EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 August 2019
EMA/PDCO/417613/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft minutes of the meeting on 23-26 July 2019

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

Disclaimers

Some of the information contained in these 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 that no restriction in the involvement of meeting participants in upcoming discussions was identified.

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.

1.2. Adoption of agenda

PDCO agenda for 23-26 July 2019 was adopted and published on EMA website.

1.3. Adoption of the minutes

PDCO minutes for 25-28 June 2019 were adopted and will be published on the EMA website.

2. Opinions

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

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 120 Opinion

Dermatology

Summary of Committee discussion:

A positive Opinion for a PIP covering the entire paediatric age-range from birth to less than 18 years for the treatment of epidermolysis bullosa was adopted.

2.1.2. Trifarotene cream HE1 - EMEA-001492-PIP02-18

Premier Research Group SLU; Lamellar ichthyosis / Treatment of lamellar ichthyosis

Day 120 Opinion

Dermatology

Summary of Committee discussion:

A positive Opinion was adopted.

2.1.3. Levonorgestrel - EMEA-002474-PIP02-18

Chemo Research, S.L.; Contraception / Prevention of pregnancy

Day 120 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO re-discussed the application taking into consideration the additional information received on the issues previously identified at Day 90. The PDCO considered that the Applicant's proposal could be accepted. Overall, a positive Opinion was adopted.

2.1.4. Tirzepatide - EMEA-002360-PIP01-18

Eli Lilly and Company; Type 2 diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 120 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

At the July 2019 meeting, the PDCO discussed the clarification received by the Applicant. In conclusion the PDCO agreed on a positive Opinion for tirzepatide in the treatment of type 2 diabetes mellitus with a deferral.

2.1.5. PEGylated-fibroblast growth factor 21 - EMEA-002448-PIP01-18

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

Day 120 Opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

The Applicant's response to the Day 90 issue was considered acceptable and a positive Opinion was adopted.

2.1.6. Liposomal ciclosporin A (L-CsA) – Orphan – EMEA-002344-PIP02-18

Breath Therapeutics GmbH; Treatment of bronchiolitis obliterans syndrome (BOS)

Day 120 Opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The Committee confirmed the outcome of the Day 90 discussion.
In conclusion, the PDCO adopted a positive Opinion for liposomal ciclosporin A (L-CsA) for the treatment of bronchiolitis obliterans syndrome (BOS).

2.1.7. Baloxavir marboxil - EMEA-002440-PIP01-18

Roche Registration GmbH; Prevention of influenza / Treatment of influenza / Treatment of influenza type A/B in otherwise healthy, high risk and hospitalised patients / Prevention (post-exposure prophylaxis) of influenza type A/B. Reduction of transmission of influenza type A/B

Day 120 Opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO reviewed the Applicant's additional information provided between Day 90 and Day 120.
The PDCO issued a positive Opinion on this PIP containing two conditions: 'Prevention of influenza infection' and 'Treatment of influenza infection'.

2.1.8. Equine Immunoglobulin F(ab')2 fragments targeting Shiga toxin - Orphan - EMEA-002444-PIP02-18

Chemo Research, S.L.; Prevention of haemolytic uremic syndrome

Day 120 Opinion

Infectious Diseases

Summary of Committee discussion:

During its July 2019 plenary meeting the PDCO adopted a positive Opinion for (equine immunoglobulin F(ab')2 fragments targeting Shiga toxin 1+2) for the 'Prevention of haemolytic uremic syndrome' (induced by Shiga-Toxin Producing Escherichia Coli), for children from birth to less than 18 years of age (EMEA-002444-PIP02-18).

2.1.9. Eptinezumab - EMEA-002243-PIP01-17

Alder BioPharmaceuticals Limited; Prevention of migraine headaches / Prophylaxis of migraine

Day 120 Opinion

Neurology

Summary of Committee discussion:

At the July 2019 meeting the PDCO noted the last clarifications provided by the Applicant and agreed a positive Opinion for the PIP for eptinezumab in 'Prevention of migraine headaches' with a deferral.

2.1.10. Fosmetpantotenate - Orphan - EMEA-002036-PIP01-16

Retrophin Europe Limited; Treatment of pantothenate kinase associated neurodegeneration (PKAN)

Day 120 Opinion

Neurology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the conclusions from Day 90.
Overall, a positive Opinion has been adopted.

2.1.11. Bempegaldesleukin - EMEA-002492-PIP01-18

Nektar Therapeutics; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoetic, and lymphoid tissue) / In combination with nivolumab (or with nivolumab and ipilimumab) for the treatment of a relapsed or refractory paediatric malignant solid tumour in paediatric patients less than 18 years old / In combination with nivolumab (or with nivolumab and ipilimumab) for the treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old

Day 120 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this procedure at Day 120 during the July 2019 plenary meeting. The PDCO was informed that the recommended international non-proprietary name for this product has been published and it is bempegaldesleukin. The PDCO Opinion will include this name.
Taking the above into consideration the PDCO adopted a positive Opinion at Day 120.

2.1.12. Niraparib (as tosylate monohydrate) - Orphan - EMEA-002268-PIP02-18

Tesaro UK Ltd; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / The combination therapy is for the treatment of patients ≥6 months to <18 years of age who have been diagnosed with recurrent solid tumours that exhibit a breast cancer susceptibility gene (BRCA)ness mutational signature (mutational signature 3)

Day 120 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this procedure in line with the comments made at Day 90. Overall, a positive Opinion was adopted.

2.1.13. Pegvorhyaluronidase alfa - Orphan - EMEA-001883-PIP03-17

Halozyme Inc; Treatment of solid malignant tumours (except central nervous system tumours, haematopoietic and lymphoid tissue tumours) / Pegvorhyaluronidase alfa is indicated in combination with cytotoxic cancer therapies for the treatment of paediatric patients aged from birth to less than 18 years with relapsed or refractory solid tumours that accumulate high levels of hyaluronan.

Day 120 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed the proposed development for pegvorhyaluronidase alfa In conclusion, the PDCO recommends granting a paediatric investigation plan for pegvorhyaluronidase alfa for all subsets of the paediatric population (from birth to less than 18 years of age) with a deferral in the condition 'treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue neoplasms)'.

2.1.14. Dostarlimab - EMEA-002463-PIP01-18

Tesaro UK Ltd; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / The combination therapy is for the treatment of patients ≥6 months to <18 years of age who have been diagnosed with recurrent solid tumours that exhibit a breast cancer susceptibility gene (BRCA)ness mutational signature (mutational signature 3).

Day 120 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this procedure in line with the comments made at Day 90. The Committee noted the additional information and justifications received.
Overall, a positive Opinion was adopted.

2.1.15. Nebivolol / zofenopril - EMEA-002593-PIP01-19

Piero Rijli; Treatment of essential hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and the discussions at the Paediatric Committee, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for zofenopril / nebivolol for all subsets of the paediatric population (0 to less than 18 years of age) in the condition 'Treatment of hypertension', on the grounds of lack of significant therapeutic benefit over existing treatments.

2.1.16. Ramipril / rosuvastatin - EMEA-002569-PIP01-19

Egis Pharmaceuticals PLC; Treatment of cardiovascular disease

Day 60 Opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and the discussions at the Paediatric Committee, the PDCO agrees with the Applicant's request for a waiver. The PDCO recommends granting a waiver for rosuvastatin / ramipril for all subsets of the paediatric population (0 to less than 18 years of age) in the conditions 'Treatment of hypertension', 'Treatment of lipid metabolism disorders' and 'Prevention of cardiovascular events' on the grounds of lack of significant therapeutic benefit over existing treatments.

2.1.17. Belantamab mafodotin - Orphan - EMEA-002468-PIP04-19

GlaxoSmithKline Trading Services; Treatment of multiple myeloma

Day 60 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this procedure at Day 60 at the July 2019 plenary and confirmed all the conclusions reached at Day 30.
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 belantamab mafodotin for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Treatment of multiple myeloma'.
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. Infigratinib - EMEA-002594-PIP01-19

QED THERAPEUTICS; Cholangiocarcinoma

Day 60 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the conclusions at Day 30.
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 infigratinib for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Treatment of cholangiocarcinoma'.
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. Obidoxime chloride / atropine sulfate - EMEA-002570-PIP01-19

Emergent Netherlands B.V.; Organophosphate nerve agent poisonings

Day 60 Opinion

Other

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 atropine (sulfate)/ obidoxime (chloride) for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Treatment of organophosphates poisoning'. The ground of the waiver is a 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.

2.1.20. Fexapotide triflutate - EMEA-002598-PIP01-19

FGK Representative Service GmbH; Treatment of patients with benign prostatic hyperplasia (BPH)

Day 60 Opinion

Uro-nephrology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the conclusion at Day 30.
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 fexapotide triflutate for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of patients with benign prostatic hyperplasia (BPH).
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. Recombinant hepatitis B vaccine - EMEA-002157-PIP01-17

VBI Vaccines Inc.; Prevention of infection of hepatitis B virus / Secondary immunization of non-responder (anti-HBs levels< 10 mIU/mL) or hypo-responder (anti-HBs levels 10-99 mIU/mL) children age 2-18 years old to prior immunization against hepatitis B virus (HBV) infection / Primary active immunization of children from birth to 18 years old for the prevention of hepatitis B infection

Day 60 Opinion

Vaccines

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed on a waiver on its own motion for recombinant Hepatitis B vaccine for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Prevention of infection of Hepatitis B virus'. The ground of the waiver is the 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.

2.1.22. Botulinum toxin type A - EMEA-002628-PIP01-19

Allergan Pharmaceutical International Limited; Post-operative atrial fibrillation in patients undergoing open-chest cardiac surgery

Day 30 Opinion

Cardiovascular Diseases

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 Botulinum toxin type A for all subsets of the paediatric population (0 to 18 years of age) in the condition of Post-operative atrial fibrillation in patients undergoing open-chest cardiac surgery.
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.

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. Etravirine - EMEA-C-000222-PIP01-08-M09

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

Day 60 Opinion

Infectious Diseases

Summary of Committee discussion:

This procedure was concluded in June 2019. See June 2019 PDCO minutes for further information.

2.2.2. Terbinafine (hydrochloride) - EMEA-C-001259-PIP02-13-M02

Polichem, S.A; Treatment of onychomycosis

Day 30 discussion

Dermatology

Summary of Committee discussion:

The PDCO adopted on 26 July 2019 an Opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0221/2019) of 17/06/2019.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Sacubitril / valsartan - EMEA-000316-PIP02-11-M04

Novartis Europharm Ltd.; Heart failure / Treatment of heart failure

Day 60 Opinion

Cardiovascular Diseases

Summary of Committee discussion:

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

2.3.2. Liquid ethanolic extract 30 per cent (w/w) of Allium cepa L. (fresh bulb) and Citrus limon (L.) Burm. f. (fresh fruit), Paullinia cupana Kunth, Theobroma cacao L. - EMEA-001835-PIP01-15-M04

LEGACY HEALTHCARE; Treatment of alopecia

Day 60 Opinion

Dermatology

Summary of Committee discussion:

The PDCO’s views expressed on Day 30 were re-discussed and 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/0138/2018 of 7 May 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. 2-hydroxypropyl-ß-cyclodextrin (HP-ß-CD) - Orphan - EMEA-001866-PIP01-15-M04

Mallinckrodt Pharmaceuticals Ireland Ltd; Treatment of Niemann-Pick disease, type C

Day 60 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO adopted a positive Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0027/2019 of 29/01/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Empagliflozin - EMEA-000828-PIP04-16-M03

Boehringer Ingelheim International GmbH; Treatment of type 1 diabetes mellitus

Day 60 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

During its plenary meeting on 26 July 2019, the PDCO adopted a positive Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0031/2019 of 11/02/2019) for empagliflozin for treatment of type 1 diabetes, as adjunct to insulin.
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 new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Semaglutide - EMEA-001441-PIP03-17-M01

Novo Nordisk A/S; Treatment of obesity

Day 60 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

During its July 2019 plenary meeting, the PDCO adopted a positive Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0007/2019 of 03/01/2019) for semaglutide, a glucagon-like-peptide-1 (GLP-1) receptor agonist, for the 'Treatment of obesity'.
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 new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Etrolizumab - EMEA-001434-PIP01-13-M02

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 60 Opinion

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 all 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/0148/2015 of 10/07/2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Odevixibat - Orphan - EMEA-002054-PIP01-16-M01

Albireo AB; Progressive familial intrahepatic cholestasis (PFIC)

Day 60 Opinion

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/0306/2017 of 31 October 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Ferric maltol - EMEA-001195-PIP01-11-M04

Norgine BV; Iron deficiency anaemia / Treatment for iron deficiency anaemia (IDA)

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.
Moreover, the PDCO agreed to change the wording of the condition of the PIP from Treatment of iron deficiency anaemia' to the wider scope 'Treatment of iron deficiency' in line with the authorised indication of Feraccru (ferric maltol). 'Treatment of iron deficiency' is understood to cover 'Treatment of iron deficiency anaemia'.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0164/2017 of 03/07/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Lonoctocog alfa - EMEA-001215-PIP01-11-M07

CSL Behring GmbH; Haemophila A

Day 60 Opinion

Haematology-Hemostaseology

Summary of Committee discussion:

During its July 2019 plenary meeting, the PDCO adopted a positive Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0058/2018 of 16/03/2018) for lonoctocog alfa in the condition 'Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)'. 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 new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Upadacitinib - EMEA-001741-PIP01-14-M02

AbbVie Ltd; Treatment of chronic idiopathic arthritis / Treatment of juvenile idiopathic arthritis

Day 60 Opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO's view expressed at Day 30 was re-discussed and 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/0363/2017 of 01 December 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Lamivudine (3TC) / dolutegrvir (DTG) – EMEA-001940-PIP01-160-M02

ViiV Healthcare BV; Human immunodeficiency virus (HIV-1) infection / Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 Opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO at the July 2019 meeting noted the replies provided by the Applicant at Day 30.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0151/2018 of 18/5/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Nirsevimab - anti-respiratory syncytial virus human IgG1κ monoclonal antibody - EMEA-001784-PIP01-15-M01

AstraZeneca AB; Prevention of respiratory syncytial viral infections

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 and on the Committee's discussion on Day 30, 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/0141/2016 of 20 May 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Pretomanid - Orphan - EMEA-002115-PIP01-17-M01

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

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 thus 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/0058/2019 of 26 February 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Ataluren – Orphan – EMEA-000115-PIP01-07-M10

PTC Therapeutics International, Limited; Treatment of dystrophinopathy ICD-10: G71.0 / Muscular dystrophy [of Duchenne and Becker] / Treatment of nonsense-mutation dystrophinopathy

Day 60 Opinion

Neurology

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/0393/2017 of 19/12/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M14

UCB Pharma S.A.; Treatment of neonatal seizures / Treatment of epilepsy with partial onset seizures / Treatment of paediatric patients with partial onset seizures / Treatment of neonatal seizures with adjunctive administration of brivaracetam

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 thus considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as

set in the Agency's latest decision (P/0051/2018 of 22 February 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M02

AveXis Netherlands B.V.; Treatment of spinal muscular atrophy / Treatment of spinal muscular atrophy type 1

Day 60 Opinion

Neurology

Summary of Committee discussion:

The PDCO re-discussed this procedure at Day 60 during the July 2019 plenary meeting.
The PDCO adopted a positive Opinion on this modification procedure at Day 60.
Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0162/2019 of 17 April 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Risdiplam - Orphan - EMEA-002070-PIP01-16-M03

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 60 Opinion

Neurology

Summary of Committee discussion:

During its July 2019 plenary meeting, the PDCO discussed the Applicant's additional clarifications and considered the Request for Modification for risdiplam for the 'Treatment of spinal muscular atrophy' in patients from birth to less than 18 years of age.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0360/2018 of 07/12/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M02

Ipsen Pharma; Treatment of malignant solid tumours

Day 60 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application, taking into account and acknowledging the additional information received after the Day 30 discussion. Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0134/2016 of 20 May 2016).

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

2.3.19. Cobimetinib - EMEA-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 / Treatment of children with a paediatric solid malignant tumour with known or expected Ras, Raf or MEK pathway activation, at first relapse or refractory to initial treatment

Day 60 Opinion

Oncology

Summary of Committee discussion:

The PDCO's views expressed at Day 30 were confirmed.
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/0216/2018 of 17 July 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Larotrectinib - Orphan - EMEA-001971-PIP02-16-M02

Bayer AG; 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 birth to less than 18 years of age with advanced solid tumours harbouring an NTRK fusion

Day 60 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed the proposed modification taking into account the clarification provided by the Applicant after the Day 30 discussion.
All pending issues were therefore considered solved and the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0182/2018 of 15 June 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.21. Pazopanib - EMEA-000601-PIP01-09-M06

Novartis Europharm Limited; Ewing sarcoma family of tumours / Rhabdomyosarcoma / Non-rhabdomyosarcoma soft tissue sarcoma / Treatment of paediatric patients with rhabdomyosarcoma / Treatment of paediatric patients with Ewing sarcoma family of tumours / Treatment of paediatric patients with non-rhabdomyosarcoma soft tissue sarcoma

Day 60 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this procedure at Day 60 during the July 2019 plenary meeting. 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/0405/2018 of 20 December 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.22. Tisagenlecleucel - Orphan - EMEA-001654-PIP02-17-M01

Novartis Europharm Limited; Mature B-cell neoplasm / Treatment of paediatric patients with CD19+ relapsed or refractory mature B-cell non-Hodgkin's lymphoma

Day 60 Opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this procedure at Day 60 during the July 2019 plenary meeting. The Applicant provided the clarifications 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/0266/2017 of 21 July 2017).

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

2.3.23. Human thrombin / human fibrinogen - EMEA-001149-PIP01-11-M05

Omrix Biopharmaceuticals N.V.; Treatment of cerebrospinal fluid leakage resulting from a surgical procedure, Treatment of haemorrhage resulting from a surgical procedure / Indicated for suture line sealing in dura mater closure, indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis, indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis.

Day 60 Opinion

Other

Summary of Committee discussion:

The PDCO re-discussed this procedure in line with the conclusions at Day 30. The PDCO took into account the additional information received.
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/0399/2017 of 19/12/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.24. Lomitapide (as lomitapide mesylate) - EMEA-001124-PIP01-10-M04

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia / Treatment of homozygous familial hypercholesterolaemia, Treatment of heterozygous familial hypercholesterolemia

Day 60 Opinion

Other

Summary of Committee discussion:

During its July 2019 plenary meeting, the PDCO adopted a positive Opinion on the PIP modification request for lomitapide for the treatment of homozygous familial hypercholesterolaemia (HoFH) in patients from 5 to less than 18 years of age.

In line with the above, the PDCO adopted a favourable Opinion on the modification of the agreed PIP, as set in the Agency's latest decision (P/0282/2015 of 27/11/2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.25. Dermatophagoides farinae / Dermatophagoides pteronyssinus - EMEA-001258-PIP01-11-M05

ALK-Abelló A/S; Treatment of allergic rhinitis / Treatment of asthma / Indicated in house dust mite allergic asthma / indicated in house dust mite allergic rhinitis

Day 60 Opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the justification submitted by the Applicant, the PDCO considered that the proposed change 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/0056/2018 of 16 March 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.26. Fevipiprant - EMEA-001315-PIP02-16-M02

Novartis EuroPharm Limited; Asthma / Treatment of uncontrolled persistent asthma

Day 60 Opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan and the Committee's discussion on Day 30, the PDCO considered that the proposed change could be accepted.

The PDCO therefore adopted a favourable Opinion accordingly on the modification of the agreed PIP as set in the Agency's latest decision (P/0157/2019 of 17 April 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.27. Finerenone - EMEA-001623-PIP01-14-M03

Bayer AG; Chronic kidney disease / Treatment of chronic kidney disease associated with proteinuria in addition to a therapy with angiotensin converting enzyme inhibitor (ACEi) or angiotensin receptor blocker (ARB)

Day 60 Opinion

Uro-nephrology

Summary of Committee discussion:

Based on the review of the rationale submitted by the Applicant for modifying the agreed paediatric investigation plan, and the discussions on Day 30 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/0160/2019 of 17 April 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.28. Influenza virus surface antigens (haemagglutinin) of strain A (H1N1) / influenza virus surface antigens (haemagglutinin) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin) of strain B (Yamagata lineage) - EMEA-002027-PIP02-17-M01

Adimmune Corporation; 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/0294/2017 of 4 October 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.29. Outer membrane vesicles (OMV) from *neisseria meningitidis* serogroup B strain NZ98/254 measured as amount of total protein containing the PorA P1.4 / Recombinant *neisseria meningitidis* serogroup B fHbp fusion protein / Recombinant *neisseria meningitidis* serogroup B NadA protein / Recombinant *neisseria meningitidis* serogroup B NHBA fusion protein - EMEA-000139-PIP01-07-M03

GSK Vaccines S.r.l.; Prevention of meningococcal meningitis

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/0040/2018 of 16 February 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.30. Prepandrix: A/Indonesia/05/2005 (H5N1) like strain used / Adjupanrix: split influenza virus, inactivated, containing antigen: A/VietNam/1194/2004 (H5N1) like strain used - EMEA-000160-PIP01-07-M05

GlaxoSmithKline Biologicals SA; 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/0087/2015 of 8 May 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.4. Opinions on Re-examinations

No items

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

For the following partial compliance checks, no need to refer them to PDCO Committee for discussion, were identified by the PME coordinator and PDCO Rapporteur. The PDCO has been informed in writing.

2.7.1. Quizartinib - EMEA-C3-001821-PIP01-15-M03

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 30 letter

Oncology

Summary of Committee discussion:

The partial compliance check has been finalised with a confirmation of compliance of all those studies contained in the agreed paediatric investigation plan that were subject of this procedure.

2.7.2. Peanut (Arachis hypogaea) allergens (previously known as Peanut flour) - EMEA-C2-001734-PIP01-14-M04

Aimmune Therapeutics Netherlands B.V.; Treatment of peanut allergy

Day 1 letter

Pneumology – Allergology

Summary of Committee discussion:

The relevant Study is hereby confirmed to be compliant as set out in the EMA's Decision (P/0114/2019 of 29 March 2019).

The PDCO has been informed about the outcome.

2.7.3. Canagliflozin hemihydrate - EMEA-C1-001030-PIP01-10-M07

Janssen-Cilag International NV; Treatment of type 2 diabetes mellitus

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The relevant PIP Study is confirmed by EMA to be compliant as set out in the EMA's Decision P/0205/2017 of 09 August 2017.

2.7.4. Satralizumab - EMEA-C1-001625-PIP01-14-M03

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 30 letter

Neurology

Summary of Committee discussion:

All relevant PIP Studies are confirmed by EMA to be compliant as set out in the latest EMA's Decision.

2.8. Revision of PDCO Opinions

2.8.1. Recombinant adeno-associated viral vector serotype 2 carrying the gene for the human aromatic L-amino acid decarboxylase protein - Orphan - EMEA-002435-PIP01-18

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

Neurology

Summary of Committee discussion:

The Revision of the Opinion is a procedure to rectify a content error in PDCO Opinion before the adoption of the EMA Decision pertaining to the Opinion. The adoption of the revised Opinion by the PDCO is required and the date of the revised PDCO Opinion will change

correspondingly to the date of adoption by the PDCO.

After adoption of the Opinion, the Applicant noticed an administrative mistake.

Taking the above into consideration, the PDCO adopted a positive Opinion on the Revision.

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

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

Day 60 discussion

Dermatology

3.1.2. Lebrikizumab - EMEA-002536-PIP01-18

Treatment of atopic dermatitis

Day 60 discussion

Dermatology

3.1.3. Deoxycytidine - Orphan - EMEA-002513-PIP01-18

Modis Therapeutics, Inc.; Treatment of thymidine kinase 2 deficiency

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Deoxythymidine - Orphan - EMEA-002624-PIP01-19

Modis Therapeutics, Inc.; Treatment of thymidine kinase 2 deficiency

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Haematology-Hemostaseology

3.1.6. Baricitinib - EMEA-001220-PIP05-19

Treatment of systemic lupus erythematosus

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Mavorixafor - EMEA-002490-PIP01-18

Treatment of warts, hypogammaglobulinemia, infections, and myelokathexis (WHIM) syndrome / Treatment of WHIM syndrome in paediatric patients aged 6 years and above

Day 60 discussion

Immunology-Rheumatology-Transplantation

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

Treatment of infections due to aerobic Gram-negative bacteria

Day 60 discussion

Infectious Diseases

3.1.9. Zoliflodacin - EMEA-002599-PIP01-19

Treatment of gonococcal infection / Treatment of uncomplicated gonorrhoea

Day 60 discussion

Infectious Diseases

3.1.10. EMEA-002572-PIP01-19

Chromosome 15q duplication syndrome / Cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder / Treatment of seizures associated with chromosome 15q duplication syndrome / Treatment of seizures associated with CDKL5 deficiency disorder

Day 60 discussion

Neurology

3.1.11. Adeno-associated viral vector serotype 8 containing the human RPGR gene - Orphan - EMEA-002601-PIP01-19

Nightstar Europa Limited; Treatment of X-linked retinitis pigmentosa

Day 60 discussion

Ophthalmology

3.1.12. Aprocitentan - EMEA-001978-PIP02-19

Hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.13. EMEA-002608-PIP01-19

Prostate cancer - diagnosis

Day 30 discussion

Diagnostic / Oncology

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

Chemotherapy-induced thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in patients receiving myelosuppressive chemotherapy for solid tumours

Day 30 discussion

Haematology-Hemostaseology

3.1.15. EMEA-001931-PIP02-19

Treatment of acquired fibrinogen deficiency

Day 30 discussion

Haematology-Hemostaseology

3.1.16. Marzeptacog alfa (activated) - Orphan - EMEA-002270-PIP01-19

Voisin Consulting S.A.R.L; Treatment of haemophilia B

Day 30 discussion

Haematology-Hemostaseology

3.1.17. Marzeptacog alfa (activated) - EMEA-002270-PIP02-19

Treatment of haemophilia A

Day 30 discussion

Haematology-Hemostaseology

3.1.18. Allogeneic, non-expanded, umbilical cord blood-derived, haematopoietic mature myeloid and lymphoid cells (NF[1]) / Allogeneic, ex vivo expanded, umbilical cord blood-derived, haematopoietic CD34+progenitor cells (CF[2]) - Orphan - EMEA-001913-PIP02-18

Gamida Cell Ltd; Treatment in haematopoietic stem cell transplantation / Haematopoietic reconstitution of patients who are medically indicated for allogeneic haematopoietic stem cell transplantation

Day 30 discussion

[1] Non cultured fraction
[2] Cultured fraction

Immunology-Rheumatology-Transplantation

3.1.19. C1-esterase inhibitor human - Orphan - EMEA-002316-PIP03-19

CSL Behring GmbH; Treatment of antibody mediated rejection (AMR) in kidney transplantation

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.20. Ibrexafungerp citrate - EMEA-002535-PIP02-19

Vulvovaginal candidiasis

Day 30 discussion

Infectious Diseases

3.1.21. EMEA-002566-PIP01-19

Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.1.22. EMEA-002424-PIP02-19

Treatment of Lewy body dementia

Day 30 discussion

Neurology

3.1.23. Arimoclomol citrate - Orphan - EMEA-001748-PIP03-19

Orphazyme A/S; Treatment of amyloid lateral sclerosis

Day 30 discussion

Neurology

3.1.24. Cannabidiol – EMEA-001964-PIP02-19 ss

Treatment of Rett Syndrome / Rett Syndrome

Day 30 discussion

Neurology

3.1.25. Dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19

EryDel S.p.A; Treatment of ataxia telangiectasia (AT) / Treatment of neurological symptoms in patients with AT

Day 30 discussion

Neurology

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

argenx BVBA; Treatment of myasthenia gravis

Day 30 discussion

Neurology

3.1.27. Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain - Orphan - EMEA-002314-PIP01-17

DNAtrix, Inc.; High-Grade Glioma / Treatment of unresectable high-grade glioma in first recurrence, and diffuse intrinsic pontine glioma after failure of radiotherapy

Day 30 discussion

Oncology

3.1.28. EMEA-002585-PIP01-19

Multiple myeloma

Day 30 discussion

Oncology

3.1.29. 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 30 discussion

Oncology

3.1.30. Emixustat - EMEA-002581-PIP01-19

Stargardt disease

Day 30 discussion

Ophthalmology

3.1.31. Timolol / bimatoprost - EMEA-002583-PIP01-19

Ocular hypertension / Primary open-angle glaucoma

Day 30 discussion

Ophthalmology

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

Nightstar Europa Limited; Treatment of choroideremia

Day 30 discussion

Ophthalmology

3.1.33. EMEA-002559-PIP02-19

Myasthenia gravis

Day 30 discussion

Other

3.1.34. Emiplacel - EMEA-002539-PIP02-19

Treatment of muscle injury

Day 30 discussion

Other

3.1.35. Adeno-associated viral vector serotype 8 containing the human MTM1 gene - Orphan - EMEA-002571-PIP01-19

Audentes Therapeutics, Inc.; X-linked myotubular myopathy (XLMTM)

Day 30 discussion

Other

3.1.36. 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 fibrosis, Treatment of interstitial lung disease with fibrosis in children

Day 30 discussion

Pneumology - Allergology

3.1.37. EMEA-002612-PIP01-19

Prevention of pulmonary dysfunction / Prevention of cardiopulmonary bypass (CPB) induced postoperative pulmonary dysfunction (PPD)

Day 30 discussion

Pneumology - Allergology

3.1.38. Benralizumab - EMEA-001214-PIP03-19

Treatment of vasculitides / Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 30 discussion

Pneumology - Allergology

3.1.39. EMEA-002121-PIP03-19

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

Day 30 discussion

Psychiatry

3.1.40. Dasotraline hydrochloride - EMEA-002590-PIP01-19

Binge eating disorder / Moderate to severe binge eating disorder

Day 30 discussion

Psychiatry

3.1.41. Ecopipam (hydrochloride) - EMEA-002564-PIP01-19

Tourette syndrome

Day 30 discussion

Psychiatry

3.1.42. EMEA-002589-PIP01-19

Schizophrenia / Treatment of schizophrenia

Day 30 discussion

Psychiatry

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. Perampanel - EMEA-C6-000467-PIP01-08-M11

Eisai Europe Ltd; Treatment of treatment-resistant epilepsies

Day 30 discussion

Neurology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

No items

4. Nominations

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 17 September 2019 for Nomination of Rapporteur and Peer reviewer

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Information related to this section cannot be 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

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. EMEA-10-2019

All classes of medicinal products for treatment of Alzheimer's disease /

Treatment of Alzheimer's disease

Summary of Committee discussion:

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

Other potential paediatric interests of this medicine suggested by PDCO: PDE9 inhibitors have been investigated as "cognition enhancers" in Alzheimer´s disease and schizophrenia as intracellular cGMP is involved in glutamatergic neurotransmission. Some PIPs on cognitive deficits in schizophrenia are ongoing.

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 items

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

The PDCO Committee noted the nomination of Arnes Resic as the new Alternate for Croatia.

The PDCO Chair welcomed Milivoj Novak and Roel Bolt the new Member for Croatia and for the Netherlands respectively.

The PDCO Chair welcomed Maaike van Dartel as the new Alternate for the Netherlands.

9.1.2. PDCO Chairperson - election

Summary of Committee discussion:

The mandate of the PDCO Chairperson, Dirk Mentzer, ends on 13 September 2019 having served the maximum of two 3-year mandates. The EMA Secretariat thanked Dirk Mentzer for

his enormous achievements during his chairmanship, his leadership as Chair to the PDCO as well as his contributions to the EU network.

The election of the new Chairperson took place in accordance to the PDCO rules of procedure. The PDCO elected Koenraad Norga as new PDCO Chair, for a three-year mandate, starting on 14 September 2019.

The PDCO and the Agency congratulated Koenraad Norga on his election and wished him all the best in his new role at the helm of the Committee.

9.1.3. August written procedures: process reminder and timelines

Summary of Committee discussion:

EMA secretariat presented the timelines for the August written procedure which will take place from 16 to 23 August included. The timelines were adopted.

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 medicinal products with recommended paediatric indications adopted in June 2019. These included Dupixent (dupilumab), Fiasp (insulin aspart), Victoza (liraglutide) and Zinforo (ceftaroline fosamil).

The list of procedures with paediatric indications to be evaluated by the CHMP, starting in June 2019, 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 (NCWG) evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of Committee discussion:

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

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA): introduction to the European Patient Forum (Youth Group)

Summary of Committee discussion:

On joint invitation by the European network of paediatric research at the EMA (Enpr-EMA) and the EMA/PDCO a young patient representative of the Youth Group of the European Patient Forum (EPF) presented an overview of the EPF, an independent, non-governmental umbrella organisation of more than 70 European member patient organisations. Moreover, the EPF's Youth Group's mission and priority activities were introduced to the Committee: The Youth Group was established in 2012 and aims to strengthen the involvement and representation of young patients in patient organisations, promote young patients' rights and recognition of their needs and expectations within and beyond the area of health care and health policy, and to empower them to develop confidence and advocacy skills. The PDCO stressed that the 'real-life' experience and specific knowledge of young patients is of high value for its work and that it would welcome the strengthening of collaboration with the EPF Youth Group and other patient organisations.

9.5. Cooperation with International Regulators

No items

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

9.6.1. Strategic Review and Learning Meeting (SRLM): Selection of therapeutic area/condition overview for November meeting

PDCO: Dirk Mentzer (Chair)

Summary of Committee discussion:

The PDCO discussed this topic from the latest SRLM meeting. The therapeutic area overview selected was asthma. Other topics for the future include perinatal asphyxia, type 2 diabetes and non-alcoholic steatohepatitis.

9.6.2. Conect4Children multistakeholder meetings

Summary of Committee discussion:

The discussion was postponed.

9.6.3. Unmet medical needs: summary of Malta SRLM presentation and discussion, agreement on the way forward

PDCO members: Karl-Heinz Huemer, Eleni Katsomiti

Summary of Committee discussion:

The Chair of the paediatric inventory group presented to the PDCO the main points of his presentation on unmet medical needs that took place at the Malta SRLM.

9.6.4. Strategic Review and Learning Meeting (SRLM) under the Finnish Presidency to be held in Helsinki on 20-22 November 2019

PDCO members: Pia Annunen, Ann-Marie Tötterman

Summary of Committee discussion:

The draft agenda for the SRLM meeting was presented and suggestions for additional topics were requested from the PDCO members.

9.7. PDCO work plan

No items

9.8. Report from the Paediatric Cluster Teleconference

Summary of Committee discussion:

Postponed to next meeting

9.9. Planning and reporting

No items

10. Any other business

10.1.1. Future EMA Relocation, meeting dates and location 2019 - 2020

Summary of Committee discussion:

The PDCO was updated on the plans for the future relocation of the Agency and the impact on the Committees meeting dates and location in 2019-2020. The PDCO December meeting dates will be changed from 11-13 December to 9-11 December 2019.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of Committee discussion:

The Group discussed topics relevant to paediatric oncology development.

11.1.2. Neonatology

Summary of Committee discussion:

The Neonatology breakout session was updated on the PRAC assessment of the signal of adverse outcomes in neonates treated with parenteral nutrition solutions not protected from light, following the discussion at the March breakout session.

The group also considered the next steps in the Guideline revision process in preparation for when the business continuity plan will be lifted.

11.1.3. Inventory

Summary of Committee discussion:

The inventory group convened on the margins of the PDCO plenary meeting to discuss organisational matters and continued discussion on the assessment of unmet needs.

11.1.4. Systemic lupus erythematosus

Summary of Committee discussion:

The systemic lupus erythematosus joint session was attended by members of the PDCO and CHMP to discuss data requirements for the paediatric development of medicines in this condition.

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 23-26 July 2019

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dirk Mentzer	Chair	Germany	No interests declared	N/A
Karl-Heinz Huemer	Member	Austria	No interests declared	N/A
Koenraad Norga	Member (Vice-Chair)	Belgium	When chairing the meeting: To be replaced for discussions, final deliberations and voting When not chairing the meeting: No participation in final deliberations and voting	- Outer membrane vescicles (OMV) from neisseria meningitidis serogroup B strain - EMEA-000139-PIP01-07-M03 - Prepandrix: A/Indonesia/05/2005 (H5N1) like strain used / Adjupanrix: split influenza virus, inactivated, containing antigen: A/VietNam/1194/2004 (H5N1) like strain used - EMEA-000160-PIP01-07-M05 - Niraparib (as tosylate monohydrate) - Orphan - EMEA-002268-PIP02-18 - Anti PD-1 monoclonal antibody - EMEA-002463-PIP01-18
Karen Van Malderen	Alternate	Belgium	No interests declared	N/A
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	None
Milivoj Novak	Member	Croatia	No interests declared	N/A
Petra Dominikova	Alternate	Czech Republic	No interests declared	N/A
Kirstine Moll Harboe	Member	Denmark	No interests declared	N/A
Irja Lutsar	Member	Estonia	No interests declared	N/A
Pia Annunen	Alternate	Finland	No participation in discussion, final deliberations and voting on:	EMEA-002448-PIP01-18
Sylvie Benchetrit	Member	France	No interests declared	N/A
Dominique Ploin	Alternate	France	No interests declared	N/A
Sabine	Member	Germany	No interests declared	N/A

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Scherer				
Yuansheng Sun	Alternate	Germany	No interests declared	N/A
Eleni Katsomiti	Member	Greece	No interests declared	N/A
Anastasia Mountaki	Alternate	Greece	No interests declared	N/A
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	N/A
Brian Aylward	Member	Ireland	No interests declared	N/A
Sara Galluzzo	Member	Italy	No interests declared	N/A
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	None
Sigita Burokiene	Member	Lithuania	No interests declared	N/A
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	N/A
Herbert Lenicker	Alternate	Malta	No interests declared	N/A
Roel Bolt	Member	Netherlands	No interests declared	
Maaike van Dartel	Alternate	Netherlands	No interests declared	N/A
Siri Wang	Member	Norway	No interests declared	N/A
Anette Solli Karlsen	Alternate	Norway	No interests declared	N/A
Marek Migdal	Member	Poland	No interests declared	N/A
Helena Fonseca	Member	Portugal	No interests declared	N/A
Hugo Tavares	Alternate	Portugal	No interests declared	N/A
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	N/A
Peter Sisovsky	Member	Slovakia	No interests declared	N/A
Peter Szitanyi	Alternate	Slovakia	No interests declared	N/A
Stefan Grosek	Member	Slovenia	No interests declared	N/A
Fernando de Andrés Trelles	Member	Spain	No interests declared	N/A
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	N/A
Ninna Gullberg	Member	Sweden	No interests declared	N/A
Angeliki Siapkara	Member	United Kingdom	No interests declared	N/A
Fernando Cabanas	Member	Healthcare Professionals'	No participation in final deliberations	Nirsevimab - anti-respiratory syncytial

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
		Representative	and voting on:	virus human IgG1κ monoclonal antibody EMEA-001784-PIP01-15-M01
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	N/A
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	N/A
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	N/A
Doina Plesca	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	none
Günter Karl-Heinz Auerswald	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	None
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	N/A
Maria Estela Moreno Martin	Expert - in person*	Spain - AGEMED/AEMPS	No interests declared	N/A
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/