14 October 2022
EMA/PDCO/775544/2022
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 06-09 September 2022

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

Health and safety information

In accordance with the Agency's health and safety policy, delegates were briefed on health, safety and emergency information and procedures prior to the start of the 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 Chair opened the meeting by welcoming all participants. Due to the current coronavirus (COVID-19) pandemic, and the associated EMA Business Continuity Plan (BCP), the meeting was held in-person with some members connected remotely (hybrid setting).

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 on the topics in the agenda of the meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda topics.

Participants were asked to declare any changes, omissions or errors to their declared interests concerning the matters for discussion. Francesca Rocchi declared a new or additional competing interest. Restrictions applicable to this meeting are captured in the List of participants included in the minutes.

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. All decisions taken at this meeting were made in the presence of a quorum of members. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The Chair thanked the departing alternate for her contributions to the Committee.

1.2. Adoption of agenda

The agenda for 06-09 September 2022 meeting was adopted.

1.3. Adoption of the minutes

The minutes for 19-22 July 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. Treprostinil (sodium) - EMEA-003182-PIP01-22

AOP Orphan Pharmaceuticals GmbH; Treatment of pulmonary arterial hypertension

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 was adopted by the PDCO for the PIP for treprostinil (sodium) for paediatric patients from birth to less than 18 years of age, in the condition of treatment of pulmonary arterial hypertension. The waiver initially requested by the applicant was not granted, since the applicant removed this request during the procedure and agreed to include all paediatric subsets in the PIP.

2.1.2. Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22

Krystal Biotech, Inc.; Treatment of dystrophic epidermolysis bullosa

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 was adopted on a PIP for beremagene geperpavec for children from birth to less than 18 years of age in the condition of treatment of dystrophic epidermolysis bullosa.

2.1.3. Recombinant fusion protein linking iduronate 2-sulfatase to engineered Fc with binding site for transferrin receptor - EMEA-002845-PIP01-20

Denali Therapeutics Inc.; Treatment of mucopolysaccharidosis II (Hunter syndrome)

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

The PDCO re-discussed at Day 120, during the September 2022 plenary meeting, a PIP application for recombinant fusion protein linking iduronate 2-sulfatase to engineered Fc with binding site for transferrin receptor for the treatment of mucopolysaccharidosis II (Hunter syndrome).
The PDCO agreed with all conclusions reached at Day 90 and adopted a positive opinion on a paediatric investigation plan for the entire paediatric population for the treatment of mucopolysaccharidosis II (Hunter syndrome).

2.1.4. Resmetirom - EMEA-003087-PIP01-21

Madrigal Pharmaceuticals EU Limited; Treatment of non-alcoholic steatohepatitis

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 and adolescents from 8 years to less than 18 years of age in the condition of treatment of non-alcoholic steatohepatitis was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 8 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for the completion of this PIP.

2.1.5. Cenerimod - EMEA-003108-PIP01-21

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of systemic lupus erythematosus (SLE)

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

In September 2022, 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 paediatric patients from 5 years to less than 18 years of age, in the condition of systemic lupus erythematosus 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 over existing treatments. The PDCO granted a deferral for the completion of this PIP.

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

Amgen Europe B.V.; Treatment of systemic lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and additional clarifications by the applicant, and further discussions at the Paediatric Committee, a positive opinion was adopted by the PDCO for the PIP for efavaleukin alfa for paediatric patients from birth to less than 18 years of age, in the condition of treatment of systemic lupus erythematosus. A deferral was granted for the completion of this PIP.
The PDCO recommended granting a waiver for efavaleukin alfa for the paediatric population from birth to less than 5 years of age in the condition of treatment of systemic lupus erythematosus.

2.1.7. Ianalumab - EMEA-002338-PIP03-21

Novartis Europharm Limited; Treatment of systemic lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The applicant addressed the remaining issues raised by the PDCO at Day 90.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion on a PIP for ianalumab for children from 5 years to less than 18 years of age, in the condition of treatment of active lupus nephritis was adopted. The Committee agreed on a waiver in a subset of children on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). The PDCO granted a deferral for the completion of this PIP.

2.1.8. Branaplam - EMEA-002204-PIP02-20

Novartis Europharm Limited; Treatment of Huntington's disease

Day 120 opinion

Neurology

Summary of Committee discussion:

In September 2022, based on the assessment of this application and the additional information provided by the applicant, the PDCO adopted a positive opinion for the PIP for the proposed medicine for paediatric patients from 2 years to less than 18 years of age, in the condition of treatment of Huntington's disease. The PDCO agreed on a waiver in a subset of children below 2 years of age on the grounds that the specific medicinal product on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

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

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

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 the proposed medicine for children from 2 years to less than 18 years of age, in the condition of treatment of myelin oligodendrocyte glycoprotein antibody-associated disease was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of lack of significant therapeutic benefit as clinical studies are not feasible. The PDCO granted a deferral for the completion of this PIP.

2.1.10. Odronextamab - EMEA-003149-PIP01-21

Regeneron Ireland DAC; Treatment of mature B cell malignancies

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed this application, taking into consideration the additional information received by the applicant.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for odronextamab for children from birth to less than 18 years of age, in the condition of treatment of mature B cell malignancies.
The PDCO granted a deferral for the completion of this PIP.

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

ROXALL Medizin GmbH; Diagnosis of IgE mediated allergy to tree pollen of the birch group

Day 120 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion on a PIP for freeze-dried allergen extract of *Betula pendula* pollen for children from 2 years to less than 18 years of age, in the condition of diagnosis of IgE-mediated allergy to tree pollen of the birch group was adopted.
The Committee agreed on a waiver in a subset of children on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2.1.12. Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512) - Orphan - EMEA-003157-PIP01-21

Pfizer Europe MA EEIG; Treatment of focal segmental glomerulosclerosis (FSGS)

Day 120 opinion

Uro-nephrology

Summary of Committee discussion:

The applicant addressed all outstanding issues satisfactorily in the draft opinion prior to Day 120. The PDCO agreed on a positive opinion for a PIP for PF-06730512 for the treatment of focal segmental glomerulosclerosis in children from 1 year of age. A waiver was agreed for the age group below 1 year on the grounds that the specific medicinal product is likely to be unsafe. The completion of the paediatric development plan was deferred.

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

Sanofi Pasteur; Prevention of yellow fever disease

Day 120 opinion

Vaccines

Summary of Committee discussion:

In September 2022, based on the assessment of this application and the additional information provided by the applicant, adopted a positive opinion for the PIP for the proposed medicine for paediatric patients from 6 months to less than 18 years of age, in the condition prevention of yellow fever disease. 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 over existing treatments. The PDCO granted a deferral for the completion of this PIP.

2.1.14. Telmisartan / rosuvastatin (calcium)- EMEA-003262-PIP01-22

Adamed Pharma S.A.; Prevention of cardiovascular events / 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 / rosuvastatin (calcium) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of hypertension and prevention of cardiovascular events 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. Fluorine (18F) PSMA-1007 - EMEA-003250-PIP01-22

BIOKOSMOS S.A.; Visualisation of prostate specific membrane antigen in prostate cancer

Day 60 opinion

Diagnostic

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 fluorine (18F) PSMA-1007 for all subsets of the paediatric population (0 to 18 years of age) in the condition of visualisation of prostate specific membrane antigen in prostate cancer based on the ground that the disease does not occur in children.

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. 3-(1,3-benzodioxol-5-yl)-5-(3-bromophenyl)-1H-pyrazole - EMEA-003252-PIP01-22

Teva B.V.; Treatment of multiple system atrophy

Day 60 opinion

Neurology

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 3-(1,3-benzodioxol-5-yl)-5-(3-bromophenyl)-1H-pyrazole for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of multiple system atrophy. 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.

2.1.17. Pridopidine (hydrochloride) - Orphan - EMEA-003174-PIP02-22

Prilenia Therapeutics B.V.; Treatment of amyotrophic lateral sclerosis (ALS)

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 assessment of this application and discussions at the Paediatric Committee the PDCO agreed with the applicant's request for a waiver. The PDCO recommended 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 of treatment of amyotrophic lateral sclerosis on the ground of lack of significant therapeutic benefit.

2.1.18. Derivative of (3S,3'S,3a'S,10a'S)-3'-phenyl-3',3a',10',10a'-tetrahydro-1'H-spiro[indoline-3,2'-pyrrolo[2',3':4,5]pyrrolo[1,2-b]indazol]-2-one - EMEA-003260-PIP01-22

Boehringer Ingelheim International GmbH; Treatment of liposarcoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the September 2022 plenary meeting, an application for a product specific waiver for a derivative of (3S,3'S,3a'S,10a'S)-3'-phenyl-3',3a',10',10a'-tetrahydro-1'H-spiro[indoline-3,2'-pyrrolo[2',3':4,5]pyrrolo[1,2-b]indazol]-2-one for the treatment of liposarcoma based on lack of safety and on the grounds that the disease or condition for which the specific medicinal product is intended to occur only in adult populations.
The PDCO confirmed all conclusions reached at Day 30 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 conditions of "treatment of liposarcoma" on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations.
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. EMEA-003197-PIP02-22

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

Day 60 opinion

Oncology

Note: Withdrawal request received on 9 September 2022

2.1.20. Trilaciclib (dihydrochloride) - EMEA-002534-PIP03-22

G1 Therapeutics, Inc.; Treatment of breast cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the September 2022 plenary meeting, an application for a product specific waiver for trilaciclib (dihydrochloride) for the treatment of breast malignant neoplasms 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 conditions of "treatment of breast cancer" on the grounds that the disease or condition for which the specific

medicinal product is intended occurs only in adult populations.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.21. Valemetostat tosilate - Orphan - EMEA-003256-PIP01-22

Daiichi Sankyo Europe GmbH; Treatment of mature T cell neoplasms

Day 60 opinion

Oncology

Note: Withdrawal request received on 9 September 2022

2.1.22. Batoclimab - EMEA-003162-PIP02-22

Immunovant Sciences, GmbH; Treatment of thyroid eye disease (TED)

Day 60 opinion

Ophthalmology

Summary of Committee discussion:

The PDCO confirmed the outcome of Day 30 discussion and 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 batoclimab for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition of treatment of thyroid eye disease on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2.1.23. Finasteride / tadalafil - EMEA-003261-PIP01-22

Adamed Pharma S.A.; Treatment of patients with benign prostatic hyperplasia

Day 60 opinion

Other

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 finasteride / tadalafil for all subsets of the paediatric population (birth to less than 18 years of age) in the condition of treatment of patients with benign prostatic hyperplasia. Since the agreed waiver ground is that the disease does not occur 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 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.

2.1.24. Lidocaine (hydrochloride monohydrate) - EMEA-003255-PIP01-22

Acumen Dental Limited; Local anaesthesia

Day 60 opinion

Pain

Summary of Committee discussion:

The PDCO discussed at Day 60, during the September 2022 plenary meeting, an application for a waiver for lidocaine (hydrochloride monohydrate) for all subsets of the paediatric population (from birth to 18 years of age) in the condition of prevention of pain of the oral mucosa, prior to injection of a local anaesthetic agent for dental procedures. The PDCO considered that the indication should be phrased as 'local anaesthesia'.
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 lidocaine (hydrochloride monohydrate) for all subsets of the paediatric population (from birth to 18 years of age) in the condition of local anaesthesia.
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.

2.1.25. Human papillomavirus type 58 L1 protein / human papillomavirus type 52 L1 protein / human papillomavirus type 45 L1 protein / human papillomavirus type 33 L1 protein / human papillomavirus type 31 L1 protein / human papillomavirus type 18 L1 protein / human papillomavirus type 16 L1 protein / human papillomavirus type 11 L1 protein / human papillomavirus type 6 L1 protein - EMEA-003209-PIP01-22

Prevention of infection by human papillomavirus (HPV)

Day 60 opinion

Vaccines / Infectious Diseases

Note: Withdrawal request received on 8 September 2022

2.2. Opinions on Compliance Check

2.2.1. Artesunate - EMEA-C-002710-PIP01-19

Amivas Ireland Limited; Treatment of malaria

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO adopted on 9 September 2022 an opinion confirming the compliance of Study 1 (development of an age-appropriate formulation with co-packaged diluent) and Study 2 (population pharmacokinetic modelling and simulation study for paediatric dose-finding) in the agreed paediatric investigation plan as set out in the latest Agency's decision (P/0328/2020) of 14 August 2020.

2.2.2. Lanadelumab - EMEA-C-001864-PIP01-15-M07

Takeda Pharmaceuticals International AG Ireland Branch; Prevention of hereditary angioedema attacks

Day 60 opinion

Other

Summary of Committee discussion:

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's decision (P/0214/2022) of 10 June 2022.

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

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

The PDCO adopted on 9 September 2022 an opinion confirming the compliance of Study 1 (V89_11) in the agreed paediatric investigation plan as set out in the latest Agency's decision (P/0141/2022) of 13 April 2022.

2.2.4. Recombinant influenza hemagglutinin-strain B (Victoria lineage) / recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (yamagata lineage) - EMEA-C3-002418-PIP01-18-M02

Sanofi Pasteur; Prevention of influenza infection

Day 60 letter

Vaccines

Summary of Committee discussion:

The PDCO discussed the completed Study 2 (LIO-04-16) and considered that this is compliant with the latest Agency's decision (P/0100/2022) of 18 March 2022.
The PDCO finalised this partially completed compliance procedure on 9 September 2022.

2.2.5. Nifurtimox - EMEA-C-003134-PIP01-21

Bayer AG; Treatment of Chagas disease

Day 30 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO adopted on 9 September 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/0081/2022) of 11 March 2022.

2.2.6. Lebrikizumab - EMEA-C1-002536-PIP01-18-M01

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 letter

Dermatology

Summary of Committee discussion:

The PDCO discussed the compliance check request. It was considered that the minor discrepancies do not affect the scientific validity of the study therefore Studies 1, 2, 3 and 6 are considered compliant and the procedure was concluded positively.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M09

Glaxo Group Limited; Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular 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 some 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/0370/2019 of 8 November 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Sotatercept - Orphan - EMEA-002756-PIP01-19-M01

Merck Sharp & Dohme B.V.; Treatment of pulmonary arterial hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, and the additional clarifications received after D30, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0227/2021 of 8 June 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Vericiguat - EMEA-001636-PIP01-14-M03

Bayer AG; Treatment of left ventricular failure

Day 60 opinion

Cardiovascular 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/0476/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Ritlecitinib - EMEA-002451-PIP01-18-M01

Pfizer Europe MA EEIG; Treatment of alopecia areata

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0147/2021 of 14 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. 3,6-diamino-2,5-bis{N-[(1R)-1-carboxy-2-hydroxyethyl]carbamoyl}pyrazine (MB-102) - EMEA-001983-PIP01-16-M01

MediBeacon Inc.; Monitoring of renal function

Day 60 opinion

Diagnostic / 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/0117/2017 of 5 May 2017).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Levonorgestrel - EMEA-002474-PIP02-18-M01

Chemo Research, S.L.; Contraception

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/0302/2019 of 10 September 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Etrasimod L-arginine - EMEA-002713-PIP01-19-M02

Arena Pharmaceuticals, Inc.; 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/0053/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Golimumab - EMEA-000265-PIP02-11-M04

Janssen Biologics 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/0157/2021 of 14 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Insulin human (NTRA-2112/ELGN-2112) - EMEA-002116-PIP01-17-M01

ELGAN Pharma Ltd; Treatment of intestinal malabsorption in preterm infants

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/0079/2018 of 16 March 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Maralixibat chloride - Orphan - EMEA-001475-PIP03-17-M03

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

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/0245/2021 of 9 July 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Tofacitinib - EMEA-000576-PIP03-12-M06

Pfizer Europe MA EEIG; 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/0009/2021 of 15 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Marstacimab - Orphan - EMEA-002285-PIP02-19-M02

Pfizer Europe MAA EEIG; Treatment of congenital haemophilia B / Treatment of congenital haemophilia A

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/0516/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Guselkumab - EMEA-001523-PIP03-18-M02

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:

Between Day 30 and Day 60 the applicant provided the requested information. 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/0065/2022 of 11 March 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Secukinumab - EMEA-000380-PIP06-19-M01

Novartis Europharm Limited; Treatment of systemic lupus erythematosus

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO confirmed the outcome of the Day 30 discussion and agreed with the proposed delay of the completion dates of the PIP studies. 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/0130/2022 of 13 April 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Cefiderocol - EMEA-002133-PIP01-17-M03

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

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 delays in timelines for completion of the Studies 3, 5 and 6 and for PIP completion, along with the proposed change on the length of infusion of Study 4, and the proposed change in Study 3 to consider pooling of data coming from a similar study conducted outside of the PIP, 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/0163/2022 of 13 May 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Gepotidacin - EMEA-002443-PIP01-18-M01

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urinary tract 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/0213/2020 of 16 June 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Gepotidacin - EMEA-002443-PIP02-18-M01

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urogenital gonorrhoea (GC)

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/0213/2020 of 16 June 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Maribavir - Orphan - EMEA-000353-PIP02-16-M02

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of cytomegalovirus (CMV) 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 including the clarification provided after D30, 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/0335/2020 of 31 August 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Taniborbactam / cefepime - EMEA-002576-PIP01-19-M01

Venatorx Pharmaceuticals, Inc.; 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 confirmed the views expressed at Day 30 and 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/0455/2020 of 4 December 2020).

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

opinion.

2.3.20. D-sorbitol / naltrexone HCl / (RS)-baclofen - Orphan - EMEA-002164-PIP01-17-M03

Pharnext SA; Treatment of Charcot-Marie-Tooth disease type 1A

Day 60 opinion

Neurology

Summary of Committee discussion:

In an oral explanation meeting the applicant provided information about the encountered issues to the PDCO and obtained general advice about the way forward. The Committee maintained that a full and solidly justified revision of the entire PIP is necessary, submitted in a future PIP modification procedure.
Based on the above, the PDCO adopted a negative opinion refusing the currently proposed changes.

2.3.21. Diroximel fumarate - EMEA-002685-PIP02-19-M01

Biogen Netherlands B.V; 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 presently a clear significant benefit of the product for the paediatric population could not be identified, concluding that the deletion of all the PIP measures and the granting of a product specific waiver could be accepted on the ground of lack of significant therapeutic benefit.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0381/2020 of 9 September 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Ocrelizumab - EMEA-000310-PIP03-10-M06

Roche Registration GmbH; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO re-discussed at Day 30, during the September 2022 plenary meeting, this request for modification for ocrelizumab for the treatment of multiple sclerosis.
The applicant proposed to delay completion of a clinical study and to modify two key elements of another clinical study.

Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that some of the proposed changes could be acceptable. Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0305/2021 of 13 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Rozanolixizumab - Orphan - EMEA-002681-PIP01-19-M01

UCB Pharma S.A.; Treatment of myasthenia gravis

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/0118/2022 of 13 April 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Enasidenib - Orphan - EMEA-001798-PIP02-16-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO discussed at Day 30, during the July 2022 plenary meeting, this request for modification for enasidenib for the treatment of acute myeloid leukaemia.
The applicant requests to delay completion of two clinical studies and two modelling and simulation studies.
The PDCO agreed to the changes and adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0293/2017 of 4 October 2017).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.25. Atropine sulphate - EMEA-002545-PIP01-19-M01

Fondazione Per La Ricerca Farmacologica Gianni Benzi Onlus; Treatment of myopia

Day 60 opinion

Ophthalmology

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.

2.3.26. Alpelisib - Orphan - EMEA-002016-PIP03-19-M02

Novartis Europharm Limited; Treatment of PIK3CA related overgrowth spectrum

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 and of the additional clarifications after D30, 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/0536/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.27. Atidarsagene autotemcel - Orphan - EMEA-001765-PIP02-15-M04

Orchard Therapeutics (Netherlands) B.V.; Treatment of metachromatic leukodystrophy

Day 60 opinion

Other

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the September 2022 plenary meeting, this request for modification for atidarsagene autotemcel for the treatment of metachromatic leukodystrophy.
The applicant requests to delay initiation and completion of a clinical study.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be acceptable. Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0212/2018 of 17 July 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Burosumab: Human recombinant IgG1 monoclonal antibody to fibroblast growth factor 23 (FGF23); KRN23 - Orphan - EMEA-001659-PIP01-15-M06

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

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/0491/2020 of 21 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.29. Eliglustat - Orphan - EMEA-000461-PIP02-11-M05

Genzyme Europe B.V.; Treatment of Gaucher disease type 1 and type 3 / Treatment of Gaucher disease type 2

Day 60 opinion

Other

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the September 2022 plenary meeting, this request for modification for eliglustat for the treatment of Gaucher disease type 1 and type 3.
The applicant requested changes in the pharmaceutical form to be developed and in one of the clinical studies.
Based on the review of the documentation submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be acceptable.
Therefore, the PDCO adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0385/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.30. Begelomab - Orphan - EMEA-001744-PIP01-14-M01

ADIENNE S.r.l SU; Treatment of acute graft versus host disease (aGvHD)

Day 60 opinion

Other / Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 31 August 2022

2.3.31. Bupivacaine - EMEA-000877-PIP03-17-M04

Pacira Ltd; Postsurgical analgesia

Day 60 opinion

Pain

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, 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/0342/2021 of 9 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.32. Finerenone - EMEA-001623-PIP01-14-M05

Bayer AG; Treatment of chronic kidney disease

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/0298/2021 of 11 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.33. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis* group B Protein 961c / Recombinant *Neisseria meningitis* group B Protein 287- 953 / meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis* group B Protein 936-741 / outer membrane vesicles (OMV) from *N. meningitidis* strain NZ 98/254 - EMEA-001260-PIP01-11-M02

GlaxoSmithKline Biologicals SA; Prevention of meningococcal meningitis

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 such substantial changes to the PIP should be in the future proposed prospectively to avoid retrospective inclusion and in order to allow for sufficient scientific discussion of the proposal. However, in this case the PDCO concluded 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/0231/2020 of 19 June 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.34. Monovalent, recombinant, replication-incompetent human adenovirus serotype 26-vectored vaccine encoding the pre-fusion conformation-stabilised F protein derived from the RSV A2 strain - EMEA-002172-PIP02-17-M02

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

Day 60 opinion

Vaccines / 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 including the addition of the waiver for the age subset from 1 year to less than year of age and the targeted population limited to children who are at increased risk of severe RSV disease 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/0197/2020 of 20 May 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.35. Epcoritamab - Orphan - EMEA-002907-PIP01-20-M02

AbbVie Ltd; Treatment of mature B cell lymphoma

Day 30 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/0257/2022 of 8 July 2022).
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. Leniolisib - EMEA-C2-002989-PIP01-21-M01

Pharming Technologies B.V.; Treatment of activated phosphoinositide 3-kinase delta syndrome (APDS)

Day 30 letter

Other

2.7.2. Danicopan - EMEA-C1-002310-PIP01-17

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 letter

Haematology-Hemostaseology

2.7.3. Ravulizumab - EMEA-C1-001943-PIP04-20

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorders

Day 30 letter

Neurology

2.7.4. Influenza virus (inactivated, split) Hemagglutinin-strain A (H3N2 subtype) / Influenza virus (inactivated, split) Hemagglutinin-strain B (Yamagata lineage) / Influenza virus (inactivated, split) Hemagglutinin-strain A (H1N1 subtype) / Influenza virus (inactivated, split) Hemagglutinin-strain B (Victoria lineage) - EMEA-C2-002359-PIP01-18-M04

Sanofi Pasteur; Prevention of influenza infection

Day 30 letter

Vaccines

2.7.5. Iptacopan - EMEA-C1-002705-PIP03-20

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 letter

Other / Haematology-Hemostaseology

2.7.6. Baricitinib - EMEA-C2-001220-PIP01-11-M06

Eli Lilly and Company; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 letter

Immunology-Rheumatology-Transplantation

2.7.7. Vamorolone - EMEA-C1-001794-PIP02-16-M05

ReveraGen BioPharma Ltd; Treatment of Duchenne muscular dystrophy

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. Cedirogant - EMEA-003142-PIP02-21

Treatment of psoriasis

Day 90 discussion

Dermatology

3.1.2. Ruxolitinib - EMEA-002618-PIP03-21

Treatment of atopic dermatitis

Day 90 discussion

Dermatology

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

Desitin Arzneimittel GmbH; Treatment of tuberous sclerosis

Day 90 discussion

Dermatology

3.1.4. Manganese - EMEA-003035-PIP02-21

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

Day 90 discussion

Diagnostic

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

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

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.6. Semaglutide - EMEA-001441-PIP07-21

Treatment of obesity

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. Batoclimab - EMEA-003162-PIP01-21

Treatment of myasthenia gravis

Day 90 discussion

Neurology

3.1.8. Exenatide acetate - Orphan - EMEA-003183-PIP02-22

Invex Therapeutics Ltd; Treatment of idiopathic intracranial hypertension

Day 90 discussion

Neurology

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

Treatment of Hodgkin lymphoma

Day 90 discussion

Oncology

3.1.10. Emactuzumab - Orphan - EMEA-003172-PIP01-21

Synox Therapeutics Limited; Treatment of tenosynovial giant cell tumour, local and diffuse type / Treatment of tenosynovial giant cell tumour (TGCT)

Day 90 discussion

Oncology

3.1.11. Efavaleukin alfa - EMEA-003156-PIP02-22

Treatment of ulcerative colitis

Day 90 discussion

Other

3.1.12. EMEA-003165-PIP01-21

Treatment of chronic kidney disease

Day 90 discussion

Uro-nephrology

3.1.13. 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 90 discussion

Vaccines

3.1.14. COVID-19 vaccine (recombinant, adjuvanted) - EMEA-003191-PIP01-22

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Vaccines / Infectious Diseases

3.1.15. Adapalene, micronised / benzoyl peroxide, hydrous / clindamycin - EMEA-003263-PIP01-22

Treatment of acne vulgaris

Day 60 discussion

Dermatology

3.1.16. Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 - Orphan - EMEA-003253-PIP01-22

Genzyme Europe B.V.; Treatment of achondroplasia

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.17. Crovalimab - EMEA-002709-PIP03-22

Treatment of Guillain-Barré syndrome (GBS)

Day 60 discussion

Haematology-Hemostaseology

3.1.18. Luspatercept - Orphan - EMEA-001521-PIP03-22

Bristol-Myers Squibb Pharma EEIG; Treatment of alpha-thalassaemia

Day 60 discussion

Haematology-Hemostaseology

3.1.19. Obinutuzumab - Orphan - EMEA-001207-PIP05-22

Roche Registration GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.20. Zilovertamab vedotin - Orphan - EMEA-003257-PIP01-22

Merck Sharp & Dohme (Europe) Inc.; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue)

Day 60 discussion

Oncology

3.1.21. Retatrutide - EMEA-003258-PIP01-22

Treatment of obesity

Day 60 discussion

Other

3.1.22. Aticaprant - EMEA-003251-PIP01-22

Treatment of major depressive disorder

Day 60 discussion

Psychiatry

3.1.23. Soluble guanylate cyclase (sGC) stimulator - EMEA-003266-PIP01-22

Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.24. Tigulixostat - EMEA-003272-PIP01-22

Treatment of hyperuricemia in primary gout

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. EMEA-002612-PIP02-22

Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.1.26. 2-[4-methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid - Orphan - EMEA-003282-PIP01-22

Horizon Therapeutics Ireland DAC; Treatment of systemic sclerosis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.27. Efgartigimod alfa - EMEA-002597-PIP08-22

Treatment of dermatomyositis / Treatment of polymyositis (including antisynthetase syndrome) / Treatment of immune-mediated necrotising myopathy

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.28. Namilumab - EMEA-003275-PIP01-22

Treatment of sarcoidosis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.29. Albaconazole - EMEA-003279-PIP01-22

Treatment of vulvovaginal candidiasis

Day 30 discussion

Infectious Diseases

3.1.30. Asunercept - Orphan - EMEA-003201-PIP01-22

Apogenix AG; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.1.31. Fosmanogepix - EMEA-003280-PIP01-22

Treatment of invasive fungal infections

Day 30 discussion

Infectious Diseases

3.1.32. Vilobelimab - EMEA-003080-PIP03-22

Treatment of severe coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.1.33. Nipocalimab - EMEA-002559-PIP06-22

Treatment of chronic inflammatory demyelinating polyradiculoneuropathy

Day 30 discussion

Neurology

3.1.34. Ocrelizumab - EMEA-000310-PIP05-22

Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.1.35. EMEA-003271-PIP01-22

Treatment of focal onset seizures

Day 30 discussion

Neurology

3.1.36. EMEA-003278-PIP01-22

Treatment of small cell lung cancer / Treatment of non-small cell lung cancer (NSCLC)

Day 30 discussion

Oncology

3.1.37. Adult differentiated autologous T cells from peripheral blood, expanded and transduced with a lentivirus to express a chimeric antigen receptor with anti-CD19 specificity (A3B1) conjugated with the co-stimulatory regions 4-1BB and CD3z - EMEA-003264-PIP01-22

Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Oncology

3.1.38. EMEA-003274-PIP01-22

Treatment of melanoma

Day 30 discussion

Oncology

3.1.39. Cobolimab - EMEA-003273-PIP01-22

Treatment of non-small cell lung cancer

Day 30 discussion

Oncology

3.1.40. Lacutamab - Orphan - EMEA-003281-PIP01-22

Innate Pharma SA; Treatment of cutaneous T cell lymphoma

Day 30 discussion

Oncology

3.1.41. Pembrolizumab / favezelimab - EMEA-003104-PIP02-22

Treatment of Hodgkin lymphoma

Day 30 discussion

Oncology

3.1.42. Pembrolizumab / vibostolimab - EMEA-003063-PIP02-22

Treatment of Hodgkin lymphoma

Day 30 discussion

Oncology

3.1.43. Pemigatinib - Orphan - EMEA-002370-PIP03-22

Incyte Biosciences Distribution B.V.; Treatment of myeloid/lymphoid neoplasms

Day 30 discussion

Oncology

3.1.44. Vusolimogene oderparepvec - EMEA-003265-PIP01-22

Treatment of cutaneous squamous cell carcinoma

Day 30 discussion

Oncology

3.1.45. EMEA-003269-PIP01-22

Treatment of mastocytosis

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.1.46. 1-(4-(6-chloropyridazin-3-yl)piperazin-1-yl)-2-(4-cyclopropyl-3-fluorophenyl)ethan-1-one - Orphan - EMEA-003268-PIP01-22

Bridge Bio Europe B.V.; Treatment of pantothenate kinase-associated neurodegeneration

Day 30 discussion

Other

3.1.47. EMEA-002612-PIP03-22

Prevention of cardiopulmonary bypass (CPB)-induced postoperative pulmonary dysfunction

Day 30 discussion

Pneumology - Allergology

3.1.48. Immunoglobulin G4 [228-proline], anti-(*Betula alleghaniensis* allergen Bet v 1) (human monoclonal γ4-chain), disulphide with human monoclonal κ-chain, dimer / immunoglobulin G4 [227-proline], anti-(*Betula alleghaniensis* allergen Bet v 1) (human monoclonal γ4-chain), disulphide with human monoclonal κ-chain, dimer / immunoglobulin G4 [224-proline], anti-(*Betula alleghaniensis* allergen Bet v 1) (human monoclonal γ4-chain), disulphide with human monoclonal κ-chain, dimer - EMEA-003270-PIP01-22

Treatment of allergic rhinitis with or without conjunctivitis in birch tree pollen allergic patients

Day 30 discussion

Pneumology - Allergology

3.1.49. EMEA-003276-PIP01-22

Treatment of post-traumatic stress disorder

Day 30 discussion

Psychiatry

3.1.50. Live attenuated respiratory syncytial virus (RSV) - EMEA-003277-PIP01-22

Prevention of respiratory syncytial virus (RSV) diseases

Day 30 discussion

Vaccines

3.1.51. MVA-BN-RSV - EMEA-003185-PIP01-22

Treatment of lower respiratory tract disease (LRTD) caused by RSV

Day 30 discussion

Vaccines / Infectious Diseases

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. Dopamine hydrochloride - EMEA-C-001105-PIP01-10-M06

BrePco Biopharma Limited; Treatment of vascular hypotensive disorders

Day 30 discussion

Neonatology - Paediatric Intensive Care

3.2.2. Brivaracetam - EMEA-C-000332-PIP01-08-M16

UCB Pharma S.A.; Treatment of epilepsy with partial onset seizures

Day 30 discussion

Neurology

3.2.3. Eladocagene exuparvovec - EMEA-C-002435-PIP01-18-M02

PTC Therapeutics International Limited; Treatment of aromatic L-amino acid decarboxylase deficiency

Day 30 discussion

Neurology

3.2.4. Vosoritide - EMEA-C4-002033-PIP01-16-M02

BioMarin International Limited; Treatment of achondroplasia

Day 30 discussion

Other

3.2.5. Agomelatine - EMEA-C-001181-PIP01-11-M06

Les Laboratoires Servier; Treatment of major depressive episodes

Day 30 discussion

Psychiatry

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Birch bark extract - EMEA-001299-PIP01-12-M01

Amryt AG; Treatment of skin injuries

Day 30 discussion

Dermatology

3.3.2. Brodalumab - EMEA-001089-PIP02-13-M03

LEO Pharma A/S; Treatment of psoriasis

Day 30 discussion

Dermatology

3.3.3. Glycopyrronium bromide - EMEA-002383-PIP01-18-M02

Dr. August Wolff GmbH & Co. KG - Arzneimittel; Treatment of hyperhidrosis

Day 30 discussion

Dermatology

3.3.4. Gadoquatrane - EMEA-002778-PIP01-20-M01

Bayer AG; Diagnosis by evaluation of any known or suspected clinical condition with contrast enhanced magnetic resonance imaging

Day 30 discussion

Diagnostic

3.3.5. Inclisiran sodium - EMEA-002214-PIP01-17-M01

Novartis Europharm Ltd.; Treatment of elevated cholesterol

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Saxagliptin - EMEA-000200-PIP01-08-M10

AstraZeneca AB; Treatment of type 2 diabetes

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Vedolizumab - EMEA-000645-PIP04-20-M01

Takeda Pharma A/S; Treatment of pouchitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.8. Recombinant human A disintegrin and metalloprotease with thrombospondin type-1 motifs 13 (rADAMTS13) - Orphan - EMEA-001160-PIP01-11-M03

Baxalta Innovations GmbH; Treatment of thrombotic thrombocytopenic purpura

Day 30 discussion

Haematology-Hemostaseology

3.3.9. Voxelotor - Orphan - EMEA-002356-PIP02-20-M01

Global Blood Therapeutics Netherlands B. V.; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.3.10. Risankizumab - EMEA-001776-PIP01-15-M01

AbbVie Ltd; Treatment of psoriasis

Day 30 discussion

Immunology-Rheumatology-Transplantation / Dermatology / Gastroenterology-Hepatology

3.3.11. Bedaquiline (fumarate) - Orphan - EMEA-000912-PIP01-10-M06

Janssen-Cilag International NV; Treatment of multi-drug resistant tuberculosis

Day 30 discussion

Infectious Diseases

3.3.12. Dolutegravir (DGT) / rilpivirine (RPV) - EMEA-001750-PIP01-15-M06

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.3.13. Sulbactam / durlobactam - EMEA-002807-PIP01-20-M01

Entasis Therapeutics Inc.; Treatment of infections due to organisms of the *Acinetobacter baumannii-calcoaceticus* complex

Day 30 discussion

Infectious Diseases

3.3.14. Ataluren - Orphan - EMEA-000115-PIP01-07-M12

PTC Therapeutics International, Limited; Treatment of dystrophinopathy

Day 30 discussion

Neurology

3.3.15. Binimetinib - EMEA-001454-PIP03-15-M02

Pierre Fabre Médicament; Treatment of melanoma

Day 30 discussion

Oncology

3.3.16. Encorafenib - EMEA-001588-PIP01-13-M02

Pierre Fabre Médicament; Treatment of melanoma

Day 30 discussion

Oncology

3.3.17. Isatuximab - EMEA-002205-PIP01-17-M03

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

Day 30 discussion

Oncology

3.3.18. Bilastine - EMEA-000347-PIP02-16-M04

Faes Farma S.A.; Treatment of allergic conjunctivitis

Day 30 discussion

Ophthalmology

3.3.19. Sodium chloride solution 4.2% (w/v) / idrevloride, 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide - Orphan - EMEA-002935-PIP01-20-M02

Parion Sciences, Inc.; Treatment of primary ciliary dyskinesia

Day 30 discussion

Pneumology - Allergology

3.3.20. Lanthanum carbonate hydrate - EMEA-000637-PIP02-10-M07

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of hyperphosphataemia

Day 30 discussion

Uro-nephrology

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 12 September 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:

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

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

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 thanked Catherine Cornu representing Healthcare professionals nominated by the EC as an Alternate Member.

9.1.2. Vote by Proxy

No item

9.1.3. Election of PDCO Vice-Chairperson

Summary of Committee discussion:

The mandate of the PDCO Vice-Chair, Sabine Scherer, will expire on 15 October 2022.

The election of the new Vice-Chair took place in accordance with the PDCO rules of procedure.

The nominations received were presented to the Committee.

The PDCO elected Sylvie Benchetrit as PDCO Vice-Chair for a three-year mandate starting on 16 October 2022.

The PDCO and the Agency congratulated Sylvie Benchetrit on her election and wished her all the best in her new role as Vice-Chair of the Committee.

9.1.4. Strategic Review and Learning Meeting (SRLM) – Prague, 6 – 7 October 2022

PDCO member: Tomáš Boráň

Summary of Committee discussion:

PDCO members were invited to the next strategic review and learning meeting in Prague and the draft agenda was presented.

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 Party: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

The Vice-Chair of the Non-clinical Working Party (NcWP) identified the products which will require NcWP 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. EMA Emergency task force (ETF) – PDCO nominations

PDCO member: Koenraad Norga

Summary of Committee discussion:

The Chair provided the Committee an update on the call for nominations for the EMA ETF following the departure of Eva Agurell.

9.4. Cooperation within the EU regulatory network

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

Summary of Committee discussion:

The PDCO was informed about the annual meeting of Enpr-EMA, which will take place as a virtual event on 4 October 2022. An outline of the agenda was presented and PDCO members were invited to attend.

9.4.2. PDCO / Health technology assessment (HTA) interaction – update on current initiatives

Summary of Committee discussion:

The PDCO was informed about ongoing initiatives discussing access considerations with a focus on paediatrics in context of HTA decision making (particularly via ACCELERATE and the paediatric MRCT project).

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

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

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q3/2022 Update of the Business Pipeline report for the human scientific committees

Summary of Committee discussion:

The Q3-2022 forecast report was presented to the PDCO for information. The report listed and presented the initial MAAs expected for the remainder of the year, highlighting the resources allocated to the expected products.

To note, the Q4-2022 forecast report, covering MAAs expected in 2023, is due at the beginning of December 2022.

10. Any other business

10.1. COVID-19 update

Summary of Committee discussion:

The PDCO was updated on COVID therapeutics and vaccines of paediatric relevance.

10.2. PIP-related CHMP procedures

PDCO member: Sabine Scherer

Summary of Committee discussion:

The PDCO discussed a proposal to further intensify the involvement in the PIP-related CHMP procedures and agreed to start a pilot.

10.3. Webinar series on 'Improving academia-industry collaborative trials' organised by Accelerate

Summary of Committee discussion:

The Committee was informed about an upcoming webinar series on the topic of academia industry collaboration and how to ensure academic clinical trials are designed and conducted so that the data can be used for marketing authorisation applications. This follows a recent publication on the critical role of academic clinical trials in paediatric cancer drug approvals, initiated by Accelerate and co-authored by EMA and FDA.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The PDCO was informed about international initiatives addressing non-clinical studies relevant to paediatric oncology.

11.2. Neonatology

Summary of Committee discussion:

The PDCO discussed guideline development as well topics of the upcoming International Neonatal Consortium (INC) annual workshop.

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 06-09 September 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	
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	
Tomas Boran	Member	Czechia	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Louisa Braun Exner	Alternate	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 alternate)	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	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
Zacharkiene				
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
Olivier Moes	Alternate	Luxembourg	No interests declared	
John-Joseph Borg	Member	Malta	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No restrictions applicable to this meeting	
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 participation in discussion, final deliberations and voting on:	3.1.3. Sirolimus - Orphan - EMEA-003168-PIP01-21
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Kristin Karlsson	Member	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
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	No restrictions applicable to this	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
		Representative	meeting	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Tomasz Grybek	Alternate	Patients' Organisation Representative	No interests declared	
María Estela Moreno Martín	Expert - via telephone*	Spain	No interests declared	
Celine Chu	Expert - via telephone*	France	No interests declared	
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/