27 March 2020
EMA/PDCO/164431/2020

Paediatric Committee (PDCO)

Minutes for the meeting on 24-27 March 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

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 these minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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

Table of contents

1. Introductions

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

At the beginning of the meeting participants were informed that EMA has invoked the Business Continuity Plan and exceptional measures have been taken to protect the staff members and all delegates, experts and members of the Committee. Amongst those measures, it was decided to hold a virtual meeting. The participants had no objection to hold the meeting and to take decisions (by consensus or by voting) in such a way.

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. 22 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

Due to restricted involvement, the Chair Koenraad Norga was replaced by the vice-chair Sabine Scherer for the discussion on agenda topic 2.3.19, 3.1.4, 3.1.5, 3.3.20.

1.2. Adoption of agenda

PDCO agenda for 24-27 March 2020

The agenda of the PDCO meeting 24th-27th March was adopted.

1.3. Adoption of the minutes

PDCO minutes for 25-28 February 2020

The minutes of the PDCO 25th-28th February were adopted and will be published on the EMA website.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Livoletide - Orphan - EMEA-002455-PIP01-18

Millendo Therapeutics SAS; Treatment of Prader-Willi syndrome

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO discussed in March 2020 the responses received by the applicant on the points raised at D90. In conclusion the PDCO recommended granting a PIP for livoletide in the treatment of Prader-Willi Syndrome with a waiver on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.2. Cyclophosphamide - EMEA-002644-PIP01-19

Accord Healthcare S.L.U.; Treatment of all malignant neoplasms

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the D90 conclusion.

Overall the PDCO expressed a positive Opinion for this PIP, a PUMA, for the condition of treatment of all malignant neoplasms, for all subsets of the paediatric population.

2.1.3. Imatinib - EMEA-002643-PIP01-19

Accord Healthcare S.L.U.; Treatment of acute lymphoblastic leukaemia / Treatment of chronic myeloid leukaemia

Day 120 opinion

Oncology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's paediatric investigation plan to support a future PUMA application for imatinib for the treatment of chronic myeloid leukaemia (CML) and the treatment of acute lymphoblastic leukaemia (ALL).

The development is focused on children and adolescents, and consists of a quality measure, as well as two clinical measures, namely a comparative bioavailability study in healthy adult volunteers and an open-label trial to evaluate acceptability and palatability in children and adolescents with leukaemia for whom a treatment with imatinib is indicated. As this is a PUMA PIP, the development is not deferred and due to complete later this year.

2.1.4. Rosuvastatin calcium / fenofibrate / - EMEA-002743-PIP01-19

Accord Healthcare S.L.U.; Treatment of mixed dyslipidaemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for rosuvastatin (calcium) / fenofibrate for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of mixed dyslipidaemia.

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.

2.1.5. Hydrocortisone (acetate) / benzocaine / - EMEA-002739-PIP01-19

FAES FARMA, S.A.; Treatment of haemorrhoidal disease

Day 60 opinion

Gastroenterology-Hepatology

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 hydrocortisone (acetate) / benzocaine for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of haemorrhoidal disease.

The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.6. Arfolitixorin - EMEA-002223-PIP01-19

Isofol Medical AB; Treatment of colorectal cancer

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO’s view expressed at D30 was re-discussed and endorsed.

Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for arfolitixorin for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of colorectal cancer.

2.1.7. Propan-2-yl 2-[5-(acryloylamino)-4-{[2-(dimethylamino)ethyl](methyl)amino}-2-methoxyanilino]-4-(1methyl-1H-indol-3-yl)pyrimidine-5-carboxylate (TAK-788) - EMEA-002716-PIP01-19

Takeda Pharma A/S; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this waiver application for an EGFR/HER inhibitor requested for a broad solid tumour condition. The committee recapitulated its D30 view, which was to consider a conservative approach, favouring a narrow condition, in line with the adult indication, of treatment of lung cancer, in order not to close any paediatric development efforts in the future.

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 Propan-2-yl2-[5-(acryloylamino)-4-{[2-(dimethylamino)ethyl](methyl)amino}-2-methoxyanilino]-4-(1methyl-1H-indol-3-yl)pyrimidine-5-carboxylate (TAK-788) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms).
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.

2.1.8. Tiragolumab - EMEA-002721-PIP01-19

Roche Registration GmbH; Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 60 opinion

Oncology

Summary of committee discussion:

A request for modification was adopted in line with the Day 30 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 Tiragolumab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of lung carcinoma (small cell and non-small cell carcinoma).

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.

2.1.9. Idasanutlin - Orphan - EMEA-001489-PIP02-19

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

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the conclusions at D30.

The committee overall agreed to a positive Opinion at D60 for this PIP, with a deferral, for all subsets of the paediatric population, for treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue); split from the initial PIP 1489-PIP01.

2.1.10. Spesolimab - EMEA-002475-PIP02-19

Prevention of generalized pustular psoriasis / Treatment of generalized pustular psoriasis

Day 60 opinion

Dermatology

Summary of committee discussion:

The applicant provided a clarification following the D30 discussion. The applicant's response was considered acceptable and a positive opinion was adopted on D60 for spesolimab in the prevention and treatment of generalised pustular psoriasis.

2.1.11. Difelikefalin - EMEA-002565-PIP02-19

Vifor Fresenius Medical Care Renal Pharma France; Treatment of chronic kidney disease associated pruritus

Day 60 opinion

Other

Summary of committee discussion:

The PDCO adopted a positive opinion at Day 60 on this PIP for the treatment of chronic kidney disease (CKD)-associated pruritus in children from 12 years of age, as well as on a waiver based on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). Following additional information provided by the applicant regarding the study timelines the PDCO agreed to defer both initiation and completion of the paediatric clinical study in order not to delay the adult marketing authorisation.

2.1.12. Ibrexafungerp - EMEA-002535-PIP03-19

SCYNEXIS, Inc.; Prevention of recurrent vulvovaginal candidiasis / Treatment of vulvovaginal candidiasis

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO agreed on a positive opinion for a PIP for the treatment of vulvovaginal candidiasis and for the prevention of recurrent vulvovaginal candidiasis for girls from menarche to less than 18 years of age. A waiver was granted for boys on the grounds that the disease does not occur in boys and for pre-menarche girls due to lack of significant therapeutic benefit.

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. Lonafarnib - EMEA-C-002516-PIP01-18

EigerBio Europe Limited; For the treatment of Hutchinson-Gilford progeria syndrome (HGPS) and progeroid laminopathies

Day 1 opinion

Summary of committee discussion:

The PDCO discussed the compliance check request along with the assessors' comments and adopted on 27 March 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision.

2.2.2. Romiplostim - EMEA-C-000653-PIP01-09-M05

Amgen Europe B.V.; Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura) / Treatment of disease-related thrombocytopenia in myelodysplastic syndrome

Day 30 opinion

Haematology-Hemostaseology

Summary of committee discussion:

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

- EMEA-C1-000653-PIP01-09-M04
- EMEA-C2-000653-PIP01-09-M04

The PDCO adopted on 27 March 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0233/2017) of 11 August 2017.

2.2.3. Mometasone (furoate monohydrate) / olopatadine (hydrochloride) (GSP 301 NS) - EMEA-C-002514-PIP01-18

Glenmark Pharmaceuticals Europe Ltd.; Treatment of allergic rhinitis / rhino-conjunctivitis

Day 30 opinion

Oto-rhino-laryngology

Summary of committee discussion:

The PDCO discussed the compliance check request and considered that the completed studies are compliant with the latest PIP.

The Committee adopted a positive opinion at Day 30.

2.2.4. Erenumab - EMEA-C2-001664-PIP02-15-M03

Novartis Europharm Limited; Prevention of migraine headaches

Day 30 letter

Neurology

Summary of committee discussion:

The PDCO discussed the completed study.

The PDCO considered that the Study is compliant with the latest Agency's Decision (P/0107/2019) of 22/3/2019.

The PDCO finalised this partially completed compliance procedure on 27/3/2020.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Crisaborole - EMEA-002065-PIP01-16-M02

Pfizer Europe MA EEIG; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of committee discussion:

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

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0101/2018 of 15 March 2018).

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

2.3.2. Avalglucosidase alfa - Orphan - EMEA-001945-PIP01-16-M02

Genzyme Europe B.V.; Treatment of Pompe disease

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/0073/2019 of 22 March 2019).

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

2.3.3. Dulaglutide - EMEA-000783-PIP01-09-M05

Eli Lilly and Company; Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO confirmed the outcome of the Day 30 discussion and agreed with the changes to the key elements.

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/0227/2016 of 26/08/2016).

2.3.4. Romosozumab - EMEA-001075-PIP04-15-M02

UCB Pharma S.A.; Treatment of osteoporosis

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

In March 2020 the PDCO discussed the responses of the applicant to the issues raised at D30. The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0247/2018 of 15/8/2018).

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

2.3.5. Volanesorsen - Orphan - EMEA-001915-PIP01-15-M02

Akcea Therapeutics; Treatment of familial chylomicronemia syndrome

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO re-discussed this procedure at Day 60 during the March 2020 plenary meeting.

The PDCO therefore adopted a favourable Opinion on this modification of the agreed PIP as set out in the Agency's latest decision (P/0404/2019 of 4 December 2019).

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

2.3.6. Alicaforsen (sodium salt) - Orphan - EMEA-002060-PIP02-17-M01

Atlantic Healthcare Europe B.V.; Treatment of pouchitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The Committee discussed the clarification received from the applicant before D60.

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/0265/2018 of 1/10/2018).

2.3.7. Tofacitinib - EMEA-000576-PIP03-12-M03

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO view expressed at Day 30 was re-discussed and endorsed.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0071/2019 of 22 March 2019).

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

2.3.8. 2-iminobiotin - Orphan - EMEA-001070-PIP01-10-M02

Neurophyxia BV; Treatment of perinatal asphyxia

Day 60 opinion

Neonatology - Paediatric Intensive Care

Summary of committee discussion:

The applicant's responses to the D60 issues were considered acceptable.

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

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/035/2012 of 3 February 2012).

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

2.3.9. Isoflurane - EMEA-002320-PIP01-17-M01

Sedana Medical AB; Sedation of mechanically ventilated patients

Day 60 opinion

Neonatology - Paediatric Intensive Care

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, including the correction of the definition of the pharmaceutical form.

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.

2.3.10. Dimethyl fumarate - EMEA-000832-PIP01-10-M05

Biogen Idec Ltd.; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of committee discussion:

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

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

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

2.3.11. Pitolisant - Orphan - EMEA-001176-PIP01-11-M04

BIOPROJET PHARMA; Treatment of narcolepsy

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.

2.3.12. Setmelanotide - Orphan - EMEA-002209-PIP01-17-M01

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

Day 60 opinion

Nutrition

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/0164/2018 of 15/06/2018).

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

2.3.13. Afatinib - EMEA-001596-PIP02-17-M02

Boehringer Ingelheim International GmbH; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue neoplasms) / Treatment of malignant neoplasms of the central nervous system

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO views expressed at D30 were endorsed and the opinion finalised taking also into account the comments received by the applicant on the draft opinion.

In conclusion, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0235/2019 of 16 July 2019).

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

2.3.14. Talimogene laherparepvec - EMEA-001251-PIP01-11-M04

Amgen Europe B.V.; Treatment of solid malignant non-CNS tumours

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the modification request for talimogene laherparepvec considering also the additional clarifications provided by the applicant after the D30 discussion and the comments received by the applicant on the draft opinion.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0087/2017 of 24 March 2017).

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

2.3.15. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M02

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukemia

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO re-discussed the proposed modifications taking into account the clarifications provided by the applicant after the D30 discussion and input received by experts of the NcWG.

All the pending issues identified at D30 were considered solved.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0194/2019 of 15 May 2019).

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

2.3.16. Ivacaftor - Orphan - EMEA-000335-PIP01-08-M14

Vertex Pharmaceuticals (Ireland) Ltd; Treatment of cystic fibrosis

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 most but not 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/0353/2018 of 22 November 2018).

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

2.3.17. Rolapitant - EMEA-001768-PIP02-15-M03

Tesaro Bio Netherlands B.V.; prevention of nausea and vomiting

Day 60 opinion

Other

Summary of committee discussion:

The PDCO's views expressed at D30 were endorsed and the extension of the waiver to entire paediatric population was agreed based on the grounds that the specific medicinal product is likely to be unsafe.

In conclusion the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0085/2018 of 16 March 2018).

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

2.3.18. Methoxyflurane - EMEA-000334-PIP01-08-M09

Medical Developments UK Ltd; Treatment of acute pain

Day 60 opinion

Pain

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, including the supplementary information received after 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. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Tezavaftor / ivacaftor / - Orphan - EMEA-001640-PIP01-14-M06

Vertex Pharmaceuticals (Europe) Ltd.; Treatment of cystic fibrosis

Day 60 opinion

Pneumology – Allergology

Summary of committee discussion:

Based on the review and the committee's day 30 discussion of the rationale submitted by the application for modifying the agreed paediatric investigation plan and the applicant's additional clarifications, the PDCO considered that the 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/0250/2019 of 17 July 2019).

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

2.3.20. Influenza virus surface antigens - A/turkey/Turkey/1/05 (H5N1) - EMEA-000599-PIP01-09-M07

Seqirus S.r.l.; Prevention of influenza

Day 60 opinion

Vaccines

Summary of committee discussion:

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

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0249/2018 of 15 August 2018).

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

2.3.21. Pandemic influenza vaccine (H5N1) (surface antigen, inactivated, adjuvanted) - EMEA-001830-PIP01-15-M02

Seqirus S.r.l.; Prevention of influenza

Day 60 opinion

Vaccines

Summary of committee discussion:

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

paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0260/2018 of 15 August 2018).

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

2.3.22. Roxadustat - EMEA-001557-PIP01-13-M04

Astellas Pharma Europe B.V.; Treatment of anaemia due to chronic disorders

Day 30 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the request and considered that the delay of Study is acceptable.

The PDCO therefore adopted at Day 30 a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0146/2019 of 17 April 2019).

2.4. Opinions on Re-examinations

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

Aeterna Zentaris GmbH; Diagnosis of growth hormone deficiency

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic

Summary of committee discussion:

On 12th March 2020 the applicant submitted their detailed grounds for re-examination as regards the currently agreed PIP Opinion for procedure EMEA-001988-PIP01-16-M01 from 31/01/2020, for macimorelin (synthetic mimetic of the growth hormone secretagogue ghrelin) for the diagnosis of growth hormone deficiency.

In conclusion, the PDCO adopted a positive Opinion following this re-examination procedure, along the lines of the above discussion on the modification of the agreed PIP as set in the Agency's latest decision (P/0105/2017 of 11/04/2017).

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

2.5. 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. 6-cyclopropaneamido-4-{[2-methoxy-3-(1-methyl-1H-1,2,4 triazol-3-yl)phenyl]amino}-N-(2H3)methylpyridazine-3-carboxamide (BMS-986165) - EMEA-C1-002350-PIP01-18

Bristol-Myers Squibb International Corporation; Treatment of psoriasis

Day 1 letter

Dermatology

2.7.2. Upadacitinib - EMEA-C2-001741-PIP01-14-M02

AbbVie Ltd; Treatment of chronic idiopathic arthritis (including rheumathoid arthritis, psoriatic arthritis, spondyloarthritis and juvenile idiopathic arthritis)

Day 1 letter

Immunology-Rheumatology-Transplantation

2.7.3. Linagliptin - EMEA-C1-000498-PIP01-08-M08

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 1 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.4. Lumasiran sodium - EMEA-C1-002079-PIP01-16-M01

Alnylam UK Limited; Treatment hyperoxaluria

Day 1 letter

Uro-nephrology

2.8. Revision of PDCO Opinions

No items

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. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19

Alexion Europe S.A.S.; Wilson disease

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Concizumab - Orphan - EMEA-002326-PIP03-18

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

Day 90 discussion

Haematology-Hemostaseology

3.1.3. 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 90 discussion

Haematology-Hemostaseology

3.1.4. Gepotidacin - EMEA-002443-PIP01-18

Treatment of uncomplicated urinary tract infection (uUTI) / Treatment of uncomplicated urinary tract infection (acute cystitis) in children aged >2 years to <18 years

Day 90 discussion

Infectious Diseases

3.1.5. Gepotidacin - EMEA-002443-PIP02-18

Treatment of uncomplicated urogenital gonorrhea (GC) / Treatment of uncomplicated urogenital gonorrhea in children ≥ 12 to <18 years

Day 90 discussion

Infectious Diseases

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

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

Day 90 discussion

Neurology

3.1.7. Autologous inactivated glioma cells - Orphan - EMEA-002663-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma

Day 90 discussion

Oncology

3.1.8. Autologous inactivated glioma cells - Orphan - EMEA-002662-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma Day 90 discussion

Oncology

3.1.9. Autologous inactivated glioma cells - Orphan - EMEA-002664-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma Day 90 discussion

Oncology

3.1.10. Autologous inactivated glioma cells - Orphan - EMEA-002661-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma

Day 90 discussion

Oncology

3.1.11. Lenvatinib - EMEA-001119-PIP03-19

Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue

Day 90 discussion

Oncology

3.1.12. Trilaciclib - EMEA-002534-PIP02-19

Prevention of chemotherapy induced myelosuppression

Day 90 discussion

Oncology

3.1.13. 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 90 discussion

Ophthalmology

3.1.14. Atropine - EMEA-002545-PIP01-19

Myopia

Day 90 discussion

Ophthalmology

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

AlgiPharma AS; Symptomatic treatment of cystic fibrosis

Day 90 discussion

Pneumology – Allergology

3.1.16. Macitentan - Orphan - EMEA-001032-PIP03-19

Janssen-Cilag International N.V.; Fontan-palliated patients

Day 60 discussion

Cardiovascular Diseases

3.1.17. Cotadutide - EMEA-002712-PIP01-19

Treatment of non-cirrhotic non-alcoholic steatohepatitis (NASH) or non-alcoholic fatty liver disease (NAFLD) / For the resolution of steatohepatitis with no worsening of fibrosis in obese children and adolescents with non-cirrhotic non-alcoholic steatohepatitis (NASH) and non-alcoholic fatty liver disease (NAFLD)

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. DTX401 - Orphan - EMEA-002734-PIP01-19

Ultragenyx Germany GmbH; Treatment of glycogen storage disease type Ia

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. Etrasimod L-arginine - EMEA-002713-PIP01-19

Treatment of ulcerative colitis / Treatment of moderately or severely active ulcerative colitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.20. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the *BCL11A* gene - Orphan - EMEA-002730-PIP01-19

Vertex Pharmaceuticals (Ireland) Limited; Treatment of beta-thalassemia / Treatment of transfusion-dependent beta-thalassemia

Day 60 discussion

Haematology-Hemostaseology

3.1.21. Etranacogene dezaparvovec - Orphan - EMEA-002722-PIP01-19

uniQure biopharma B.V.; Treatment of haemophilia B

Day 60 discussion

Haematology-Hemostaseology

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

Treatment of hereditary angioedema

Day 60 discussion

Haematology-Hemostaseology

3.1.23. Adeno-associated virus serotype rh74 containing a human micro-dystrophin gene - EMEA-002677-PIP01-19

Duchenne muscular dystrophy

Day 60 discussion

Neurology

3.1.24. Diroximel - EMEA-002685-PIP02-19

Treatment of multiple sclerosis

Day 60 discussion

Neurology

3.1.25. Padsevonil - EMEA-002466-PIP02-19

Treatment of fixation off sensitivity (FOS) in patients with epilepsy / Adjunctive treatment of FOS in paediatric patients with epilepsy

Day 60 discussion

Neurology

3.1.26. 17-mer, 2'-O-methyl modified phosphorothioate RNA oligonucleotide - Orphan - EMEA-002717-PIP01-19

ProQR Therapeutics; Treatment of inherited retinal disorders / Treatment of Leber's congenital amaurosis due to the p.Cys998X mutation (C2991 +1655A>G) in the CEP290 gene

Day 60 discussion

Ophthalmology

3.1.27. Recifercept - Orphan - EMEA-002715-PIP01-19

Pfizer Europe MA EEIG; Treatment of achondroplasia

Day 60 discussion

Other

3.1.28. EMEA-002731-PIP01-19

Treatment of schizophrenia

Day 60 discussion

Psychiatry

3.1.29. Chloroprocaine - EMEA-000639-PIP06-20

Ocular surface anaesthesia

Day 30 discussion

Anaesthesiology

3.1.30. Ezetimibe / atorvastatin - EMEA-002649-PIP02-20

Prevention of cardiovascular events / Atorvastatin/ezetimibe is indicated as substitution therapy to reduce the risk of cardiovascular events in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS), for adults receiving atorvastatin and ezetimibe concurrently at the same dose level

Day 30 discussion

Cardiovascular Diseases

3.1.31. Bilastine - EMEA-000347-PIP05-20

Acute urticaria / Short-term treatment as single therapy or in severe cases as additional therapy option of histamine-mediated type I hypersensitivity reactions, such as acute urticaria, when immediate action is required or parenteral formulation is preferred

Day 30 discussion

Dermatology / Pneumology - Allergology / Oto-rhino-laryngology

3.1.32. Semaglutide - EMEA-001441-PIP04-20

Treatment of non-alcoholic fatty liver disease (NAFLD)

Day 30 discussion

Gastroenterology-Hepatology

3.1.33. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the *BCL11A* gene - Orphan - EMEA-002730-PIP02-19

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

Day 30 discussion

Haematology-Hemostaseology

3.1.34. N-[(2S)-5-{[(1R, 2S)-2-(4-fluorophenyl)cyclopropyl] amino}-1-(4-methylpiperazin-1-yl)-1-oxopentan-2-yl]-4-(1H-1,2,3-triazol-1-yl)benzamide, bis-tosylate salt - Orphan - EMEA-002752-PIP01-19

Imago Biosciences BV; Treatment of myeloproliferative neoplasms

Day 30 discussion

Haematology-Hemostaseology

3.1.35. Bimezikumab - EMEA-002189-PIP03-19

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

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.36. EMEA-002742-PIP01-19

Treatment of parainfluenza infection

Day 30 discussion

Infectious Diseases

3.1.37. Adeno-associated virus serotype 2 (AAV2) encoding human aromatic L-amino acid decarboxylase (hAADC) - EMEA-002753-PIP01-19

Parkinson's disease

Day 30 discussion

Neurology

3.1.38. 1-[({4-[(4-fluoro-2-methyl-1H-indol-5-yl)oxy]-6-methoxyquinolin-7-yl}oxy)methyl]cyclopropan-1-amine - Orphan - EMEA-002486-PIP03-20

Advenchen Laboratories, LLC.; Treatment of soft tissue sarcomas / Treatment of alveolar soft part sarcoma / Treatment of Synovial sarcoma

Day 30 discussion

Oncology

3.1.39. Monalizumab - EMEA-002751-PIP01-19

Head and neck epithelial malignant neoplasms

Day 30 discussion

Oncology

3.1.40. Ribociclib - EMEA-002765-PIP01-19

Treatment of breast cancer

Day 30 discussion

Oncology

3.1.41. Atropine - EMEA-002744-PIP01-19

Myopia / Treatment to slow myopia progression

Day 30 discussion

Ophthalmology

3.1.42. EMEA-002748-PIP01-20

Secondary lymphedema associated with the treatment of breast cancer

Day 30 discussion

Other

3.1.43. Rituximab / CD3+CD4+CD25+CD127-FoxP3+ regulatory T cells - EMEA-002737-PIP01-19

Treatment of type 1 diabetes mellitus (T1DM)

Day 30 discussion

Other / Endocrinology-Gynaecology-Fertility-Metabolism

3.1.44. Betahistine - EMEA-002652-PIP01-19

Acute peripheral vertigo

Day 30 discussion

Oto-rhino-laryngology / Neurology

3.1.45. Dexmedetomidine - EMEA-002758-PIP01-19

Treatment of acute agitation in bipolar disorder / Treatment of acute agitation in schizophrenia

Day 30 discussion

Psychiatry

3.1.46. Allopurinol / verinurad - EMEA-002754-PIP01-19

Chronic kidney disease / Treatment of chronic kidney disease in children and adolescents (6 to <18 years old) with hyperuricaemia and albuminuria

Day 30 discussion

Uro-nephrology

3.1.47. EMEA-002630-PIP01-19

Chikungunya virus infection

Day 30 discussion

Vaccines / Infectious Diseases

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

Novartis Europharm Limited; Treatment of osteoporosis

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Adalimumab - EMEA-C-000366-PIP02-09-M06

AbbVie Limited; Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.2.3. (3R,4R)-4-methyl-3-(methyl-7H-pyrrolo[2,3-d]pyrimidin-4-ylamino)-β-oxo-1-piperidinepropanenitrile, 2-hydroxy-1,2,3-propanetricarboxylate (1:1) - EMEA-C4-000576-PIP01-09-M10

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.4. Cilastatin sodium / relebactam / imipenem monohydrate - EMEA-C2-001809-PIP01-15-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of Gram-negative bacterial infections

Day 30 discussion

Infectious Diseases

3.2.5. Inebilizumab - EMEA-C3-001911-PIP01-15-M02

Viela Bio; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

Neurology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Apixaban - EMEA-000183-PIP01-08-M08

Bristol-Myers Squibb / Pfizer EEIG; Prevention of arterial thromboembolism / Prevention of venous thromboembolism / Prevention of TE in paediatric patients with cardiac disease / Prevention of venous thromboembolism VTE) in paediatric subjects with a newly diagnosed acute lymphoblastic leukemia (ALL) or lymphoma (T or B cell), a functioning central venous access device (CVAD) and receiving asparaginase during chemotherapy induction

Day 30 discussion

Cardiovascular Diseases

3.3.2. Apixaban - EMEA-000183-PIP02-12-M03

Bristol-Myers Squibb / Pfizer EEIG; Treatment of venous thromboembolism

Day 30 discussion

Cardiovascular Diseases

3.3.3. Etripamil - EMEA-002303-PIP01-17-M01

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

Day 30 discussion

Cardiovascular Diseases

3.3.4. Ticagrelor - EMEA-000480-PIP01-08-M13

AstraZeneca AB; Thromboembolic events (children) / Acute coronary syndrome /History of myocardial infarction / Reduction in occurrence of vaso-occlusive crises in paediatric patients with sickle cell disease

Day 30 discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.5. Ligelizumab - EMEA-001811-PIP02-15-M03

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

3.3.6. Lucerastat - Orphan - EMEA-002095-PIP01-16-M01

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of Fabry disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Darvadstrocel - Orphan - EMEA-001561-PIP01-13-M01

Takeda Pharma A/S; Anal fistula

Day 30 discussion

Gastroenterology-Hepatology

3.3.8. Autologous CD34+ cells transduced with lentiviral vector encoding the human beta-globin gene - Orphan - EMEA-001933-PIP01-16-M01

Orchard Therapeutics (Europe) Ltd; Beta-thalassemia major and intermedia / Treatment of beta-thalassemia major and intermedia

Day 30 discussion

Haematology-Hemostaseology

3.3.9. Human fibrinogen concentrate - EMEA-001931-PIP01-16-M02

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3.10. Tofacitinib citrate - EMEA-000576-PIP01-09-M12

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis) / Juvenile idiopathic arthritis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.11. Upadacitinib - EMEA-001741-PIP04-17-M01

AbbVie Ltd; Treatment of atopic dermatitis

Day 30 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.3.12. Tenofovir alafenamide / emtricitabine / cobicistat / elvitegravir - EMEA-001460-PIP01-13-M0

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

Day 30 discussion

Infectious Diseases

3.3.13. Eptinezumab - EMEA-002243-PIP01-17-M01

H. Lundbeck A/S; Prevention of migraine headaches / Prophylaxis of migraine

Day 30 discussion

Neurology

3.3.14. Erenumab - EMEA-001664-PIP02-15-M04

Novartis Europharm Limited; Prevention of migraine headaches / Prophylaxis of migraine

Day 30 discussion

Neurology

3.3.15. Eslicarbazepine acetate - EMEA-000696-PIP02-10-M07

BIAL - Portela & Ca, SA; Treatment of epilepsy with partial onset seizures / Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as adjunctive therapy / Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as monotherapy

Day 30 discussion

Neurology

3.3.16. Humanised anti-IL-6 receptor (IL-6R) monoclonal antibody (INN: satralizumab) - Orphan - EMEA-001625-PIP01-14-M05

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 30 discussion

Neurology

3.3.17. Meningococcal group Y oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein (MenABCWY) / recombinant *Neisseria meningitis* group B Protein 961c / recombinant *Neisseria meningitis* group B Protein 287- 953 / meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Outer Membrane Vesicles (OMV) from *Neisseria meningitidis* Strain NZ 98/254 / meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis* group B Protein 936-741 - EMEA-001260-PIP01-11-M01

GSK Vaccines s.r.l.; A39.9 Meningococcal infection in adults and paediatrics patients

Day 30 discussion

Vaccines

3.3.18. *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-M07

Pfizer Europe MA EEIG; Invasive meningococcal disease caused by *Neisseria meningitidis*

serogroup B

Day 30 discussion

Vaccines

3.3.19. Monovalent, recombinant, replication-incompetent human adenovirus serotype 26-vectored vaccine encoding the pre-fusion conformation-stabilised F protein derived from the RSV A2 strain - EMEA-002172-PIP02-17-M01

Janssen-Cilag International NV; Prevention of lower respiratory tract disease caused by respiratory syncytial virus (RSV)

Day 30 discussion

Vaccines / Infectious Diseases

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 31 March 2020 for Nomination of Rapporteur and Peer reviewer

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

No items

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

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

5.1. New Scientific Advice

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

5.2. Ongoing Scientific Advice

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

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

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

6. Discussion on the applicability of class waivers

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

6.1. Discussions on the applicability of class waiver for products

6.1.1. Selective estrogen receptor degrader - EMEA-02-2020

AstraZeneca AB; The classes of oestrogen receptor modulator medicinal products for treatment of breast malignant neoplasms / Treatment of oestrogen receptor positive, locally advanced or metastatic breast cancer

Summary of committee discussion:

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

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

6.1.2. Brolucizumab - EMEA-03-2020

Novartis Europharm Ltd; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema/ Treatment of visual impairment due to diabetic macular oedema

Summary of committee discussion:

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

Other potential paediatric interests of this medicine suggested by PDCO: retinopathy of prematurity

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

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

No items

8. Annual reports on deferrals

Note: The annual reports on deferrals to be noted by the members of the PDCO are flagged in the Annex B.

No items

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

PDCO Chair: Koen Norga

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 5 medicinal products adopted in February 2020 for which a paediatric investigation plan is ongoing. These included Fetcroja (Cefiderocol), Alunbrig (Brigatinib), OFEV (Nintedanib), Otezla (Apremilast), Entyvio (Vedolizumab).

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

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Summary of committee discussion:

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

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

Relevant products for FWG discussion were identified.

9.4. Cooperation within the EU regulatory network

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

Summary of committee discussion:

The PDCO proposed Sabine Scherer (PDCO vice chair and German PDCO member) to become the new PDCO delegate in the coordinating group of Enpr-EMA. Moreover, it was proposed that Annette Solli Karlsen with her special expertise in clinical trial assessment could become an additional member of the coordinating group.

9.5. Cooperation with International Regulators

9.5.1. ICH S11 – Guideline on nonclinical safety testing in support of development of paediatric pharmaceuticals S11

PDCO Member: Karen van Malderen

Summary of committee discussion:

The guideline will now be formally adopted by the PDCO and the CHMP. The ICH S11 guideline will be included in the PDCO post-mail of the March 2020 plenary.

A presentation by the Non-clinical Working Group chair is envisaged to inform PDCO members on the general content of the ICHS11 guideline and in specific on relevant aspects for the PDCO to consider during the evaluation of the non-clinical measures to support the paediatric clinical development.

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) – follow up from the Helsinki meeting on 20-22 November 2019

PDCO members: Pia Annunen, Ann-Marie Tötterman

Summary of committee discussion:

The draft document summarising the outcome of the SRLM in Helsinki was presented to the committee.

9.7. PDCO work plan

No items

9.8. Planning and reporting

9.8.1. Marketing authorisation applications (MAA) forecast for 2020 – planning update dated Q1 2020

Summary of committee discussion:

Relevant products for FWG discussion were identified.

10. Any other business

10.1 Temporary working arrangements of committees – updated rules of procedure

Summary of committee discussion:

An update of the rules of procedures was presented at the plenary PDCO.

10.2 Template to accompany Proxy votes

Summary of committee discussion:

The new rules on proxy voting were presented as well as the relevant templates to use when members inform the Committee secretariat of the proxy vote.

10.3 COVID updates of paediatric relevance

Summary of committee discussion:

An update was presented on the current status of ongoing activities at the EMA and other Committees related to paediatric aspects concerning Covid-19 (SARS-CoV-2). The presentation included an overview of therapeutics and vaccines being developed for the treatment of COVID29 and of initiatives to harmonize clinical trials protocols for Covid-19 across EU countries.

11. Breakout sessions

No items

12. List of participants

List of participants including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the DD Month YEAR meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium	When chairing the meeting: To be replaced for discussions, final deliberations and voting on: When not chairing the meeting: No participation in final deliberations and voting on:	- 2.3.19 ROLAPITANT - EMEA-001768-PIP02-15-M03; -3.3.20. Meningococcal group Y oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein (MenABCWY) / recombinant Neisseria meningitis group B Protein 961c / recombinant Neisseria meningitis group B Protein 287- 953 / meningococcal group C oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / meningococcal group A oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / Outer Membrane Vesicles (OMV) from Neisseria meningitidis Strain NZ 98/254 / meningococcal group W-135 oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / recombinant Neisseria meningitis group B Protein 936-741 - EMEA-001260-PIP01-11-M01; - 3.1.4. Gepotidacin - EMEA-002443-PIP01-18; -3.1.5. Gepotidacin - EMEA-002443-PIP02-18
Karl-Heinz	Member	Austria	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Huemer				
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	N/A
Georgios Savva	Member	Cyprus	No interests declared	
Lucie Kravackova	Member	Czech Republic	No interests declared	
Petra Dominikova	Alternate	Czech Republic	No interests declared	
Kirstine Moll Harboe	Member	Denmark	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 discussions, final deliberations and voting on:	- 3.3.1. Apixaban - EMEA-000183-PIP01-08-M08; - 3.3.2. Apixaban - EMEA-000183-PIP02-12-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	
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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Marek Migdal	Member	Poland	No interests declared	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Peter Szitanyi	Alternate	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Ninna Gullberg	Member	Sweden	No interests declared	
Eva Agurell	Alternate	Sweden	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	N/A
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:	- 3.2.10. Lumasiran sodium - EMEA-C1-002079-PIP01-16-M01; - 2.3.12. Pitolisant - Orphan - EMEA-001176-PIP01-11-M04
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 restrictions applicable to this meeting	N/A
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

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Milena Stevanovic	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	N/A
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Viviana Giannuzzi	Alternate	Patients' Organisation Representative	No participation in final deliberations and voting on:	- 3.1.14. Atropine - EMEA-002545-PIP01-19
Maria Estela Moreno Martin	Expert - in person*	Spain	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/