25 March 2022
EMA/PDCO/134913/2022
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 22-25 February 2022

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

Disclaimers

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

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

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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Table of contents

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.

1.2. Adoption of agenda

The agenda for 22-25 February 2022 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 18-21 January 2022 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. Abelacimab - EMEA-003017-PIP01-21

Anthos Therapeutics, Inc.; Treatment of thromboembolic events / Prevention of

thromboembolic events

Day 120 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee a positive opinion for the PIP for abelacimab in children from 28 days to less than 18 years of age, in the condition of prevention of thromboembolic events was adopted. The PDCO agreed on a waiver from birth to less than 28 days on the grounds that the specific medicinal product is likely to be ineffective. The PDCO granted a deferral for the completion of this PIP.
Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO also agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for abelacimab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of thromboembolic events based on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as the needs are already covered.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.2. Dupilumab - EMEA-001501-PIP09-21

Sanofi-Aventis Groupe; Treatment of chronic inducible cold urticaria

Day 120 opinion

Dermatology

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 (dupilumab) for the paediatric population from 2 years of age in the condition of treatment of chronic inducible cold urticaria was adopted. The PDCO agreed on a waiver in children below 2 years of age on the grounds of 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. The PDCO granted a deferral for the completion of this PIP.

2.1.3. EMEA-003027-PIP01-21

Treatment of hyperphenylalaninaemia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 23 February 2022

2.1.4. Benzylamine derivative of benzofuran (BCX9930) - EMEA-002974-PIP01-21

BioCryst Ireland Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Day 120 opinion

Haematology-Hemostaseology

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 in children and adolescents from 2 years to less than 18 years of age, in the condition of treatment of paroxysmal nocturnal haemoglobinuria was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The PDCO granted a deferral for the completion of this PIP.

2.1.5. Rozanolixizumab - Orphan - EMEA-002681-PIP02-21

UCB Pharma S.A; Treatment of immune thrombocytopenia

Day 120 opinion

Haematology-Hemostaseology

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 in children and adolescents from 2 years to less than 18 years of age, in the condition of treatment of immune thrombocytopenia was adopted. The PDCO agreed on a waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit. The PDCO granted a deferral for the completion of this PIP.

2.1.6. Censavudine - EMEA-003075-PIP01-21

Transposon Therapeutics, Inc.; Treatment of Aicardi-Goutières syndrome

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the January 2022 plenary meeting, an application for a paediatric investigation plan for censavudine for treatment of Aicardi–Goutières syndrome.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant as well as the methodology assessment by MSWP between Day 90 and Day 120.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion was adopted.

2.1.7. Invimestrocel - EMEA-002317-PIP02-21

ReGenesys BVBA (Athersys); Treatment of ischaemic stroke

Day 120 opinion

Neurology

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 invimestrocel for the paediatric population from birth to less than 18 years of age, in the condition of treatment of ischaemic stroke was adopted. The PDCO granted a deferral for the completion of this PIP.

2.1.8. Catequentinib - Orphan - EMEA-002486-PIP04-21

Advenchen Laboratories, LLC; Treatment of soft tissue sarcomas / Treatment of Ewing sarcoma

Day 120 opinion

Oncology

Summary of Committee discussion:

In the written response the applicant addressed the issues raised by the Committee at Day 90. Based on the assessment of this application, the additional information provided by the applicant and the additional changes implemented in the development plan, a positive opinion for the PIP for the proposed medicine for the paediatric population from birth to less than 18 years of age, in the conditions of treatment of soft tissue sarcomas and treatment of Ewing sarcoma was adopted. The PDCO granted a deferral for the completion of this PIP.

2.1.9. Autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LLV, encoding for the human α-L-iduronidase (IDUA) gene (OTL-203) - Orphan - EMEA-003001-PIP01-21

Orchard Therapeutics (Netherlands) B.V.; Treatment of mucopolysaccharidosis type I, Hurler syndrome

Day 120 opinion

Other

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 in children from 28 days to less than 18 years of age, in the condition of treatment of mucopolysaccharidosis type I, Hurler syndrome was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 18 days on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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

FibroGen, Inc.; Treatment of Duchenne muscular dystrophy

Day 120 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant in February 2022 the PDCO agreed a PIP including a waiver for paediatric population from birth to less than 2 years of age including a deferral for pamrevlumab in treatment of Duchenne muscular dystrophy.

The PIP included one non-clinical study and four clinical studies in ambulant and non-ambulant patients, complemented by two modelling and simulation analysis also in support of extrapolation.

2.1.11. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A/H5N1 - EMEA-002869-PIP01-21

Seqirus Netherlands B.V.; Influenza due to identified zoonotic or pandemic influenza virus

Day 120 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted a positive opinion on influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A/H5N1 for the prevention of influenza due to identified zoonotic or pandemic influenza virus. The PIP covered all ages from birth to less than 18 years. Study 1 of the PIP is already concluded, while Studies 2 to 4 will be triggered in case of a declaration by the WHO of an influenza A/H5 or A/Hx virus pandemic.

2.1.12. Crovalimab - EMEA-002709-PIP02-21

Guillain–Barré syndrome

Day 60 opinion

Neurology

Note: Withdrawal request received on 14 February 2022

2.1.13. Acetylsalicylic acid / ticagrelor - EMEA-003146-PIP01-21

PharOS Pharmaceutical Oriented Services Single Member Ltd; Prevention of atherothrombotic events

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for acetylsalicylic acid / ticagrelor for all subsets of the paediatric population (0 to 18 years of age) in the condition of prevention of atherothrombotic events on the grounds that this condition does not occur in paediatric population.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.14. Telmisartan / indapamide - EMEA-003151-PIP01-21

KRKA, d.d., Novo mesto; Treatment of hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for telmisartan / indapamide for all subsets of the paediatric population (0 to 18 years of age) for the condition of treatment of hypertension on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.15. Adalimumab conjugated with (4S)-4-[2-(2-bromoacetamido)acetamido]-5-{3-[(4-{(4aR,4bS,5S,6aS,6bS,8R,9aR,10aS,10bS)-5-hydroxy-4a,6a-dimethyl-2-oxo-6b-[(phosphonooxy)acetyl]-4a,4b,5,6,6a,6b,9a,10,10a,10b,11,12-dodecahydro-2H,8H-naphtho[2',1':4,5]indeno[1,2-d][1,3]dioxol-8-yl}phenyl)methyl] anilino}-5-oxopentanoic acid; ABBV-154 - EMEA-002927-PIP02-21

AbbVie Ltd; Treatment of polymyalgia rheumatica

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for adalimumab conjugated with (4S)-4-[2-(2-bromoacetamido)acetamido]-5-{3-[(4-{(4aR,4bS,5S,6aS,6bS,8R,9aR,10aS,10bS)-5-hydroxy-4a,6a-dimethyl-2-oxo-6b-[(phosphonooxy)acetyl]-4a,4b,5,6,6a,6b,9a,10,10a,10b,11,12-dodecahydro-2H,8H-naphtho[2',1':4,5]indeno[1,2-d][1,3]dioxol-8-yl}phenyl)methyl] anilino}-5-oxopentanoic acid (ABBV-154) for all subsets

of the paediatric population (0 to 18 years of age) in the condition of polymyalgia rheumatica on the grounds that this condition does not occur in paediatric population.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.16. Secukinumab - EMEA-000380-PIP09-21

Novartis Europharm Limited; Treatment of lichen planus (including mucosal lichen planus)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for secukinumab for all subsets of the paediatric population from birth to less than 18 years of age for the condition of treatment of lichen planus (including mucosal lichen planus) on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2.1.17. Cevostamab - Orphan - EMEA-003145-PIP01-21

Roche Registration GmbH; Treatment of multiple myeloma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2022 plenary meeting, an application for a product specific waiver for cevostamab, a bispecific antibody targeting FcRH5, for the treatment of multiple myeloma on the grounds that the disease does not occur in the paediatric population.
The PDCO confirmed all conclusions reached at Day 30 and adopted a positive opinion at Day 60 on a product specific waiver for this product for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of multiple myeloma" on the grounds that this disease does not occur in the paediatric population.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.18. Human IgG4 monoclonal antibody against BCMA and CD3 - EMEA-003147-PIP01-21

AbbVie Ltd; Treatment of multiple myeloma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2022 plenary meeting, an application for a product specific waiver for a human IgG4 monoclonal bispecific antibody against B-cell maturation antigen (BCMA) and CD3 for the treatment of multiple myeloma on the grounds that the disease does not occur in the paediatric population.
The PDCO confirmed all conclusions reached at Day 30 and adopted a positive opinion at Day 60 on a product specific waiver for this product for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of multiple myeloma" on the grounds that this disease does not occur in the paediatric population.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.19. Retifanlimab - Orphan - EMEA-002798-PIP02-21

Incyte Biosciences Distribution B.V.; Treatment of endometrial carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for retifanlimab for all subsets of the paediatric population (birth to less than 18 years of age) for treatment of endometrial cancer based on the ground that the disease does not occur in children. Since the agreed waiver ground is disease not occurring, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.20. Sintilimab - EMEA-002919-PIP02-21

Eli Lilly and Company Limited; Treatment of oesophageal cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for sintilimab for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of oesophageal cancer based on the ground that the disease does not occur in children. Since the agreed waiver ground is disease not occurring, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.21. Influenza virus A/turkey/turkey/1/2005 (H5N1) NIBRG-23 strain, HA surface antigen - EMEA-002869-PIP03-21

Seqirus Netherlands B.V.; Influenza due to identified zoonotic or pandemic influenza virus

Day 60 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted a positive opinion for a PIP for influenza virus A/turkey/Turkey/1/2005 (H5N1) NIBRG-23 strain, HA surface antigen for children from 6 months of age to less than 18 years of age in the condition prevention of zoonotic influenza. A waiver was granted for children below 6 months of age.

2.2. Opinions on Compliance Check

2.2.1. Dupilumab - EMEA-C-001501-PIP01-13-M07

Regeneron Pharmaceuticals, Inc.; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

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

- EMEA-C1-001501-PIP01-13-M03
- EMEA-C2-001501-PIP01-13-M05
- EMEA-C3-001501-PIP01-13-M05
- EMEA-C4-001501-PIP01-13-M06

The PDCO adopted on 25 February 2022 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0329/2021) of 11 August 2021.

2.2.2. Ligelizumab - EMEA-C2-001811-PIP02-15-M04

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 60 letter

Dermatology

Summary of Committee discussion:

The PDCO considered the applicant's responses satisfactory.
Study 2 (CQGE031C2202) and Study 6 (Modelling and Simulation Activity 2) are therefore considered compliant with the latest Agency's Decision (P/0308/2021) of 11 August 2021.
The PDCO finalised this partially completed compliance procedure on 25 February 2022.

2.2.3. Cobicistate / atazanavir sulphate - EMEA-C2-001465-PIP01-13-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 letter

Infectious Diseases

Summary of Committee discussion:

The PDCO discussed the completed Study 4 and considered that this is compliant with the latest Agency's Decision (P/0510/2020) of 22 December 2020.
The PDCO finalised this partially completed compliance procedure on 25 February 2022.

2.2.4. Eribulin - EMEA-C-001261-PIP01-11-M07

Eisai GmbH; Treatment of soft tissue sarcoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2022 plenary meeting, an application for a compliance check for eribulin for the treatment of soft tissue sarcoma.
The PDCO took note of the information the applicant provided between Day 30 and Day 60 as well as of the outcomes of preceding partial compliance check procedures:

- EMEA-C2-001261-PIP01-11-M02

and adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0535/2021) of 6 December 2021.

2.2.5. Ibrutinib - EMEA-C-001397-PIP03-14-M06

Janssen-Cilag International NV; Treatment of mature B cell neoplasm

Day 30 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 30, during the February 2022 plenary meeting, an application for a compliance check for ibrutinib for the treatment of mature B cell neoplasms.
The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-001397-PIP03-14-M02
- EMEA-C2-001397-PIP03-14-M03

and adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0337/2021) of 11 August 2021.

2.2.6. Dexmedetomidine hydrochloride - EMEA-C2-002758-PIP01-19-M01

BioXcel Therapeutics, Inc.; Treatment of bipolar disorder / Treatment of schizophrenia

Day 30 letter

Psychiatry

Summary of Committee discussion:

The PDCO discussed the completed Study 3 and the initiation of Study 2, and considered that these are compliant with the latest Agency's Decision (P/0568/2021) of 7 January 2022.
The PDCO finalised this partially completed compliance procedure at D30.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Migalastat hydrochloride - Orphan - EMEA-001194-PIP01-11-M05

Amicus Therapeutics Europe Limited; Treatment of Fabry 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/0137/2019 of 17 April 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Saxagliptin - EMEA-000200-PIP01-08-M09

AstraZeneca AB; Treatment of type 2 diabetes

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 one of two 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/0277/2019 of 16 August 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Eluxadoline - EMEA-001579-PIP01-13-M05

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

Day 60 opinion

Gastroenterology-Hepatology

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 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/0107/2020 of 18 March 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Ozanimod - EMEA-001710-PIP04-17-M03

Celgene Europe B.V.; Treatment of Crohn's disease

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/0067/2021 of 18 February 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Bezlotoxumab - EMEA-001645-PIP01-14-M04

Merck Sharp & Dohme (Europe), Inc.; Prevention of recurrence *of Clostridioides difficile* 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/0104/2020 of 20 March 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Brincidofovir - Orphan - EMEA-001904-PIP02-17-M01

SymBio Pharmaceuticals Limited; Treatment of adenovirus in immunocompromised patients

Day 60 opinion

Infectious Diseases

Note: Withdrawal request received on 4 February 2022

2.3.7. Cabotegravir - EMEA-001418-PIP02-15-M03

ViiV Healthcare UK Limited; Prevention 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 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/0118/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Rilpivirine (hydrochloride) - EMEA-000317-PIP01-08-M13

Janssen-Cilag International NV; 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 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/0291/2020 issued on 12 August 2020. The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Cannabidiol / delta-9-tetrahydrocannabinol - EMEA-000181-PIP01-08-M06

GW Pharma (International) B.V; Treatment of spasticity

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 (P/0316/2016 of 2 December 2016). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion. The PDCO wishes to remind the applicant once more of the importance to update the product's SmPC with the results of the negative trial.

2.3.10. Galcanezumab - EMEA-001860-PIP03-16-M07

Eli Lilly and Company Limited; Prevention of migraine headaches

Day 60 opinion

Neurology

Summary of Committee discussion:

In February 2022 the PDCO re-discussed the modification request taking into account additional information provided following the D30 discussion. PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0449/2021 of 29 October 2021).

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

2.3.11. Leriglitazone - Orphan - EMEA-002106-PIP01-16-M02

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 60 opinion

Neurology

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 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/0412/2021 of 29 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Ofatumumab - EMEA-002397-PIP01-18-M02

Novartis Ireland Limited; 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 (P/0042/2021 of 29 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Siponimod (hemifumarate) - EMEA-000716-PIP01-09-M04

Novartis Europharm 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 (P/0014/2021 of 28 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. (4S,7aR,9aR,10S,11E,14S,15R)-6'-chloro-10-methoxy-14,15-dimethyl-3',4',7a,8,9,9a,10,13,14,15-decahydro-2'H,7H-spiro[1,19-ethenocyclobuta[i][1,4]oxazepino[3,4f][1,2,7]thiadiazacyclohexadecine-4,1'-naphthalen]-18(17H)-one 16,16-dioxide (AMG 176) - EMEA-002631-PIP01-19-M01

Amgen Europe BV; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as

set in the Agency's latest decision (P/0331/2020 of 24 August 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Axicabtagene ciloleucel - Orphan - EMEA-002010-PIP01-16-M03

Kite Pharma EU B.V.; Treatment of mature B cell neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2022 plenary meeting, this request for modification for axicabtagene ciloleucel for the treatment of mature B cell neoplasms. The applicant requested to convert this PIP into a product-specific waiver on the grounds of lack of significant therapeutic benefit.
The PDCO confirmed all conclusions reached at Day 30 and adopted a positive opinion at Day 60 on the modification of the agreed PIP as set in the Agency's latest decision (P/0132/2020 of 15 April 2020) to a product specific waiver for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of mature B cell neoplasms" on the grounds of lack of significant therapeutic benefit over existing treatments for paediatric patients.

2.3.16. Bempegaldesleukin - EMEA-002492-PIP01-18-M02

Nektar Therapeutics; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue neoplasms)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2022 plenary meeting, this request for modification for bempegaldesleukin for the treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue neoplasms). The applicant requested to defer completion of a PIP study.
The PDCO confirmed all conclusions reached at Day 30, took into consideration information provided by the applicant between Day 30 and Day 60 and adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0273/2020 of 15 July 2020).

2.3.17. Palbociclib - EMEA-002146-PIP01-17-M04

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma

Day 60 opinion

Oncology

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 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/0426/2021 of 29 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Ponatinib - Orphan - EMEA-001186-PIP01-11-M03

Incyte Biosciences Distribution B.V.; Treatment of Philadelphia chromosome positive acute lymphoblastic leukaemia / Treatment of chronic myeloid leukaemia

Day 60 opinion

Oncology

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/0293/2018 of 12 September 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M05

Pfizer Europe MA EEIG; Treatment of B cell acute lymphoblastic leukaemia

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the February 2022 plenary meeting, a request for modification for inotuzumab ozogamicin for the treatment of B cell acute lymphoblastic leukaemia.
The applicant requested to modify the description of the study population in clinical Study 2 and to clarify how patients will be followed up in the same study.
The PDCO took into consideration the conclusions reached at Day 30 and further information the applicant provided between Day 30 and Day 60 and adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0478/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Fosdenopterin - Orphan - EMEA-001491-PIP01-13-M02

Comharsa Life Sciences Limited; Treatment of molybdenum cofactor deficiency type A

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 change to adapt the term of the pharmaceutical form from "powder for solution for infusion" to "powder for solution for injection" 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/0048/2021 of 27 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Amikacin sulfate - Orphan - EMEA-000525-PIP01-08-M08

Insmed Netherlands B.V.; Treatment of pseudomonas aeruginosa lung infection/colonisation in cystic fibrosis patients / Treatment of nontuberculous mycobacterial (NTM) lung infection

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could not be accepted as proposed. The PDCO agreed a waiver on own motion for the condition 'treatment of pseudomonas aeruginosa lung infection/colonisation in cystic fibrosis patients' for the paediatric population from birth to less than 18 years of age on the grounds that the specific medicinal product is likely to be ineffective.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0358/2021 of 8 September 2021). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Benralizumab - EMEA-001214-PIP01-11-M11

AstraZeneca AB; Treatment of asthma

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

In a written response the applicant addressed the remaining issues raised by the Committee.
The PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0244/2018 of 15 August 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Daridorexant - EMEA-002121-PIP03-19-M01

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of insomnia

Day 60 opinion

Psychiatry

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/0131/2020 of 15 April 2020). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Esketamine (hydrochloride) - EMEA-001428-PIP03-15-M02

Janssen-Cilag International NV; Treatment of major depressive disorder

Day 60 opinion

Psychiatry

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/0238/2019 of 16 July 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.25. Lisdexamfetamine dimesylate - EMEA-000553-PIP01-09-M05

Shire Pharmaceuticals Contract Limited; Treatment of attention deficit hyperactivity disorder

Day 60 opinion

Psychiatry

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/0321/2013 of 19 December 2013).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. Etelcalcetide - EMEA-001554-PIP01-13-M03

Amgen Europe B.V.; Treatment of hyperparathyroidism

Day 60 opinion

Uro-nephrology

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/0173/2018 of 15 June 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.27. Ferumoxytol - EMEA-000373-PIP02-09-M05

Covis Pharma Europe B.V.; Treatment of iron deficiency anaemia

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/0060/2015 of 1 April 2015).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Recombinant influenza hemagglutinin-strain B (Yamagata lineage) / Recombinant influenza hemagglutinin-strain B (Victoria lineage) / Recombinant influenza hemagglutinin-strain A (H3N2 subtype) / Recombinant influenza hemagglutinin-strain A (H1N1 subtype) - EMEA-002418-PIP01-18-M02

Sanofi Pasteur; Prevention of influenza infection

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/0219/2019 of 17 June 2019).

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

2.4. Opinions on Re-examinations

2.4.1. L-carnitine / glucose/calcium chloride dihydrate / magnesium chloride hexahydrate / sodium lactate / sodium chloride - EMEA-003049-PIP01-21

Iperboreal Pharma Srl; Treatment of renal failure with carnitine deficiency

Day 30 opinion

Uro-nephrology

Summary of Committee discussion:

The PDCO discussed the applicant's request for re-examination to grant a deferral for the initiation of Study 1 (IP-001-2021).

An oral explanation, as requested by the applicant, took place on 23 February 2022.

After further internal discussion the PDCO concluded by vote not to agree to grant a deferral for the initiation of Study 1, maintaining the previous PDCO position.

The PDCO adopted a positive opinion on the PIP for L-carnitine / glucose / calcium chloride dihydrate / magnesium chloride hexahydrate / sodium lactate / sodium chloride for treatment of renal failure with carnitine deficiency in the paediatric population from birth to less than 18 years of age, including a deferral for the PIP studies except for the initiation of Study 1.

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. Gadopiclenol - EMEA-C2-001949-PIP01-16-M04

Guerbet; Detection and visualisation of areas with disruption of the blood brain barrier and/or abnormal vascularity for diagnostic purposes

Day 30 letter

Diagnostic

2.7.2. Gadopiclenol - EMEA-C2-001949-PIP02-18-M01

Guerbet; Detection and visualisation of disorders or lesions with suspected abnormal vascularity in various body regions for diagnostic purposes

Day 30 letter

Diagnostic

2.7.3. Dalbavancin hydrochloride - EMEA-C2-000016-PIP01-07-M08

Allergan Pharmaceuticals International Limited; Treatment of acute bacterial skin and skin structure infections

Day 30 letter

Infectious Diseases

2.7.4. Dupilumab - EMEA-C1-001501-PIP04-19-M01

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 30 letter

Gastroenterology-Hepatology

2.7.5. Sacubitril / valsartan - EMEA-C2-000316-PIP02-11-M05

Novartis Europharm Ltd; Treatment of heart failure

Day 30 letter

Cardiovascular Diseases

2.7.6. Recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2 (INN: narsoplimab) - EMEA-C2-002479-PIP01-18-M01

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

Day 30 letter

Haematology-Hemostaseology

2.7.7. Alpelisib - EMEA-C1-002016-PIP03-19-M01

Novartis Europharm Limited; Treatment of PIK3CA related overgrowth spectrum

Day 30 letter

Other

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. Ibutamoren mesylate - Orphan - EMEA-003032-PIP01-21

Lumos Pharma, Inc.; Treatment of growth hormone deficiency

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP02-20

Treatment of obesity

Day 90 discussion

Gastroenterology-Hepatology

3.1.3. Alisitol / retinyl palmitate / zinc gluconate - Orphan - EMEA-002198-PIP01-21

Vanessa Research Magyarorszag Kft/Vanessa Research Hungary ltd; Treatment of microvillus inclusion disease

Day 90 discussion

Gastroenterology-Hepatology

3.1.4. Mitapivat - Orphan - EMEA-002684-PIP02-21

Agios Netherlands B.V.; Treatment of thalassaemia

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Deucravacitinib - EMEA-002350-PIP02-20

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.6. Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase - Orphan - EMEA-003020-PIP01-21

Lysogene; Treatment of GM1 gangliosidosis

Day 90 discussion

Neurology

3.1.7. Lorlatinib - EMEA-002669-PIP03-21

ALK-aberrant neuroblastoma

Day 90 discussion

Oncology

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

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Vaccines

3.1.9. SARS-CoV-2 virus, beta-propiolactone inactivated - EMEA-003077-PIP01-21

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Vaccines

3.1.10. Asundexian - EMEA-003144-PIP01-21

Prevention of arterial thromboembolism

Day 60 discussion

Cardiovascular Diseases

3.1.11. Oxytocin - Orphan - EMEA-003148-PIP01-21

OT4B; Treatment of Prader-Willi syndrome

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.12. Etrasimod L-arginine - EMEA-002713-PIP02-21

Treatment of Crohn's disease

Day 60 discussion

Gastroenterology-Hepatology

3.1.13. EMEA-003143-PIP01-21

Treatment of ulcerative colitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.14. Ianalumab - EMEA-002338-PIP03-21

Treatment of systemic lupus erythematosus (SLE)

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.15. Tocilizumab - EMEA-000309-PIP09-21

Treatment of systemic sclerosis

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.16. A fully human IgG1 monoclonal antibody targeting an epitope in the receptor-binding domain of the spike glycoprotein of SARS-CoV-2 - EMEA-003118-PIP01-21

Treatment and prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Infectious Diseases

Note: Withdrawal request received on 11 February 2022

3.1.17. Ensovibep - EMEA-003150-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Infectious Diseases

3.1.18. Remibrutinib - EMEA-002582-PIP02-21

Treatment of multiple sclerosis

Day 60 discussion

Neurology

3.1.19. EMEA-003110-PIP02-21

Treatment of neurofibromatosis type 1

Day 60 discussion

Oncology

3.1.20. Odronextamab - EMEA-003149-PIP01-21

Aggressive mature B cell non-Hodgkin lymphoma (B-NHL)

Day 60 discussion

Oncology

3.1.21. EMEA-003141-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Other

Note: Withdrawal request received on 10 February 2022

3.1.22. Apitegromab - Orphan - EMEA-002951-PIP02-21

Scholar Rock, Inc.; Spinal muscular atrophy

Day 60 discussion

Other / Neurology

3.1.23. RSV F protein - EMEA-003094-PIP02-21

Prevention of respiratory tract disease caused by respiratory syncytial virus

Day 60 discussion

Vaccines / Infectious Diseases

3.1.24. Amlodipine / indapamide / perindopril arginine / atorvastatin - EMEA-003173-PIP01-21

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.25. Cedirogant - EMEA-003142-PIP02-21

Treatment of moderate to severe psoriasis

Day 30 discussion

Dermatology

3.1.26. Dupilumab - EMEA-001501-PIP11-21

Treatment of chronic pruritus of unknown origin

Day 30 discussion

Dermatology

3.1.27. Sirolimus - Orphan - EMEA-003168-PIP01-21

Desitin Arzneimittel GmbH; Treatment of tuberous sclerosis

Day 30 discussion

Dermatology

3.1.28. Manganese chloride tetrahydrate - EMEA-003035-PIP02-21

Diagnostic evaluation of liver lesions by magnetic resonance imaging (MRI)

Day 30 discussion

Diagnostic

3.1.29. Avexitide acetate - Orphan - EMEA-003125-PIP02-21

EigerBio Europe Limited; Treatment of congenital hyperinsulinism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.30. Glucagon analogue linked to a human immunoglobulin Fc fragment - Orphan - EMEA-003170-PIP01-21

Hanmi Pharm. Co., Ltd.; Treatment of congenital hyperinsulinism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.31. Insulin lispro - EMEA-003166-PIP01-21

Treatment of diabetes mellitus type 2 / Treatment of diabetes mellitus type 1

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.32. Semaglutide - EMEA-001441-PIP07-21

Treatment of obesity

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Efavaleukin alfa - EMEA-003156-PIP01-21

Systemic lupus erythematosus (SLE)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.34. Vilobelimab - EMEA-003080-PIP02-21

SARS-Cov-2 induced severe pneumonia

Day 30 discussion

Immunology-Rheumatology-Transplantation / Infectious Diseases / Dermatology / Oncology

Note: Withdrawal request received on 3 February 2022

3.1.35. HIV-1 maturation Inhibitor - EMEA-003153-PIP01-21

Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.1.36. HIV-1 maturation inhibitor / dolutegravir - EMEA-003152-PIP01-21

Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.1.37. Interferon beta-1a - EMEA-003056-PIP01-22

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.1.38. Adeno-associated virus vector serotype 1 containing the human GRN gene - Orphan - EMEA-003167-PIP01-21

Passage Bio, Inc.; Treatment of frontotemporal dementia

Day 30 discussion

Neurology

3.1.39. Anti-CD40L humanized monoclonal antibody - EMEA-002945-PIP02-21

Multiple sclerosis

Day 30 discussion

Neurology

3.1.40. Batoclimab - EMEA-003162-PIP01-21

Generalised myasthenia gravis

Day 30 discussion

Neurology

3.1.41. Izaflortaucipir (^{18}F) - EMEA-003040-PIP02-21

Diagnosis of Alzheimer's disease

Day 30 discussion

Neurology

3.1.42. Latozinemab - Orphan - EMEA-002997-PIP02-22

Alector, Inc.; Treatment of amyotrophic lateral sclerosis

Day 30 discussion

Neurology

3.1.43. Pridopidine hydrochloride - Orphan - EMEA-003174-PIP01-21

Prilenia Therapeutics B.V.; Treatment of Huntington disease (HD)

Day 30 discussion

Neurology

3.1.44. Satralizumab - Orphan - EMEA-001625-PIP03-21

Roche Registration GmbH; Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 30 discussion

Neurology

3.1.45. EMEA-003161-PIP01-21

Treatment of mature B cell neoplasms

Day 30 discussion

Oncology

3.1.46. EMEA-003169-PIP01-21

Treatment of gastrointestinal stromal tumours

Day 30 discussion

Oncology

3.1.47. Camidanlumab tesirine - EMEA-003160-PIP01-21

Treatment of Hodgkin lymphoma

Day 30 discussion

Oncology

3.1.48. Emactuzumab - EMEA-003172-PIP01-21

Treatment of tenosynovial giant cell tumour, local and diffuse type

Day 30 discussion

Oncology

3.1.49. Human IgG4-based bispecific antibody binding to both B-cell maturation antigen (BCMA) and cluster of differentiation 3 (CD3) - EMEA-003175-PIP01-21

Treatment of multiple myeloma

Day 30 discussion

Oncology

3.1.50. Infigratinib - Orphan - EMEA-002594-PIP03-21

Helsinn Birex Pharmaceuticals Ltd.; Treatment of urothelial carcinoma

Day 30 discussion

Oncology

3.1.51. Obecabtagene autoleucel - EMEA-003171-PIP01-21

Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Oncology

3.1.52. Sacituzumab govitecan - Orphan - EMEA-002645-PIP03-21

Gilead Sciences International Ltd.; Treatment of lung carcinoma

Day 30 discussion

Oncology

3.1.53. Zandelisib - EMEA-003158-PIP01-21

Mature B cell neoplasms

Day 30 discussion

Oncology

3.1.54. EMEA-003159-PIP01-21

Treatment of idiopathic pulmonary fibrosis (IPF)

Day 30 discussion

Pneumology - Allergology

3.1.55. Freeze-dried allergen extract of *Betula pendula* pollen - EMEA-003117-PIP02-21

Diagnosis of IgE mediated allergy to tree pollen of the birch group

Day 30 discussion

Pneumology - Allergology

3.1.56. Pseudoephedrine / bilastine - EMEA-003164-PIP01-21

Treatment of allergic rhinitis

Day 30 discussion

Pneumology - Allergology

3.1.57. Cannabidiol - EMEA-003176-PIP01-21

Treatment of fragile X syndrome (FXS)

Day 30 discussion

Psychiatry

3.1.58. Atrasentan - Orphan - EMEA-001666-PIP02-21

Chinook Therapeutics, Inc.; Treatment of primary IgA nephropathy

Day 30 discussion

Uro-nephrology

3.1.59. EMEA-003165-PIP01-21

Treatment of chronic kidney disease

Day 30 discussion

Uro-nephrology

3.1.60. Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc - EMEA-003157-PIP01-21

Treatment of focal segmental glomerulosclerosis (FSGS)

Day 30 discussion

Uro-nephrology

3.1.61. Repagermanium - Orphan - EMEA-003154-PIP01-21

Dimerix Bioscience Pty Ltd; Treatment of focal segmental glomerulosclerosis (FSGS)

Day 30 discussion

Uro-nephrology

3.1.62. Pneumococcal polysaccharide serotype 35B – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 31 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 24F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 23B – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 23A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 16F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 15C – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 15A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 20 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 17F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 12F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 11A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 10A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 9N – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 8 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate - EMEA-003155-PIP01-21

Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Vaccines

3.1.63. Yellow fever virus, strain vYF-247 - EMEA-003030-PIP02-21

Prevention of yellow fever disease

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. Treosulfan - EMEA-C-000883-PIP01-10-M05

medac Gesellschaft für klinische Spezialpräparate mbH; Conditioning treatment prior to haematopoietic progenitor cell transplantation

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.2. Casirivimab - EMEA-C2-002964-PIP01-21

Regeneron Ireland DAC; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

Note: Withdrawal request received on 3 February 2022

3.2.3. Imdevimab - EMEA-C2-002965-PIP01-21

Regeneron Ireland DAC; Treatment of coronavirus disease 2019 (COVID-19)

Infectious Diseases

Note: Withdrawal request received on 3 February 2022

3.2.4. Oritavancin - EMEA-C2-001270-PIP01-12-M04

Menarini International Operations Luxembourg S.A.; Treatment of acute bacterial skin and skin structure infections

Day 30 discussion

Infectious Diseases

3.2.5. Dabrafenib - EMEA-C3-001147-PIP01-11-M07

Novartis Europharm Limited; Treatment of solid malignant tumours (excluding melanoma and glioma)

Day 30 discussion

Oncology

3.2.6. Trametinib - EMEA-C2-001177-PIP01-11-M06

Novartis Europharm Limited; Treatment of melanoma

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Regdanvimab - EMEA-002961-PIP01-21-M01

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

Day 30 discussion

Vaccines

3.3.2. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20-M01

Amgen Europe B.V.; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.3.3. Remibrutinib - EMEA-002582-PIP01-19-M01

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

3.3.4. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19-M02

Alexion Europe S.A.S.; Treatment of Wilson disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Dapagliflozin - EMEA-000694-PIP02-14-M03

AstraZeneca AB; Treatment of type 1 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Tirzepatide - EMEA-002360-PIP01-18-M02

Eli Lilly and Company Ltd; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Tolvaptan - EMEA-001231-PIP02-13-M09

Otsuka Pharmaceutical Netherlands B.V.; Treatment of polycystic kidney disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

3.3.8. Odevixibat - Orphan - EMEA-002054-PIP01-16-M03

Albireo AB; Treatment of progressive familial intrahepatic cholestasis (PFIC)

Day 30 discussion

Gastroenterology-Hepatology

3.3.9. Potassium sulphate / magnesium sulphate heptahydrate / sodium sulphate anhydrous - EMEA-000816-PIP02-10-M03

IPSEN Consumer Healthcare; Diagnostic of organic and/or functional bowel diseases

Day 30 discussion

Gastroenterology-Hepatology

3.3.10. Betibeglogene autotemcel - Orphan - EMEA-001665-PIP01-14-M06

bluebird bio (Netherlands) B.V.; Treatment of beta-thalassaemia

Day 30 discussion

Haematology-Hemostaseology

3.3.11. Luspatercept - Orphan - EMEA-001521-PIP01-13-M06

Bristol-Myers Squibb Pharma EEIG; Treatment of beta-thalassaemia / Treatment of myelodysplastic syndromes

Day 30 discussion

Haematology-Hemostaseology

3.3.12. Brincidofovir - Orphan - EMEA-001904-PIP03-18-M02

Chimerix IRL Limited; Treatment of smallpox

Day 30 discussion

Infectious Diseases

3.3.13. Cefiderocol - EMEA-002133-PIP01-17-M02

Shionogi B.V.; Treatment of infections due to aerobic gram-negative bacteria

Day 30 discussion

Infectious Diseases

3.3.14. Eslicarbazepine acetate - EMEA-000696-PIP02-10-M08

BIAL - Portela & Ca, SA; Treatment of epilepsy with partial onset seizures

Day 30 discussion

Neurology

3.3.15. Cemiplimab - EMEA-002007-PIP02-17-M02

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

Day 30 discussion

Oncology

3.3.16. Lenvatinib - EMEA-001119-PIP03-19-M02

Eisai GmbH; Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue neoplasms, papillary thyroid cancer, follicular thyroid cancer and osteosarcoma

Day 30 discussion

Oncology

3.3.17. Venetoclax - Orphan - EMEA-002018-PIP02-16-M05

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms / Treatment of solid tumour malignant neoplasms

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.3.18. Sonlicromanol - Orphan - EMEA-002113-PIP01-16-M01

Khondrion BV; Treatment of mitochondrial respiratory chain/oxidative phosphorylation defects

Day 30 discussion

Other

3.3.19. Ketamine / sufentanil - EMEA-001739-PIP02-16-M01

Cessatech A/S; Treatment of acute pain

Day 30 discussion

Pain

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 submissions of applications with start of procedure 21 February 2022 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:

No item

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

6.1.1. ANGPTL3 agonist - EMEA-16-2021

Novartis Europharm Limited; All classes of medicinal products for treatment of primary and secondary osteoarthrosis / Treatment of osteoarthritis (OA) of the knee

Summary of committee discussion:

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

Other potential paediatric interests of this medicine suggested by PDCO: juvenile idiopathic arthritis.

6.1.2. Astegolimab - EMEA-17-2021

Roche Registration GmbH; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft versus-host disease after [bone-marrow] transplantation) / Long-term maintenance treatment to reduce the risk of exacerbations of COPD in patients with a history of exacerbations

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 identified at this stage.

6.1.3. Plasmid expressing variant of human interleukin 10 - EMEA-18-2022

Xalud Therapeutics, Inc; All classes of medicinal products for treatment of primary and secondary osteoarthrosis / Treatment of moderate-to-severe pain; Treatment of reduced function due to osteoarthritis; and modification of osteoarthritis disease

Summary of committee discussion:

The PDCO decided to ask for additional information on the mechanism of action of action of the medicinal product before concluding on the applicability of the class-waiver request.

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 item

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

The PDCO Chair welcomed Ms Zena Gunther and Ms Maria Eleni Avraamidou representing Cyprus as the new Member and Alternate Member respectively.

The PDCO Chair expressed his thanks to Mr Georgios Savva and Ms Elena Kaisis for their contribution to the PDCO as they step down from their representation from Cyprus.

9.1.2. Vote by Proxy

No item

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 list of procedures related to medicinal products with paediatric indications to be evaluated by the CHMP, starting in January 2022, was presented to the PDCO members.

9.2.2. mRNA vaccines booster in adolescents – request from CHMP

Summary of Committee discussion:

The PDCO adopted a response to a request by CHMP on the use of real world evidence data from young adults and any additional data requirement to support boosters of mRNA vaccines in adolescents.

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.

9.3.3. Patients and Consumers Working Party (PCWP)/Healthcare Professionals Working Party (HCPWP)

Summary of Committee discussion:

The meeting summary – Annual PCWP/HCPWP meeting with all eligible organisations on 24 November 2021 and the Draft Agenda - PCWP/HCPWP joint meeting on 2-3 March 2022 were presented for information.

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

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. Accelerating Clinical Trials in the EU (ACT EU)

Summary of Committee discussion:

On the topic of clinical trial approval this remains a member state competency. The CTR does however put in place processes that facilitate a national decision through the CTCG. Advice on methodology can be given on a European level or national level. How to address the administrative hurdles of the clinical practice aspects of clinical trials could be introduced as a topic through the multi-stakeholder platform.

10.2. Multi-Stakeholder Meeting on Paediatric Atopic Dermatitis

Conect4Children meeting on 1-2 March 2022

Summary of Committee discussion:

The PDCO members were notified about the forthcoming multi-stakeholder meeting on atopic dermatitis in children and adolescents on March 1-2, 2022.

Further details have been provided on:

- scientific programme
- facilitation of new medicines evaluation in children and adolescents with atopic dermatitis
- participation of PDCO members

10.3. COVID-19 update

Summary of Committee discussion:

The Committee was updated on the most relevant paediatric developments in the area of COVID-19 vaccines.

10.4. Working party implementation update - call for nominations

Summary of Committee discussion:

The Committee was informed about the progress on the implementation of the new operational model on the working parties. The timetable for the nomination of experts was presented, including the criteria for the expertise required for experts in the different working

parties. The Committee will be kept informed of the ongoing activities of the implementation plan as well as the new composition of the working parties in Q2 2022.

11. Breakout sessions

11.1. Internal PDCO Operations

Summary of Committee discussion:

PDCO members discussed matters relating to PDCO internal operations.

11.2. Neonatology

Summary of Committee discussion:

The group discussed issues related to stem-cell related developments in the area of neonatology.

11.3. Paediatric oncology

Summary of Committee discussion:

The group discussed issues related to the inclusion of adolescents in adult clinical studies.

11.4. Vaccines

Summary of Committee discussion:

The PDCO discussed the development of new COVID vaccines that will be used as boosters in adults but may be developed also as primary vaccination series in the paediatric population.

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 22-25 February 2022 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium	No interests declared	
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:	3.2.6. Ibrutinib - EMEA-C-001397-PIP03-14-M06
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Zena Gunther	Member	Cyprus	No interests declared	
Maria Eleni Avraamidou	Alternate	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	
Pauliina Lehtolainen-Dalkilic	Member	Finland	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 Sun	Alternate	Germany	No interests declared	
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 restrictions applicable to this meeting	
Dovile Zacharkiene	Member	Lithuania	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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: No participation in final deliberations and voting on:	3.3.4. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19-M02 3.3.8. Odevixibat - Orphan - EMEA-002054-PIP01-16-M03
Maaike van Dartel	Alternate	Netherlands	No interests declared	
Siri Wang	Member	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	
Eva Agurell	Member	Sweden	No interests declared	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jaroslav Sterba	Member	Patients' Organisation Representative	No interests declared	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Alternate	Patients' Organisation	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
		Representative		
Michal Odermarsky	Alternate	Patients' Organisation Representative	No participation in final deliberations and voting on:	2.7.5. Sacubitril / valsartan - EMEA-C2-000316-PIP02-11-M05
María Estela Moreno Martín	Expert	Spain	No interests declared	
Mogens Westergaard	Expert	Denmark	No interests declared	
Filip Josephson	Expert	Sweden	No interests declared	
Jan Mueller-Berghaus	Expert	Germany	No interests declared	
Charlotta Bergquist	Expert	Sweden	No interests declared	
Annette Lommel	Expert	Germany	No interests declared	
Helena Back	Expert	Sweden	No interests declared	
Agustin Portela Moreira	Expert	Spain	No interests declared	
A representative from the European Commission attended the meeting Meeting run with support from relevant EMA staff				
* Experts were 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/