31 May 2024
EMA/PDCO/222146/2024
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 23-26 April 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 member.

1.2. Adoption of agenda

The agenda for 23-26 April 2024 meeting was adopted with amendments.

Topic added:

10.5 Judgments of the Court of Justice of 22 June 2023 (C-6/21 P and C-16/21 P) and 14 March 2024 (Case C-291/22 P)

1.3. Adoption of the minutes

The minutes for the 19-22 March 2024 meeting were adopted with amendments 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. Derivative of pyrindin-2-yl)cyclopropanecarboxamide hydrochloride - EMEA-003480-PIP01-23

Alumis, Inc.; Treatment of psoriasis

Day 120 opinion

Dermatology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for derivative of pyrindin-2-yl)cyclopropanecarboxamide hydrochloride (ESK-001), for the paediatric population from 6 years to less than 18 years of age in the condition treatment of psoriasis was adopted.
The PDCO agreed on a waiver in the paediatric population from birth to less than 6 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO granted a deferral for some measures contained in this PIP.

2.1.2. Zasocitinib - EMEA-003478-PIP01-23

Takeda Pharma A/S; Treatment of psoriasis

Day 120 opinion

Dermatology

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 zasocitinib in the condition of treatment of psoriasis with a waiver for the paediatric population from birth to less than 6 years on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments, and a deferral of one or more measures contained in the PIP.

2.1.3. Human alpha-1 proteinase inhibitor, modified (SerpinPC) - EMEA-003463-PIP01-23

ApcinteX Ltd; Treatment of haemophilia B

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 human alpha-1 proteinase inhibitor, modified (SerpinPC) for children aged 6 months to less than 18 years of age, in the condition of treatment of haemophilia B 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. The PDCO granted a deferral for one or more measures contained in this PIP.

2.1.4. Ianalumab - EMEA-002338-PIP05-23

Novartis Europharm Limited; Treatment of immune thrombocytopenia

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

In the written response, the applicant addressed some of 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 ianalumab for the paediatric population from 5 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 children from birth to less than 5 years of age 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.
The PDCO granted a deferral for one or more measures contained in this paediatric investigation plan (PIP).

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

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

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for mavorixafor for children aged 2 years to less than 18 years, in the condition of treatment of warts, hypogammaglobulinemia, infections and myelokathexis (WHIM) syndrome, was adopted.
The PDCO agreed on a waiver in a subset of children from birth to less than 2 years on the grounds that the specific medicinal product is likely to be unsafe. The PDCO granted a deferral for one of the measures contained in this PIP.

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

Marinus Pharmaceuticals, Inc.; Treatment of tuberous sclerosis complex

Day 120 opinion

Neurology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for the proposed medicine for children from 1 months to less than 18 years of age, in the condition of treatment of tuberous sclerosis complex was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 1 month of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.7. Radiprodil - EMEA-003462-PIP01-23

GRIN Therapeutics, Inc.; Treatment of GRIN-related disorders

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the April 2024 plenary meeting, an application for a stepwise paediatric investigation plan (sPIP) (within the sPIP pilot) and request for a waiver and a deferral for radiprodil for treatment of GRIN related disorder.
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 waiver for children less than 28 days of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible in the treatment of GRIN related disorder. The deferral was recommended to be refused as the product is being developed for a condition predominantly or exclusively affecting the paediatric population.

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

Myrtelle,Inc.; Treatment of Canavan disease

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the April 2024 plenary meeting the application for a paediatric investigation plan (PIP) with a deferral request for a recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA (MYR-101) for treatment of Canavan disease (CD).
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 stepwise PIP and refused to grant the deferral of the completion of this PIP.

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

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

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the April 2024 plenary meeting, a paediatric investigation plan (PIP) for brigimadlin for the treatment of soft-tissue sarcoma excluding liposarcoma.
The PDCO confirmed all conclusions reached at Day 90 and took into consideration the information the applicant provided between Day 90 and Day 120. Based on the assessment of this application, the PDCO adopted a positive opinion on a PIP for children from birth to less than 18 years of age, with a deferral for the treatment of soft tissue sarcoma excluding liposarcoma.

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

Prevention of migraine

Day 120 opinion

Pain / Neurology

Note: Withdrawal request received on 23 April 2024

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

Verisfield Single Member SA; Treatment of hypercholesterolaemia / Prevention of cardiovascular events

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

2.1.12. Gallium (^{68}Ga) boclatixafortide - EMEA-003408-PIP02-24

Pentixapharm AG; Diagnosis of primary aldosteronism

Day 60 opinion

Diagnostic

Summary of Committee discussion:

The PDCO discussed at Day 60, during the April 2024 plenary meeting the application for a product specific full waiver for gallium (^{68}Ga) boclatixafortide for diagnosis of a subtype of primary aldosteronism.
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 gallium (^{68}Ga) boclatixafortide for all subsets of the paediatric population (from birth to 18 years of age) for subtype diagnosis of primary aldosteronism based on lack of significant therapeutic benefit.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

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

Althera Laboratories Ltd; Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

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

2.1.14. 6-(4-((1s,3s)-1-amino-3-hydroxycyclobutyl)phenyl)-1-ethyl-7-phenyl-1H-pyrido [2,3-b][1,4]oxazin-2(3H)-one, L-tartrate salt - Orphan - EMEA-003585-PIP01-24

Vaderis Therapeutics AG; Treatment of hereditary haemorrhagic telangiectasia

Day 60 opinion

Haematology-Hemostaseology

Note: Withdrawal request received on 8 April 2024

2.1.15. (5aSa,17aRa)-20-chloro-2-[(2S,5R)-2,5-dimethyl-4-(prop-2-enoyl)piperazin-1-yl]-14,17-difluoro-6-(propan-2-yl)-11,12-dihydro-4H-1,18-(ethanediylidene)pyrido[4,3-e]pyrimido[1,6-g][1,4,7,9]benzodioxadiazacyclododecin-4-one (MK-1084) - EMEA-003586-PIP01-24

MSD Europe Belgium SRL; Treatment of all solid and haematological malignancies

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of all solid and haematological malignancies on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2.1.16. Actinium-225-2-(4,7,10-tris-carboxymethyl-1,4,7,10 tetraaza-cyclododec-1-yl)-pentanedioic acid 3-iodo-D-Tyr-D-Phe-D-Lys-OH)-8-oyl-ε-(HO-Glu-ureido-Lys-OH) - EMEA-003576-PIP01-24

Elvis Osei Tutu; Treatment of PSMA-expressing prostate 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 actinium-225-2-(4,7,10-tris-carboxymethyl-1,4,7,10 tetraaza-cyclododec-1-yl)-pentanedioic acid 3-iodo-D-Tyr-D-Phe-D-Lys-OH)-8-oyl-ε-(HO-Glu-ureido-Lys-OH) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of PSMA-expressing prostate cancer 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.

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

Lisata Therapeutics Ireland Limited; Treatment of pancreatic cancer

Day 60 opinion

Oncology

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

2.1.18. Derivative of 1,5,6,7-tetrahydro-4H-pyrrolo[3,2-c]pyridin-4-one - EMEA-003581-PIP01-24

Bayer AG; Treatment of lung cancer (small cell and non-small cell lung cancer)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the April 2024 plenary meeting, a product-specific waiver request for a derivative of 1,5,6,7-tetrahydro-4H-pyrrolo[3,2-c]pyridin-4-one for the treatment of lung cancer on the grounds that the disease does not occur in the paediatric population.
The PDCO confirmed all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 for 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.

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

XNK Therapeutics AB; Treatment of multiple myeloma

Day 60 opinion

Oncology

Note: Withdrawal request received on 9 April 2024

2.1.20. Rivoceranib - EMEA-002489-PIP02-24

Elevar Therapeutics Inc.; Treatment of hepatocellular carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

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

2.1.21. Telisotuzumab conjugated to (2S)-2-(2-bromoacetamido)-N-[(2S)-1-({3-[(7S)-7-ethyl-7-hydroxy-8,11-dioxo-7,8,11,13-tetrahydro-2H,10H-[1,3]dioxolo[4,5-g]pyrano[3',4':6,7]indolizino[1,2-b]quinolin-14-yl]bicyclo[1.1.1]pentan-1-yl}amino)-1-oxopropan-2-yl]-3-methylbutanamide (ABBV-400) - EMEA-003574-PIP01-24

AbbVie Ltd; Treatment of colorectal carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for telisotuzumab conjugated to (2S)-2-(2-bromoacetamido)-N-[(2S)-1-({3-[(7S)-7-ethyl-7-hydroxy-8,11-dioxo-7,8,11,13-tetrahydro-2H,10H-[1,3]dioxolo[4,5-g]pyrano[3',4':6,7]indolizino[1,2-b]quinolin-14-yl]bicyclo[1.1.1]pentan-1-yl}amino)-1-oxopropan-2-yl]-3-methylbutanamide (ABBV-400) for the treatment of colorectal carcinoma 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.

2.1.22. Lebrikizumab – EMEA-002536-PIP02-24

Almirall SA; Treatment of nasal polyposis

Day 60 opinion

Pneumology – Allergology

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 lebrikizumab for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of nasal polyposis on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.23. Fenofibrate / rosuvastatin calcium - EMEA-003591-PIP01-24

Verisfield Single Member S.A.; Treatment of elevated cholesterol with elevated triglycerides

Day 30 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO discussed at Day 30, during the April 2024 plenary meeting, an application for a full waiver for fenofibrate / rosuvastatin calcium for treatment of elevated cholesterol with elevated triglycerides.
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 fenofibrate / rosuvastatin calcium for all subsets of the paediatric population (birth to 18 years of age) in the condition of treatment of elevated cholesterol with elevated triglycerides on the grounds that no significant therapeutic benefit is expected in the paediatric population.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.24. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-003624-PIP01-24

Seqirus Netherlands B.V.; Prevention of influenza infection

Day 30 opinion

Vaccines

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 prevention of influenza infection on the grounds on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

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

Acadia Pharmaceuticals Inc.; Treatment of Rett syndrome

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 assessment of this application and the additional information provided by the applicant, the PDCO agreed in April 2024 on a positive opinion for the paediatric investigation plan (PIP) for trofinetide for the paediatric population from 2 years to less than 18 years of age in the condition of treatment of Rett syndrome. The PDCO agreed on a waiver in a subset of children below 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.26. Autologous T-cells from a melanoma metastasis (TM001) - EMEA-003535-PIP02-24

Netherlands Cancer Institute (NKI); Treatment of melanoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the April 2024 plenary meeting, a paediatric investigation plan (PIP) for autologous T-cells from a melanoma metastasis (TM001) for the treatment of melanoma.
The PDCO confirmed all the conclusions reached at Day 30, refused the PIP and granted a product-specific waiver on its own motion at Day 60 for this product for the treatment of melanoma on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients because clinical studies would not be 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.

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

Seqirus Netherlands; 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 influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) (surface antigen, inactivated, prepared in cell cultures) for children from 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 on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2.2. Opinions on Compliance Check

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

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

The PDCO adopted on 26 April 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/0488/2023) of 4 December 2023.

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

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

Day 60 letter

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO discussed the completed study(ies) and considered that these are compliant with the latest Agency's Decision (P/0178/2023) of 15 May 2023.
The PDCO finalised this partially completed compliance procedure on 26 April 2024.

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

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

Day 60 letter

Infectious Diseases

Summary of Committee discussion:

The PDCO rediscussed the completed Study 2 and the interim report of Study 6 and considered that these are compliant with the latest Agency's Decision (P/0257/2023) of 14 July 2023.
The PDCO finalised this partially completed compliance procedure on 26 May 2024.

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

Pierre Fabre Médicament; Treatment of melanoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO adopted on 26 April 2024 an opinion confirming the compliance of Study 3 (modelling and simulation study to evaluate and to determine the dose of binimetinib to be used in combination with encorafenib which matches adult plasma exposure and for the use of the products in the treatment of melanoma in adolescents from 12 to less than 18 years of age with unresectable or metastatic BRAF V600 mutant melanoma) in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0147/2023) of 21 April 2023.

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

Pierre Fabre Médicament; Treatment of melanoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO adopted on 26 April 2024 an opinion confirming the compliance of Study 3 (modelling and simulation study to evaluate and to determine the dose of binimetinib to be used in combination with encorafenib which matches adult plasma exposure and for the use of the products in the treatment of melanoma in adolescents from 12 to less than 18 years of age with unresectable or metastatic BRAF V600 mutant melanoma) in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0148/2023) of 21 April 2023.

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

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

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

- EMEA-C1-002330-PIP01-18
- EMEA-C2-002330-PIP01-18-M02
- EMEA-C3-002330-PIP01-18-M02

The PDCO adopted on 26 April 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/0246/2023) of 14 July 2023.

2.2.7. Baloxavir marboxil - EMEA-C-002440-PIP01-18-M05

Roche Registration GmbH; Prevention of influenza

Day 30 opinion

Infectious Diseases

Summary of Committee discussion:

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

- EMEA-C1-002440-PIP01-18

The PDCO adopted on 26 April 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/0359/2023) of 8 September 2023.

2.2.8. Canagliflozin - EMEA-C-001030-PIP01-10-M10

Janssen-Cilag International NV; Treatment of type 2 diabetes mellitus

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

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

- EMEA-C1-001030-PIP01-10-M07

The PDCO adopted on 26 April 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/0208/2022) of 10 June 2022.

2.2.9. Methoxyflurane - EMEA-C-000334-PIP01-08-M11

Medical Developments UK Ltd; Treatment of acute pain

Day 30 opinion

Pain

Summary of Committee discussion:

The PDCO adopted on 26 April 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/0080/2023) of 13 March 2023.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

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

MSD Europe Belgium SRL; Treatment of type 2 diabetes mellitus

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 changes on the definition of the study population to be included in the paediatric clinical trial, PIP 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/0141/2019 of 17 April 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification the applicant requested to revise the planned dates of completion for Studies 5 and 9, and amend endpoints and statistical analysis of Study 7.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0529/2022 of 30 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Horizon Therapeutics Ireland Designated Activity Company (DAC); Treatment of immunoglobulin G4-related 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/0531/2023 of 29 December 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 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 (timelines of all studies, and some elements of Studies 2 and 3) 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/0189/2012 of 22 August 2012).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0090/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

OHB Neonatology Ltd; Prevention of chronic lung disease of prematurity

Day 60 opinion

Neonatology - Paediatric Intensive Care

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/0066/2020 of 28 February 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Horizon Therapeutics Ireland Designated Activity Company (DAC); 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/0471/2023 of 1 December 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Soticlestat – Orphan – EMEA-002572-PIP02-19-M05

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

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 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/0251/2023 of 14 July 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Neurology

Note: Withdrawal request received on 16 April 2024

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

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO's view expressed at Day 30 was endorsed.
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/0259/2023 of 13 July 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed 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/0503/2020 of 22 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (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/0284/2022 of 11 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 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/0222/2022 of 24 June 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

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

Day 60 opinion

Vaccines / Infectious Diseases

Note: Withdrawal request received on 4 April 2024

2.3.15. Obefazimod - EMEA-003196-PIP01-22-M01

Abivax; Treatment of ulcerative colitis

Day 30 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. Within this modification the applicant requested to remove the condition Crohn's disease and the studies associated with this condition and therefore splitting the PIP into

two separate PIPs for ulcerative colitis and Crohn's disease. A separate PIP will be submitted for the condition treatment of Crohn's disease.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0519/2022 of 30 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

2.4.1. Orforglipron - EMEA-003299-PIP01-22

Eli Lilly and Company; Treatment of type 2 diabetes mellitus

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The re-examination consisted of requesting the deletion of the secondary endpoint of continuous glucose monitoring assessments for the clinical trial paediatric investigation plan (PIP) Study 1.
Based on the review of the grounds for re-examination the PDCO concluded that the proposed changes on the continuous glucose monitoring endpoint could not be accepted
The PDCO therefore maintained its previous opinion on the PIP.

2.4.2. Sevasemten - EMEA-003394-PIP01-23

FGK Representative Service GmbH Germany; Treatment of dystrophinopathies

Day 30 opinion

Other / Neurology

Summary of Committee discussion:

The PDCO discussed the grounds for the re-examinations provided by the applicant on the previously agreed paediatric investigation plan (PIP) opinion per sevasemten and agreed to revise its opinion and to agree to the PIP, to grant a deferral and to grant a waiver for the paediatric population from birth to less than 6 months of age in treatment of dystrophinopathies on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit and following re-examination, and to amend the measures.

2.4.3. 13 Grass aqueous extract - EMEA-000813-PIP01-09-M01

Allergy Therapeutics (UK) Ltd.; Treatment of allergic rhinitis/rhino-conjunctivitis

Day 30 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the assessment of grounds for re-examination of opinion and the additional information provided by the applicant, the PDCO considered that the opinion should be maintained.

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

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

Day 30 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed during the April 2024 plenary meeting, a request for re-examination for brexucabtagene autoleucel for the treatment of mature B-cell neoplasms.
The PDCO considered the arguments provided by the applicant and agreed with their request. Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0002/2021 of 5 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.5. 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. Chikungunya virus virus-like particle vaccine / aluminum hydroxide - EMEA-C1-002656-PIP01-19-M01

Bavarian Nordic A/S; Prevention of chikungunya disease

Day 30 letter

Vaccines / Infectious Diseases

2.7.2. Zuranolone - EMEA-C1-003119-PIP01-21-M01

Biogen Netherlands B.V.; Treatment of postpartum depression

Day 30 letter

Psychiatry

2.7.3. Ixazomib citrate - EMEA-C1-001410-PIP02-17-M04

Takeda Pharma A/S; Treatment of lymphoid malignancies (excluding multiple myeloma)

Day 30 letter

Oncology

2.7.4. Pridopidine (hydrochloride) - EMEA-C1-003174-PIP01-21-M01

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

Day 30 letter

Neurology

2.7.5. Chloroprocaine hydrochloride - EMEA-C2-000639-PIP03-16-M03

Sintetica GmbH; Peripheral nerve block (local anaesthesia by perineural injection)

Day 30 letter

Anaesthesiology

2.7.6. Fully human IgG1 RB-1 YTE anti-RSV F monoclonal antibody - EMEA-C1-002755-PIP01-19-M02

MSD Europe Belgium S.R.L.; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 30 letter

Infectious Diseases

2.7.7. Sepiapterin – Orphan - EMEA-C1-003027-PIP02-23

PTC Therapeutics International Limited; Treatment of hyperphenylalaninemia

Day 30 letter

Endocrinology – Gynaecology – Fertility – Metabolism

2.7.8. Sargramostim - EMEA-C1-003568-PIP01-23

Sargramostim Partner Therapeutics; Treatment of patients acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome – H-ARS)

Day 30 letter

Immunology – Rheumatology – Transplantation

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. Ensitrelvir - EMEA-003192-PIP02-23

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Infectious Diseases

3.1.2. Lenacapavir - EMEA-002740-PIP02-23

Prevention of human immunodeficiency virus (HIV-1) infection

Day 90 discussion

Infectious Diseases

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

Recordati Rare Diseases; Treatment of neurotrophic keratitis

Day 90 discussion

Ophthalmology

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

Springworks Therapeutics Ireland Limited; Treatment of neurofibromatosis type 1 - plexiform neurofibroma / Treatment of neurofibromatosis type 1

Day 90 discussion

Other

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

Treatment of bronchiectasis

Day 90 discussion

Pneumology - Allergology

3.1.6. EMEA-003580-PIP01-24

Treatment of elevated cholesterol / Treatment of mixed dyslipidaemia

Day 60 discussion

Cardiovascular Diseases

3.1.7. Ersodetug – Orphan - EMEA-002813-PIP02-24

Rezolute (Bio) Ireland Limited; Treatment of congenital hyperinsulinism

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 60 discussion

Gastroenterology-Hepatology

3.1.9. Mezagitamab - EMEA-003502-PIP02-24

Treatment of primary IgA nephropathy

Day 60 discussion

Haematology-Hemostaseology

3.1.10. Tildrakizumab - EMEA-001451-PIP02-24

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

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.11. 3,3-Dimethyl-N-(6-methyl-5-{[2-(1-methyl-1H-pyrazol-4-yl)pyridine-4-yl]oxy}pyridine-2-yl)-2-oxopyrrolidine-1-carboxamide hydrochloride hydrate - Orphan - EMEA-003495-PIP02-24

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

Day 60 discussion

Oncology

3.1.12. 7-ethyl-10-hydroxy-camptothecin - Orphan - EMEA-003588-PIP01-24

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

Day 60 discussion

Oncology

3.1.13. Ravulizumab - EMEA-001943-PIP07-24

Treatment of primary IgA nephropathy

Day 60 discussion

Uro-nephrology

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

Influenza immunisation

Day 60 discussion

Vaccines

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

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

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

Treatment of venous leg ulcer

Day 30 discussion

Dermatology

3.1.17. 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

Treatment of systemic light chain amyloidosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. Teplizumab - EMEA-000524-PIP02-24

Prevention of stage 3 type 1 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. 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 30 discussion

Immunology-Rheumatology-Transplantation

3.1.20. Zasocitinib - EMEA-003478-PIP02-24

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.21. EMEA-003601-PIP01-24

Prevention of *Clostridioides difficile* infection

Day 30 discussion

Infectious Diseases

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

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy

Day 30 discussion

Neurology

3.1.23. EMEA-003596-PIP01-24

Treatment of mature B-cell neoplasms

Day 30 discussion

Oncology

3.1.24. EMEA-003597-PIP01-24

Treatment of pancreatic cancer

Day 30 discussion

Oncology

3.1.25. Anti-human LAG-3 mAb, human IgG4 isotype - EMEA-003598-PIP01-24

Treatment of lung cancer

Day 30 discussion

Oncology

3.1.26. 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

Treatment of multiple myeloma

Day 30 discussion

Oncology

3.1.27. Fulzerasib - EMEA-003594-PIP01-24

Treatment of colorectal cancer

Day 30 discussion

Oncology

3.1.28. HER2 antibody drug conjugate - EMEA-003599-PIP01-24

Treatment of breast cancer / Treatment of endometrial cancer

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

3.1.30. 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 30 discussion

Other

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

Precigen, Inc.; Treatment of respiratory papillomatosis

Day 30 discussion

Oto-rhino-laryngology

3.1.32. Lebrikizumab - EMEA-002536-PIP03-24

Treatment of perennial allergic rhinitis

Day 30 discussion

Pneumology - Allergology

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

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

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Infectious Diseases

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

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

Pneumology - Allergology

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

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Neurology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

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

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

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

GE Healthcare AS; Diagnosis of myocardial perfusion disturbances

Day 30 discussion

Diagnostic / Cardiovascular Diseases

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

BioMarin International Limited; Treatment of hyperphenylalaninaemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. RAAV8-hUGT1A1 – Orphan – EMEA-002021-PIP01-16-M01

GENETHON; Treatment of Crigler-Najjar syndrome

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Gastroenterology-Hepatology

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

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

Day 30 discussion

Haematology-Hemostaseology

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

Baxalta Innovations GmBH; Treatment of Von Willebrand disease

Day 30 discussion

Haematology-Hemostaseology

3.3.8. 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 30 discussion

Immunology-Rheumatology-Transplantation

3.3.9. Tofacitinib citrate - 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 30 discussion

Immunology-Rheumatology-Transplantation

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

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

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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

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

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Neurology

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

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

Day 30 discussion

Oncology

3.3.15. 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 30 discussion

Oncology

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

AbbVie Ltd; Treatment of mature B-cell lymphoma

Day 30 discussion

Oncology

3.3.17. 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 30 discussion

Oncology

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

Novartis Europharm Limited; Neuroblastoma

Day 30 discussion

Oncology

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

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

Day 30 discussion

Ophthalmology

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

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Day 30 discussion

Other

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

Lupin Europe GmbH; Treatment of myotonic disorders

Day 30 discussion

Other

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

Mereo BioPharma Ireland Limited; Treatment of osteogenesis imperfecta

Day 30 discussion

Other

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

MIT Gesundheit GmbH; Cardioplegia

Day 30 discussion

Other

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

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

Day 30 discussion

Pneumology - Allergology

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

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

Day 30 discussion

Pneumology - Allergology

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

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

Day 30 discussion

Pneumology - Allergology

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

Gedeon Richter Plc.; Treatment of schizophrenia

Day 30 discussion

Psychiatry

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

Alexion Europe SAS; Treatment in haematopoietic stem cell transplant

Day 30 discussion

Uro-nephrology / Haematology-Hemostaseology

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

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

Day 30 discussion

Vaccines

3.3.30. COVID-19 Vaccine, recombinant, adjuvanted - EMEA-003191-PIP01-22-M01

HIPRA Human Health S.L.U.; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 29 April 2024 for Nomination of Rapporteur and Peer reviewer

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

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

5.1. New Scientific Advice

No item

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

No item

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

6.1.1. Cisplatin - EMEA-03- 2024

InhaTarget Therapeutics SRL; The class of first- and second generation platinum-containing medicinal products for treatment of lung malignant neoplasms; Treatment of non-small cell lung cancer (NSCLC)

Summary of Committee discussion:

The applicability of the class waiver as referred to in the Agency's decision CW/0001/2015 to the planned therapeutic indication was confirmed.
Other potential paediatric interests of this medicine suggested by PDCO: none identified at this stage.

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

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

7.1.1. Zibotentan / dapagliflozin (propanediol monohydrate) - EMEA-002969-PIP01-21

AstraZeneca AB; Treatment of chronic kidney disease

Proposed indication: Treatment of adults with primary immunoglobulin A nephropathy (IgAN) with a urine protein-to-creatinine ratio ≥1g/g

Summary of Committee discussion:

The PDCO confirmed that the proposed indication 'treatment of adults with primary immunoglobulin A nephropathy (IgAN) with a urine protein-to-creatinine ratio ≥1g/g', falls under the scope of the Agency Decision p/0249/2021, as the indication is considered to be covered by the condition 'treatment of chronic kidney disease' listed in the Agency Decision.

7.1.2. Autologous CD3+CD4+CD25+CD127-FoxP3+ polyclonal regulatory T cells ex vivo expanded - EMEA-002737-PIP01-19

PolTreg SA; Treatment of type 1 diabetes mellitus

Proposed indication: Treatment of presymptomatic (stage 1) diabetes mellitus type 1 (T1DM stage 1)

Summary of Committee discussion:

The Committee confirmed that the proposed indication of treatment of presymptomatic

(stage 1) diabetes mellitus type 1 does not fall under the agreed PIP condition of treatment of type 1 diabetes mellitus, but under the condition of prevention of type 1 diabetes mellitus.

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 Eric Vermeulen as a member representing patients' organisations.

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:

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

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Summary of Committee discussion:

The list of PIP-related CHMP procedures starting in March 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 April 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 member: Peter Sisovsky

Summary of Committee discussion:

The PDCO members emphasised concerns regarding the text in the reflection paper and the established EMA's understanding of extrapolation (including the measures developers need to adopt), as expressed in the EMA guidance documents, the ICH guideline E11A on paediatric extrapolation (draft, April 2022), and associated templates. In this regard, further analysis of possible changes to this issue on how to extrapolate in herbal medicinal products (HMPs) is needed, and eventually the involvement of other relevant EMA Committees/Working Groups/Working Parties as appropriate, to clarify the key points of the extrapolation paradigm that would need to be accurately reflected for HMPs.

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:

Two 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

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The April 2024 agenda of the cluster was shared with the PDCO members for information.

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

No item

9.8.2. Marketing Authorisation Applications (MAAs) 3-year forecast report (March 2024 to December 2026)

Summary of Committee discussion:

The Committee was informed about the availability of the 3-year forecast report for initial marketing authorisations (MAAs). The report covers MAAs expected up to December 2026, highlighting key feature of the pipeline and include information on Variations Type 2 and Line Extensions.

10. Any other business

10.1. Accelerating Clinical Trials (ACT) EU PA7 pilot between Scientific Advice Working Party (SAWP) and Clinical Trials Coordination Group (CTCG)

Summary of Committee discussion:

The Committee was informed about the upcoming pilot involving an interaction between SAWP and CTCG on relevant scientific advice procedures.

10.2. Onboarding of Paediatric processes on IRIS

Summary of Committee discussion:

The Committee was informed about the progress of onboarding paediatric procedures in IRIS and a brief live demo was provided.

10.3. Update on the Accelerating Clinical Trials (ACT) EU PA8 workshop

Summary of Committee discussion:

The PDCO members were updated on the paediatric related activities developed during the ACT EU PA8 workshop.

10.4. Feedback on training on PIPs for the Clinical Trials Coordination Group (CTCG) assessor round table

PDCO member: Anette Solli Karlsen

Summary of Committee discussion:

The Committee noted the feedback on the training that was provided to the CTCG assessor round table (ART) on 18 April 2024. The ART is a discussion forum for national competent authority (NCA) and Ethics Committee assessors. The training was recorded and will become available in due time. The training included: Use of medicinal products in children, Paediatric regulation and the paediatric committee (PDCO), The paediatric investigation plan (PIP) and assessment, Content of the agreed PIP, Key binding elements of a PIP and some reflections on aspects to consider when assessing a clinical trial that is part of a PIP.

Feedback that was received from the ART and included in the feedback to the PDCO:

- CTCG/commission will elaborate on whether the entire 'decision with annexes' document can be requested through validation of a CTA – to be followed up by the CTCG.
- There remains a need for training/discussion on the interpretation on CTR Article 32.
- The limited time frame for clarification/discussion with the PDCO complicates discussion on individual PIPs.
- It was also discussed how to handle assessment of clinical trials part of a PIP in clock stop/not yet submitted.

10.5. Judgments of the Court of Justice of 22 June 2023 (C-6/21 P and C-16/21 P) and 14 March 2024 (Case C-291/22 P)

Summary of Committee discussion:

The Committee was informed of two recent judgments of the Court of Justice on the application of the principle of (objective) impartiality to EMA's Scientific Advisory Groups (SAGs).

11. Breakout sessions

11.1. Internal PDCO Operations

Summary of Committee discussion:

The Committee discussed topics related to the internal PDCO operations and in particular the topic from the 2024 PDCO workplan on mechanism of action PIPs.

11.2. Neonatology

Summary of Committee discussion:

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

11.3. HIV

Summary of Committee discussion:

The group discussed procedures related to the HIV therapeutic area under discussion at the current meeting.

11.4. Paediatric oncology

Summary of Committee discussion:

Recent workshops related to paediatric oncology was discussed within the group.

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 23-26 April 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 restrictions applicable to this meeting	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Miroslav Weiss	Member	Croatia	No interests declared	
Irena Senecic-Cala	Alternate	Croatia	No participation in discussion, final deliberations and voting on:	2.3.2. Linaclotide - EMEA-000927-PIP01-10-M08
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	
Liisa Saare	Alternate	Estonia	No interests declared	
Pauliina Lehtolainen-Dalkilic	Member	Finland	No interests declared	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Mountaki	Alternate	Greece	No interests declared	
Adrienn Horváth	Member	Hungary	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Cinzia Ciceroni	Alternate	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No restrictions applicable to this meeting	
Maaike Van Dartel	Alternate	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No restrictions applicable to this meeting	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Peter Szitanyi	Alternate	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Sara Vennberg	Member	Sweden	No interests declared	
David Khan	Alternate	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
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:	2.4.4. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP03-20-M02 3.3.14. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M04 3.3.17. Niraparib tosylate monohydrate - EMEA-002268-PIP02-18-M02
Viviana Giannuzzi	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Patricia Felgueiras Seabra Durao	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Victoria Romero Pazos	Alternate	Patients' Organisation Representative	No interests declared	
Celine Chu	Expert	France	No interests declared	
Birgit Ahrens	Expert	Germany	No restrictions applicable to this meeting	
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/