PIP01-23

Treatment of psoriasis

Day 90 discussion

Dermatology

3.1.3. Human alpha-1 proteinase inhibitor, modified - EMEA-003463-PIP01-23

Treatment of haemophilia B

Day 90 discussion

Haematology-Hemostaseology

3.1.4. Ianalumab - EMEA-002338-PIP05-23

Treatment of immune thrombocytopenia (ITP)

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Mavorixafor - Orphan - EMEA-002490-PIP01-18

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

Day 90 discussion

Immunology-Rheumatology-Transplantation

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

Marinus Pharmaceuticals, Inc.; Treatment of tuberous sclerosis complex

Day 90 discussion

Neurology

3.1.7. Radiprodil - EMEA-003462-PIP01-23

Treatment of GRIN-related disorders

Day 90 discussion

Neurology

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

Myrtelle,Inc.; Treatment of Canavan disease

Day 90 discussion

Neurology

3.1.9. Brigimadlin - Orphan - EMEA-003260-PIP03-23

Boehringer Ingelheim International GmbH; Treatment of soft tissue sarcoma excluding liposarcoma

Day 90 discussion

Oncology

3.1.10. Humanised IgG1 monoclonal antibody against pituitary adenylate cyclase-activating polypeptide - EMEA-003483-PIP01-23

Prevention of migraine

Day 90 discussion

Pain / Neurology

3.1.11. Povorcitinib - EMEA-003313-PIP02-23

Treatment of vitiligo

Day 60 discussion

Dermatology

3.1.12. Sonelokimab - EMEA-002568-PIP02-23

Treatment of hidradenitis suppurativa

Day 60 discussion

Dermatology

3.1.13. Linaprazan - EMEA-003558-PIP01-23

Treatment of gastro-oesophageal reflux disease

Day 60 discussion

Gastroenterology-Hepatology

3.1.14. Humanised IgG4 monoclonal antibody against FIXa and FX - EMEA-003550-PIP01-23

Treatment of coagulation factor deficiencies

Day 60 discussion

Haematology-Hemostaseology

3.1.15. Tulisokibart - EMEA-003556-PIP01-23

Treatment of ulcerative colitis

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.16. Olorofim - Orphan - EMEA-003564-PIP01-23

Shionogi BV; Treatment of fungal infectious disorders

Day 60 discussion

Infectious Diseases

3.1.17. N-{(2S,3R)-4,4-difluoro-1-(2-hydroxy-2-methylpropanoyl)-2-[(2,3',5'-trifluoro[1,1'-biphenyl]-3-yl)methyl]pyrrolidin-3-yl}ethanesulfonamide - Orphan - EMEA-003553-PIP01-23

Takeda Pharma A/S; Treatment of idiopathic hypersomnia / Treatment of narcolepsy

Day 60 discussion

Neurology

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

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

Day 60 discussion

Oncology

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

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

Day 60 discussion

Other / Cardiovascular Diseases

3.1.20. Atogepant - EMEA-002530-PIP02-23

Treatment of migraine headaches

Day 60 discussion

Pain

3.1.21. EMEA-003560-PIP01-23

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

Day 60 discussion

Vaccines / Infectious Diseases

3.1.22. EMEA-003580-PIP01-24

Treatment of elevated cholesterol / Treatment of mixed dyslipidaemia

Day 30 discussion

Cardiovascular Diseases

3.1.23. Ezetimibe / rosuvastatin - EMEA-003582-PIP01-24

Treatment of hypercholesterolaemia / Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

3.1.24. Gallium (68Ga) boclatixafortide - EMEA-003408-PIP02-24

Diagnosis of primary aldosteronism

Day 30 discussion

Diagnostic

3.1.25. Ersodetug - Orphan - EMEA-002813-PIP02-24

Rezolute (Bio) Ireland Limited; Treatment of congenital hyperinsulinism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.26. Sitagliptin / dapagliflozin - EMEA-003572-PIP01-23

Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.27. Crofelemer - Orphan - EMEA-003296-PIP02-24

Napo Therapeutics S.p.A.; Treatment of microvillus inclusion disease

Day 30 discussion

Gastroenterology-Hepatology

3.1.28. 6-(4-((1s,3s)-1-amino-3-hydroxycyclobutyl)phenyl)-1-ethyl-7-phenyl-1H-pyrido [2,3-b][1,4]oxazin-2(3H)-one, L-tartrate salt - Orphan - EMEA-003585-PIP01-24

Vaderis Therapeutics AG; Treatment of hereditary haemorrhagic telangiectasia

Day 30 discussion

Haematology-Hemostaseology

3.1.29. Mezagitamab - EMEA-003502-PIP02-24

Treatment of primary IgA nephropathy

Day 30 discussion

Haematology-Hemostaseology

3.1.30. Tildrakizumab - EMEA-001451-PIP02-24

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.31. Tulisokibart - EMEA-003556-PIP02-24

Treatment of Crohn's disease

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.32. Trofinetide - Orphan - EMEA-003587-PIP01-24

Acadia Pharmaceuticals Inc.; Treatment of Rett syndrome

Day 30 discussion

Neurology

3.1.33. EMEA-003586-PIP01-24

Treatment of all solid and haematological malignancies

Day 30 discussion

Oncology

3.1.34. 3,3-Dimethyl-N-(6-methyl-5-{[2-(1-methyl-1H-pyrazol-4-yl)pyridine-4-yl]oxy}pyridine-2-yl)-2-oxopyrrolidine-1-carboxamide hydrochloride hydrate - Orphan - EMEA-003495-PIP02-24

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

Day 30 discussion

Oncology

3.1.35. 7-Ethyl-10-hydroxy-camptothecin - Orphan - EMEA-003588-PIP01-24

CEBIOTEX S.L. Biomedical Nanofibers; Treatment of soft tissue neoplasms

Day 30 discussion

Oncology

3.1.36. EMEA-003576-PIP01-24

Treatment of prostate cancer

Day 30 discussion

Oncology

3.1.37. Autologous T-cells from a melanoma metastasis - EMEA-003535-PIP02-24

Treatment of melanoma

Day 30 discussion

Oncology

3.1.38. Certepetide - Orphan - EMEA-003577-PIP01-24

Lisata Therapeutics Ireland Limited; Treatment of pancreatic cancer

Day 30 discussion

Oncology

3.1.39. EMEA-003581-PIP01-24

Treatment of lung cancer

Day 30 discussion

Oncology

3.1.40. Evencaleucel - Orphan - EMEA-003579-PIP01-24

XNK Therapeutics AB; Treatment of multiple myeloma

Day 30 discussion

Oncology

3.1.41. Rivoceranib - EMEA-002489-PIP02-24

Treatment of hepatocellular carcinoma

Day 30 discussion

Oncology

3.1.42. EMEA-003574-PIP01-24

Treatment of colorectal cancer

Day 30 discussion

Oncology

3.1.43. Lebrikizumab - EMEA-002536-PIP02-24

Treatment of chronic rhinosinusitis with nasal polyps

Day 30 discussion

Pneumology - Allergology

3.1.44. Ravulizumab - EMEA-001943-PIP07-24

Treatment of primary IgA nephropathy

Day 30 discussion

Uro-nephrology

3.1.45. mRNA encoding the influenza virus B/Victoria strain neuraminidase / mRNA encoding the influenza virus B/Victoria strain hemagglutinin / mRNA encoding the influenza virus H3N2 strain neuraminidase / mRNA encoding the influenza virus H3N2 strain hemagglutinin / mRNA encoding the influenza virus H1N1 strain neuraminidase / mRNA encoding the influenza virus H1N1 strain hemagglutinin - EMEA-003578-PIP01-24

Influenza immunisation

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. Maralixibat chloride - EMEA-C-001475-PIP03-17-M04

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 discussion

Gastroenterology-Hepatology

3.2.2. Itolizumab - EMEA-C1-003208-PIP02-22

Biocon Pharma Malta I Limited; Treatment of acute graft versus host disease

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.3. Cobicistat / darunavir - EMEA-C4-001280-PIP01-12-M06

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

Day 30 discussion

Infectious Diseases

3.2.4. Binimetinib - EMEA-C-001454-PIP03-15-M03

Pierre Fabre Médicament; Treatment of melanoma

Day 30 discussion

Oncology

3.2.5. Encorafenib - EMEA-C-001588-PIP01-13-M03

Pierre Fabre Médicament; Treatment of melanoma

Day 30 discussion

Oncology

3.2.6. Pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed) - EMEA-C-002330-PIP01-18-M03

Pfizer Europe MA EEIG; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Ertugliflozin - EMEA-001533-PIP01-13-M03

MSD Europe Belgium SRL; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.2. Linaclotide - EMEA-000927-PIP01-10-M08

AbbVie Deutschland GmbH & Co. KG; Treatment of functional constipation

Day 30 discussion

Gastroenterology-Hepatology

3.3.3. Inebilizumab - EMEA-001911-PIP03-23-M01

Horizon Therapeutics Ireland Designated Activity Company (DAC); Treatment of immunoglobulin G4-related disease (IgG4-RD)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.4. Olokizumab - EMEA-001222-PIP01-11-M01

Accelsiors GmbH; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.5. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M05

MSD Europe Belgium SRL; Treatment of gram-negative bacterial infections

Day 30 discussion

Infectious Diseases

3.3.6. Mecasermin rinfabate - Orphan - EMEA-000534-PIP03-17-M01

OHB Neonatology Ltd; Prevention of chronic lung disease of prematurity

Day 30 discussion

Neonatology - Paediatric Intensive Care

3.3.7. Inebilizumab - EMEA-001911-PIP02-22-M01

Horizon Therapeutics Ireland Designated Activity Company (DAC); Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.3.8. Soticlestat - Orphan - EMEA-002572-PIP02-19-M05

Takeda Pharma A/S; Treatment of Dravet syndrome / Treatment of Lennox-Gastaut syndrome

Day 30 discussion

Neurology

3.3.9. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M05

Vanda Pharmaceuticals Netherlands B.V.; Treatment of non-24-hour sleep-wake disorder in the totally blind

Day 30 discussion

Neurology

3.3.10. Quizartinib - EMEA-001821-PIP01-15-M08

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology

3.3.11. Calcifediol - EMEA-002093-PIP02-17-M02

Vifor Fresenius Medical Care Renal Pharma France; Treatment of secondary hyperparathyroidism (SHPT)

Day 30 discussion

Uro-nephrology

3.3.12. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21-M02

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 30 discussion

Uro-nephrology

3.3.13. *Neisseria meningitidis* serogroup W polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup Y polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup C polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M05

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 30 discussion

Vaccines

3.3.14. mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 (mRNA-1283) - EMEA-003426-PIP01-23-M01

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

Day 30 discussion

Vaccines / Infectious Diseases

Note: Withdrawal request received on 4 April 2024

4. Nominations

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

4.1. List of submissions of applications with start of procedure 2 April 2024 for Nomination of Rapporteur and Peer reviewer

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

Information related to this section cannot be disclosed at the present time as it is deemed

to contain commercially confidential information.

5.1. New Scientific Advice

No item

5.2. Final Scientific Advice (Reports and Scientific Advice letters)

No item

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

6.1.1. Zanidatamab – EMEA-17-2023

Jazz Pharmaceuticals Ireland Limited; The class of Her- / Epidermal growth factor-receptor antibody medicinal products for treatment of intestinal malignant neoplasms.

Zanidatamab in combination with capecitabine plus oxaliplatin (CAPOX) or 5-fluorouracil (5-FU) plus cisplatin (FP), with or without tislelizumab as first line (1L) treatment in patients with human epidermal growth factor receptor 2 (HER2)-positive, unresectable locally advanced or metastatic gastroesophageal adenocarcinoma (GEA).

Summary of Committee discussion:

Taking into account the 'heterogeneity' of the classification of gastric/gastroesophageal junction carcinomas and oesophageal carcinomas, 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: osteosarcoma (mainly relapsed/refractory and primary metastatic disease other than lungs only) as about 30% of osteosarcomas are HER2+. Although HER2 expression so far does not seem to have prognostic impact in osteosarcomas and there was a recently reported negative trial with trastuzumab deruxtecan, despite encouraging preclinical data, in view of the specific mechanism of action of the product, the applicant is encouraged to consider whether there might be a role for this product in treating paediatric patients with osteosarcoma.

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. Tirzepatide - EMEA-002360-PIP02-22-M02

Eli Lilly and Company; Treatment of obesity

Proposed indication: Treatment of obstructive sleep apnoea (OSA) in paediatric patients with obesity

Summary of Committee discussion:

The PDCO was of the view that the proposed indication 'treatment of obstructive sleep apnoea (OSA) in paediatric patients with obesity' falls under the scope of the Decision, as the indication is considered to be covered by the condition 'treatment of obesity' listed in the Agency Decision.

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 Maja Pavlovic as a member for Croatia.

9.1.2. Vote by Proxy

None

9.1.3. Strategic Review and Learning Meeting (SRLM) - Leuven, Belgium 16-17 May 2024

PDCO member: Karen van Malderen

Summary of Committee discussion:

The Committee was updated about the next SRLM to be held in person on 16-17 May 2024 in Leuven, Belgium.

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 February 2024, was presented to the PDCO members.

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

An overview of discussions on PIP-related procedures, held by the CHMP in March 2024, was provided by a CHMP / PDCO member.

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

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

9.3.2. Paediatric Formulation Operational Expert Group (PFOEG)

PDCO member: Brian Aylward (*ad interim*)

Summary of Committee discussion:

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

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

No item

9.3.4. Overview of Innovation Task Force (ITF) activities for the year 2023

Summary of Committee discussion:

An overview of the ITF briefing meetings held in 2023 was presented to the Committee for information.

9.3.5. Upcoming Innovation Task Force (ITF) meetings

Summary of Committee discussion:

Three upcoming ITF briefing meetings (BM) were presented to the Committee for information.

9.4. Cooperation within the EU regulatory network

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

No item

9.4.2. EU Network Training Centre (NTC): training webinar on the regulatory/health technology assessment (HTA) interface under the HTA Regulation

Summary of Committee discussion:

On 2 May 2024, an EU NTC training webinar on the regulatory/HTA interface under the HTA Regulation will be held. EMA would like to raise awareness within the wider regulatory network, whilst extending the target audience also to HTA colleagues, given the mutual learnings in view of the future collaborations. The agenda will cover an overview of the new HTA Regulation, outline of collaboration between regulators and HTAs under the new legal framework, followed by mutual learning about the respective assessment scopes. The webinar will be delivered by colleagues from the EC, HTAs and regulatory network and EMA respectively.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The February 2024 minutes, March 2024 agenda and minutes of the cluster were shared with the PDCO members for information.

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q1/2024 Update of the Business Pipeline report for the human scientific committees

Summary of Committee discussion:

The Q1 2024 forecast report was presented for information. The report is an update of the Marketing Authorisation Applications (MAAs) expected for the remainder of the year.

10. Any other business

10.1. Real World Evidence, including DARWIN EU®

Summary of Committee discussion:

The Committee received an update on the progress of DARWIN EU including the most recently onboarded additional 10 data partners, as well as 2 recent DARWIN EU studies likely to be of interest to the Committee. The first one is a descriptive cohort study on age-specific incidence rates of RSV-related disease in Europe; the second study is a comparative effectiveness study of human papillomavirus vaccines (HPV) to prevent cervical cancer. Both studies triggered interest and were discussed in detail, comments provided during the plenary discussion were duly noted. The group then discussed a call for new use cases to test the RWE extrapolation framework, as well as the ongoing selection of data partners within DARWIN EU, with some Committee members expressing their willingness to contribute more to the process of identifying and selecting data sources with high potential for addressing paediatric needs. Finally, an update was given on recent and upcoming events, including the launch of the HMA-EMA catalogues of real-world data sources and studies, the pharmacoepidemiology and real-world evidence training curriculum (available via EU NTC), the introduction of a new knowledge sharing event series (called Real-World Academy) and the recent joint HMA-EMA multistakeholder workshop on patient registries, which took place on 12 and 13 February 2024. The PDCO welcomed the update.

10.2. PDCO and Clinical Trials Coordination Group (CTCG) interaction

Summary of Committee discussion:

The EMA reported back from the discussion that took place on the 7 March 2024 CTCG meeting where action points to improve mutual awareness and collaboration were agreed.

10.3. PIP Focusing on Child-Centric Medicines Developments

Summary of Committee discussion:

A summary of the discussion on this topic at the recent DIA Europe meeting was presented to the Committee.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The group discussed ongoing oncology procedures and was informed about the upcoming paediatric strategy forum on novel ways to target and enhance efficiency of anti-GD2 therapies.

11.2. Neonatology

Summary of Committee discussion:

The group discussed topics related to the revision of the neonatal guideline.

11.3. HIV

Summary of Committee discussion:

The groups discussed aspects related to procedures pertaining to the therapeutic area.

The Chair thanked all participants and closed the meeting.

12. List of participants

List of participants including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 19-22 March 2024 meeting PDCO meeting which was held in-person.

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

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Brian Aylward	Chair	Ireland	No interests declared	
Johanna Wernsperger	Member*	Austria	No interests declared	
Agnes Gyurasics	Alternate	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in final deliberations and voting on:	2.3.1. Apixaban - EMEA-000183-PIP02-12-M05
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Miroslav Weiss	Member	Croatia	No interests declared	
Irena Senecic-Cala	Alternate*	Croatia	No participation in discussion, final deliberations and voting on:	3.3.2. Linaclotide - EMEA-000927-PIP01-10-M08
Zena Gunther	Member	Cyprus	No interests declared	
Tereza Bazantova	Member	Czechia	No interests declared	
Pavlina Chladova	Alternate	Czechia	No interests declared	
Louisa Braun Exner	Member	Denmark	No interests declared	
Liisa Saare	Alternate	Estonia	No interests declared	
Pauliina Lehtolainen-Dalkilic	Member	Finland	No interests declared	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Adrienn Horvath	Member*	Hungary	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Cinzia Ciceroni	Alternate	Italy	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	
Greta Budukeviciute	Member*	Lithuania	No interests declared	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
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 Andres Trelles	Member*	Spain	No interests declared	
Maria Jesus Fernandez 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 restrictions applicable to this meeting	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member*	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Tomasz	Member	Patients'	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Grybek		Organisation Representative		
Jaroslav Sterba	Alternate	Patients' Organisation Representative	No participation in final deliberations and voting on:	2.3.8. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP03-20-M02
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	
Meeting run with support from relevant EMA staff				

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

13. Explanatory notes

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

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

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

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

Class waiver (*section 6 Discussion on the applicability of class waiver*)
As some diseases do not affect children (for example Parkinson's disease), the development of

medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived**.** For more information on the classes of diseases subject to waivers, see class waivers.

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

For a list of acronyms and abbreviations, see:
Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA's regulatory activities

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