10 November 2023
EMA/PDCO/479731/2023
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 10-13 October 2023

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(s) and alternate(s) and thanked the departing members/alternates for their contributions to the Committee.

1.2. Adoption of agenda

The agenda for 10-13 October 2023 meeting was adopted with amendments.

1.3. Adoption of the minutes

The minutes for 05-08 October 2023 meeting were adopted and will be published on the EMA website.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Amlitelimab - EMEA-003233-PIP01-22

Sanofi Winthrop Industrie; Treatment of atopic dermatitis

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 was adopted for amlitelimab in children from 6 months to less than 18 years of age in the condition of 'treatment of atopic dermatitis'.
The PDCO granted a waiver in children from birth to less than 6 months 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(s).
The PDCO granted a deferral for completion of this PIP.

2.1.2. Upadacitinib - EMEA-001741-PIP08-22

AbbVie Ltd; Treatment of hidradenitis suppurativa

Day 120 opinion

Dermatology

Summary of Committee discussion:

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

2.1.3. Upadacitinib - EMEA-001741-PIP09-23

AbbVie Ltd; Treatment of systemic lupus erythematosus

Day 120 opinion

Dermatology

Summary of Committee discussion:

The Paediatric Committee agreed on a PIP for upadacitinib for the treatment of systemic lupus erythematosus in paediatric patients from 5 years of age. A waiver was granted for the population below 5 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 one or more measures contained in the PIP.

2.1.4. *Escherichia coli*, expressing high affinity phenylalanine transporter, modified phenylalanine ammonia lyase and L-amino acid deaminase - EMEA-003381-PIP01-22

Treatment of hyperphenylalaninemia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 11 October 2023

2.1.5. Recombinant human monoclonal antibody to insulin receptor - Orphan - EMEA-002813-PIP01-23

Rezolute (Bio) Ireland Limited; Treatment of congenital hyperinsulinism

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 12 October 2023

2.1.6. Venglustat - Orphan - EMEA-001716-PIP07-22

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

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

In the written response in the framework of a consultation of the draft opinion, 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 PIP for the proposed medicine for the treatment of Gaucher disease type 3 above 2 years of age was adopted. The PDCO agreed on a waiver in Gaucher disease type 2 in all subsets of the paediatric population from birth to less than 18 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible and in the treatment of Gaucher disease type 3 for the paediatric population from birth to less than 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.7. Cemdisiran - Orphan - EMEA-003237-PIP02-22

Regeneron Ireland DAC; Treatment of paroxysmal nocturnal haemoglobinuria

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application, the additional information provided by the applicant and further discussions at the Paediatric Committee, the PDCO adopted a positive opinion on Day 120 for cemdisiran for treatment of paroxysmal nocturnal haemoglobinuria (PNH) for paediatric patients from 2 years to less than 18 years of age.
A waiver was granted for paediatric population 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 one or more measures contained in this PIP.

2.1.8. Pozelimab - EMEA-003238-PIP02-22

Regeneron Ireland DAC; Treatment of paroxysmal nocturnal haemoglobinuria

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application, the additional information provided by the applicant and further discussions at the Paediatric Committee, the PDCO adopted a positive opinion on Day 120 for pozelimab for treatment of paroxysmal nocturnal haemoglobinuria (PNH) for paediatric patients from 2 years to less than 18 years of age.
A waiver was granted for paediatric population 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 one or more measures contained in this PIP.

2.1.9. Tetrahydrouridine / decitabine - Orphan - EMEA-003404-PIP01-23

Novo Nordisk A/S; Treatment of sickle cell disease (SCD)

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 PIP for the proposed medicine for children from 6 months to less than 18 years of age in the condition of treatment of sickle cell disease was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.10. Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP01-23

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy

Day 120 opinion

Neurology

Note: Withdrawal request received on 13 October 2023

2.1.11. Inebilizumab - EMEA-001911-PIP02-22

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

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 PIP for the proposed medicine for children from 2 years to less than 18 years of age in the condition of treatment of myasthenia gravis was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.12. Cobolimab - EMEA-003273-PIP02-22

GlaxoSmithKline Trading Services Limited; Treatment of all conditions included in the category of malignant neoplasms including lymphoma (except lung cancers and haematopoietic malignancies)

Day 120 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for cobolimab for all subsets of the paediatric population, in the condition of treatment of all conditions included in the category of malignant neoplasms including lymphoma (except lung cancers and haematopoietic malignancies) was adopted. The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.13. Iptacopan - EMEA-002705-PIP05-23

Novartis Europharm Limited; Treatment of immune-complex mediated membranoproliferative glomerulonephritis

Day 120 opinion

Other

Summary of Committee discussion:

The Paediatric Committee agreed on a PIP for iptacopan for the treatment of immune-complex mediated membranoproliferative glomerulonephritis in paediatric patients from 2 years of age. A waiver was granted for the population below 2 years of age on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). The PDCO granted a deferral for one or more measures contained in the PIP.

2.1.14. Candesartan / atorvastatin / amlodipine - EMEA-003466-PIP01-23

Teva B.V.; 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 candesartan / atorvastatin / amlodipine for all subsets of the paediatric population (0 to 18 years of age) in the condition of 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.

2.1.15. Rosuvastatin / ezetimibe - EMEA-003472-PIP01-23

Pharmaplot PC; 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.

2.1.16. Fusion protein consisting of relaxin and Fc domain of IgG1 - EMEA-003476-PIP01-23

AstraZeneca AB; Treatment of pulmonary hypertension due to left heart 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 fusion protein consisting of relaxin and Fc domain of IgG1 for all subsets of the paediatric population (0 to 18 years of age) in the condition of

treatment of pulmonary hypertension due to left heart 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 other types of pulmonary hypertension (e.g., PAH), where there is a paediatric unmet need. Moreover, this product could have other indications in adults, and in that case, they must request another PIP, which depending on the indications should be discussed in the PDCO for its possible application in the paediatric age. 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. Glyceryl trinitrate - EMEA-003479-PIP01-23

Egis Pharmaceuticals PLC; Treatment of ischaemic coronary artery disorders / Treatment of heart failure / Prevention of cardiac and vascular procedural complications

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 glyceryl trinitrate for all subsets of the paediatric population (0 to 18 years of age) in the conditions of treatment of ischaemic coronary artery disorders, treatment of heart failure and prevention of cardiac and vascular procedural complications. 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 a benzoimidazole substituted pyrimidine - EMEA-003470-PIP01-23

Pfizer Europe MA EEIG; Treatment of breast malignant neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the October 2023 plenary meeting a request for a product-specific waiver for a derivative of a benzoimidazole substituted pyrimidine (PF-07220060) for the treatment of breast malignant neoplasms on the grounds that the disease does not occur in children. 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 breast malignant neoplasms on the grounds that the disease does not occur in children.
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.

2.1.19. Gotistobart - EMEA-003467-PIP01-23

OncoC4, Inc.; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

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

2.1.20. Humanised IgG1 monoclonal antibody against integrin beta-6 conjugated to monomethyl auristatin E via a valine-citrulline linker - EMEA-003471-PIP01-23

Seagen B.V.; Treatment of non-small cell lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the October 2023 plenary meeting a request for a product-specific waiver for a humanised IgG1 monoclonal antibody against integrin beta-6 conjugated to monomethyl auristatin E via a valine-citrulline linker (SGN-B6A) for the treatment of solid tumours on the grounds that the product is likely to lack efficacy 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 and agreed a product-specific waiver for the treatment of non-small cell lung cancer on the grounds that the disease does not occur in children. The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition. The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.21. Ilginatinib maleate - EMEA-003468-PIP01-23

NS Pharma, Inc.; Treatment of myelofibrosis

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 myelofibrosis on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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. Relatlimab / nivolumab - EMEA-002727-PIP02-23

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms and melanoma)

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 conditions included in the category of malignant neoplasms (except nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms and melanoma) on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.23. Humanised IgG1 blocking monoclonal antibody targeting CD39 (IPH5201) - EMEA-003469-PIP01-23

Innate Pharma SA; Treatment of lung cancer

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 IPH5201 for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of lung cancer. 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.

2.1.24. Naproxen / paracetamol - EMEA-003475-PIP01-23

GSK Consumer Healthcare SARL, a Haleon group company; Treatment of rheumatoid arthritis / Treatment of febrile disorders / Treatment of acute pain

Day 60 opinion

Pain

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 naproxen / paracetamol for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of acute pain, treatment of febrile disorders and treatment of rheumatoid arthritis, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.25. Povidone, iodinated - EMEA-003484-PIP01-23

Medivis Srl; Preoperative preparation for ophthalmic surgeries

Day 30 opinion

Ophthalmology

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 povidone, iodinated for all subsets of the paediatric population (0 to 18 years of age) in the condition of preoperative preparation for ophthalmic surgeries. 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. Indapamide / ramipril - EMEA-003498-PIP01-23

Egis Pharmaceuticals PLC; Treatment of hypertension

Day 30 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for indapamide / ramipril for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of hypertension. The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.2. Opinions on Compliance Check

2.2.1. Macitentan - EMEA-C3-001032-PIP01-10-M05

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 60 letter

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO considered the completed Studies 8 and 10 and agreed that these, for children aged 2 to less than 18 years, are compliant with the latest Agency's Decision (P/0012/2023) of 31 January 2023. Compliance cannot be confirmed in children aged 1 month to less than 2 years of age as the data are not included in the report.
The PDCO considered the completed Study 9 and agreed that this is compliant with the latest Agency's Decision (P/0012/2023) of 31 January 2023. It was noted that the studies being used as data sources for this study are not concluded, however the key elements of the study design are considered fulfilled.

2.2.2. Nirsevimab - EMEA-C-001784-PIP01-15-M04

AstraZeneca AB; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

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

- EMEA-C1-001784-PIP01-15-M02
- EMEA-C1-001784-PIP01-15-M03
- EMEA-C3-001784-PIP01-15-M04

The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0243/2022) of 8 July 2022.

2.2.3. Ataluren - EMEA-C-000115-PIP01-07-M13

PTC Therapeutics International, Limited; Treatment of dystrophinopathy

Day 60 opinion

Neurology

Summary of Committee discussion:

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

- EMEA-C1-000115-PIP01-07-M03
- EMEA-C2-000115-PIP01-07-M08
- EMEA-C3-000115-PIP01-07-M09

The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0196/2023) of 15 May 2023.

2.2.4. Lenvatinib - EMEA-C-001119-PIP03-19-M03

Eisai GmbH; Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue neoplasms, papillary thyroid cancer, follicular thyroid cancer and osteosarcoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0528/2022) of 30 December 2022.

2.2.5. Durvalumab - EMEA-C-002028-PIP01-16-M04

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

Day 30 opinion

Oncology

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures (EMEA-C1-002028-PIP01-16-M01 and EMEA-C2-002028-PIP01-16-M02) and that Study 2 (Multi-centre, open-label study, with a dose finding phase (phase 1) and an expansion phase (phase 2), to evaluate the safety, tolerability, pharmacokinetics and antitumour activity of durvalumab monotherapy, and durvalumab used in combination with tremelimumab in paediatric patients from birth to less than 18 years of age with a relapsed/refractory solid tumour or a paediatric solid tumour for whom no curative standard treatment is available) was completed in agreement with the latest Decision.
The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0302/2023) of 13 August 2023.

2.2.6. Tremelimumab - EMEA-C-002029-PIP01-16-M04

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

Day 30 opinion

Oncology

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures (EMEA-C1-002029-PIP01-16-M01 and EMEA-C2-002029-PIP01-16-M02) and that Study 2 (Multi-centre, open-label study, with a dose finding phase (phase 1) and an expansion phase (phase 2), to evaluate the safety, tolerability, pharmacokinetics and antitumour activity of durvalumab monotherapy, and durvalumab used in combination with tremelimumab in paediatric patients from birth to less than 18 years of age with a relapsed/refractory solid tumour or a paediatric solid tumour for whom no curative standard treatment is available) was completed in agreement with the latest Decision.
The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0302/2023) of 13 August 2023.

2.2.7. Ruxolitinib (phosphate) - EMEA-C-000901-PIP03-16-M02

Novartis Europharm Limited; Treatment of acute graft versus host disease (aGvHD)

Day 30 opinion

Oncology

Summary of Committee discussion:

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

- EMEA-C1-000901-PIP03-16-M01
- EMEA-C2-000901-PIP03-16-M02

The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0172/2021) of 9 April 2021.

2.2.8. Ruxolitinib (phosphate) - EMEA-C-000901-PIP04-17-M02

Novartis Europharm Limited; Treatment of chronic graft versus host disease

Day 30 opinion

Oncology

Summary of Committee discussion:

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

- EMEA-C1-000901-PIP04-17-M01
- EMEA-C2-000901-PIP04-17-M02

The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0527/2021) of 3 December 2021.

2.2.9. Ixekizumab - EMEA-C-001050-PIP01-10-M05

Eli Lilly and Company; Treatment of psoriasis

Day 30 opinion

Dermatology

Summary of Committee discussion:

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

- EMEA-C1-001050-PIP01-10, EMEA-C3-001050-PIP01-10-M05

The PDCO adopted on 13 October 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0087/2020) of 18 March 2020.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Macitentan - Orphan - EMEA-001032-PIP01-10-M07

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension / Treatment of systemic sclerosis / Treatment of idiopathic pulmonary fibrosis

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. The modification concerned a reduction of the minimum number of patients below 2 years of age to be recruited as part of the PIP. The PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision by majority. The Norwegian PDCO member was in agreement. Divergent position(s) will be appended to the opinion. The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Treprostinil sodium - EMEA-003182-PIP01-22-M01

AOP Orphan Pharmaceuticals GmbH; Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), and taken into account further clarifications by the applicant, the PDCO considered that the proposed changes to 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/0427/2022 of 28 October 2022). The requested deferral for completion of the clinical study was not granted, as it did not meet the grounds detailed in Article 20(1) of the Regulation.
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Abrocitinib - EMEA-002312-PIP01-17-M02

Pfizer Europe MA EEIG; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

In the written response the applicant addressed 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 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/0023/2020 of 3 January 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Remibrutinib - EMEA-002582-PIP01-19-M03

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 60 opinion

Dermatology

Summary of Committee discussion:

The applicant addressed 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 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/0132/2023 of 13 April 2023). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Liraglutide - EMEA-000128-PIP02-09-M05

Novo Nordisk A/S; Treatment of obesity

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 (deletion of quality-related study) could be accepted. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0309/2021 of 13 August 2021). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Pegzilarginase - Orphan - EMEA-001925-PIP02-19-M01

Immedica Pharma AB; Treatment of hyperargininaemia

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 (delay of the Study 6 completion) 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/0252/2020 of 15 July 2020). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Maralixibat chloride - Orphan - EMEA-001475-PIP03-17-M04

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

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. This is a modification to include the pharmaceutical form "tablet" which is being developed. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0436/2022 of 28 October 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Denecimig - EMEA-002762-PIP02-20-M02

Novo Nordisk A/S; Treatment of haemophilia A

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0215/2023 of 14 June 2023). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Human fibrinogen concentrate - EMEA-001931-PIP01-16-M03

Biotest AG; Treatment of congenital fibrinogen deficiency

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/0232/2020 of 19 June 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Sebetralstat - Orphan - EMEA-002723-PIP01-19-M02

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

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/0379/2022 of 27 September 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Risankizumab - EMEA-001776-PIP02-17-M02

AbbVie Ltd; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, spondylarthritis 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 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/0039/2021 of 27 January 2021). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Upadacitinib – EMEA-001741-PIP03-16-M04

AbbVie Ltd; Treatment of Crohn's disease

Day 60 opinion

Immunology-Rheumatology-Transplantation / 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 update elements of Study 4 and an overall delay to the completion of the PIP. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0263/2022 of 13 July 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Bemnifosbuvir - EMEA-002963-PIP01-21-M01

Atea Pharmaceuticals, Inc; Treatment 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), 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/0463/2021 of 29 October 2021). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Cilgavimab (AZD1061) - EMEA-002925-PIP01-20-M02

AstraZeneca AB; Prevention and treatment 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), the PDCO considered that for children below 12 years of age a waiver on own motion was appropriate, based on lack of significant therapeutic benefit as studies are considered not feasible, due to the current epidemiology of COVID-19. The PDCO therefore adopted an opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0048/2022 of 11 February 2022), containing a waiver on own motion in children from birth to less than 12 years of age. The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Dalbavancin hydrochloride - EMEA-000016-PIP01-07-M09

AbbVie Ltd; Treatment of acute bacterial skin and skin structure infections (ABSSSI)

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO noted the replies provided by the applicant to the issues raised at Day 30 and adopted a favourable opinion on the modification of the agreed paediatric investigation plan (PIP) as set in the Agency's latest decision (P/0522/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Letermovir - Orphan - EMEA-001631-PIP01-14-M05

Merck Sharp & Dohme (Europe), Inc.; Prevention of cytomegalovirus infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO reviewed and accepted the information provided by the applicant since Day 30. Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0362/2019 of 4 November 2019). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of gram-negative bacterial infections

Day 60 opinion

Infectious Diseases

Note: Withdrawal request received on 12 October 2023

2.3.18. Ritonavir / nirmatrelvir - EMEA-003081-PIP01-21-M03

Pfizer Europe MA EEIG; Prevention of coronavirus disease 2019 (COVID-19) / Treatment 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), 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/0503/2022 of 1 December 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Tixagevimab (AZD8895) - EMEA-002900-PIP01-20-M02

AstraZeneca AB; Prevention and treatment 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), the PDCO considered that for children below 12 years of age a waiver on the Committee's motion was appropriate, based on lack of significant therapeutic benefit as studies are considered not feasible, due to the current epidemiology of COVID-19. The PDCO therefore adopted an opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0048/2022 of 11 February 2022), containing a waiver on own motion in children from birth to less than 12 years of age. The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Zanamivir - EMEA-001318-PIP01-12-M05

GlaxoSmithKline Trading Services Limited; Treatment of influenza / Prevention of influenza

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/0391/2020 of 23 October 2020). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Alprazolam - EMEA-003043-PIP01-21-M01

UCB Pharma SA.; Treatment of epileptic seizures

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/0220/2022 of 10 June 2022). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Ravulizumab - EMEA-001943-PIP04-20-M01

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorders

Day 60 opinion

Neurology

Summary of Committee discussion:

The applicant addressed most 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 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/0474/2021 of 21 December 2021).

2.3.23. Satralizumab - Orphan - EMEA-001625-PIP02-21-M02

Roche Registration GmbH; Treatment of generalised myasthenia gravis

Day 60 opinion

Neurology

Note: Withdrawal request received on 13 October 2023

2.3.24. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M04

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

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from the Day 30 discussion. Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0143/2020 of 18 April 2020). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.25. Lisocabtagene maraleucel - EMEA-001995-PIP01-16-M04

Bristol-Myers Squibb Pharma EEIG; Treatment of B-lymphoblastic leukaemia/lymphoma / Treatment of mature B-cell neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the October 2023 plenary meeting, a request for modification for lisocabtagene maraleucel for the treatment of B-lymphoblastic leukaemia/lymphoma and for the treatment of mature B-cell neoplasms. Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be acceptable. Therefore, the PDCO adopted a positive opinion on the conversion of the agreed PIP as set in the Agency's latest decision (P/0558/2021 of 31 December 2021) into a product-specific waiver for the treatment of B-lymphoblastic leukaemia/lymphoma and for the treatment of mature B-cell neoplasms on the grounds of lack of significant therapeutic benefit. The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. Lutetium (^{177}Lu) oxodotreotide - Orphan - EMEA-002950-PIP01-20-M01

Advanced Accelerator Applications; Treatment of gastroenteropancreatic neuroendocrine tumours

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the October 2023 plenary meeting, a request for modification for lutetium (^{177}Lu) oxodotreotide for the treatment of gastroenteropancreatic neuroendocrine tumours. The PDCO confirmed all the conclusions reached at Day 30. 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/0503/2021 of 3 December 2021). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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

Eli Lilly and Company; Treatment of obesity

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could be accepted. Within this modification procedure elements of Study 2 were updated.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0285/2023 of 24 July 2023). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Tapentadol (hydrochloride) - EMEA-000325-PIP01-08-M11

Grünenthal GmbH; Treatment of chronic pain

Day 60 opinion

Pain

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes on the timeline for PIP Studies 1, 5, 6, 7 could not be accepted. However, the PDCO considered that the timeline for Study 5 could be delayed by one year, to July 2024. Subsequently, the dependent Studies 1, 6 and 7 could be changed from February 2024 to February 2025, delaying the overall completion of the PIP. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0258/2020 of 15 July 2020). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.29. Mometasone (furoate) / glycopyrronium bromide / indacaterol - EMEA-001812-PIP01-15-M02

Novartis Europharm Limited; Treatment of asthma

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

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

Studies 2 and 3. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0362/2021 of 8 September 2021). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.30. Seltorexant - EMEA-002746-PIP01-20-M02

Janssen-Cilag International NV; Treatment of major depressive disorder

Day 60 opinion

Psychiatry

Summary of Committee discussion:

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

2.3.31. Lacosamide - EMEA-000402-PIP03-17-M08

UCB Pharma S.A.; Treatment of generalised epilepsy and epileptic syndromes

Day 30 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan (PIP), the PDCO considered that the proposed changes could not be accepted.
Postponing the agreed completion date of a study that is known to face recruitment and other challenges is not agreeable without a revision of the study design that will inevitably affect the timelines. Any such revision should be done in view of the study as a whole, not just the timelines in isolation.
The PDCO therefore adopted a negative opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0247/2022 of 8 July 2022).

2.3.32. Ebola Zaire Vaccine (rVSVΔG-ZEBOV-GP, live) - EMEA-001786-PIP01-15-M03

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

Day 30 opinion

Vaccines

Summary of Committee discussion:

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

agreed PIP as set in the Agency's latest decision (P/0429/2021 of 29 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

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

2.7.1. Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - EMEA-C1-002699-PIP01-19-M01

YES Pharmaceutical Development Services GmbH; Treatment of burns

Day 30 letter

Dermatology

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. EMEA-002674-PIP01-19

Treatment of acne vulgaris

Day 90 discussion

Dermatology

3.1.2. Modified messenger ribonucleic acid encoding human propionyl-coenzyme A carboxylase alpha and beta subunits encapsulated into lipid nanoparticles - Orphan - EMEA-003419-PIP01-23

Moderna Biotech Spain, S.L.; Treatment of propionic acidaemia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Modified mRNA encoding human methylmalonyl-coenzyme A mutase containing a polymorphism at position 671 - Orphan - EMEA-003437-PIP01-23

Moderna Biotech Spain, S.L.; Treatment of methylmalonic acidaemia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. EMEA-003350-PIP01-23

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.5. Inebilizumab - EMEA-001911-PIP03-23

Treatment of immunoglobulin G4-related disease (IgG4-RD)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.6. Broadly neutralising anti-HIV human monoclonal antibody - EMEA-003392-PIP01-23

Treatment of human immunodeficiency virus (HIV-1) infection

Day 90 discussion

Infectious Diseases

3.1.7. Vemircopan - Orphan - EMEA-002863-PIP02-23

Alexion Europe SAS; Treatment of generalised myasthenia gravis

Day 90 discussion

Neurology

3.1.8. Olutasidenib - Orphan - EMEA-003421-PIP01-23

Rigel Pharmaceuticals B.V.; Treatment of acute myeloid leukaemia

Day 90 discussion

Oncology

3.1.9. Tamibarotene - Orphan - EMEA-003329-PIP02-22

Syros Pharmaceutical (Ireland) Limited; Treatment of acute myeloid leukaemia / Treatment of myelodysplastic syndromes

Day 90 discussion

Oncology

3.1.10. Cedazuridine / decitabine - EMEA-003071-PIP02-23

Treatment of myelodysplastic syndromes

Day 90 discussion

Oncology / Haematology-Hemostaseology

3.1.11. Taldefgrobep alfa - Orphan - EMEA-003386-PIP01-22

Biohaven Bioscience Ireland Limited; Treatment of spinal muscular atrophy

Day 90 discussion

Other

3.1.12. Complement factor B antisense oligonucleotide - EMEA-003396-PIP01-23

Treatment of immunoglobulin A nephropathy (IgAN)

Day 90 discussion

Uro-nephrology

3.1.13. EMEA-003480-PIP01-23

Treatment of psoriasis

Day 60 discussion

Dermatology

3.1.14. Lutikizumab - EMEA-003481-PIP01-23

Treatment of hidradenitis suppurativa

Day 60 discussion

Dermatology

3.1.15. EMEA-003478-PIP01-23

Treatment of psoriasis

Day 60 discussion

Dermatology

3.1.16. Bitopertin - Orphan - EMEA-000439-PIP03-23

Disc Medicine B.V.; Treatment of erythropoietic protoporphyria / Treatment of X-linked protoporphyria

Day 60 discussion

Dermatology / Haematology-Hemostaseology

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

Novo Nordisk A/S; Treatment of sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

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

Treatment of haemophilia B

Day 60 discussion

Haematology-Hemostaseology

3.1.19. Ianalumab - EMEA-002338-PIP05-23

Treatment of immune thrombocytopenia (ITP)

Day 60 discussion

Haematology-Hemostaseology

3.1.20. EMEA-003002-PIP04-23

Treatment of systemic sclerosis (SSc)

Day 60 discussion

Immunology-Rheumatology-Transplantation

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

Immedica Pharma AB; Treatment in allogenic stem cell transplantation

Day 60 discussion

Immunology-Rheumatology-Transplantation / Oncology

3.1.22. Radiprodil - EMEA-003462-PIP01-23

Treatment of GRIN-related disorders

Day 60 discussion

Neurology

3.1.23. EMEA-003477-PIP01-23

Treatment of appetite and general nutrition disorders

Day 60 discussion

Nutrition

3.1.24. (3S,3'S,3a'S,10a'S)-6-chloro-3'-(3-chloro-2-fluorophenyl)-1'-(cyclopropylmethyl)-6'-methyl-2-oxo-1,2,3',3a',10',10a'-hexahydro-1'H-spiro[indole-3,2'-pyrrolo[2',3':4,5]pyrrolo[1,2-b]indazole]-7'-carboxylic acid - Orphan - EMEA-003260-PIP03-23

Boehringer Ingelheim International GmbH; Treatment of soft tissue sarcoma

Day 60 discussion

Oncology

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

Treatment of melanoma

Day 60 discussion

Oncology

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

Treatment of hereditary angioedema (HAE)

Day 60 discussion

Other

3.1.27. Imlifidase - Orphan - EMEA-002183-PIP03-23

Hansa Biopharma AB; Treatment of anti–glomerular basement membrane (anti-GBM) disease

Day 60 discussion

Other / Uro-nephrology

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

Prevention of migraine

Day 60 discussion

Pain / Neurology

3.1.29. Atacicept - EMEA-002004-PIP04-23

Treatment immunoglobulin A nephropathy

Day 60 discussion

Uro-nephrology

3.1.30. EMEA-003487-PIP01-23

Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

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

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.32. Elinzanetant - EMEA-003500-PIP01-23

Treatment of vasomotor symptoms caused by endocrine therapy in women with, or at high risk for developing hormone-receptor positive breast cancer

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Humanised IgG1 kappa monoclonal antibody directed against IGF-1R - EMEA-003499-PIP01-23

Treatment of thyroid eye disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.34. Sitagliptin / dapagliflozin - EMEA-003486-PIP01-23

Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. Govorestat - Orphan - EMEA-003365-PIP02-23

Applied Therapeutics, Inc; Treatment of galactosemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

3.1.36. Mitapivat - Orphan - EMEA-002684-PIP03-23

Agios Netherlands B.V.; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.1.37. Allogeneic cultured postnatal thymus-derived tissue - Orphan - EMEA-003496-PIP01-23

Enzyvant Therapeutics Ireland Limited; Treatment of congenital athymia

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.39. *Betula pendula* pollen allergoid, mannan-conjugated, polymerised - EMEA-003492-PIP01-23

Treatment of allergic rhinitis/rhino-conjunctivitis / Treatment of birch pollen allergic rhinitis/rhino-conjunctivitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.40. *Dermatophagoides pteronyssinus* allergoid, mannan-conjugated, polymerised / *Dermatophagoides farinae* allergoid, mannan-conjugated, polymerised - EMEA-003493-PIP01-23

Treatment of allergic rhinitis/rhino-conjunctivitis / Treatment of house dust mite allergic rhinitis/rhino-conjunctivitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.41. *Phleum pratense* pollen allergoid, mannan-conjugated, polymerised / *Dactylis glomerata* pollen allergoid, mannan-conjugated, polymerised - EMEA-003491-PIP01-23

Treatment of grass pollen allergic rhinitis/rhino-conjunctivitis / Treatment of allergic rhinitis/rhino-conjunctivitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.42. Proline derivative - EMEA-003440-PIP01-23

Treatment of type 1 interferonopathies

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.43. Diflunisal - Orphan - EMEA-003490-PIP01-23

AO Pharma AB; Treatment of transthyretin amyloid amyloidosis

Day 30 discussion

Neurology

3.1.44. EMEA-003495-PIP01-23

Treatment of tenosynovial giant cell tumours

Day 30 discussion

Oncology

3.1.45. EMEA-003489-PIP01-23

Treatment of relapsed and refractory solid malignant tumours / Treatment of relapsed and refractory malignant neoplasms of the hematopoietic and lymphoid tissue / Treatment of malignant neoplasms of the hematopoietic and lymphoid tissue

Day 30 discussion

Oncology

3.1.46. Oregovomab - EMEA-003497-PIP01-23

Treatment of ovarian cancer

Day 30 discussion

Oncology

3.1.47. Rilvegostomig - EMEA-003501-PIP01-23

Treatment of biliary tract cancer / Treatment of lung cancer

Day 30 discussion

Oncology

3.1.48. Lotilaner - EMEA-003488-PIP01-23

Treatment of *Demodex* blepharitis

Day 30 discussion

Ophthalmology / Infectious Diseases

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

Prevention of respiratory syncytial virus (RSV) disease

Day 30 discussion

Vaccines

3.1.50. DNA plasmid encoding HPV type 18 consensus E6 and E7 proteins / DNA plasmid encoding HPV type 16 consensus E6 and E7 - EMEA-002022-PIP02-23

Treatment of low-grade squamous intraepithelial lesions (LSIL) of the anus caused by HPV types 16 and 18 / Treatment of high-grade squamous intraepithelial lesions (HSIL) of the anus caused by HPV types 16 and 18

Day 30 discussion

Vaccines / Infectious Diseases

3.1.51. Human papillomavirus type 58 L1 protein / human papillomavirus type 52 L1 protein / human papillomavirus type 45 L1 protein / human papillomavirus type 33 L1 protein / human papillomavirus type 31 L1 protein / human papillomavirus type 18 L1 protein / human papillomavirus type 16 L1 protein / human papillomavirus type 11 L1 protein / human papillomavirus type 6 L1 protein - EMEA-003209-PIP02-23

Prevention of infection by human papillomavirus (HPV)

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. Dupilumab - EMEA-C1-001501-PIP07-20-M01

Sanofi Winthrop Industrie; Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

3.2.2. Doxribtimine / doxecitine - EMEA-C1-003210-PIP01-22

UCB Pharma S.A.; Treatment of thymidine kinase 2 deficiency

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Avibactam / ceftazidime - EMEA-C-001313-PIP01-12-M13

Pfizer Europe MA EEIG; Treatment of intra-abdominal infections

Day 30 discussion

Infectious Diseases

3.2.4. Eliglustat - EMEA-C-000461-PIP02-11-M05

Sanofi B.V.; Treatment of Gaucher disease type 1 and type 3

Day 30 discussion

Other

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Birch Bark Extract - EMEA-001299-PIP01-12-M02

Amryt Pharmaceuticals DAC; Treatment of skin injuries

Day 30 discussion

Dermatology

3.3.2. Delgocitinib - EMEA-002329-PIP02-20-M03

LEO Pharma A/S; Treatment of chronic hand eczema

Day 30 discussion

Dermatology

3.3.3. Glycopyrronium bromide - EMEA-002383-PIP01-18-M03

Dr. August Wolff GmbH & Co. KG - Arzneimittel; Treatment of hyperhidrosis

Day 30 discussion

Dermatology

3.3.4. Interleukin-23 receptor antagonist peptide - EMEA-003301-PIP01-22-M01

Janssen-Cilag International NV; Treatment of psoriasis

Day 30 discussion

Dermatology

3.3.5. Ritlecitinib - EMEA-002451-PIP01-18-M02

Pfizer Europe MA EEIG; Treatment of alopecia areata

Day 30 discussion

Dermatology

3.3.6. Venglustat - Orphan - EMEA-001716-PIP04-19-M01

Sanofi B.V.; Treatment of GM1 gangliosidosis / Treatment of GM2 gangliosidosis / Treatment of galactosialidosis / Treatment of sialidosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Filgotinib - EMEA-001619-PIP03-16-M02

Galapagos NV; Treatment of ulcerative colitis / Treatment of Crohn’s disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.8. Mirikizumab - EMEA-002208-PIP01-17-M03

Eli Lilly and Company; Treatment of ulcerative colitis / Treatment of Crohn's disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.9. Potassium chloride / sodium chloride / ascorbic acid / sodium sulfate / sodium ascorbate / polyethylene glycol 3350 – EMEA-001705-PIP02-15-M05

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 30 discussion

Gastroenterology-Hepatology

3.3.10. Zinc gluconate / alisitol / retinyl palmitate - Orphan - EMEA-002198-PIP01-21-M01

Vanessa Research Spain S.L.; Treatment of microvillus inclusion disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.11. Iron as ferric maltol - EMEA-001195-PIP01-11-M07

Norgine BV; Treatment of iron deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3.12. Baricitinib - EMEA-001220-PIP01-11-M09

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.13. Cendakimab - EMEA-002640-PIP01-19-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of eosinophilic esophagitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.14. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19-M03

Sanofi B.V.; Treatment of immune thrombocytopenia

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Merck Sharp & Dohme (Europe), Inc.; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 30 discussion

Infectious Diseases

3.3.16. Vaborbactam / meropenem - EMEA-001731-PIP01-14-M04

Menarini International Operations Luxembourg S.A.; Treatment of gram-negative bacterial infections

Day 30 discussion

Infectious Diseases

3.3.17. Inebilizumab - EMEA-001911-PIP01-15-M05

Horizon Therapeutics Ireland DAC; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

Neurology

3.3.18. Pridopidine (hydrochloride) - Orphan - EMEA-003174-PIP01-21-M01

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

Day 30 discussion

Neurology

3.3.19. Vamorolone - Orphan - EMEA-001794-PIP02-16-M06

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

Day 30 discussion

Other

3.3.20. Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M04

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

Day 30 discussion

Pain

3.3.21. Mirabegron - EMEA-000597-PIP02-10-M10

Astellas Pharma Europe B.V.; Treatment of idiopathic overactive bladder

Day 30 discussion

Uro-nephrology

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

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Vaccines

3.3.23. Recombinant respiratory syncytial virus pre-fusion F protein, adjuvanted with AS01E - EMEA-002904-PIP01-20-M01

GlaxoSmithKline Biologicals S.A.; Prevention of lower respiratory tract disease caused by respiratory syncytial virus

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 16 October 2023 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

No item

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. Lasofoxifene- EMEA-08-2023

Sermonix Pharmaceuticals, INC; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant neoplasms / Treatment of adults with oestrogen receptor positive, human epidermal receptor negative advanced or metastatic breast cancer with an ESR1 mutation

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.

6.1.2. Anti-microtubule binding region tau antibody - EMEA-09-2023

Eisai Limited; All classes of medicinal products for treatment of Alzheimer's disease / Treatment of Alzheimer's disease (AD) / Treatment of dominantly inherited Alzheimer's disease (DIAD)

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 indications was not 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

No item

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

The Chair welcomed Agnes Gyurasics as alternate for Austria.

The following members/alternates have been appointed by the European Commission to the PDCO to represent patients' organisations for a 3-year term from 01 August 2023:

- Member: Eric Vermeulen
- Alternate: Victoria Romero Pazos

The Chair thanked Vessela Boudinova for her contribution as alternate for Bulgaria.

9.1.2. Vote by Proxy

None

9.1.3. Strategic Review and Learning Meeting (SRLM) - Madrid, Spain 17-18 October 2023

Summary of Committee discussion:

The Committee was updated about the next strategic review and learning meeting in Madrid.

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:

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

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. Formulation Working Group

PDCO member: Brian Aylward (*ad interim*)

Summary of Committee discussion:

The Chair of the Formulation Working Group (FWG) identified the products which will require FWG 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:

The upcoming ITF meeting was presented to the Committee for information.

9.3.5. EMA's Working Parties in the New Model – Formulation Working Group integration

PDCO Chair: Brian Aylward

Summary of Committee discussion:

The PDCO Chair updated the Committee about the reorganisation of the EMA's Working Parties and the integration of the Formulation Working Group (FWG) into the new model within the Quality Domain, following discussion on the 3 Year workplan and the prioritisation and expertise required for the FWG held in September 2023. A launch for call for nominations at PDCO and Quality Working Party was done during the October 2023 plenaries.

The FWG will become the Paediatric Formulation Organisational Expert Group (PFOEG) in the new model.

9.4. Cooperation within the EU regulatory network

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

Summary of Committee discussion:

The Committee was given an overview of the main discussion points and outcomes of the annual meeting of Enpr-EMA members and the annual Enpr-EMA workshop which took place on 09 and 10 October 2023. Members were informed that the meeting reports will be published on the Enpr-EMA website in due course and shared with the Committee members.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The 21 September 2023 and ad hoc 22 September 2023 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. 14 years of standard allergen PIP

Update on the 16th International Paul-Ehrlich-Seminar held on 07 September 2023

Experts: Susanne Kaul, Birgit Ahrens

Summary of Committee discussion:

Feedback on the 16th International Paul-Ehrlich Seminar in Langen, 06-09 September 2023 was presented to the PDCO.

The PDCO position, expressed in the letter to Bundesverband der Pharmazeutischen Industrie (BPI), was presented at the seminar.

10.2. ACT EU priority action (PA) 8 on methodology

Expert: Ditte Zerlang Andersen

Summary of Committee discussion:

Committee members were provided with an update of the ACT-EU Priority Action 8 on methodology guidance. Moreover, members were invited to express their interest to participate in the ACT EU methodology workshop, which is planned to take place on 22-23 November 2023 at the EMA.

10.3. Patient Experience Data (PED) project

Summary of Committee discussion:

The PDCO noted the presentation on PED and its relevance for paediatric medicines. Patients' views or preferences on medicines or living with a condition is particularly important for special populations such as children, who are often underrepresented in clinical trials. During the evaluation phase, collection of PED data by reliable and validated methodologies can contribute to the knowledge as supportive data to primary or secondary endpoints. PED may inform young people's preferences for formulations, adverse drug reactions (ADRs), risk minimisation measures (RMMs) or clinical outcomes. In the post authorisation phase, PED can be collected as part of real-world evidence (RWD) (e.g. in registries) to generate supportive evidence.

The two key deliverables of EMA's 2023 PED work are:

- Reflection paper on the best EU approach to generate, collect and analyse PED;
- Explore how to improve transparency.

On the basis of the 2022 workshop's outcomes, EMA will enable discussions within the Network on current status, next steps and how to monitor progress. Furthermore, EMA is preparing a list of actions and priorities that will be discussed with the EU Network and also shared with stakeholders, including PCWP and HCPWP.

Other actions are:

- EMA has set up an internal working group to coordinate cross-Agency expertise and draft the reflection paper, in collaboration with Network experts;
- Drafting expected to start in Q4 2023 and public consultation expected by Q1 2024.

ICH guidance:

- EMA supports PED global development and will contribute to ICH work on PED guidelines.

Currently, the priority for EMA is to draft the reflection paper and publish it for consultation, however it was agreed to continue multilateral stakeholder cooperation to obtain the best regulatory outcomes, and to explore additional engagement opportunities as needed (e.g., focus groups or workshops) for key topics. Any other multistakeholder discussion (such as focus groups or workshops) will be considered during the public consultation.

EU regulators will explore how to better reflect in the assessment report (AR) the way PED is assessed as well as the rationale for acceptance/exclusion for Benefit/Risk decision-making.

Finally a call for PDCO experts to join PED expertise community was made and PDCO members were invited to express their interest.

10.4. Request for feedback on recommendation for inclusion of adolescents in adult trials from the Clinical Trials Coordination Group (CTCG)

PDCO member: Anette Solli Karlsen

Summary of Committee discussion:

The PDCO was informed about recent interactions with CTCG.

10.5. Internal review of the ICH M15 MIDD Guideline

Summary of Committee discussion:

The Committee was informed about the objective of this guideline which is to outline general model-informed drug development (MIDD) principles and describe recommended

practices with respect to planning, conduct, reporting, interpretation, and alignment on the impact on regulatory decision-making.

10.6. PDCO Paediatric Efficacy and Safety presentation at WHO Paediatric Regulatory Network Meeting

PDCO member: Sara Vennberg

Summary of Committee discussion:

The presentation given by Sara Vennberg via remote participation at last month's WHO Paediatric Regulatory Network Meeting in Nairobi, was made available to PDCO members. The presentation was well received by an audience of researchers and regulators from outside of the EU. It was highlighted that a wealth of regulatory science and principles can be found in the EPARs of products authorised centrally in the EU. It was noted that PDCO is a leading Scientific Committee with valuable knowledge and experience to share with recently established and fledgling regulatory agencies across the world.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The group discussed general issues related to ongoing paediatric procedures.

11.2. Neonatology

Summary of Committee discussion:

The group discussed topics for revision of the neonatal guideline.

11.3. HIV

Summary of Committee discussion:

The group discussed ongoing paediatric procedures.

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 10-13 October 2023 PDCO meeting, which was held remotely.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Brian Aylward	Chair	Ireland	No interests declared	
Johanna Wernsperger	Member	Austria	No interests declared	
Agnes Gyurasics	Alternate	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in discussion, final deliberations and voting on:	2.2.7. Ruxolitinib (phosphate) - EMEA-C-000901-PIP03-16-M02 2.2.8. Ruxolitinib (phosphate) - EMEA-C-000901-PIP04-17-M02
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
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	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	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Anastasia Mountaki	Alternate	Greece	No interests declared	
Adrienn Horváth	Member	Hungary	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Cinzia Ciceroni	Alternate	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No participation in discussion, final deliberations and	2.2.2. Nirsevimab - EMEA-C-001784-PIP01-15-M04

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
			voting on:	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
John-Joseph Borg	Member	Malta	No interests declared	
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 participation in final deliberations and voting on:	2.2.2. Nirsevimab - EMEA-C-001784-PIP01-15-M04 3.3.15. Fully human IgG1 RB-1 YTE anti-RSV F monoclonal antibody - EMEA-002755-PIP01-19-M02
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
Tomasz Grybek	Member	Patients' Organisation Representative	No interests declared	
Jaroslav Sterba	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Viviana Giannuzzi	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Patricia Felgueiras Seabra Durao	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Victoria Romero Pazos	Alternate	Patients' Organisation Representative	No interests declared	
Celine Chu	Expert	France	No interests declared	
María Estela Moreno Martín	Expert	Spain	No interests declared	
Ditte Zerlang Andersen	Expert	Denmark	No interests declared	
Susanne Kaul	Expert	Germany	No interests declared	
Birgit Ahrens	Expert	Germany	No interests declared	
Karen Rösner-Friese	Expert	Germany	No interests declared	
Olga Kholmanskikh	Expert	Belgium	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.

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