4 September 2020
EMA/PDCO/466199/2020
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 1-4 September 2020

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.

Further information with relevant explanatory notes can be found at the end of this document.

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
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1. Introductions

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

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 was replaced by the Vice-Chair Sabine Scherer for the discussion on agenda topic 2.3.7, 2.3.9, 3.1.32, 3.1.34.

1.2. Adoption of agenda

PDCO agenda for 1-4 September 2020

The agenda of the PDCO meeting 1-4 September 2020 was adopted.

1.3. Adoption of the minutes

PDCO minutes for extraordinary PDCO virtual meeting 9 July 2020 on tocilizumab

PDCO minutes for 21-24 July 2020

The minutes of the PDCO extraordinary virtual meeting of 9 July and 21-24 July 2020 were adopted and will be published on the EMA website.

2. Opinions

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

2.1. Opinions on Products

2.1.1. Glycopyrronium bromide - EMEA-002383-PIP01-18

Dr. August Wolff GmbH & Co. KG - Arzneimittel; Hyperhidrosis / Treatment of primary axillary hyperhidrosis

Day 120 opinion

Dermatology

Summary of committee discussion:

The PDCO discussed the clarification received from the applicant on the outstanding issues. In conclusion, the PDCO adopted a positive opinion for glycopyrronium bromide for treatment of hyperhidrosis with a deferral and a waiver for a subset of children on the ground of lack of significant therapeutic benefit.

2.1.2. Danicopan - Orphan - EMEA-002310-PIP01-17

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the information provided by the applicant on their intent to pursue the restricted indication of "Treatment of paroxysmal nocturnal haemoglobinuria as add-on therapy to a C5 Inhibitor in patients with signs or symptoms of extravascular haemolysis" which is aligned with the current development. This is supported and in line with the PRIME designation, discussed by the EMA earlier this year.
The PDCO adopted a positive opinion, including a deferral and a waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2.1.3. Plasma kallikrein inhibitor - EMEA-002723-PIP01-19

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The Committee adopted a positive opinion, including a deferral for the clinical development in a subset of children and a waiver for the paediatric population in a subset of children on the grounds of lack of significant therapeutic benefit.

2.1.4. Doravirine / islatravir - EMEA-002707-PIP01-19

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

infection / Doravirine/islatravir is indicated alone or in combination with other antiretroviral medicinal products for the treatment of HIV-1 infection in paediatric patients from 28 days to less than 18 years of age.

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

Whilst the majority of points were resolved the PDCO did not agree on a few remaining issues. In conclusion the PDCO agreed a PIP with a waiver for a subset of the paediatric population and a deferral for the FDC doravirine / islatravir as single regimen for the treatment of HIV-1.

2.1.5. Mixture of 2 synthetic double-stranded N-acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against the hepatitis B virus - EMEA-002694-PIP01-19

Janssen-Cilag International NV; Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

A positive opinion on a paediatric investigation plan for a subset of with a deferral in the treatment of chronic viral hepatitis B was adopted. A waiver for a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients was also adopted.

2.1.6. N-(3-cyano-4-fluorophenyl)-1-methyl-4-[[(2S)-1,1,1-trifluoro-2-propanyl]sulfamoyl]-1H-pyrrole-2-carboxamide - EMEA-002693-PIP01-19

Janssen-Cilag International NV; Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

A positive opinion on a paediatric investigation plan for a subset of children with a deferral in the treatment of chronic viral hepatitis B was adopted. A waiver for a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients was also adopted.

2.1.7. The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood (Hau-UCB-mnc) - Orphan - EMEA-001799-PIP03-19

BrainRepair UG (haftungsbeschränkt); Periventricular leukomalacia (PVL)

Day 120 opinion

Neonatology - Paediatric Intensive Care

Summary of committee discussion:

The PDCO adopted a positive opinion for a PIP for "The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood (Hau-UCB-mnc)" for the treatment of preterm neonates from birth to less than 37+0 weeks gestational age (GA) for the treatment of periventricular leukomalacia (PVL). A waiver was granted for the remaining paediatric population 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). A deferral was not applied for and not granted.

2.1.8. RAAVrh74.MHCK7.microdystrophin (SRP-9001) - Orphan - EMEA-002677-PIP01-19

Sarepta Therapeutics Ireland; Duchenne Muscular Dystrophy

Day 120 opinion

Neurology

Summary of committee discussion:

The PDCO agreed a PIP for rAAVrh74.MHCK7.microdystrophin in the condition of Duchenne Muscular Dystrophy with a deferral.

2.1.9. Recombinant human granulocyte colony-stimulating factor – human immunoglobulin Fc fusion protein (rhG-CSF-Fc) - EMEA-002507-PIP02-19

Generon (Shanghai) Corporation; Treatment of chemotherapy-induced neutropenia and prevention of chemotherapy-induced febrile neutropenia / Reduction in the duration of neutropenia and the incidence of febrile neutropenia in patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO discussed the application for recombinant human granulocyte colony-stimulating factor – human immunoglobulin Fc fusion protein (rhG-CSF-Fc, proposed INN: efbemalenograstim alfa) taking into account the clarifications provided by the applicant after D90 and the applicant's comments on the draft opinion.
All pending issues identified at Day 90 were therefore considered solved.
In conclusion, the PDCO recommended granting a paediatric investigation plan for recombinant human granulocyte colony-stimulating factor – human immunoglobulin Fc fusion protein (rhG-CSF-Fc) for the entire paediatric population (from birth to less than 18 years of age) and a deferral for treatment of chemotherapy-induced neutropenia and prevention of chemotherapy-induced febrile neutropenia.

2.1.10. (2S,4S)-2-(4-Carboxyphenyl)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-1-ium chloride—water (1/1) - Orphan - EMEA-002705-PIP01-19

Novartis Europharm Limited; C3 Glomerulopathy

Day 120 opinion

Other

Summary of committee discussion:

Based on the assessment of this application and the discussions at the Paediatric Committee, the PDCO adopted a positive opinion with a waiver for conducting studies in a subset of the paediatric population and a deferral for the paediatric study in a subset of children in the condition of "treatment of C3 glomerulopathy".

2.1.11. (2S,4S)-2-(4-Carboxyphenyl)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-1-ium chloride—water (1/1)- - EMEA-002705-PIP02-19

Novartis Europharm Limited; IgA Nephropathy

Day 120 opinion

Other

Summary of committee discussion:

The PDCO endorsed its views expressed on day 90.
Based on the assessment of this application and the discussions at the Paediatric Committee, the PDCO adopted a positive opinion with a waiver for conducting studies in a subset of children and a deferral for the paediatric study in a subset of children in the condition of "treatment of IgA nephropathy".

2.1.12. Colchicine - EMEA-002837-PIP01-20

Disphar International B.V.; Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The applicant agreed to changing the condition to 'prevention of cardiovascular events'.
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 colchicine for all subsets of the paediatric population (0 to 18 years of age) in the condition of prevention of cardiovascular events on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as studies are not feasible.
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.

2.1.13. Fenofibrate / Ezetimibe / Pravastatin sodium - EMEA-002835-PIP01-20

Laboratoires SMB S.A.; Treatment of mixed hyperlipidaemia

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application, the PDCO agrees with the applicant's request for a waiver. A waiver for fenofibrate / pravastatin sodium / ezetimibe was granted for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mixed hyperlipidaemia.

2.1.14. Dupilumab - EMEA-001501-PIP06-20

sanofi-aventis recherche & développement; Prurigo Nodularis

Day 60 opinion

Dermatology

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 dupilumab for all subsets of the paediatric population (from birth to less than18 years of age) in the condition of "treatment of prurigo nodularis on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.
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.

2.1.15. Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains (CT053) - EMEA-002823-PIP01-20

CARsgen Therapeutics Corporation; Treatment of multiple myeloma / Treatment of patients with relapsed and/or refractory multiple myeloma (RRMM) who have received at least one proteasome inhibitor (PI), one immunomodulatory agent (IMiD), and one anti-CD38 monoclonal antibody (mAb). The patients shall have received at least 3 prior lines of therapy for MM.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO confirmed all conclusions reached at Day 30 and agreed to grant a product specific waiver for autologous T lymphocyte-enriched population of cells transduced with a lentiviral

vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains (CT053) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of multiple myeloma on the grounds that the disease does not occur in the paediatric population.

2.1.16. Sacituzumab govitecan - EMEA-002645-PIP02-20

Immunomedics GmbH; Treatment of urothelial carcinoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO confirmed all conclusions reached at Day 30 and agreed to grant a product specific waiver for sacituzumab govitecan for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of urothelial carcinoma on the grounds that urothelial carcinoma does not occur in the paediatric population.

2.1.17. Tiragolumab - EMEA-002721-PIP02-20

Roche Registration GmbH; Treatment of cervical cancer

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO confirmed all conclusions reached at Day 30 and agreed to grant a product specific waiver for tiragolumab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of cervical cancer on the grounds that the disease does not occur in the paediatric population.

2.1.18. Tiragolumab - EMEA-002721-PIP03-20

Roche Registration GmbH; Treatment of oesophageal carcinoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO confirmed all conclusions reached at Day 30 and agreed to grant a product specific waiver for tiragolumab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of oesophageal carcinoma on the grounds that the disease does not occur in the paediatric population.

2.1.19. Alpha-R-lipoic acid choline ester tosilate - EMEA-002811-PIP01-20

Novartis Europharm Limited; Treatment of presbyopia

Day 60 opinion

Ophthalmology

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 alpha-R-lipoic acid choline ester tosilate for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of presbyopia on the grounds that the disease only occurs in the adult population.
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.

2.1.20. Brolucizumab - EMEA-002691-PIP02-20

Novartis Europharm Limited; Diabetic retinopathy

Day 60 opinion

Ophthalmology

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

2.1.21. Dabrafenib - EMEA-001147-PIP02-20

Treatment of glioma / Treatment of paediatric patients with glioma with a BRAF V600 mutation in combination with trametinib

Day 60 Opinion

Oncology

The PDCO's view expressed at D30 was re-discussed and endorsed.
In conclusion, the PDCO adopted a positive opinion at D60 for dabrafenib for treatment of glioma with a waiver for a subset of children on the ground that the specific medicinal product is likely to be unsafe in the specified paediatric subsets.

2.1.22. Trametinib - EMEA-001177-PIP02-20

Treatment of glioma / Treatment of paediatric patients with glioma with a BRAF V600 mutation

in combination with dabrafenib

Day 60 Opinion

Oncology

The PDCO's view expressed at D30 was re-discussed and endorsed.
In conclusion, the PDCO adopted a positive opinion at D60 for trametinib for treatment of glioma with a waiver for a subset of children on the ground that the condition for which the specific medicinal product is intended does not occur in the specified paediatric subset.

2.1.23. Recombinant humanised monoclonal antibody (IgG1, Kappa) to IL-5 - EMEA-002836-PIP01-20

Treatment of Asthma / Add-on treatment for patients (adolescents aged 12 years and older) with severe asthma and an eosinophilic phenotype

Day 60 discussion

Pneumology - Allergology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agreed on a waiver on its own motion, based on lack of significant therapeutic benefit. The PDCO recommends granting a waiver for Recombinant humanised monoclonal antibody (IgG1, Kappa) to IL-5 for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of Asthma.

2.2. Opinions on Compliance Check

The following compliance checks have been put up for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance.

2.2.1. Evinacumab - EMEA-C1-002298-PIP01-17-M01

Regeneron Ireland DAC; Treatment of elevated cholesterol

Day 60 letter

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

Summary of committee discussion:

The PDCO discussed at their September 2020 meeting the responses provided by the applicant and input from Non-Clinical Working Group experts concluded that the completed studies 1, 2, 3, and 4 as well as initiation of Study 5 and 7 and considered that were compliant with the latest Agency's Decision P/0105/2020 of 18/3/2020.
The PDCO finalised this partially completed compliance procedure on 4/9/2020.

2.2.2. Elivaldogene autotemcel - EMEA-C-001244-PIP01-11-M02

bluebird bio (Netherlands) B.V.; Treatment of adrenoleukodystrophy

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO's view expressed at D30 were endorsed.
The PDCO adopted on 4 September 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0290/2018 of 12 September 2018).

2.2.3. Posaconazole - EMEA-C2-000468-PIP02-12-M06

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections

Day 30 letter

Infectious Diseases

Summary of committee discussion:

The PDCO discussed the compliance check request.
The PDCO discussed the completed studies 1,2,5,8 and 9, and considered that these are compliant with the latest Agency's Decision P/0101/2020 of 18 March 2020.
The PDCO finalised this partially completed compliance procedure on 4 September 2020.

2.2.4. Arimoclomol citrate - EMEA-C1-001748-PIP01-15

Orphazyme A/S; Treatment of Niemann-Pick disease, type C

Day 30 letter

Neurology

Summary of committee discussion:

The PDCO discussed the compliance check request.
The PDCO discussed the completed studies 1-8 and the initiation of study 9 and considered that these are compliant with the latest Agency's Decision P/0213/2018 of 17 July 2018.
The PDCO finalised this partially completed compliance procedure on 4 September 2020.

2.2.5. Cobimetinib - EMEA-C3-001425-PIP01-13-M04

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 30 letter

Oncology

Summary of committee discussion:

The PDCO discussed the completed studies Study 1 and Study 4 and considered that these are compliant with the latest Agency's Decision (P/0306/2019) of 10 September 2019.
The PDCO finalised this partially completed compliance procedure on 4 September 2020.

2.2.6. Ad26.ZEBOV (recombinant, replication-incompetent) - EMEA-C2-002307-PIP01-17-M01

Janssen-Cilag International NV; Prevention of Ebola Virus Disease

Day 30 letter

Vaccines

Summary of committee discussion:

The PDCO discussed the completed study and considered that it is compliant with the latest Agency's Decision (P/0138/2020) of 17 April 2020.
The PDCO finalised this partially completed compliance procedure on 04 September 2020.

2.2.7. MVA-BN-Filo (recombinant, non-replicating) - EMEA-C2-002308-PIP01-17-M01

Janssen-Cilag International NV; Prevention of Ebola Virus Disease

Day 30 letter

Vaccines

Summary of committee discussion:

The PDCO discussed the completed study and considered that it is compliant with the latest Agency's Decision (P/0138/2020) of 17 April 2020.
The PDCO finalised this partially completed compliance procedure on 04 September 2020.

2.2.8. Lonapegsomatropin - EMEA-C-002692-PIP01-19

Ascendis Pharma Endocrinology Division A/S; Treatment of growth hormone deficiency

Day 30 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO finalised this full compliance check procedure on 04 September 2020 and 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/0275/2020) of 15/07/2020.

2.2.9. Eptacog beta (activated) - EMEA-C-001203-PIP02-14-M02

LFB SA; Treatment of congenital coagulation disorders

Day 30 Opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO finalised this full compliance check procedure on 04 September 2020 and 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/0214/2017) of 09/08/2017.

2.2.10. Posaconazole - EMEA-C2-000468-PIP02-12-M06

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections

Day 30 letter

Infectious Diseases

Summary of committee discussion:

The PDCO discussed the compliance check request.

The PDCO discussed the completed studies 1,2,5,8 and 9, and considered that these are compliant with the latest Agency's Decision P/0101/2020 of 18 March 2020.

The PDCO finalised this partially completed compliance procedure on 4 September 2020.

2.2.11. Influenza virus surface antigens (haemagglutinin and neuraminidase) of the following strains: A/(H1N1), A/(H3N2), B/Yamagata lineage, B/Victoria lineage - EMEA-C-001782-PIP01-15-M03

Abbott Biologicals B.V.; Prevention of influenza infection

Day 30 Opinion

Vaccines

Summary of committee discussion:

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

- EMEA-C2-001782-PIP01-15-M02
- EMEA-C1-001782-PIP01-15

The PDCO adopted on 4 September 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0211/2018) of 17 July 2018.

2.2.12. Arimoclomol citrate - EMEA-C1-001748-PIP01-15

Orphazyme A/S; Treatment of Niemann-Pick disease, type C

Day 30 letter

Neurology

Summary of committee discussion:

The PDCO discussed the compliance check request.

The PDCO discussed the completed studies 1-8 and the initiation of study 9 and considered that these are compliant with the latest Agency's Decision P/0213/2018 of 17 July 2018.

The PDCO finalised this partially completed compliance procedure on 4 September 2020.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Etripamil - EMEA-002303-PIP01-17-M02

Milestone Pharmaceuticals, Inc.; Treatment of supraventricular tachychardia / Treatment of acute paroxysmal supraventricular tachycardia (PSVT)

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/0042/2019 of 29 January 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Edoxaban (tosylate) - EMEA-000788-PIP02-11-M10

Daiichi Sankyo Europe GmbH; Prevention of arterial thromboembolism, Prevention of venous thromboembolism, Treatment of venous thromboembolism / Prevention of arterial thromboembolism in paediatric cardiac patients at risk of thrombotic events, Acute treatment & secondary prevention of symptomatic recurrent venous thrombotic events (VTE) in paediatric patients at risk

Day 60 opinion

Cardiovascular Diseases / Haematology-Hemostaseology

Summary of committee discussion:

Following the receipt of additional information and justification prior to Day 60 the PDCO confirmed the outcome of the discussion 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/0013/2020 of 6 January 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Birch bark extract - Orphan - EMEA-001299-PIP03-17-M01

Amryt Research Limited; Treatment of epidermolysis bullosa / Treatment of epidermolysis bullosa

Day 60 opinion

Dermatology

Summary of committee discussion:

The PDCO confirmed the outcome of the discussion 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/0338/2019 of 11 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Velmanase alfa - Orphan - EMEA-001056-PIP02-12-M01

Chiesi Farmaceutici S.p.A.; Treatment of alpha-mannosidosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO confirmed all the conclusions reached 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/0122/2014 of 7 May 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Ozanimod hydrochloride - EMEA-001710-PIP03-17-M02

Celgene Europe B.V.; Treatment of ulcerative colitis / Treatment of moderate to severely active ulcerative colitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0008/2020 of 3 January 2020.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Pegylated-fibroblast growth factor 21 (BMS-986036) - EMEA-002448-PIP01-18-M01

Bristol-Myers Squibb International Corporation; Treatment of non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant's response to the D30 issues was acceptable.
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/0336/2019 issued on 10 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Mepolizumab - Orphan - EMEA-000069-PIP01-07-M07

GSK Trading Services Limited; Treatment of Hypereosinophilic Syndrome / Treatment of Hypereosinophilic Syndrome (HES)

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Overall a positive Opinion was agreed by the PDCO, accepting the requested waiver for a subset of childrenbased on the ground of lack of significant therapeutic benefit, because studies are not feasible.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0007/2016 of 29 January 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Vadadustat - EMEA-001944-PIP01-16-M02

Akebia Therapeutics, Inc.; Treatment of anaemia due to chronic disorders / Treatment of anaemia secondary to chronic kidney disease

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO confirmed all the conclusions reached 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/0074/2020 of 21 March 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Zanamivir - EMEA-001318-PIP01-12-M04

GlaxoSmithKline Trading Services Limited; Treatment of influenza, Prevention of influenza / Treatment of influenza A and B virus infection, Prevention of influenza A and B virus 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/0415/2019 of 04/12/2019)
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Copanlisib - Orphan - EMEA-001757-PIP02-15-M02

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of mature B-cell neoplasms / Treatment of children with a relapsed or refractory neuroblastoma, Ewing sarcoma, osteosarcoma or rhabdomyosarcoma including at first relapse, in combination with chemotherapy

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's views expressed at D30 were endorsed.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0133/2020 of 15 April 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Dabrafenib - EMEA-001147-PIP01-11-M07

Novartis Europharm Limited; Treatment of solid malignant tumours (excluding melanoma and glioma) / Treatment of melanoma / Treatment of paediatric patients with solid malignant tumours containing BRAF V600 mutations / Treatment of adolescent patients with melanoma with a BRAF V600 mutation

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's view expressed at D30 was re-discussed and endorsed.
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/0065/2019 of 22 March 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Daratumumab - Orphan - EMEA-002152-PIP01-17-M02

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with a lymphoid malignancy (except mature B-cell neoplasms)

Day 60 opinion

Oncology

Summary of committee discussion:

The committee re-discussed this procedure in line with the conclusion from the D30 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/0206/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Trametinib - EMEA-001177-PIP01-11-M06

Novartis Europharm Limited; Treatment of all conditions included in the category of malignant neoplasms (except melanoma, glioma, haematopoietic and lymphoid tissue), Treatment of melanoma / Treatment of paediatric patients with a solid malignant tumour with known or expected RAS, RAF or MEK pathway activation / Treatment of adolescent patients with melanoma containing BRAF V600 activating mutations

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's view expressed at D30 was re-discussed and endorsed.
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/0076/2019 of 22 March 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Dupilumab - EMEA-001501-PIP02-13-M06

sanofi-aventis recherche & développement; Treatment of asthma

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The applicant's responses to the D30 issues were considered acceptable.
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/0011/2020 issued on 6 January 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Ravulizumab - Orphan - EMEA-001943-PIP01-16-M05

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 60 opinion

Uro-nephrology / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO confirmed the outcome of the discussion 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/0199/2019 of 12 June 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Ravulizumab - Orphan - EMEA-002077-PIP01-16-M03

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 opinion

Uro-nephrology / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO confirmed the outcome of the discussion 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/0166/2019 of 15 May 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Filgotinib - EMEA-001619-PIP03-16-M01

Gilead Sciences International Ltd.; Treatment of Crohn's disease, Treatment of ulcerative colitis / Treatment of paediatric patients 2 years of age and older with moderately-to-severely active ulcerative colitis, Treatment of paediatric patients 2 years of age and older with moderately-to-severely active Crohn's disease

Day 30 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

This is a purely administrative modification to link the agreed two PIPs: ulcerative colitis (EMEA-001619-PIP03-16) to the chronic idiopathic arthritis PIP (EMEA-001619-PIP04-17-M01, EMA Decision P/0371/2018) by adding a cross reference to the decision. The PDCO agreed with the applicant's request to link the decisions of these two PIPs.
A positive opinion was therefore adopted at D 30.

2.3.18. Eculizumab - Orphan - EMEA-000876-PIP05-15-M04

Alexion Europe SAS; Treatment of myasthenia gravis / Treatment of refractory acetylcholine

receptor antibody (AChR-Ab) - positive generalised myasthenia gravis

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO' s views expressed at D30 were endorsed.
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/0200/2018 of 19 July 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Lenvatinib - EMEA-001119-PIP02-12-M07

Eisai GmbH; Treatment of osteosarcoma / Treatment of papillary thyroid carcinoma / Treatment of follicular carcinoma / Treatment of refractory or relapsed osteosarcoma in children and adolescents / Treatment of progressive, radioiodine-refractory differentiated thyroid cancer in children and adolescents

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO' s view expressed at D30 were endorsed.
Therefore, 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 adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0033/2020 of 29 January 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M01

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 60 opinion

Other / Pneumology – Allergology

Summary of committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0091/2019 of 22 March 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.21. Peanut allergen extract - EMEA-001481-PIP01-13-M04

DBV Technologies S.A.; Peanut allergy

Day 60 opinion

Pneumology – Allergology

Summary of committee discussion:

The PDCO discussed the Applicant's responses after Day 30 and found them satisfactory. All issues raised at Day 30 have now been resolved.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0342/2018 of 9 November 2018).
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

2.6.1. BAY 1747846 - EMEA-002778-PIP01-20

Bayer AG; Diagnosis by evaluation of any known or suspected clinical condition with contrast enhanced magnetic resonance imaging

Revision of the opinion – written procedure

Diagnostic

Summary of committee discussion:

The opinion was revised to reflect the withdrawal of the request for deferral, as well as to correct study timelines.

2.7. Partial Compliance Checks completed by EMA

The following partial compliance checks have concluded positively without PDCO discussion. The Committee has been informed in writing.

2.7.1. Palonosetron / fosnetupitant - EMEA-C2-001198-PIP03-17-M04

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

Day 30 letter

Gastroenterology-Hepatology

2.7.2. Lasmiditan - EMEA-C2-002166-PIP01-17-M04

Eli Lilly Nederland B.V.; Migraine with and without aura

Day 30 letter

Neurology

2.7.3. Pneumococcal polyssacharide serotype 5, 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 33F, 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 19F, 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 10A, 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 15B, 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 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 3, 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 11A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 1, 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 22F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polyssacharide serotype 14, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate - EMEA-C1-002330-PIP01-18

Pfizer Europe MA EEIG; Disease caused by *streptococcus pneumoniae*

Day 30 letter

Vaccines

2.7.4. Ivacaftor / tezacaftor / elexacaftor - EMEA-C2-002324-PIP01-17

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 letter

Other

2.7.5. Dermatophagoides pteronyssinus / Dermatophagoides farinae - EMEA-C2-001258-PIP01-11-M06

ALK-Abelló A/S; Treatment of asthma

Day 30 letter

Pneumology - Allergology

3. Discussion of applications

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

3.1. Discussions on Products D90-D60-D30

3.1.1. Recombinant human acid alpha-glucosidase - Orphan - EMEA-002447-PIP01-18

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

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Venglustat - Orphan - EMEA-001716-PIP04-19

Genzyme Europe B.V.; GM2 gangliosidosis, Other gangliosidosis, GM1, GM3, Defects in glycoprotein degradation, sialidosis / Long term treatment of patients with a confirmed diagnosis of late onset GM2 gangliosidosis / Long term treatment in patients within the same biochemical pathway as GM2 gangliosidosis / Long term treatment in patients with Juvenile (Subacute) and Adolescent (Late-Onset) GM2 gangliosidosis ages 2 years old and older, males/females

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

3.1.3. Guselkumab - EMEA-001523-PIP04-19

Treatment of Ulcerative Colitis

Day 90 discussion

Gastroenterology-Hepatology

3.1.4. Bispecific antibody binding to clotting factor IX and X - EMEA-002762-PIP02-20

Treatment of haemophilia A / Routine prophylaxis to prevent or reduce frequency of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) with or without factor VIII inhibitors

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Garadacimab - EMEA-002726-PIP01-19

Hereditary angioedema attacks (HAE)

Day 90 discussion

Haematology-Hemostaseology

3.1.6. Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of acute graft-versus-host disease (aGVHD)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Alpha1-proteinase inhibitor (human) - EMEA-001312-PIP03-19

Treatment of acute graft-versus-host disease (GVHD)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.8. Bimekizumab - EMEA-002189-PIP03-19

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis) / Treatment of JIA (enthesitis-related arthritis [ERA] and juvenile psoriatic arthritis [JPsA]) in patients from ≥2 years to <18 years of age

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.9. Cotadutide - EMEA-002712-PIP01-19

Treatment of non-cirrhotic non-alcoholic steatohepatitis (NASH) with fibrosis / Treatment of non-cirrhotic non-alcoholic steatohepatitis (NASH) with fibrosis in children and adolescents

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.10. Dapirolizumab pegol - EMEA-002702-PIP01-19

Treatment of systemic lupus erythematosus (SLE) / Treatment of children and adolescents ≥5

years to <18 years of age with active SLE despite standard therapy

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.11. Taniborbactam / cefepime - EMEA-002576-PIP01-19

Treatment of gram-negative bacterial infections / Treatment of cUTI including acute pyelonephritis

Day 90 discussion

Infectious Diseases

3.1.12. Vonoprazan - EMEA-002703-PIP01-19

Treatment of reflux oesophagitis / Treatment of Helicobacter pylori infection / Eradication of H. pylori concurrently given with appropriate antibiotic therapy / Treatment of erosive reflux oesophagitis / Maintenance of healed erosive reflux oesophagitis

Day 90 discussion

Infectious Diseases / Gastroenterology-Hepatology

3.1.13. Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene - Orphan - EMEA-002741-PIP01-20

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 90 discussion

Neurology

3.1.14. Lanadelumab - Orphan - EMEA-001864-PIP03-19

Shire Pharmaceuticals Ireland Limited (a Takeda company); Prevention of attacks of Idiopathic non-histaminergic angioedema (INHA)

Day 90 discussion

Other

3.1.15. Adrenaline (epinephrine) - EMEA-002749-PIP01-19

Treatment of allergic reactions / The emergency treatment of allergic reactions, including anaphylaxis.

Day 90 discussion

Pneumology - Allergology

3.1.16. EMEA-002653-PIP01-19

Treatment of Schizophrenia / Treatment of cognitive impairment associated with schizophrenia in patients 13 to <18 years of age

Day 90 discussion

Psychiatry

3.1.17. Dupilumab - EMEA-001501-PIP07-20

treatment of chronic spontaneous urticaria

Day 60 discussion

Dermatology

3.1.18. LM-030 - Orphan - EMEA-002770-PIP02-20

LifeMax Laboratories, Inc.; Netherton Syndrome

Day 60 discussion

Dermatology

3.1.19. Adeno-associated virus serotype 5- (AAV5-) based vector that contains the human phenylalanine hydroxylase (hPAH) gene - Orphan - EMEA-002833-PIP01-20

BioMarin International Limited; Treatment of phenylalanine hydroxylase deficiency / Treatment of patients with Phenylketonuria

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. Dasiglucagon - Orphan - EMEA-002233-PIP02-20

Zealand Pharma; Congenital Hyperinsulinism

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.21. Hydroxypropyl-β-cyclodextrin (HPβCD) - Orphan - EMEA-002839-PIP01-20

Cyclo Therapeutics Inc; Niemann Pick disease type C / Treatment of Niemann Pick type C1 disease in children, adolescents and adults

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.22. Liposomal annamycin - EMEA-002810-PIP01-20

Treatment of acute myeloid leukaemia / Treatment of relapsed acute myeloid leukaemia

Day 60 discussion

Haematology-Hemostaseology

3.1.23. EMEA-002350-PIP02-20

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis) / Treatment of juvenile psoriatic arthritis (JPsA) in paediatric patients 6 years of age and older.

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.24. Human plasma derived c1-inhibitor - EMEA-002818-PIP01-20

Pre-procedure prevention of acute hereditary angioedema (HAE) / Treatment of hereditary angioedema (HAE)

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.25. Bimekizumab - EMEA-002189-PIP04-20

Treatment of hidradenitis suppurativa / Treatment of moderate to severe hidradenitis suppurativa in adolescents from 12 years of age

Day 60 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.26. Risankizumab - EMEA-001776-PIP05-20

Hidradenitis suppurativa

Day 60 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.27. 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting glial fibrillary acidic protein pre-mRNA - Orphan - EMEA-002822-PIP01-20

Ionis Pharmaceuticals; Alexander disease

Day 60 discussion

Neurology

3.1.28. Erdafitinib - EMEA-002042-PIP02-20

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of locally advanced or metastatic solid tumors, including primary CNS tumors, harboring susceptible FGFR alterations in patients who have progressed following prior therapies and those who have no acceptable standard therapies

Day 60 discussion

Oncology

3.1.29. Imetelstat - Orphan - EMEA-001910-PIP03-20

Geron Corporation; Treatment of Acute Myeloid Leukemia (AML)/ Treatment of Myelodysplastic Syndromes (MDS), including Juvenile Myelomonocytic Leukemia (JMML) / Treatment of paediatric patients with relapsed or refractory AML or MDS, including JMML, from 1 year to less than 18 years of age

Day 60 discussion

Oncology

3.1.30. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002821-PIP01-20

Prevention of RSV- associated lower respiratory tract illness through maternal immunization / Active immunization of pregnant women during the second and third trimester of pregnancy to prevent respiratory syncytial virus (RSV) -associated lower respiratory tract illness (LRTI) in infants by transfer of maternal antibodies.

Day 60 discussion

Vaccines

3.1.31. Ezetimibe / Atorvastatin - EMEA-002852-PIP01-20

Prevention of cardiovascular events, Treatment of hypercholesterolemia / The combination of Atorvastatin and Ezetimibe is indicated for the treatment of hypercholesterolemia as substitution therapy in adult patients adequately controlled with the individual substances given concurrently at the same dose level as in the fixed dose combination, but as separate products / Atorvastatin/Ezetimibe is indicated as substitution therapy to reduce the risk of cardiovascular events in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS), for adults receiving atorvastatin and ezetimibe concurrently at the same dose level.

Day 30 discussion

Cardiovascular Diseases

3.1.32. Perindopril arginine / indapamide / amlodipine besilate - EMEA-002849-PIP01-20

Hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.33. Ralinepag - Orphan - EMEA-002432-PIP02-20

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension / Treatment of pulmonary arterial hypertension WHO Group I to improve exercise capacity and to delay clinical worsening

Day 30 discussion

Cardiovascular Diseases

3.1.34. Sotatercept - EMEA-002756-PIP01-19

Pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.35. Chimeric fibril-reactive IgG1k monoclonal antibody 11-1F4 - Orphan - EMEA-002791-PIP01-20

Real Regulatory Limited; AL Amyloidosis / Treatment of AL Amyloidosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.36. Indometacin / levonorgestrel - EMEA-002820-PIP01-20

Contraceptive management

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.37. Pioglitazone / spironolactone / metformin - EMEA-002187-PIP01-17

Treatment of polycystic ovary syndrome (PCOS) / Treatment of adolescent polycystic ovary syndrome (PCOS) in post-menarche adolescents <18 yrs and young adult women ≥18 yrs and <24.0 yrs

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Treatment of mucopolysaccharidosis II (Hunter syndrome)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.39. EMEA-001475-PIP04-20

Treatment of Biliary atresia

Day 30 discussion

Gastroenterology-Hepatology

3.1.40. Recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8 - Orphan - EMEA-002856-PIP01-20

Allakos Inc; Treatment of eosinophilic gastrointestinal inflammatory disorders / Treatment of eosinophilic esophagitis / Treatment of eosinophilic gastritis and/or eosinophilic gastroenteritis

Day 30 discussion

Gastroenterology-Hepatology

3.1.41. Pegfilgrastim - EMEA-002671-PIP02-20

Treatment of chemotherapy-induced neutropenia and Prevention of chemotherapy-induced febrile neutropenia / Reduction in the duration of neutropenia and the incidence of febrile neutropenia in paediatric patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 30 discussion

Haematology-Hemostaseology

3.1.42. EMEA-002350-PIP03-20

Treatment of systemic lupus erythematosus (SLE) / Treatment of lupus nephritis (LN) despite receiving SoC / Treatment of systemic lupus erythematosus (SLE) despite receiving standard of care (SoC)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.43. Iscalimab - EMEA-002842-PIP01-20

Prophylaxis against transplant rejection / Prophylaxis of graft rejection in paediatric renal transplant patients 2 years of age and older

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.44. Fasudil - EMEA-002841-PIP01-20

Non-traumatic subarachnoid haemorrhage

Day 30 discussion

Neurology

3.1.45. Rimegepant - EMEA-002812-PIP02-20

Acute treatment of migraine

Day 30 discussion

Neurology

3.1.46. Viltolarsen - Orphan - EMEA-002853-PIP01-20

NS Pharma, Inc.; Treatment of Duchenne muscular dystrophy / Treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping

Day 30 discussion

Neurology

3.1.47. Allogeneic BCMA-directed chimeric antigen receptor T Cell - EMEA-002834-PIP01-20

Multiple myeloma

Day 30 discussion

Oncology

3.1.48. Lurbinectedin - Orphan - EMEA-002846-PIP01-20

Pharma Mar, S.A.; Treatment of small cell lung cancer

Day 30 discussion

Oncology

3.1.49. Serplulimab - EMEA-002859-PIP01-20

Lung cancer (SCLC and NSCLC)

Day 30 discussion

Oncology

3.1.50. Talazoparib - EMEA-002066-PIP01-20

Treatment of Ewing sarcoma / Treatment of refractory or recurrent Ewing Sarcoma

Day 30 discussion

Oncology

3.1.51. EMEA-002843-PIP01-20

Treatment of ovarian cancer

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.52. EMEA-002843-PIP02-20

Treatment of breast cancer

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.53. Flotetuzumab - EMEA-002855-PIP01-20

Acute myeloid leukemia (AML)

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.54. Atropine - EMEA-002858-PIP01-20

Prevention of myopic progression

Day 30 discussion

Ophthalmology

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

Recordati Rare Diseases; Neurotrophic keratitis

Day 30 discussion

Ophthalmology

3.1.56. (1R,2R,3S,4S,5R,6S)-cyclohexane-1,2,3,4,5,6-hexayl-hexakis (dihydrogen phosphate) - Orphan - EMEA-002854-PIP01-20

Sanifit Therapeutics S.A.; Treatment of calciphylaxis

Day 30 discussion

Other

3.1.57. Dapagliflozin - EMEA-000694-PIP05-20

COVID-19, virus identified, COVID-19, virus not identified

Day 30 discussion

Other

3.1.58. Garetosmab - Orphan - EMEA-002736-PIP01-19

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

Day 30 discussion

Other

3.1.59. Ravulizumab - EMEA-001943-PIP02-20

Hematopoietic stem cell transplantation associated / Thrombotic Microangiopathy (HSCT-TMA) / Treatment of Thrombotic Microangiopathy after Haematopoietic Stem Cell Transplantation (HSCT-TMA)

Day 30 discussion

Uro-nephrology / Haematology-Hemostaseology

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

Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Vaccines

3.2. Discussions on Compliance Check

The following compliance checks have been put up for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance.

3.2.1. Sofosbuvir / voxilaprevir / velpatasvir - EMEA-C-001822-PIP01-15-M01

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C

Day 30 discussion

Infectious Diseases

3.2.2. Regorafenib - EMEA-C2-001178-PIP01-11-M05

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

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Bilastine - EMEA-000347-PIP02-16-M02

Faes Farma S.A.; Treatment of allergic conjunctivitis

Day 30 discussion

3.3.2. Tralokinumab - EMEA-001900-PIP02-17-M04

LEO Pharma A/S; Treatment of Atopic Dermatitis

Day 30 discussion

Dermatology

3.3.3. Canagliflozin (1s)-1,5-anhydro-1-[3-[[5-(4-fluorophenyl)-2-thienyl]methyl]-4-methylphenyl]-D-glucitol hemihydrate - EMEA-001030-PIP01-10-M08

Janssen-Cilag International NV; Type 2 Diabetes Mellitus / Treatment of Type 2 Diabetes Mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Drospirenone / estetrol monohydrate - EMEA-001332-PIP01-12-M04

Estetra SPRL; Prevention of pregnancy

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Lixisenatide - EMEA-000916-PIP01-10-M07

sanofi-aventis R&D; Type 2 diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Olipudase alfa - Orphan - EMEA-001600-PIP01-13-M02

Genzyme Europe B.V.; Treatment of Niemann-Pick Disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Recombinant parathyroid hormone: rhPTH (1-84) - Orphan - EMEA-001526-PIP01-13-M04

Shire Pharmaceuticals Ireland Limited; Hypoparathyroidism / Treatment of hypoparathyroidism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.8. Filgotinib - EMEA-001619-PIP03-16-M01

Gilead Sciences International Ltd.; Treatment of paediatric patients 2 years of age and older with moderately-to-severely active ulcerative colitis / Treatment of paediatric patients 2 years of age and older with moderately-to-severely active Crohn’ s disease

Day 30 discussion

Gastroenterology-Hepatology

3.3.9. Potassium Chloride / Sodium Chloride / Ascorbic Acid / Sodium Sulfate / Sodium Ascorbate / Polyethylene Glycol 3350 - EMEA-001705-PIP02-15-M03

Norgine Limited; Bowel cleansing prior to any procedure requiring a clean bowel / Bowel cleansing prior to clinical procedures

Day 30 discussion

Gastroenterology-Hepatology

3.3.10. Teduglutide ([gly2] recombinant human glucagon-like peptide) - Orphan - EMEA-000482-PIP01-08-M06

Shire Pharmaceuticals Ireland Limited; Other and unspecified post-surgical non absorption - Syndrome Short Bowel / Treatment of Short Bowel Syndrome

Day 30 discussion

Gastroenterology-Hepatology

3.3.11. Vonicog alfa - EMEA-001164-PIP01-11-M04

Baxalta Innovations GmbH; Von Willebrand disease / Prevention and treatment of bleeding episodes and for surgical and invasive procedures in paediatric patients (less than 18 years of age) with von Willebrand disease

Day 30 discussion

Haematology-Hemostaseology

3.3.12. Apremilast - EMEA-000715-PIP03-11-M06

Amgen Europe B.V.; Treatment of psoriasis / Treatment of moderate to severe psoriasis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.13. Ustekinumab - EMEA-000311-PIP03-11-M06

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, PsA and juvenile idiopathic arthritis [JIA]) / Treatment of juvenile idiopathic arthritis (juvenile psoriatic arthritis [jPsA]

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.14. Tazobactam / ceftolozane - EMEA-001142-PIP01-11-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of urinary tract infections / Treatment of intra-abdominal infections / Treatment of complicated urinary tract infections (cUTI) / Treatment of complicated intra-abdominal infections (cIAI)

Day 30 discussion

Infectious Diseases

3.3.15. Tedizolid - EMEA-001379-PIP01-12-M05

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

Day 30 discussion

Infectious Diseases

3.3.16. Tenofovir alafenamide / emtricitabine / elvitegravir / cobicistat - EMEA-001460-PIP01-13-M05

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection / For the treatment of HIV-1 infection in paediatric patients from 6 years to less than 18 years.

Day 30 discussion

Infectious Diseases

3.3.17. Tenofovir disoproxil (as fumarate) - EMEA-000533-PIP01-08-M09

Gilead Sciences International Limited; Treatment of chronic viral hepatitis B / For treatment of chronic hepatitis B in paediatric patients from 2 years of age with compensated liver disease.

Day 30 discussion

Infectious Diseases

3.3.18. Fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16-M03

Zogenix International Ltd; Dravet syndrome / The adjunctive treatment of seizures in paediatric patients at least 1 year of age with Dravet syndrome

Day 30 discussion

Neurology

3.3.19. Humanised anti-IL-6 receptor (IL-6R) monoclonal antibody - Orphan - EMEA-001625-PIP01-14-M06

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 30 discussion

Neurology

3.3.20. Nivolumab - EMEA-001407-PIP01-12-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old / Treatment of a paediatric malignant solid tumour in paediatric patients from 6 months to less than 18 years old

Day 30 discussion

Oncology

3.3.21. Nivolumab - EMEA-001407-PIP02-15-M04

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue, Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients with a relapsed or refractory non-Hodgkin lymphoma in the age group from 6 months to less than 18 years old / Treatment of paediatric patients from 6 months to less than 18 years of age with a recurrent or progressive high-grade glioma / Treatment of paediatric patients with a relapsed or refractory Hodgkin lymphoma in the age group from 5 years to less than 18 years

Day 30 discussion

Oncology

3.3.22. Veliparib - EMEA-000499-PIP02-10-M01

AbbVie Ltd; Treatment of high-grade glioma

Day 30 discussion

Oncology

3.3.23. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M10

MediWound Germany GmbH; Treatment of burns of external body surfaces / Removal of eschar in deep partial and/or full thickness burns

Day 30 discussion

Other

3.3.24. Palovarotene - Orphan - EMEA-001662-PIP01-14-M04

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 30 discussion

Other

3.3.25. Benralizumab - EMEA-001214-PIP01-11-M10

AstraZeneca AB; Asthma / Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3.26. Birch pollen extract (Betula verrucosa) - EMEA-001879-PIP01-15-M02

ALK-Abelló A/S; Treatment of allergic rhinitis / rhino-conjunctivitis / Treatment of tree pollen allergic rhinitis / rhino-conjunctivitis

Day 30 discussion

Pneumology - Allergology

3.3.27. Brexpiprazole - EMEA-001185-PIP01-11-M06

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of schizophrenia from 13 to less than 18 years of age

Day 30 discussion

Psychiatry

3.3.28. Dengue virus serotype 4 (live, attenuated) / Dengue virus serotype 3 (live, attenuated) / Dengue virus serotype 2 (live, attenuated) / Dengue virus serotype 1 (live, attenuated) - EMEA-001888-PIP01-15-M01

Takeda Vaccines, Inc.; Prevention of dengue fever

Day 30 discussion

Vaccines

4. Nominations

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

4.1. List of submission of applications with start of procedure 15 September 2020 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:

No item

4.3. Nominations for other activities

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

PDCO representatives at SAWP are Karl-Heinz Huemer, Sara Galluzzo, as PDCO / SAWP members and Johanna Wernsperger, Dina Apele-Freimane as their PDCO / SAWP Alternates respectively.

5.1. New Scientific Advice

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

5.2. Ongoing Scientific Advice

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

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

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

6. Discussion on the applicability of class waivers

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Discussions on the applicability of class waiver for products

6.1.1. Bevacizumab (ophthalmic formulation) - EMEA-07-2020

Le4d Global Regulatory Science Limited; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema/ Treatment of neovascular (wet) 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: retinopathy of prematurity.

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

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

No item

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

The following members/alternates have been appointed by the European Commission to the PDCO to represent health professionals for a 3-year term from 1st August 2020:

- Member: Michael Fruehwald

– Alternate: Johannes Taminiau

– Member: Fernando Cabañas

– Alternate: Doina Anca Plesca

– Member: Francesca Rocchi

– Alternate: Catherine Cornu

The following members/alternates have been appointed by the European Commission to the PDCO to represent patients' organisations for a 3-year term from 1st August 2020:

– Member: Jaroslav Sterba

- Alternate: Milena Stevanovic

– Member: Dimitrios Athanasiou

– Alternate: Tomasz Grybek

- Member: Katrin Claus

– Alternate: Nora Kriauzaite

The following members have expired their mandate:

– Viviana Giannuzzi

– Jorrit Gerritsen

– Paola Baiardi

– Günter Karl-Heinz Auerswald

- Michal Odermarsky

Post-meeting note: Michael Fruehwald and Katrin Claus have resigned as members of the PDCO. Following Katrin Claus' resignation, the European Commission appointed Nora Kriauzaite as member and Michal Odermarsky as alternate to represent patients' organisations.

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 PDCO members were informed about the final CHMP Opinions on four medicinal products with recommended paediatric indications adopted in July 2020 by CHMP. These include Crisvita (borusumab), Latuda (Lurasidone) and Votubia (everolimus).

The CHMP adopted one negative opinion on Gamifant (emapalumab).

The list of procedures with paediatric indications to be evaluated by the CHMP, starting in July 2020, was presented to the PDCO members.

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 identified the products which will require Non-Clinical Working Group evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

Relevant products for FWG discussion were identified.

9.4. Cooperation within the EU regulatory network

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

Summary of committee discussion:

The committee was informed of the modifications to the mandate of Enpr-EMA's Coordinating Group, which were being proposed as part of the regular review of the mandate and which will be adopted by Enpr-EMA's Coordinating Group at their next meeting.

9.5. Cooperation with International Regulators

No item

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

9.8.1. Strategic Review and Learning Meeting (SRLM) under the German Presidency to be held virtually on 22nd October 2020

The PDCO was informed about the planned sessions and discussions at the next SRLM.

10. Any other business

10.1.1. Covid-19 update

Updates were provided on the development of therapeutics and vaccines for COVID-19.

10.1.2. Implementation of extrapolation - update

The PDCO was informed about the extrapolation topic discussion during the last EMA-EuNetHTA bilateral and updated on recent activities around the implementation exercise within the network.

10.1.3. Guidance supporting the discussions on waiver grounds

The PDCO discussed structured guidance on the choice of waiver grounds.

10.1.4. Publication on the evaluation of the orphan and paediatric regulation - next steps

The PDCO was updated on the evaluation of the legislations on medicines for children and rare diseases.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

General topics (e.g. dose-findings studies and combination studies) on oncology paediatric investigation plans were discussed.

11.1.2. Neonatology

Summary of committee discussion:

Preparatory discussions in relation to the upcoming annual workshop of the International Neonatal Consortium on 28/29 October 2020.

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 1-4th September 2020 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 on:	2.3.7. Mepolizumab - Orphan - EMEA-000069-PIP01-07 -M07 2.3.9. Zanamivir - EMEA-001318-PIP01-12 -M04 3.1.32. Recombinant humanised monoclonal antibody (IgG1, Kappa) to IL-5 - EMEA-002836-PIP01-20 3.1.34. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002821-PIP01-20
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Lucie Kravackova	Member	Czech Republic	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Ann Marie Totterman	Member	Finland	No interest 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	
Anastasia Mountaki	Alternate	Greece	No interests declared	
Ágnes	Member (CHMP	Hungary	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Gyurasics	member)			
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	
Sigita Burokiene	Member	Lithuania	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roel Bolt	Member	Netherlands	No interests declared	
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 interests declared	
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 interest declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Eva Agurell	Alternate	Sweden	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Günter Karl-Heinz	Expert	Patients' Organisation	No restrictions applicable to this	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Auerswald		Representative	meeting	
Paola Baiardi	Expert	Patients' Organisation Representative	No restrictions applicable to this meeting	
Michal Odermarsky	Expert	Patients' Organisation Representative	No restrictions applicable to this meeting	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Viviana Giannuzzi	Expert	Patients' Organisation Representative	No restrictions applicable to this meeting	
Lutz Wiesner	Expert	BfArM	No interests declared	
María Estela Moreno Martín	Expert	AEMPS	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/