21 May 2021
EMA/PDCO/298607/2021
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 18-21 May 2021

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

Virtual Meeting

Disclaimers

Some of the information contained in this set of minutes is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Of note, this set of minutes is a working document primarily designed for PDCO members and the work the Committee undertakes.

Note on access to documents

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

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1. Introductions

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

The Chairperson opened the meeting by welcoming all participants. Due to the current coronavirus (COVID-19) outbreak, and the associated EMA Business Continuity Plan (BCP), the meeting was held remotely.

In accordance with the Agency's policy on handling of declarations of interests of scientific Committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and based on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some meeting participants in upcoming discussions as included in the pre-meeting list of participants and restrictions.

Participants in this meeting were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure and as included in the list of participants. All decisions taken at this meeting were made in the presence of a quorum of members (i.e. 17 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

Due to restricted involvement, the Chair Koenraad Norga deputised chairing the meeting to the Vice-Chair Sabine Scherer for the agenda topic 3.1.45.

1.2. Adoption of agenda

The agenda for 18-21 May 2021 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 20-23 April 2021 meeting were adopted and will be published on the EMA website.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Finerenone - EMEA-001623-PIP03-20

Bayer AG; Treatment of heart failure

Day 120 opinion

Cardiovascular Diseases

Summary of Committee discussion:

In line with its Day 90 discussion, the PDCO concluded on a positive opinion for a PIP and a deferral for finerenone in the condition of treatment of heart failure in children from birth to less than 18 years of age.

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

Janssen-Cilag International N.V.; Treatment of functional single ventricle heart disease with total cavo-pulmonary connection

Day 120 opinion

Cardiovascular Diseases

Summary of Committee discussion:

In line with the Day 90 discussion, the PDCO concluded on a positive opinion for a PIP and a deferral for macitentan in the condition of treatment of functional single ventricle heart disease with total cavo-pulmonary connection, and recommends granting a waiver in children without total cavo-pulmonary connection on the grounds that the condition for which the specific medicinal product is developed does not occur in this population.

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

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension

Day 120 opinion

Cardiovascular Diseases

Summary of Committee discussion:

All outstanding issues were considered resolved. The PDCO therefore concluded on a positive opinion for a PIP and a deferral for ralinepag in the condition of treatment of pulmonary arterial hypertension in children from 1 year to less than 18 years of age, and recommends granting a waiver in children from birth to less than 1 year of age on the grounds that the specific medicinal product is likely to be unsafe in this paediatric age subset.

2.1.4. Allogeneic skin-derived ABCB5-positive mesenchymal stem cells - Orphan - EMEA-002875-PIP01-20

RHEACELL GmbH & Co. KG; Treatment of epidermolysis bullosa

Day 120 opinion

Dermatology

Summary of Committee discussion:

Based on the assessment of this application, the PDCO adopted a positive PIP Opinion during its May 2021 plenary, for the product allogeneic skin-derived ABCB5-positive mesenchymal stem cells for children from birth to less than 18 years of age, in the condition of treatment of epidermolysis bullosa (EB).

2.1.5. Adeno-associated viral vector serotype 8 containing the human glucose-6-phosphatase gene (DTX401) - Orphan - EMEA-002734-PIP01-19

Ultragenyx Germany GmbH; Treatment of glycogen storage disease type Ia / Treatment of glycogen storage disease type Ia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the assessment of this application, the PDCO adopted a positive opinion for the PIP for the proposed medicine for children from 2 to less than 18 years of age, in the condition treatment of glycogen storage disease type Ia. The PDCO granted a deferral for the completion of this PIP.
The PDCO granted a waiver for the paediatric population from birth to less than 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.6. Pyridoxal 5'-phosphate monohydrate - Orphan - EMEA-002404-PIP01-18

Medicure Pharma Europe Limited; Pyridox(am)ine 5'-phosphate oxidase (PNPO) deficiency

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Note: Withdrawal request received on 7 May 2021

2.1.7. Maralixibat Chloride - Orphan - EMEA-001475-PIP04-20

Mirum Pharmaceuticals Inc.; Treatment of biliary atresia

Day 120 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

In the written response following the D90 discussion the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the

proposed medicine for the paediatric population from birth to less than 18 years in the condition of treatment of biliary atresia was adopted.

2.1.8. Odevixibat - Orphan - EMEA-002054-PIP03-20

Albireo AB; Treatment of Alagille syndrome

Day 120 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from birth to less than 18 years of age, in the condition of treatment of Alagille syndrome was adopted.

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

Allakos Inc; Treatment of eosinophilic gastrointestinal inflammatory disorders / Treatment of eosinophilic gastrointestinal inflammatory disorders

Day 120 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

The issues discussed at Day 90 are now resolved following the responses submitted by the Applicant on 6 May 2021.
The PDCO adopted a positive opinion at Day 120, including a waiver in children from birth to less than 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible and a deferral for the clinical and extrapolation studies in children from 2 years to less than 12 years of age.

2.1.10. Cilgavimab (AZD1061) - EMEA-002925-PIP01-20

AstraZeneca AB; Prevention or treatment of COVID-19

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the responses of the applicant to the day 90 outstanding issues and considered that the remaining issues were solved. An opinion was adopted on a PIP and a deferral. The PIP covers the paediatric population from birth (29 weeks gestational age) to less than 18 years of age for the conditions 'treatment of coronavirus-19 (COVID-19) and prevention of coronavirus-19 (COVID-19) '.

2.1.11. Tixagevimab (AZD8895) - EMEA-002900-PIP01-20

AstraZeneca AB; Prevention or treatment of COVID-19

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the responses of the applicant to the day 90 outstanding issues and considered that the remaining issues were solved. An opinion was adopted on a PIP and a deferral. The PIP covers the paediatric population from birth (29 weeks gestational age) to less than 18 years of age for the conditions 'treatment of coronavirus-19 (COVID-19) and prevention of coronavirus-19 (COVID-19)'.

2.1.12. Pralsetinib - EMEA-002575-PIP02-20

Roche Registration GmbH; Treatment of thyroid neoplasms

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 120 during the May 2021 plenary a PIP for pralsetinib for the treatment of thyroid neoplasms.
The Committee confirmed all the conclusions reached at Day 90.
Based on the assessment of this application and the additional information provided by the applicant, the PDCO adopted a positive PIP opinion for pralsetinib for children from 12 years to less than 18 years of age, in the condition of treatment of thyroid neoplasms. The PDCO agreed on a waiver in a subset of children on the grounds that this product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for the completion of this PIP.

2.1.13. Autologous selected renal cells - EMEA-002844-PIP01-20

ProKidney; Treatment of chronic kidney disease

Day 120 opinion

Uro-nephrology

Summary of Committee discussion:

Responses to the PDCO's Day 90 comments were received from the applicant.
Subsequently, all outstanding issues were considered resolved. The PDCO therefore concluded on a positive opinion for a PIP and a deferral for autologous selected renal cells in the condition of treatment of chronic kidney disease in children from 2 years to less than 18 years of age, and recommends granting a waiver in children from birth to less than 2 years of age on the grounds that the specific medicinal product is likely to be unsafe in this paediatric age subset.

2.1.14. Selatogrel - EMEA-002967-PIP01-21

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of acute myocardial infarction (AMI)

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

The applicant requests a waiver for selatogrel for all subsets of the paediatric population in the condition, treatment of acute myocardial infarction, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as studies are not feasible.

The PDCO reviewed this request and found it to be overall acceptable.

2.1.15. Vupanorsen - EMEA-002973-PIP01-21

Pfizer Europe MA EEIG; Treatment of hypertriglyceridaemia / Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application, the PDCO agrees with the applicant's request for a waiver for vupanorsen for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of hypertriglyceridaemia on the grounds of a lack of significant therapeutic benefit as the needs are already covered, as well as in prevention of cardiovascular events on the grounds of a 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 sporadic cases in the condition prevention of cardiovascular events 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. Mannitol - EMEA-002968-PIP01-21

NTC Srl; Bowel cleansing prior to clinical procedure

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 mannitol for all subsets of the paediatric population (0 to

less than 18 years of age) in the condition of bowel cleansing prior to clinical procedure.

2.1.17. Anti-C1s Humanized IgG4 Monoclonal Antibody - EMEA-002903-PIP02-21

Genzyme Europe B.V.; Treatment of Cold Agglutinin Disease

Day 60 opinion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology / Neurology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of cold agglutinin disease on the grounds the disease or condition for which the specific medicinal product is intended occurs only in adult populations.

2.1.18. [18F]CTT1057 - EMEA-002975-PIP01-21

Advanced Accelerator Applications SA; Visualisation of prostate-specific membrane antigen in adenocarcinoma of the prostate

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from D30. 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 [18F]CTT1057 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 based on the ground that the disease does not occur in children. Since the most appropriate waiver ground is considered to be disease not occurring in the paediatric population, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant. The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.19. Datopotamab deruxtecan - EMEA-002976-PIP01-21

Daiichi Sankyo Europe GmbH; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from D30. 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 datopotamab deruxtecan for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of cancer based on the ground that the disease does not occur in children. Since the most appropriate waiver ground is considered to be disease not occurring in the paediatric population, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.20. Human Papilloma Virus Type 16 E6 001-032/Human Papilloma Virus Type 16 E6 019-050/Human Papilloma Virus Type 16 E6 041-065/Human Papilloma Virus Type 16 E6 055-080/Human Papilloma Virus Type 16 E6 085-109/Human Papilloma Virus Type 16 E6 091-122/Human Papilloma Virus Type 16 E6 127-158 / Human Papilloma Virus Type 16 E6 071-095/Human Papilloma Virus Type 16 E6 109-140/Human Papilloma Virus Type 16 E7 001-035/Human Papilloma Virus Type 16 E7 022-056/Human Papilloma Virus Type 16 E7 064-098 - EMEA-001055-PIP02-21

ISA Therapeutics B.V.; Treatment of HPV16 positive malignancies

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from D30. 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 Human Papilloma Virus Type 16 E6 001-032/Human Papilloma Virus Type 16 E6 019-050/Human Papilloma Virus Type 16 E6 041-065/Human Papilloma Virus Type 16 E6 055-080/Human Papilloma Virus Type 16 E6 085-109/Human Papilloma Virus Type 16 E6 091-122/Human Papilloma Virus Type 16 E6 127-158 / Human Papilloma Virus Type 16 E6 071-095/Human Papilloma Virus Type 16 E6 109-140/Human Papilloma Virus Type 16 E7 001-035/Human Papilloma Virus Type 16 E7 022-056/Human Papilloma Virus Type 16 E7 064-098 for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of HPV16 positive malignancies based on the ground of disease not occurring in children from birth to less than 14 years of age and lack of significant therapeutic benefit in patients age 14 years to less than 18 years of age.
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. Patritumab deruxtecan - EMEA-002977-PIP01-21

Daiichi Sankyo Europe GmbH; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from D30. 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 patritumab deruxtecan for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of lung cancer based on the ground that the disease does not occur in children. Since the most appropriate waiver ground is considered to be disease not occurring in the paediatric population, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
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. Trastuzumab deruxtecan - EMEA-002978-PIP01-21

Daiichi Sankyo Europe GmbH; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from D30. 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 trastuzumab deruxtecan for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of lung cancer based on the ground that the disease does not occur. Since the most appropriate waiver ground is considered to be disease not occurring in the paediatric population, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
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. Dapagliflozin (propanediol monohydrate) / Zibotentan - EMEA-002969-PIP01-21

AstraZeneca AB; Treatment of chronic kidney disease

Day 60 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for zibotentan / dapagliflozin (propanediol monohydrate) for all subsets of the paediatric population (0 to less than 18 years of age) in the condition of treatment of chronic kidney 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.

2.1.24. Regdanvimab - EMEA-002961-PIP01-21

Celltrion Healthcare Hungary Kft.; Treatment of coronavirus disease 2019 (COVID-19)

Day 90 opinion

Infectious Diseases

Summary of Committee discussion:

In May 2021 the PDCO agreed an Opinion for a PIP for regdanvimab for the treatment of COVID-19 with a deferral.

2.1.25. Fully human neutralizing immunoglobulin G-1 kappa monoclonal antibody directed against a conserved epitope on the SARS CoV1 and 2 spike protein - EMEA-002899-PIP01-20

GlaxoSmithKline (Ireland) Ltd; Treatment of coronavirus disease 2019 (COVID-19)

Day 90 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the responses provided by the applicant. All other issues raised in the request for modification were considered solved.
Based on the assessment of this application and further discussions at the Paediatric Committee, a positive opinion was adopted for fully human neutralizing immunoglobulin G-1 kappa monoclonal antibody directed against a conserved epitope on the SARS CoV1 and 2 spike protein for all subsets of the paediatric population (birth to 18 years of age, with age at birth starting from 32 weeks gestational age) in the condition treatment of coronavirus disease 2019 (COVID-19).

2.2. Opinions on Compliance Check

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

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Nemolizumab - EMEA-001624-PIP01-14-M03

Galderma International S.A.; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0106/2015 of 13 May 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19-M01

Alexion Europe S.A.S.; Treatment of Wilson 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/0234/2020 of 19 June 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Canagliflozin (1s)-1,5-anhydro-1-[3-[[5-(4-fluorophenyl)-2-thienyl]methyl]-4-methylphenyl]-D-glucitol hemihydrate - EMEA-001030-PIP01-10-M09

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

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted, in line with the day 30 PDCO discussion.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision during its May 2021 plenary meeting.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Cotadutide - EMEA-002287-PIP01-17-M02

AstraZeneca AB; Treatment of Type 2 Diabetes Mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

Based on the review of the rationale submitted by the 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/0502/2020 of 22/12/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Romosozumab - EMEA-001075-PIP04-15-M03

UCB Pharma S.A.; Treatment of osteoporosis

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/0183/2020 of 13/5/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Eluxadoline - EMEA-001579-PIP01-13-M04

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

Day 60 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Note: Withdrawal request received on 19 May 2021

2.3.7. Ozanimod hydrochloride - EMEA-001710-PIP03-17-M03

Celgene Europe B.V.; Treatment of ulcerative colitis

Day 60 opinion

Gastroenterology-Hepatology

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/0383/2020 of 24 September 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Garadacimab - EMEA-002726-PIP01-19-M01

CSL Behring GmbH; Prevention of hereditary angioedema attacks

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/0451/2020 of 01/12/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Cotadutide - EMEA-002712-PIP01-19-M01

AstraZeneca AB; Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 opinion

Immunology-Rheumatology-Transplantation

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/0452/2020 of 01/12/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Guselkumab - EMEA-001523-PIP03-18-M01

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

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/0025/2019 of 22 February 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19-M01

Principia Biopharma, Inc.; Treatment of immune thrombocytopenia

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that most of the proposed changes could be accepted. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0306/2020 of 12 August 2020.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Cobicistat / darunavir - EMEA-001280-PIP01-12-M04

Janssen-Cilag International NV; Treatment of HIV-1 infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

In the written response the applicant addressed the issues raised by the Committee at D30. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0049/2021 of 27 January 2021.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Dolutegravir - EMEA-000409-PIP01-08-M06

ViiV Healthcare UK Limited; Human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

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

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (3/1/2019 of P/0017/2019).

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

2.3.14. Ibalizumab - EMEA-002311-PIP01-17-M02

Theratechnologies Europe Limited; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0271/2018 of 17/8/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Oseltamivir phosphate - EMEA-000365-PIP01-08-M12

Roche Registration GmbH; Treatment and prevention of influenza

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

In the written response the applicant addressed the remaining issues raised by the Committee.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0341/2020 of 9 September 2020.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Oteseconazole - EMEA-002392-PIP01-18-M01

Gedeon Richter Plc.; ICD-10 Code B373, Treatment of vulvovaginal candidiasis

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Note: Withdrawal request received on 21 May 2021

2.3.17. Peramivir - EMEA-001856-PIP02-16-M02

BioCryst Ireland Limited; Treatment of influenza

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0340/2016 of 25 November 2016.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. Tenofovir alafenamide - EMEA-001584-PIP01-13-M06

Gilead Sciences International Ltd.; Treatment of chronic viral hepatitis B

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0063/2020 issued on 20 February 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Vaborbactam / meropenem - EMEA-001731-PIP01-14-M03

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0267/2020 of 15/7/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Nivolumab - EMEA-001407-PIP02-15-M05

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

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0433/2020 of 5 November 2020.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.21. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M05

Shire Pharmaceuticals Ireland Limited; Treatment of hereditary angioedema

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/0265/2019 of 23/07/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.22. Ravulizumab - Orphan - EMEA-001943-PIP01-16-M06

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 60 opinion

Uro-nephrology / 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/0400/2020 of 23 October 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.23. Ravulizumab - Orphan - EMEA-002077-PIP01-16-M04

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 opinion

Uro-nephrology / 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/0399/2020 of 23 October 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.24. COVID-19 Vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M01

AstraZeneca AB; COVID-19

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that most of the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0003/2021 of 5 January 2021).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

The following partial compliance checks have been identified by the PME coordinator and PDCO rapporteur as not needing to be referred to the PDCO for discussion. The PDCO has been informed in writing.

2.7.1. Maralixibat Chloride - EMEA-C1-001475-PIP02-13-M01

Mirum Phamraceuticals; Treatment of Alagille syndrome

Day 30 letter

Gastroenterology-Hepatology

2.7.2. Valoctocogene roxaparvovec - EMEA-C2-002427-PIP01-18-M01

BioMarin International Ltd.; Treatment of haemophilia A

Day 30 letter

Haematology-Hemostaseology

2.7.3. Tenofovir (disoproxil fumarate) - EMEA-C5-000533-PIP01-08-M10

Gilead Sciences International Limited; Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 letter

Infectious Diseases

2.7.4. Crizotinib - EMEA-C2-001493-PIP03-18-M01

Pfizer Europe MA EEIG; Treatment of inflammatory myofibroblastic tumour

Day 30 letter

Oncology

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20

Atopic dermatitis / Atopic dermatitis

Day 90 discussion

Dermatology

3.1.2. Infigratinib - EMEA-002594-PIP02-20

Achondroplasia / Treatment of achondroplasia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. (1R,3S,5R)-2-(2-(3-acetyl-5-(2-methylpyrimidin-5-yl)-1H-indazol-1-yl)acetyl)-N-(6-bromo-3-methylpyridin-2-yl)-5-methyl-2-azabicyclo[3.1.0]hexane-3-carboxamide - Orphan - EMEA-002863-PIP01-20

Alexion Europe SAS; Paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Haematology-Hemostaseology

3.1.4. EMEA-002350-PIP03-20

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.5. Fenebrutinib - EMEA-002349-PIP03-20

Treatment of multiple sclerosis / Multiple sclerosis

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.6. Itraconazole - Orphan - EMEA-002787-PIP01-20

Laboratoires S.M.B. S.A.; Prevention of invasive mould disease

Day 90 discussion

Infectious Diseases / Oncology

3.1.7. Trimodulin - EMEA-002883-PIP01-20

Treatment of infections

Day 90 discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Pneumology - Allergology

3.1.8. Zidebactam / cefepime - EMEA-002892-PIP01-20

Treatment of complicated urinary tract infections

Day 90 discussion

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

3.1.9. Ublituximab - EMEA-002889-PIP02-20

Treatment of multiple sclerosis

Day 90 discussion

Neurology

3.1.10. Vatiquinone - Orphan - EMEA-001238-PIP02-20

PTC Therapeutics International Limited; Treatment of mitochondrial disease / Treatment of mitochondrial epilepsy, excluding Leigh Syndrome

Day 90 discussion

Neurology

3.1.11. Afamitresgene autoleucel - Orphan - EMEA-002867-PIP01-20

Adaptimmune Ltd; Treatment of soft tissue sarcoma

Day 90 discussion

Oncology

3.1.12. Allogeneic anti-CD19 CAR T cells produced using CRISPR/Cas9 to disrupt the T cell receptor alpha constant (TRAC) and β2-microglobulin (B2M) genomic loci and a recombinant adeno-associated viral vector to deliver donor template for insertion of the anti-CD19 CAR expression cassette into the TRAC locus (CTX110) - EMEA-002881-PIP01-20

Mature B cell neoplasms and B cell lymphoblastic leukemia/lymphoma / B cell lymphoblastic leukemia/lymphoma / Mature B cell neoplasms

Day 90 discussion

Oncology

3.1.13. Iptacopan - Orphan - EMEA-002705-PIP03-20

Novartis Europharm Limited; Paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Other / Haematology-Hemostaseology

3.1.14. Bardoxolone methyl - Orphan - EMEA-002488-PIP01-18

Reata Pharmaceuticals Inc.; Treatment of Alport syndrome

Day 90 discussion

Uro-nephrology

3.1.15. SERALUTINIB - Orphan - EMEA-002972-PIP01-21

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension.

Day 60 discussion

Cardiovascular Diseases

3.1.16. Ruxolitinib - EMEA-002618-PIP03-21

Atopic dermatitis

Day 60 discussion

Dermatology

3.1.17. Benzylamine derivative of benzofuran - EMEA-002974-PIP01-21

Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Haematology-Hemostaseology

3.1.18. Ravulizumab - EMEA-001943-PIP04-20

Aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder

Day 60 discussion

Neurology

3.1.19. Satralizumab - Orphan - EMEA-001625-PIP02-21

Roche Registration GmbH; Treatment of myasthenia gravis

Day 60 discussion

Neurology

3.1.20. Vatiquinone - EMEA-001238-PIP03-21

Treatment of Friedreich’s ataxia

Day 60 discussion

Neurology

3.1.21. Nirogacestat hydrobromide - Orphan - EMEA-002971-PIP01-21

SpringWorks Therapeutics, Inc; Treatment of desmoid tumours

Day 60 discussion

Oncology

3.1.22. Ribociclib - EMEA-002765-PIP02-21

Treatment of neuroblastoma

Day 60 discussion

Oncology

3.1.23. 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA - Orphan - EMEA-002981-PIP01-21

Antisense Therapeutics Limited; Treatment of Duchenne muscular dystrophy

Day 60 discussion

Other

3.1.24. Pamrevlumab - Orphan - EMEA-002979-PIP01-21

FibroGen, Inc.; Treatment of Duchenne muscular dystrophy

Day 60 discussion

Other

3.1.25. Evenamide - EMEA-002519-PIP03-21

Treatment of schizophrenia

Day 60 discussion

Psychiatry

3.1.26. Neisseria meningitidis serogroup B Protein-based active substance / Recombinant Neisseria meningitidis serogroup B protein 1 / Recombinant Neisseria meningitidis serogroup B protein 3 / Recombinant Neisseria meningitidis serogroup B protein 2 - EMEA-002954-PIP02-21

Prevention of meningococcal disease (serogroup B)

Day 60 discussion

Vaccines

3.1.27. Bentracimab - EMEA-002766-PIP02-21

Treatment of ticagrelor associated haemorrhage / Prevention of ticagrelor associated haemorrhage

Day 30 discussion

Cardiovascular Diseases

3.1.28. Hydrochlorothiazide / Amlodipine / Ramipril - EMEA-002998-PIP01-21

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.29. Brepocitinib - EMEA-003011-PIP01-21

Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.1.30. Human Immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein - Orphan - EMEA-002995-PIP01-21

EspeRare Foundation; X-linked hypohidrotic ectodermal dysplasia (XLHED)

Day 30 discussion

Dermatology

3.1.31. Ulobetasol propionate - EMEA-003000-PIP01-21

Treatment of psoriasis. / The proposed condition is moderate to severe plaque psoriasis. The proposed medicinal product is aimed as "treatment".

Day 30 discussion

Dermatology

3.1.32. EMEA-002992-PIP01-21

Treatment of Fibrodysplasia Ossificans Progressiva (FOP).

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Empagliflozin - EMEA-000828-PIP07-21

Treatment of ischaemic heart disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.34. Ethinyl estradiol / Dienogest - EMEA-002229-PIP02-21

Treatment of polycystic ovary syndrome

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. Semaglutide - EMEA-001441-PIP06-21

Treatment of obesity

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.36. Semaglutide / insulin icodec - EMEA-002988-PIP01-21

Type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.37. Aldafermin - EMEA-003005-PIP01-21

Treatment of Non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Gastroenterology-Hepatology

3.1.38. Recombinant adeno-associated viral (rAAV) vector expressing the human ornithine transcarbamylase (hOTC) gene - EMEA-002983-PIP01-21

Ornithine Transcarbamylase Deficiency

Day 30 discussion

Gastroenterology-Hepatology

3.1.39. Sirolimus - EMEA-002982-PIP01-21

Ornithine Transcarbamylase Deficiency (OTCD)

Day 30 discussion

Gastroenterology-Hepatology

3.1.40. 6-[(3S,4S)-4-methyl-1-(pyrimidin-2-ylmethyl)pyrrolidin-3-yl]-3-tetrahydropyran-4-yl-7H-imidazo[1,5-a]pyrazin-8-one - Orphan - EMEA-002991-PIP01-21

IMARA Inc; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.1.41. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP03-21

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

Day 30 discussion

Haematology-Hemostaseology

3.1.42. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP04-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of beta-thalassemia intermedia and major

Day 30 discussion

Haematology-Hemostaseology

3.1.43. Efgartigimod alfa - Orphan - EMEA-002597-PIP04-21

argenx BV; Treatment of immune thrombocytopenia

Day 30 discussion

Haematology-Hemostaseology

3.1.44. Apremilast - EMEA-000715-PIP06-21

Treatment of coronavirus disease (COVID-19)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.45. Leniolisib phosphate - Orphan - EMEA-002989-PIP01-21

Pharming Group N.V.; Activated phosphoinositide 3-kinase δ syndrome (APDS)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.46. Favipiravir - EMEA-002985-PIP01-21

Treatment of coronavirus disease (COVID-19)

Day 30 discussion

Infectious Diseases

3.1.47. Givinostat - Orphan - EMEA-000551-PIP04-21

Italfarmaco S.p.A.; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Neurology

3.1.48. Immunoglobulin G1 anti-SORT1 human monoclonal antibody - EMEA-002997-PIP01-21

Treatment of dementia

Day 30 discussion

Neurology

3.1.49. EMEA-002993-PIP01-21

Treatment of narcolepsy

Day 30 discussion

Neurology

3.1.50. EMEA-002635-PIP02-21

Treatment of advanced or metastatic malignancies harbouring ALK, ROS1, or NTRK1-3 alterations

Day 30 discussion

Oncology

3.1.51. Alectinib - EMEA-002431-PIP02-21

Treatment of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Oncology

3.1.52. Autologous T cells transduced with lentiviral vector containing a tandem chimeric antigen receptor directed against CD20 and CD19 - Orphan - EMEA-003009-PIP01-21

Miltenyi Biomedicine GmbH; Diffuse large B cell lymphoma / Mature aggressive B cell non-Hodgkin lymphoma

Day 30 discussion

Oncology

3.1.53. Cetrelimab - EMEA-003006-PIP01-21

Treatment of urothelial carcinoma

Day 30 discussion

Oncology

3.1.54. Datopotamab deruxtecan - EMEA-002976-PIP02-21

Treatment of breast cancer

Day 30 discussion

Oncology

3.1.55. Gemcitabine HCl - EMEA-003007-PIP01-21

Treatment of urothelial carcinoma

Day 30 discussion

Oncology

3.1.56. Autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LV, encoding for the human α-L-iduronidase (IDUA) cDNA - Orphan - EMEA-003001-PIP01-21

Orchard Therapeutics Netherlands B.V.; Treatment of Mucopolysaccharidosis type I, Hurler syndrome (MPS-IH)

Day 30 discussion

Other

3.1.57. Gabapentin - EMEA-002994-PIP01-21

Treatment of postherpetic neuralgia

Day 30 discussion

Pain / Neurology

3.1.58. Sivopixant - EMEA-003010-PIP01-21

Unexplained or refractory chronic cough

Day 30 discussion

Pneumology - Allergology

3.1.59. EMEA-002990-PIP01-21

Major depressive disorder

Day 30 discussion

Psychiatry

3.1.60. Ralmitaront - EMEA-003003-PIP01-21

Treatment of schizophrenia

Day 30 discussion

Psychiatry

3.1.61. EMEA-003002-PIP01-21

Treatment of proteinuric chronic kidney disease

Day 30 discussion

Uro-nephrology

3.1.62. Escherichia coli vaccine - EMEA-002996-PIP01-21

Prevention of E.coli infections

Day 30 discussion

Vaccines

3.1.63. Live, attenuated, dengue virus, serotype 4 (DENV4) / Live, attenuated, dengue virus, serotype 3 (DENV3) / Live, attenuated, chimeric dengue virus, serotype 2 (DENV2) / Live, attenuated, dengue virus, serotype 1 (DENV1) - EMEA-002999-PIP01-21

Prevention of dengue disease

Day 30 discussion

Vaccines

3.1.64. Recombinant SARS-CoV-2 spike (S)-protein virus-like particle - EMEA-003008-PIP01-21

Prevention of Coronavirus disease 2019 (COVID-19)

Day 30 discussion

Vaccines

3.2. Discussions on Compliance Check

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

3.2.1. Enalapril maleate - EMEA-C-001706-PIP01-14-M03

Proveca Pharma Limited; Treatment of heart failure

Day 30 discussion

Cardiovascular Diseases

3.2.2. Exenatide - EMEA-C-000689-PIP01-09-M11

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Maralixibat Chloride - EMEA-C1-001475-PIP02-13-M01

Mirum Pharmaceuticals; Treatment of Alagille syndrome

Day 30 discussion

Gastroenterology-Hepatology

The PDCO finalised this partially completed compliance procedure on 21 May 2021.

3.2.4. Cobimetinib - EMEA-C-001425-PIP01-13-M05

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) with Ras, Raf or MEK pathway activation

Day 30 discussion

Oncology

3.2.5. Tezepelumab - EMEA-C1-001613-PIP01-14-M04

AstraZeneca AB; Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Clevidipine - EMEA-000282-PIP01-08-M03

Chiesi Farmaceutici S.p.A.; Treatment of hypertensive disease

Day 30 discussion

Cardiovascular Diseases

3.3.2. Sacubitril/valsartan - EMEA-000316-PIP02-11-M05

Novartis Europharm Ltd.; Heart failure

Day 30 discussion

Cardiovascular Diseases

3.3.3. Baricitinib - EMEA-001220-PIP03-16-M02

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.4. Dupilumab - EMEA-001501-PIP01-13-M07

Regeneron Pharmaceuticals, Inc; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.5. Lebrikizumab - EMEA-002536-PIP01-18-M01

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.6. Ligelizumab - EMEA-001811-PIP02-15-M04

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

3.3.7. Tralokinumab - EMEA-001900-PIP02-17-M05

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.8. Liraglutide - EMEA-000128-PIP02-09-M04

Novo Nordisk A/S; E66 Obesity

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.9. Ferric maltol - EMEA-001195-PIP01-11-M05

Norgine BV; Treatment of iron deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3.10. Giroctocogene fitelparvovec - Orphan - EMEA-002724-PIP01-19-M01

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 30 discussion

Haematology-Hemostaseology

3.3.11. Rurioctocog alfa pegol - EMEA-001296-PIP01-12-M04

Baxalta Innovations GmbH; Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)

Day 30 discussion

Haematology-Hemostaseology

3.3.12. Tocilizumab - EMEA-000309-PIP04-17-M03

Roche Registration GmbH; Treatment of cytokine release syndrome

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.13. Cenobamate - EMEA-002563-PIP02-19-M01

A.C.R.A.F. SpA; Treatment of epilepsy

Day 30 discussion

Neurology

3.3.14. Ocrelizumab - EMEA-000310-PIP03-10-M05

Roche Registration GmbH; Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.3.15. Phenobarbital - EMEA-002532-PIP01-18-M01

Proveca Pharma Limited; Treatment of epilepsy

Day 30 discussion

Neurology

3.3.16. Temelimab - EMEA-002127-PIP01-17-M01

GeNeuro SA; Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.3.17. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M03

Ipsen Pharma; Treatment of malignant solid tumours

Day 30 discussion

Oncology

3.3.18. Cemiplimab - EMEA-002007-PIP02-17-M01

Regeneron Ireland DAC; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 30 discussion

Oncology

3.3.19. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M06

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasm

Day 30 discussion

Oncology

3.3.20. Orphan - EMEA-001821-PIP01-15-M05

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 30 discussion

Oncology

3.3.21. Sirolimus - Orphan - EMEA-001416-PIP01-12-M03

Santen Incorporated; Treatment of non-infectious uveitis affecting the posterior segment of the eye

Day 30 discussion

Ophthalmology

3.3.22. Andexanet alfa - EMEA-001902-PIP01-15-M05

Alexion Europe SAS; Treatment of factor Xa inhibitor associated haemorrhage / Prevention

of factor Xa inhibitor associated haemorrhage

Day 30 discussion

Other

3.3.23. In vitro expanded autologous human articular chondrocytes - EMEA-002217-PIP01-17-M02

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 30 discussion

Other

3.3.24. Selexipag - EMEA-000997-PIP01-10-M05

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 discussion

Other

3.3.25. Xylitol / Procaine hydrochloride / Magnesium sulphate heptahydrate / Potassium chloride - EMEA-001171-PIP01-11-M02

MIT Gesundheit GmbH; Cardioplegia

Day 30 discussion

Other

3.3.26. Lactobacillus reuteri - Orphan - EMEA-001895-PIP01-15-M01

Infant Bacterial Therapeutics AB; Prevention of necrotising enterocolitis

Day 30 discussion

Other / Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.3.27. Fasinumab - EMEA-002059-PIP02-19-M01

Regeneron Ireland D.A.C.; Chronic musculoskeletal pain / Chronic pain / Chronic non-musculoskeletal pain

Day 30 discussion

Pain

3.3.28. Seltorexant - EMEA-002746-PIP01-20-M01

Janssen-Cilag International NV; Major Depressive Disorder (MDD)

Day 30 discussion

Psychiatry

3.3.29. Vortioxetine - EMEA-000455-PIP02-10-M08

H. Lundbeck A/S; Major Depressive Disorder

Day 30 discussion

Psychiatry

3.3.30. Finerenone - EMEA-001623-PIP01-14-M04

Bayer AG; Chronic Kidney Disease

Day 30 discussion

Uro-nephrology

3.3.31. Ad26.ZEBOV - EMEA-002307-PIP01-17-M02

Janssen Cilag International NV; Prevention of Ebola virus disease

Day 30 discussion

Vaccines

3.3.32. Hepatitis B (rDNA) surface antigen adjuvanted - EMEA-001127-PIP02-11-M01

Dynavax GmBH; Prevention of hepatitis B virus infection

Day 30 discussion

Vaccines

3.3.33. MVA-BN-Filo - EMEA-002308-PIP01-17-M02

Janssen Cilag International NV; Prevention of Ebola virus disease

Day 30 discussion

Vaccines

3.3.34. Neisseria meningitidis serogroup W polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup C polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M03

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 30 discussion

Vaccines

3.3.35. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03

AstraZeneca AB; Prevention of respiratory syncytial viral infections

Day 30 discussion

Vaccines

4. Nominations

Information related to this section cannot be disclosed 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 25 May 2021 for Nomination of Rapporteur and Peer reviewer

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

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

5.1. New Scientific Advice

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

No item

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

No item

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

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

7.1.1. Tisagenlecleucel - EMEA-001654-PIP02-17-M01

Novartis Europharm Limited; Treatment of mature B-cell neoplasms

Proposed indications: Treatment of adult patients with r/r follicular lymphoma after two lines of therapies and treatment of adult patients with relapse or refractory aggressive B-cell non-Hodgkin lymphoma after one line of systemic therapy

Oncology

Summary of Committee discussion:

The PDCO was of the view that the proposed indications "treatment of adult patients with r/r follicular lymphoma after two lines of therapies" and "treatment of adult patients with relapse or refractory aggressive B-cell non-Hodgkin lymphoma after one line of systemic therapy", fall under the scope of the above mentioned Decision, as the indications are considered to be covered by the condition "treatment of mature B-cell neoplasms" listed in the Agency Decision.

7.1.2. Somapacitan - EMEA-001469-PIP02-17

Novo Nordisk A/S; Treatment of short stature

The applicant wishes to receive PDCO's feedback that their newly planned indications "Treatment of Short Stature associated with Noonan Syndrome (NS) / Treatment of Short Stature associated with Turner Syndrome (TS) / Treatment of Idiopathic short stature (ISS)," are covered by the agreed full Waiver with the condition "Treatment of short stature" listed in the Agency Decision

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO has reviewed the company request during the plenary meeting held on 18 – 21 May 2021. The PDCO is of the view that the proposed indications "Treatment of Short Stature associated with Noonan Syndrome (NS) / Treatment of Short Stature associated with Turner Syndrome (TS) / Treatment of Idiopathic short stature (ISS)", fall under the scope of the above mentioned Decision, as the indications are considered to be covered by the condition "treatment of short stature" listed in the Agency Decision.

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

No item

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

No item

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 (NcWG) identified the products which will require NcWG evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of Committee discussion:

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

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:

No item

9.5. Cooperation with International Regulators

No item

9.5.1. Report from the Paediatric Cluster Teleconference

Summary of Committee discussion:

No item

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

No item

10. Any other business

10.1. COVID -19 update

Summary of Committee discussion:

The PDCO was updated on COVID vaccines, and in particular on the available data in paediatric populations.

10.2. Marketing authorisation applications 3-year forecast report

Summary of Committee discussion:

The new features and content of the annual 3-year forecast report was presented to the PDCO. To complete the set of information on future marketing authorisation applications (MAAs) it was suggested to consider including information on PIPs in the Annex I – MAAs listing – of the report.

10.3. Introduction by the Research and Innovation Workstream

Summary of Committee discussion:

Colleagues presented a summary of recent activities in the Workstream's areas of Innovation Task Force, Horizon Scanning and Business Pipeline / Forecasting, and opened opportunities for Committee members to contribute to upcoming activities.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The session was cancelled.

11.2. Neonatology

Summary of Committee discussion:

The session was cancelled.

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 18-21 May 2021 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	2.1.25. Fully human neutralizing immunoglobulin G-1 kappa monoclonal antibody directed against a conserved epitope on the SARS CoV1 and 2 spike protein - EMEA-002899-PIP01-20
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Marleen Renard	Member	Belgium	No participation in final deliberations and voting on: No participation in discussion, final deliberations and voting on:	3.3.19. Ibrutinib - Orphan - EMEA-001397-PIP03-14 -M06 3.1.16. Ruxolitinib - EMEA-002618-PIP03-21
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Georgios Savva	Member	Cyprus	No interests declared	
Tereza Bazantova	Alternate	Czechia	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Anne Paavola	Alternate	Finland	No interests declared	
Sylvie Benchetrit	Member	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member (Vice-Chair)	Germany	No interests declared	
Yuansheng	Alternate	Germany	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Sun				
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Mountaki	Alternate	Greece	No interests declared	
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No participation in discussion, final deliberations and voting on:	3.3.35. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03
Dovile Zacharkiene	Member	Lithuania	No interests declared	
Silvijus Abramaviciu s	Alternate	Lithuania	No restrictions applicable to this meeting	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No participation in discussion, final deliberations and voting on:	2.3.2. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19-M01
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 restrictions applicable to this meeting	
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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Eva Agurell	Member	Sweden	No interests declared	
Sara Vennberg	Alternate	Sweden	No interests declared	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	3.3.35. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	3.3.35. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03 2.3.24. COVID-19 Vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M01
Jaroslav Sterba	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Alternate	Patients' Organisation Representative	No interests declared	
Michal Odermarsky	Alternate	Patients' Organisation Representative	No participation in final deliberations and voting on:	3.3.24. Selexipag - EMEA-000997-PIP01-10-M05 3.3.2.Sacubitril/valsartan - EMEA-000316-PIP02-11-M05
María Estela Moreno Martín	Expert - via telephone*	Spain	No interests declared	
Lutz Wiesner	Expert - via telephone*	Germany	No interests declared	
Franziska Wolter	Expert - via telephone*	Germany - BfArM	No interests declared	
Ann Marie Janson Lang	Expert - via telephone*	Sweden - MPA	No interests declared	
Gaby Lydia Wangorsch	Expert - via telephone*	Germany - PEI	No interests declared	
Victor Mangas Sanjuan	Expert - via telephone*	Spain	No interests declared	
Flora Musuamba Tshinanu	Expert - via telephone*	Belgium	No restrictions applicable to this meeting	
Jean-Michel Dogné	Expert - via telephone*	Belgium	No interests declared	
Martine	Expert - via	Belgium	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Sabbe	telephone*			
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/