25 June 2021
EMA/PDCO/372631/2021
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 22-25 June 2021

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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

Table of contents

1. Introductions

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

The Chairperson opened the meeting by welcoming all participants. Due to the current coronavirus (COVID-19) outbreak, 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 based on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some meeting participants in upcoming discussions as included in the pre-meeting list of participants and restrictions.

Participants in this meeting were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions 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 and as included in the list of participants. All decisions taken at this meeting were made in the presence of a quorum of members (i.e. 17 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

Due to restricted involvement, the Chair Koenraad Norga deputised chairing the meeting to the Vice-Chair Sabine Scherer for the agenda topic(s) 2.7.3.

1.2. Adoption of agenda

The agenda for 22-25 June 2021 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 18-21 May 2021 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. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20

Kyowa Kirin Pharmaceutical Development Limited; Treatment of atopic dermatitis

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 the proposed medicine for children, in the condition of treatment of atopic dermatitis was adopted. The PDCO agreed on a waiver. The PDCO granted a deferral for the completion of this PIP.

2.1.2. Infigratinib - EMEA-002594-PIP02-20

QED Therapeutics Inc.; Treatment of achondroplasia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for patients, in the condition of treatment of achondroplasia was adopted. The PDCO granted a deferral for the completion of this PIP.

2.1.3. (1R,3S,5R)-2-(2-(3-acetyl-5-(2-methylpyrimidin-5-yl)-1H-indazol-1-yl)acetyl)-N-(6-bromo-3-methylpyridin-2-yl)-5-methyl-2-azabicyclo[3.1.0]hexane-3-carboxamide - Orphan - EMEA-002863-PIP01-20

Alexion Europe SAS; Treatment of Paroxysmal Nocturnal Haemoglobinuria

Day 120 opinion

Haematology-Hemostaseology

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 and adolescents, in the condition of treatment of paroxysmal nocturnal haemoglobinuria was adopted by majority. The PDCO agreed on a waiver. The PDCO granted a deferral for the completion of this PIP.

2.1.4. Deucravacitinib - EMEA-002350-PIP03-20

Bristol-Myers Squibb International Corporation; Treatment of systemic lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

In a 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, in the condition of treatment of systemic lupus erythematosus was adopted. The PDCO agreed on a waiver. The PDCO granted a deferral for the completion of this PIP.

2.1.5. Fenebrutinib - EMEA-002349-PIP03-20

Roche Registration GmbH; Treatment of multiple sclerosis

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO discussed the responses submitted by the Applicant at Day 120.
The Committee is still of the view that a deferral for the initiation of Study 2 cannot be agreed at this time.
The PDCO adopted a positive opinion, including a waiver in children and granted a deferral for completion of all studies in the PIP.

2.1.6. Itraconazole - Orphan - EMEA-002787-PIP01-20

Laboratoires S.M.B. S.A.; Prevention of invasive mould disease

Day 120 opinion

Action: For adoption

Infectious Diseases / Oncology

Note: Withdrawal request received on 24 June 2021

2.1.7. Zidebactam / cefepime - EMEA-002892-PIP01-20

Wockhardt Bio AG; Treatment of complicated urinary tract infections

Day 120 opinion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Uro-nephrology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant via the draft opinion, a positive opinion for the PIP for the proposed medicine, in the condition of treatment of complicated urinary tract infections was adopted. The PDCO

granted a deferral for the completion of this PIP.

2.1.8. Ublituximab - EMEA-002889-PIP02-20

CambPharma Solutions (CY) Ltd; Treatment of multiple sclerosis

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO re-discussed at Day 120 during the June 2021 plenary meeting a PIP for ublituximab, for the treatment of multiple sclerosis.
The PDCO confirmed all conclusions reached at Day 90 and took into consideration the information the applicant provided between Day 90 and Day 120.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for ublituximab was adopted. The PDCO agreed on a waiver in a subset of children. The PDCO granted a deferral for the completion of this PIP.

2.1.9. Vatiquinone - Orphan - EMEA-001238-PIP02-20

PTC Therapeutics International Limited; Treatment of mitochondrial disease

Day 120 opinion

Neurology

Summary of Committee discussion:

In June 2021 the PDCO noted that almost all remaining outstanding issues had been addressed by the applicant. Therefore the PDCO agreed a PIP for vatiquinone in the treatment of mithocondrial disease.

2.1.10. Afamitresgene autoleucel - Orphan - EMEA-002867-PIP01-20

Adaptimmune Ltd; Treatment of soft tissue sarcoma

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 120 during the June 2021 plenary meeting a PIP for afamitresgene autoleucel for the treatment of soft tissue sarcoma.
The Committee confirmed all conclusions reached at Day 90.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for autologous CD4+ and CD8+ T cells transduced with a lentiviral vector containing an affinity-enhanced T-cell receptor targeting the MAGE-A4 antigen for the treatment of soft tissue sarcoma was adopted. The PDCO agreed on a waiver in a subset of children. The PDCO granted a deferral for the completion of this PIP.

2.1.11. Allogeneic anti-CD19 CAR T cells produced using CRISPR/Cas9 to disrupt the T cell receptor alpha constant (TRAC) and β2-microglobulin (B2M) genomic loci and a recombinant adeno-associated viral vector to deliver donor template for insertion of the anti-CD19 CAR expression cassette into the TRAC locus - EMEA-002881-PIP01-20

CRISPR Therapeutics AG; Treatment of B-lymphoblastic leukaemia/lymphoma / treatment of mature B cell neoplasms

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 120 during the June 2021 plenary meeting a PIP for allogeneic anti-CD19 CAR T cells produced using CRISPR/Cas9 to disrupt the T cell receptor alpha constant (TRAC) and β2-microglobulin (B2M) genomic loci and a recombinant adeno-associated viral vector to deliver donor template for insertion of the anti-CD19 CAR expression cassette into the TRAC locus (CTX110) for the treatment of B-lymphoblastic leukaemia/lymphoma and for the treatment of mature B-cell neoplasms.
The PDCO confirmed all conclusions reached at Day 90 and took into consideration the information the applicant provided between Day 90 and Day 120.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for allogeneic anti-CD19 CAR T cells produced using CRISPR/Cas9 to disrupt the T cell receptor alpha constant (TRAC) and β2-microglobulin (B2M) genomic loci and a recombinant adeno-associated viral vector to deliver donor template for insertion of the anti-CD19 CAR expression cassette into the TRAC locus (CTX110) was adopted at day 120. The PDCO granted a deferral for the completion of this PIP.

2.1.12. Iptacopan - Orphan - EMEA-002705-PIP03-20

Novartis Europharm Limited; Paroxysmal Nocturnal Haemoglobinuria

Day 120 opinion

Other / Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application, the PDCO adopted a positive opinion at day 120 during their June 2021 plenary, for the PIP for the proposed medicine, in the condition treatment of paroxysmal nocturnal haemoglobinuria. The PDCO granted a deferral for the completion of this PIP.
The PDCO granted a waiver for the paediatric population.

2.1.13. Bardoxolone (methyl) - Orphan - EMEA-002488-PIP01-18

Reata Pharmaceuticals Inc.; Treatment of Alport syndrome

Day 120 opinion

Uro-nephrology

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, in the condition of treatment of Alport syndrome was adopted. The PDCO agreed on a waiver. The PDCO granted a deferral for the completion of this PIP.

2.1.14. Ramipril / amlodipine / hydrochlorothiazide - EMEA-002998-PIP01-21

Swyssi AG; 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 agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for ramipril / amlodipine / hydrochlorothiazide for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of hypertension.

2.1.15. Empagliflozin - EMEA-000828-PIP07-21

Boehringer Ingelheim International GmbH; Treatment of ischaemic heart disease

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

During its June 2021 plenary, the PDCO adopted a positive Opinion for a full waiver in all paediatric age subsets from birth to less than 18 years of age for the SGLT-2 transporter inhibitor empagliflozin for the condition “treatment of ischaemic heart disease”, on the grounds of likely lack of safety.

2.1.16. Semaglutide / insulin icodec - EMEA-002988-PIP01-21

Novo Nordisk A/S; Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for insulin icodec / semaglutide for the paediatric population.

2.1.17. Aldafermin - EMEA-003005-PIP01-21

NGM Biopharmaceuticals, Inc.; Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for the proposed product for all subsets of the paediatric population (0 to 18 years of age) in the condition of non-alcoholic steatohepatitis (NASH) on the grounds that the specific product is likely to be unsafe.

2.1.18. Immunoglobulin G1 anti-SORT1 human monoclonal antibody - EMEA-002997-PIP01-21

Alector, Inc.; Treatment of clinically symptomatic or pre-symptomatic frontotemporal dementia

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed the clarification submitted by the Applicant at Day 60.
The Committee tend to agree with the proposed by the Applicant condition 'Treatment of clinically symptomatic or pre-symptomatic frontotemporal dementia' covering the planned adult indication.
Based on the assessment of this application the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for immunoglobulin G1 anti-SORT1 human monoclonal antibody for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of clinically symptomatic or pre-symptomatic frontotemporal dementia.

2.1.19. Cetrelimab - EMEA-003006-PIP01-21

Janssen-Cilag International NV; Treatment of urothelial carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agrees with the applicant's request for a waiver. The PDCO recommends 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 urothelial carcinoma' 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).

2.1.20. Gemcitabine (hydrochloride) - EMEA-003007-PIP01-21

Janssen-Cilag International NV; Treatment of urothelial carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agrees with the applicant's request for a waiver. The PDCO recommends 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 urothelial carcinoma' 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).

2.1.21. Sivopixant - EMEA-003010-PIP01-21

Shionogi B.V.; Treatment of unexplained or refractory chronic cough

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

The PDCO's view expressed at Day 30 was re-discussed and endorsed.
Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for sivopixant for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of unexplained or refractory chronic cough.

2.1.22. Human Immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein - Orphan - EMEA-002995-PIP01-21

EspeRare Foundation; X-linked hypohidrotic ectodermal dysplasia (XLHED)

Day 60 opinion

Dermatology

Summary of Committee discussion:

The PDCO adopted a positive opinion on a PIP and a waiver in all age groups, at D60.

2.1.23. Gabapentin - EMEA-002994-PIP01-21

Treatment of postherpetic neuralgia

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition of treatment of postherpetic neuralgia on the grounds of lack of significant therapeutic benefit.

The PDCO emphasises 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 treatment of neuropathic and mixed pain 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. Datopotamab deruxtecan - EMEA-002976-PIP02-21

Treatment of breast cancer

Day 60 opinion

Action: For adoption

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition of treatment of breast cancer on the ground that the disease does not occur in children. Since the most appropriate waiver ground is considered to be disease not occurring in the paediatric population, the possibility to apply this to all pharmaceutical forms and all routes of administration was agreed to by the applicant.

The PDCO emphasises 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

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.

2.2.1. Enalapril maleate - EMEA-C-001706-PIP01-14-M03

Proveca Pharma Limited; Treatment of heart failure

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The PDCO adopted on D60 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0093/2021) of 17 March 2021.

2.2.2. Cobimetinib - EMEA-C-001425-PIP01-13-M05

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) with Ras, Raf or MEK pathway activation

Day 60 opinion

Oncology

Summary of Committee discussion:

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

- EMEA-C1-001425-PIP01-13-M01
- EMEA-C2-001425-PIP01-13-M03
- EMEA-C3-001425-PIP01-13-M04

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision P/0119/2021 of 17 March 2021.

2.2.3. Elvitegravir / Cobicistat / emtricitabine / Tenofovir alafenamide - EMEA-C-001460-PIP01-13-M05

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

Day 30 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures.
The following studies were assessed and considered compliant with the agreed paediatric investigation plan as part of this procedure:

- Study 1
- Study 2

The PDCO adopted on 25/06/2021 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0435/2020) of 13 November 2020.

2.2.4. Secukinumab - EMEA-C-000380-PIP02-09-M04

Novartis Europharm Ltd; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

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

- EMEA-C1-000380-PIP02-09-M02 - withdrawn
- EMEA-C2-000380-PIP02-09-M03

The PDCO adopted on 25 June 2021 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0372/2018) of 7 December 2018.

2.2.5. Dimethyl fumarate - EMEA-C-000832-PIP01-10-M05

Biogen Idec Ltd; Treatment of multiple sclerosis

Day 30 opinion

Action: For information. Adopted via written procedure on 14 June 2021

Neurology

2.2.6. Ticagrelor - EMEA-C-000480-PIP01-08-M14

AstraZeneca AB; Prevention of thromboembolic events

Day 30 letter

Cardiovascular Diseases / Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO discussed at Day 30 during the June 2021 plenary meeting a final compliance check for ticagrelor for the prevention of thromboembolic events.
The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-000480-PIP01-08-M07
- EMEA-C2-000480-PIP01-08-M11
- EMEA-C3-000480-PIP01-08-M11
- EMEA-C4-000480-PIP01-08-M13

The PDCO adopted on 25 June 2021 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0175/2021) of 9 April 2021.

2.2.7. Cipaglucosidase alfa - EMEA-C1-002447-PIP01-18-M01

Amicus Therapeutics Europe Limited; Treatment of glycogen storage disease Type II (Pompe's disease)

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Studies confirmed to be compliant as set out in the EMA's Decision P/0204/2021 of 10 May 2021.

2.2.8. Brentuximab vedotin - EMEA-C-000980-PIP01-10-M07

Takeda Pharma A/S; Treatment of Hodgkin lymphoma

Day 30 opinion

Oncology

Summary of Committee discussion:

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

- EMEA-C1-000980-PIP01-10-M02
- EMEA-C2-000980-PIP01-10-M04
- EMEA-C3-000980-PIP01-10-M05
- EMEA-C4-000980-PIP01-10-M05 – withdrawn
- EMEA-C5-000980-PIP01-10-M07

The PDCO adopted on 25 June 2021 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0013/2021) of 28 January 2021.

2.2.9. Ceftolozane / Tazobactam - EMEA-C-001142-PIP01-11-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of urinary tract infections

Day 30 opinion

Infectious Diseases

Summary of Committee discussion:

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

- EMEA-C1-001142-PIP01-11
- EMEA-C2-001142-PIP01-11-M01

The PDCO adopted on 25 June 2021 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0436/2020) of 13 November 2020.

2.2.10. Doravirine - EMEA-C2-001676-PIP01-14-M04

Merck Sharp & Dohme (Europe), Inc; Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 30 letter

Infectious Diseases

Summary of Committee discussion:

Study confirmed to be compliant as set out in the EMA's Decision (P/0177/2021) of 12 April 2021.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Clevidipine - EMEA-000282-PIP01-08-M03

Chiesi Farmaceutici S.p.A.; Treatment of hypertensive disease

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

Note: Withdrawal request received on 24 June 2021

2.3.2. Sacubitril/valsartan - EMEA-000316-PIP02-11-M05

Novartis Europharm Ltd.; Treatment of heart failure

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, 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/0344/2019 of 11 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Baricitinib - EMEA-001220-PIP03-16-M02

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0239/2019 of 16 July 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Dupilumab - EMEA-001501-PIP01-13-M07

Regeneron Pharmaceuticals, Inc; treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0374/2019 of 22/11/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Lebrikizumab - EMEA-002536-PIP01-18-M01

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0151/2020 of 17 April 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Ligelizumab - EMEA-001811-PIP02-15-M04

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 60 opinion

Dermatology

Summary of Committee discussion:

The PDCO considered Applicant's responses after Day 30 satisfactory.
The Committee therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0208/2020 of 16 June 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Tralokinumab - EMEA-001900-PIP02-17-M05

LEO Pharma A/S; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0440/2020 of 1 December 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Liraglutide - EMEA-000128-PIP02-09-M04

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, 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/0383/2019 of 04 December 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Ferric maltol - EMEA-001195-PIP01-11-M05

Norgine BV; Treatment of iron 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 and taking into account additional information received between Day 30 and Day 60 of the procedure, 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/0330/2019 of 11/09/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Giroctocogene fitelparvovec - Orphan - EMEA-002724-PIP01-19-M01

Pfizer Europe MA EEIG; Treatment of haemophilia A

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 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/0362/2020 of 9 September 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Rurioctocog alfa pegol - EMEA-001296-PIP01-12-M04

Baxalta Innovations GmbH; Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)

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 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/0001/2016 of 08 January 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Tocilizumab - EMEA-000309-PIP04-17-M03

Roche Registration GmbH; Treatment of cytokine release syndrome associated with chimeric antigen receptor (CAR) T cell therapy or T-cell-engaging bispecific antibody therapy

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

In June 2021 the PDCO noted the responses provided by the applicant that overall were considered satisfactory. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0253/2019 of 16/7/2019) for tocilizumab in treatment of cytokine release syndrome associated with chimeric antigen receptor (CAR) T cell therapy or T-cell-engaging bispecific antibody therapy. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Cenobamate - EMEA-002563-PIP02-19-M01

A.C.R.A.F. SpA; Treatment of epilepsy

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/0120/2020 of 18 March 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Ocrelizumab - EMEA-000310-PIP03-10-M05

Roche Registration GmbH; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO re-discussed at Day 60 during the June 2021 plenary meeting a modification for ocrelizumab for the treatment of multiple sclerosis.
The Committee took into consideration the information the applicant provided between Day 30 and Day 60. 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/0493/2020 of 22 December 2020. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Phenobarbital - EMEA-002532-PIP01-18-M01

Proveca Pharma Limited; Treatment of epilepsy

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/0422/2019 of 6 December 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Temelimab - EMEA-002127-PIP01-17-M01

GeNeuro SA; 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/0371/2017 of 11/12/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M03

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 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/0134/2016 of 23 May 2016. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. Cemiplimab - EMEA-002007-PIP02-17-M01

Regeneron Ireland DAC; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes with minor adjustment 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/0385/2017 of 19 December 2017.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M06

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasm

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60 during the June 2021 plenary meeting a modification for ibrutinib for the treatment of mature B-cell neoplasm.
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/0117/2021 of 17 March 2021. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Quizartinib - Orphan - EMEA-001821-PIP01-15-M05

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

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/0343/2020 of 9 September 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion

2.3.21. Sirolimus - Orphan - EMEA-001416-PIP01-12-M03

Santen Incorporated; Treatment of non-infectious uveitis

Day 60 opinion

Ophthalmology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0197/2018 of 19 July 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.22. In vitro expanded autologous human articular chondrocytes - EMEA-002217-PIP01-17-M02

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 60 opinion

Other

Summary of Committee discussion:

The PDCO re-discussed at Day 60 during the June 2021 plenary meeting a modification for autologous cartilage-derived cultured chondrocytes for the treatment of cartilage disorders. The Committee took into consideration the information the applicant provided between Day 30 and Day 60 and while accepting the administrative changes requested initially, it refused changes to the paediatric investigation plan, it refused the deferral and recommended to grant a product-specific waiver on its own motion on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.3.23. Selexipag - EMEA-000997-PIP01-10-M05

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

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/0123/2019 of 17/04/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.24. Xylitol / Procaine hydrochloride / Magnesium sulphate heptahydrate / Potassium chloride - EMEA-001171-PIP01-11-M02

MIT Gesundheit GmbH; Cardioplegia

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/0293/2016 of 4 November 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.25. Lactobacillus reuteri (IBP-9414) - Orphan - EMEA-001895-PIP01-15-M01

Infant Bacterial Therapeutics AB; Prevention of necrotising enterocolitis

Day 60 opinion

Other / Neonatology - Paediatric Intensive Care / 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/0330/2017 of 31 October 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion

2.3.26. Fasinumab - EMEA-002059-PIP02-19-M01

Regeneron Ireland D.A.C.; Treatment of chronic musculoskeletal pain / treatment of chronic non-musculoskeletal 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/0307/2020 of 12/08/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.27. Seltorexant - EMEA-002746-PIP01-20-M01

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 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/0079/2021 of 17 March 2021).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.28. Vortioxetine - EMEA-000455-PIP02-10-M08

H. Lundbeck A/S; Treatment of major depressive disorder

Day 60 opinion

Psychiatry

Summary of Committee discussion:

The PDCO's view expressed at Day 30 was re-discussed and endorsed.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0032/2021 of 27 January 2021).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.29. Finerenone - EMEA-001623-PIP01-14-M04

Bayer AG; Treatment of chronic kidney disease

Day 60 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0324/2019 of 10 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.30. Ad26.ZEBOV - EMEA-002307-PIP01-17-M02

Janssen Cilag International NV; Prevention of Ebola virus disease

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0155/2020 of 17/04/2020)
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.31. Hepatitis B (rDNA) surface antigen adjuvanted - EMEA-001127-PIP02-11-M01

Dynavax GmbH; Prevention of Hepatitis B virus infection

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0051/2012 of 2 March 2012).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.32. MVA-BN-Filo - EMEA-002308-PIP01-17-M02

Janssen Cilag International NV; Prevention of Ebola virus disease

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0138/2020 of 17/04/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.33. Neisseria meningitidis serogroup A polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup C polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup W polysaccharide conjugated to tetanus toxoid (MenACYW) - EMEA-001930-PIP01-16-M03

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the 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/0169/2019 issued on 11 October 2019.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.
Scientific discussion
The PDCO re-discussed this application in line with the outcome conclusion from D30, taking into account the additional information received.

2.3.34. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan including the discussion at D30, 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/0082/2020 issued on 18 March 2020).
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. Deucravacitinib - EMEA-C2-002350-PIP01-18-M01

Bristol-Myers Squibb International Corporation; Treatment of psoriasis

Day 30 letter

Dermatology

2.7.2. Obeticholic acid - EMEA-C1-001304-PIP03-17

Intercept Pharma International Limited; Treatment of non-alcoholic steatohepatitis

Day 30 letter

Gastroenterology-Hepatology

2.7.3. Niraparib (as tosylate monohydrate) - EMEA-C1-002268-PIP02-18-M01

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

Day 1 letter

Oncology

2.7.4. Efgartigimod alfa - EMEA-C1-002597-PIP01-19-M01

Argenx BV; Treatment of myasthenia gravis

Day 30 letter

Neurology

2.7.5. Tezepelumab - EMEA-C1-001613-PIP01-14-M04

AstraZeneca AB; Treatment of asthma

Day 60 letter

Pneumology – Allergology

Note: Adopted via written procedure on 14 June.

2.7.6. Liraglutide - EMEA-C3-000128-PIP02-09-M03

Novo Nordisk A/S; Treatment of obesity

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.7. Cipaglucosidase alfa - EMEA-C1-002447-PIP01-18-M01

Amicus Therapeutics Europe Limited; Treatment of glycogen storage disease Type II (Pompe's disease)

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.8. Doravirine / tenofovir disoproxil (fumarate) / lamivudine - EMEA-C2-001695-PIP01-14-M04

Merck Sharp & Dohme (Europe), Inc; Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 30 letter

Infectious Diseases

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-002870-PIP01-20

General anaesthesia

Day 90 discussion

Anaesthesiology

3.1.2. Benralizumab - EMEA-001214-PIP05-19

Treatment of Eosinophilic Esophagitis (EoE)

Day 90 discussion

Gastroenterology-Hepatology

3.1.3. (S)-1-(5-((2,3-dihydro-[1,4]dioxino[2,3-b]pyridin-7-yl)sulfonyl)-3,4,5,6-tetrahydropyrrolo[3,4-c]pyrrol-2(1H)-yl)-3-hydroxy-2-phenylpropan-1-one - Orphan - EMEA-002924-PIP01-20

Forma Therapeutics, Inc.; Treatment of sickle cell disease

Day 90 discussion

Haematology-Hemostaseology

3.1.4. Concizumab - Orphan - EMEA-002326-PIP04-20

Novo Nordisk A/S; Treatment of congenital haemophilia A / treatment of congenital haemophilia B

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Baricitinib - EMEA-001220-PIP08-20

Treatment of alopecia areata

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.6. Casirivimab - EMEA-002964-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19) / Prophylaxis of SARS-CoV-2 infection / Treatment and decreased transmission of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Infectious Diseases

3.1.7. Imdevimab - EMEA-002965-PIP01-21

Treatment and decreased transmission of coronavirus disease 2019 (COVID-19) / prophylaxis of SARS-CoV-2 infection / treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Infectious Diseases

3.1.8. Potassium Bitartrate / Citric Acid / L-Lactic Acid - EMEA-002917-PIP01-20

Prevention of urogenital Chlamydia trachomatis (CT) infection and Neisseria gonorrhoeae (GC) infections in females / prevention of urogenital Chlamydia trachomatis (CT) infection and Neisseria gonorrhoeae (GC) infection in females / prevention of urogenital Chlamydia trachomatis (CT) infection and Neisseria gonorrhoeae (GC) infection

Day 90 discussion

Infectious Diseases

3.1.9. Epcoritamab - EMEA-002907-PIP01-20

Treatment of mature B-cell lymphoma

Day 90 discussion

Oncology

3.1.10. Ligelizumab - EMEA-001811-PIP03-20

Treatment of food allergy

Day 90 discussion

Pneumology - Allergology

3.1.11. Single chain urokinase plasminogen activator (scuPA) - Orphan - EMEA-002896-PIP01-20

Lung Therapeutics, Inc.; Treatment of infectious pleural effusion

Day 90 discussion

Pneumology - Allergology

3.1.12. Sodium chloride solution 4.2% (w/v) / 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide - Orphan - EMEA-002935-PIP01-20

Parion Sciences, Inc.; Primary Ciliary Dyskinesia (PCD)

Day 90 discussion

Pneumology - Allergology

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

Prevention of disease caused by *Streptococcus pneumoniae*

Day 90 discussion

Vaccines

3.1.14. Bentracimab - EMEA-002766-PIP02-21

Treatment of ticagrelor associated haemorrhage / prevention of ticagrelor associated haemorrhage

Day 60 discussion

Cardiovascular Diseases

3.1.15. Brepocitinib - EMEA-003011-PIP01-21

Treatment of atopic dermatitis

Day 60 discussion

Dermatology

3.1.16. Ulobetasol propionate - EMEA-003000-PIP01-21

Treatment of psoriasis

Day 60 discussion

Dermatology

3.1.17. EMEA-002992-PIP01-21

Treatment of Fibrodysplasia Ossificans Progressiva (FOP)

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. Ethinyl estradiol / Dienogest - EMEA-002229-PIP02-21

Treatment of polycystic ovary syndrome

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. Semaglutide - EMEA-001441-PIP06-21

Treatment of obesity

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 3 June 2021

3.1.20. Recombinant adeno-associated viral (rAAV) vector expressing the human ornithine transcarbamylase (hOTC) gene - EMEA-002983-PIP01-21

Ornithine Transcarbamylase Deficiency

Day 60 discussion

Gastroenterology-Hepatology

3.1.21. Sirolimus - EMEA-002982-PIP01-21

Ornithine Transcarbamylase Deficiency (OTCD)

Day 60 discussion

Gastroenterology-Hepatology

3.1.22. 6-[(3S,4S)-4-methyl-1-(pyrimidin-2-ylmethyl)pyrrolidin-3-yl]-3-tetrahydropyran-4-yl-7H-imidazo[1,5-a]pyrazin-8-one - Orphan - EMEA-002991-PIP01-21

IMARA Inc; Treatment of sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

3.1.23. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP03-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of severe sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

3.1.24. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP04-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of beta-thalassemia intermedia and major

Day 60 discussion

Haematology-Hemostaseology

3.1.25. Efgartigimod alfa - Orphan - EMEA-002597-PIP04-21

argenx BV; Treatment of Immune Thrombocytopenia

Day 60 discussion

Haematology-Hemostaseology

3.1.26. Apremilast - EMEA-000715-PIP06-21

Treatment of coronavirus disease (COVID-19)

Day 60 discussion

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 17 June 2021

3.1.27. Leniolisib phosphate - Orphan - EMEA-002989-PIP01-21

Pharming Group N.V.; Activated phosphoinositide 3-kinase δ syndrome (APDS)

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.28. Favipiravir - EMEA-002985-PIP01-21

Treatment of coronavirus disease (COVID-19)

Day 60 discussion

Infectious Diseases

3.1.29. Givinostat - Orphan - EMEA-000551-PIP04-21

Italfarmaco S.p.A.; Treatment of Duchenne muscular dystrophy

Day 60 discussion

Neurology

3.1.30. EMEA-002993-PIP01-21

Treatment of narcolepsy

Day 60 discussion

Neurology

3.1.31. EMEA-002635-PIP02-21

Treatment of advanced or metastatic malignancies harbouring ALK, ROS1, or NTRK1-3 alterations

Day 60 discussion

Oncology

3.1.32. Alectinib - EMEA-002431-PIP02-21

Treatment of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 discussion

Oncology

3.1.33. Autologous T cells transduced with lentiviral vector containing a tandem chimeric antigen receptor directed against CD20 and CD19 - Orphan - EMEA-003009-PIP01-21

Miltenyi Biomedicine GmbH; Mature aggressive B cell non-Hodgkin lymphoma / Diffuse large B cell lymphoma

Day 60 discussion

Oncology

3.1.34. Autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LV, encoding for the human α-L-iduronidase (IDUA) cDNA - Orphan - EMEA-003001-PIP01-21

Orchard Therapeutics Netherlands B.V.; Treatment of Mucopolysaccharidosis type I, Hurler syndrome (MPS-IH)

Day 60 discussion

Other

3.1.35. EMEA-002990-PIP01-21

Major depressive disorder

Day 60 discussion

Psychiatry

Note: Withdrawal request received on 24 June 2021

3.1.36. Ralmitaront - EMEA-003003-PIP01-21

Treatment of schizophrenia

Day 60 discussion

Psychiatry

3.1.37. EMEA-003002-PIP01-21

Treatment of proteinuric chronic kidney disease

Day 60 discussion

Uro-nephrology

3.1.38. Escherichia coli vaccine - EMEA-002996-PIP01-21

Prevention of *E.coli* infections

Day 60 discussion

Vaccines

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

Prevention of dengue disease

Day 60 discussion

Vaccines

3.1.40. Recombinant SARS-CoV-2 spike (S)-protein virus-like particle - EMEA-003008-PIP01-21

Prevention of Coronavirus disease 2019 (COVID-19)

Day 60 discussion

Vaccines

3.1.41. Abelacimab - EMEA-003017-PIP01-21

Prevention of venous thromboembolism associated with cancer

Day 30 discussion

Cardiovascular Diseases

3.1.42. Bisoprolol / amlodipine / indapamide / perindopril - EMEA-003015-PIP01-21

Treatment of (essential) primary hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.43. Dersimelagon - EMEA-002850-PIP02-21

Treatment of X-linked protoporphyria / Treatment of erythropoietic protoporphyria

Day 30 discussion

Dermatology

3.1.44. EMEA-003027-PIP01-21

Treatment of hyperphenylalaninaemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.45. Prasterone / levonorgestrel / ethinylestradiol - EMEA-002960-PIP02-21

Treatment of hypoactive sexual desire disorder (HSDD) secondary to COC use in women requiring contraception

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.46. EMEA-003019-PIP01-21

Treatment of inborn errors of amino acid metabolism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.47. Apraglutide - Orphan - EMEA-003016-PIP01-21

VectivBio AG; Treatment of short bowel syndrome

Day 30 discussion

Gastroenterology-Hepatology

3.1.48. Benralizumab - EMEA-001214-PIP07-21

Treatment of Eosinophilic Gastritis/Eosinophilic Gastroenteritis

Day 30 discussion

Gastroenterology-Hepatology

3.1.49. Izencitinib - EMEA-002757-PIP02-21

Treatment of Crohn's disease

Day 30 discussion

Gastroenterology-Hepatology

3.1.50. Autologous CD34+ cells transduced ex vivo with a lentiviral vector containing a modified gamma-globin gene - Orphan - EMEA-003029-PIP01-21

Aruvant Sciences GmbH; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.1.51. Mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus - EMEA-002694-PIP02-21

Treatment of chronic hepatitis D infection

Day 30 discussion

Infectious Diseases

3.1.52. Azithromycin - EMEA-003021-PIP01-21

Prevention of bronchopulmonary dysplasia

Day 30 discussion

Neonatology - Paediatric Intensive Care

3.1.53. Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase - Orphan - EMEA-003020-PIP01-21

Lysogene; Treatment of GM1 gangliosidosis

Day 30 discussion

Neurology

3.1.54. Efgartigimod alfa - Orphan - EMEA-002597-PIP05-21

argenx BV; Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.1.55. 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting fused in sarcoma (FUS) pre-mRNA - EMEA-003024-PIP01-21

Amyotrophic lateral sclerosis (ALS) patients with fused in sarcoma (FUS) mutations (FUS-ALS) >=12 years of age

Day 30 discussion

Neurology

3.1.56. Recombinant fusion protein linking human frataxin to TAT cell-penetrant peptide. - Orphan - EMEA-003022-PIP01-21

Larimar Therapeutics Inc.; Friedreich's ataxia

Day 30 discussion

Neurology

3.1.57. Atezolizumab - EMEA-001638-PIP02-21

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

Day 30 discussion

Oncology

3.1.58. Lorlatinib - EMEA-002669-PIP03-21

ALK-aberrant neuroblastoma

Day 30 discussion

Oncology

3.1.59. Ociperlimab - EMEA-003028-PIP01-21

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

Day 30 discussion

Oncology

3.1.60. Prednisolone - EMEA-003004-PIP01-21

Treatment of prostate cancer

Day 30 discussion

Oncology

3.1.61. Selinexor - EMEA-002387-PIP02-21

Recurrent or advanced endometrial cancer

Day 30 discussion

Oncology

3.1.62. Vodobatinib - EMEA-003014-PIP01-21

Chronic Myeloid Leukemia

Day 30 discussion

Oncology

3.1.63. Vorasidenib - EMEA-002932-PIP02-21

Treatment of glioma

Day 30 discussion

Oncology

3.1.64. Recombinant humanized anti-blood dendritic cell antigen 2 (BDCA2) monoclonal antibody - EMEA-002555-PIP02-21

Lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.65. Ligelizumab - EMEA-001811-PIP04-21

Treatment of chronic inducible urticaria

Day 30 discussion

Dermatology

3.1.66. Florbetaben (18F) - Orphan - EMEA-001090-PIP02-21

Life Molecular Imaging GmbH; Diagnosis of cardiac amyloidosis

Day 30 discussion

3.1.67. A recombinant SARS-CoV-2 Spike (S)-trimer fusion protein - EMEA-002987-PIP01-21

Prevention of COVID-19 disease

Day 30 discussion

Vaccines / Infectious Diseases

3.1.68. Pemigatinib - Orphan - EMEA-002370-PIP02-21

Incyte Biosciences Distribution B.V.; Treatment of myeloid/lymphoid neoplasms with eosinophilia and FGFR1 rearrangement

Day 30 discussion

Oncology

3.1.69. Cendakimab - EMEA-002640-PIP01-19

Eosinophilic sophagitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.70. Ribitol - Orphan - EMEA-002887-PIP01-20

Premier Research Group S.L.; Treatment of Limb-Girdle Muscular Dystrophy

Day 90 discussion

Other

3.1.71. Molnupiravir - EMEA-002940-PIP01-20

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

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. Octocog alfa - EMEA-C-001064-PIP01-10-M03

Bayer AG; Treatment of hereditary Factor VIII deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Entrectinib - EMEA-002096-PIP01-16-M03

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

3.3.2. Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M01

Mundipharma Corporation (Ireland) Limited; Treatment of invasive candidiasis

Day 30 discussion

3.3.3. Brodalumab - EMEA-001089-PIP02-13-M02

LEO Pharma A/S; Treatment of psoriasis

Day 30 discussion

Dermatology

3.3.4. Rubidium Rb-82 Chloride - EMEA-000882-PIP03-11-M05

Jubilant DraxImage Inc., dba Jubilant Radiopharma; Visualisation of myocardial perfusion for diagnostic purposes

Day 30 discussion

Diagnostic

3.3.5. Avalglucosidase alfa - Orphan - EMEA-001945-PIP01-16-M03

Genzyme Europe B.V.; CD-10: E74.0 / Glycogen storage disease (Pompe disease)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Metreleptin - Orphan - EMEA-001701-PIP01-14-M02

Amryt Pharmaceuticals DAC; Treatment of lipodystrophy

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Recombinant parathyroid hormone: rhPTH (1-84) - Orphan - EMEA-001526-PIP01-13-M05

Shire Pharmaceuticals Ireland Limited; Hypoparathyroidism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.8. Tolvaptan - EMEA-001231-PIP02-13-M08

Otsuka Pharmaceutical Netherlands B.V.; Polycystic Kidney Disease (PKD)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

3.3.9. Dupilumab - EMEA-001501-PIP04-19-M01

Regeneron Ireland DAC; Eosinophilic Esophagitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.10. Naldemedine - EMEA-001893-PIP01-15-M02

Shionogi B.V.; Opioid-induced Constipation (OIC)

Day 30 discussion

Gastroenterology-Hepatology

3.3.11. Pegylated-fibroblast growth factor 21 (BMS-986036) - EMEA-002448-PIP01-18-M02

Bristol-Myers Squibb International Corporation; Treatment of non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Gastroenterology-Hepatology

3.3.12. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19-M01

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

Day 30 discussion

Haematology-Hemostaseology

3.3.13. Narsoplimab - Orphan - EMEA-002479-PIP01-18-M01

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

Day 30 discussion

Haematology-Hemostaseology

3.3.14. Aztreonam / Avibactam - EMEA-002283-PIP01-17-M02

Pfizer Europe MA EEIG; Treatment of infections caused by aerobic gram-negative bacteria

Day 30 discussion

Infectious Diseases

3.3.15. Cabotegravir - EMEA-001418-PIP01-13-M03

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

Day 30 discussion

Infectious Diseases

3.3.16. Cabotegravir - EMEA-001418-PIP02-15-M02

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

Day 30 discussion

Infectious Diseases

3.3.17. Remdesivir - EMEA-002826-PIP01-20-M02

Gilead Sciences International Ltd.; Coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.18. Bumetanide - EMEA-001303-PIP01-12-M04

Les Laboratoires Servier; Autism Spectrum Disorder

Day 30 discussion

Neurology

3.3.19. Lacosamide - EMEA-000402-PIP03-17-M05

UCB Pharma S.A.; Treatment of Generalized Epilepsy and Epilepsy Syndromes.

Day 30 discussion

Neurology

3.3.20. Autologous tumor-infiltrating lymphocytes (LN-144/LN-145) - EMEA-002776-PIP01-20-M01

Iovance Biotherapeutics, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Oncology

3.3.21. Bintrafusp alfa - Orphan - EMEA-002586-PIP01-19-M02

Merck Europe B.V.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Oncology

3.3.22. Bosutinib - EMEA-000727-PIP01-09-M05

Pfizer Europe MA EEIG; treatment of chronic myeloid leukaemia

Day 30 discussion

Oncology

3.3.23. Selumetinib - Orphan - EMEA-001585-PIP01-13-M05

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

Day 30 discussion

Oncology

3.3.24. Bupropion HCl / Naltrexone HCl - EMEA-001373-PIP01-12-M04

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 30 discussion

Other

3.3.25. Eliglustat - Orphan - EMEA-000461-PIP02-11-M04

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

Day 30 discussion

Other

3.3.26. Human Thrombin / Human Fibrinogen - EMEA-001149-PIP01-11-M07

Omrix Biopharmaceuticals N.V.; Treatment of cerebrospinal fluid leakage resulting from a surgical procedure. / Treatment of haemorrhage resulting from a surgical procedure.

Day 30 discussion

Other

3.3.27. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16-M03

Lupin Europe GmbH; Treatment of myotonic disorders

Day 30 discussion

Other

3.3.28. Patiromer calcium - EMEA-001720-PIP01-14-M02

Vifor Fresenius Medical Care Renal Pharma France; treatment of hyperkalaemia

Day 30 discussion

Other

3.3.29. Bupivacaine - EMEA-000877-PIP03-17-M03

Pacira Ltd; postsurgical analgesia

Day 30 discussion

Pain

3.3.30. Amikacin sulfate - Orphan - EMEA-000525-PIP01-08-M07

Insmed Netherlands B.V.; Treatment of nontuberculous mycobacterial (NTM) lung infection / treatment of Pseudomonas aeruginosa lung infection/colonisation in cystic fibrosis patients

Day 30 discussion

Pneumology - Allergology

3.3.31. Berotralstat - EMEA-002449-PIP02-18-M01

BioCryst Ireland Limited; Treatment of Hereditary Angioedema

Day 30 discussion

Pneumology - Allergology

3.3.32. Mometasone (furoate) / Glycopyrronium bromide / Indacaterol - EMEA-001812-PIP01-15-M01

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3.33. Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Yamagata lineage) / Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Victoria lineage) / Split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain - EMEA-002359-PIP01-18-M03

Sanofi Pasteur; Prevention of influenza infection

Day 30 discussion

Vaccines

3.3.34. Recombinant Clostridioides difficile Toxoids A and B - EMEA-002112-PIP01-16-M01

Pfizer Europe MA EEIG; Prevention of Clostridioides difficile infection (CDI)

Day 30 discussion

Vaccines

3.3.35. (R)-2-amino-3-phenylpropylcarbamate hydrochloride - EMEA-002184-PIP01-17-M01

Jazz Pharmaceuticals Ireland Limited; Treatment of narcolepsy / Treatment of obstructive sleep apnoea

Day 30 discussion

Neurology

3.3.36. Dasiglucagon - Orphan - EMEA-002233-PIP01-17-M01

Zealand Pharma A/S; Treatment of hypoglycaemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.37. Pevonedistat - Orphan - EMEA-002117-PIP01-17-M02

Takeda Pharma A/S; Treatment of myelodysplastic syndromes / Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology

3.3.38. Pneumococcal polyssacharide serotype 33F conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 23F conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 22F conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 19F conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 19A conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 18C conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 15B conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 14 conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 12F conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 11A conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 10A conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 9V conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 8 conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 7F conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 6B conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 6A conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 5 conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 4 conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 3 conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 1 conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate - EMEA-002330-PIP01-18-M01

Pfizer Europe MA EEIG; Disease caused by Streptococcus pneumoniae

Day 30 discussion

Vaccines

3.3.39. Pneumococcal Polysaccharide Serotype 33F - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 23F - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 22F - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 19F - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 19A - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 18C - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 14 - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 9V - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 7F - Diphtheria CRM197 Conjugate /

Pneumococcal Polysaccharide Serotype 6B - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 6A - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 5 - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 4 - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 3 - Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 1 - Diphtheria CRM197 Conjugate - EMEA-002215-PIP01-17-M03

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

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 letters of intent received for submission of applications with start of procedure 12 July 2021 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:

No item

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. Pegcetacoplan - EMEA-08-2021

Apellis Ireland Limited; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of geographic atrophy secondary to age related macular degeneration

Summary of Committee discussion:

The applicability of the class waiver as referred to in the 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. Darolutamide - EMEA-02-2021

Bayer AG; The classes of androgen receptor modulator for treatment prostate malignant neoplasms / Treatment of adult men with metastatic hormone-sensitive prostate cancer (mHSPC)

Summary of Committee discussion:

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

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

6.1.3. Trastuzumab deruxtecan - EMEA-03-2021

Daiichi Sankyo Europe GmbH; The class of HER / Epidermal growth factor-receptor antibody medicinal products for treatment intestinal malignant neoplasms / Treatment of adult patients with human epidermal growth factor receptor (HER)2-positive gastric cancer

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: HER2 is essential for normal embryonic development and has a critical function in oncogenesis. Increased expression was documented in Wilms tumours, neuroblastoma and osteosarcoma, but mostly without gene amplification. The PDCO encourages the applicant to conduct preclinical studies assessing paediatric tumours as well.

6.1.4. humanized monoclonal anti-tau antibody - EMEA-05-2021

Janssen-Cilag International NV; All classes of medicinal products for treatment of Alzheimer's disease / Treatment of Alzheimer's disease

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.

6.1.5. Abiraterone - EMEA-09-2021

ALFRED E. TIEFENBACHER (GmbH & Co. KG); The classes of androgen 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 prostate malignant neoplasms / Treatment of newly diagnosed high risk metastatic hormone sensitive prostate cancer (mHSPC) in adult men in combination with androgen deprivation therapy / Treatment of metastatic castration resistant prostate cancer (mCRPC) in adult men who are asymptomatic or mildly symptomatic after failure of androgen deprivation therapy in whom chemotherapy is not yet clinically indicated / Treatment of mCRPC in adult men whose disease has progressed on or after a docetaxel-based chemotherapy regimen

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

No item

8. Annual reports on deferrals

No item

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. Update on PDCO member(s)/alternate(s) mandate status

The PDCO Committee noted the re-nomination of Yuansheng Sun as the alternate of Germany.

9.1.2. Revision of the Summary Report template (B-F scientific document)

Summary of Committee discussion:

The revision of the B-F scientific document was presented to the PDCO for comments requested by 12 July 2021. Follow-up discussion will be scheduled at the next PDCO meeting.

9.1.3. Deadlines for paediatric applications 2020-2024

Summary of Committee discussion:

The deadlines for paediatric applications 2020-2024 were presented and adopted by the PDCO.

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

No item

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

The chair of the Non-clinical Working Group (NcWG) identified the products which will require NcWG 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.

9.3.3. Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) on 1-2 June 2021

The agenda for the Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) on 1-2 June 2021 was presented for information.

9.3.4. EMA Working Party review

Summary of Committee discussion:

An update on the EMA working party review was presented to the committee.

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. Exchange between Clinical Trials Facilitation and Coordination Group (CTFG) and PDCO

PDCO Member: Anette Solli Karlsen

Summary of Committee discussion:

Collaboration aspects between CTFG and PDCO were discussed, including continuing the information exchange, specifically on an ad hoc basis on COVID-19 vaccines procedures, and planning interactions for the next year.

9.4.3. PDCO / Health technology assessment (HTA) interaction – update on current initiatives

Summary of Committee discussion:

The committee was informed about initiatives with HTA bodies with relevance to paediatrics.

9.5. Cooperation with International Regulators

No item

9.5.1. Feedback from 6th Paediatric Oncology Strategy Forum on CAR T cell development

Summary of Committee discussion:

The PDCO was informed about the conclusion from CAR T cell Paediatric Oncology Strategy Forum.

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 Committee was updated on COVID-19 vaccines and therapeutics. Main focus of this update was the myocarditis events in young people with mRNA COVID vaccines.

10.2. EU innovation network Horizon Scanning report on Genome Editing

Summary of Committee discussion:

The Committee was made aware of the short report and of implementation activities started by the EU IN.

10.3. EMA Business Pipeline activity and Horizon scanning

Q2/2021 Update of the Business Pipeline report for the human scientific committees

Summary of Committee discussion:

The Q-21 report was presented for information. The report covers the list of non-COVID-19 related MAAs expected for the remainder of 2021, highlighting products with no appointed rapporteur.

11. Breakout sessions

11.1. Internal PDCO Operations

Summary of Committee discussion:

Members discussed topics to optimise PDCO internal operations.

11.2. Vaccines

Summary of Committee discussion:

The break out session was focused on the future needs and challenges of paediatric COVID vaccines, including the expected duration of protection, how to deal with the development of variant vaccines from the point of view of paediatrics, and when it is expected that comparison with authorized COVID vaccines may be needed/appropriate in the paediatric studies of new vaccines.

11.3. Paediatric oncology

Summary of Committee discussion:

The breakout session was cancelled as no topic of relevance was identified.

11.4. Neonatology

Summary of Committee discussion:

Discussion on the role of modelling and simulation for development of medicines for neonates.

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 22-25 June 2021 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	When chairing the meeting: To be replaced for discussions, final deliberations and voting When not chairing the meeting: No participation in final deliberations and voting	2.7.3. Niraparib (as tosylate monohydrate) - EMEA-C1-002268-PIP02-18-M01
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in final deliberations and voting on: No participation in discussion, final deliberations and voting on:	2.3.19. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M06
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Tereza Bazantova	Alternate	Czechia	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Pauliina Lehtolainen-Dalkilic	Member	Finland	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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Mountaki	Alternate	Greece	No interests declared	
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No participation in discussion, final deliberations and voting on:	2.3.34. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03
Dovile Zacharkiene	Member	Lithuania	No interests declared	
Silvijus Abramaviciu s	Alternate	Lithuania	No restrictions applicable to this meeting	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Eva Agurell	Member	Sweden	No interests declared	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	2.3.34. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	2.3.34. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03
Jaroslav Sterba	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Alternate	Patients' Organisation Representative	No interests declared	
María Estela Moreno Martín	Expert - via telephone*	Spain	No interests declared	
Lutz Wiesner	Expert - via telephone*	Germany	No interests declared	
Charlotta Bergquist	Expert - via telephone*	Sweden - MPA	No interests declared	
Tomas Radimersky	CHMP alternate - via telephone*	Czechia - SUKL	No interests declared	
Gaby Wangorsch	Expert - via telephone*	Germany - PEI	No interests declared	
A representative from the European Commission attended the meeting Meeting run with support from relevant EMA staff				
* Experts were only 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/