EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

31 January 2020
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Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes for the meeting on 09-11 December 2019

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

Disclaimers

Some of the information contained in the 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, the minutes are 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
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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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 2.1.11 and 2.3.14.

1.2. Adoption of agenda

The agenda for the PDCO meeting 9-11th December 2019 was adopted.

1.3. Adoption of the minutes

The minutes of November 2019 PDCO meeting 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. Asciminib - EMEA-002347-PIP01-18

Novartis Europharm Limited; Treatment of chronic myeloid leukaemia

Day 120 opinion

Oncology

Summary of committee discussion:

The PDOC re-discussed this application in line with the outcome conclusion from day 90.
The Committee acknowledged that all outstanding quality issues were considered acceptable as reflected in the Opinion.
Overall the PDCO agreed a PIP for the condition of treatment of chronic myeloid leukaemia and a deferral.

2.1.2. Anti-IL-17A/F Nanobody (MK1095) - EMEA-002568-PIP01-19

Bond Avillion 2 Development LP; Psoriasis / Treatment of psoriasis

Day 120 opinion

Dermatology

Summary of committee discussion:

The applicant's responses to the D90 issues were acceptable and a positive opinion was adopted on D120.

2.1.3. Dupilumab - EMEA-001501-PIP04-19

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant addressed the issues raised by the PDCO at Day 90The members expressed doubts that a primary endpoint reflecting only histological improvement would generate sufficient efficacy and safety data on dupilumab use in the proposed indication in children

Therefore, the PDCO adopted a negative opinion.

2.1.4. Norursodeoxycholic acid - Orphan - EMEA-002485-PIP01-18

Dr. Falk Pharma GmbH; Treatment of primary sclerosing cholangitis / Treatment of autoimmune sclerosing cholangitis

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO discussed this procedure on D120.
The applicant's responses to the D90 issues were acknowledged and the applicant's proposal was welcomed. The totality of the data will be important when assessing the data at the time of the marketing authorisation application and improvement in the biochemical endpoints in addition to histology and clinical endpoints will be important.
A positive opinion was adopted.

2.1.5. Factor VIII Fc – von Willebrand factor – XTEN fusion protein (rFVIIIFc-VWF-XTEN) - EMEA-002501-PIP01-18

Bioverativ Therapeutics, Inc., a Sanofi Company; Treatment of congenital haemophilia A

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the clarification provided by the applicant after D90.
A positive opinion was adopted at D120.

2.1.6. Anti-CD7 mAb conjugated to ricin toxin A chain / anti-CD3 mAb conjugated to ricin toxin A chain (T-Guard) - Orphan - EMEA-002087-PIP01-16

Xenikos BV; Treatmeant of acute graft versus host disease

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

A positive opinion was adopted on D120.

2.1.7. Mecasermin rinfabate - Orphan - EMEA-000534-PIP03-17

Premacure AB, a member of the Shire group of companies; Prevention of chronic lung disease of prematurity

Day 120 opinion

Neonatology - Paediatric Intensive Care

Summary of committee discussion:

An oral explanation meeting (OEM) took place on 10 December 2019. A positive opinion was adopted at D120.

2.1.8. Atogepant - EMEA-002530-PIP01-18

Allergan Pharmaceuticals International Limited; Prevention of migraine headaches

Day 120 opinion

Pain

Summary of committee discussion:

The PDCO noted the replies of the applicant to the points raised at D90 and considered they were satisfactorily addressed. In conclusion the PDCO adopted a positive opinion for the PIP for atogepant with a deferral in the condition prevention of migraine headaches.

2.1.9. (R)-1-(3-(aminomethyl) phenyl)-N-(5-((3 cyanophenyl)(cyclopropylmethylamino)methyl)-2-fluorophenyl)-3-(trifluoromethyl)-1H-pyrazole-5-carboxamide dihydrochloride (BCX7353) - Orphan - EMEA-002449-PIP02-18

BioCryst UK; Treatment of hereditary angioedema (HAE)

Day 120 opinion

Action: For adoption

Pneumology - Allergology

Summary of committee discussion:

All the other issues having been addressed and the PDCO adopted a positive opinion with a waiver and deferral for BCX7353 in treatment of hereditary angioedema at their December 2019 meeting.

2.1.10. 2-[[8-chloro-3-[(4-chlorophenyl)methyl]-4-(difluoromethoxy)-2-ethyl-5-quinolinyl]oxy]acetic acid L-lysine salt (GB001) - EMEA-002484-PIP01-18

GB001, Inc (A wholly-owned subsidiary of Gossamer Bio, Inc.); Treatment of asthma

Day 120 opinion

Pneumology - Allergology

Summary of committee discussion:

Based on the assessment of this application, taking into account the additional information and clarifications submitted by the applicant, the PDCO agrees with the proposed paediatric development plan and adopted a positive opinion for 2-[[8-chloro-3-[(4-chlorophenyl)methyl]-4-(difluoromethoxy)-2-ethyl-5- quinolinyl]oxy]acetic acid L-lysine salt in the condition ”treatment of asthma”.

2.1.11. EMEA-002515-PIP01-18

GlaxoSmithKline Trading Services Limited; Treatment of asthma

Day 120 opinion

Pneumology - Allergology

Summary of committee discussion:

The Committee considered the proposal by the applicant.

The PDCO adopted a positive opinion.

2.1.12. Budesonide - Orphan - EMEA-002500-PIP01-18

Calliditas Therapeutics AB; Treatment of primary IgA nephropathy

Day 120 opinion

Uro-nephrology

Summary of committee discussion:

A positive opinion was adopted at D120.

2.1.13. RhPSMA-7.3 (18F) - EMEA-002657-PIP01-19

Blue Earth Diagnostics Ltd; Visualisation of prostate-specific membrane antigen in adenocarcinoma of the prostate

Day 60 opinion

Diagnostic

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 rhPSMA-7.3 (18F) for all subsets of the paediatric population (0 to 18 years of age) in the condition of Visualisation of prostate-specific membrane antigen in adenocarcinoma of the prostate.
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.

2.1.14. 3-[4-(4-Aminopiperidin-1-yl)-3-(3,5-difluorophenyl)quinolin-6-yl]-2-hydroxybenzonitrile (CRN00808) - EMEA-002682-PIP01-19

Crinetics Pharmaceuticals Inc; Treatment of acromegaly / Treatment of pituitary gigantism

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The PDCO therefore adopted a positive Opinion on this full waiver request on 11 December 2019, for 3-[4-(4-Aminopiperidin-1-yl)-3-(3,5-difluorophenyl)quinolin-6-yl]-2-hydroxybenzonitrile (Lab code: CRN00808) for all subsets of the paediatric population (0 to 18 years of age) in the two conditions of:
Treatment of Acromegaly, on the grounds that the disease or condition does not occur in the paediatric population
Treatment of Gigantism on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2.1.15. Glibenclamide - EMEA-002651-PIP01-19

Biogen Idec Limited; Treatment of large hemispheric infarction

Day 60 opinion

Neurology

Summary of committee discussion:

In conclusion, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for glibenclamide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of large hemispheric infarction on the grounds of lack of significant therapeutic benefit as studies are not feasible.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. The PDCO identified hemorrhagic progression of contusion following brain injury 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.

2.1.16. Hyoscine / Physostigmine - EMEA-002678-PIP01-19

Defence Science and Technology Laboratories; Prevention of organophosphate poisoning

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 physostigmine / hyoscine for all subsets of the paediatric population (0 to 18 years of age) in the condition of Prevention of organophosphate poisoning.
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. Autologous dendritic cells activated by transient exposure to killed prostate cancer cells ex vivo (DCVAC/PCa) - EMEA-002679-PIP01-19

SOTIO a.s.; Treatment of prostate cancer

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed this product at day 60 during the December 2019 plenary.
Based on the assessment of this application, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for autologous dendritic cells activated by transient exposure to killed prostate cancer cells ex vivo (DCVAC/PCa) for all subsets of the paediatric population (0 to 18 years of age) in the condition of prostate cancer.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the

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

2.1.18. Canakinumab - EMEA-000060-PIP08-19

Novartis Europharm Limited; Treatment of lung carcinoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's views expressed at D30 were endorsed.

The PDCO agreed with the applicant's request for a waiver and recommended granting a waiver for canakinumab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of lung carcinoma based on the grounds that the disease for which the specific medicinal product is intended 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.

2.1.19. Humanized immunoglobulin (Ig) G4 proline, alanine, alanine (IgG4 PAA) based bispecific antibody directed against cluster of differentiation (CD) 3 receptor complex and B-cell maturation antigen (BCMA) - EMEA-002650-PIP01-19

Janssen-Cilag International N.V.; treatment of multiple myeloma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed this product at day 60 during the December 2019 plenary.

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver.

The PDCO recommended granting a waiver for Humanized immunoglobulin (Ig) G4 proline, alanine, alanine (IgG4 PAA) based bispecific antibody directed against cluster of differentiation (CD) 3 receptor complex and B-cell maturation antigen (BCMA) for all subsets of the paediatric population (0 to 18 years of age) in the condition 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. The PDCO identified treatment of mature B-cell malignancies 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.

2.1.20. Futibatinib - Orphan - EMEA-002647-PIP01-19

Taiho Pharma Europe Lt; Treatment of cholangiocarcinoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure in line with the outcome 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 futibatinib for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of cholangiocarcinoma based on the ground that the disease does not occur in children.
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. Ripasudil - EMEA-002676-PIP01-19

Kowa Pharmaceutical Europe Co. Ltd.; Treatment of corneal dystrophy

Day 60 opinion

Ophthalmology

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 ripasudil for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of corneal dystrophy 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.

2.1.22. Modified human papillomavirus capsid protein conjugated to the near-infrared dye silicate(5-),bis[N-[3-[(hydroxy-.kappa.O)dimethylsilyl]propyl]-3-sulfo-N,N-bis(3-sulfopropyl)-1-propanaminiumato(4-)][6-[[[3-[(29H,31H-phthalocyanin-yl-.kappa.N29,.kappa.N30,.kappa.N31,.kappa.N32)oxy]propoxy]carbonyl]amino]hexanoato(3-)]-, sodium (1:5) - EMEA-002658-PIP01-19

Aura Biosciences Inc; Treatment of ocular melanoma

Day 60 opinion

Ophthalmology / Oncology

Summary of committee discussion:

The PDCO discussed this product at day 60 during the December 2019 plenary.
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 Modified human papillomavirus capsid protein conjugated to the near-infrared dye silicate(5-),bis[N-[3-[(hydroxy-.kappa.O)dimethylsilyl]propyl]-3-sulfo-N,N-bis(3-sulfopropyl)-1-propanaminiumato(4-)][6-[[[3-[(29H,31H-phthalocyanin-yl-.kappa.N29,.kappa.N30,.kappa.N31,.kappa.N32)oxy]propoxy]carbonyl]amino]hexanoato(3-)]-, sodium (1:5) for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of ocular melanoma.
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. Benzocaine - EMEA-002654-PIP02-19

Johnson and Johnson; Treatment of oropharyngeal pain;

Day 60 opinion

Oto-rhino-laryngology

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 benzocaine for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of oropharyngeal pain on the ground of lack of significant therapeutic benefit.

2.1.24. Levocetirizine / montelukast - EMEA-002646-PIP01-19

Abbott Laboratories Limited; Treatment of allergic rhinitis

Day 60 opinion

Pneumology - Allergology / Oto-rhino-laryngology

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 Levocetirizine / montelukast for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of allergic rhinitis.

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. Ertugliflozin - EMEA-C2-001533-PIP01-13-M02

MSD (Europe) Inc.; Treatment of type 2 diabetes mellitus

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Studies are confirmed to be compliant as set out in the EMA's Decision ((P/0141/2019 from 17/04/2019):
Compliance for PIP Study confirmed.

2.2.2. Glecaprevir / pibrentasvir - EMEA-C-001832-PIP01-15-M02

AbbVie Ltd; Treatment of chronic hepatitis C

Day 30 Opinion

Infectious Diseases

Summary of committee discussion:

The PDCO adopted on 13 December 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/0128/2019 of 17 April 2019.

2.2.3. Setmelanotide - EMEA-C1-002209-PIP01-17

Rhythm Pharmaceuticals, Inc.; Treatment of appetite and general nutrition disorders

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Studies are confirmed to be compliant as set out in the EMA's Decision (P/0164/2018 of 15/06/2018).

2.2.4. Sodium thiosulfate (STS) - EMEA-C-002147-PIP02-17-M01

Fennec Pharmaceuticals, Inc.; Prevention of platinum-induced ototoxic hearing loss

Day 30 Opinion

Oncology / Oto-rhino-laryngology

Summary of committee discussion:

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

2.2.5. Lurasidone (hydrochloride) - EMEA-C-001230-PIP01-11-M05

Aziende Chimiche Riunite Angelini Francesco -ACRAF S.p.A; Treatment of schizophrenia

Day 30 Opinion

Psychiatry

Summary of committee discussion:

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

2.2.6. Vestronidase alfa - EMEA-C-001540-PIP01-13-M01

Ultragenyx Germany GmbH; Treatment of mucopolysaccharidosis type VII

Day 30 Opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO adopted on 11 December 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/0202/2016) of 28 July 2016.

2.2.7. Octenidine (dihydrochloride) - EMEA-C-001514-PIP01-13-M01

Cassella-med GmbH & Co. KG; Treatment of upper respiratory tract infections

Day 30 Opinion

Oto-rhino-laryngology

Summary of committee discussion:

The PDCO adopted on 11 December 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/0269/2019 of 14 August 2019.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Zoledronic acid - EMEA-000057-PIP01-07-M07

Novartis Europharm Limited; Treatment of osteoporosis / Treatment of Paget’s disease of the bone

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be

accepted.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0140/2013 of 3 July 2013).

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

2.3.2. Albutrepenonacog alfa - Orphan - EMEA-001107-PIP01-10-M04

CSL Behring GmbH; Haemophilia B / 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 application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0230/2017 of 09/08/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Cannabidiol (CBD)- Orphan - EMEA-001964-PIP01-16-M01

GW Pharma (International) B.V.; Treatment of seizures associated with Lennox-Gastaut Syndrome / Treatment of seizures associated with Dravet syndrome / Treatment of seizures associated with tuberous sclerosis complex /Treatment of seizures associated with infantile spasms

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the majority of the proposed changes could be accepted.
The PDCO thus adopted a positive opinion endorsing the majority of the proposed modifications. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. (RS)-Baclofen / Naltrexone HCl /D-Sorbitol (PXT3003) - Orphan - EMEA-002164-PIP01-17-M02

Pharnext S.A.; Charcot-Marie-Tooth disease Type 1A / Treatment of Charcot-Marie-Tooth type 1A

Day 60 opinion

Neurology

Summary of committee discussion:

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

2.3.5. Lenvatinib - EMEA-001119-PIP02-12-M06

Eisai GmbH; Treatment of papillary thyroid cancer / Treatment of osteosarcoma / Treatment of follicular thyroid cancer

Day 60 opinion

Oncology

Summary of committee discussion:

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

In conclusion, the PDCO 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 (P/0209/2019 of 12 June 2019).

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

2.3.6. Lipegfilgrastim - EMEA-001019-PIP01-10-M05

UAB "Sicor Biotech"; Treatment of chemotherapy-induced neutropenia / Prevention of chemotherapy-induced febrile neutropenia

Day 60 opinion

Oncology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0261/2017 of 09/09/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Nivolumab - EMEA-001407-PIP01-12-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)

Day 60 opinion

Oncology

Summary of committee discussion:

In conclusion, the PDCO 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 (P/0049/2018 of 22 February 2018).

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

2.3.8. Nivolumab - EMEA-001407-PIP02-15-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue / Treatment of malignant neoplasms of the central nervous system

Day 60 opinion

Summary of committee discussion:

In conclusion, the PDCO 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 (P/0050/2018 of 22 February 2018).

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

2.3.9. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M03

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

Day 60 opinion

Other

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0267/2019 of 25 July 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Vamorolone - Orphan - EMEA-001794-PIP02-16-M02

ReveraGen BioPharma Ltd.; Treatment of Duchenne muscular dystrophy

Day 60 opinion

Other

Summary of committee discussion:

Based on the review of the application submitted 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/0288/2018 of 12 September 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Vosoritide (BMN 111) - Orphan - EMEA-002033-PIP01-16-M01

BioMarin International Limited; Treatment of achondroplasia

Day 60 opinion

Action: For adoption

Other

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Bupivacaine - EMEA-000877-PIP03-17-M01

Pacira Ltd; Postsurgical analgesia

Day 60 opinion

Action: For adoption

Pain

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Fluticasone furoate / umeclidinium bromide / vilanterol trifenatate / - EMEA-002153-PIP01-17-M01

GlaxoSmithKline Trading Services Limited; Treatment of asthma

Day 60 opinion

Action: For adoption

Pneumology – Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed

paediatric investigation plan, the PDCO considered that the proposed changes could not be accepted at this point in time.

The PDCO therefore adopted a negative Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0076/2018 of 16 March 2018).

The PDCO Opinion on the modified agreed PIP therefore remains unchanged to the previous PDCO Opinion.

2.3.14. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-001715-PIP01-14-M03

Seqirus Netherlands B.V.; Influenza / Prevention of influenza infection

Day 60 opinion

Action: For adoption

Vaccines

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/0057/2019 of 25 February 2019).

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

2.4. Opinions on Re-examinations

2.4.1. 3-[5-[(1R,2S)-2-(2,2-difluoropropanoylamino)-1-(2,3-dihydro-1,4-benzodioxin-6-yl)propoxy]indazol-1-yl]-N-[(3R)-tetrahydrofuran-3-yl]benzamide (AZD7594) - EMEA-001976-PIP02-18

AstraZeneca AB; Asthma / Treatment of asthma

Day 30 opinion

Pneumology – Allergology

Summary of committee discussion:

Re-examination discussion

An oral explanation meeting (OEM) took place on 10 December 2019. The PDCO agreed at their October 2019 meeting a PIP. The applicant requested a revision, and the proposal of the applicant was supported. Nevertheless, some of the members did not agree with this conclusion and had divergent views, hence the PDCO agreed on a positive opinion on the re-examination by a majority of 23 votes out of 31 at their December 2019 meeting.

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. Teriflunomide - EMEA-C2-001094-PIP01-10-M04

Genzyme Europe B.V. / Sanofi-Aventis groupe; Treatment of multiple sclerosis

Day 1 letter

Neurology

2.7.2. Dupilumab - EMEA-C4-001501-PIP01-13-M06

Regeneron Pharmaceuticals, Inc.,; Treatment of atopic dermatitis

Day 1 letter

Dermatology

2.7.3. Eladocagene exuparvovec - EMEA-C1-002435-PIP01-18

PTC Therapeutics International Limited; Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency

Day 1 letter

Neurology

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. EMEA-002481-PIP01-18

Moderate to severe atopic dermatitis

Day 90 discussion

Dermatology

3.1.2. EMEA-002552-PIP01-19

Treatment of non-alcoholic steatohepatitis (NASH)

Day 90 discussion

Gastroenterology-Hepatology

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

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

Day 90 discussion

Haematology-Hemostaseology

3.1.4. CD34+enriched cells from patients with Fanconi anemia subtype A (FA-A) transduced ex vivo with lentiviral vector carrying the FANCA gene, - Orphan - EMEA-002578-PIP01-19

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

Day 90 discussion

Haematology-Hemostaseology

3.1.5. 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) - 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 90 discussion

Immunology-Rheumatology-Transplantation

3.1.6. Baricitinib - EMEA-001220-PIP05-19

Treatment of systemic lupus erythematosus / Treatment of systemic lupus erythematosus

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.7. Hematopoietic stem cells modified 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 (LAD-I)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.8. Zoliflodacin - EMEA-002599-PIP01-19

Treatment of gonococcal infection / Treatment of uncomplicated gonorrhoea

Day 90 discussion

Infectious Diseases

3.1.9. Tazobactam sodium / cefepime hydrochloride - EMEA-002483-PIP01-18

Treatment of complicated urinary tract infections (cUTI) or acute pyelonephritis

Day 90 discussion

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

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

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

Day 90 discussion

Neurology

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

Argenx BVBA; treatment of myasthenia gravis

Day 90 discussion

Neurology

3.1.12. EMEA-002572-PIP01-19

Chromosome 15q Duplication Syndrome, Cyclin-Dependent Kinase-Like 5 deficiency disorder / Treatment of seizures associated with Chromosome 15q Duplication Syndrome, Treatment of seizures associated with Cyclin-Dependent Kinase-Like 5 (CDKL5) deficiency disorder

Day 90 discussion

Neurology

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

Nightstar Europa Limited; Treatment of choroideremia

Day 90 discussion

Ophthalmology

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

Momenta Pharmaceuticals, Inc.; Myasthenia gravis

Day 90 discussion

Other

3.1.15. Rebisufligene etisparvovec - Orphan - EMEA-002206-PIP02-19

Abeona Therapeutics Inc.; Treatment of Mucopolysaccharidosis IIIA (ICD-10 E76.2) / Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome) in children from 6 months to less than 18 years of age

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.16. Naltrexone hydrochloride - EMEA-002670-PIP01-19

Treatment of Crohn's disease / in the treatment of moderate to severe active Crohn's disease as an adjuvant therapy in paediatric patients (from 6 years of age)

Day 60 discussion

Gastroenterology-Hepatology

3.1.17. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19

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

Day 60 discussion

Haematology-Hemostaseology

3.1.18. Evobrutinib - EMEA-002284-PIP02-19

Treatment of systemic lupus erythematosus

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.19. Ritonavir / darunavir - EMEA-002537-PIP02-19

Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Infectious Diseases

3.1.20. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19

SIGA Technologies, Inc.; Orthopoxvirus disease (smallpox, monkeypox, cowpox, and vaccinia complications)

Day 60 discussion

Infectious Diseases

3.1.21. 1-(2,2-DIPHENYLTETRAHYDROFURAN-3-YL)-N,N-DIMETHYLMETHANAMINE HYDROCHLORIDE - Orphan - EMEA-002688-PIP01-19

Anavex Germany GmbH; Rett Syndrome

Day 60 discussion

Other

3.1.22. Fasinumab - EMEA-002059-PIP02-19

Chronic pain / Chronic musculoskeletal pain / Chronic non-musculoskeletal pain / Treatment of chronic cancer pain in a palliative care setting / Treatment of moderate to severe chronic pain associated with osteoarthritis (OA) of the knee or hip in patients who achieve an inadequate response to or are intolerant to currently available analgesics (Adults only)

Day 60 discussion

Pain

3.1.23. Sodium alginate oligosaccharide - Orphan - EMEA-002321-PIP01-17

AlgiPharma AS; Symptomatic treatment of cystic fibrosis

Day 60 discussion

Pneumology - Allergology

3.1.24. EMEA-002653-PIP01-19

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

Day 60 discussion

Psychiatry

3.1.25. Ezetimibe / Rosuvastatin - EMEA-002257-PIP02-19

Prevention of cardiovascular events

Indicated to reduce the risk of cardiovascular events as substitution therapy in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS), who are adequately controlled with the individual substances given concurrently at the same dose level as in the fixed dose combination, but as separate products

Day 30 discussion

Cardiovascular Diseases

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

CUTISS AG; Treatment of burns

Day 30 discussion

Dermatology

3.1.27. Nemolizumab - EMEA-001624-PIP02-19

Prurigo Nodularis

Day 30 discussion

Dermatology

3.1.28. C-type natriuretic peptide conjugated to a multi-arm polyethylene glycol carrier molecule through a cleavable linker - EMEA-002689-PIP01-19

Achondroplasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.29. Gefapixant - EMEA-002267-PIP03-19

Treatment of endometriosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.30. Lonapegsomatropin - EMEA-002692-PIP01-19

Growth hormone deficiency

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Gastroenterology-Hepatology

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

Albireo AB; Biliary atresia

Day 30 discussion

Gastroenterology-Hepatology

3.1.33. 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 intermedia and major

Day 30 discussion

Haematology-Hemostaseology

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

Treatment of immune thrombocytopenia

Day 30 discussion

Haematology-Hemostaseology

3.1.35. 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 30 discussion

Haematology-Hemostaseology

3.1.36. Mitapivat - EMEA-002684-PIP01-19

Pyruvate Kinase Deficiency / Treatment of paediatric patients with Pyruvate Kinase Deficiency

Day 30 discussion

Haematology-Hemostaseology

3.1.37. 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 30 discussion

Immunology-Rheumatology-Transplantation

3.1.38. 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 30 discussion

Immunology-Rheumatology-Transplantation

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

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.40. Artesunate - EMEA-002710-PIP01-19

Malaria

Day 30 discussion

Infectious Diseases

3.1.41. EMEA-002694-PIP01-19

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

Day 30 discussion

Infectious Diseases

3.1.42. EMEA-002693-PIP01-19

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

Day 30 discussion

Infectious Diseases

3.1.43. Pritelivir - EMEA-002180-PIP02-19

Treatment of herpes simplex virus disease

Day 30 discussion

Infectious Diseases

3.1.44. Carbidopa / levodopa - EMEA-002687-PIP01-19

Parkinson’s disease

Day 30 discussion

Neurology

3.1.45. Elezanumab - EMEA-002675-PIP01-19

Multiple Sclerosis

Day 30 discussion

Neurology

3.1.46. 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 30 discussion

Neurology

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

Biohaven Pharmaceuticals, Inc.; Multiple system atrophy

Day 30 discussion

Neurology

3.1.48. EMEA-002686-PIP01-19

Treatment of breast Cancer

Day 30 discussion

Oncology

3.1.49. EMEA-002690-PIP01-19

Treatment of non-small cell lung cancer

Day 30 discussion

Oncology

3.1.50. Daratumumab - Orphan - EMEA-002152-PIP03-19

Janssen-Cilag International N.V.; Systemic light chain amyloidosis

Day 30 discussion

Oncology

3.1.51. Eftilagimod alpha - EMEA-002698-PIP01-19

Treatment of breast tumours / Treatment of benign breast tumours, Treatment of primary breast cancer, Treatment of secondary breast cancer (primary cancer metastasizing to the breast)

Day 30 discussion

Oncology

3.1.52. 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 30 discussion

Oncology

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

Incyte Biosciences Distribution B.V.; Treatment of Mature B-cell neoplasm

Day 30 discussion

Oncology

3.1.54. 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, and diffuse large B-cell lymphoma (DLBCL).

Day 30 discussion

Oncology

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

Astellas Pharma Europe B.V.; Treatment of gastric and gastroesophageal junction adenocarcinoma / Treatment of pancreatic adenocarcinoma

Day 30 discussion

Oncology

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

Blueprint Medicines (Netherlands) B.V.; Treatment of mastocytosis / Treatment of indolent systemic mastocytosis, Treatment of smoldering systemic mastocytosis

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.57. Brolucizumab - EMEA-002691-PIP01-19

Central Retinal Vein Occlusion (CRVO), Branch Retinal Vein Occlusion (BRVO)

Day 30 discussion

Ophthalmology

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

Momenta Pharmaceuticals, Inc.; Autoimmune haemolytic anaemia

Day 30 discussion

Other

3.1.59. EMEA-002668-PIP01-19

Treatment of chronic pain in patients, who have not sufficiently responded to different analgesic therapies incl. at least one opioid (adult population only)

Day 30 discussion

Pain

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. Bimekizumab - EMEA-C1-002189-PIP01-17-M01

UCB Biopharma SPRL; Treatment of Psoriasis

Day 30 discussion

Dermatology

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

Gilead Sciences International Ltd.; Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.2.3. Itacitinib - EMEA-C2-002178-PIP01-17-M01

Incyte Biosciences Distribution B.V.; Treatment of acute Graft versus Host Disease

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

TETEC AG; Treatment of cartilage disorders

Day 30 discussion

Other

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. 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) / For the treatment of paediatric patients with NTRK fusion-positive solid tumours

Day 30 discussion

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

Eli Lilly Nederland B.V.; Treatment of psoriasis

Day 30 discussion

Dermatology

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

Galderma International S.A.; Atopic dermatitis / Treatment of moderate to severe atopic dermatitis not adequately controlled with topical treatments

Day 30 discussion

Dermatology

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

AstraZeneca AB; E11 Type 2 Diabetes / Treatment of Type 2 Diabetes

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Amgen Europe B.V.; Treatment of Osteoporosis / Treatment of osteogenesis imperfecta, Treatment of glucocortcoid induced osteoporosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Regeneron Ireland DAC; Treatment of elevated cholesterol / Treatment of homozygous familial hypercholesterolemia (HoFH)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

AstraZeneca AB; Hyperkalemia / Treatment of hyperkalemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Aeterna Zentaris GmbH; Growth hormone deficiency / Diagnosis of growth hormone deficiency

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic

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

Swedish Orphan Biovitrum AB (publ); Hereditary Factor IX Deficiency - D67

Day 30 discussion

Haematology-Hemostaseology

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

Allergan Pharmaceuticals International Limited; Irritable bowel syndrome with diarrhoea

Day 30 discussion

Gastroenterology-Hepatology

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

Swedish Orphan Biovitrum AB (publ); Hereditary factor IX deficiency - D67

Day 30 discussion

Haematology-Hemostaseology

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

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

Day 30 discussion

Haematology-Hemostaseology

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

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Novimmune BV; Treatment of Haemophagocytic Lymphohistiocytosis

Day 30 discussion

Immunology-Rheumatology-Transplantation

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

Merck Sharp & Dohme (Europe), Inc.; Treatment of Clostridium difficile infection / ZINPLAVA is indicated for the prevention of recurrence of Clostridium difficile infection (CDI) in paediatric patients at high risk for recurrence of CDI

Day 30 discussion

Infectious Diseases

3.3.16. Nirsevimab - anti-respiratory syncytial virus human IgG1κ monoclonal antibody - EMEA-001784-PIP01-15-M02

AstraZeneca AB; Prevention of respiratory syncytial viral infections

Day 30 discussion

Infectious Diseases

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

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections / Treatment of invasive fungal infections / For treatment of invasive fungal infections in the following paediatric patients: Invasive aspergillosis in patients with disease that is refractory to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products / Treatment of Invasive Aspergillosis / Patients receiving remission-induction chemotherapy for acute myelogenous leukemia (AML) or myelodysplastic syndromes (MDS) expected to result in prolonged neutropenia and who are at high risk of developing invasive fungal infections / Hematopoietic stem cell transplant (HSCT) recipients who are undergoing high-dose immunosuppressive therapy for graft versus host disease and who are at high risk of developing invasive fungal infections

Day 30 discussion

Infectious Diseases

3.3.18. Tenofovir alafenamide - EMEA-001584-PIP01-13-M05

Gilead Sciences International Ltd.; Treatment of chronic Hepatitis B

Day 30 discussion

Infectious Diseases

3.3.19. Vaborbactam / meropenem - EMEA-001731-PIP01-14-M01

Menarini International Opernations Luxembourg S.A.; Treatment of Gram-negative bacterial infections

Day 30 discussion

Infectious Diseases

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

Alexion Europe SAS; Neuromyelitis optica spectrum disorders / Treatment of paediatric patients with relapsing neuromyelitis optica spectrum disorders

Day 30 discussion

Neurology

3.3.21. 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 (AADC) deficiency

Day 30 discussion

Neurology

3.3.22. Ozanimod - EMEA-001710-PIP02-14-M04

Celgene Europe B.V.; Treatment of multiple sclerosis / Treatment of relapsing remitting forms of multiple sclerosis

Day 30 discussion

Neurology

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

Eisai Europe Limited; Treatment of treatment-resistant epilepsies / Adjunctive therapy in patients with other paediatric epilepsies, Adjunctive therapy in patients with refractory partial onset seizures including secondarily generalised seizures

Day 30 discussion

Neurology

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

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 30 discussion

Neurology

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

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue), Treatment of acute lymphoblastic leukaemia, Treatment of acute myeloid leukaemia. / Treatment of children with a solid malignant tumour and which is newly-diagnosed and metastatic, or refractory to first-line treatment, Treatment of children with first relapse of, or with frontline-refractory acute myeloid leukaemia, Treatment of children with first relapse of, or with frontline-refractory acute lymphoblastic leukaemia.

Day 30 discussion

Oncology

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

Sun Pharmaceutical Industries Europe B.V.; Basal cell carcinoma / Medulloblastoma

Day 30 discussion

Oncology

3.3.27. Burosumab - Human recombinant IgG1 monoclonal antibody to fibroblast growth factor 23 (FGF23); - Orphan - EMEA-001659-PIP01-15-M04

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

Day 30 discussion

Other

3.3.28. Meloxicam / Bupivacaine - EMEA-002246-PIP01-17-M01

Heron Therapeutics B.V.; Acute Postoperative Pain

Day 30 discussion

Pain

3.3.29. Ravulizumab (ALXN1210) - Orphan - EMEA-001943-PIP01-16-M04

Alexion Europe SAS; Atypical Haemolytic Uremic Syndrome / Treatment of atypical Haemolytic Uremic Syndrome

Day 30 discussion

Uro-nephrology / Haematology-Hemostaseology

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

Seqirus UK Limited; Influenza / Prevention of influenza

Day 30 discussion

Vaccines

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

Sanofi Pasteur; Prevention of dengue disease

Day 30 discussion

Vaccines

3.3.32. Neisseria meningitidis serogroup B recombinant lipoprotein (rLP2086; subfamily B; Escherichia coli) / Neisseria meningitidis serogroup B recombinant lipoprotein (rLP2086; subfamily A; Escherichia coli) - EMEA-001037-PIP02-11-M06

Pfizer Europe MA EEIG; Invasive meningococcal disease caused by N meningitidis serogroup B.

Day 30 discussion

Vaccines

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

Sanofi Pasteur; Prevention of influenza infection

Day 30 discussion

Vaccines

4. Nominations

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

4.1. List of letters of intent received for submission of applications with start of procedure 28 January 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.

No items

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

No items

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

No items

9.2. Coordination with EMA Scientific Committees or CMDh-v

No items

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Summary of committee discussion:

The chair of the Non-clinical Working Group identified the products which will require Non-clinical Working Group evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

Relevant products for FWG discussion were identified.

9.4. Cooperation within the EU regulatory network

No items

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

No items

9.7. PDCO work plan

9.7.1. PDCO Work Plan 2020

PDCO Chair: Koenraad Norga

Summary of committee discussion:

The PDCO workplan was discussed and the topics for the next year have been endorsed by the PDCO.

9.8. Planning and reporting

9.8.1. Marketing authorisation applications (MAA) forecast for 2019 – planning update dated Q4 2019

Summary of committee discussion:

A presentation on the forecast of new initial marketing authorisation applications was tabled for information.

10. Any other business

10.1.1. Update on EMA relocation

An update on the relocation of the EMA from the Spark building to the new permanent location in Amsterdam Zuidas was presented to the PDCO.

11. Breakout sessions

No items

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 09-11 December 2019 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium	When chairing the meeting: To be replaced for discussions, final deliberations and voting When not chairing the meeting: No participation in final deliberations and voting	- 2.1.11. - EMEA-002515-PIP01-18; - 2.3.14. Vilanterol trifenatate / umeclidinium bromide / fluticasone furoate - EMEA-002153-PIP01-17-M01;
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
Petra Dominikova	Alternate	Czech Republic	No interests declared	
Kirstine Moll Harboe	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Ann Marie Totterman	Member	Finland	No interests declared	
Pia Annunen	Alternate	Finland	No participation in final deliberations and voting on:	- 2.3.8. Nivolumab - EMEA-001407-PIP01-12-M02; - 2.3.9. Nivolumab - EMEA-001407-PIP02-15-M03
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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	N/A
Sigita Burokiene	Member	Lithuania	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roel Bolt	Member	Netherlands	No interests declared	
Maaike van Dartel	Alternate	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No interests declared	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
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 final deliberations and voting on:	- 3.3.16 Nirsevimab
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 final deliberations and voting on:	- 3.3.16 Nirsevimab

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Günter Karl-Heinz Auerswald	Member	Patients' Organisation Representative	No participation in final deliberations and voting on:	- 3.3.11 eftrenonacog alfa
Paola Baiardi	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	N/A
Michal Odermarsky	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	N/A
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Johanna Lähteenvuo	Expert - via telephone*	Finland	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/