28 February 2020
EMA/PDCO/52138/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes for the meeting on 28-31 January 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).

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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. 23 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 3.2.6 and 3.1.52.

1.2. Adoption of agenda

PDCO agenda for 28-31 January 2020

The agenda of the PDCO meeting 28th -31st January was adopted.

1.3. Adoption of the minutes

PDCO minutes for 09-11 December 2019

The minutes from the PDCO plenary 09-11 December 2019 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. Tris(hydroxymethyl)aminomethane trihydrate (PF-05221304-82) - EMEA-002552-PIP01-19

Pfizer Europe MA EEIG; Treatment of non-alcoholic steatohepatitis (NASH)

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO discussed this procedure on D120 and the applicant's responses to the D90 issues were generally considered acceptable. A positive opinion was adopted.

2.1.2. Avatrombopag maleate - EMEA-001136-PIP02-19

Dova Pharmaceuticals Ireland Limited; Chemotherapy-induced thrombocytopenia

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO's views expressed at Day 90 were endorsed and the opinion finalised. In conclusion, the PDCO adopted a positive opinion for a paediatric investigation plan for avatrombopag maleate in the condition 'treatment of chemotherapy-induced thrombocytopenia'.

2.1.3. Autologous CD34+ enriched cells from patients with Fanconi anaemia subtype A (FA-A) transduced *ex vivo* with lentiviral vector carrying the FANCA gene (PGKFANCA-WPRE) - Orphan - EMEA-002578-PIP01-19

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

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the assessment of this application the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant for autologous CD34+enriched cells from patients with Fanconi anaemia subtype A transduced *ex vivo* with a lentiviral vector carrying the FANCA gene (PGK-FANCA-WPRE) in the condition of treatment of Fanconi anaemia subtype A. The plan is not deferred.

2.1.4. Allogeneic, *ex vivo* expanded, umbilical cord blood-derived, haematopoietic CD34+progenitor cells (CF)/ Allogeneic, non-expanded, umbilical cord blood-

derived, haematopoietic mature myeloid and lymphoid cells (NF) - Orphan - EMEA-001913-PIP02-18

Gamida Cell Ltd; Treatment in haematopoietic stem cell transplantation

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed this procedure at D120 during the January 2020 plenary meeting. The PDCO noted that the applicant provided clarifications for all the points that were unclear.
The Committee adopted a positive Opinion at Day 120 for Allogeneic,non-expanded,umbilical cord blood-derived,haematopoietic mature myeloid and lymphoid cells (NF) / Allogeneic,*ex vivo* expanded,umbilical cord blood-derived,haematopoietic CD34+progenitor cells (CF) for the treatment in haematopoietic stem cell transplantation.

2.1.5. Baricitinib - EMEA-001220-PIP05-19

Eli Lilly and Company Limited; Treatment of systemic lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

Between Day 90 and Day 120 the applicant provided satisfactory responses to the PDCO's requests concerning aspects of the study plan. A positive opinion was adopted.

2.1.6. Autologous CD34+ haematopoietic stem cells transduced *ex vivo* with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene - Orphan - EMEA-002562-PIP01-19

Rocket Pharmaceuticals, Inc.; Leukocyte adhesion deficiency type I

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

In conclusion, based on the assessment of this application the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant for autologous CD34+ haematopoietic stem cells transduced *ex vivo* with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene in the condition of treatment of leukocyte adhesion deficiency type I (LAD-I). The plan is due for completion in 2026.

2.1.7. Zoliflodacin - EMEA-002599-PIP01-19

Entasis Therapeutic Inc.; Treatment of gonococcal infection

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

In conclusion, based on the assessment of this application the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant for zoliflodacin in the condition of treatment of gonococcal infection. The plan is not deferred and expected to complete mid next year.

2.1.8. Cannabidiol - Orphan - EMEA-001964-PIP02-19

GW Pharma (International) B.V; Treatment of Rett syndrome

Day 120 opinion

Neurology

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 modified proposal for a paediatric investigation plan. A positive opinion endorsing the PIP has therefore been adopted.

2.1.9. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19

Argenx BVBA; Treatment of myasthenia gravis

Day 120 opinion

Neurology

Summary of committee discussion:

Based on the assessment of this application the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant for efgartigimod alfa in the condition of treatment of myasthenia gravis. Except for initiation of the paediatric study the plan is fully deferred and expected to complete in 2025.

2.1.10. Soticlestat - EMEA-002572-PIP01-19

Takeda Pharma A/S; Treatment of chromosome 15q duplication syndrome / Treatment of cyclin-dependent kinase-like 5 deficiency disorder

Day 120 opinion

Neurology

Summary of committee discussion:

The PDCO noted that the outstanding issues in relation to formulation development have now been resolved and confirmed that the modified PIP proposal is now adequate and agreeable. A positive opinion confirming the PIP has therefore been adopted.

2.1.11. Timrepigene emparvovec - Orphan - EMEA-002430-PIP01-18

Nightstar Europa Limited; Treatment of choroideremia

Day 120 opinion

Ophthalmology

Summary of committee discussion:

The PDCO discussed this procedure on D120 and the applicant's responses to the D90 issues were considered acceptable. A positive opinion was adopted.

2.1.12. Anti-neonatal Fc receptor human monoclonal antibody - Orphan - EMEA-002559-PIP02-19

Momenta Pharmaceuticals, Inc.; Treatment of myasthenia gravis

Day 120 opinion

Other

Summary of committee discussion:

Based on the assessment of this application the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant for anti-neonatal Fc receptor human monoclonal antibody in the condition of treatment of myasthenia gravis. Except for initiation of the paediatric study the plan is fully deferred and expected to complete in 2025.

2.1.13. Rosuvastatin / ezetimibe - EMEA-002257-PIP02-19

ELPEN Pharmaceutical Co. Inc.; Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application, the PDCO adopted a positive opinion on the applicant's request of a product-specific waiver for Rosuvastatin/Ezetimibe for all subsets of the paediatric population for the prevention of cardiovascular events on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.14. Nemolizumab - EMEA-001624-PIP02-19

Galderma International S.A.; Treatment of prurigo nodularis

Day 60 opinion

Dermatology

Summary of committee discussion:

Based on the submitted responses by the Applicant after Day 30, the PDCO agrees with the request for a waiver.

The PDCO granted a waiver for nemolizumab for all subsets of the paediatric population (0 to 18 years of age) for Treatment of prurigo nodularis.

2.1.15. Gefapixant (citrate salt) - EMEA-002267-PIP03-19

Merck Sharp & Dohme (Europe), Inc.; Treatment of endometriosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO's view expressed at D30 was re-discussed and endorsed.
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 gefapixant (citrate salt) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of endometriosis.

2.1.16. Levodopa / Carbidopa - EMEA-002687-PIP01-19

Neuroderm Ltd; Treatment of Parkinson's disease

Day 60 opinion

Neurology

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 levodopa / carbidopa for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of Parkinson's disease.
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.

2.1.17. Verdiperstat - Orphan - EMEA-002708-PIP01-19

Biohaven Pharmaceuticals, Inc.; Treatment of multiple system atrophy

Day 60 opinion

Neurology

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 verdiperstat for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of multiple system atrophy" on the grounds that the disease does not occur in the paediatric 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.

2.1.18. (2S)-2-[[2-[(4S)-4-(difluoromethyl)-2-oxo-oxazolidin-3-yl]-5,6-dihydroimidazo[1,2-d][1,4]benzoxazepin-9-yl]amino]propanamide - EMEA-002686-PIP01-19

Roche Registration GmbH; Treatment of breast cancer

Day 60 opinion

Action: For adoption

Oncology

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 (2S)-2-[[2-[(4S)-4-(difluoromethyl)-2-oxo-oxazolidin-3-yl]-5,6-dihydroimidazo[1,2-d][1,4]benzoxazepin-9-yl]amino]propanamide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of breast cancer based on the ground that the disease does not occur in the paediatric 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.

2.1.19. 6 fluoro-7-(2-fluoro-6-hydroxyphenyl)-(1M)-1-[4-methyl-2-(propan-2-yl)pyridin-3-yl]-4-[(2S)-2- methyl-4-(prop-2-enoyl)piperazin-1-yl]pyrido[2,3-d]pyrimidin-2(1H)-one - EMEA-002690-PIP01-19

Amgen Europe BV; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee , the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for 6 fluoro-7-(2-fluoro-6-hydroxyphenyl)-(1M)-1-[4-methyl-2-(propan-2-yl)pyridin-3-yl]-4-[(2S)-2- methyl-4-(prop-2-enoyl)piperazin-1-yl]pyrido[2,3-d]pyrimidin-2(1H)-one for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of lung cancer based on the ground that the disease does not occur in the paediatric 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. Daratumumab - Orphan - EMEA-002152-PIP03-19

Janssen-Cilag International N.V.; Treatment of systemic light chain amyloidosis

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO agrees with the applicant's request for a waiver and recommends granting a waiver for daratumumab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of systemic light chain amyloidosis.

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. Eftilagimod alpha - EMEA-002698-PIP01-19

Immutep SAS; Treatment of breast cancer

Day 60 opinion

Oncology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for eftilagimod alpha for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of breast cancer based on the ground that the disease does not occur in the paediatric 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.

2.1.22. Parsaclisib - Orphan - EMEA-002696-PIP01-19

Incyte Biosciences Distribution B.V.; Treatment of mature B-cell malignancies

Day 60 opinion

Oncology

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 parsaclisib for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mature B-cell malignancies based on the ground 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. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.23. Zolbetuximab - Orphan - EMEA-002695-PIP01-19

Astellas Pharma Europe B.V.; Treatment of gastric cancer and gastro-oesophageal junction adenocarcinoma / Treatment of pancreatic cancer

Day 60 opinion

Oncology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for zolbetuximab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of gastric cancer and gastro-oesophageal junction adenocarcinoma and treatment of pancreatic cancer based on the ground that the diseases do not occur in the paediatric 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.

2.1.24. Brolucizumab - EMEA-002691-PIP01-19

Novartis Europharm Limited; Treatment of visual impairment due tomacular edema associated with retinal vein occlusion (branch (RVO) or central (RVO))

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 (0 to 18 years of age) in the condition of "treatment of visual impairment due to macular edema associated with retinal vein occlusion (branch RVO or central RVO)" on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible due to the rarity of this condition in the paediatric 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. The PDCO identified retinopathy of prematurity 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.

2.1.25. 5%-Δ9-Tetrahydrocannabinol standardised Cannabis extract - EMEA-002668-PIP01-19

Vertanical GmbH; Treatment of chronic pain

Day 60 opinion

Pain

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 5%-Δ9-Tetrahydrocannabinol standardised Cannabis extract for all subsets of the paediatric population (0 to 18 years of age) in the condition Treatment of chronic pain.
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.

2.1.26. Avapritinib - Orphan - EMEA-002358-PIP03-19

Blueprint Medicines (Netherlands) B.V.; Treatment of mastocytosis

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

The Committee granted a waiver on its own motion for avapritinib in all subsets of the paediatric population for 'Treatment of mastocytosis' on the grounds that clinical studies with avapritinib cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subsets.

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. Filgotinib - EMEA-C1-001619-PIP03-16

Gilead Sciences International Ltd.; Treatment of ulcerative colitis

Day 60 letter

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO finalised this partially compliance procedure on 31 January 2020.

2.2.2. Autologous cartilage derived cultured chondrocytes - EMEA-C1-001823-PIP01-15-M01

TETEC AG; Treatment of cartilage disorders

Day 60 letter

Other

Summary of committee discussion:

In light of all the information provided, the PDCO considered that the Study is compliant with the latest Agency's Decision (P/0074/2019) of 22 March 2019.
The PDCO finalised this partially completed compliance procedure on 31 January 2020.

2.2.3. Liraglutide - EMEA-C2-000128-PIP02-09-M03

Novo Nordisk A/S; Treatment of obesity

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Studies are hereby confirmed to be compliant as set out in the EMA's Decision (P/0383/2019) of 04/12/2019.

2.2.4. Pitolisant hydrochloride - EMEA-C2-001176-PIP01-11-M03

Bioprojet Pharma; Treatment of narcolepsy

Day 30 letter

Neurology

Summary of committee discussion:

Compliance of the checked measure was confirmed by the PDCO.

2.2.5. Adjupanrix: Purified antigen fractions of inactivated split virion influenza A/VietNam/1194/2004 (H5N1) like strain used (NIBRG-14) / Prepandrix: Purified antigen fractions of inactivated split virion influenza A/Indonesia/05/2005 like strain used (PR8-IBCDC-RG2) - EMEA-C-000160-PIP01-07-M05

GlaxoSmithKline Biologicals SA; Prevention of influenza infection

Day 30 letter

Vaccines

Summary of committee discussion:

The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0299/2019) of 14 August 2019.
The PDCO adopted on 31 January 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/0299/2019) of 14 August 2019.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Entrectinib - EMEA-002096-PIP01-16-M02

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

Day 60 opinion

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/0010/2019 of 4 January 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Ixekizumab - EMEA-001050-PIP01-10-M05

Eli Lilly Nederland B.V.; Treatment of psoriasis

Day 60 opinion

Dermatology

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/0351/2018> of 20/11/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Nemolizumab - EMEA-001624-PIP01-14-M02

Galderma International S.A.; Atopic dermatitis / Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of committee discussion:

The PDCO discussed the applicant’s response after Day 30.
Many of the proposed modifications were not considered acceptable.
The PDCO therefore adopted a negative Opinion on the modification of the agreed PIP.

2.3.4. Alogliptin - EMEA-000496-PIP01-08-M07

Takeda Development Centre Europe Ltd; Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO adopted a favourable Opinion on this Request for Modification of the agreed PIP as set in the Agency's latest decision (P/0097/2019 of 22/03/2019), for Alogliptin (dipeptidyl peptidase-4 inhibitor) for treatment of type 2 diabetes (T2D) during its plenary on 31 January 2020.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Dapagliflozin - EMEA-000694-PIP01-09-M08

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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/0307/2018 of 12/09/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Denosumab - EMEA-000145-PIP02-12-M02

Amgen Europe B.V.; Treatment of osteoporosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO discussed at their January 2020 meeting the replies that the applicant provided to the issues raised at D30. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0058/2016 of 18/3/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Evinacumab - EMEA-002298-PIP01-17-M01

Regeneron Ireland DAC; Treatment of elevated cholesterol

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO discussed the responses of the applicant to the points raised at D30.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0404/2018 of 20/12/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M04

AstraZeneca AB; Hyperkalaemia / Treatment of hyperkalaemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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/0053/2018 of 1 March 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Macimorelin - EMEA-001988-PIP01-16-M01

Aeterna Zentaris GmbH; Diagnosis of growth hormone deficiency

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic

Summary of committee discussion:

A positive Opinion has been adopted by the PDCO on the modification of the agreed PIP as set in the Agency's latest decision (P/0105/2017 of 11/04/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Eluxadoline - EMEA-001579-PIP01-13-M03

Allergan Pharmaceuticals International Limited; Treatment of diarrhoea-predominant irritable bowel syndrome

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

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/0388/2017 of 19/12/2017).

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

2.3.11. Eftrenonacog alfa - Orphan - EMEA-000914-PIP01-10-M05

Swedish Orphan Biovitrum AB (publ); Treatment of hereditary factor IX deficiency

Day 60 opinion

Haematology-Hemostaseology

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/0296/2018 of 12/09/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Vadadustat - EMEA-001944-PIP01-16-M01

Akebia Therapeutics, Inc.; Treatment of anaemia due to chronic disorders

Day 60 opinion

Haematology-Hemostaseology

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/0035/2017 of 31/01/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Avacopan - Orphan - EMEA-002023-PIP01-16-M04

ChemoCentryx Ireland Ltd.; Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

Day 60 opinion

Immunology-Rheumatology-Transplantation

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 completion of Studies 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/0360/2019 of 4 November 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Emapalumab - Orphan - EMEA-002031-PIP01-16-M03

Novimmune BV; Treatment of haemophagocytic lymphohistiocytosis

Day 60 opinion

Immunology-Rheumatology-Transplantation

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/0306/2018 of 12/09/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Bezlotoxumab - EMEA-001645-PIP01-14-M03

Merck Sharp & Dohme (Europe), Inc.; Prevention of recurrence of *Clostridium difficile* infection

Day 60 opinion

Infectious Diseases

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/0387/2017 of 19/12/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Anti-respiratory syncytial virus human IgG1κ monoclonal antibody (MEDI8897) - EMEA-001784-PIP01-15-M02

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

Day 60 opinion

Infectious Diseases

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/0305/2019 of 10 September 2019).

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

2.3.17. Posaconazole - EMEA-000468-PIP02-12-M06

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

Day 60 opinion

Action: For adoption

Infectious Diseases

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0223/2019 of 21 June 2019).

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

2.3.18. Tenofovir alafenamide (as fumarate) - EMEA-001584-PIP01-13-M05

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B

Day 60 opinion

Infectious Diseases

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/0133/2019 of 17 April 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Eculizumab - Orphan - EMEA-000876-PIP03-14-M04

Alexion Europe SAS; Neuromyelitis optica spectrum disorders / Treatment neuromyelitis optica spectrum disorders

Day 60 opinion

Neurology

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/0252/2019 of 16 July 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Eladocagene exuparvovec - Orphan - EMEA-002435-PIP01-18-M01

PTC Therapeutic International Limited; Aromatic L-amino acid decarboxylase (AADC) Deficiency / Treatment of aromatic L-amino acid decarboxylase deficiency

Day 60 opinion

Neurology

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/0310/2019 of 10 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.21. Perampanel - EMEA-000467-PIP01-08-M13

Eisai Europe Limited; Treatment of treatment-resistant epilepsies

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, including the responses to the questions raised during the Day 30 discussion, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0217/2019 of 12 June 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion

2.3.22. Idasanutlin - Orphan - EMEA-001489-PIP01-13-M02

Roche Registration GmbH; Treatment of acute lymphoblastic leukaemia / Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this modification request acknowledging the additional delay of start of initiation of study being requested.
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/0366/2018 of 07/12/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.23. Risdiplam - Orphan - EMEA-002070-PIP01-16-M04

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 60 opinion

Action: For adoption

Neurology

Summary of committee discussion:

During its plenary on 31 January 2020, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0349/2019 of 16/09/2019), for risdiplam for the treatment of spinal muscular atrophy in patients from birth to less than 18 years of age.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.24. Sonidegib - EMEA-000880-PIP02-11-M04

Sun Pharmaceutical Industries Europe B.V.;Treatment of medulloblastoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's views expressed at D30 were endorsed.
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/0018/2016 of 29 January 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.25. Burosumab - Orphan - EMEA-001659-PIP01-15-M04

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

Day 60 opinion

Other

Summary of committee discussion:

The PDCO confirmed the outcome of the Day 30 discussion and agreed with the requested delay of a Study.
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/0007/2018 of 30/01/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.26. Bupivacaine / meloxicam (HTX-011) - EMEA-002246-PIP01-17-M01

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

Day 60 opinion

Pain

Summary of committee discussion:

The PDCO reviewed the additional information received since Day 30 and concluded that the request to change is well justified and agreeable, and the proposed measures are acceptable. Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO therefore considered that the proposed changes could be accepted and adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.27. Live, attenuated, chimeric dengue virus, serotype 1 / Live, attenuated, chimeric dengue virus, serotype 2 / Live, attenuated, chimeric dengue virus, serotype 3 / Live, attenuated, chimeric dengue virus, serotype 4 - EMEA-001545-PIP01-13-M02

Sanofi Pasteur; Prevention of dengue

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/0174/2015 of 07/08/2015

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

2.3.28. Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain, A/H3N2-like strain, B-like strain (Victoria lineage) and B-like strain (Yamagata lineage) - EMEA-002359-PIP01-18-M01

Sanofi Pasteur; Prevention of influenza infection

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/0023/2019 of 4 January 2019).

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

2.3.29. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) [QIVc]- EMEA-002068-PIP01-16-M03

Seqirus UK Limited; Influenza / Prevention of influenza

Day 60 opinion

Vaccines

Summary of committee discussion:

The PDCO re-discussed the requested modification taking into account the clarifications provided by the applicant after the D30 discussion.

All pending issues were considered solved.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0387/2018 of 06 December 2018).

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

2.4. Opinions on Re-examinations

2.4.1. Dupilumab - EMEA-001501-PIP04-19

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 30 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

An oral explanation meeting (OEM) took place on 29 January 2020 to address the outstanding issues remaining in the procedure. The Committee issued an opinion implementing the changes accepted with the revision.

2.4.2. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M03

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia / For the treatment of relapsed or refractory B cell precursor acute lymphoblastic leukaemia

Day 30 opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

An Oral Explanation with the applicant took place on 29th January 2020. During the oral explanation the applicant further discussed the reasoning behind the request to use the

primary endpoint.

Looking at the design of the trial and the complex treatment landscape, and in light of the additional justifications/changes provided by the applicant, the PDCO revised its opinion.

The Committee issued an opinion implementing the changes accepted with the revision.

2.4.3. Vosoritide - Orphan - EMEA-002033-PIP01-16-M01

BioMarin International Limited; Treatment of achondroplasia

Day 30 opinion

Other

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for the re-examination of the adopted opinion, the PDCO acknowledged the editing error and considered that the proposed changes should be accepted.
The PDCO therefore revised its previous opinion and implemented the change proposed in the re-examination request. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.4.4. (R)-1-(3-(aminomethyl) phenyl)-N-(5-((3 cyanophenyl)(cyclopropylmethylamino)methyl)-2-fluorophenyl)-3-(trifluoromethyl)-1H-pyrazole-5-carboxamide dihydrochloride - Orphan - EMEA-002449-PIP02-18

BioCryst UK; Treatment of hereditary angioedema

Day 30 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO discussed the re-examination request of the PIP at their January 2020 meeting. The PDCO agreed with the proposed re-examination requested to change the deferral status of the clinical studies of the PIP in order not to delay the initial MAA submission for adults.

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of opinions

No items

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. Selpercatinib - EMEA-C1-002544-PIP01-18

Eli Lilly and Company; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 1 Letter

Oncology

2.7.2. Mometasone (furoate) / Indacaterol (acetate) - EMEA-C3-001217-PIP01-11-M05

Novartis Europharm Ltd; Treatment of asthma

Day 1 letter

Pneumology – Allergology

2.7.3. Recombinant varicella zoster virus (VZV) glycoprotein E - EMEA-C1-001426-PIP01-13-M02

GlaxoSmithKline Biologicals SA; Prevention of varicella zoster virus (VZV) reactivation / Prevention of herpes zoster in immunocompromised subjects

Day 1 letter

Vaccines

2.7.4. Idecabtagene vicleucel - EMEA-C1-002369-PIP01-18-M01

Celgene Europe B.V.; Treatment of mature B-cell neoplasms

Day 30 letter

Oncology

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. Lenadogene nolparvovec - Orphan - EMEA-001992-PIP02-16

GenSight-Biologics; Leber Hereditary Optic Neuropathy (LHON)

Day 90 discussion

3.1.2. Dihomo-γ-linolenic acid (DGLA) - EMEA-002364-PIP03-19

Treatment of atopic dermatitis / Treatment of moderate to severe atopic dermatitis

Day 90 discussion

Dermatology

3.1.3. EMEA-002327-PIP02-19

Prevention of oral mucositis

Day 90 discussion

Dermatology

3.1.4. EMEA-002582-PIP01-19

Treatment of chronic spontaneous urticaria

Day 90 discussion

Dermatology

3.1.5. Tezepelumab - EMEA-002579-PIP01-18

Atopic dermatitis

Day 90 discussion

Dermatology

3.1.6. Ladarixin - EMEA-002642-PIP01-19

Treatment of type 1 diabetes / Treatment of new-onset type 1 diabetes mellitus

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. Alpha1-proteinase inhibitor EMEA-001312-PIP02-19

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

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.8. Polymyxin B - EMEA-002595-PIP01-19

Treatment of infections due to aerobic Gram-negative bacteria

Day 90 discussion

Infectious Diseases

3.1.9. 5-[[4-[2-[5-(1-Hydroxyethyl)-2-pyridinyl]ethoxy]phenyl]methyl]-2,4-thiazolidinedione hydrochloride - Orphan - EMEA-002106-PIP01-16

Minoryx Therapeutics S.L.; Adrenoleukodystrophy (ALD) / Treatment of adrenomyeloneuropathy (AMN)

Day 90 discussion

Neurology

3.1.10. 1-[4-bromo-5-[1-ethyl-7-(methylamino)-2-oxo-1,2-dihydro-1,6-naphthyridin-3-yl]-2-fluorophenyl]-3-phenylurea - Orphan - EMEA-002526-PIP02-19

Deciphera Pharmaceuticals LLC; Treatment of gastrointestinal stromal tumours

Day 90 discussion

Oncology

3.1.11. Bintrafusp alfa - Orphan - EMEA-002586-PIP01-19

Merck Europe B.V.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 3 to <18 years of age with solid malignant tumors

Day 90 discussion

Oncology

3.1.12. Pracinostat - Orphan - EMEA-002567-PIP01-19

Helsinn Birex Pharmaceuticals limited; Acute myeloid leukemia

Day 90 discussion

Oncology

3.1.13. Immunoglobulin G4 - EMEA-002290-PIP01-17

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / In combination with nivolumab for the treatment of malignant solid tumours in paediatric patients from birth to less than 18 years old.

Day 90 discussion

Oncology

3.1.14. 2-[[2-ethyl-6-[4-[2-(3-hydroxyazetidin-1-yl)-2-oxoethyl]piperazin-1-yl]-8-methylimidazo[1,2-a]pyridin-3-yl](methyl)amino]-4-(4-fluorophenyl)-1,3-thiazole-5-carbonitrile - Orphan - EMEA-002333-PIP02-19

Galapagos NV; Treatment of idiopathic pulmonary fribrosis, Treatment of interstitial lung disease with fibrosis in children

Day 90 discussion

Pneumology - Allergology

3.1.15. Tosatoxumab - Orphan - EMEA-002506-PIP01-18

Aridis Pharmaceuticals Inc.; Pneumonia caused by *Staphylococcus aureus*

Day 90 discussion

Pneumology - Allergology

3.1.16. EMEA-002121-PIP03-19

Treatment of insomnia / Treatment of insomnia in children with comorbid neurodevelopmental and psychiatric disorders

Day 90 discussion

Psychiatry

3.1.17. Multivalent Pneumococcal vaccine EMEA-002641-PIP01-19

Prevention of pneumococcal disease caused by *S. pneumoniae* / For the active immunisation for the prevention of invasive pneumococcal diseases (IPD) caused by *S. pneumoniae* in infants, children and adolescents from 6 weeks to < 18 years of age

Day 90 discussion

Vaccines

3.1.18. 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

Disease caused by *Streptococcus pneumoniae*

Day 90 discussion

Vaccines

3.1.19. Temozolomide - EMEA-002634-PIP01-19

Treatment of malignant glioma / Children from the age of three years and adolescent patients with malignant glioma, such as glioblastoma multiform or anaplastic astrocytoma, showing recurrence or progression after standard therapy, who have difficulty swallowing

Day 90 discussion

Oncology

3.1.20. Cenobamate - EMEA-002563-PIP02-19

Treatment of epilepsy

Day 90 discussion

Neurology

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

CUTISS AG; Treatment of burns

Day 60 discussion

Dermatology

3.1.22. EMEA-002689-PIP01-19

Achondroplasia

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.23. Lonapegsomatropin - EMEA-002692-PIP01-19

Growth hormone deficiency

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. Cilofexor - Orphan - EMEA-002554-PIP02-19

Gilead Sciences International Ltd.; Treatment of primary sclerosing cholangitis (PSC) (DB96.2)

Day 60 discussion

Gastroenterology-Hepatology

3.1.25. Odevixibat - Orphan - EMEA-002054-PIP02-18

Albireo AB; Biliary atresia

Day 60 discussion

Gastroenterology-Hepatology

3.1.26. 2-(2-{[2-(1H-benzimidazol-2-yl)ethyl]amino}ethyl)-N-[(3-fluoropyridin-2-yl)methyl]-1,3-oxazole-4-carboxamide trihydrochloride - Orphan - EMEA-002704-PIP01-19

Vifor France; Beta-thalassaemia / Treatment of beta-thalassaemia

Day 60 discussion

Haematology-Hemostaseology

3.1.27. Efgartigimod alfa - EMEA-002597-PIP02-19

Treatment of immune thrombocytopenia

Day 60 discussion

Haematology-Hemostaseology

3.1.28. Marstacimab - Orphan - EMEA-002285-PIP02-19

Pfizer Europe MA EEIG; Treatment of haemophilia B / Treatment of haemophilia A / Prophylaxis of bleeding in haemophilia B / Prophylaxis of bleeding in haemophilia A

Day 60 discussion

Haematology-Hemostaseology

3.1.29. Mitapivat - EMEA-002684-PIP01-19

Pyruvate kinase deficiency / Treatment of paediatric patients with pyruvate kinase deficiency

Day 60 discussion

Haematology-Hemostaseology

3.1.30. 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

Day 60 discussion

Immunology-Rheumatology-Transplantation

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

Treatment of systemic lupus erythematosus (SLE) / Treatment of children and adolescents ≥7 years to <18 years of age with active SLE despite standard therapy

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.32. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19

Principia Biopharma, Inc.; Immune thrombocytopenia / Treatment of immune thrombocytopenia

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.33. Artesunate - EMEA-002710-PIP01-19

Malaria

Day 60 discussion

Infectious Diseases

3.1.34. EMEA-002694-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 60 discussion

Infectious Diseases

3.1.35. EMEA-002693-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 60 discussion

Infectious Diseases

3.1.36. Pritelivir - EMEA-002180-PIP02-19

Treatment of *herpes simplex* virus disease

Day 60 discussion

Infectious Diseases

3.1.37. Soticlestat - EMEA-002572-PIP02-19

Dravet Syndrome / Lennox-Gastaut syndrome / Treatment of seizures associated with Dravet Syndrome / Treatment of seizures associated with Lennox-Gastaut syndrome

Day 60 discussion

Neurology

3.1.38. Mosunetuzumab - EMEA-002524-PIP02-19

Treatment of mature B-cell neoplasms / Treatment of children from 6 months to less than 18 years of age with relapsed or refractory high-grade mature B-cell non-Hodgkin lymphoma (B-NHL), including Burkitt lymphoma (BL), Burkitt leukaemia (mature B-cell acute lymphoblastic leukaemia FAB L3; B-AL) and diffuse large B-cell lymphoma (DLBCL)

Day 60 discussion

Oncology

3.1.39. Recombinant anti-human CD20 and anti-human CD3 monoclonal antibody - EMEA-002648-PIP01-19

Treatment of mature B-cell neoplasms / Treatment of children from 6 months to less than 18 years of age with relapsed or refractory high-grade mature B-cell non-Hodgkin lymphoma (B-NHL), including Burkitt lymphoma (BL), Burkitt leukaemia (mature B-cell acute lymphoblastic leukaemia FAB L3; B-AL), and diffuse large B-cell lymphoma (DLBCL)

Day 60 discussion

Oncology

3.1.40. Anti-neonatal Fc receptor human monoclonal antibody - Orphan - EMEA-002559-PIP03-19

Momenta Pharmaceuticals, Inc.; Autoimmune haemolytic aneamia

Day 60 discussion

Other

3.1.41. Urea / glycerol - EMEA-002511-PIP02-19

Treatment of dry skin

Day 30 discussion

Dermatology

3.1.42. Lerodalcibep - EMEA-002720-PIP01-19

Treatment of elevated cholesterol / Treatment of elevated low-density lipoprotein cholesterol (LDL-C) in children from 6 to less than 18 years of age with heterozygous familial hypercholesterolaemia (HeFH) or with homozygous familial hypercholesterolaemia (HoFH)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.43. 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 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.44. Garadacimab - EMEA-002726-PIP01-19

Hereditary angioedema attacks (HAE)

Day 30 discussion

Haematology-Hemostaseology

3.1.45. Recombinant adeno-associated viral vector serotype 6 encoding the B-domain-deleted human factor VIII - Orphan - EMEA-002724-PIP01-19

Pfizer Europe MA EEIG; Treatment of haemophilia A (congenital FVIII deficiency)

Day 30 discussion

Haematology-Hemostaseology

3.1.46. Alpha1-proteinase inhibitor - EMEA-001312-PIP03-19

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

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 30 discussion

Infectious Diseases

3.1.48. Vonoprazan - EMEA-002703-PIP01-19

Helicobacter pylori / Reflux oesophagitis / Treatment of erosive reflux oesophagitis and relief of heartburn / Eradication of *Helicobacter pylori* (H. pylori) concurrently given with appropriate antibiotic therapy

Day 30 discussion

Infectious Diseases / Gastroenterology-Hepatology

3.1.49. Natalizumab - EMEA-001095-PIP03-19

Treatment of multiple sclerosis (MS) / Treatment of relapsing-remitting multiple sclerosis (MS)

Day 30 discussion

Neurology

3.1.50. Autologous CD4 and CD8 T-cells that have been transduced with a self-inactivating lentiviral vector expressing an affinity enhanced NY-ESO-1 specific T-Cell Receptor - Orphan - EMEA-002476-PIP02-19

GlaxoSmithKline Trading Services Limited; Soft tissue sarcoma

Day 30 discussion

Oncology

3.1.51. Efbemalenograstim alfa - EMEA-002507-PIP02-19

Prevention of chemotherapy-induced neutropenia and 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 30 discussion

Oncology

3.1.52. Lazertinib - EMEA-002725-PIP01-19

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 30 discussion

Oncology

3.1.53. Relatlimab / nivolumab - EMEA-002727-PIP01-19

Treatment of melanoma / Relatlimab/nivolumab fixed dose combination for treatment of unresectable or metastatic melanoma in patients from 12 to 18 years

Day 30 discussion

Oncology

3.1.54. 4-{(2S,4S)-4-Ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid hydrochloride(1/1) - Orphan - EMEA-002705-PIP01-19

Novartis Europharm Limited; C3 Glomerulopathy

Day 30 discussion

Other

3.1.55. EMEA-002705-PIP02-19

IgA nephropathy

Day 30 discussion

Other

3.1.56. Alpelisib - EMEA-002016-PIP03-19

PIK3CA related overgrowth spectrum (PROS)

Day 30 discussion

Other

3.1.57. Lanadelumab - Orphan - EMEA-001864-PIP02-19

Shire Pharmaceuticals Ireland Limited (a Takeda company); Prevention of acquired angioedema attacks (AAE) / Prevention of attacks of acquired angioedema (AAE) due to C1-INH deficiency

Day 30 discussion

Other

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

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

Day 30 discussion

Other

3.1.59. Crinecerfont - Orphan - EMEA-002700-PIP01-19

Neurocrine Therapeutics Ltd; Treatment of congenital adrenal hyperplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.60. EMEA-002714-PIP01-19

Treatment of mature B-cell neoplasms

Day 30 discussion

Oncology

3.1.61. EMEA-002674-PIP01-19

Treatment of acne vulgaris

Day 30 discussion

Dermatology

3.1.62. Lebrikizumab - EMEA-002536-PIP01-18

Treatment of atopic dermatitis

Day 90 discussion

Dermatology

3.1.63. EMEA-002619-PIP01-19

Treatment of renal tumors

Day 30 discussion

Oncology

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

Genzyme Europe B.V.; GM2 gangliosidosis, Other gangliosidosis / GM1, GM3, Defects in

glycoprotein degradation, Sialidosis / The planned indication for venglustat is 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 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

3.1.65. Romiplostim - EMEA-000653-PIP02-19

Secondary thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in children <18 years of age with solid tumours

Day 30 discussion

Oncology / Haematology-Hemostaseology

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. Lubiprostone - EMEA-C-000245-PIP01-08-M06

Sucampo AG; Chronic Idiopathic Constipation

Day 30 discussion

Gastroenterology-Hepatology

3.2.2. Avatrombopag (maleate) - EMEA-C1-001136-PIP01-11-M01

Dova Pharmaceuticals Ireland Ltd.; Treatment of idiopathic thrombocytopenic purpura

Day 30 discussion

Haematology-Hemostaseology

3.2.3. Palovarotene - EMEA-C1-001662-PIP01-14-M02

Ipsen Pharma; Fibrodysplasia Ossificans Progressiva

Day 30 discussion

Other

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Tralokinumab - EMEA-001900-PIP02-17-M03

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.2. Mirikizumab - EMEA-002208-PIP01-17-M01

Eli Lilly and Company; Treatment of psoriasis / Treatment of Crohn's disease / Treatment of ulcerative colitis / Treatment of moderate to severely active ulcerative colitis / Treatment of moderate-to-severe plaque psoriasis / Treatment of moderate to severely active Crohn's disease

Day 30 discussion

Dermatology / Gastroenterology-Hepatology

3.3.3. Semaglutide - EMEA-001441-PIP01-13-M03

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

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Linaclotide - EMEA-000927-PIP01-10-M05

Allergan Pharmaceuticals International Limited; Functional constipation in children

Day 30 discussion

Gastroenterology-Hepatology

3.3.5. Boceprevir - EMEA-000583-PIP01-09-M08

Merck Sharp & Dohme (Europe), Inc; Treatment of chronic hepatitis C

Day 30 discussion

Infectious Diseases

3.3.6. Tenofovir disoproxil (as fumarate) - EMEA-000533-PIP01-08-M08

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

3.3.7. Galcanezumab - EMEA-001860-PIP03-16-M04

Eli Lilly and Company Limited; Prevention of migraine headaches

Day 30 discussion

Neurology

3.3.8. Lasmiditan - EMEA-002166-PIP01-17-M03

Eli Lilly and Company Limited; Migraine with and without aura

Day 30 discussion

Neurology

3.3.9. Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti CD19 CD28/CD3-zeta chimeric antigen receptor and cultured - Orphan - EMEA-001862-PIP01-15-M02

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia / Treatment of relapsed or refractory B-precursor acute lymphoblastic leukaemia (r/r ALL)

Day 30 discussion

Oncology

3.3.10. Axicabtagene ciloleucel - Orphan - EMEA-002010-PIP01-16-M02

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms / Treatment of children less than 18 years of age and weighing at least 6 kg with relapsed or refractory aggressive B-cell non-hodgkin lymphoma (NHL) who are ineligible for autologous stem cell transplant

Day 30 discussion

Oncology

3.3.11. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M03

Amgen Europe B.V.; Treatment of Acute Lymphoblastic Leukaemia / Treatment of children previously untreated with high-risk first relapse of B-precursor acute lymphoblastic leukaemia

Day 30 discussion

Oncology

3.3.12. Copanlisib - Orphan - EMEA-001757-PIP02-15-M01

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of children with a relapsed or

refractory neuroblastoma, Ewing sarcoma, osteosarcoma or rhabdomyosarcoma including at first relapse, in combination with chemotherapy

Day 30 discussion

Oncology

3.3.13. Clostridium botulinum neurotoxin type A (150 kD), free from complexing proteins - EMEA-001039-PIP02-12-M04

Merz Pharmaceuticals GmbH; Treatment of sialorrhea / Treatment of chronic troublesome sialorrhea associated with neurological conditions (e.g. cerebral palsy, traumatic brain injury) and/or intellectual disability in children and adolescents aged 2 – 17 years

Day 30 discussion

Ophthalmology / Neurology

3.3.14. Palovarotene - Orphan - EMEA-001662-PIP01-14-M03

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 30 discussion

Other

3.3.15. Ad26.ZEBOV - EMEA-002307-PIP01-17-M01

Janssen Cilag International NV; Prevention of ebola virus disease (EVD)/ Prevention of EVD in children aged ≥1 year

Day 30 discussion

Vaccines

3.3.16. MVA-BN-Filo - EMEA-002308-PIP01-17-M01

Janssen Cilag International NV; Prevention of ebola virus disease (EVD) / Prevention of EVD in children aged ≥1 year

Day 30 discussion

Vaccines

3.3.17. Regorafenib - EMEA-001178-PIP01-11-M05

Bayer AG; Treatment of all conditions contained in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of paediatric patients with a solid malignant tumour(s) integrated with anti-cancer therapy

Day 30 discussion

Oncology

3.3.18. Etrolizumab - EMEA-001434-PIP01-13-M03

Roche Registration GmbH; Treatment of ulcerative colitis / Treatment of Crohn's disease / Treatment of moderately to severely active Crohn's disease / Treatment of moderately to severely active ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.19. Elafibranor - EMEA-001857-PIP01-15-M01

GENFIT SA; Non-alcoholic fatty liver disease (NAFLD) including non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Gastroenterology-Hepatology

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 letters of intent received for submission of applications with start of procedure 02 March 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:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

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

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. Pentosan polysulfate - EMEA-18-2019

Paradigm Biopharmaceuticals (Ireland) Ltd; All classes of medicinal products for the treatment primary and secondary osteoarthrosis/ treatment of osteoarthritis of the knee associated with pain and bone marrow lesions

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: juvenile idiopathic arthritis, mucopolysaccaroidosis, sickle cell disease, Fabry disease, Gaucher disease.

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

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

7.1.1. Macitentan - EMEA-001032-PIP01-10

Treatment of pulmonary arterial hypertension (PAH)

Proposed indication: Treatment of portopulmonary hypertension (POPH)

Summary of committee discussion:

The PDCO is of the view that the proposed indication "treatment of portopulmonary hypertension (POPH)" or "treatment of pulmonary arterial hypertension (PAH) associated with portal hypertension", falls under the scope of the above mentioned Decision, as the indication is considered to be covered by the condition "treatment of pulmonary arterial hypertension (PAH)" listed in the Agency Decision.

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

No items

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 3 medicinal products with recommended paediatric indications adopted in December 2019. These included Dificlir (fidaxomicin), Sirturo (bedaquiline) and Stelara (ustekinumab).

The CHMP also recommended approval of a new pharmaceutical form: Dificlir, granules for oral suspension (40 mg/ml) for use in children with a body weight of at least 12.5 kg.

The list of procedures with paediatric indications to be evaluated by the CHMP, starting in December 2019, was presented to the PDCO members.

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

CHMP/PDCO joint session

Summary of committee discussion:

The relevant members of the PDCO/CHMP attended the PDCO/CHMP joint meeting to discuss the related topics.

9.2.3. Committee for Advanced Therapies (CAT)

Update on the reflection paper on haemophilia

Summary of committee discussion:

The PDCO endorsed the comments provided on the reflection paper on haemophilia. The current version will be sent to the CAT secretariat for consideration.

9.2.4. Pharmacovigilance Risk Assessment Committee (PRAC)

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.

9.3.3. Scientific Advice Working Party: nominations of PDCO members to SAWP

Summary of committee discussion:

A presentation was given on the re-examination of the PDCO member representatives at the SAWP. The current challenges encountered relate to the high number of scientific advices received in 2019, a trend which has been on the increase since 2017 and is expected to be high in 2020.

9.3.4. Meeting Summary from the Annual Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) with all eligible organisations - 20 November 2019

Summary of committee discussion:

The meeting summary from the PCWP/HCPWP was noted for information.

9.3.5. Agenda for the Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) on 03-04 March 2020

Summary of committee discussion:

The agenda for the PCWP/HCPWP meeting in March 2020 was noted for information.

9.4. Cooperation within the EU regulatory network

9.4.1. CTFG: Outcome of TC

CTFG Chair: Ann Marie Janson Lang

Summary of committee discussion:

A summary of the teleconference between some PDCO members and some members of the CTFG was presented to the committee.

9.5. Cooperation with International Regulators

9.5.1. Report from the Paediatric Cluster Teleconference

Summary of committee discussion:

The Committee was informed about the discussions at the Paediatric Cluster teleconference.

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

No items

9.7. PDCO work plan

No items

9.8. Planning and reporting

No items

10. Any other business

No items

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The group discussed paediatric investigation plans under assessment and the main conclusions of the 5th Paediatric Strategy Forum.

11.1.2. Neonatology

Summary of committee discussion:

The breakout session discussed planned activities for the upcoming year.

11.1.3. Inventory

Summary of committee discussion:

The inventory group discussed the current template for the scientific document (parts B to F) of the submission package and how to best gather data on unmet needs from applicants.

The Chair thanked all participants, especially the UK members, Ms Angeliki Siapkara and Ms Martina Riegl, for their involvement in the Committee activities. The professionalism as well as commitment of UK participants contributed greatly to the functioning and development of the PDCO, and their collaboration has been highly appreciated.

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 DD Month YEAR meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium	No participation in discussion, final deliberations and voting on:	- 3.2.6 - Adjupanrix: Purified antigen fractions of inactivated split virion influenza A/VietNam/1194/2004 (H5N1) like strain used (NIBRG-14) / Prepandrix: Purified antigen fractions of inactivated split virion influenza A/Indonesia/05/2005 like strain I13used (PR8-IBCDC-RG2) - EMEA-C-000160-PIP01-07-M05; - 3.1.52. -Autologous CD4 and CD8 T-cells that have been transduced with a self-inactivating lentiviral vector expressing an affinity enhanced NY-ESO-1 specific T-Cell Receptor - Orphan - EMEA-002476-PIP02 -19
Karl-Heinz Huemer	Member	Austria	No interests declared	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	N/A
Georgios Savva	Member	Cyprus	No interests declared	
Petra Dominikova	Alternate	Czech Republic	No interests declared	
Kirstine Moll Harboe	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Ann Marie Totterman	Member	Finland	No interests declared	
Pia Annunen	Alternate	Finland		-3.1.55. Relatlimab / nivolumab - EMEA-002727-PIP01-19;

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
				- 3.1.13. Immunoglobulin G4, EMEA-002290-PIP01-17
Sylvie Benchetrit	Member	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	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:	- 2.3.16. Nirsevimab - anti-respiratory syncytial virus human IgG1κ monoclonal antibody (MEDI8897) - EMEA-001784-PIP01-15-M02
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 interests declared	
Maria Jesus	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
Fernández Cortizo				
Ninna Gullberg	Member	Sweden	No interests declared	
Eva Agurell	Alternate	Sweden	No interests declared	
Angeliki Siapkara	Member	United Kingdom	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in discussion, final deliberations and voting on:	- 2.3.16. Nirsevimab - anti-respiratory syncytial virus human IgG1κ monoclonal antibody (MEDI8897) - EMEA-001784-PIP01-15-M02
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	N/A
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No participation in discussion, final deliberations and voting on:	- 2.3.16. Nirsevimab - anti-respiratory syncytial virus human IgG1κ monoclonal antibody (MEDI8897) - EMEA-001784-PIP01-15-M02; - 3.2.4. Pitolisant hydrochloride - EMEA-C2-001176-PIP01-11-M03
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Paola Baiardi	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	N/A
Michal Odermarsky	Member	Patients' Organisation Representative	No participation in final deliberations and voting on:	- 7.1.1. Macitentan - EMEA-001032-PIP01-10
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Violeta Stoyanova-Beninska	Expert - in person*	COMP Chair	No interests declared	
Maria Estela Moreno Martin	Expert - in person*	Spain	No interests declared	
Angeliki Roboti	Expert - via telephone*	Greece	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Konstantinos Gkirtis	Expert - via telephone*	Greece	No interests declared	
Ann Marie Jason Lang	Expert - via telephone*	CTFG Chair	No interests declared	
Beatrice Panico	Expert - via telephone*	CTFG member	No interests declared	
Elena Prokofyeva	Expert - via telephone*	CTFG member	No interests declared	
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/