9 September 2022
EMA/PDCO/674607/2022
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 19-22 July 2022

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

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. Due to the coronavirus (COVID-19) pandemic, and the associated EMA Business Continuity Plan (BCP), 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 interests were declared.

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

The Chair thanked the departing alternate for his contributions to the Committee.

The Chair announced the start of the Czech Republic presidency of the Council of the European Union (EU).

1.2. Adoption of agenda

The agenda for 19-22 July 2022 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 21-24 June 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. Ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA (Hologene 5) - Orphan - EMEA-003137-PIP01-21

Holostem Terapie Avanzate s.r.l.; Treatment of epidermolysis bullosa

Day 120 opinion

Dermatology

Summary of Committee discussion:

The PDCO agreed on a positive opinion for a PIP for ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA (Hologene 5) for the treatment of epidermolysis bullosa in all paediatric age groups from birth to less than 18 years of age. The completion of the paediatric development plan is not deferred.

2.1.2. Tezepelumab - EMEA-001613-PIP04-21

Amgen Europe B.V.; Treatment of chronic spontaneous urticaria

Day 120 opinion

Dermatology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for tezepelumab for the paediatric population from 2 years to less than 18 years of age, in the condition of treatment of chronic spontaneous urticaria was adopted.
The PDCO agreed on a waiver in a subset of children from birth to less than 2 years of age on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset.
The PDCO granted a deferral for the completion of this PIP.

2.1.3. Clazakizumab - EMEA-001371-PIP02-21

CSL Behring GmbH; Prevention and treatment of rejection of transplanted kidney

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO agreed on a positive opinion for a PIP for clazakizumab for the prevention and treatment of rejection of transplanted kidney in children from 2 years to less than 18 years of age. A waiver was agreed for the age group below 2 years 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 completion of the paediatric development plan was deferred.

2.1.4. Tocilizumab - EMEA-000309-PIP09-21

Roche Registration GmbH; Treatment of systemic sclerosis

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, the Paediatric Committee adopted a positive opinion on D120 for tocilizumab for treatment of systemic sclerosis in paediatric population from 5 years to less than 18 years of age.
A waiver was granted for the paediatric population from birth to less than 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 the completion of this PIP.

2.1.5. Ibrexafungerp citrate - EMEA-002535-PIP04-21

SCYNEXIS, Inc.; Treatment of invasive candidiasis

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from birth to less than 18 years of age in the condition of treatment of invasive candidiasis was adopted. The PDCO granted a deferral for the completion of this PIP.

2.1.6. Corticotropin - EMEA-003097-PIP01-21

Amzell B.V.; Treatment of infantile spasms

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the July 2022 plenary meeting, an application for a paediatric investigation plan for corticotropin for the treatment of infantile spasms in children from 4 weeks to less than 25 months of age.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.

The PDCO adopted a positive opinion on a paediatric investigation plan with a partial waiver 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) below 4 weeks and above 25 months of age.

2.1.7. Cannabidiol - EMEA-003176-PIP01-21

Treatment of fragile X syndrome (FXS)

Day 120 opinion

Psychiatry

Note: Withdrawal request received on 12 July 2022

2.1.8. Troriluzole - EMEA-003084-PIP02-21

Treatment of obsessive-compulsive disorder

Day 120 opinion

Psychiatry

Note: Withdrawal request received on 12 July 2022

2.1.9. Zuranolone - EMEA-003119-PIP01-21

Biogen Netherlands B.V.; Treatment of postpartum depression

Day 120 opinion

Psychiatry

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for zuranolone for post pubertal females less than 18 years of age, in the condition of treatment of postpartum depression was adopted.
The PDCO agreed on a waiver for males from birth to less than 18 years of age and prepubertal females on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subsets.
The PDCO granted a deferral for the completion of this PIP.

2.1.10. Telmisartan / amlodipine / hydrochlorothiazide - EMEA-003229-PIP01-22

Krka, d.d., Novo mesto; Treatment of hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for telmisartan / amlodipine / hydrochlorothiazide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of hypertension on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.11. Rosuvastatin (calcium) / perindopril (tert-butylamin) - EMEA-003228-PIP01-22

KRKA, d.d., Novo mesto; Prevention of cardiovascular events / Treatment of hypertension / Treatment of dyslipidaemia

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO's view expressed at Day 30 was re-discussed and endorsed.
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 (calcium) / perindopril (tert-butylamin) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of dyslipidaemia, treatment of hypertension, prevention of cardiovascular events.
The PDCO emphasised that the granting of a waiver for the conditions mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.12. Rosuvastatin / valsartan - EMEA-003240-PIP01-22

Teva B.V.; Treatment of dyslipidaemia / Treatment of hypertension / Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO's view expressed at Day 30 was re-discussed and endorsed.
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 / valsartan for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of dyslipidaemia, treatment of hypertension, prevention of cardiovascular events.
The PDCO emphasised that the granting of a waiver for the conditions mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the

Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.13. Bismuth (subcitrate) / tetracycline (hydrochloride) / metronidazole (benzoate) - EMEA-003224-PIP01-22

Verisfield Single Member S.A.; Treatment of *Helicobacter pylori* infection

Day 60 opinion

Infectious Diseases / Gastroenterology-Hepatology

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 bismuth (subcitrate) / tetracycline (hydrochloride) / metronidazole (benzoate) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of *Helicobacter pylori* infection.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.14. Pegcetacoplan - EMEA-002600-PIP04-22

Apellis Netherlands B.V.; Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for pegcetacoplan for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of amyotrophic lateral sclerosis on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2.1.15. Suvecaltamide (hydrochloride) - EMEA-003248-PIP01-22

Jazz Pharmaceuticals Ireland Ltd; Treatment of essential tremor

Day 60 opinion

Neurology

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

2.1.16. Autologous CD4+ and CD8+ T cells transduced with lentiviral vector encoding a chimeric antigen receptor (CAR) directed against human B cell maturation antigen (BCMA) and preserving the T cell phenotype of the leukapheresis starting material (PHE885) - EMEA-003231-PIP01-22

Novartis Europharm Limited; Treatment of multiple myeloma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2022 plenary meeting, an application for a product specific waiver for autologous CD4+ and CD8+ T cells transduced with lentiviral vector encoding a chimeric antigen receptor (CAR) directed against human B cell maturation antigen (BCMA) and preserving the T cell phenotype of the leukapheresis starting material (PHE885) for the treatment of multiple myeloma on the grounds that the disease does not occur in children.
The PDCO agreed with all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 on a product specific waiver for this product for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of multiple myeloma" on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.17. Gallium 68-labelled prostate-specific membrane antigen-11 (68Ga-PSMA-11) - EMEA-003236-PIP01-22

ROTOP Pharmaka GmbH; Visualisation of prostate specific membrane antigen in prostate cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2022 plenary meeting, an application for a product specific waiver for gallium 68-labelled prostate-specific membrane antigen-11 (68Ga-PSMA-11) for the visualisation of prostate specific membrane antigen in prostate cancer based on lack of safety and on the grounds that the disease does not occur in

children.
The PDCO agreed with all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 on a product specific waiver for this product for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "visualisation of prostate specific membrane antigen in prostate cancer" on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.18. Golidocitinib - EMEA-003246-PIP01-22

Dizal (Jiangsu) Pharmaceuticals Co., Ltd.; Treatment of peripheral T cell lymphoma

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 golidocitinib for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of peripheral T cell lymphoma based on the ground of lack of significant therapeutic benefit because studies are unfeasible.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.19. Monalizumab - EMEA-002751-PIP02-22

AstraZeneca AB; 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 monalizumab 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 that the disease does not occur in the paediatric population, 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. Oleclumab - EMEA-003234-PIP01-22

AstraZeneca AB; Treatment of lung cancer / Treatment of pancreatic cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

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

2.1.21. Toripalimab - EMEA-003243-PIP01-22

TopAlliance Biosciences, Inc.; Treatment of all conditions in the category of malignant neoplasms (except CNS, haematopoietic and lymphoid tissue and melanoma)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2022 plenary meeting, an application for a product specific waiver for toripalimab for the treatment of all conditions in the category of malignant neoplasms (except CNS, haematopoietic and lymphoid tissue) on the grounds of lack of significant therapeutic benefit.
The PDCO agreed with all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 on a product specific waiver for this product for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of all conditions in the category of malignant neoplasms (except CNS, haematopoietic and lymphoid tissue and melanoma)" on the grounds that this medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.22. Vepsitamab - EMEA-003230-PIP01-22

Amgen Europe B.V.; Treatment of colorectal carcinoma / Treatment of gastric cancer and gastro-oesophageal junction cancer / Treatment of pancreatic cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2022 plenary meeting, an application for a product specific waiver for vepsitamab for the "treatment of gastrointestinal solid tumours malignant and unspecified expressing MUC17" on the grounds that the disease does not occur in the paediatric population.
The PDCO agreed with all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 on a product specific waiver for this product for all subsets of the paediatric population (from birth to less than 18 years of age) in the conditions of "treatment of gastric cancer and gastro-oesophageal junction cancer", "treatment of colorectal carcinoma" and "treatment of pancreatic cancer" on the grounds that the diseases or conditions for which the specific medicinal product is intended do not occur in the specified paediatric subset(s).
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.23. Xevinapant - Orphan - EMEA-003235-PIP01-22

Merck Healthcare KGaA; Treatment of head and neck epithelial malignant neoplasms

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 xevinapant for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of head and neck epithelial malignant neoplasms based on the ground 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. The PDCO identified patients with neuroblastoma and patients with rhabdomyosarcoma as an unmet need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.24. Dimeric protein comprised of two disulfide-linked monomers, each being a fully human fusion protein consisting of a modified extracellular domain of the human activin receptor type IIA fused to the fragment crystallizable domain of human IgG1 Fc including the hinge region, CH2 and CH3 domains (KER-050) - EMEA-003239-PIP01-22

Keros Therapeutics, Inc.; Treatment of myelodysplastic syndrome / Treatment of myelofibrosis

Day 60 opinion

Oncology / Haematology-Hemostaseology

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 conditions 'treatment of myelodysplastic syndrome' based 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) and 'treatment of myelofibrosis' based on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2.2. Opinions on Compliance Check

2.2.1. Nintedanib esylate - EMEA-C-001006-PIP05-18-M01

Boehringer Ingelheim International GmbH; Treatment of fibrosing interstitial lung diseases

Day 30 opinion

Pneumology - Allergology

Summary of Committee discussion:

The PDCO adopted on 22 July 2022 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0438/2021) of 29 October 2021.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Treprostinil - EMEA-000207-PIP01-08-M07

Ferrer Internacional, S.A.; Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular Diseases

Note: Withdrawal request received on 20 July 2022

2.3.2. Evinacumab - EMEA-002298-PIP01-17-M04

Regeneron Ireland DAC; Treatment of elevated cholesterol

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, 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/0514/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Guselkumab - EMEA-001523-PIP04-19-M01

Janssen-Cilag International N.V.; Treatment of ulcerative colitis

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, 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/0461/2020 of 4 December 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Odevixibat - Orphan - EMEA-002054-PIP03-20-M01

Albireo AB; Treatment of Alagille syndrome

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, the PDCO considered that some of the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0246/2021 of 9 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Ustekinumab - EMEA-000311-PIP04-13-M05

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

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0083/2021 of 19 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Plasma kallikrein inhibitor - EMEA-002723-PIP01-19-M01

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 60 opinion

Haematology-Hemostaseology

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, 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/0416/2020 of 23 October 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Roxadustat - EMEA-001557-PIP01-13-M06

Astellas Pharma Europe B.V.; Treatment of anaemia due to chronic disorders

Day 60 opinion

Haematology-Hemostaseology

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, 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/0103/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Vonicog alfa - EMEA-001164-PIP01-11-M06

Baxalta Innovations GmbH; Treatment of Von Willebrand disease

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that proposed changes to timelines of Study 1 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/0494/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Apremilast - EMEA-000715-PIP02-11-M06

Amgen Europe B.V.; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0388/2019 of 4 December 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Apremilast - EMEA-000715-PIP05-13-M05

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

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0388/2019 of 4 December 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Baricitinib - EMEA-001220-PIP07-20-M01

Eli Lilly and Company Limited; Treatment of coronavirus disease 2019 (COVID-19)

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, the PDCO considered that some of the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0062/2021 of 5 February 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Belimumab - EMEA-000520-PIP02-13-M04

Glaxo Group Limited; Treatment of systemic lupus erythematosus

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, 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/0003/2020 of 6 January 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Grisnilimab setaritox / dafsolimab setaritox - Orphan - EMEA-002087-PIP01-16-M01

Xenikos B.V.; Treatment of acute graft versus host disease

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0053/2020 of 29 January 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Upadacitinib - EMEA-001741-PIP04-17-M03

AbbVie Ltd; Treatment of atopic dermatitis

Day 60 opinion

Immunology-Rheumatology-Transplantation / Dermatology

Note: Withdrawal request received on 15 July 2022

2.3.15. Baloxavir marboxil - EMEA-002440-PIP01-18-M03

Roche Registration GmbH; Prevention of influenza / Treatment 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, 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/0486/2021 of 3 December 2021).

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

2.3.16. BNT162b2 / tozinameran - EMEA-002861-PIP02-20-M04

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0547/2021 of 31 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Cabotegravir - EMEA-001418-PIP01-13-M05

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

Day 60 opinion

Infectious Diseases

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, 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/0040/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Cobicistat - EMEA-000969-PIP01-10-M06

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0007/2021 of 15 January 2021).

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

2.3.19. Emtricitabine / tenofovir alafenamide - EMEA-001577-PIP02-14-M05

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0034/2021 of 27 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M04

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0372/2020 of 9 September 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Rilpivirine - EMEA-000317-PIP02-18-M01

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

Day 60 opinion

Infectious Diseases

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 application for modifying the agreed paediatric investigation plan, 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/0039/2019 of 29 January 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Tenofovir alafenamide / rilpivirine / emtricitabine - EMEA-001679-PIP01-14-M02

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0107/2015 of 13 May 2015).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Zoliflodacin - EMEA-002599-PIP01-19-M01

Entasis Therapeutic Inc.; Treatment of gonococcal infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO agreed that a delay of the completion date of the PIP was needed. The proposed changes to Study 1 related to the timing and definition of some of the endpoints could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0073/2020 of 21 March 2020). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Fenfluramine (hydrochloride) - Orphan - EMEA-001990-PIP01-16-M05

Zogenix International Ltd; Treatment of Dravet syndrome

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0502/2021 of 2 December 2021).

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

2.3.25. Ozanimod (hydrochloride) - EMEA-001710-PIP02-14-M07

Bristol-Myers Squibb Pharma EEIG; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0415/2021 of 29 October 2021. The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. Peginterferon beta-1a - EMEA-001129-PIP01-11-M05

Biogen Idec Ltd; Treatment of multiple sclerosis

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, 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/0371/2019 of 14 November 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.27. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M05

Ipsen Pharma; Treatment of malignant solid tumours

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0282/2021 of 19 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Copanlisib - Orphan - EMEA-001757-PIP02-15-M03

Bayer AG; Treatment of mature B cell neoplasms / Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 60 opinion

Oncology

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, the PDCO considered 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/0402/2020 of 22 October 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.29. Durvalumab - EMEA-002028-PIP01-16-M03

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

Day 60 opinion

Oncology

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, the PDCO considered that the proposed changes could be accepted and the scope of the paediatric investigation plan was amended to exclude a condition. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0106/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.30. Tremelimumab - Orphan - EMEA-002029-PIP01-16-M03

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

Day 60 opinion

Oncology

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, the PDCO considered that the proposed changes could be accepted and the scope of the paediatric investigation plan was amended to exclude a condition. The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0107/2021 of 17 March 2021).

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

2.3.31. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M06

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the July 2022 plenary meeting, this request for modification for inotuzumab ozogamicin for the treatment of B cell acute lymphoblastic leukaemia.
The applicant proposed changes in two clinical studies, including modification of the start date of a study and completion of another.
The PDCO agreed to the changes and adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0134/2022 of 13 April 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.32. Lomitapide (as lomitapide mesylate) - EMEA-001124-PIP01-10-M05

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia

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, 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/0332/2019 of 10 September 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.33. Cannabidiol / delta-9-tetrahydrocannabinol - EMEA-000181-PIP02-13-M01

GW Pharma (International) B.V; Treatment of 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, 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/0298/2014 of 24 November 2014).

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

2.3.34. Adrenaline (epinephrine) - EMEA-002749-PIP01-19-M02

ARS Pharmaceuticals IRL, Limited; Treatment of allergic reactions

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, 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/0054/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.35. Cholera vaccine, recombinant, live, oral (strain CVD 103-HgR) - EMEA-001490-PIP01-13-M03

Emergent Netherlands B.V.; Treatment of cholera disease caused by *Vibrio cholerae* serogroup O1

Day 60 opinion

Vaccines

Note: Withdrawal request received on 21 July 2022

2.3.36. Finerenone - EMEA-001623-PIP03-20-M01

Bayer AG; Treatment of heart failure

Day 30 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, the PDCO considered that the proposed changes in the timelines of the agreed 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/0243/2021 of 9 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.37. Gadopiclenol - EMEA-001949-PIP02-18-M02

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

Day 30 opinion

Diagnostic

Summary of Committee discussion:

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

2.3.38. Efanesoctocog alfa - Orphan - EMEA-002501-PIP01-18-M03

Swedish Orphan Biovitrum AB (Publ).; Treatment of haemophilia A

Day 30 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, 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/0095/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.39. Etranacogene dezaparvovec - Orphan - EMEA-002722-PIP01-19-M02

CSL Behring GmbH; Treatment of haemophilia B

Day 30 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, 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/0104/2022 of 25 March 2022).
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. Asciminib - EMEA-C2-002347-PIP01-18

Novartis Europharm Limited; Treatment of chronic myeloid leukaemia

Day 30 letter

Oncology / Haematology-Hemostaseology

2.7.2. Leriglitazone - EMEA-C1-002106-PIP01-16-M02

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 30 letter

Neurology

2.7.3. Upadacitinib - EMEA-C1-001741-PIP03-16-M02

AbbVie Ltd; Treatment of Crohn's disease

Day 30 letter

Gastroenterology-Hepatology

2.7.4. Ruxolitinib (phosphate) - EMEA-C2-000901-PIP03-16-M02

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

Day 30 letter

Oncology

2.7.5. Ruxolitinib (phosphate) - EMEA-C2-000901-PIP04-17-M02

Novartis Europharm Limited; Treatment of chronic graft versus host disease

Day 30 letter

Oncology

2.7.6. Modified Vaccinia Ankara - Bavarian Nordic virus (smallpox) - EMEA-C1-001161-PIP02-11-M01

Bavarian Nordic A/S; Prevention of smallpox, monkeypox and related orthopoxvirus infection

Day 30 letter

Vaccines

2.7.7. Fluticasone furoate / triphenylacetic acid - 4-{(1R)-2-[(6-{2-[(2,6-dichlorobenzyl)oxy]ethoxy}hexyl)amino]-1-hydroxyethyl}-2-(hydroxymethyl)phenol - EMEA-C4-000431-PIP01-08-M12

Glaxo Group Limited; Treatment of asthma

Day 30 letter

Pneumology - Allergology

2.7.8. Edoxaban tosilate - EMEA-C4-000788-PIP02-11-M11

Daiichi-Sankyo Europe GmbH; Treatment of venous thromboembolism

Day 30 letter

Cardiovascular Diseases

2.7.9. Semaglutide - EMEA-C2-001441-PIP03-17-M02

Novo Nordisk A/S; Treatment of obesity

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.10. Ebola Zaire vaccine (rVSVΔG-ZEBOV-GP, live) - EMEA-C1-001786-PIP01-15-M02

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

Day 30 letter

Vaccines

2.7.11. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A/H5N1 - EMEA-C1-002869-PIP01-21

Seqirus Netherlands B.V.; Treatment of influenza due to identified zoonotic or pandemic influenza virus

Day 30 letter

Vaccines

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. Treprostinil - EMEA-003182-PIP01-22

Treatment of pulmonary arterial hypertension

Day 90 discussion

Cardiovascular Diseases

3.1.2. Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22

Krystal Biotech, Inc.; Treatment of dystrophic epidermolysis bullosa

Day 90 discussion

Dermatology

3.1.3. Recombinant fusion protein linking iduronate 2-sulfatase to engineered Fc with binding site for transferrin receptor - EMEA-002845-PIP01-20

Treatment of mucopolysaccharidosis II (Hunter syndrome)

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Resmetirom - EMEA-003087-PIP01-21

Treatment of non-alcoholic steatohepatitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.5. Cenerimod - EMEA-003108-PIP01-21

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.6. Efavaleukin alfa - EMEA-003156-PIP01-21

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Ianalumab - EMEA-002338-PIP03-21

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.8. Branaplam - EMEA-002204-PIP02-20

Treatment of Huntington's disease

Day 90 discussion

Neurology

3.1.9. Satralizumab - Orphan - EMEA-001625-PIP03-21

Roche Registration GmbH; Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 90 discussion

Neurology

3.1.10. Odronextamab - EMEA-003149-PIP01-21

Treatment of aggressive mature B cell non-Hodgkin lymphoma (B-NHL)

Day 90 discussion

Oncology

3.1.11. Freeze-dried allergen extract of *Betula pendula* pollen - EMEA-003117-PIP02-21

Diagnosis of IgE mediated allergy to tree pollen of the birch group

Day 90 discussion

Pneumology - Allergology

3.1.12. Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512) - Orphan - EMEA-003157-PIP01-21

Pfizer Europe MA EEIG; Treatment of focal segmental glomerulosclerosis (FSGS)

Day 90 discussion

Uro-nephrology

3.1.13. Yellow fever virus, strain vYF-247 - EMEA-003030-PIP02-21

Prevention of yellow fever disease

Day 90 discussion

Vaccines

3.1.14. Amlitelimab - EMEA-003233-PIP01-22

Treatment of atopic dermatitis

Day 60 discussion

Dermatology

3.1.15. CRN04777 - Orphan - EMEA-003242-PIP01-22

Crinetics Pharmaceuticals, Inc.; Treatment of congenital hyperinsulinism

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.16. EMEA-003241-PIP01-22

Treatment of sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

3.1.17. Itolizumab - Orphan - EMEA-003208-PIP02-22

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

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.18. Cilgavimab / tixagevimab - EMEA-003079-PIP01-22

Treatment of coronavirus disease 2019 (COVID-19) / Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Infectious Diseases

3.1.19. Opelconazole - EMEA-003249-PIP01-22

Treatment of bronchopulmonary aspergillosis

Day 60 discussion

Infectious Diseases

3.1.20. Enibarcimab - EMEA-003244-PIP01-22

Treatment of sepsis

Day 60 discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

3.1.21. Cemdisiran sodium - Orphan - EMEA-003237-PIP01-22

Regeneron Ireland DAC; Treatment of generalised myasthenia gravis (gMG)

Day 60 discussion

Neurology

3.1.22. Pozelimab - EMEA-003238-PIP01-22

Treatment of generalised myasthenia gravis (gMG)

Day 60 discussion

Neurology

3.1.23. Lutetium (177Lu) edotreotide - Orphan - EMEA-003245-PIP01-22

ITM Solucin GmbH; Treatment of somatostatin receptor (SSTR)-positive tumours

Day 60 discussion

Oncology

3.1.24. Obinutuzumab - Orphan - EMEA-001207-PIP04-22

Roche Registration GmbH; Prevention of cytokine release syndrome

Day 60 discussion

Oncology

Note: Withdrawal request received on 18 July 2022

3.1.25. Tinlarebant - Orphan - EMEA-003225-PIP01-22

Belite Bio, Inc; Treatment of Stargardt disease

Day 60 discussion

Ophthalmology

3.1.26. Tirzepatide - EMEA-002360-PIP02-22

Treatment of obesity

Day 60 discussion

Other

3.1.27. Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 - Orphan - EMEA-003232-PIP01-22

Inozyme Pharma Ireland Limited; Treatment of ectonucleotide pyrophosphatase / phosphodiesterase 1 (ENPP1) deficiency

Day 60 discussion

Other / Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.1.28. ABNCoV2 (AV2-cVLP-RBD SARS-CoV-2) - EMEA-003184-PIP01-22

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Vaccines / Infectious Diseases

3.1.29. Rosuvastatin / telmisartan - EMEA-003262-PIP01-22

Treatment of hypertension / Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

3.1.30. Adapalene, micronised / benzoyl peroxide, hydrous / clindamycin - EMEA-003263-PIP01-22

Treatment of acne vulgaris

Day 30 discussion

Dermatology

3.1.31. Fluorine (18F) PSMA-1007 - EMEA-003250-PIP01-22

For the diagnosis of prostate cancer recurrence after previous definitive treatment

Day 30 discussion

Diagnostic

3.1.32. Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 - Orphan - EMEA-003253-PIP01-22

Genzyme Europe B.V.; Treatment of achondroplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Crovalimab - EMEA-002709-PIP03-22

Treatment of Guillain-Barré syndrome (GBS)

Day 30 discussion

Haematology-Hemostaseology

3.1.34. Luspatercept - Orphan - EMEA-001521-PIP03-22

Bristol-Myers Squibb Pharma EEIG; Treatment of alpha-thalassaemia

Day 30 discussion

Haematology-Hemostaseology

3.1.35. Obinutuzumab - Orphan - EMEA-001207-PIP05-22

Roche Registration GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.36. EMEA-003252-PIP01-22

Treatment of multiple system atrophy

Day 30 discussion

Neurology

3.1.37. Pridopidine HCl - Orphan - EMEA-003174-PIP02-22

Prilenia Therapeutics B.V.; Treatment of amyotrophic lateral sclerosis (ALS)

Day 30 discussion

Neurology

3.1.38. EMEA-003260-PIP01-22

Treatment of dedifferentiated liposarcoma

Day 30 discussion

Oncology

3.1.39. EMEA-003197-PIP02-22

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

Day 30 discussion

Oncology

3.1.40. Trilaciclib - EMEA-002534-PIP03-22

Treatment of breast malignant neoplasms

Day 30 discussion

Oncology

3.1.41. Valemetostat tosilate - Orphan - EMEA-003256-PIP01-22

Daiichi Sankyo Europe GmbH; Treatment of mature T cell neoplasms

Day 30 discussion

Oncology

3.1.42. Zilovertamab vedotin - Orphan - EMEA-003257-PIP01-22

Merck Sharp & Dohme (Europe) Inc.; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue)

Day 30 discussion

Oncology

3.1.43. Batoclimab - EMEA-003162-PIP02-22

Treatment of thyroid eye disease (TED)

Day 30 discussion

Ophthalmology

3.1.44. Retatrutide - EMEA-003258-PIP01-22

Treatment of obesity

Day 30 discussion

Other

3.1.45. Tadalafil / finasteride - EMEA-003261-PIP01-22

Treatment of the signs and symptoms of benign prostatic hyperplasia (BPH) in adult males with an enlarged prostate

Day 30 discussion

Other

3.1.46. Lidocaine - EMEA-003255-PIP01-22

Local anaesthesia

Day 30 discussion

Pain

3.1.47. EMEA-003254-PIP01-22

Prevention of pulmonary infection/colonisation by improvement in airway mucosal clearance in patients with cystic fibrosis

Day 30 discussion

Pneumology - Allergology

Note: Withdrawal request received on 16 August 2022

3.1.48. Aticaprant - EMEA-003251-PIP01-22

Treatment of major depressive disorder

Day 30 discussion

Psychiatry

3.1.49. 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-PIP01-22

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. Artesunate - EMEA-C-002710-PIP01-19

Amivas Ireland Limited; Treatment of malaria

Day 30 discussion

Infectious Diseases

3.2.2. Lanadelumab - EMEA-C-001864-PIP01-15-M07

Takeda Pharmaceuticals International AG Ireland Branch; Prevention of hereditary angioedema attacks

Day 30 discussion

Other

3.2.3. Influenza virus A / turkey / turkey / 1 / 2005 (H5N1) NIBRG-23 strain, HA surface antigen - EMEA-C-002869-PIP03-21

Seqirus Netherlands B.V.; Treatment of influenza due to identified zoonotic or pandemic influenza virus

Day 30 discussion

Vaccines

3.2.4. Recombinant influenza hemagglutinin-strain B (Victoria lineage) / recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (Yamagata lineage) - EMEA-C3-002418-PIP01-18-M02

Sanofi Pasteur; Prevention of influenza infection

Day 30 discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M09

Glaxo Group Limited; Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.3.2. Finerenone - EMEA-001623-PIP03-20-M01

Bayer AG; Treatment of heart failure

Day 30 discussion

Cardiovascular Diseases

3.3.3. Sotatercept - Orphan - EMEA-002756-PIP01-19-M01

Merck Sharp & Dohme B.V.; Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.3.4. Vericiguat - EMEA-001636-PIP01-14-M03

Bayer AG; Treatment of left ventricular failure

Day 30 discussion

Cardiovascular Diseases

3.3.5. Ritlecitinib - EMEA-002451-PIP01-18-M01

Pfizer Europe MA EEIG; Treatment of alopecia areata

Day 30 discussion

Dermatology

3.3.6. 3,6-diamino-2,5-bis{N-[(1R)-1-carboxy-2-hydroxyethyl]carbamoyl} pyrazine (designated MB-102) - EMEA-001983-PIP01-16-M01

MediBeacon Inc.; Monitoring of renal function

Day 30 discussion

Diagnostic / Uro-nephrology

3.3.7. Levonorgestrel - EMEA-002474-PIP02-18-M01

Chemo Research, S.L.; Contraception

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.8. Etrasimod L-arginine - EMEA-002713-PIP01-19-M02

Arena Pharmaceuticals, Inc.; Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.9. Golimumab - EMEA-000265-PIP02-11-M04

Janssen Biologics B.V.; Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.10. Insulin human - EMEA-002116-PIP01-17-M01

ELGAN Pharma Ltd; Treatment of intestinal malabsorption in preterm infants

Day 30 discussion

Gastroenterology-Hepatology

3.3.11. Maralixibat chloride - Orphan - EMEA-001475-PIP03-17-M03

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 discussion

Gastroenterology-Hepatology

3.3.12. Tofacitinib - EMEA-000576-PIP03-12-M06

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.13. Marstacimab - Orphan - EMEA-002285-PIP02-19-M02

Pfizer Europe MAA EEIG; Treatment of congenital haemophilia A / Treatment of congenital haemophilia B

Day 30 discussion

Haematology-Hemostaseology

3.3.14. Guselkumab - EMEA-001523-PIP03-18-M02

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.15. Secukinumab - EMEA-000380-PIP06-19-M01

Novartis Europharm Limited; Treatment of systemic lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.16. Cefiderocol - EMEA-002133-PIP01-17-M03

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

Day 30 discussion

Infectious Diseases

3.3.17. Gepotidacin - EMEA-002443-PIP01-18-M01

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urinary tract infections (uUTI)

Day 30 discussion

Infectious Diseases

3.3.18. Gepotidacin - EMEA-002443-PIP02-18-M01

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urogenital gonorrhoea (GC)

Day 30 discussion

Infectious Diseases

3.3.19. Maribavir - Orphan - EMEA-000353-PIP02-16-M02

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of cytomegalovirus (CMV) infection

Day 30 discussion

Infectious Diseases

3.3.20. Taniborbactam / cefepime - EMEA-002576-PIP01-19-M01

Venatorx Pharmaceuticals, Inc.; Treatment of gram-negative bacterial infections

Day 30 discussion

Infectious Diseases

3.3.21. D-Sorbitol / naltrexone HCl / (RS)-baclofen - Orphan - EMEA-002164-PIP01-17-M03

Pharnext SA; Treatment of Charcot-Marie-Tooth disease type 1A

Day 30 discussion

Neurology

3.3.22. Diroximel fumarate - EMEA-002685-PIP02-19-M01

Biogen Netherlands B.V; Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.3.23. Ocrelizumab - EMEA-000310-PIP03-10-M06

Roche Registration GmbH; Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.3.24. Rozanolixizumab - Orphan - EMEA-002681-PIP01-19-M01

UCB Pharma S.A.; Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.3.25. Enasidenib - Orphan - EMEA-001798-PIP02-16-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.3.26. Atropine sulphate - EMEA-002545-PIP01-19-M01

Fondazione Per La Ricerca Farmacologica Gianni Benzi Onlus; Treatment of myopia

Day 30 discussion

Ophthalmology

3.3.27. Alpelisib - Orphan - EMEA-002016-PIP03-19-M02

Novartis Europharm Limited; Treatment of PIK3CA related overgrowth spectrum

Day 30 discussion

Other

3.3.28. Atidarsagene autotemcel - Orphan - EMEA-001765-PIP02-15-M04

Orchard Therapeutics (Netherlands) B.V.; Treatment of metachromatic leukodystrophy

Day 30 discussion

Other

3.3.29. Burosumab: Human recombinant IgG1 monoclonal antibody to fibroblast growth factor 23 (FGF23); KRN23 - Orphan - EMEA-001659-PIP01-15-M06

Kyowa Kirin Holdings B.V.; Treatment of X-linked hypophosphataemia

Day 30 discussion

Other

3.3.30. Eliglustat - Orphan - EMEA-000461-PIP02-11-M05

Genzyme Europe B.V.; Treatment of Gaucher disease (ICD-9-CM Diagnosis 272.7, Lipidoses) type 3 / Treatment of Gaucher disease (ICD-9-CM Diagnosis 272.7, Lipidoses) type 1

Day 30 discussion

Other

3.3.31. Begelomab - Orphan - EMEA-001744-PIP01-14-M01

ADIENNE S.r.l SU; Treatment of acute graft-versus-host disease (aGvHD)

Day 30 discussion

Other / Immunology-Rheumatology-Transplantation

3.3.32. Bupivacaine - EMEA-000877-PIP03-17-M04

Pacira Ltd; Postsurgical analgesia

Day 30 discussion

Pain

3.3.33. Finerenone - EMEA-001623-PIP01-14-M05

Bayer AG; Treatment of chronic kidney disease

Day 30 discussion

Uro-nephrology

3.3.34. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis* group B Protein 961c / recombinant *Neisseria meningitis* group B Protein 287- 953 / meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis* group B Protein 936-741 / outer membrane vesicles (OMV) from *N. meningitidis* strain NZ 98/254 - EMEA-001260-PIP01-11-M02

GlaxoSmithKline Biologicals SA; Prevention of meningococcal meningitis

Day 30 discussion

Vaccines

3.3.35. Ad26.RSV.preF - EMEA-002172-PIP02-17-M02

Janssen-Cilag International NV; Prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV)

Day 30 discussion

Vaccines / Infectious Diseases

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 August 2022 for Nomination of Rapporteur and Peer reviewer

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

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

5.1. New Scientific Advice

No item

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

No item

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

6.1.1. Adeno-associated viral vector serotype 2 expressing sCD59 (JNJ-81201887) – EMEA-02-2022

Janssen-Cilag International N.V; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of geographic atrophy (GA) secondary to dry age-related macular degeneration (AMD)

Summary of Committee discussion:

The applicability of the class waiver as referred to in the Agency's Decision CW/0001/2015 to the planned therapeutic indication was confirmed.

Other potential paediatric interests of this medicine suggested by PDCO: none identified at this stage.

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

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

7.1.1. Pyrrol-hydroxyethylpyridin-3-ol derivative (MIJ821) - EMEA-002946-PIP01-20

Novartis Europharm Limited

The request for the PDCO is to confirm whether treatment resistant depression (TRD) is covered by the condition major depressive disorder (MDD) in the PIP.

Summary of Committee discussion:

Based on the assessment of this application, the PDCO agreed a PIP for MIJ821 for the treatment of major depressive disorder (MDD) with a deferral and a waiver for the paediatric population from birth to less than 7 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), and from 7 years to less than 12 years of age on the grounds that the specific medicinal product is likely to be unsafe.

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 PDCO Chair thanked Michal Odermarsky for his work at the PDCO as he stepped down from his membership.

9.1.2. Vote by Proxy

No item

9.1.3. Election of PDCO Chairperson

Summary of Committee discussion:

The mandate of the PDCO Chair, Koenraad Norga, will expire on 13 September 2022.

The election of the new Chair took place in accordance with the PDCO rules of procedure.

The nominations received were presented to the Committee.

The PDCO elected Brian Aylward as PDCO Chair for a three-year mandate starting on 14 September 2022.

The PDCO and the Agency congratulated Brian Aylward on his election and wished him all the best in his new role as Chair of the Committee.

9.1.4. Strategic Review and Learning Meeting (SRLM) – Prague, 6 – 7 October 2022

PDCO member: Tomáš Boráň

Summary of Committee discussion:

PDCO members were invited to the next strategic review and learning meeting in Prague and the draft agenda was presented.

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Summary of Committee discussion:

The list of PIP-related CHMP procedures starting in June 2022, was presented to the PDCO members.

The PDCO Vice-Chair presented to the Committee members a proposal for the PDCO involvement in PIP-related CHMP procedures, including the following steps:

- Nomination of PDCO expert during plenary;

- Access to the assessment report (AR) for members from NCAs and for members not affiliated with NCA;

- Assessment of CHMP AR;

- Submission of comment with CHMP member's comment as PDCO expert comment;

- Feedback to PDCO.

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

Summary of Committee discussion:

The Chair of the Formulation Working Group (FWG) identified the products which will require FWG evaluation and discussion.

9.4. Cooperation within the EU regulatory network

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

No item

9.4.2. C4C Multistakeholder meeting on type I diabetes

Summary of Committee discussion:

C4C (Conect4Children) is organizing a multistakeholder meeting on type I diabetes mellitus, to be held attached to the international conference in advanced technologies and treatment for diabetes (ATDD) that is going to be conducted between the 22nd and 25th February 2023 in Berlin as a hybrid meeting, allowing face to face interaction and remote connection.

The programme committee will start the preparation meetings in September 2022, with a monthly meeting until the conference takes place. The topics are still under discussion, ranging through devices, type 2 diabetes, diabetes guideline, etc.

For this purpose, a call for interest is made in order to identify PDCO members that could participate in the activities related to this meeting.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The July 2022 agenda and minutes of the cluster were shared with the PDCO members for information.

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

No item

10. Any other business

10.1. COVID-19 update

Summary of Committee discussion:

The PDCO was updated on COVID treatments and vaccines of relevance for the paediatric population.

10.2. COVID-19 disease incidence in paediatric populations study

Summary of Committee discussion:

A presentation from the University Medical Center Utrecht (EU PE&PV (Pharmacoepidemiology and Pharmacovigilance) Research Network) was provided to the PDCO members on a study on COVID-19 incidence in the paediatric population and hospitalisation rates in the EU that they had conducted. The PDCO welcomed the initiative and underlined the great value in providing support to PDCO work in the area.

10.3. Revision of the guideline on clinical investigation of medicinal products in the treatment or prevention of diabetes mellitus

PDCO members: Carine de Beaufort, Agnes Gyurasics; CHMP member: Kristina Dunder

Summary of Committee discussion:

The guideline on clinical investigation of medicinal products in the treatment or prevention of diabetes mellitus is under revision, and paediatric specificities have been reviewed by PDCO representatives and were discussed during the plenary meeting.

PDCO feedback was well received by the CHMP drafting group who will resume the revision of the guideline in September 2022, any further comments could be provided until that time.

10.4. EMA records management system – update on Sharepoint migration

Summary of Committee discussion:

This point was postponed until further notice.

10.5. Exparel liposomal - EMEA/H/C/004586/II/0005 PDCO consultation from CHMP

Summary of Committee discussion:

The PDCO discussed a question raised by CHMP in relation to an ongoing marketing authorisation assessment procedure.

10.6. ICH E11A case example publication

PDCO member: Kristin Karlsson

Summary of Committee discussion:

The PDCO noted that the case study was published as part of the overall ICH document, and that comments on it will be sought as part of the public consultation. A link to the main ICH webpage will be provided on the EMA webpage.

It was noted that the ICH topic leaders had reached out to experts in advance and they had raised no major concerns with the example and the way it had been presented. PDCO members could follow up after the meeting with the topic leaders if the members wish to make additional comments.

10.7. Real World Evidence update, including DARWIN EU®

Summary of Committee discussion:

The PDCO noted the update on Real World Evidence (RWE).

Regarding the DARWIN EU®, it was noted that the Phase I is currently half-way through and first benefits will be delivered with pilot studies performed in 2022. Starting in 2024, the majority of committees in their decision-making will be routinely supported with reliable RWE. The DARWIN EU® should be fully operational in 2025/2026.

There is currently selection of data partners ongoing. 10 partners will be selected to onboard this year. Primary criteria for prioritisation were presented. The secondary criteria include non-EU data sources, databases which provide linkage and continuous follow up between primary and secondary care. Around 26 million EU patients is estimated to be covered in this selection.

The second part of the presentation described, which analyses and studies DARWIN EU® will deliver.

The third part gave an update on the RWE studies for PDCO. Status of studies requested by PDCO was presented.

Finally, as a reminder it was explained how to request RWE – email should be sent with a specific template.

A call for volunteers was made to be the liaison with the RWE team and work more closely with the project team on RWE studies.

11. Breakout sessions

11.1. Internal PDCO Operations

Summary of Committee discussion:

During this breakout session, matters relating the PDCO internal operations were discussed.

11.2. Neonatology

Summary of Committee discussion:

This breakout session was cancelled.

11.3. Paediatric oncology

Summary of Committee discussion:

The group discussed issues related to ongoing oncology procedures.

11.4. Vaccines

Summary of Committee discussion:

The group discussed general principles of COVID vaccines in children of very young age, and boosters.

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 19-22 July 2022 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium	No interests declared	
Karl-Heinz Huemer	Member	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in discussion, final deliberations and voting on:	2.7.4. Ruxolitinib (phosphate) - EMEA-C2-000901-PIP03-16-M02 2.7.5. Ruxolitinib (phosphate) - EMEA-C2-000901-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	
Tomas Boran	Member	Czechia	No interests declared	
Tereza Bazantova	Alternate	Czechia	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Louisa Braun Exner	Alternate	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Anne Paavola	Alternate	Finland	No interests declared	
Sylvie Benchetrit	Member	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member (Vice-Chair)	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Ágnes Gyurasics	Member (CHMP alternate)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-	Member	Latvia	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Freimane			applicable to this meeting	
Dovile Zacharkiene	Member	Lithuania	No interests declared	
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
Olivier Moes	Alternate	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No participation in final deliberations and voting on:	2.3.4. Odevixibat - Orphan - EMEA-002054-PIP03-20-M01
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	
Kristin Karlsson	Member	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jaroslav Sterba	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Alternate	Patients' Organisation Representative	No interests declared	
Michal Odermarsky	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
María Estela Moreno Martín	Expert	Spain	No interests declared	
Kristina Dunder	Expert	Sweden	No interests declared	
Susanne Brendler-Schwaab	Expert	Germany	No interests declared	
Ineta Popena	Expert	Latvia	No interests declared	
Janis Kurlovics	Expert	Latvia	No interests declared	
Ieva Rutkovska	Expert	Latvia	No interests declared	
Elita Poplavska	Expert	Latvia	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/