22 March 2024
EMA/PDCO/98496/2024
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 20-23 February 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 welcomed the new member and alternate.

1.2. Adoption of agenda

The agenda for 20-23 February 2024 meeting was adopted with amendments.

Topic added in section 5.1.

1.3. Adoption of the minutes

The minutes for 16-19 January 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. Navepegritide - Orphan - EMEA-002689-PIP02-23

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the final version of this modified paediatric investigation plan (PIP) with a deferral. A positive opinion was adopted accordingly.

2.1.2. Orforglipron - EMEA-003299-PIP01-22

Eli Lilly and Company; Treatment of type 2 diabetes mellitus

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the paediatric investigation plan (PIP) for orforglipron, for the paediatric population from 10 years to less than 18 years of age in the condition treatment of type 2 diabetes mellitus was adopted.
The PDCO agreed on a waiver in the paediatric population from birth to less than 10 years of age on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subsets.
The PDCO granted a deferral for some measures contained in this PIP.

2.1.3. Belimumab - EMEA-000520-PIP03-23

GlaxoSmithKline (Ireland) Limited; Treatment of systemic sclerosis

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

A paediatric investigation plan (PIP) was agreed for belimumab for the treatment of systemic sclerosis in patients from 8 years to less than 18 years of age. A waiver was granted for the paediatric population from birth to less than 8 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible. The agreed PIP includes one clinical study (single-arm PK, PD study) and a modelling and simulation study, which are part of an extrapolation plan covering the paediatric population from 8 years to less than 18 years of age. A deferral for one or more measures contained in the PIP has been granted.

2.1.4. Iodine (^{131}I) apamistamab - Orphan - EMEA-003395-PIP02-23

Immedica Pharma AB; Treatment in allogenic stem cell transplantation

Day 120 opinion

Immunology-Rheumatology-Transplantation / Oncology

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 patients from 3 years to less than 18 years of age in the condition of treatment in haematopoietic stem cell transplantation was adopted. The PDCO agreed on a waiver in a subset of children less than 3 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.5. Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP03-23

Biotest AG; Treatment of lower respiratory tract and lung infections

Day 120 opinion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Pneumology - Allergology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed on a paediatric investigation plan (PIP) with a deferral for trimodulin (human IgM, IgA, IgG solution) for all subsets of the paediatric population from birth to less than 18 years of age in the condition treatment of lower respiratory tract and lung infections. The PIP contains two clinical studies and four modelling and simulation studies.

2.1.6. EMEA-003477-PIP01-23

Treatment of appetite and general nutrition disorders

Day 120 opinion

Action: For adoption

Nutrition

Note: Withdrawal request received on 6 February 2024

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

SEAGEN BV; Treatment of solid tumours, including central nervous system malignancies

Day 120 opinion

Oncology

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 all paediatric patients in the condition of treatment of solid tumours, including central nervous system malignancies was adopted. The PDCO granted a deferral for one or more measures contained in the PIP.

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

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

Day 120 opinion

Other

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 alpelisib for paediatric patients from birth to less than 18 years of age, in the condition of treatment of lymphatic malformations associated with a PIK3CA mutation.

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

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

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 children and adolescents from 2 years to less than 18 years of age in the condition of treatment of fibrodysplasia ossificans progressiva was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the specific medicinal product is likely to be unsafe. The PDCO granted a deferral for one or more measures contained in the PIP.

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

Fulcrum Therapeutics, Inc.; Treatment of facioscapulohumeral muscular dystrophy

Day 120 opinion

Other

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 losmapimod for children from 2 to less than 18 years, in the condition of treatment of facioscapulohumeral muscular dystrophy was adopted.
The PDCO agreed on a waiver in a subset of children from birth to less than 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The PDCO granted a deferral for the completion of this PIP.

2.1.11. Sevasemten - EMEA-003394-PIP01-23

FGK Representative Service GmbH Germany; Treatment of dystrophinopathies

Day 120 opinion

Other / Neurology

Summary of Committee discussion:

In February 2024, the PDCO discussed the remaining issues that were raised at Day 90.
The PDCO adopted a positive opinion on a paediatric investigation plan (PIP) for sevasemten for a subset of children from 6 months to less than 18 years of age, with a deferral in treatment of dystrophinopathies (including treatment of Duchenne muscular dystrophy and treatment of Becker muscular dystrophy) and a waiver for a subset of children from birth to less than 6 months on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.12. Cannabidiol - Orphan - EMEA-003176-PIP02-22

Zynerba Pharmaceuticals Inc; Treatment of fragile X syndrome (FXS)

Day 120 opinion

Psychiatry

Summary of Committee discussion:

The PDCO discussed at Day 120, during the February 2024 plenary meeting, an application for a paediatric investigation plan (PIP) with a deferral and a waiver for zamtocabtagene autoleucel for cannabidiol for the treatment of fragile X syndrome.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.
The PDCO adopted a positive opinion on a PIP with a deferral and a waiver for children less than 3 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible in the treatment of fragile X syndrome.

2.1.13. CX-000667 mRNA encoding CMV gB / CX-005282 mRNA encoding the gH protein in the CMV glycoprotein complex pentamer / CX-005128 mRNA encoding the UL131A protein in the CMV glycoprotein complex pentamer / CX-000712 mRNA encoding the UL130 protein in the CMV glycoprotein complex pentamer / CX-000594 mRNA

encoding the gL protein in the CMV glycoprotein complex pentamer / CX-000359 mRNA encoding the UL128 protein in the CMV glycoprotein complex pentamer - EMEA-003405-PIP01-23

Moderna Biotech Spain, S.L.; Prevention of cytomegalovirus infection

Day 120 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 CX-000359 mRNA encoding the UL128 protein in the CMV glycoprotein complex pentamer / CX-000594 mRNA encoding the gL protein in the CMV glycoprotein complex pentamer / CX-000712 mRNA encoding the UL130 protein in the CMV glycoprotein complex pentamer / CX-005128 mRNA encoding the UL131A protein in the CMV glycoprotein complex pentamer / CX-005282 mRNA encoding the gH protein in the CMV glycoprotein complex pentamer / CX-000667 mRNA encoding CMV gB (mRNA-1647) for children aged 6 months to less than 18 years of age, in the condition of prevention of prevention of cytomegalovirus 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 does not represent a significant therapeutic benefit in this age group. The PDCO granted a deferral for the completion of this PIP.

2.1.14. Nebivolol / ramipril - EMEA-003530-PIP01-23

Zakłady Farmaceutyczne Polpharma S.A.; Treatment of hypertension / Treatment of heart failure / Treatment of coronary artery disease / Treatment of hypertension with coexisting heart failure / Treatment of hypertension with coexisting coronary artery disease

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 nebivolol / ramipril for all subsets of the paediatric population (0 to 18 years of age) in the conditions of treatment of hypertension, treatment of heart failure, treatment of coronary artery disease, treatment of hypertension with coexisting heart failure and treatment of hypertension with coexisting coronary artery disease.
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. Human IgG1 monoclonal antibody targeting amyloid transthyretin - EMEA-003548-PIP01-23

Alexion Europe S.A.S.; Treatment of transthyretin amyloidosis

Day 60 opinion

Cardiovascular Diseases / Neurology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for human IgG1 monoclonal antibody targeting amyloid transthyretin for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of transthyretin amyloidosis, 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.

2.1.16. Propylene glycol / urea - EMEA-003542-PIP01-23

ACO HUD Nordic AB; Treatment of dry skin

Day 60 opinion

Dermatology

Summary of Committee discussion:

In the written response, the applicant addressed some of the remaining issues raised by the Committee at Day 30.
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 propylene glycol / urea for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of dry skin 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).

2.1.17. Sitagliptin / dapagliflozin - EMEA-003534-PIP01-23

Rontis Hellas Medical and Pharmaceutical Products S.A.; Treatment of type II 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.

2.1.18. Efgartigimod alfa - EMEA-002597-PIP10-23

argenx BV; Treatment of thyroid eye disease

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

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

2.1.19. Human HSD17B1 enzyme inhibitor (OG-6219) - EMEA-003537-PIP01-23

N.V. Organon; Treatment of endometriosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for human HSD17B1 enzyme inhibitor (OG-6219) for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of endometriosis on the grounds of disease not occurring in boys and pre-menarcheal girls and on the ground that the specific medicinal product does not represent a

significant therapeutic benefit as clinical studies are not feasible for girls from menarche to less than 18 years of age.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.20. 2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid (fipaxalparant) - Orphan - EMEA-003539-PIP01-23

Horizon Therapeutics Ireland DAC; Treatment of progressive fibrosing interstitial lung disease

Day 60 opinion

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 21 February 2024

2.1.21. Empasiprubart - EMEA-003528-PIP01-23

argenx BV; Treatment of multifocal motor neuropathy

Day 60 opinion

Neurology

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 empasiprubart for all subsets of the paediatric population (0 to 18 years of age) in the condition of multifocal motor neuropathy.
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.

2.1.22. Suvecaltamide hydrochloride - EMEA-003248-PIP02-23

Jazz Pharmaceuticals Ireland Ltd; Treatment of tremor in patients with Parkinson’s disease

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition of treatment of tremor in patients with Parkinson’s disease on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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

2.1.23. Sodium phenylbutyrate / ursodoxicoltaurine – Orphan – EMEA-002876-PIP02-23

Amylyx Pharmaceuticals EMEA B.V.; Treatment of progressive supranuclear palsy

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the February 2024 plenary meeting the application for a product specific full waiver for sodium phenylbutyrate / ursodoxicoltaurine for treatment of progressive supranuclear palsy.
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 sodium phenylbutyrate / ursodoxicoltaurine for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of progressive supranuclear palsy on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subsets. The applicant agreed to generalising the opinion to all pharmaceutical forms and 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.

2.1.24. Autologous T-cells expressing a chimeric antigenic receptor against G protein coupled receptor class C group 5 member D (GPRC5D) (BMS-986393) - EMEA-003543-PIP01-23

Bristol-Myers Squibb Pharma EEIG; Treatment of multiple myeloma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2024 plenary meeting, a product-specific waiver request for autologous T-cells expressing a chimeric antigenic receptor against G protein coupled receptor family C group 5 member D (GPRC5D) (BMS-986393) for the treatment of multiple myeloma on the grounds that the disease does not occur in the paediatric population.
The PDCO confirmed all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 for this product for the treatment of multiple myeloma on the grounds that the disease does not occur in the paediatric population.
The PDCO emphasised that the granting of a waiver for the condition mentioned above

should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.25. Zongertinib - EMEA-003546-PIP01-23

Boehringer Ingelheim International GmbH; Treatment of non-small cell lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

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

2.1.26. Netupitant / palonosetron - EMEA-001198-PIP04-23

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

Day 60 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for netupitant / palonosetron for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of prevention of chemotherapy-induced nausea and vomiting on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.27. Vixarelimab - EMEA-003540-PIP01-23

Roche Registration GmbH; Treatment of idiopathic pulmonary fibrosis

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 vixarelimab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of idiopathic pulmonary fibrosis.

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.

2.1.28. Maplirpacept - EMEA-003551-PIP01-23

Pfizer Europe MA EEIG; Treatment of multiple myeloma

Day 30 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for maplirpacept for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of multiple myeloma 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 the extension 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.

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

AstraZeneca AB; Prevention of influenza infection

Day 1 opinion

Vaccines

The PDCO adopted the opinion by written procedure on 8 February 2024

2.1.30. Povorcitinib - EMEA-003313-PIP03-23

Incyte Biosciences Distribution B.V.; Treatment of prurigo nodularis

Day 30 opinion

Dermatology

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

2.2. Opinions on Compliance Check

2.2.1. Letermovir - EMEA-C-001631-PIP01-14-M05

Merck Sharp & Dohme B.V.; Prevention of cytomegalovirus infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

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

- EMEA-C1-001631-PIP14-M04

The PDCO adopted on 23 February 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/0455/2023) of 17 November 2023.

2.2.2. Tedizolid phosphate - EMEA-C2-001379-PIP01-12-M08

Merck Sharp & Dohme (Europe) Inc; Treatment of acute bacterial skin and skin structure infections

Day 60 letter

Infectious Diseases

Summary of Committee discussion:

The PDCO rediscussed the completed Study 6 (MK-1986-014) and considered that the study is compliant with the latest Agency's Decision (P/0340/2023) of 17 August 2023.
The PDCO finalised this partial compliance procedure on 23 February 2023.

2.2.3. Hydrocortisone - EMEA-C-002305-PIP01-17-M01

Laboratoire AGUETTANT; Prevention of bronchopulmonary dysplasia

Day 60 opinion

Neonatology - Paediatric Intensive Care

Summary of Committee discussion:

The PDCO took note of the applicant's clarifications and considered issues, raised at Day 30, resolved.
The PDCO adopted on 23 February 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/0358/2020) of 9 September 2020.

2.2.4. Blinatumomab - EMEA-C-000574-PIP02-12-M04

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO adopted on 23 February 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/0449/2023) of 27 October 2023.

2.2.5. Evinacumab - EMEA-C-002298-PIP01-17-M05

Ultragenyx Germany GmbH; Treatment of elevated cholesterol

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:
- EMEA-C1-002298-PIP01-17-M01
- EMEA-C2-002298-PIP01-17-M05

The PDCO adopted on 23 February 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/0087/2023) of 10 March 2023.

2.2.6. Macitentan - EMEA-C-001032-PIP01-10-M07

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:
- EMEA-C1-001032-PIP01-10-M01
- EMEA-C2-001032-PIP01-10-M02
- EMEA-C3-001032-PIP01-10-M05

The PDCO adopted on 23 February 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/0457/2023) of 1 December 2023.

2.2.7. Avacopan - EMEA-C5-002023-PIP01-16-M07

Amgen Europe B.V.; Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

Day 60 letter

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO discussed the applicant’s responses, received after Day 30. The members concluded that Study 6 (CL010_168) and Study 7 (CL019_168) are compliant with the latest Agency's Decision (P/0352/2023) of 8 September 2023.
The PDCO finalised this partially completed compliance procedure on 23 February 2024.

2.2.8. Human fibrinogen concentrate (BT524) - EMEA-C-001931-PIP01-16-M03

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 30 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO adopted on 23 February 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/0484/2023) of 30 November 2023.

2.2.9. Risdiplam - EMEA-C-002070-PIP01-16-M07

Roche Pharma AG; Treatment of spinal muscular atrophy

Day 30 opinion

Neurology

Summary of Committee discussion:

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

- EMEA-C1-002070-PIP01-16-M04
- EMEA-C2-002070-PIP01-16-M06

The PDCO adopted on 23 February 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/0086/2023) of 10 March 2023.

2.2.10. Mozafancogene autotemcel - EMEA-C1-002578-PIP01-19-M01

Rocket Pharmaceuticals, Inc.; Treatment of Fanconi anaemia subtype A

Day 30 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO discussed the completed Studies 1, 3 and 5 and considered that these are compliant with the latest Agency's Decision (P/0002/2024) of 7 January 2024.
The PDCO finalised this partially completed compliance procedure on 23 February 2024.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Chloroprocaine (hydrochloride) - EMEA-000639-PIP03-16-M03

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

Day 60 opinion

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/0334/2022 of 10 August 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Aficamten - EMEA-002958-PIP01-21-M01

Cytokinetics, Inc.; Treatment of hypertrophic cardiomyopathy

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0023/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Milvexian - EMEA-003220-PIP01-22-M01

Janssen-Cilag International N.V.; Prevention of thromboembolism in patients with cardiovascular diseases

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0274/2023 of 7 August 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Ruxolitinib phosphate - EMEA-002618-PIP02-20-M01

Incyte Biosciences Distribution B.V.; Treatment of vitiligo

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes (changes to key elements and the timeline of Study 4 (INCB 18424-309)) 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/0145/2021 of 16 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Glucagon analogue linked to a human immunoglobulin Fc fragment - Orphan - EMEA-003170-PIP01-21-M02

Hanmi Pharm. Co., Ltd.; Treatment of congenital hyperinsulinism

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/0100/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19-M03

Pfizer Europe MA EEIG; Treatment of congenital factor IX deficiency (haemophilia B)

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed

paediatric investigation plan (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/0277/2022 of 10 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Filgotinib - EMEA-001619-PIP04-17-M03

Galapagos NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, 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 some of the proposed changes could be accepted (sample size in Study 4, changes to secondary endpoints in Study 5).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0113/2023 of 13 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. BNT162b2 / tozinameran / famtozinameran / riltozinameran / raxtozinameran - EMEA-002861-PIP02-20-M07

BioNTech Manufacturing GmbH; Prevention of coronavirus disease 2019 (COVID-19)

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), and the additional responses received between Day 30 and Day 60 of this application, 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/0393/2023 of 28 September 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Catequentinib - Orphan - EMEA-002486-PIP04-21-M01

Advenchen Laboratories, LLC; Treatment of Ewing sarcomas / Treatment of soft tissue sarcomas

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that 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/0138/2022 of 13 April 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Cedazuridine / decitabine - EMEA-003071-PIP01-21-M01

Otsuka Pharmaceutical Netherlands B.V.; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0217/2022 of 10 June 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Vamikibart - EMEA-003215-PIP01-22-M01

Roche Registration GmbH; Treatment of uveitic macular oedema

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/0072/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Afamelanotide - Orphan - EMEA-000737-PIP02-11-M03

Clinuvel Europe Limited; Treatment of erythropoietic protoporphyria

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 on the timelines for Studies 2, 3, 4 and 5 without delaying the overall completion date of the PIP, 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/0060/2023 of 3 February 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. In vitro expanded autologous human articular chondrocytes - EMEA-001823-PIP01-15-M03

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 60 opinion

Other

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2024 plenary meeting, a request for modification for in vitro expanded autologous human articular chondrocytes for the treatment of cartilage disorders.
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/0043/2021 of 27 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. 13 Grass aqueous extract - EMEA-000813-PIP01-09-M01

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

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

The applicant addressed several of the remaining issues raised by the Committee 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 most of the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/221/2010 of 29 October 2010).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Seltorexant - EMEA-002746-PIP01-20-M03

Janssen-Cilag International NV; Treatment of major depressive disorder

Day 60 opinion

Psychiatry

Summary of Committee discussion:

In the written response, and during an oral explanation before the Committee on 21

February 2024, some of the issues were addressed.
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 on the reduction of the sample size and PK sampling for Study 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/0291/2021 of 13 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. L-Carnitine / glucose / calcium chloride dihydrate / magnesium chloride hexahydrate / sodium lactate / sodium chloride - EMEA-003049-PIP01-21-M01

Iperboreal Pharma Srl; Treatment of renal failure with carnitine deficiency

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

The applicant addressed the remaining issues raised by the Committee.
Based on the review of 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/0060/2022 of 7 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Mirabegron - EMEA-000597-PIP03-15-M06

Astellas Pharma Europe B.V.; Treatment of neurogenic detrusor overactivity

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 (changed timelines of Study 12) 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/0187/2022 of 25 May 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis* group B protein 961c / recombinant *Neisseria meningitis* group B protein 287- 953 / meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis*

group B protein 936-741 / outer membrane vesicles (OMV) from *N. meningitidis* strain NZ 98/254 - EMEA-001260-PIP01-11-M03

GlaxoSmithKline Biologicals SA; Prevention of meningococcal meningitis

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/0437/2022 of 28 October 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Baricitinib - EMEA-001220-PIP01-11-M10

Eli Lilly and Company Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

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 (eligibility criteria in Study 5 (sJIA)) 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/0520/2023 of 29 December 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

2.4.1. Frexalimab - EMEA-002945-PIP03-23

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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. Baricitinib - EMEA-C3-001220-PIP01-11-M08

Eli Lilly and Company; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 letter

Immunology-Rheumatology-Transplantation

2.7.2. Vosorotide - EMEA-C6-002033-PIP01-16-M03

BioMarin International Limited; Treatment of achondroplasia

Day 15 letter

Other

2.7.3. Obeticholic acid - EMEA-C2-001304-PIP02-13-M07

Advanz Pharma Limited; Treatment of biliary atresia

Day 30 letter

Gastroenterology-Hepatology

2.7.4. Ferric Citrate Coordination Complex (FCCC) - EMEA-C1-001213-PIP03-23

Averoa SAS; Treatment of anaemias due to chronic kidney disorders

Day 30 letter

Uro-nephrology

2.7.5. Apraglutide - EMEA-C1-003016-PIP01-21

VectivBio AG; Treatment of short bowel syndrome

Day 30 letter

Gastroenterology-Hepatology

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

Treatment of hypercholesterolaemia

Day 90 discussion

Cardiovascular Diseases

3.1.2. Lutikizumab - EMEA-003481-PIP01-23

Treatment of hidradenitis suppurativa

Day 90 discussion

Dermatology

3.1.3. Spesolimab - EMEA-002475-PIP04-23

Treatment of hidradenitis suppurativa

Day 90 discussion

Dermatology

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

Treatment of obesity

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. Plecanatide - EMEA-003441-PIP01-23

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

Day 90 discussion

Gastroenterology-Hepatology

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

Novo Nordisk A/S; Treatment of sickle cell disease

Day 90 discussion

Haematology-Hemostaseology

3.1.7. Tozorakimab - EMEA-003360-PIP01-22

Treatment of acute respiratory failure

Day 90 discussion

Infectious Diseases / Pneumology - Allergology

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

Treatment of melanoma

Day 90 discussion

Oncology

3.1.9. Laruparetigene zovaparvovec – Orphan – EMEA-003457-PIP01-23

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

Day 90 discussion

Ophthalmology

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

CSL Behring GmbH; Treatment of sickle cell disease

Day 90 discussion

Other

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

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

Day 90 discussion

Other

3.1.12. Mometasone - EMEA-003454-PIP01-23

Treatment of chronic rhinosinusitis (CRS)

Day 90 discussion

Oto-rhino-laryngology

3.1.13. Tanimilast - EMEA-003393-PIP01-23

Treatment of asthma

Day 90 discussion

Pneumology - Allergology

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

Prevention of respiratory syncytial virus (RSV) disease

Day 90 discussion

Vaccines

3.1.15. EMEA-003531-PIP01-23

Treatment of hyperphenylalaninemia

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.16. Efinopegdutide - EMEA-003549-PIP01-23

Treatment of non-alcoholic steatohepatitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.17. Ensitrelvir - EMEA-003192-PIP03-23

Post exposure prophylaxis of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Infectious Diseases

3.1.18. Glenzocimab - EMEA-003536-PIP01-23

Treatment of acute ischaemic stroke (AIS)

Day 60 discussion

Neurology

3.1.19. Unasnemab - EMEA-003529-PIP01-23

Treatment of spinal cord injury

Day 60 discussion

Neurology

3.1.20. Budigalimab - EMEA-003532-PIP01-23

Treatment of solid tumours

Day 60 discussion

Oncology

3.1.21. Livmoniplimab - EMEA-003533-PIP01-23

Treatment of solid tumours

Day 60 discussion

Oncology

3.1.22. Fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer - EMEA-003538-PIP01-23

Treatment of Duchenne muscular dystrophy

Day 60 discussion

Other

3.1.23. Retatrutide - EMEA-003258-PIP02-23

Treatment of obesity

Day 60 discussion

Other

3.1.24. Atorvastatin / fenofibrate - EMEA-003563-PIP01-23

Treatment of mixed hyperlipidaemia

Day 30 discussion

Cardiovascular Diseases

3.1.25. Fenofibrate / ezetimibe / rosuvastatin - EMEA-003562-PIP01-23

Treatment of mixed hyperlipidaemia

Day 30 discussion

Cardiovascular Diseases

3.1.26. Povorcitinib - EMEA-003313-PIP02-23

Treatment of vitiligo

Day 30 discussion

Dermatology

3.1.27. Sonelokimab - EMEA-002568-PIP02-23

Treatment of hidradenitis suppurativa

Day 30 discussion

Dermatology

3.1.28. Linaprazan - EMEA-003558-PIP01-23

Treatment of gastro-oesophageal reflux disease

Day 30 discussion

Gastroenterology-Hepatology

3.1.29. Volixibat - EMEA-003567-PIP01-23

Treatment of primary biliary cholangitis

Day 30 discussion

Gastroenterology-Hepatology

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

Treatment of coagulation factor deficiencies

Day 30 discussion

Haematology-Hemostaseology

3.1.31. Sargramostim - EMEA-003568-PIP01-23

Treatment for patients acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome [H-ARS])

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.32. Tulisokibart - EMEA-003556-PIP01-23

Treatment of ulcerative colitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Prevention of rabies infection

Day 30 discussion

Infectious Diseases

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

Shionogi BV; Treatment of fungal infectious disorders

Day 30 discussion

Infectious Diseases

3.1.35. Acetylcysteine - EMEA-003554-PIP01-23

Treatment of hereditary cystatin C amyloid angiopathy

Day 30 discussion

Neurology

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

Treatment of essential tremor

Day 30 discussion

Neurology

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

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

Day 30 discussion

Neurology

3.1.38. Amivantamab - EMEA-002573-PIP02-23

Treatment of colorectal carcinoma

Day 30 discussion

Oncology

3.1.39. Camreluzimab - EMEA-003566-PIP01-23

Treatment of hepatocellular carcinoma

Day 30 discussion

Oncology

3.1.40. Erdafitinib - EMEA-002042-PIP03-23

Treatment of urothelial carcinoma

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

3.1.42. Modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens - EMEA-003434-PIP02-23

Treatment of renal neoplasms / Treatment of urothelial carcinomas / Treatment of cutaneous squamous cell carcinoma

Day 30 discussion

Oncology

3.1.43. Mosunetuzumab - Orphan - EMEA-002524-PIP03-23

Roche Registration GmbH; Treatment of mature B-cell neoplasms

Day 30 discussion

Oncology

3.1.44. EMEA-003485-PIP01-23

Treatment of non-small cell lung cancer

Day 30 discussion

Oncology

3.1.45. Petosemtamab - EMEA-003557-PIP01-23

Treatment of head and neck epithelial neoplasms

Day 30 discussion

Oncology

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

Treatment of ovarian cancer / Treatment of primary peritoneal cancer / Treatment of fallopian tube cancer

Day 30 discussion

Oncology

3.1.47. Riletamotide / tapderimotide / alrefimotide - EMEA-003555-PIP01-23

Treatment of malignant mesothelioma

Day 30 discussion

Oncology

3.1.48. Carbachol / brimonidine - EMEA-003561-PIP01-23

Treatment of presbyopia

Day 30 discussion

Ophthalmology

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

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

Day 30 discussion

Other / Cardiovascular Diseases

3.1.50. Atogepant - EMEA-002530-PIP02-23

Treatment of migraine headaches

Day 30 discussion

Pain

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

Treatment of emphysema secondary to congenital deficiency of alpha-1 antitrypsin

Day 30 discussion

Pneumology - Allergology

3.1.52. Dapagliflozin propanediol / baxdrostat - EMEA-003559-PIP01-23

Treatment of chronic kidney disease

Day 30 discussion

Uro-nephrology

3.1.53. EMEA-003560-PIP01-23

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

Day 30 discussion

Vaccines / Infectious Diseases

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

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

Day 30 discussion

Neurology

3.2.2. Brexucabtagene autoleucel - EMEA-C-001862-PIP01-15-M03

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

Day 30 discussion

Oncology

Note: Withdrawal request received on 29 January 2024

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

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

Day 30 discussion

Ophthalmology / Pneumology - Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

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

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

Day 30 discussion

Cardiovascular Diseases

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

Chemo Research, S.L.; Contraception

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Haematology-Hemostaseology

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

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 30 discussion

Haematology-Hemostaseology

3.3.6. Pegcetacoplan – Orphan – EMEA-002600-PIP01-19-M02

Swedish Orphan Biovitrum AB (publ); Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Haematology-Hemostaseology

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

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 30 discussion

Neurology

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

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

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

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

AstraZeneca AB; Treatment of melanoma / Treatment of neurofibromatosis type 1 / Treatment of thyroid cancer

Day 30 discussion

Oncology

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

BioCryst Ireland Limited; Treatment of hereditary angioedema

Day 30 discussion

Pneumology - Allergology

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

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

Day 30 discussion

Pneumology - Allergology

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

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

Pneumology - Allergology

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

Chinook Therapeutics, Inc.; Treatment of IgA nephropathy

Day 30 discussion

Uro-nephrology

3.3.17. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-002068-PIP01-16-M05

Seqirus Netherlands; Prevention of influenza

Day 30 discussion

Vaccines

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

MSD Europe Belgium SRL; Prevention of dengue disease

Day 30 discussion

Vaccines

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

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

Day 30 discussion

Vaccines

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

Sanofi Pasteur; Prevention of influenza infection

Day 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 26 February 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

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. BI 771716 - EMEA-15-2023

Boehringer Ingelheim International GmbH; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of geographic atrophy (GA) secondary to age-related macular degeneration (AMD)

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: atypical haemolytic uremic syndrome, paroxysmal nocturnal haemoglobinuria, myasthenia gravis, graft rejection, neuromyelitis optica etc. (in an appropriate pharmaceutical form/route of administration).

6.1.2. Zanidatamab - EMEA-01-2024

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

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.

6.1.3. Tasquinimod – EMEA-16-2023

Active Biotech AB; The class of ribonucleotide reductase beta-2 inhibitor medicinal products for treatment of myeloproliferative neoplasms; the class of primarily alkylating medicinal products for treatment of myeloproliferative neoplasms and mature B, T and NK cell neoplasms; the class of immunomodulatory cytokine medicinal products for treatment of neuroendocrine malignant neoplasms, skin malignant neoplasms, myeloproliferative neoplasms and mature B, T and NK cell neoplasms / Treatment of myelofibrosis

Summary of Committee discussion:

The applicability of the class waiver as referred to in the Agency's decision CW/0001/2015 to the planned therapeutic indication was not confirmed.

This was based on the consideration that in view of its mechanism of action consisting of enhancing the host immune response and inhibiting the angiogenic response, it is considered that tasquinimod does not belong to any of the pharmacological classes of medicinal products for the treatment of myeloproliferative neoplasms included in the decision CW/0001/2015.

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. Quizartinib - EMEA-001821-PIP01-15-M07

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Proposed indication: Quizartinib is indicated in combination with standard cytarabine and anthracycline induction and standard cytarabine consolidation, for the treatment of adult patients with newly diagnosed acute myeloid leukaemia (AML) who are FLT3-ITD negative

Summary of Committee discussion:

The PDCO confirmed that the proposed new indication falls under the scope of the EMA decision P/0259/2023.

7.1.2. Abelacimab - EMEA-003017-PIP01-21

Anthos Therapeutics, Inc.; Prevention of thromboembolic events / Treatment of thromboembolic events

Proposed indication: Prevention of stroke and system embolic events in atrial fibrillation

Summary of Committee discussion:

The applicant requested confirmation that the paediatric decision P/0127/2022 for the conditions treatment and prevention of thromboembolic events covers the indication "prevention of stroke and system embolic events in atrial fibrillation", and thus, no separate PIP or waiver is required for this indication. PDCO is of the view that the proposed indication falls under the scope of the above-mentioned Decision, as the indication is considered to be covered by the conditions "treatment of thromboembolic events" or "prevention of thromboembolic events" listed in the Agency Decision and no separate PIP or waiver is required.

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

The Chair welcomed Miroslav Weiss as the new member for Croatia and Irena Senecic-Cala as the new alternate for Croatia.

9.1.2. Vote by Proxy

None

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

PDCO member: Karen van Malderen

Summary of Committee discussion:

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

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Summary of Committee discussion:

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

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

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

9.3.2. Paediatric Formulation Operational Expert Group (PFOEG)

PDCO member: Brian Aylward (*ad interim*)

Summary of Committee discussion:

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

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

Summary of Committee discussion:

The PCWP-HCPWP and all eligible organisations meeting summary report on 14-15 November 2023 and the draft agenda for PCWP-HCPWP Joint meeting on 27-28 February 2024 were shared with the Committee for information.

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

Summary of Committee discussion:

Topic has been postponed and will be discussed at the PDCO 19-22 March 2024 plenary meeting.

9.3.5. Upcoming Innovation Task Force (ITF) meetings

Summary of Committee discussion:

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

9.4. Cooperation within the EU regulatory network

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

No item

9.4.2. Chemical and Biological Quality European Specialised Expert Communities (ESECs)

Summary of Committee discussion:

PDCO noted the presentation on the background of the two quality ESECs, the objectives and how to nominate experts for membership. A question was raised if non-NCA PDCO members could also nominate experts. It was clarified that initially, the ESECs are restricted to assessors working for, or on behalf of, NCAs because there will be exposure to commercial confidential information (CCI). In the future, it may be possible to nominate academic experts.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The February 2024 agenda and the January 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. Main PDCO approaches for the condition pulmonary arterial hypertension

Summary of Committee discussion:

An overview of pulmonary arterial hypertension in the paediatric population was presented, followed by a retrospective analysis of approved PIPs for this condition. It encompassed a summary of the different study design strategies approved, along with an overview of the efficacy endpoints used. Subsequently, a discussion was held regarding the efficacy endpoints and the extrapolation strategy.

10.2. Guideline on allergen on small populations

Summary of Committee discussion:

The guideline on allergen on small populations was presented to the Committee. The members were informed on amendments implemented following previous PDCO consultation. The PDCO adopted the guideline.

10.3. EU Network Training Centre (EU NTC): Update on the Paediatric curriculum- call to action

Summary of Committee discussion:

A document outlining how PDCO members/alternates can support the update of the Paediatric curriculum to ensure it is fit for purpose and serves current and future training needs was presented.

PDCO members and alternates were encouraged to volunteer to undertake one or more of the following actions:

- Critical review of (an) existing module(s) with feedback on need to update;
- Propose new scientific module(s);
- Propose existing/external material that should be considered for inclusion in the Paediatric curriculum.

If PDCO members/alternates have any questions or considerations, or if they would like to involve other groups/colleagues, the Committee should contact the EU NTC team at EMA.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The clinical experience of some paediatric patients with Burkitt lymphoma was reported along with the challenges encountered. The group also discussed lessons learnt from recent,

relevant CHMP procedures.

11.2. Neonatology

Summary of Committee discussion:

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

11.3. Vaccines

Summary of Committee discussion:

The PDCO was informed on the background and activities related to the transition from quadrivalent to trivalent influenza vaccines.

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 20-23 February 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 final deliberations and voting on:	3.3.1. Apixaban - EMEA-000183-PIP02-12-M05
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Miroslav Weiss	Member	Croatia	No interests declared	
Zena Gunther	Member	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	
Anne Paavola	Alternate	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	
Cinzia Ciceroni	Alternate	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	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
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	
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 final deliberations and voting on:	3.2.4. Brexucabtagene autoleucel - EMEA-C-001862-PIP01-15-M03 (withdrawn)

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
				3.3.8. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP03-20-M02
Viviana Giannuzzi	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Patricia Felgueiras Seabra Durao	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Victoria Romero Pazos	Alternate	Patients' Organisation Representative	No interests declared	
María Estela Moreno Martín	Expert	Spain	No interests declared	
Olga Kholmanskikh	Expert	Belgium	No interests declared	
Andreas Bonertz	Expert	Germany	No interests declared	
Birgit Ahrens	Expert	Germany	No restrictions applicable to this meeting	
Susanne Kaul	Expert	Germany	No restrictions applicable to this meeting	
Flora Musuamba Tshinanu	Expert	Belgium	No restrictions applicable to this meeting	
Larissa Higgins	Expert	Ireland	No restrictions applicable to this meeting	
Sandra Schmidt	Expert	Germany	No restrictions applicable to this meeting	
Nathalie Morgensztjen	Expert	France	No interests declared	
Meeting run with support from relevant EMA staff				

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

13. Explanatory notes

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

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

plan.

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

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

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

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

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/