26 July 2024
EMA/PDCO/321477/2024
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 25-28 June 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 welcomed the new members.

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

1.2. Adoption of agenda

The agenda for 25-28 June 2024 meeting was adopted with amendments:

9.1.3. Strategic Review and Learning Meeting (SRLM) – Budapest, Hungary 9-10 October 2024

9.2.4. EMA engagement methodologies for patients and healthcare professionals

10.6 Spinal muscular atrophy (SMA) registry report

1.3. Adoption of the minutes

The minutes for 28-31 May 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. Transglutaminase 2 inhibitor (ZED1227) - EMEA-003513-PIP01-23

Dr. Falk Pharma GmbH; Treatment of coeliac disease

Day 120 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, a positive opinion was adopted by the PDCO for the paediatric investigation plan (PIP) for transglutaminase 2 inhibitor (ZED1227) in the condition of treatment of coeliac disease with a waiver for the paediatric population from birth to less than 2 years on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments, and a deferral of one or more measures contained in the PIP.

2.1.2. Mezagitamab - EMEA-003502-PIP01-23

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of immune thrombocytopenia

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 mezagitamab for children from 6 years to less than 18 years of age in the condition of treatment of immune thrombocytopenia 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 of lack of significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.3. Ganaxolone - EMEA-002341-PIP03-23

Treatment of refractory status epilepticus

Day 120 opinion

Neurology

Note: Withdrawal request received on 26 June 2024

2.1.4. Vosoritide - EMEA-002033-PIP02-23

BioMarin International Limited; Treatment of hypochondroplasia

Day 120 opinion

Other

Summary of Committee discussion:

The PDCO discussed at Day 120, during the June 2024 plenary meeting, an application for a paediatric investigation plan (PIP) and a deferral for vosoritide for the treatment of hypochondroplasia.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.
The PDCO adopted a positive opinion on a PIP with a deferral.

2.1.5. Single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A/H1N1, A/H3N2, and B/Victoria and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein - EMEA-003521-PIP01-23

Moderna Biotech Spain S.L.; Prevention of influenza and coronavirus disease 2019 (COVID-19)

Day 120 opinion

Vaccines

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 paediatric investigation plan (PIP) for single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A/H1N1, A/H3N2, and B/Victoria and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein for children aged 6 weeks to less than 18 years of age in the condition of prevention of influenza and coronavirus disease 2019 (COVID-19). The PDCO agreed on a waiver in a subset of children from birth to less than 6 weeks of age on the grounds that the specific medicinal product is likely to be ineffective. The PDCO granted a deferral for the completion of this PIP.

2.1.6. Tadalafil / ambrisentan - EMEA-003617-PIP01-24

Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular Diseases

Note: Withdrawal request received on 27 June 2024

2.1.7. Tadalafil / ambrisentan - EMEA-003621-PIP01-24

Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular Diseases

Note: Withdrawal request received on 28 June 2024

2.1.8. Tarumase - EMEA-003616-PIP01-24

SolasCure Limited; Treatment of chronic wounds

Day 60 opinion

Dermatology

Summary of Committee discussion:

The PDCO reviewed and endorsed the conclusions reached at Day 30 and maintained that a waiver in all subsets of the paediatric population for the 'treatment of chronic wounds' was not appropriate, and a paediatric investigation plan (PIP) should be submitted to address the identified paediatric needs. A negative opinion refusing the waiver request has therefore been adopted.

2.1.9. Copper (^{64}Cu) oxodotreotide - Orphan - EMEA-003610-PIP01-24

Curium Pet France; Diagnosis of neuroendocrine tumours (excluding neuroblastoma)

Day 60 opinion

Diagnostic

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 copper (^{64}Cu) oxodotreotide for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of diagnosis of neuroendocrine tumours (excluding neuroblastoma) on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.10. Alvelestat (tosylate) - EMEA-003605-PIP01-24

Mereo Biopharma Ireland Limited; Treatment of alpha-1 antitrypsin deficiency

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for alvelestat (tosylate) for all subsets of the paediatric population from birth to less than 18 years of age in the condition of 'treatment of alpha-1 antitrypsin deficiency' based on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.11. Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - EMEA-003420-PIP02-24

Treatment of severe hypertriglyceridaemia (SHTG)

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 18 June 2024

2.1.12. Inobrodib - EMEA-003622-PIP01-24

CellCentric Ltd; Treatment of multiple myeloma

Day 60 opinion

Haematology-Hemostaseology

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 inobrodib for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of multiple myeloma, based on the ground that the disease does 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.

2.1.13. Acasunlimab - EMEA-003606-PIP01-24

Genmab A/S; Treatment of lung cancer (small cell and non-small cell lung cancer)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the June 2024 plenary meeting, a product-specific waiver request for acasunlimab for the treatment of lung cancer on the grounds that the disease or condition 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 a product-specific waiver for this product for the treatment of lung cancer (small cell and non-small cell lung cancer) on the grounds that the disease does not occur in the paediatric population.
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.

2.1.14. Buparlisib - EMEA-003607-PIP01-24

Adlai Nortye USA, Inc.; Treatment of head and neck epithelial malignant neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO's views expressed at Day 30 were endorsed.
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 treatment of head and neck epithelial malignant neoplasms based on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.15. Ifinatamab deruxtecan - Orphan - EMEA-003613-PIP01-24

Daiichi Sankyo Europe GmbH; Treatment of lung cancer (small cell and non-small cell lung cancer)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 30, during the June 2024 plenary meeting, a product-specific waiver request for ifinatamab deruxtecan for the treatment of lung cancer on the grounds that the disease or condition 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 a product-specific waiver for this product for the treatment of lung cancer (small cell and non-small cell lung cancer) on the grounds that the disease does not occur in the paediatric population.
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.

2.1.16. Iloperidone - EMEA-000995-PIP02-24

Treatment of bipolar disorder

Day 60 opinion

Psychiatry

Note: Withdrawal request received on 19 June 2024

2.1.17. Zanubrutinib - EMEA-002354-PIP03-24

BeiGene Ireland Ltd.; Treatment of lymphoplasmacytic lymphoma / Treatment of mature B-cell neoplasms (excluding lymphocytic lymphoma)

Day 30 discussion

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. In line with the previously granted waiver for zanuburtinib hard capsules, the PDCO recommended granting a waiver for the new developed pharmaceutical form of the product, i.e. film-coated tablet, for all subsets of the paediatric population (from birth to less than 18 years of age) for the conditions 'treatment of lymphoplasmacytic lymphoma' based on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s), and for the 'treatment of mature B-cell neoplasms (excluding lymphoplasmacytic lymphoma)' based on the grounds that the disease for which the specific medicinal product is intended does not occur in the age subset from birth to less 1 year of age and on the grounds that the specific medicinal product is likely to be ineffective in the age subset from 1 year 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.

2.2. Opinions on Compliance Check

2.2.1. Recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (RIV3) - EMEA-C-003640-PIP01-24

Sanofi Winthrop Industrie; Prevention of influenza disease

Day 30 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted an opinion on 28 June 2024 confirming the compliance of all studies in

the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0220/2024) of 14 June 2024.

2.2.2. Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage - EMEA-C-003641-PIP01-24

GlaxoSmithKline Biologicals S.A.; Prevention of influenza infection

Day 30 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted an opinion on 28 June 2024 confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0219/2024) of 15 June 2024.

2.2.3. Estetrol monohydrate / drospirenone - EMEA-C-001332-PIP01-12-M06

Estetra SRL; Prevention of pregnancy

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO adopted on 28 June 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/0071/2023) of 10 March 2023.

2.2.4. Liraglutide - EMEA-C-000128-PIP02-09-M05

Novo Nordisk A/S; Treatment of obesity

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

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

- EMEA-C1-000128-PIP02-09
- EMEA-C2-000128-PIP02-09-M03
- EMEA-C3-000128-PIP02-09-M03

The PDCO adopted on 28 June 2024 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan (PIP) as set out in the latest Agency's Decision (P/0468/2023) of 13 December 2023.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Gadopiclenol - EMEA-001949-PIP01-16-M07

Guerbet; Detection and visualisation of areas with disruption of the blood brain barrier and/or abnormal vascularity for diagnostic purposes

Day 60 opinion

Diagnostic

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. This modification concerns a reduction in the sample size based on 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/0013/2024 of 31 January 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Gadopiclenol - EMEA-001949-PIP02-18-M05

Guerbet; Detection and visualisation of disorders or lesions with suspected abnormal vascularity in various body regions for diagnostic purposes

Day 60 opinion

Diagnostic

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. This modification concerns a reduction in the sample size based on 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/0013/2024 of 31 January 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Crinecerfont - Orphan - EMEA-002700-PIP01-19-M02

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

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

set in the Agency's latest decision (P/0097/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Humanised monoclonal antibody Fab fragment - Orphan - EMEA-003253-PIP01-22-M01

Sanofi B.V; Treatment of achondroplasia

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/0348/2023 of 8 September 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Navepegritide - Orphan - EMEA-002689-PIP02-23-M01

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0082/2024 of 15 March 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Teplizumab - EMEA-000524-PIP01-08-M03

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 27 June 2024

2.3.7. Survodutide - EMEA-002942-PIP02-20-M02

Boehringer Ingelheim International GmbH; Treatment of obesity

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

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

- delay of the date of initiation of Study 1;
- minor changes to wording of the data contributing to the model and delay of the date of completion of Study 2;
- delay of completion of Study 3.

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0166/2023 of 15 May 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Tezepelumab - EMEA-001613-PIP03-21-M01

AstraZeneca AB; Treatment of eosinophilic esophagitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that 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/0407/2023 of 25 October 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Vonoprazan - EMEA-002703-PIP01-19-M01

Phathom Pharmaceuticals, Inc.; Treatment of gastroesophageal reflux disease (GORD) / Treatment of *Helicobacter pylori* infection

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

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

- a waiver in neonates;
- deletion of the dispersible tablet formulation and sprinkles;
- popPK approach for Study 3 with sparse sampling rather than intensive sampling;
- delay of completion of the studies.

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0454/2020 of 4 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Fitusiran - Orphan - EMEA-001855-PIP01-15-M06

Sanofi B.V.; Treatment of haemophilia B / Treatment of haemophilia A

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 modified duration of Study 6, dose selection of Study 8 and the addition of Study 10 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/0410/2023 of 27 October 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Osivelotor - EMEA-003241-PIP01-22-M01

Pfizer Europe MA EEIG; Treatment of sickle cell 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/0074/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Narsoplimab - Orphan - EMEA-002479-PIP01-18-M02

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

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/0370/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Vadadustat - EMEA-001944-PIP01-16-M05

Medice Arzneimittel Pütter GmbH & Co KG; Treatment of anaemia due to chronic disorders

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the June 2024 plenary meeting, a request for modification for vadadustat for the treatment of anaemia due to chronic disorders.
The applicant requested to postpone completion of the study.
The PDCO confirmed all the conclusions reached at Day 30 and, based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), considered that the proposed changes could be acceptable. Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0244/2022 of 8 July 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19-M03

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of acute graft-versus-host disease

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0008/2023 of 30 January 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Human normal immunoglobulin - EMEA-001290-PIP01-12-M01

LFB Biotechnologies; Treatment of primary immunodeficiency

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. The applicant agreed with the addition of a new endpoint 'to assess the tolerability of the injections' in Study 1, in view of the increased maximum administration volume.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0305/2012 of 20 December 2012).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M07

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0027/2024 of 29 January 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Lenacapavir sodium - EMEA-002740-PIP01-19-M01

Gilead Sciences International 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 (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/0005/2021 of 15 January 2021).

2.3.18. Pretomanid - Orphan - EMEA-002115-PIP01-17-M06

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the requested delay in Study 4 could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0292/2022 of 11 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M07

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus type-1 (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 (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/0496/2022 of 2 December 2022). For the time being the planned number of patients to be recruited remained unchanged.
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M05

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (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 (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/0029/2023 of 31 January 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Cenobamate - EMEA-002563-PIP02-19-M03

Angelini Pharma S.p.A; Treatment of epilepsy

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that most of 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/0214/2023 of 14 June 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19-M01

Quince Therapeutics, S.p.A.; Treatment of ataxia telangiectasia

Day 60 opinion

Neurology

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

2.3.23. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M02

argenx BV; 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 (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/0072/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Fosigotifator sodium tromethamine - Orphan - EMEA-003344-PIP01-22-M01

AbbVie Limited; Treatment of vanishing white matter disease

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/0354/2023 of 8 September 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.25. Midostaurin - Orphan - EMEA-000780-PIP01-09-M08

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

Day 60 opinion

Oncology

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/0068/2024 of 8 March 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. Venetoclax - Orphan - EMEA-002018-PIP02-16-M06

Abbvie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms / Treatment of solid tumour malignant neoplasms

Day 60 opinion

Oncology

Note: Withdrawal request received on 14 June 2024

2.3.27. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M06

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology / 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/0267/2023 of 14 July 2023).

2.3.28. Bupropion hydrochloride / naltrexone hydrochloride - EMEA-001373-PIP01-12-M06

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed change to the

timelines of the quality 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/0294/2023 of 11 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.29. Ketamine / sufentanil - EMEA-001739-PIP02-16-M03

Cessatech A/S; Treatment of acute pain

Day 60 opinion

Pain

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 proposed changes to the timelines for Studies 6, 7 and 8 delaying the overall completion date of the PIP from August 2024 to April 2025 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/0320/2023 of 11 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.30. Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M05

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

Day 60 opinion

Pain

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/0160/2023 of 12 May 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.31. Benralizumab - EMEA-001214-PIP04-19-M01

AstraZeneca AB; Treatment of hypereosinophilic syndrome

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 most of 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/0035/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.32. Donidalorsen - Orphan - EMEA-003112-PIP01-21-M01

Otsuka Pharmaceutical Netherlands B.V.; 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 some of the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on modification of the agreed PIP as set in the Agency's latest decision (P/0274/2022 of 10 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.33. COVID-19 vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M04

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO agreed with the applicant's request to modify the PIP into a waiver for the entire paediatric population based on the grounds of lack of significant therapeutic benefit over existing vaccines.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0559/2021 of 27 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.34. *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-M01

Sanofi Pasteur; Prevention of meningococcal disease (serogroups A, B, C, W and Y)

Day 60 opinion

Vaccines

Note: Withdrawal request received on 28 June 2024

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. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-C1-002780-PIP02-20-M01

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

Day 30 letter

Vaccines

2.7.2. Selumetinib - EMEA-C2-001585-PIP01-13-M06

AstraZeneca AB; Treatment of neurofibromatosis type 1

Day 30 letter

Oncology

2.7.3. Olezarsen (sodium) - EMEA-C1-003177-PIP01-21

Ionis Ireland Limited; Treatment of familial chylomicronaemia syndrome

Day 30 letter

Cardiovascular Diseases

2.7.4. Finerenone - EMEA-C1-001623-PIP03-20-M01

Bayer AG; Treatment of heart failure

Day 30 letter

Cardiovascular Diseases

2.7.5. Glucagon - EMEA-C2-001657-PIP01-14-M01

Amphastar France Pharmaceuticals; Treatment of hypoglycaemia

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.6. Sebetralstat - EMEA-C1-002723-PIP01-19-M02

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 30 letter

Haematology-Hemostaseology

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

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 90 discussion

Cardiovascular Diseases

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

Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Efinopegdutide - EMEA-003549-PIP01-23

Treatment of non-alcoholic steatohepatitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.4. Hydroxycarbamide - EMEA-003388-PIP01-23

Treatment of sickle cell disease

Day 90 discussion

Haematology-Hemostaseology

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

Prevention of hereditary angioedema attacks

Day 90 discussion

Haematology-Hemostaseology

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

Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Iptacopan - EMEA-002705-PIP06-23

Treatment of myasthenia gravis

Day 90 discussion

Neurology

3.1.8. Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003524-PIP01-23

Sensorion SA; Treatment of otoferlin gene-mediated hearing loss

Day 90 discussion

Other

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

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

Day 90 discussion

Other

3.1.10. Retatrutide - EMEA-003258-PIP02-23

Treatment of obesity

Day 90 discussion

Other

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

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

Day 90 discussion

Other / Cardiovascular Diseases

3.1.12. Luliconazole - EMEA-003604-PIP01-24

Treatment of onychomycosis

Day 60 discussion

Dermatology

3.1.13. 2-isopropyl-3H-naphtho[1,2-d]imidazole-4,5-dione - Orphan - EMEA-003618-PIP01-24

Abliva AB; Treatment of primary mitochondrial disease

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.14. Diazoxide choline - Orphan - EMEA-003614-PIP01-24

Soleno Therapeutics Europe Ltd.; Treatment of Prader-Willi syndrome

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Psychiatry / Neurology

3.1.15. Pegozafermin - EMEA-003619-PIP01-24

Treatment of metabolic dysfunction-associated steatohepatitis (MASH)

Day 60 discussion

Gastroenterology-Hepatology

3.1.16. Telitacicept - EMEA-002824-PIP02-24

Treatment of myasthenia gravis

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.17. Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding human Wiskott-Aldrich syndrome protein - EMEA-003620-PIP01-24

Treatment of Wiskott-Aldrich syndrome

Day 60 discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.18. EMEA-003609-PIP01-24

Treatment of respiratory tract disease caused by human respiratory syncytial virus

Day 60 discussion

Infectious Diseases

3.1.19. Obeldesivir - EMEA-003306-PIP02-24

Treatment of human respiratory syncytial virus (RSV)

Day 60 discussion

Infectious Diseases

3.1.20. EMEA-003615-PIP01-24

Treatment of Prader Willi syndrome

Day 60 discussion

Neurology

3.1.21. Frexalimab - EMEA-002945-PIP04-24

Treatment of multiple sclerosis

Day 60 discussion

Neurology

3.1.22. Gedatolisib - EMEA-003612-PIP01-24

Treatment of children with solid malignancies / Treatment of haematologic malignancies / Treatment of central nervous system malignancies

Day 60 discussion

Oncology

3.1.23. Letetresgene autoleucel - Orphan - EMEA-002476-PIP03-24

Adaptimmune B. V.; Treatment of soft tissue sarcoma

Day 60 discussion

Oncology

3.1.24. Rocatinlimab - EMEA-002886-PIP03-24

Treatment of asthma

Day 60 discussion

Pneumology - Allergology

3.1.25. Sargramostim - EMEA-003568-PIP02-24

Treatment of autoimmune pulmonary alveolar proteinosis (aPAP)

Day 60 discussion

Pneumology - Allergology

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

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

Day 30 discussion

Cardiovascular Diseases

3.1.27. Zerlasiran - EMEA-003627-PIP01-24

Atherosclerotic cardiovascular disease

Day 30 discussion

Cardiovascular Diseases

3.1.28. EMEA-003632-PIP01-24

Treatment of heart failure

Day 30 discussion

Cardiovascular Diseases

3.1.29. EMEA-003633-PIP01-24

Treatment of psoriasis

Day 30 discussion

Dermatology

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

Treatment of diabetic foot ulcer / Treatment of venous leg ulcer

Day 30 discussion

Dermatology

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

Zealand Pharma A/S; Treatment of congenital hyperinsulinism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Novo Nordisk A/S; Treatment of thalassemia

Day 30 discussion

Haematology-Hemostaseology

3.1.33. Sonelokimab - EMEA-002568-PIP03-24

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Treatment of *Clostridioides difficile* infection (CDI)

Day 30 discussion

Infectious Diseases

3.1.35. EMEA-003602-PIP01-24

Treatment of tuberculosis

Day 30 discussion

Infectious Diseases

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

Taysha Gene Therapies, Inc.; Treatment of Rett syndrome

Day 30 discussion

Neurology

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

ILTOO Pharma SAS; Treatment of amyotrophic lateral sclerosis (ALS)

Day 30 discussion

Neurology

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

NovaCurie GmbH; Treatment of low-grade glioma

Day 30 discussion

Neurology

3.1.39. EMEA-003631-PIP01-24

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

Day 30 discussion

Oncology

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

Treatment of multiple myeloma

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

3.1.42. Tafasitamab - Orphan - EMEA-002499-PIP03-24

Incyte Biosciences Distribution B.V; Treatment of mature B-cell neoplasms [excluding diffuse large B-cell lymphoma (DLBCL)]

Day 30 discussion

Oncology

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

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

Day 30 discussion

Ophthalmology

3.1.44. Tacrolimus - EMEA-003625-PIP01-24

Treatment of vernal keratoconjunctivitis

Day 30 discussion

Ophthalmology

3.1.45. Rupatadine / montelukast - EMEA-003629-PIP01-24

Treatment of allergic rhinitis

Day 30 discussion

Oto-rhino-laryngology

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

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

Day 30 discussion

Vaccines

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

Prevention of norovirus acute gastroenteritis

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

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

Day 30 discussion

Infectious Diseases

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

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

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

Roche Registration GmbH; Treatment of multiple sclerosis

Day 30 discussion

Neurology

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

GlaxoSmithKline (Ireland) Limited; Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

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

Boehringer Ingelheim International GmbH; Treatment of Netherton syndrome

Day 30 discussion

Dermatology

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

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

Day 30 discussion

Diagnostic

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

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Bristol-Myers Squibb Pharma EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

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

Day 30 discussion

Infectious Diseases

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

Mundipharma GmbH; Treatment of invasive candidiasis

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Infectious Diseases

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

Eli Lilly and Company Ltd; Treatment of migraine headaches

Day 30 discussion

Neurology

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

Pfizer Europe MA EEIG; Treatment of migraine headaches

Day 30 discussion

Neurology

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

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

Day 30 discussion

Neurology

3.3.17. Vamorolone - Orphan - EMEA-001794-PIP02-16-M08

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

Day 30 discussion

Neurology

Note: Withdrawal request received on 13 June 2024

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

Sarepta Therapeutics Ireland; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Neurology

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

Merck Healthcare KGaA; Treatment of malignant neoplasms of the central nervous system

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

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

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology

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

Miltenyi Biomedicine GmbH; Treatment of mature B-cell neoplasms

Day 30 discussion

Oncology

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

Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

Day 30 discussion

Other

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

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Day 30 discussion

Other / Haematology-Hemostaseology

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

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

Day 30 discussion

Vaccines

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

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

Day 30 discussion

Vaccines

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

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

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 08 July 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. Depemokimab - EMEA-06-2024

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

Add-on to inhaled corticosteroid-based maintenance therapy for COPD patients.

Summary of Committee discussion:

The applicability of the class waiver as referred to in the EMA decision CW/0001/2015, (all classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft-versus-host disease (bone-marrow) transplantation) to the planned therapeutic indication(s) was confirmed.
Other potential paediatric interests of this medicine suggested by PDCO: asthma, hypereosinophilic syndrome, eosinophilic granulomatosis with polyangiitis.

6.1.2. Leuprorelin acetate - EMEA-07-2024

Recordati Industria Chimica e Farmaceutica S.p.A.; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of prostate malignant neoplasms.

Treatment of hormone dependent advanced prostate cancer and for the treatment of high-risk localised and locally advanced hormone dependent prostate cancer in combination with

radiotherapy.

Summary of Committee discussion:

The applicability of the class waiver as referred to in the EMA decision CW/0001/2015 (all classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of prostate malignant neoplasms) to the planned therapeutic indication(s) was confirmed.
Other potential paediatric interests of this medicine suggested by PDCO: central precocious puberty.

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

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

No item

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

The Chair welcomed Shteryu Boyadzhiev as the new alternate for Bulgaria, Sima Naujokiene as the new alternate for Lithuania and Sine Buhl Næss-Schmidt as the new alternate for Denmark.

9.1.2. Vote by Proxy

None

9.1.3. Strategic Review and Learning Meeting (SRLM) – Budapest, Hungary 9-10 October 2024

Summary of Committee discussion:

The PDCO members were informed that SRLM under the Hungarian Presidency of the Council of the EU will be held in person on 9-10 October 2024 in Budapest, Hungary.

9.2. Coordination with EMA Scientific Committees or CMDh-v

9.2.1. Committee for Medicinal Products for Human Use (CHMP)

Summary of Committee discussion:

The list of PIP-related CHMP procedures starting in May 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 June 2024, was provided by a CHMP / PDCO member.

9.2.2. Pharmacovigilance Risk Assessment Committee (PRAC)

Scope: Committee consultation of the PRAC-approved final revision of GVP Module XVI on risk minimisation measures post-public consultation

Summary of Committee discussion:

The revision 3 of the GVP Module XVI and its new Addendum II, containing guidance on risk minimisation measures (Module) and methods for the effectiveness evaluation of the measures (Addendum) was shared with PDCO and adopted.

9.2.3. Joint resolution on the principles of conduct within EMA Scientific Committees, WPs and CMDx

Summary of Committee discussion:

The PDCO members noted the presentation on the principles of conduct within EMA Scientific Committees, WPs and CMDx.

9.2.4. EMA engagement methodologies for patients and healthcare professionals

Summary of Committee discussion:

The various methodologies available for engaging with patients, healthcare professionals and their organisations on medicine-specific issues was described to the PDCO. The Committee was invited to follow up with the Public and Stakeholder Engagement department with any questions or requests for patients and healthcare professionals.

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

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

9.3.2. Paediatric Formulation Operational Expert Group (PFOEG)

PDCO member: Brian Aylward (*ad interim*)

Summary of Committee discussion:

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

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

No item

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Summary of Committee discussion:

A presentation of two upcoming ITF meetings was circulated by email

9.3.5. Cardiovascular Working Party (CVS WP)

PDCO Chair: Brian Aylward

Summary of Committee discussion:

Under the supervision of the CVS WP a temporary drafting group (DG) was established. The DG is working on the revision of the Paediatric Addendum on pulmonary arterial hypertension. The Committee was informed about the two PDCO representatives nominated to contribute to the DG.

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. Feedback and status update from the PaedForm Project (EDQM)

Scope: Feedback from the Paediatric Formulary meeting held in Strasbourg (FR) on 5-6 March 2024 and current status

PDCO member: Siri Wang

Summary of Committee discussion:

Update on the PaedForm Project (EDQM) and the website European Paediatric Formulary

(edqm.eu) have been shared with the Committee for information.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The June 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

Summary of Committee discussion:

No item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q2/2024 Update of the Business Pipeline report for the human scientific committees

Summary of Committee discussion:

The business pipeline report for Q2/2024 was provided for information.

10. Any other business

10.1. Health Technology Assessment body (HTAb) / EMA workshop on uncertainty management

Scope: To inform the Committee on an upcoming HTAb / EMA workshop series on uncertainty management

Summary of Committee discussion:

The Committee was informed about a technical EMA/HTA workshop series on evidence needs.

10.2. Potential pitfalls from uncritical use of artificial intelligence (AI)

Summary of Committee discussion:

The current use of AI in regulatory medicine was presented, including its advantages and disadvantages. AI tools are getting better exponentially. These tools could bring a lot of

practical benefit if being mindful of the pitfalls.

10.3. Practical aspects of IRIS for PDCO Members

Scope: The PDCO will be informed about the practical aspects associated with the onboarding of paediatric procedures in IRIS

Summary of Committee discussion:

The Committee was instructed on the practical aspects associated with the Paediatric procedures being onboarded in IRIS.

10.4. Presenting efforts/work ongoing on a facilitation framework

Scope: The PDCO will be informed about ongoing considerations towards building a paediatric drug development facilitation framework

Summary of Committee discussion:

The Committee was informed about ongoing collaborative efforts in developing a facilitation framework, enabling science focused feasibility discussions in areas of high unmet medical needs between multiple industry and academia, with regulatory oversight, such that it supports timely completion of development efforts.

10.5. Real World Evidence, including DARWIN EU®

Summary of Committee discussion:

EMA provided the quarterly update on Real World Evidence (RWE)/DARWIN EU. The following topics were discussed: Paediatric specific data source, Pharmacogenetics pilot in DARWIN EU, upcoming/recent events. The PDCO noted the ongoing efforts to increase the number of paediatric data sources available in the DARWIN EU network. The National Neonate Database from the UK (NNRD) was specifically mentioned, as it was discussed in detail by the Neonates Working group (WG) breakout of the PDCO meeting. It was agreed that Neonates WG will come forward with 2 to 3 potential research questions to pilot in this NNRD database, in order to assess its suitability to answer critical regulatory questions in this special population.
In addition, EMA invited Committee members to bring forward potential evidence gaps encountered during ongoing or upcoming assessments where RWE might contribute. Proposals for research questions in the paediatric population should be sent via mail.

10.6. Real World Evidence (RWE) spinal muscular atrophy (SMA) registry study report

PDCO: Sabine Scherer

Summary of Committee discussion:

The PDCO members were updated about the discussion on the outcome of the SMA registry study and problems with the registry held at the COMP June 2024 plenary. The Committee was informed that the final report can be found in the HMA-EMA catalogue and a manuscript is under preparation.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

Procedures under assessment by the CHMP and relevant to paediatric patients were discussed.

11.2. Neonatology

Summary of Committee discussion:

The group discussed RWE databases in the area of neonatology, product related content as well as the progression of the draft of the neonatal guideline revision.

11.3. HIV

Summary of Committee discussion:

HIV breakout session was cancelled.

The Chair thanked all participants and closed the meeting.

12. List of participants

List of participants including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 25-28 June 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 discussion, final deliberations and voting on:	2.1.17. Zanubrutinib - EMEA-002354-PIP03-24 2.3.26. Venetoclax - Orphan - EMEA-002018-PIP02-16-M06 (withdrawn) 3.3.18. Avelumab - EMEA-001849-PIP02-15-M05
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No participation in discussion, final deliberations and voting on:	3.3.22. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04
Miroslav Weiss	Member	Croatia	No interests declared	
Irena Senecic-Cala	Alternate*	Croatia	No restrictions applicable to this meeting	
Maria Eleni Avraamidou	Alternate	Cyprus	No interests declared	
Tereza Bazantova	Member	Czechia	No interests declared	
Pavlina Chladová	Alternate	Czechia	No interests declared	
Louisa Braun Exner	Member	Denmark	No interests declared	
Jana Lass	Member	Estonia	No interests declared	
Liisa Saare	Alternate	Estonia	No interests declared	
Pauliina Lehtolainen-Dalkilic	Member	Finland	No interests declared	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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 participation in discussion, final deliberations and voting on:	2.3.16. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M07 3.1.18. EMEA-003609-PIP01-24 3.1.19. Obeldesivir - EMEA-003306-PIP02-24 3.1.46. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24
Sima Naujokiene	Alternate*	Lithuania	No interests declared	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
David Khan	Alternate	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in discussion, final deliberations and voting on:	3.1.18. EMEA-003609-PIP01-24 3.1.19. Obeldesivir - EMEA-003306-PIP02-24 3.1.46. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24
Francesca Rocchi	Member*	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jose Ignacio Malagon Calle	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Tomasz Grybek	Member	Patients' Organisation Representative	No interests declared	
Jaroslav Sterba	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Viviana Giannuzzi	Member*	Patients' Organisation Representative	No restrictions applicable to this meeting	
Patricia Felgueiras Seabra Durao	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Victoria Romero Pazos	Alternate	Patients' Organisation Representative	No interests declared	
Celine Chu	Expert	France	No interests declared	
Franziska Wolter	Expert	Germany	No interests declared	
Olga Kholmanskikh	Expert	Belgium	No interests declared	
Meeting run with support from relevant EMA staff				

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

13. Explanatory notes

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

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

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

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

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

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

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

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