26 January 2018
EMA/PDCO/49067/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes of the meeting on 23 – 26 January 2018

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

23 January 2018, 14:00- 19:00, room 2A

24 January 2018, 08:30- 19:00, room 2A

25 January 2018, 08:30- 19:00, room 2A

26 January 2018, 08:30- 13:00, room 2A

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.

Further information with relevant explanatory notes can be found at the end of this document.

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

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. 23 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 PDCO agenda was adopted.

1.3. Adoption of the minutes

The minutes of the December 2017 PDCO were adopted and will be published on the EMA website.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Tralokinumab - EMEA-001900-PIP02-17

LEO Pharma A/S; Treatment of Atopic Dermatitis

Day 120 opinion

Dermatology

Summary of committee discussion:

All the issues raised at Day 60 have been addressed and resolved satisfactorily now. The Committee adopted a positive opinion.

2.1.2. Non-Pathogenic Bacterial Lysate of Escherichia coli and Enterococcus faecalis - EMEA-002155-PIP01-17

SymbioPharm GmbH; Irritable bowel syndrome (IBS)

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

A waiver for all subsets of the paediatric population (0 to 18 years of age) in the condition of irritable bowel syndrome (IBS) 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 subsets was adopted.

2.1.3. Crizanlizumab - Orphan - EMEA-002141-PIP01-17

Novartis Europharm Limited; Treatment of sickle cell disease / Prevention of vaso-occlusive crises in patients with sickle cell disease

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The applicant's response to the outstanding issues remaining at Day 90 was received on 12 January 2018.
As all the issues were thus solved satisfactorily the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant. The Committee also agreed a deferral.

2.1.4. Anifrolumab - EMEA-001435-PIP02-16

AstraZeneca AB; Lupus nephritis, Systemic lupus erythematosis

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The applicant took into account the outcome of the PDCO's Day 90. Based on the review of the further rationale submitted by the applicant, the PDCO deemed the proposed programme acceptable and adopted a positive opinion.

2.1.5. Upadacitinib - EMEA-001741-PIP03-16

AbbVie Ltd; Treatment of Crohn's Disease

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The applicant's response to the D90 issue was acceptable and a positive opinion was adopted at Day 120.

2.1.6. Insulin human - EMEA-002116-PIP01-17

Nutrinia, Ltd.; Treatment of intestinal malabsorption in preterm infants

Day 120 opinion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

Summary of committee discussion:

The applicant's responses to the D90 issues were considered acceptable and a positive opinion was adopted.

2.1.7. Adeno-Associated Viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA - Orphan - EMEA-002122-PIP02-17

LYSOGENE; Mucopolysaccharidosis type IIIA

Day 120 opinion

Neurology

Summary of committee discussion:

Based on the assessment of this application and further assessment of the clarifications provided by the applicant after Day 90 and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's position.
The PDCO recommended granting a PIP with a deferral for 'adeno-associated viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA

2.1.8. D-Sorbitol / Naltrexone HCl / (RS)-Bacoflen - Orphan - EMEA-002164-PIP01-17

Pharnext SA; Charcot-Marie-Tooth disease Type 1A / Treatment of Charcot-Marie-Tooth Type 1A in symptomatic paediatric patients

Day 120 opinion

Neurology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO concluded that all issues have now been resolved and the modified PIP is acceptable. A positive opinion endorsing the applicant's latest proposal has been adopted accordingly.

2.1.9. Durvalumab - EMEA-002028-PIP01-16

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue), Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumours Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy

Day 120 opinion

Oncology

Summary of committee discussion:

The application for durvalumab was re-discussed taken into account the clarifications provided by the applicant after D90 and the comments received by the applicant on the draft opinion.
The justifications and clarifications provided by the applicant on the concerns raised at D90 were considered adequate and all issues considered solved.
In conclusion, the PDCO recommends granting a paediatric investigation plan and a deferral for durvalumab for the 'treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue)' and 'treatment of malignant neoplasms of haematopoietic and lymphoid tissue'.

2.1.10. Pevonedistat - EMEA-002117-PIP01-17

Takeda Pharma A/S; Acute Myeloid Leukemia (AML), Myelodysplastic Syndromes (MDS) / Treatment of paediatric patients with relapsed or refractory (R/R) MDS (including juvenile myelomonocytic leukemia)/Treatment of paediatric patients with relapsed or refractory (R/R) AML

Day 120 opinion

Oncology

Summary of committee discussion:

The applicant's response to the outstanding issues remaining at Day 90 was received on 12 January 2018.
As all the issues were thus solved satisfactorily the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant.

2.1.11. Tremelimumab - EMEA-002029-PIP01-16

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue), Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumour, Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy.

Day 120 opinion

Oncology

Summary of committee discussion:

The application for tremelimumab was re-discussed taken into account the clarifications provided by the applicant after D90 and the comments received by the applicant on the draft opinion.
The justifications and clarifications provided by the applicant on the concerns raised at D90 were considered adequate and all issues considered solved.
In conclusion, the PDCO recommends granting a paediatric investigation plan and a deferral for tremelimumab for the 'treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue)' and 'treatment of malignant neoplasms of haematopoietic and lymphoid tissue'.

2.1.12. Vamorolone - Orphan - EMEA-001794-PIP02-16

ReveraGen BioPharma Ltd; Treatment of duchenne muscular dystrophy

Day 120 opinion

Other

Summary of committee discussion:

Following additional information received by the applicant, in reply to the points raised at D90 and following other exchange of information, the PDCO agreed a favourable opinion at their January 2018 meeting for vamorolone for the condition treatment of Duchenne muscular dystrophy, with a deferral.

2.1.13. Tanezumab - EMEA-001635-PIP03-17

Pfizer Limited; Treatment of chronic pain

Day 120 opinion

Pain

Summary of committee discussion:

The remaining issues in the opinion regarding time points of assessment had been solved between Day 90 and Day 120. The committee adopted a positive opinion.

2.1.14. Vilanterol trifenatate / Umeclidinium bromide / Fluticasone furoate - EMEA-002153-PIP01-17

GlaxoSmithKline Trading Services Limited; ICD-10 J45.5x severe persistent asthma

Day 120 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO's views expressed at day 90 were re-discussed taking into account the applicant clarifications.

Based on the assessment of this application and further discussions, the Paediatric Committee adopted a positive opinion.

2.1.15. Candesartan (cilexetil) / amlodipine (besylate) - EMEA-002248-PIP01-17

Midas Pharma GmbH; Treatment of essential hypertension (ICD9: 401, ICD10: I10)

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO confirmed the outcome of the Day 30 discussion and based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Candesartan cilexetil / Amlodipine besylate for all subsets of the paediatric population (0 to less than 18 years of age) in the condition of Treatment of hypertension.

2.1.16. Ezetimibe / Rosuvastatin - EMEA-002257-PIP01-17

ELPEN Pharmaceutical Co. Inc; Treatment of hypercholesterolemia / The combination of Rosuvastatin and Ezetimibe is indicated for the treatment of hypercholesterolemia as substitution therapy in adult patients adequately controlled with the individual substances given concurrently at the same dose level as in the fixed dose combination, but as separate products.

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 agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Rosuvastatin / Ezetimibe for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of hypercholesterolemia.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.17. Treprostinil sodium - Orphan - EMEA-002254-PIP01-17

SciPharm Sàrl; Treatment of (inoperable) chronic thromboembolic pulmonary hypertension (CTEPH)

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 agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Treprostinil sodium for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of (inoperable) chronic thromboembolic pulmonary hypertension (CTEPH).

2.1.18. Levothyroxine sodium - EMEA-002259-PIP01-17

IBSA Farmaceutici Italia Srl; Benign thyroid nodules, Goitre, Hypothyroidism

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 agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for levothyroxine (sodium) for all subsets of the paediatric population (0 to 18 years of age) in the conditions of benign thyroid nodules, goitre and hypothyroidism.

2.1.19. Metformin hydrochloride / dapagliflozin - EMEA-001151-PIP02-17

AstraZeneca AB; Type 2 diabetes (E11)

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

During its plenary on 26 January 2018 the Paediatric Committee (PDCO) recommended granting a waiver for dapagliflozin / metformin hydrochloride for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of type 2 diabetes mellitus.
The single components of this fixed dose combination are already available (Metformin). A separate paediatric development was not deemed to fulfil a medical need. The PDCO adopted a waiver in children from 10-18 years of age on the grounds of lack of significant therapeutic benefit and in children from birth-10 years based on the grounds of the disease not occurring.

2.1.20. Metformin hydrochloride / saxagliptin / dapagliflozin - EMEA-002249-PIP01-17

AstraZeneca AB; Type 2 diabetes (E11)

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

During its plenary on 26 January 2018 the Paediatric Committee (PDCO) recommended granting a waiver for the fixed dose combination (FDC) Metformin/Saxagliptin/Dapagliflozin for all subsets of the paediatric population (0 to 18 years of age) in the condition treatment of type 2 diabetes mellitus.
The single components of this FDC are already available (Metformin).
A triple combination of glucose lowering agents is not considered to meet a paediatric medical need, specifically not when it is a FDC without dosing flexibility.
The PDCO adopted a waiver in children from 10-18 years of age on the grounds of lack of significant therapeutic benefit and in children from birth-10 years based on the grounds of the disease not occurring.

2.1.21. Pemafibrate - EMEA-001573-PIP02-17

Kowa Research Europe Ltd; Treatment of hypertriglyceridaemia, Prevention of cardiovascular events in patients with elevated triglycerides levels

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for pemafibrate for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of hypertriglyceridaemia, Prevention of cardiovascular events in patients with elevated triglycerides levels.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.22. Venglustat - EMEA-001716-PIP02-17

Genzyme Europe B.V.; ICD-10: G20; Disease of the nervous system; Extrapyramidal and movement disorders (G20-G26); Parkinson disease.

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for venglustat for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of Parkinson's disease.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population

are available even if a waiver has been granted in another condition.

2.1.23. Entinostat Polymorph B - EMEA-002272-PIP01-17

Syndax Pharmaceuticals, Inc.; Treatment of breast cancer

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed its views expressed at Day 30 taking into account the applicant's clarifications. The Committee agreed to grant a waiver for conducting studies in the paediatric population in the condition "Treatment of breast malignant neoplasm" on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.24. Niraparib - Orphan - EMEA-002268-PIP01-17

Janssen Research & Development; Treatment of prostate malignant neoplasms

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed its views expressed at Day 30 taking into account the applicant's clarifications.
Based on these clarifications and timelines for a PIP submission the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for niraparib for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of prostate malignant neoplasms on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations
As stated above the PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.25. T-cell bispecific antibody targeting carcinoembryonic antigen expressed on tumor cells and CD3 epsilon chain present on T-cells - EMEA-002252-PIP01-17

Roche Registration Limited; Treatment of all conditions included in the category of

malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms)

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's view expressed at D30 was endorsed.
Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for 'T-cell bispecific antibody targeting carcinoembryonic antigen expressed on tumor cells and CD3 epsilon chain present on T-cells' for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of 'treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms)'.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need, if any will be identified in the future. 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.

2.1.26. Veliparib - Orphan - EMEA-000499-PIP04-17

AbbVie Ltd; Treatment of lung carcinoma (SCLC and NSCLC)

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the requested product specific waiver for veliparib for the treatment of lung carcinoma (small cell and non-small cell carcinoma) taking into consideration the additional clarifications provided by the applicant after the D30 discussion.
The members concluded that at this stage it is unclear what could be the possible role of veliparib in treatment of paediatric tumours used in monotherapy or in combination and therefore considered adequate to limit the scope of this application to treatment of lung carcinoma (small cell and non-small cell carcinoma).
In conclusion, based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for veliparib 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 occurs only in the adult population.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle, according to the Paediatric Regulation, incentives for the development for use in the paediatric population

are available even if a waiver has been granted in another condition.

2.1.27. Gabapentin / Trazodone hydrochloride - EMEA-002263-PIP01-17

Aziende Chimiche Riunite Angelini Francesco - A.C.R.A.F. - S.p.A.; Painful diabetic neuropathy

Day 60 opinion

Pain

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver.
The PDCO emphasises that the granting of a waiver for the condition discussed 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. Ibuprofen - EMEA-002302-PIP01-17

Farmalíder, S.A.; R52, R50.9 / Fever, unspecified, Pain, unspecified

Day 30 opinion

Other / Pain

Summary of committee discussion:

The PDCO reviewed the application along with the assessors' comments. Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for ibuprofen for all subsets of the paediatric population.
The PDCO emphasises that the granting of a waiver for a condition should not prevent the applicant from considering 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.

2.1.29. Brivaracetam - Orphan - EMEA-000332-PIP02-17

UCB Pharma S.A.; treatment of paediatric epilepsy syndromes / treatment of refractory paediatric epilepsy syndromes with adjunctive administration of brivaracetam

Day 30 opinion

Neurology

Summary of committee discussion:

The PDCO agreed with the request to split the PIP per conditions and adopted a positive opinion at Day 30.

2.1.30. Ruxolitinib phosphate - EMEA-000901-PIP04-17

Chronic graft versus host disease / Treatment of chronic Graft vs Host Disease (GvHD) after allogeneic hematopoietic stem cell transplantation (alloSCT) in paediatric patients aged 28 days and above.

Day 60 opinion

Oncology

Summary of committee discussion:

The applicant's response to the issues raised at Day 30 was received on 12 January 2018 and further responses were received on 19 January 2018.
As all the issues were thus solved satisfactorily the PDCO adopted a positive opinion on the paediatric plan proposed by the applicant.

2.2. Opinions on Compliance Check

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

2.2.1. Tolvaptan - EMEA-C1-001231-PIP02-13-M05

Otsuka Pharmaceutical Europe Ltd.; Treatment of polycystic kidney disease

Day 60 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO adopted the report of the applicant. The partial compliance check is positive.

2.2.2. Guselkumab - EMEA-C1-001523-PIP02-14-M01

Janssen Cilag International NV; Treatment of Psoriasis

Day 30 letter

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed the compliance request at D30. The study was conducted in accordance with the PIP opinion and a positive opinion on this partial compliance check was adopted.

2.2.3. Ozanimod - EMEA-C2-001710-PIP02-14-M02

Celgene Europe Limited; Treatment of Multiple Sclerosis

Day 30 letter

Neurology

Summary of committee discussion:

The PDCO finalised on 24 January 2018 this partially completed compliance procedure and confirmed the compliance of all those studies contained in the agreed paediatric investigation plan that were to be completed until this date.

2.2.4. L-Asparaginase encapsulated in Erythrocytes - EMEA-C3-000341-PIP02-09-M05

ERYTECH Pharma S.A.; Treatment of acute lymphoblastic leukaemia

Day 30 letter

Oncology

Summary of committee discussion:

The PDCO discussed the completed study and considered that this was compliant with the latest Agency's Decision (P/0004/2018) of 04 January 2018.
The PDCO finalised on 26 January 2018 this partially completed compliance procedure and confirmed the compliance of all those studies contained in the agreed paediatric investigation plan that were to be completed until this date.

2.2.5. Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human Arylsulfatase A (ARSA) cDNA sequence - EMEA-C2-001765-PIP02-15-M01

GlaxoSmithKline Trading Services Limited; Treatment of metachromatic leukodystrophy (MLD)

Day 30 letter

Other

Summary of committee discussion:

The completed study was checked for compliance.
The PDCO discussed the completed study and considered that this is compliant with the latest Agency's Decision (P/0160/2017) of 30 June 2017.
The PDCO finalised on 26 January 2018 this partially completed compliance procedure and confirmed the compliance of the completed non-clinical study contained in the agreed Paediatric Investigation Plan.

2.2.6. Ivacaftor/ Lumacaftor - EMEA-C5-001582-PIP01-13-M06

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 letter

Other

Summary of committee discussion:

The following completed studies were checked for compliance. Compliance of all measures of studies can be confirmed.

Compliance with the following studies had been previously confirmed in the partial compliance procedures EMEA-C1-001582-PIP01-13 (compliance report EMA/604209/2014), EMEA-C2-001582-PIP01-13 (compliance report EMA/515971/2016), EMEA-C3-001582-PIP01-13 (compliance report EMA/733410/2016) and EMEA-C4-001582-PIP01-13 (compliance report EMA/25130/2017).

In conclusion, the PDCO finalised on 26 January 2018 this fifth partially completed compliance procedure and confirmed the compliance of all those studies contained in the agreed paediatric investigation plan (P/0198/2017 of 14 July 2017) that were to be completed until this date.

2.2.7. Dupilumab - EMEA-C1-001501-PIP02-13-M02

sanofi-aventis recherche & développement; Treatment of asthma

Day 30 letter

Pneumology - Allergology

Summary of committee discussion:

The PDCO discussed the compliance request on D30. Studies have been conducted in compliance with the key binding elements in the PIP opinion.

2.2.8. Human coagulation factor X - EMEA-C-000971-PIP01-10-M03

Bio Products Laboratory Ltd; Treatment of hereditary factor X deficiency

Day 30 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The completed study was checked for compliance.
The PDCO took note of preceding procedure and report on partially completed compliance (EMEA-C2-000971-PIP01-10-M02).
The PDCO adopted on 26 January 2018 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0389/2017) of 19 December 2017.

2.2.9. Plerixafor - EMEA-C-000174-PIP01-07-M03

Genzyme Europe B.V.; Myelosuppression caused by chemotherapy to treat malignant disorders, which requires an autologous haematopoietic stem cell transplant

Day 30 opinion

Oncology

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/0253/2013 of

29 October 2013).

2.2.10. Piperaquine tetraphosphate / artenimol - EMEA-C-000153-PIP01-07-M05

Alfasigma S.p.A.; Uncomplicated malaria caused by Plasmodium faciparum

Day 30 opinion

Infectious Diseases

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/0002/2018) of 04 January 2018.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Alirocumab - EMEA-001169-PIP01-11-M04

Sanofi-aventis Recherche & Developpement; Treatment of elevated cholesterol / Treatment of heterozygous and homozygous familial hypercholesterolemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
In particular, the Paediatric Committee reviewed the additional information provided by the applicant between D30 and D60.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0269/2017 of 04 September 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M01

Novartis Europharm Limited; Treatment of adrenal cortical hyperfunctions / Treatment of Cushing's disease in adolescents and children aged 6 years and older

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

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

accepted.

2.3.3. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M03

AstraZeneca AB; Treatment of hyperkalemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO discussed this modification procedure at D60. The applicant's response to the D30 issues was considered acceptable.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0226/2017 of 11 August 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human betaA-T87Q-globin gene - Orphan - EMEA-001665-PIP01-14-M02

Bluebird bio France; β-thalassaemia

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0297/2016 of 04/11/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Lonoctocog alfa - EMEA-001215-PIP01-11-M06

CSL Behring GmbH; Haemophilia A

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0183/2017of 03/07/2017), during its plenary

meeting on 26 January 2018.

2.3.6. Octocog alfa - EMEA-001064-PIP01-10-M03

Bayer AG; Treatment of hereditary factor VIII deficiency / Treatment and prophylaxis of bleeding in patients with haemophilia A

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0107/2014 of 05 May 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Rolapitant - EMEA-001768-PIP02-15-M01

Tesaro UK Ltd; Prevention of nausea and vomiting/ Prevention of acute and delayed nausea and vomiting associated with initial and repeat courses of highly emetogenic cisplatin-based cancer therapy and moderately emetogenic cancer therapy

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The application was re-discussed after the applicant answered the questions raised on D30.
In conclusion, the PDCO granted a positive opinion on the proposed changes.

2.3.8. Golimumab - EMEA-000265-PIP02-11-M02

Janssen Biologics B.V.; Ulcerative Colitis ICD: K51 / Treatment of ulcerative colitis

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed this modification at D60.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0073/2014 of 2 April 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO

Opinion.

2.3.9. Denosumab - EMEA-000145-PIP01-07-M09

Amgen Europe B.V.; Prevention of skeletal related events in patients with bone metastases, Treatment of hypercalcemia of malignancy, Treatment of chronic idiopathic artritis, Treatment of bone loss associated with sex hormone ablative therapy, Treatment of giant cell tumour of bone / Treatment of giant cell tumour of bone in children (12-17 years old).

Day 60 opinion

Immunology-Rheumatology-Transplantation / Endocrinology-Gynaecology-Fertility-Metabolism / Oncology

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0125/2016 of 20/05/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion

2.3.10. Fidaxomicin - EMEA-000636-PIP01-09-M07

Astellas Pharma Europe B.V.; Treatment of enterocolitis caused by clostridium difficile / Treatment of Clostridium difficile infections (CDI) also known as C. difficile-associated diarrhoea (CDAD)

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO confirmed the outcome of the discussion at Day 30. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0243/2017 of 4/9/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Erenumab - EMEA-001664-PIP02-15-M02

Novartis Europharm Limited; Prevention of migraine headaches

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO at their January 2018 meeting noted further feed-back received by the applicant.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0310/2017 of 31/10/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Lacosamide - EMEA-000402-PIP03-17-M02

UCB Pharma S.A.; Treatment of Generalized Epilepsy and Epilepsy Syndromes: Epilepsy - generalized idiopathic epilepsy and epilepsy syndromes [G40.3] Epilepsy - Other generalized epilepsy and epileptic syndromes [G40.4] / Adjunctive therapy in the treatment of primary generalized tonic-clonic seizures (PGTCS) in pediatric patients with idiopathic generalized epilepsy (IGE)(4 years to <18 years), Adjunctive therapy in the treatment of epileptic syndromes associated with generalized seizures in pediatric patients with epilepsy birth to <18 years (specific epileptic syndrome(s) to be based on future clinical development further to exploratory study results)

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the assessment of this application including the new information received since Day 30 and further discussions at the Paediatric Committee, the PDCO concluded that all issues have now been resolved and the modified PIP is acceptable. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Pyridopyrimidione SMN2 Splicing Modifier - EMEA-002070-PIP01-16-M01

Roche Registration Limited; Treatment of spinal muscular atrophy

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0284/2017 of 04 October 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Binimetinib - EMEA-001454-PIP03-15-M01

PIERRE FABRE MEDICAMENT; Treatment of melanoma / Binimetinib in combination with encorafenib is indicated for the treatment of patients aged 12 years and older with unresectable or metastatic melanoma harbouring BRAF V600 mutations.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the proposed modification for binimetinib taking into account the clarifications provided by the applicant after D30.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0051/2016 of 18 March 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Encorafenib - EMEA-001588-PIP01-13-M01

PIERRE FABRE MEDICAMENT; Treatment of melanoma / Encorafenib in combination with binimetinib is indicated for the treatment of patients aged 12 years and older with unresectable or metastatic melanoma harbouring BRAF V600 mutations.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the proposed modification for encorafenib (to be used in combination with binimetinib, EMEA-001454-PIP03-15) taking into account the clarifications provided by the applicant after D30.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0054/2016 of 18 March 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Nivolumab - EMEA-001407-PIP01-12-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old., Treatment of a paediatric malignant solid tumour in paediatric patients from 6 months to less than 18 years old.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the proposed modification taking into account the clarifications provided by the applicant after D30, including also the comments on the draft opinion. In conclusion, based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0064/2014 of 07 March 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Nivolumab - EMEA-001407-PIP02-15-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue, Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients with relapsed or refractory Hodgkin lymphoma in the age group from 5 years to < 18 years., Treatment of paediatric patients with a relapsed or refractory non-Hodgkin lymphoma in the age group from 6 months to less than 18 years old., Treatment of paediatric patients from 6 months to less than 18 years of age with a recurrent or progressive high-grade glioma.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the proposed modification taking into account the clarifications provided by the applicant after D30, including also the comments on the draft opinion. Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0004/2017 of 13 January 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. Pembrolizumab - EMEA-001474-PIP01-13-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of advanced, untreated or previously treated, malignant melanoma in children from 12 year old to less than 18 years of age / Treatment as monotherapy of a PD-L1 positive paediatric malignant solid tumor in children from 6 months to less than 18 years of age.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's view expressed at D30 was endorsed.

In conclusion, based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0059/2014 of 07 March 2014).

2.3.19. Selumetinib - EMEA-001585-PIP01-13-M02

AstraZeneca AB; Treatment of Thyroid Cancer, Treatment of Neurofibromatosis-Type 1 / Selumetinib in combination with adjuvant radioactive iodine therapy is medicated for the treatment of adolescents newly diagnosed with differentiated thyroid cancer who are at h1gh risk of primary treatment failure., Selumetinib is indicated for the treatment of inoperable NFl related plexiform neurofibroma in children and adolescents

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO issued a positive opinion for the modification.

2.3.20. Ivacaftor - Orphan - EMEA-000335-PIP01-08-M12

Vertex Pharmaceuticals (Europe) Limited; Treatment of Cystic Fibrosis

Day 60 opinion

Other

Summary of committee discussion:

The PDCO's views expressed at Day 30 were re-discussed taking into account the applicant's supplementary information and clarifications.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0147/2017 of 7 June 2017), accepting some but not all proposed changes.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.21. Dermatophagoides farinae / Dermatophagoides pteronyssinus - EMEA-001258-PIP01-11-M03

ALK-Abelló A/S; Treatment of allergic rhinitis, Treatment of asthma / indicated in house dust mite allergic asthma,

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that most of the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0284/2015 of 27 November 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.22. Ivacaftor - Orphan - EMEA-001640-PIP01-14-M04

Vertex Pharmaceuticals (Europe) Ltd.; Treatment of Cystic Fibrosis

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO re-discussed the proposed modifications taking into account the applicant's clarifications.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that some, but not all 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/0311/2017 of 31 October 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.23. Mometasone furoate / Indacaterol acetate - EMEA-001217-PIP01-11-M04

NOVARTIS EUROPHARM LTD.; Treatment of asthma

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO's views expressed at day 30 were re-discussed taking into account the applicant's additional clarifications as well as the clarifying T-conference between Day 30 and Day 60.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0141/2017 of 7 June 2017), accepting some, but not all proposed changes.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.24. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M13

UCB Pharma S.A.; Treatment of epilepsy with partial onset seizures, Treatment of neonatal seizures / Treatment of neonatal seizures with adjunctive administration of brivaracetam, Treatment of paediatric patients with partial onset seizures

Day 30 opinion

Neurology

Summary of committee discussion:

The PDCO agreed with the request to split the PIP per conditions and adopted a positive opinion at Day 30. The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.25. Dasatinib (as monohydrate) - Orphan - EMEA-000567-PIP01-09-M05

Bristol-Myers Squibb Pharma EEIG; Treatment of Philadelphia chromosome (BCR-ABL translocation)-positive acute lymphoblastic leukaemia, Treatment of Philadelphia chromosome (BCR-ABL translocation)-positive chronic myeloid leukaemia / Treatment of Philadelphia chromosome (BCR-ABL translocation)-positive (Ph+) chronic myeloid leukaemia, Treatment of Philadelphia chromosome (BCR-ABL translocation)-positive (Ph+) acute lymphoblastic leukaemia

Day 30 opinion

Oncology

Summary of committee discussion:

The PDCO discussed the modification request on 24 January 2018.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0118/2013 of 02 May 2013).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.26. Influenza virus surface antigens (haemagglutinin and neuraminidase) of the following strains: A/<Official Strain>(H1N1), A/<Official Strain>(H3N2), B/<Official Strain>Yamagata lineage, B/<Official Strain>Victoria lineage based on annual recommendations by WHO, CHMP (EU) and other regional or local authorities - EMEA-001782-PIP01-15-M02

Abbott Biologicals B.V.; Prevention of Influenza infection / Prophylaxis of influenza; especially in those who run an increased risk of associated complications

Day 30 opinion

Vaccines

Summary of committee discussion:

A positive Opinion was adopted at D30.

2.4. Opinions on Re-examinations

No items.

2.5. Finalisation and adoption of opinions

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. EMEA-002162-PIP01-17

Type 2 diabetes mellitus

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Obeticholic Acid - EMEA-001304-PIP03-17

NASH / NASH with Fibrosis

Day 90 discussion

Gastroenterology-Hepatology

3.1.3. Plazomicin Sulfate - EMEA-001639-PIP02-17

Infections due to enterobacteriaceae in patients with limited treatment options, complicated urinary tract infections including pyelonephritis

Day 90 discussion

Infectious Diseases

3.1.4. Ixazomib - Orphan - EMEA-001410-PIP02-17

Takeda Pharm A/S; Treatment of Acute Lymphoblastic Leukaemia (ALL), Treatment of Multiple Myeloma / Treatment of adult patients with Newly Diagnosed Multiple Myeloma (NDMM), Treatment of paediatric patients diagnosed with relapsed precursor B-ALL or T-ALL, Maintenance treatment of paediatric patients with newly diagnosed intermediate-risk or very high risk T-ALL/LLy

Day 90 discussion

Oncology

3.1.5. Taselisib - EMEA-002210-PIP01-17

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumors, hematopoietic and lymphoid tissue neoplasms) / Treatment of children with solid tumors with known or anticipated PI3K activation.

Day 90 discussion

Oncology

3.1.6. (R) - azasetron (as besylate) - Orphan - EMEA-002165-PIP01-17

Sensorion SA; Prevention of cisplatin-Induced otoxicity

Day 90 discussion

Oto-rhino-laryngology

3.1.7. Fevipiprant - EMEA-001315-PIP02-16

Asthma / Treatment of uncontrolled persistent asthma

Day 90 discussion

Pneumology - Allergology

3.1.8. B from Yamagata VLP Influenza Drug Substance (4 of 4) / B from Victoria lineage VLP Influenza Drug Substance (3 of 4) / H3 VLP Influenza Drug Substance (2 of 4) / Plant-derived Quadrivalent VLP Influenza vaccine composed of 4 active substances: H1 VLP Influenza Drug Substance (1 of 4) - EMEA-002220-PIP01-17

Prevention of influenza / For active immunization of persons six months of age and older for the prevention of influenza caused by influenza virus subtypes A and type B covered by the vaccine.

Day 90 discussion

Vaccines

3.1.9. Birch bark extract - Orphan - EMEA-001299-PIP03-17

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 60 discussion

Dermatology

3.1.10. Genetically modified Lactococcus lactis - EMEA-002237-PIP01-17

Treatment of Type 1 diabetes mellitus

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.11. EMEA-001710-PIP03-17

Treatment of ulcerative colitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.12. Risankizumab - EMEA-001776-PIP03-17

Crohn's Disease

Day 60 discussion

Gastroenterology-Hepatology

3.1.13. Risankizumab - EMEA-001776-PIP04-17

Ulcerative Colitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.14. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene - Orphan - EMEA-001665-PIP02-17

Bluebird bio France; Sickle Cell Disease

Day 60 discussion

Haematology-Hemostaseology

3.1.15. Eptinezumab - EMEA-002243-PIP01-17

Prevention of migraine headaches

Day 60 discussion

Neurology

3.1.16. Bilastine - EMEA-000347-PIP02-16

Treatment of allergic conjunctivitis

Day 60 discussion

Ophthalmology

3.1.17. Human Plasminogen - Orphan - EMEA-002253-PIP01-17

Kedrion S.p.A.; Treatment of Ligneous Conjunctivitis and prevention of pseudomembranes recurrence in patients affected by Ligneous Conjunctivitis

Day 60 discussion

Ophthalmology

3.1.18. Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin - Orphan - EMEA-002169-PIP01-17

Mereo Biopharma 3 Ltd; Treatment of osteogenesis imperfecta / Treatment of osteogenesis imperfecta, types 1, 3 and 4

Day 60 discussion

Other

3.1.19. Interferon beta-1a - Orphan - EMEA-002238-PIP01-17

Faron Pharmaceuticals Ltd; Treatment of Acute Respiratory Distress Syndrome

Day 60 discussion

Pneumology - Allergology

3.1.20. EMEA-002172-PIP02-17

Prevention of lower respiratory tract disease caused by respiratory syncytial virus

Day 60 discussion

Vaccines / Infectious Diseases

3.1.21. Neladenoson bialanate - EMEA-002262-PIP01-17

Treatment of Heart Failure

Day 30 discussion

Cardiovascular Diseases

3.1.22. Rosuvastatin / ezetimibe - EMEA-001344-PIP02-17

Prevention of Cardiovascular Events

Day 30 discussion

Cardiovascular Diseases

3.1.23. EMEA-002287-PIP01-17

Treatment of Type 2 Diabetes Mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. EMEA-002310-PIP01-17

Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Haematology-Hemostaseology

3.1.25. Baricitinib - EMEA-001220-PIP04-17

Treatment of systemic lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.26. Recombinant IgG degrading enzyme of Streptococcus pyogenes - Orphan - EMEA-002183-PIP01-17

Hansa Medical AB; Patients with chronic kidney disease in need of kidney transplantation / Prevention of graft rejection following solid organ transplantation

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.27. Aztreonam / Avibactam sodium - EMEA-002283-PIP01-17

Infections caused by Gram-negative bacteria, including those that produce metallo-β-lactamases, for which there are limited or no treatment options. / For the treatment of complicated urinary tract infections, For the treatment of Ventilator associated pneumonia, For the treatment of complicated intra-abdominal infections, For the treatment of hospital-acquired pneumonia

Day 30 discussion

Infectious Diseases

3.1.28. Ridinilazole - EMEA-002250-PIP02-17

Clostridium difficile Infection (CDI) and recurrence of CDI

Day 30 discussion

Infectious Diseases

3.1.29. Tafenoquine - EMEA-002301-PIP01-17

Prevention of malaria

Day 30 discussion

Infectious Diseases

3.1.30. Humanized recombinant IgG4 anti-human tau antibody - Orphan - EMEA-002226-PIP02-17

AbbVie Ltd; Progressive Supranuclear Palsy

Day 30 discussion

Neurology

3.1.31. Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes - Orphan - EMEA-002025-PIP03-17

Atara Biotherapeutics, Inc.; Treatment of Epstein-Barr virus associated lymphoproliferative diseases in patients with primary immune disorders

Day 30 discussion

Oncology

3.1.32. Enfortumab vedotin - EMEA-002299-PIP01-17

Treatment of locally advanced or metastatic urothelial cancer

Day 30 discussion

Oncology

3.1.33. Ipilimumab / nivolumab - EMEA-002049-PIP01-16

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms). / Treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old., Treatment of a paediatric malignant solid tumour in paediatric patients from 6 months to less than 18 years old.

Day 30 discussion

Oncology

3.1.34. Ivosidenib - EMEA-002247-PIP02-17

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms), Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients (2 to less than 18 years of age) with recurrent or progressive (R/P) malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms), including central nervous system tumours, with an isocitrate dehydrogenase-1 (IDH1) mutation.

Day 30 discussion

Oncology

3.1.35. Ivosidenib - Orphan - EMEA-002247-PIP03-17

Agios Pharmaceuticals, Inc.; Treatment of Acute Myeloid Leukaemia / Treatment of paediatric patients from 2 to less than 18 years of age with newly diagnosed and relapsed or refractory (R/R) AML with an isocitrate dehydrogenase-1 (IDH1) mutation.

Day 30 discussion

Oncology

3.1.36. Olaparib - Orphan - EMEA-002269-PIP01-17

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system [CNS], haematopoietic, and lymphoid tissue). / Treatment of paediatric patients from 6 months to ≤18 years old with homologous recombination repair (HRR) mutated solid tumours

Day 30 discussion

Oncology

3.1.37. Polatuzumab vedotin - EMEA-002255-PIP01-17

Treatment of Diffuse Large B-Cell lymphoma (DLBCL), Treatment of Burkitt lymphoma, Burkitt leukemia (BL/B-ALL), Treatment of Follicular lymphoma (FL)

Day 30 discussion

Oncology

3.1.38. Rovalpituzumab tesirine - Orphan - EMEA-002292-PIP01-17

AbbVie Ltd; Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 30 discussion

Oncology

3.1.39. Recombinant human acid ceramidase - Orphan - EMEA-002266-PIP01-17

Enzyvant Farber Ireland Ltd; Farber disease

Day 30 discussion

Other

3.1.40. EMEA-002293-PIP01-17

Oxaliplatin induced periperal neuropathy (CIPN)

Day 30 discussion

Other / Oncology

3.1.41. Olodanrigan - EMEA-002286-PIP01-17

Peripheral neuropathic pain / Treatment of moderate to severe peripheral neuropathic pain

Day 30 discussion

Pain

3.1.42. 3-pentylbenzeneacetic acid sodium salt - Orphan - EMEA-002265-PIP01-17

Prometic Pharma SMT Limited; Idiopathic pulmonary fibrosis

Day 30 discussion

Pneumology - Allergology

3.1.43. EMEA-002310-PIP02-17

Treatment of C3 glomerulopathy

Day 30 discussion

Uro-nephrology

3.1.44. Ferric Pyrophosphate Citrate - EMEA-002261-PIP01-17

Iron deficient anaemia / Treatment of iron deficient anaemia in haemodialysis patients

Day 30 discussion

Uro-nephrology / Haematology-Hemostaseology

3.2. Discussions on Compliance Check

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

3.2.1. Belatacept - EMEA-C3-000157-PIP01-07-M03

Bristol-Myers Squibb Pharma EEIG; Prevention of rejection of transplanted kidney

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.2. Crisaborole - EMEA-C2-002065-PIP01-16

Pfizer Limited; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.2.3. Lubiprostone - EMEA-C3-000245-PIP01-08-M04

Sucampo AG; Treatment of Constipation

Day 30 discussion

Gastroenterology-Hepatology

3.2.4. Fc- and CDR-modified humanised monoclonal antibody against C5 - EMEA-C1-002077-PIP01-16-M01

Alexion Europe SAS; Treatment of Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Haematology-Hemostaseology

3.2.5. Tocilizumab - EMEA-C-000309-PIP01-08-M07

Roche Registration Limited; Chronic Idiopathic Arthritis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.6. Lanadelumab - EMEA-C1-001864-PIP01-15-M01

Shire Pharmaceuticals Ireland Limited; Prevention against Acute Attacks of Hereditary Angioedema

Day 30 discussion

Other

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Dopamine hydrochloride - EMEA-001105-PIP01-10-M04

BrePco Biopharma Limited; Other hypotension (I95.8) / Treatment of hypotension in neonates including the extremely low gestational age newborn / Treatment of hypotension in infants and children

Day 30 discussion

Cardiovascular Diseases

3.3.2. Evolocumab - EMEA-001268-PIP01-12-M05

Amgen Europe B.V.; Treatment of mixed dyslipidaemia, Treatment of elevated cholesterol / Heterozygous Familial Hypercholesterolaemia (HeFH) and Homozygous Familial Hypercholesterolaemia (HoFH) after Prior Lipid-Lowering Therapy in paediatric subjects aged 10 years and above

Day 30 discussion

Cardiovascular Diseases

3.3.3. Crisaborole - EMEA-002065-PIP01-16-M01

Pfizer Ltd; Mild to moderate atopic dermatitis

Day 30 discussion

Dermatology

3.3.4. Tilmanocept - EMEA-001255-PIP01-11-M03

Norgine BV; Visualisation of lymphatic drainage of solid tumours for diagnostic purposes / Visualisation of lymphatic drainage of rhabdomyosarcoma and melanoma for diagnostic purposes

Day 30 discussion

Diagnostic / Oncology

3.3.5. Vedolizumab - EMEA-000645-PIP01-09-M06

Takeda Pharma A/S; Crohn's Disease, Ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.6. Luspatercept - Orphan - EMEA-001521-PIP01-13-M02

Celgene Europe Ltd; Anemias due to chronic disorders / Treatment of anemia in patients with b-thalassemia

Day 30 discussion

Haematology-Hemostaseology

3.3.7. Abatacept - EMEA-000118-PIP02-10-M03

Bristol-Myers Squibb Pharma EEIG; Chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis and juvenile idiopathic arthitis)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.8. Dalbavancin - EMEA-000016-PIP01-07-M06

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

Day 30 discussion

Infectious Diseases

3.3.9. Nusinersen - Orphan - EMEA-001448-PIP01-13-M03

Biogen Idec Ltd; Spinal muscular atrophy

Day 30 discussion

Neurology

3.3.10. Peginterferon beta-1a - EMEA-001129-PIP01-11-M02

Biogen Idec Ltd; Multiple Sclerosis / Treatment of relapsing remitting forms of Multiple Sclerosis

Day 30 discussion

Neurology

3.3.11. Ponesimod - EMEA-000798-PIP01-09-M01

Actelion Registration Ltd; Multiple Sclerosis / Relapsing Remitting forms of multiple sclerosis

Day 30 discussion

Neurology

3.3.12. Autologous CD4+ and CD8+ T cells Expressing a CD19-Specific Chimeric Antigen Receptor - Orphan - EMEA-001995-PIP01-16-M01

Celgene Europe Limited; Treatment of B-lymphoblastic leukemia/lymphoma, Treatment of mature B-cell neoplasms / Treatment of paediatric patients with CD19+ relapsed or refractory B-cell acute lymphoblastic leukaemia, Treatment of paediatric patients with CD19+ relapsed or refractory diffuse-large B-cell lymphoma, Burkitt lymphoma or primary mediastinal large B-cell lymphoma

Day 30 discussion

Oncology

3.3.13. Quizartinib - Orphan - EMEA-001821-PIP01-15-M01

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia / For the treatment of paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed AML with FLT3-ITD mutations., For the treatment of paediatric patients aged from 1 month to less than 18 years of age with refractory or relapsed AML with FLT3-ITD mutations after failure of front line intensive chemotherapy regimen, in combination with standard chemotherapy.

Day 30 discussion

Oncology

3.3.14. Andexanet alfa - EMEA-001902-PIP01-15-M02

Portola Pharma UK Limited; prevention of factor Xa inhibitor associated haemorrhage, treatment of factor Xa inhibitor associated haemorrhage / For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients requiring urgent surgery, For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients experiencing an acute major bleeding episode

Day 30 discussion

Other

3.3.15. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M06

MediWound Germany GmbH; treatment of burns of external body surfaces / Removal of eschar in deep partial and/or full thickness burns

Day 30 discussion

Other

3.3.16. Palovarotene - Orphan - EMEA-001662-PIP01-14-M02

Clementia Pharmaceuticals Inc.; Treatment of Fibrodysplasia Ossificans Progressiva (FOP)

Day 30 discussion

Other

3.3.17. Methoxyflurane - EMEA-000334-PIP01-08-M07

Medical Developments UK Ltd; Treatment of acute pain / Self administration to conscious patients with minor trauma and associated pain, under supervision of personnel trained in its use, 2. For the management of acute pain associated with short surgical procedures, such as the change of dressings, dislocations and injections.

Day 30 discussion

Pain

3.3.18. Tapentadol - EMEA-000325-PIP01-08-M09

Grünenthal GmbH; Treatment of chronic pain

Day 30 discussion

Pain

3.3.19. Benralizumab - EMEA-001214-PIP01-11-M07

AstraZeneca AB; Asthma

Day 30 discussion

Pneumology - Allergology

4. Nominations

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

4.1. List of letters of intent received for submission of applications with start of procedure 3 April 2018 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

None

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

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

6. Discussion on the applicability of class waivers

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

6.1. Discussions on the applicability of class waiver for products

6.1.1. 5-fluorouracil - EMEA-18-2017

PIERRE FABRE DERMATOLOGIE; Topical treatment of actinic keratosis lesions of the face, ears and/or scalp / Treatment of actinic keratosis

Summary of committee discussion:

The applicability of the class waiver as referred to in the Agency's Decision CW/1/2011 to the planned therapeutic indication was confirmed.

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

6.1.2. Trastuzumab emtansine - EMEA-19-2017

Roche Registration Limited; Single agent for the adjuvant treatment of adult patients with HER2-positive early breast cancer / Class of Her- / Epidermal growth factor-receptor antibody medicinal products for the treatment of breast malignant neoplasms

Summary of committee discussion:

The applicability of the class waiver as referred to in the Agency's Decision CW/001/2015 to the planned therapeutic indication was confirmed.

Other potential paediatric interests of this medicine suggested by PDCO: even the applicability of the class-waiver was confirmed; HER2 is essential for normal embryonic development and has a critical function in oncogenesis. Increased expression was documented in Wilms tumors, neuroblastoma and osteosarcoma, but mostly without gene amplification.

The applicant is therefore strongly encouraged to conduct preclinical studies assessing pediatric tumors as well.

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. Tafamidis meglumine - EMEA-000884-PIP01-10

Pfizer Limited; neuropathic heredofamilial amyloidosis

Proposed indication: cardiomyopathy (due to wild-type or variant transthyretin)

Summary of committee discussion:

The PDCO has reviewed the request during the plenary meeting held on 24 January 2018.

The PDCO was of the view that the indication “Treatment of cardiomyopathy due to wild-type or variant transthyretin in adults” falls under the scope of the above mentioned Decision as the indication is considered to be covered by the condition “Neuropathic heredofamilial amyloidosis” listed in the Agency Decision.

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

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 2017 was presented to the PDCO members.

The members were also informed about 4 medicinal products, Alkindi, Crysvita, Truvada and Yervoy for which the CHMP adopted a positive opinion recommending a paediatric indication during their meeting in December 2017.

9.2.2. Coordination Group for Mutual Recognition and Decentralised Procedures - Human (CMDh)

CMDh Question to PDCO

PDCO member: Hugo Tavares

Summary of committee discussion:

After further discussions, the Committee adopted the written response to CMDh.

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Summary of committee discussion:

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

9.3.2. PDCO confirmation of new NcWG experts: January 2018 PDCO

PDCO member: Karen van Malderen

Summary of committee discussion:

The PDCO confirmed the new members of the NcWG.

9.3.3. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

The chair of the Formulation Working Group identified the products which will require Formulation Working Group evaluation and discussion.

9.3.4. Guideline on the clinical investigation of recombinant and 4 human plasma-derived factor VIII products

Postponed

9.3.5. Draft Guideline on good pharmacovigilance practices (GVP) Product or Population Specific Considerations IV: Paediatric Pharmacovigilance

9.3.6. Pilot phase on the Inventory of unmet needs

PDCO member: Karl-Heinz Huemer

Summary of committee discussion:

The chair of the Inventory group presented the progress on this topic to the Committee.

9.3.7. Risk Management Plan and PIP studies: synergies or overlap

Summary of committee discussion:

EMA gave an update on the revised GVP V and on current understanding regarding how to report information on the paediatric subpopulation in the RMP for various types of products in relation to their indication. The updated RMP template incorporates this revised guidance.

9.3.8. Patients and Consumers Working Party (PCWP)

- Agenda of the PCWP meeting with all eligible organisations – 22 November 2017
- Meeting Summary PCWP meeting with all eligible organisations – 22 November 2017

The documents were tabled for information

9.3.9. Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP)

- AMR info session report – 19 September 2017
- Agenda of the PCO & HCPO training - 21 November 2017

The documents were tabled for information

9.3.10. Questions and answers on ethanol used as an excipient in medicinal products for human use

Summary of committee discussion:

The Committee briefly discussed, on the clinical labelling point of view, the use of ethanol as an excipient in children, in light of the Draft 'Questions and answers on ethanol used as an excipient in medicinal products for human use' currently under internal consultation.

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's work plan for 2018 includes the implementation of the EMA "Principles on the involvement of young patients/consumers within EMA activities", to facilitate and promote involvement of young patients within PDCO activities as needed. To contribute to this activity, the European network of young people advisory groups (eYPAGnet) was

invited to the PDCO plenary to inform the committee about their activities and to discuss how they could promote the involvement of young people in PDCO activities.

The meeting started with a short presentation by EMA's public engagement department to inform the PDCO about the recently adopted "Principles on the involvement of young patients/consumers within EMA activities" and to provide some examples of patients' involvement along the medicine lifecycle at EMA.

eYPAGnet is currently formed of 9 teams: 1 in Barcelona, 6 in the UK, 1 team in France (Lyon) and one in Scotland (Scottish Children's Research Network). Several new teams are in development across Europe. eYPAGnet is a member of Enpr-EMA as well as of the International children's advisory network (iCAN). eYPAGnet has established one single point of contact (coordinator Hospital Sant Joan de Deu, Barcelona). Monthly meetings will allow timely feedback to questions of PDCO.

The committee agreed to establish a small group of PDCO members, together with PCPWP members, to look at how and when it would be beneficial for PDCO to consult with young people in their work.

9.5. Cooperation with International Regulators

None

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

None

9.7. PDCO work plan

None

9.8. Planning and reporting

None

10. Any other business

10.1. AOB topic

10.1.1. Involvement of young people at PDCO

Summary of committee discussion:

The recently adopted 'Principals for involvement of young people at EMA', which provides the basis by which the EMA and its committees may consult and involve young people in their work, was presented. A well-established patient involvement at the Agency was explained and PDCO members invited to take advantage of the infrastructure in place

within the public engagement department to accommodate requests for involvement in PDCO activities.

It was proposed to set up a group of interested PDCO members to discuss how, and when would be most beneficial to consult young people. The YPAGnet group, who have established youth groups, would also be involved. Next steps will be shared with the group shortly.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The group discussed planned activities and upcoming events in 2018.

11.1.2. Neonatology

Summary of committee discussion:

The group discussed planned activities and upcoming events in 2018.

11.1.3. Inventory

Summary of committee discussion:

The inventory group continued discussing the methodology to be used to identify paediatric unmet needs.

11.1.4. Overview of Duchenne PIPs

Summary of committee discussion:

A group of PDCO members reviewed the pipeline for Duchenne PIPs.

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 23 – 26 January 2018 meeting.

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dirk Mentzer	Chair	Germany	No interests declared	
Karl-Heinz Huemer	Member	Austria	No interests declared	
Koenraad Norga	Member (Vice-Chair)	Belgium	When chairing the meeting: To be replaced for discussions, final deliberations and voting When not chairing the meeting: No participation in final deliberations and voting	EMEA-002153-PIP01-17 EMEA-C2-001765-PIP02-15-M01
Karen Van Malderen	Alternate	Belgium		
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Adriana Andrić	Member	Croatia	No interests declared	
Suzana Mimica Matanovic	Alternate	Croatia	No interests declared	
Georgios Savva	Member	Cyprus	No interests declared	
Peter Szitanyi	Alternate	Czech Republic	No interests declared	
Kirstine Moll Harboe	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Ann Marie Kaukonen	Member	Finland	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Maija Pihlajamaki	Alternate	Finland	No interests declared	
Sylvie Benchetrit	Member	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Immanuel Barth	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Mountaki	Alternate	Greece	No interests declared	
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No restrictions applicable to this meeting	
Sigita Burokiene	Member	Lithuania	No interests declared	
Goda Vaitkeviciene	Alternate	Lithuania	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Maaike van Dartel	Member	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No interests declared	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Peter Sisovsky	Member	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesús Fernández Cortizo	Alternate	Spain	No interests declared	
Ninna Gullberg	Member	Sweden	No interests declared	
Angeliki Siapkara	Member	United Kingdom	No interests declared	
Martina Riegl	Alternate	United Kingdom	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No interests declared	
Riccardo Riccardi	Alternate	Healthcare Professionals' Representative	No participation in discussion, final deliberations and voting on:	EMEA-001716-PIP02-17 EMEA-C-000174-PIP01-07-M03
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Günter Karl-Heinz Auerswald	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Viviana Giannuzzi	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Shiva Ramroop	Expert - in person*	United Kingdom	No restrictions applicable to this meeting	
Catriona Baker	Expert - in person*	United Kingdom	No interests declared	
Sabine Kudicke	Expert - via telephone*	Germany	No interests declared	
Begonya Nafira Escalera	Expert - in person*	Spain	n/a	
Pamela Dicks	Expert - in person*	United Kingdom	No interests declared	
Eleni Gaki	Expert - in person*	United Kingdom	No interests declared	
Representative from the European Commission participated in the meeting				
Meeting run with support from relevant EMA staff				

* Experts were only evaluated against the agenda topics or activities they participated in

13. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Paediatric investigation plan (PIP) (section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs)
A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, when it is safe to do so, to support the authorisation of a medicine for children. Pharmaceutical companies submit proposals for PIPs to the European Medicines Agency's Paediatric Committee (PDCO). This Committee is responsible for agreeing or refusing the plan.

Compliance checks (section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check)
A compliance check may be necessary before any application for marketing authorisation (even for an adult indication) can be considered valid, if there was no deferral for at least one of the studies agreed in the PIP, or after the due date of initiation or completion of a study/measure. The same applies to some regulatory applications for authorised products, as described above.

Modification of an Agreed Paediatric Investigation Plan (section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP)
The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.
In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

Class waiver (section 6 Discussion on the applicability of class waiver)
As some diseases do not affect children (for example Parkinson's disease), the development of medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived**.** For more information on the classes of diseases subject to waivers, see class waivers.

Annual reports on deferrals (section 8)
If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

More detailed information on the above terms can be found on the EMA website:
www.ema.europa.eu/