28 June 2024
EMA/PDCO/274736/2024
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 28-31 May 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

Disclaimers

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

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

Note on access to documents

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

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1. Introductions

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

The Chair opened the meeting by welcoming all participants. The meeting was held remotely.

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

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

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

The Chair thanked the departing alternate for her contribution to the Committee.

1.2. Adoption of agenda

The agenda for 28-31 May 2024 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 23-26 April 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. Ensitrelvir - EMEA-003192-PIP02-23

Shionogi B.V.; Treatment of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee and taking into account the responses of the applicant to the Day 90 questions, the PDCO agreed on a paediatric investigation plan (PIP) with a deferral for ensitrelvir for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of coronavirus 2019 (COVID-19).

2.1.2. Lenacapavir - EMEA-002740-PIP02-23

Prevention of human immunodeficiency virus (HIV-1) infection

Day 120 opinion

Infectious Diseases

Note: Withdrawal request received on 21 May 2024

2.1.3. Udonitrectag lysine - Orphan - EMEA-002848-PIP01-20

Recordati Rare Diseases; Treatment of neurotrophic keratitis

Day 120 opinion

Ophthalmology

Note: Withdrawal request received on 21 May 2024

2.1.4. Mirdametinib - Orphan - EMEA-003525-PIP01-23

Springworks Therapeutics Ireland Limited; Treatment of neurofibromatosis type 1

Day 120 opinion

Other

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 the proposed medicine for the entire paediatric population from birth to less than 18 years of age, in the condition of treatment of neurofibromatosis type 1 was adopted. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.5. Derivative of azabicycloheptane-carboxamide - EMEA-003451-PIP01-23

Boehringer Ingelheim International GmbH; Treatment of bronchiectasis

Day 120 opinion

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 derivative of azabicycloheptane-carboxamide for children from 1 year of age to less than 18 years of age in the condition of treatment of bronchiectasis. The PIP contains one quality study, two clinical studies, one modelling study, and one extrapolation plan and a deferral. A waiver was also granted for children below 1 year of age, based on the fact that the disease does not occur in this age group.

2.1.6. Indapamide / ramipril - EMEA-003600-PIP01-24

Adamed Pharma S.A.; Treatment of hypertension

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

2.1.7. Allogeneic skin-derived ABCB5-positive dermal mesenchymal stromal cells - EMEA-002875-PIP02-24

RHEACELL GmbH & Co. KG; Treatment of venous leg ulcer

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of venous leg ulcer on the ground that the disease or condition does not occur in the paediatric population. The applicant agreed with the proposed extension of 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.

2.1.8. Autologous CD3-positive T cells transduced with a retroviral vector containing an anti-B cell maturation agent chimeric antigen receptor gene - EMEA-003593-PIP01-24

Nexcella, Inc.; Treatment of systemic light chain amyloidosis

Day 60 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 autologous CD3-positive T cells transduced with a retroviral vector containing an anti-B cell maturation agent chimeric antigen receptor gene, for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of systemic light chain amyloidosis, on the grounds that the disease or condition only occurs in adults. The PDCO recommended to expand the waiver to all pharmaceutical forms and all routes of administration, to which the applicant agreed.
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.

2.1.9. 3-tert-butyl-N-{(1R)-1-[4-(6-{6-[4-({1-[4-(2,4-dioxo-1,3-diazinan-1-yl)phenyl]piperidin-4-yl}methyl)piperazin-1-yl]pyridin-3-yl}-7H-pyrrolo[2,3-d]pyrimidin-4-yl)-2-methylphenyl]ethyl}-1,2,4-oxadiazole-5-carboxamide - EMEA-003596-PIP01-24

BeiGene Ireland Limited; Treatment of mature B-cell 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 condition 'treatment of mature B-cell neoplasms' on the grounds that the specific medicinal product is likely to be ineffective.
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 B-cell acute lymphoblastic leukaemia 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.

2.1.10. 4-Benzoyl-D-phenylalanyl-D-seryl-D-tryptophyl-D-seryl-2,3,4,5,6-pentafluoro-D-phenylalanyl-3-cyclohexyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-glutaminyl-D-arginyl-D arginine acetate - EMEA-003597-PIP01-24

CanBas Co., Ltd.; Treatment of pancreatic cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for 4-benzoyl-D-phenylalanyl-D-seryl-D-tryptophyl-D-seryl-2,3,4,5,6-pentafluoro-D-phenylalanyl-3-cyclohexyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-glutaminyl-D-arginyl-D arginine acetate for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of pancreatic cancer, on the grounds that the specified condition occurs only in adult population.
The applicant agreed to the extension of the scope of the waiver to cover 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.

2.1.11. Anti-human LAG-3 monoclonal antibody, human IgG4 isotype - EMEA-003598-PIP01-24

Beigene Ireland Ltd; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for anti-human LAG-3 monoclonal antibody, human IgG4 isotype for the treatment of lung cancer on the grounds that the disease does not occur in the paediatric population.
The PDCO recommended to grant the waiver for all pharmaceutical forms and all routes of administration to which the applicant agreed.
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.

2.1.12. Autologous CD3-positive T cells transduced with a retroviral vector containing an anti-B cell maturation agent chimeric antigen receptor gene - EMEA-003593-PIP02-24

Nexcella, Inc.; Treatment of multiple myeloma

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 autologous CD3-positive T cells transduced with a retroviral vector containing an anti-B cell maturation agent chimeric antigen receptor gene for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of multiple myeloma, on the grounds that the specified condition occurs only in adult population.
The applicant agreed to the extension of the scope of the waiver to cover 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.

2.1.13. Fulzerasib - EMEA-003594-PIP01-24

Zhejiang Genfleet Therapeutics Co. Ltd.; Treatment of colorectal cancer

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 for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of colorectal cancer on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).
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.

2.1.14. Trastuzumab-exatecan derivative antibody-drug conjugate - EMEA-003599-PIP01-24

BioNTech SE; Treatment of breast cancer / Treatment of endometrial cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for trastuzumab-exatecan derivative antibody-drug conjugate for all subsets of the paediatric population (0 to 18 years of age) in the conditions of treatment of breast cancer, treatment of endometrial cancer, on the grounds that these conditions occur only in adult population.
The applicant agreed to the extension of the scope of the waiver to cover 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.

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

Sanofi Winthrop Industrie; Prevention of influenza disease

Day 30 opinion

Vaccines

Summary of Committee discussion:

Based on the assessment of this application, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from 9 years of age to less than 18 years of age, in the condition of prevention of influenza disease was adopted.
The PDCO agreed on a waiver in a subset of children from birth to less than 9 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.16. Diclofenac (sodium) / thiocolchicoside - EMEA-003611-PIP01-24

DOC Generici S.r.l.; Treatment of musculoskeletal and connective tissue pain and discomfort

Day 30 opinion

Pain

Summary of Committee discussion:

The PDCO discussed at Day 30, during the May 2024 plenary meeting, an application for a full waiver for diclofenac (sodium) / thiocolchicoside for treatment of musculoskeletal and connective tissue pain and discomfort.
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 diclofenac (sodium) / thiocolchicoside for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of musculoskeletal and connective tissue pain and discomfort.
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. Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage - EMEA-003641-PIP01-24

GlaxoSmithKline Biologicals S.A.; Prevention of influenza infection

Day 30 opinion

Vaccines

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 aged 6 months to less than 18 years of age, in the condition of prevention of influenza infection was adopted.
The PDCO agreed on a waiver in a subset of children from birth to less than 6 months of age on the grounds that the specific medicinal product is likely to be ineffective.

2.2. Opinions on Compliance Check

2.2.1. Olipudase alfa - EMEA-C-001600-PIP01-13-M02

Sanofi B.V.; Treatment of Niemann-Pick disease

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:
- EMEA-C1-001600-PIP01-13-M02

The PDCO adopted on 31 May 2024 an opinion confirming the compliance of all studies (Study 1 - DFI13803, Study 2 - LTS13632 and Study 3 - Extrapolation measure to confirm extrapolability of adult results to paediatrics) in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0459/2020) of 1 December 2020.

2.2.2. Tedizolid phosphate - EMEA-C3-001379-PIP01-12-M08

MSD Europe Belgium SRL; Treatment of acute bacterial skin and skin structure infections

Day 60 letter

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the completed Studies 2 and 9 and considered that these are compliant with the latest Agency's Decision (P/0340/2023) of 17 August 2023.
The PDCO finalised this partially completed compliance procedure on 31 May 2024.

2.2.3. Vanzacaftor / tezacaftor / deutivacaftor - EMEA-C1-003052-PIP01-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 60 letter

Pneumology – Allergology

Summary of Committee discussion:

Based on the responses provided by the applicant, study listed in part B of this report were hereby confirmed to be compliant as set out in the Agency's Decision (P/0071/2022) of 11 March 2022.

2.2.4. Landiolol (hydrochloride) - EMEA-C-001150-PIP02-13-M05

AOP Orphan Pharmaceuticals GmbH; Treatment of supraventricular arrythmias

Day 30 opinion

Cardiovascular Diseases

Summary of Committee discussion:

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

- EMEA-C2-001150-PIP02-13-M04

The PDCO adopted on 31 May 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/0221/2023) of 14 June 2023.

2.2.5. Fordadistrogene movaparvovec - EMEA-C1-002741-PIP01-20-M02

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 60 letter

Neurology

Summary of Committee discussion:

The PDCO discussed Study 1 (C3391001) and considered that the measures which could be checked for compliance at this point in time (i.e. all elements except for study duration for

participants, statistical plan (i.e. 5-year post-treatment analyses), completion date) were compliant with the latest Agency's Decision (P/0073/2024) of 9 March 2024.

2.2.6. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) - EMEA-C-003623-PIP01-24

Seqirus Netherlands B.V.; Prevention of influenza infection

Day 30 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted on 31 May 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/0181/2024) of 16 May 2024.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Ralinepag - Orphan - EMEA-002432-PIP02-20-M01

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that 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/0244/2021 of 9 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Regadenoson - EMEA-000410-PIP01-08-M07

GE Healthcare AS; Diagnosis of myocardial perfusion disturbances

Day 60 opinion

Diagnostic / 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 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/0029/2024 of 29 January 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Pegvaliase - Orphan - EMEA-001951-PIP01-16-M03

BioMarin International Limited; Treatment of hyperphenylalaninaemia

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/0089/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. RAAV8-hUGT1A1 - Orphan - EMEA-002021-PIP01-16-M01

GENETHON; Treatment of Crigler-Najjar syndrome

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. Within this modification some elements of the non-clinical study and clinical studies were updated along with a delay in the completion.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0194/2021 of 10 May 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Vedolizumab - EMEA-000645-PIP01-09-M09

Takeda Pharma A/S; Treatment of ulcerative colitis / Treatment of Crohn's disease

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. The modification concerns some minor updates including delay in the completion dates of clinical studies.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0552/2021 of 31 December 2021).

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

2.3.6. Luspatercept - Orphan - EMEA-001521-PIP01-13-M07

Bristol-Myers Squibb Pharma EEIG; Treatment of myelodysplastic syndromes / Treatment of beta-thalassaemia

Day 60 opinion

Haematology-Hemostaseology

Note: Withdrawal request received on 30 May 2024

2.3.7. Vonicog alfa - EMEA-001164-PIP01-11-M08

Baxalta Innovations GmBH; Treatment of Von Willebrand 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 requested delays, some updates on the sample size and some updates on the modelling and simulation 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/0416/2023 of 25 October 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Sarilumab - EMEA-001045-PIP01-10-M04

Sanofi Winthrop Industrie; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 30 May 2024

2.3.9. Tofacitinib - EMEA-000576-PIP01-09-M16

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

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

timelines and updated wording for the statistical analysis of Study 7 and age-appropriate formulation 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/0308/2023 of 7 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Islatravir / doravirine - EMEA-002707-PIP01-19-M02

MSD Europe Belgium SRL; Treatment of human immunodeficiency virus-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/0073/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M06

ViiV Healthcare UK Limited; 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/0121/2023 of 14 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Tenofovir disoproxil fumarate / lamivudine / doravirine - EMEA-001695-PIP01-14-M06

MSD Europe Belgium SRL; Treatment of human immunodeficiency virus-1 (HIV-1) infection

Day 60 opinion

Infectious Diseases

Note: Withdrawal request received on 30 May 2024

2.3.13. Ublituximab - EMEA-002889-PIP02-20-M01

Neuraxpharm Pharmaceuticals, S.L.; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the May 2024 plenary meeting, a request for modification for ublituximab for the treatment of multiple sclerosis.
The PDCO confirmed all the conclusions reached at Day 30 and assessed the information the applicant provided between Day 30 and Day 60. 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/0317/2021 of 11 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M04

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

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the May 2024 plenary meeting, a request for modification for brexucabtagene autoleucel for the treatment of acute lymphoblastic leukaemia (ALL).
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/0377/2023 of 8 September 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Dostarlimab - EMEA-002463-PIP01-18-M02

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from the Day 30 discussion, taking also into account the additional information received by the applicant. 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/0211/2021 of 10 May 2021).

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

2.3.16. Epcoritamab - Orphan - EMEA-002907-PIP01-20-M03

AbbVie Ltd; Treatment of mature B-cell lymphoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from the Day 30 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/0415/2022 of 29 September 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Niraparib tosylate monohydrate - EMEA-002268-PIP02-18-M02

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from the Day 30 discussion, taking also into account the additional information received by the applicant.
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/0184/2021 of 10 May 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Ribociclib - EMEA-002765-PIP02-21-M01

Novartis Europharm Limited; Treatment of neuroblastoma

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 agreed to the modification request to closing the early phase PIP Study 3 and delete the included pivotal clinical trial in light of the

generated data indicating lack of potential significant therapeutic benefit in context of current treatment options in use and under development for the specific PIP condition.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0020/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Botaretigene sparoparvovec - Orphan - EMEA-002827-PIP01-20-M03

Janssen-Cilag International NV Turnhoutseweg 30; Treatment of retinitis pigmentosa

Day 60 opinion

Ophthalmology

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/0389/2023 of 8 September 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Iptacopan - Orphan - EMEA-002705-PIP01-19-M02

Novartis Europharm Limited; Treatment of C3 glomerulopathy

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 requested delays and the inclusion of cardiovascular monitoring measures 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/0164/2023 of 15 May 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16-M05

Lupin Europe GmbH; Treatment of myotonic disorders

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes (timeline changes to some clinical studies and the extrapolation study, as well as changes to secondary endpoints of Study 5 and dosing instructions for Study 7) 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/0300/2023 of 11 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Setrusumab - Orphan - EMEA-002169-PIP01-17-M03

Mereo BioPharma Ireland Limited; Treatment of osteogenesis imperfecta

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0536/2022 of 30 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Xylitol / procaine hydrochloride / magnesium sulphate heptahydrate / potassium chloride - EMEA-001171-PIP01-11-M03

MIT Gesundheit GmbH; Cardioplegia

Day 60 opinion

Other

Note: Withdrawal request received on 29 May 2024

2.3.24. *Dermatophagoides farinae* extracts - EMEA-000834-PIP01-10-M01

Allergopharma GmbH & Co. KG; Treatment of allergic rhinitis / rhinoconjunctivitis

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 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 (P/285/2010 of 3 December 2010).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.25. *Dermatophagoides pteronyssinus* extracts 100% - EMEA-000835-PIP01-10-M01

Allergopharma GmbH & Co. KG; Treatment of allergic rhinitis / rhinoconjunctivitis

Day 60 opinion

Pneumology - Allergology

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 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 (P/286/2010 of 3 December 2010).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. *Dermatophagoides pteronyssinus / Dermatophagoides farinae* extracts (50%/50%) - EMEA-000836-PIP01-10-M01

Allergopharma GmbH & Co. KG; Treatment of allergic rhinitis / rhinoconjunctivitis

Day 60 opinion

Pneumology - Allergology

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 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 (P/287/2010 of 3 December 2010).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.27. Cariprazine hydrochloride - EMEA-001652-PIP01-14-M06

Gedeon Richter Plc.; Treatment of schizophrenia

Day 60 opinion

Psychiatry

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 partially accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0162/2023 of 12 May 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Ravulizumab (ALXN1210) - EMEA-001943-PIP02-20-M03

Alexion Europe SAS; Treatment in haematopoietic stem cell transplant

Day 60 opinion

Uro-nephrology / Haematology-Hemostaseology

Note: Withdrawal request received on 14 May 2024

2.3.29. NVX-CoV2373 - EMEA-002941-PIP01-20-M05

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

The applicant agreed to the requests made by the PDCO at Day 30. Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0423/2023 of 27 October 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.30. SARS-CoV-2 virus recombinant spike protein receptor binding domain fusion homodimer – XBB.1.16-XBB.1.16 variant / SARS-CoV-2 virus recombinant spike protein receptor binding domain fusion heterodimer (B.1.351 and B.1.1.7 strains) (beta-alpha variants) - EMEA-003191-PIP01-22-M01

HIPRA Human Health S.L.U.; 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 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/0465/2022 of 4 November 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.31. 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-M04

Sanofi Pasteur; Prevention of influenza disease

Day 30 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/0098/2024 of 5 April 2024).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.32. Ibrexafungerp - EMEA-002535-PIP03-19-M01

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

Day 30 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 (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/0164/2020 of 17 April 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.33. Gefurulimab - EMEA-003302-PIP01-22-M01

Alexion Europe SAS; Treatment of myasthenia gravis

Day 30 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes (minor changes to Study 1 and addition of deferral to Study 2) 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/0198/2023 of 22 May 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. Onasemnogene abeparvovec - EMEA-C2-002168-PIP01-17-M05

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 30 letter

Neurology

2.7.2. Gadoquatrane - EMEA-C1-002778-PIP01-20-M01

Bayer AG; Diagnosis by evaluation of any known or suspected clinical condition with contrast enhanced magnetic resonance imaging

Day 30 letter

Diagnostic

2.7.3. Ustekinumab - EMEA-C2-000311-PIP04-13-M06

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

Day 30 letter

Gastroenterology-Hepatology

2.7.4. Acetyl-L-leucine - EMEA-C1-002796-PIP01-20-M02

IntraBio Ltd; Treatment of Niemann-Pick disease type C

Day 30 letter

Neurology

2.7.5. Iptacopan - EMEA-C1-002705-PIP02-19-M01

Novartis Europharm Limited; Treatment of IgA nephropathy

Day 30 letter

Uro-nephrology

2.7.6. Lomitapide - EMEA-C1-001124-PIP01-10-M05

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.7. Brexpiprazole - EMEA-C3-001185-PIP01-11-M08

Otsuka Pharmaceutical Development & commercialisation Europe GmbH; Treatment of

schizophrenia

Day 30 letter

Psychiatry

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. Transglutaminase 2 inhibitor - EMEA-003513-PIP01-23

Treatment of coeliac disease

Day 90 discussion

Gastroenterology-Hepatology

3.1.2. Mezagitamab - EMEA-003502-PIP01-23

Treatment of immune thrombocytopenia (ITP)

Day 90 discussion

Haematology-Hemostaseology

3.1.3. Ganaxolone - EMEA-002341-PIP03-23

Treatment of refractory status epilepticus

Day 90 discussion

Neurology

3.1.4. Vosoritide - EMEA-002033-PIP02-23

Treatment of hypochondroplasia

Day 90 discussion

Other

3.1.5. A/H1N1, A/H3N2, and B/Victoria and 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

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

Day 90 discussion

Vaccines

3.1.6. Teplizumab - EMEA-000524-PIP02-24

Prevention of stage 3 type 1 diabetes mellitus

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. Interleukin-23 receptor antagonist peptide - EMEA-003301-PIP02-24

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

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.8. EMEA-003478-PIP02-24

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

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.9. EMEA-003601-PIP01-24

Prevention of *Clostridioides difficile* infection

Day 60 discussion

Infectious Diseases

3.1.10. Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP02-24

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy

Day 60 discussion

Neurology

3.1.11. Trastuzumab deruxtecan - EMEA-002978-PIP02-24

Treatment of all conditions in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 discussion

Oncology

3.1.12. 2′-O, 4′-C-methylene-P-thio-adenylyl-(3′→5′)-2′-O, 4′-C-methylene-P-thio-guanylyl-(3′→5′)-2′-O, 4′-C-methylene-P-thio-adenylyl-(3′→5′)-2′-deoxy-P-thio-adenylyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-guanylyl-(3′→5′)-2′-deoxy-P-thio-guanylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenylyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)- 2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-O, 4′-C-methylene-5-methyl-P-thio-cytidylyl-(3′→5′)-2′-O, 4′-C-methylene-5-methyl-P-thio-uridylyl-(3′→5′)-2′-O, 4′-C-methylene-5-methyl-P-thio-uridylyl-(3′→5′)-2′-O, 4′-C-methyleneguanosine, heptadecasodium salt - Orphan - EMEA-003595-PIP01-24

Ultragenyx Germany GmbH; Treatment of Angelman syndrome

Day 60 discussion

Other

3.1.13. Gorilla adenovirus vector expressing HPV6 and HPV11 antigens - Orphan - EMEA-003592-PIP01-24

Precigen, Inc.; Treatment of respiratory papillomatosis

Day 60 discussion

Oto-rhino-laryngology

3.1.14. Lebrikizumab - EMEA-002536-PIP03-24

Treatment of perennial allergic rhinitis

Day 60 discussion

Pneumology - Allergology

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

Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

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

Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.17. Luliconazole - EMEA-003604-PIP01-24

Treatment of onychomycosis

Day 30 discussion

Dermatology

3.1.18. Tarumase - EMEA-003616-PIP01-24

Treatment of chronic wounds

Day 30 discussion

Dermatology

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

Curium Pet France; Diagnosis of neuroendocrine neoplasms

Day 30 discussion

Diagnostic

3.1.20. 2-isopropyl-3H-naphtho[1,2-d]imidazole-4,5-dione - Orphan - EMEA-003618-PIP01-24

Abliva AB; Treatment of primary mitochondrial disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.21. Alvelestat - EMEA-003605-PIP01-24

Treatment of alpha-1 antitrypsin deficiency

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.22. 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 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Psychiatry / Neurology

3.1.24. Pegozafermin - EMEA-003619-PIP01-24

Treatment of metabolic dysfunction-associated steatohepatitis (MASH)

Day 30 discussion

Gastroenterology-Hepatology

3.1.25. Inobrodib - EMEA-003622-PIP01-24

Treatment of multiple myeloma

Day 30 discussion

Haematology-Hemostaseology

3.1.26. Telitacicept - EMEA-002824-PIP02-24

Treatment of myasthenia gravis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.27. 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 30 discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.28. EMEA-003609-PIP01-24

Treatment of respiratory tract disease caused by human respiratory syncytial virus

Day 30 discussion

Infectious Diseases

3.1.29. Obeldesivir - EMEA-003306-PIP02-24

Treatment of human respiratory syncytial virus (RSV)

Day 30 discussion

Infectious Diseases

3.1.30. EMEA-003615-PIP01-24

Treatment of Prader Willi syndrome

Day 30 discussion

Neurology

3.1.31. Frexalimab - EMEA-002945-PIP04-24

Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.1.32. Acasunlimab - EMEA-003606-PIP01-24

Treatment of lung cancer

Day 30 discussion

Oncology

3.1.33. Buparlisib - EMEA-003607-PIP01-24

Treatment of head and neck epithelial malignant neoplasms

Day 30 discussion

Oncology

3.1.34. Gedatolisib - EMEA-003612-PIP01-24

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

Day 30 discussion

Oncology

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

Daiichi Sankyo Europe GmbH; Treatment of lung cancer

Day 30 discussion

Oncology

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

Adaptimmune B. V.; Treatment of soft tissue sarcoma

Day 30 discussion

Oncology

3.1.37. Rocatinlimab - EMEA-002886-PIP03-24

Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.1.38. Sargramostim - EMEA-003568-PIP02-24

Treatment of autoimmune pulmonary alveolar proteinosis (aPAP)

Day 30 discussion

Pneumology - Allergology

3.1.39. Iloperidone - EMEA-000995-PIP02-24

Treatment of bipolar disorder

Day 30 discussion

Psychiatry

3.2. Discussions on Compliance Check

The following compliance checks have been identified for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance.

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. 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 30 discussion

Diagnostic

3.3.2. 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 30 discussion

Diagnostic

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

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Sanofi B.V; Treatment of achondroplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Boehringer Ingelheim International GmbH; Treatment of obesity

Day 30 discussion

Gastroenterology-Hepatology

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

AstraZeneca AB; Treatment of eosinophilic esophagitis

Day 30 discussion

Gastroenterology-Hepatology

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

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

Day 30 discussion

Gastroenterology-Hepatology

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

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

Day 30 discussion

Haematology-Hemostaseology

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

Pfizer Europe MA EEIG; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

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

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

Day 30 discussion

Haematology-Hemostaseology

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

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

Day 30 discussion

Haematology-Hemostaseology

3.3.14. 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 30 discussion

Immunology-Rheumatology-Transplantation

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

LFB Biotechnologies; Treatment of primary immunodeficiency (PID)

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Infectious Diseases

3.3.20. 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 30 discussion

Infectious Diseases

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

Angelini Pharma S.p.A; Treatment of focal onset seizures (FOS) / Treatment of primary generalised tonic clonic seizures (PGTC)

Day 30 discussion

Neurology

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

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

Day 30 discussion

Neurology

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

argenx BV; Treatment of generalised myasthenia gravis

Day 30 discussion

Neurology

3.3.24. Sodium ({(2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2-yl}oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) - Orphan - EMEA-003344-PIP01-22-M01

AbbVie Limited; Treatment of vanishing white matter disease

Day 30 discussion

Neurology

3.3.25. 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 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

Note: Withdrawal request received on 13 June 2024

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

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

Day 30 discussion

Oncology / Haematology-Hemostaseology

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

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 30 discussion

Other

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

Cessatech A/S; Treatment of acute pain

Day 30 discussion

Pain

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

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

Day 30 discussion

Pain

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

AstraZeneca AB; Treatment of hypereosinophilic syndrome

Day 30 discussion

Pneumology - Allergology

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

Otsuka Pharmaceutical Netherlands B.V.; Treatment of hereditary angioedema

Day 30 discussion

Pneumology - Allergology

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

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

Day 30 discussion

Vaccines

3.3.34. *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 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 27 May 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. Fezolinetant - EMEA-04-2024

Astellas Pharma Europe B.V.; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms.

Treatment of moderate to severe vasomotor symptoms in women with breast cancer on adjuvant endocrine therapy.

Summary of Committee discussion:

The applicability of the class waiver as referred to in the EMA decision (CW/0001/2015; classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant neoplasms), to the planned therapeutic indication(s) was not confirmed.

This was based on the consideration that in view of its mechanism of action consisting of blocking neurokinin B (NKB) binding on the kisspeptin/neurokinin B/dynorphin (KNDy) neurons to modulate neuronal activity in the thermoregulatory centre of the brain, it is considered that fezolinetant does not belong to any of the pharmacological classes of medicinal products for the treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant neoplasms in the decision CW/0001/2015.
Other potential paediatric interests of this medicine suggested by PDCO: polycystic ovary syndrome.

6.1.2. AVD-104 (PLGA-PEG-PSA) a ligand-presenting nanoparticle - EMEA-05-2024

Aviceda Therapeutics, Inc.; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema.

Treatment of geographic atrophy secondary to age-related macular degeneration.

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 age-related macular degeneration and diabetic macular oedema) to the planned therapeutic indication(s) was confirmed.

Other potential paediatric interests of this medicine suggested by PDCO: treatment of diabetic macular oedema.

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 thanked Anne Paavola for her contribution as an alternate for Finland.

9.1.2. Vote by Proxy

None

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

Summary of Committee discussion:

Feedback was noted from the PDCO SRLM under the Belgian Presidency of the Council of the EU held in person on 16-17 May 2024 in Leuven, Belgium.

9.1.4. Scientific Committee Meetings – alternating face-to-face and virtual meetings schedule for 2025

Summary of Committee discussion:

The Committee was informed on the alternating face-to-face and virtual meetings schedule for 2025.

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 April 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 May 2024, was provided by a CHMP / PDCO member.

9.2.2. Committee on Herbal Medicinal Products (HMPC) - Reflection paper on data recommendations for (traditional) herbal medicinal products in children/adolescents

PDCO Chair: Brian Aylward

Summary of Committee discussion:

The Committee members were informed about the next steps of the reflection paper.

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

Action: For information

Summary of Committee discussion:

Three upcoming ITF meetings 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. European Directorate for the Quality of Medicines & HealthCare (EDQM)

PDCO member: Siri Wang

Summary of Committee discussion:

The topic has been postponed to July 2024 PDCO meeting.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The May 2024 agenda and April 2024 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

No item

10. Any other business

10.1. Changes to the labelling of benzyl alcohol in paediatric formulations

PDCO member: Karen Van Malderen

Summary of Committee discussion:

The PDCO agreed to issue a formal request to the Non-clinical Working Party

(NcWP)/Excipients drafting group to revisit the labelling for medicinal products (including IV formulations) containing benzyl alcohol.

10.2. Real World Evidence, including DARWIN EU®

Summary of Committee discussion:

The PDCO noted the feedback on the Joint Heads of Medicines Agencies (HMA)/European Medicines Agency (EMA) Multistakeholder Workshop on Patient Registries, held on 12-13 February 2024. The report is planned to be published in June 2024.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

Paediatric oncology breakout session was cancelled.

11.2. Neonatology

Summary of Committee discussion:

The group continued its discussion on the revision of the neonatal guideline.

11.3. Internal PDCO Operations

Summary of Committee discussion:

The Committee discussed topics related to internal operations.

11.4. HIV

Summary of Committee discussion:

The groups discussed ongoing procedures for PIPs in the treatment and preventions therapeutic area of HIV infection 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 28-31 May 2024 PDCO meeting, which was held remotely.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Brian Aylward	Chair	Ireland	No interests declared	
Johanna Wernsperger	Member	Austria	No interests declared	
Agnes Gyurasics	Alternate	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in discussion, final deliberations and voting on:	2.3.16. Epcoritamab - Orphan - EMEA-002907-PIP01-20-M03 3.3.26. Venetoclax - Orphan - EMEA-002018-PIP02-16-M06
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 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	
Vacant	Alternate	France		
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Adrienn Horváth	Member	Hungary	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Sara Galluzzo	Member	Italy	No interests declared	
Cinzia Ciceroni	Alternate	Italy	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	
Marek Migdal	Member	Poland	No restrictions applicable to this meeting	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Peter Szitanyi	Alternate	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Sara Vennberg	Member	Sweden	No interests declared	
David Khan	Alternate	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in discussion, final deliberations and voting on:	3.1.28. EMEA-003609-PIP01-24 3.1.29. EMEA-003306-PIP02-24
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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 participation in discussion, final deliberations and voting on:	2.3.14. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M04 2.3.17. Niraparib tosylate monohydrate - EMEA-002268-PIP02-18-M02 2.3.18. Ribociclib - EMEA-002765-PIP02-21-M01
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	
Susanne Kaul	Expert	Germany	No restrictions applicable to this meeting	
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/