25 February 2022
EMA/PDCO/54982/2022
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 18-21 January 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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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 18-21 January 2022 meeting was adopted with amendments.

1.3. Adoption of the minutes

The minutes for 14-17 December 2021 meeting were adopted and will be published on the EMA website.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Phospholipid esters from herring roe (HRO350) - EMEA-003053-PIP01-21

Arctic Bioscience (previously Arctic Nutrition AS); Treatment of psoriasis

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

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

argenx BV; Treatment of immune thrombocytopenia

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO adopted a positive opinion for the PIP for efgartigimod alfa with a waiver for the paediatric population from birth to less than 2 years in treatment of immune thrombocytopenia. The PIP includes a clinical two-part pharmacokinetic and pharmacodynamic study of efgartigimod in paediatric patients from 2 years to less than 18 years of age with idiopathic thrombocytopenic purpura (ITP) and an extrapolation study.

2.1.3. Recombinant humanized anti-blood dendritic cell antigen 2 (BDCA2) monoclonal antibody (BIIB059) - EMEA-002555-PIP02-21

Biogen Netherlands B.V.; Treatment of lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

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 5 years of age with lupus erythematosus was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of 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.

2.1.4. Acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) - Orphan - EMEA-002796-PIP01-20

IntraBio Ltd.; Treatment of Niemann-Pick disease type C

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 with a deferral for acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) for treatment of Niemann-Pick disease type C. The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.

The PDCO discussed the potential development of the product in Niemann Pick disease type A and B and acknowledged the readiness of the applicant to conduct appropriate proof of concept (POC) studies in these subtypes of the disease. Moreover, it was considered that should these POC studies show promise, the applicant should be encouraged to come for a separate PIP to specify a separate development plan in this indication.

PDCO granted a positive opinion on the paediatric plan proposed by the applicant.

2.1.5. Firazorexton sesquihydrate - EMEA-002993-PIP01-21

Treatment of narcolepsy

Day 120 opinion

Neurology

Note: Withdrawal request received on 19 January 2022

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

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

Day 120 opinion

Neurology

Summary of Committee discussion:

The PDCO adopted a positive opinion for givinostat in treatment of Duchenne muscular dystrophy with a waiver for the paediatric population from birth to less than 2 years of age and a deferral. The PIP consists of a quality and a non-clinical study, five clinical studies (of which two RCTs in ambulant and non-ambulant patients) and five modelling and simulation analyses.

2.1.7. Viltolarsen - Orphan - EMEA-002853-PIP01-20

NS Pharma, Inc.; Treatment of Duchenne muscular dystrophy

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 and adolescents from 6 months to less than 18 years of age, in the condition treatment of Duchenne muscular dystrophy 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.

2.1.8. Pemigatinib - Orphan - EMEA-002370-PIP02-21

Incyte Biosciences Distribution B.V.; Treatment of myeloid/lymphoid neoplasms with FGFR1 rearrangement

Day 120 opinion

Oncology

Note: Withdrawal request received on 13 January 2022

2.1.9. Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21

Miltenyi Biomedicine GmbH; Treatment of mature B cell neoplasms

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 120, during the January 2022 plenary meeting, an application for a paediatric investigation plan with a deferral and a waiver for zamtocabtagene autoleucel for the treatment of mature B cell neoplasms.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.
The PDCO adopted a positive opinion on a paediatric investigation plan with a deferral and a waiver for children weighing less than 6 kg on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible in the treatment of mature B cell neoplasms.

2.1.10. Pabinafusp alfa - Orphan - EMEA-003033-PIP01-21

JCR Pharmaceuticals Co., Ltd.; Mucopolysaccharidosis type II

Day 120 opinion

Other

Note: Withdrawal request received on 13 January 2022

2.1.11. Deutivacaftor / tezacaftor / (14S)-8-[3-(2-{dispiro[2.0.2^(4).1^(3)]heptan-7-yl}ethoxy)-1H-pyrazol-1-yl]-12,12-dimethyl-2lambda^(6)-thia-3,9,11,18,23-pentaazatetracyclo[17.3.1.1^(11,14).0^(5,10)]tetracosa-1(22),5,7,9,19(23),20-hexaene-2,2,4-trione calcium salt hydrate - EMEA-003052-PIP01-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 120 opinion

Pneumology - Allergology

Summary of Committee discussion:

The PDCO considered that the responses to the remaining questions after Day 90 were satisfactory and that a positive opinion could be adopted.
Some members still expressed doubts on the significant therapeutic benefit of this product but the majority of the Committee was of the opinion that the single daily administration versus twice a day for Kaftrio could have a beneficial impact on the burden of care in cystic fibrosis (CF) paediatric patients.
Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO recommended granting a positive opinion to the Paediatric Investigation Plan for tezacaftor / deutivacaftor / (14S)-8-[3-(2-{dispiro[2.0.2^(4).1^(3)]heptan-7-yl}ethoxy)-1H-pyrazol-1-yl]-12,12-dimethyl-2lambda^(6)-thia-3,9,11,18,23-penta-azatetracyclo[17.3.1.1^(11,14).0^(5,10)]tetracosa-1(22),5,7,9,19(23),20-hexaene-2,2,4-trione calcium salt hydrate for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of cystic fibrosis. The PIP contains a waiver in children below 1 year of age based on likely lack of safety; two quality studies aiming at the development of age-appropriate formulations/presentations; one non-clinical juvenile study; and four clinical studies covering ages from 1 year to less than 18 years old.

2.1.12. Pyrrol-Hydroxyethylpyridin-3-ol derivative (MIJ821) - EMEA-002946-PIP01-20

Novartis Europharm Limited; Treatment of major depressive disorder

Day 120 opinion

Psychiatry

Summary of Committee discussion:

Based on the assessment of this application, the PDCO agreed a PIP for MIJ821 for the treatment of major depressive disorder (MDD) with a deferral and a waiver for the paediatric population from birth to less than 7 years of age 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), and from 7 years to less than 12 years of age on the grounds that the specific medicinal product is likely to be unsafe.

2.1.13. Ralmitaront - EMEA-003003-PIP01-21

Roche Registration GmbH; Treatment of schizophrenia

Day 120 opinion

Psychiatry

Summary of Committee discussion:

During its plenary on 21 January 2022, the PDCO discussed the outcome of D90 clarifications received from the applicant. Overall, the responses were found acceptable. The duration of the washout period was specified in the key binding elements. An interim analysis was introduced in Study 3 to allow for potential dose adjustment (to match the exposure with the one achieved in adults).
The PDCO granted a positive opinion on the paediatric plan proposed by the applicant.

Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agrees with the applicant's request for a waiver. The PDCO recommended granting a waiver for ralmitaront for the paediatric population (0 to 12 years of age) in the condition of treatment of schizophrenia.

2.1.14. 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 120 opinion

Uro-nephrology

Summary of Committee discussion:

Based on the assessment of this application a positive opinion on the PIP for the proposed medicine for children from birth to less than 18 years of age, in the condition of treatment of renal failure with carnitine deficiency. The PDCO granted a deferral for the completion of this PIP.

2.1.15. CpG 1018/Alum-adjuvanted recombinant SARS-CoV-2 Trimeric Spike (S) protein subunit vaccine (SCB-2019) - EMEA-002987-PIP01-21

Clover Biopharmaceuticals Ireland Limited; Prevention of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Vaccines

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO agreed on a PIP for CpG 1018/Alum-adjuvanted recombinant SARS-CoV-2 Trimeric Spike protein subunit vaccine (SCB-2019) covering all subsets of the paediatric population (from birth to less than 18 years of age) in the condition prevention of COVID-19 disease. The PIP contains 3 clinical studies, including one study in immunocompromised children.

2.1.16. *Neisseria meningitidis* serogroup B fHbp subfamily B / *Neisseria meningitidis* serogroup B fHbp subfamily A / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-002814-PIP02-21

Pfizer Europe MA EEIG; Invasive disease caused by *Neisseria meningitidis* group A, B, C, W and Y from 2 months of age

Day 120 opinion

Vaccines

Summary of Committee discussion:

The PDCO confirmed that a positive opinion could be granted to this application. The opinion was adopted on the 19th of January 2022. The PIP contains a partial waiver below 2 months of age based on lack of safety, and five clinical studies covering as a whole all age groups from 2 months to less than 18 years of age.

2.1.17. 2-{(3S)-7-fluoro-4-[(3-oxo-3,4-dihydro-2H-1,4-benzoxazin-6-yl)carbonyl]-3,4-dihydro-2H-1,4-benzoxazin-3-yl}-N-methylacetamide (AZD9977) / Dapagliflozin - EMEA-003120-PIP01-21

AstraZeneca AB; Prevention of cardiovascular events in patients with chronic heart failure

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 Dapagliflozin / 2-{(3S)-7-fluoro-4-[(3-oxo-3,4-dihydro-2H-1,4-benzoxazin-6-yl)carbonyl]-3,4-dihydro-2H-1,4-benzoxazin-3-yl}-N-methylacetamide (AZD9977) for all subsets of the paediatric population (0 to 18 years of age) in the condition of prevention of cardiovascular events in patients with chronic heart failure, on the grounds that the specific medicinal product is likely to be unsafe in all paediatric age subsets.
The PDCO recommended to grant the waiver for all pharmaceutical forms and all routes of administration to which the applicant agreed.

2.1.18. Avexitide - Orphan - EMEA-003125-PIP01-21

EigerBio Europe Limited; Treatment of postbariatric hypoglycaemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

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 product-specific waiver for avexitide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of postbariatric hypoglycaemia on the grounds that this condition occurs only in adults.
The PDCO welcomes the recent submission of PIP application for avexitide in congenital hyperinsulinism and encourages the applicant to consider a PIP for hypoglycaemia secondary to gastrointestinal surgery (unrelated to bariatric surgery).

2.1.19. Parsaclisib (as hydrochloride) - Orphan - EMEA-002696-PIP02-21

Incyte Biosciences Distribution B.V.; Treatment of autoimmune haemolytic anaemia

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed this application in line with the D30 conclusions and clarifications received from the applicant regarding the safety profile of the product.
Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a product specific waiver. The PDCO recommended granting a product specific waiver for parsaclisib (as hydrochloride) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of autoimmune haemolytic anaemia based on the ground of lack of significant therapeutic benefit.

2.1.20. Human normal immunoglobulin - EMEA-003121-PIP01-21

Instituto Grifols, S.A.; Treatment of post-polio syndrome

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 human normal immunoglobulin for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of post-polio syndrome, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in all paediatric age subsets.
The PDCO recommended to grant the waiver for all pharmaceutical forms and all routes of administration to which the applicant agreed.
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.

2.1.21. Ceralasertib - EMEA-003127-PIP01-21

AstraZeneca AB; Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the January 2022 plenary meeting, an application for a product specific waiver for ceralasertib, a selective inhibitor of the serine/threonine kinase ATR, with selectivity also against other family members of phosphatidylinositol 3-kinase-related kinases for the treatment of lung carcinoma (small cell and non-small cell carcinoma) 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 lung carcinoma (small cell and non-small cell carcinoma)" on the grounds that the 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.

2.1.22. Cosibelimab - EMEA-003041-PIP01-21

Checkpoint Therapeutics, Inc.; Treatment of cutaneous squamous cell carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for cosibelimab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of cutaneous squamous cell carcinoma.

2.1.23. Nemtabrutinib - EMEA-003135-PIP01-21

Merck Sharp & Dohme (Europe), Inc.; Treatment of mature B cell malignancies

Day 60 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the 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 'treatment of mature B cell malignancies' based on the grounds that the specific medicinal product is likely to be ineffective.

2.1.24. Sunvozertinib - EMEA-003132-PIP01-21

Dizal (Jiangsu) Pharmaceutical Co., Ltd; Treatment of non-small cell lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

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 'treatment of non-small cell lung cancer'.

2.1.25. Tarlatamab - EMEA-003138-PIP01-21

Amgen Europe BV; Treatment of prostate malignant neoplasms / Treatment of small cell lung cancer (SCLC)

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO discussed at Day 60, during the January 2022 plenary meeting, an application for a product specific waiver for tarlatamab, a bi-specific T cell engager targeting the proteins delta-like ligand 3 (DLL3) and cluster of differentiation 3 (CD3), for the treatment of small cell lung cancer (SCLC) and for the treatment of neuroendocrine prostate cancer (NEPC) 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 small cell lung cancer" and "treatment of prostate malignant neoplasms" on the grounds that these diseases do not occur in the paediatric population.
The applicant agreed for the waiver to be extended to all pharmaceutical forms and all routes of administration.

2.1.26. Tucatinib - EMEA-002242-PIP02-21

Seagen B.V.; Treatment of all solid tumours

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 tucatinib for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of 'treatment of all solid tumours' based on the ground of lack of significant therapeutic benefit.
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.

2.1.27. Vimseltinib - Orphan - EMEA-002802-PIP02-21

Deciphera Pharmaceuticals; Treatment of tenosynovial giant cell tumour

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 vimseltinib for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of tenosynovial giant cell tumour, based on the grounds of lack of safety for pre-pubertal patients and lack of significant therapeutic benefit for patients of post pubertal age to less than 18 years age.
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.

2.1.28. Bemcentinib - EMEA-003123-PIP01-21

Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology / Haematology-Hemostaseology

Note: Withdrawal request received on 14 January 2022

2.1.29. (1S,3S)-3-({2-methyl-6-[1-methyl-5-({[methyl(propyl)carbamoyl]oxy}methyl)-1H-1,2,3-triazol-4-yl]pyridin-3-yl}oxy)cyclohexane-1-carboxylic acid (BMS-986278) - EMEA-001649-PIP02-21

Bristol-Myers Squibb Pharma EEIG; Treatment of fibrosing interstitial lung diseases (ILD)

Day 60 opinion

Pneumology - Allergology

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 (1S,3S)-3-({2-methyl-6-[1-methyl-5-({[methyl(propyl)carbamoyl]oxy}methyl)-1H-1,2,3-triazol-4-yl]pyridin-3-yl}oxy)cyclohexane-1-carboxylic acid (BMS-986278) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of fibrosing interstitial lung diseases (ILD).
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.

2.1.30. Ocrelizumab - EMEA-000310-PIP04-21

Roche Registration GmbH; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Note: Withdrawal request received on 20 January 2022

2.2. Opinions on Compliance Check

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

2.2.1. Nirsevimab - EMEA-C2-001784-PIP01-15-M03

AstraZeneca AB; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 60 letter

Infectious Diseases

Summary of Committee discussion:

Taking the additional information received after Day 30 as well as the assessors' comments into account, the PDCO considered that the checked studies are compliant with the latest Agency's Decision (P/0296/2021) of 11 August 2021.

2.2.2. Nivolumab - EMEA-C-001407-PIP01-12-M03

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

Day 60 opinion

Oncology

Summary of Committee discussion:

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

- EMEA-C1-001407-PIP01-12
- EMEA-C2-001407-PIP01-12

The PDCO adopted on 21 January 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/0432/2020) of 5 November 2020.

2.2.1. Albutrepenonacog alfa - EMEA-C-001107-PIP01-10-M04

CSL Behring GmbH; Treatment of hereditary factor IX deficiency

Day 30 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO discussed the compliance check request.
The interpretation of the key binding element regarding the number of patients enrolled in the Study 6 was discussed. It was considered that despite unprecise wording confounding

assessment, this key element can be considered compliant.
Taken together, all key elements that remained at this final compliance check were fulfilled.
Therefore, the Study 6 of this PIP was considered compliant.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Delgocitinib - EMEA-002329-PIP02-20-M01

LEO Pharma A/S; Treatment of chronic hand eczema

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 adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0108/2021 of 17 March 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Spesolimab - EMEA-002475-PIP02-19-M02

Boehringer Ingelheim International GmbH; Prevention of generalized pustular psoriasis / Treatment of generalised pustular psoriasis

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/0178/2021 of 19 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Tralokinumab - EMEA-001900-PIP02-17-M06

LEO Pharma A/S; Treatment of atopic dermatitis

Day 60 opinion

Dermatology

Summary of Committee discussion:

The applicant addressed the issues raised by the PDCO at Day 30.
The delay of Study 2 completion has been justified.
The PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the

Agency's latest decision (P/0292/2021 of 11 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. Dienogest / ethinyl estradiol - EMEA-002229-PIP01-17-M03

Chemo Research; Prevention of pregnancy

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/0169/2021 of 14 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Pegvaliase - Orphan - EMEA-001951-PIP01-16-M02

BioMarin International Limited; Treatment of hyperphenylalaninaemia

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/0036/2017 of 31 January 2017).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Etrasimod L-arginine - EMEA-002713-PIP01-19-M01

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/0487/2020 of 22 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Guselkumab - EMEA-001523-PIP05-19-M01

Janssen-Cilag International N.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/0293/2020 of 12 August 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Mirikizumab - EMEA-002208-PIP01-17-M02

Eli Lilly and Company; Treatment of Crohn's disease / 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/0130/2020 of 15 April 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.9. Efanesoctocog alfa - Orphan - EMEA-002501-PIP01-18-M02

Bioverativ Therapeutics, Inc., a Sanofi Company; Treatment of 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/0238/2020 of 16 June 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Glutamine - Orphan - EMEA-001996-PIP02-16-M01

Emmaus Medical Europe Ltd.; Treatment of sickle cell disease

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/0003/2018 of 4 January 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Apremilast - EMEA-000715-PIP02-11-M05

Amgen Europe B.V.; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 20 January 2022

2.3.12. Imlifidase - Orphan - EMEA-002183-PIP01-17-M01

Hansa Biopharma AB; Prevention of graft rejection following solid organ transplantation

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0229/2018 of 30 July 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Casirivimab - EMEA-002964-PIP01-21-M01

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0347/2021 of 16 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Cilgavimab (AZD1061) - EMEA-002925-PIP01-20-M01

AstraZeneca AB; Prevention or treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

In the responses between Day 30 and Day 60 and in additional correspondence the applicant clarified the remaining issues for this modification.
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 PIP opinion was amended with the removal of the post-exposure prophylaxis indication (and respective cohort in the paediatric study), and with the addition of an indication for the treatment of severe COVID-19 as well as of the intravenous route of administration.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0236/2021 of 8 June 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Fostemsavir (tromethamine) - EMEA-001687-PIP01-14-M06

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0430/2021 of 29 October 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Imdevimab - EMEA-002965-PIP01-21-M01

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0348/2021 of 16 August 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Ridinilazole (hydrate) - EMEA-002250-PIP02-17-M01

Summit Limited; Treatment of *Clostridioides difficile* infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Between Day 30 and Day 60 the applicant had addressed all outstanding issues satisfactorily.
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/0109/2019 of 22 March 2019).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Tedizolid phosphate - EMEA-001379-PIP01-12-M06

Merck Sharp & Dohme (Europe), Inc.; Treatment of acute bacterial skin and skin structure 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/0462/2020 of 1 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. Tenofovir disoproxil - EMEA-000533-PIP01-08-M11

Gilead Sciences International Limited; Treatment of chronic viral hepatitis B / Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0156/2021 of 14 April 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Tixagevimab (AZD8895) - EMEA-002900-PIP01-20-M01

AstraZeneca AB; Prevention or treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

In the responses between Day 30 and Day 60 and in additional correspondence the applicant clarified the remaining issues for this modification.
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 PIP opinion was amended with the removal of the post-exposure prophylaxis indication (and respective cohort in the paediatric study), and with the addition of an indication for the treatment of severe COVID-19 as well as of the intravenous route of administration.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0236/2021 of 8 June 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.21. Isoflurane - EMEA-002320-PIP01-17-M02

Sedana Medical AB; Sedation of mechanically ventilated patients

Day 60 opinion

Neonatology - Paediatric Intensive Care

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0190/2020 of 15 May 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Delandistrogene moxeparvovec - Orphan - EMEA-002677-PIP01-19-M01

Roche Registration GmbH; Treatment of Duchenne muscular dystrophy

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0419/2020 of 22 October 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Eculizumab - Orphan - EMEA-000876-PIP05-15-M05

Alexion Europe SAS; 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/0388/2020 of 29 September 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Eptinezumab - EMEA-002243-PIP01-17-M02

H. Lundbeck A/S; Prevention of migraine headaches

Day 60 opinion

Neurology

Summary of Committee discussion:

The PDCO, in conclusion, adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0123/2021 of 17 March 2021).

2.3.25. Dinutuximab beta - Orphan - EMEA-001314-PIP01-12-M01

EUSA Pharma (Netherlands) BV; Treatment of neuroblastoma

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/0094/2014 of 7 April 2014).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.26. Ex vivo expanded autologous human corneal epithelium cells containing stem cells - Orphan - EMEA-001082-PIP02-11-M03

Holostem Terapie Avanzate S.r.l.; Treatment of limbal stem cell deficiency due to ocular burns

Day 60 opinion

Ophthalmology

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/0370/2018 of 7 December 2018).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.27. Vosoritide - Orphan - EMEA-002033-PIP01-16-M02

BioMarin International Limited; Treatment of achondroplasia

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, including the new information received after Day 30, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0060/2020 of 10 February 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.28. Adrenaline (epinephrine) - EMEA-002749-PIP01-19-M01

ARS Pharmaceuticals IRL, Limited; Treatment of allergic reactions

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

The applicant provided further information to support the modification.
The PDCO adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0431/2020 of 5 November 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.29. Modified allergen extract of birch pollen - EMEA-000932-PIP01-10-M02

ROXALL Medizin GmbH; Treatment of allergic rhinitis / rhino-conjunctivitis

Day 60 opinion

Pneumology - Allergology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could not be accepted.
The PDCO therefore adopted a negative opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/310/2010 of 22 December 2010).

2.3.30. Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Yamagata lineage) / Split influenza virus, inactivated containing antigens

equivalent to the B-like strain (Victoria lineage) / Split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain - EMEA-002359-PIP01-18-M04

Sanofi Pasteur; Prevention of influenza infection

Day 60 opinion

Vaccines

Summary of Committee discussion:

The applicant addressed satisfactorily the remaining minor points after D30 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/0010/2021 of 18 January 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.31. SARS CoV2 prefusion Spike delta TM (CoV2 preS dTM) protein, recombinant adjuvanted with AS03 - EMEA-002915-PIP01-20-M01

Sanofi Pasteur; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 opinion

Vaccines

Summary of Committee discussion:

The PDCO discussed the proposal of the applicant for modifications to Study VAT00003, the main paediatric clinical study of this PIP (children from birth to less than 18 years of age).
The applicant proposed the addition of a dose finding cohort in adolescents from 12 to less than 18 years of age in the study, to increase the safety dataset and choose the most adequate dose in this age group from the immunogenicity and reactogenicity perspective.
This was considered acceptable.
The PDCO considered that the changes proposed by the applicant were acceptable and that a positive opinion could be already granted at Day 30.
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/0201/2021 of 10 May 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

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

2.7.1. Risdiplam - EMEA-C2-002070-PIP01-16-M06

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 30 letter

Neurology

2.7.2. Atogepant - EMEA-C1-002530-PIP01-18

AbbVie Deutschland GmbH & Co. KG; Prevention of migraine headaches

Day 30 letter

Neurology

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. Abelacimab - EMEA-003017-PIP01-21

Prevention of venous thromboembolism associated with cancer

Day 90 discussion

Cardiovascular Diseases

3.1.2. Dupilumab - EMEA-001501-PIP09-21

Treatment of chronic inducible cold urticaria

Day 90 discussion

Dermatology

3.1.3. EMEA-003027-PIP01-21

Treatment of hyperphenylalaninaemia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Treatment of paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Haematology-Hemostaseology

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

UCB Pharma S.A; Treatment of immune thrombocytopenia

Day 90 discussion

Haematology-Hemostaseology

3.1.6. Censavudine - EMEA-003075-PIP01-21

Treatment of Aicardi-Goutières syndrome

Day 90 discussion

Neurology

3.1.7. Invimestrocel - EMEA-002317-PIP02-21

Treatment of acute ischaemic stroke

Day 90 discussion

Neurology

3.1.8. 1-[({4-[(4-fluoro-2-methyl-1H-indol-5-yl)oxy]-6- methoxyquinolin-7-yl}oxy)methyl]cyclopropan-1-amine bishydrochloride - Orphan - EMEA-002486-PIP04-21

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

Day 90 discussion

Oncology

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

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

Day 90 discussion

Other

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

FibroGen, Inc.; Treatment of Duchenne muscular dystrophy

Day 90 discussion

Other

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

Influenza due to identified zoonotic or pandemic influenza virus

Day 90 discussion

Vaccines

3.1.12. Ex vivo expanded autologous human keratinocytes containing epidermal stem cells transduced with a LAMB3-encoding retroviral vector - Orphan - EMEA-003137-PIP01-21

Holostem Terapie Avanzate s.r.l.; Treatment of junctional epidermolysis bullosa (JEB)

Day 60 discussion

Dermatology

3.1.13. Tezepelumab - EMEA-001613-PIP04-21

Treatment of chronic spontaneous urticaria

Day 60 discussion

Dermatology

3.1.14. A nonreplicating, recombinant adeno-associated virus (AAV) serotype 9 (AAV9) gene transfer vector that contains a modified human ATP7B coding sequence - Orphan - EMEA-003131-PIP01-21

Ultragenyx Germany GmbH; Treatment of Wilson disease

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.15. Elamipretide - Orphan - EMEA-003128-PIP01-21

Stealth BioTherapeutics Inc.; Treatment of Barth syndrome

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.16. EMEA-003116-PIP01-21

Treatment of coeliac disease

Day 60 discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.1.17. Ibrexafungerp citrate - EMEA-002535-PIP04-21

Invasive candidiasis

Day 60 discussion

Infectious Diseases

3.1.18. Nifurtimox - EMEA-003134-PIP01-21

Treatment of Chagas disease

Day 60 discussion

Infectious Diseases

3.1.19. Ivermectin - EMEA-003136-PIP01-21

Treatment of head lice infestations / Topical treatment of head lice infestations

Day 60 discussion

Infectious Diseases / Dermatology

Note: Withdrawal request received on 17 January 2022

3.1.20. Self-complementary adeno-associated viral vector serotype 9 containing the human CLN3 gene - Orphan - EMEA-003124-PIP01-21

Amicus Therapeutics Europe Limited; Neuronal ceroid lipofuscinosis - CLN3

Day 60 discussion

Neurology

3.1.21. EMEA-003129-PIP01-21

Osteosarcoma

Day 60 discussion

Oncology

3.1.22. (R)-tetrahydrofuran-3-yl 4-(6-(5-(4-ethoxy-1-isopropylpiperidin-4-yl)pyridin-2-yl)pyrrolo[1,2-b]pyridazin-4-yl)piperazine-1-carboxylate sesquisuccinate - Orphan - EMEA-003133-PIP01-21

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 60 discussion

Other

3.1.23. Anti TL1a mAb - EMEA-003111-PIP02-21

Treatment of asthma

Day 60 discussion

Other

3.1.24. Azelastine hydrochloride / mometasone furoate - EMEA-003122-PIP01-21

Seasonal allergic rhinitis

Day 60 discussion

Oto-rhino-laryngology

3.1.25. Zuranolone - EMEA-003119-PIP01-21

Treatment of postpartum depression

Day 60 discussion

Psychiatry

3.1.26. *Borrelia* outer surface protein A (OspA) serotypes (ST1-6) lipidated, fusion protein vaccine - EMEA-003130-PIP01-21

Prevention of Lyme disease

Day 60 discussion

Vaccines

3.1.27. Recombinant COVID-19 subunit nanoparticle - EMEA-003115-PIP01-21

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Vaccines

3.1.28. Crovalimab - EMEA-002709-PIP02-21

Guillain Barre syndrome

Day 30 discussion

Neurology

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

Prevention of atherothrombotic events

Day 30 discussion

Cardiovascular Diseases

3.1.30. Asundexian - EMEA-003144-PIP01-21

Prevention of arterial thromboembolism

Day 30 discussion

Cardiovascular Diseases

3.1.31. Indapamide / telmisartan - EMEA-003151-PIP01-21

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

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

OT4B; Treatment of Prader-Willi syndrome

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Treatment of Crohn's disease

Day 30 discussion

Gastroenterology-Hepatology

3.1.34. EMEA-003143-PIP01-21

Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.1.35. EMEA-002927-PIP02-21

Treatment of polymyalgia rheumatica

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.36. Ianalumab - EMEA-002338-PIP03-21

Treatment of systemic lupus erythematosus (SLE)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.37. Secukinumab - EMEA-000380-PIP09-21

Treatment of lichen planus (including mucosal lichen planus)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.38. Tocilizumab - EMEA-000309-PIP09-21

Treatment of systemic sclerosis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.39. 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 30 discussion

Infectious Diseases

3.1.40. Ensovibep - EMEA-003150-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.1.41. Remibrutinib - EMEA-002582-PIP02-21

Treatment of multiple sclerosis

Day 30 discussion

Neurology

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

Roche Registration GmbH; Treatment of multiple myeloma

Day 30 discussion

Oncology

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

Treatment of multiple myeloma

Day 30 discussion

Oncology

3.1.44. EMEA-003110-PIP02-21

Treatment of neurofibromatosis type 1

Day 30 discussion

Oncology

3.1.45. Odronextamab - EMEA-003149-PIP01-21

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

Day 30 discussion

Oncology

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

Incyte Biosciences Distribution B.V.; Endometrial carcinoma

Day 30 discussion

Oncology

3.1.47. Sintilimab - EMEA-002919-PIP02-21

Oesophageal cancer

Day 30 discussion

Oncology

3.1.48. EMEA-003141-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Other

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

Scholar Rock, Inc.; Spinal muscular atrophy

Day 30 discussion

Other / Neurology

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

Influenza due to identified zoonotic or pandemic influenza virus

Day 30 discussion

Vaccines

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

Prevention of respiratory tract disease caused by respiratory syncytial virus

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

Regeneron Pharmaceuticals, Inc.; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

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

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

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

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

infection

Day 30 discussion

Infectious Diseases

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

Eisai GmbH; Treatment of soft tissue sarcoma

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

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

Amicus Therapeutics Europe Limited; Treatment of Fabry disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

AstraZeneca AB; Treatment of type 2 diabetes

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Gastroenterology-Hepatology

3.3.4. Ozanimod hydrochloride - EMEA-001710-PIP04-17-M03

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

Day 30 discussion

Gastroenterology-Hepatology

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

Merck Sharp & Dohme (Europe), Inc.; Prevention of recurrence of *Clostridioides difficile*

infection

Day 30 discussion

Infectious Diseases

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

SymBio Pharmaceuticals Limited; Treatment of adenovirus in immunocompromised patients

Day 30 discussion

Infectious Diseases

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

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

Day 30 discussion

Infectious Diseases

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

Janssen-Cilag International NV; Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

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

GW Pharma (International) B.V; Spasticity

Day 30 discussion

Neurology

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

Eli Lilly and Company Limited; Prevention of migraine headache

Day 30 discussion

Neurology

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

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 30 discussion

Neurology

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

Novartis Ireland Limited; Treatment of multiple sclerosis

Day 30 discussion

Neurology

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

Novartis Europharm Ltd; Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.3.14. (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 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

3.3.16. 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 30 discussion

Oncology

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

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma

Day 30 discussion

Oncology

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

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

Day 30 discussion

Oncology

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

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Oncology / Haematology-Hemostaseology

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

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

Day 30 discussion

Other

3.3.21. Amikacin (sulfate) - Orphan - EMEA-000525-PIP01-08-M08

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

Day 30 discussion

Pneumology - Allergology

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

AstraZeneca AB; Asthma

Day 30 discussion

Pneumology - Allergology

3.3.23. (S)-(2-(5-chloro-4-methyl-1H-benzo[d]imidazol-2-yl)-2-methylpyrrolidin-1-yl)(4-methoxy-2-(2H-1,2,3-triazol-2-yl)phenyl)methanone hydrochloride - EMEA-002121-PIP03-19-M01

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of insomnia

Day 30 discussion

Psychiatry

3.3.24. Esketamine hydrochloride - EMEA-001428-PIP03-15-M02

Janssen-Cilag International NV; Major depressive disorder (MDD)

Day 30 discussion

Psychiatry

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

Shire Pharmaceuticals Contract Limited; Treatment of attention deficit hyperactivity disorder (ADHD)

Day 30 discussion

Psychiatry

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

Amgen Europe B.V.; Treatment of hyperparathyroidism

Day 30 discussion

Uro-nephrology

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

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

Day 30 discussion

Uro-nephrology / Haematology-Hemostaseology

3.3.28. 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 30 discussion

Vaccines

4. Nominations

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

4.1. List of letters of intent received for submission of applications with start of procedure 31 January 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)

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

6.1.1. Tramadol hydrochloride / magnesium lactate dihydrate - EMEA-12-2021

SciencePharma spółka z ograniczoną odpowiedzialnością spółka jawna; All classes of medicinal products for treatment of primary and secondary osteoarthrosis; Management of chronic pain in adults with osteoarthritis of the hip and/or 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 not confirmed.

Other potential paediatric interests of this medicine suggested by PDCO: treatment of pain.

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

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

7.1.1. Otilimab - EMEA-001882-PIP02-16-M02

GlaxoSmithKline Trading Services Limited; Treatment of rheumatoid arthritis (RA) in adults

Proposed PIP indication: Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis)

Summary of Committee discussion:

The PDCO confirmed the indication proposed falls within the agreed PIP condition.

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. Update on PDCO member(s)/alternate(s) mandate status

The PDCO Committee noted the nomination of Tomas Boran as the new member of Czechia.

9.1.2. Vote by proxy

No item

9.1.3. Strategic Review and Learning Meeting (SRLM) – Paris, 31 March – 1 April 2022

PDCO member: Sylvie Benchetrit

Summary of Committee discussion:

PDCO members were informed that the next strategic review and learning meeting in Paris will be held virtually rather than face to face as originally indicated to the current COVID situation in France.

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 with paediatric indications to be evaluated by the CHMP, starting in December 2021, was presented to the PDCO members.

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Summary of Committee discussion:

The Chair of the Non-clinical Working Group (NcWG) identified the products which will require NcWG evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of Committee discussion:

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

9.4. Cooperation within the EU regulatory network

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

No item

9.5. Cooperation with International Regulators

No item

9.5.1. Report from the Paediatric Cluster Teleconference

Summary of Committee discussion:

The minutes of the cluster and the list of further teleconference meeting dates in 2022 were shared with the PDCO members for information.

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

9.7.1. PDCO Work Plan 2022

PDCO Chair: Koenraad Norga

Summary of Committee discussion:

The PDCO work plan was adopted. The document will be available on the EMA website.

9.8. Planning and reporting

No item

10. Any other business

10.1. COVID-19 update

Summary of Committee discussion:

The Committee was updated on the latest developments of COVID vaccines and treatments that are relevant to paediatrics.

10.2. International Council for Harmonisation – ICH E11A – Paediatric Extrapolation

Summary of Committee discussion:

PDCO were asked for their views ahead of Step 1 of the document. These will be taken forward to the wider ICH Working Group to ensure the document is suitable for publication.

10.3. Working party implementation update - call for volunteers

Summary of Committee discussion:

The call for nominations for the experts for working parties was presented. The Committee noted the draft documents and the timetables for the call for nominations.

10.4. Paediatric Process Review – project update

Summary of Committee discussion:

In view of the action items defined in the EMA-EC action plan to improve the handling of PIP applications an update was provided to PDCO on activities to review paediatric processes and procedures. There was a call for interest for PDCO members to join a working group in this

respect.

11. Breakout sessions

11.1. Internal PDCO Operations

Summary of Committee discussion:

PDCO members discussed issues relating to internal operations.

11.2. Neonatology

Summary of Committee discussion:

The breakout session was cancelled as no topic of relevance was identified.

11.3. Paediatric oncology

Summary of Committee discussion:

Members were informed about ongoing oncology meetings and discussed about PIP-related issues.

11.4. Vaccines

Summary of Committee discussion:

Discussion on principles of COVID-19 vaccines 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 18-21 January 2021 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium	No interests declared	
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Marleen Renard	Member	Belgium	No restrictions applicable to this meeting	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Georgios Savva	Member	Cyprus	No interests declared	
Tereza Bazantova	Alternate	Czechia	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
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 participation in discussion, final deliberations and voting on:	2.2.1. Nirsevimab - EMEA-C2-001784-PIP01-15-M03

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Silvijus Abramavicius	Alternate	Lithuania	No restrictions applicable to this meeting	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No 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 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 participation in final deliberations and voting on:	2.2.1. Nirsevimab - EMEA-C2-001784-PIP01-15-M03
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 participation in final deliberations and voting on:	2.2.1. Nirsevimab - EMEA-C2-001784-PIP01-15-M03
Jaroslav Sterba	Member	Patients' Organisation Representative	No interests declared	
Dimitrios Athanasiou	Member	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		
Tomasz Grybek	Alternate	Patients' Organisation Representative	No interests declared	
Michal Odermarsky	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
María Estela Moreno Martín	Expert	Spain	No interests declared	
Lutz Wiesner	Expert	Germany	No interests declared	
Kristinn Karlsson	Expert	Sweden	No restrictions applicable to this 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/