20 June 2017
EMA/PDCO/307150/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes for the meeting on 16-19 May 2017

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

16 May 2017, 14:00- 19:00, room 2F

17 May 2017, 08:30- 19:00, room 2F

18 May 2017, 08:30- 19:00, room 2F

19 May 2017, 08:30- 13:00, room 2F

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 agenda was adopted with amendments.

1.3. Adoption of the minutes

The minutes of the May PDCO plenary meeting 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. lacosamide - EMEA-000402-PIP03-17

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 30 discussion

Neurology

Summary of committee discussion:

The splitting of the PIP to two separate PIPs per condition has been endorsed by the PDCO. A positive opinion was adopted at Day 30.

2.1.2. Empagliflozin - EMEA-000828-PIP04-16

Boehringer Ingelheim International GmbH; Treatment of type 1 diabetes mellitus

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee on the applicant's clarifications/proposals to the draft opinion, the PDCO adopted a positive PIP opinion for Empagliflozin.

2.1.3. Omadacycline - EMEA-000560-PIP02-15

Paratek UK Limited; Treatment of acute bacterial skin and skin structure infections (ABSSSI)

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO re-discussed its view expressed at day 90 taking into account the applicant's supplementary information.

Based on the assessment of this application, the PDCO adopted a positive opinion.

2.1.4. Omadacycline - EMEA-000560-PIP03-15

Paratek UK Limited; Treatment of bacterial pneumonia

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO re-discussed its view expressed at day 90 taking into account the applicant's supplementary information.

Based on the assessment of this application, the PDCO adopted a positive opinion.

2.1.5. Larotrectinib - Orphan - EMEA-001971-PIP02-16

Loxo Oncology, Inc.; Treatment of solid tumours / Treatment of adults, adolescents, children and infants with advanced solid tumours harbouring an NTRK fusion, as established prior to initiation of larotrectinib therapy

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the application for larotrectinib taking also into account the clarifications provided by the applicant after the D90 discussion.

All the pending issues were considered solved.

In conclusion the PDCO recommended granting a paediatric investigation plan for the entire paediatric population from birth to less than 18 years of age and a deferral.

2.1.6. Fluocinolone Acetonide - Orphan - EMEA-000801-PIP03-16

CAMPHARM Limited; Chronic non-infectious uveitis affecting the posterior segment of the eye

Day 120 opinion

Ophthalmology

Summary of committee discussion:

A positive opinion was adopted on D120.

2.1.7. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16

Lupin (Europe) Ltd.; Symptomatic treatment of myotonic disorders

Day 120 opinion

Other

Summary of committee discussion:

The PDCO re-discussed its view expressed at day 90 taking into account the applicant's supplementary information.

The PDCO adopted a positive opinion.

2.1.8. Live attenuated, chimeric dengue virus, serotype 4 / Live, attenuated, chimeric dengue virus, serotype 3 / Live, attenuated dengue virus, serotype 2 / Live, attenuated, chimeric dengue virus, serotype 1 - EMEA-001888-PIP01-15

Takeda Vaccines, Inc.; Prevention of dengue fever

Day 120 opinion

Vaccines

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO at their May 2017 meeting agreed a PIP for this Live attenuated, chimeric dengue virus, serotype 4 / Live, attenuated, chimeric dengue virus, serotype 3 / Live, attenuated, chimeric dengue virus, serotype 1 / Live, attenuated dengue virus, serotype 2 in the condition of prevention of dengue fever with a deferral.

2.1.9. Amlodipine / Rosuvastatin - EMEA-002130-PIP01-17

CIPROS S.R.L.; Treatment of angina and dyslipidaemia, Treatment of concomitant hypertension and dyslipidemia, Treatment of essential hypertension in patients who are estimated to have a high risk for a first cardiovascular event

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO's view expressed at day 30 was re-discussed and endorsed. Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The committee recommends to grant a waiver for rosuvastatin/amlodipine for all subsets of the paediatric population on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments in the following conditions:

- Prevention of cardiovascular events
- Treatment of ischemic coronary artery disorders
- Treatment of hypertension
- Treatment of dyslipidaemia.

2.1.10. Amlodipine / Rosuvastatin - EMEA-002136-PIP01-17

ERREKAPPA EUROTERAPICI S.p.A.; Treatment of angina and dyslipidaemia, Treatment of concomitant hypertension and dyslipidemia, Treatment of essential hypertension in patients who are estimated to have a high risk for a first cardiovascular event

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO's view expressed at day 30 was re-discussed and endorsed. Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The committee recommends to grant a waiver for rosuvastatin/amlodipine for all subsets of the paediatric population on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments in the following conditions:

- Prevention of cardiovascular events
- Treatment of ischemic coronary artery disorders
- Treatment of hypertension
- Treatment of dyslipidaemia.

2.1.11. Amlodipine besilate / Hydrochlorothiazide / Olmesartan medoxomil - EMEA-002104-PIP01-16

Accord Healthcare, S.L.U.; Essential Hypertension (MedDRA PT: 10015488)

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application the PDCO at their May 2017 meeting agreed with the applicant's request for a waiver. The PDCO recommends granting a waiver for Hydrochlorothiazide / Amlodipine / Olmesartan medoxomil for all subsets of the paediatric population (0 to 18 years of age) in the condition of Essential Hypertension with grounds based on lack of therapeutic benefit over existing treatments.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.12. dezamizumab - Orphan - EMEA-002110-PIP02-17

GlaxoSmithKline Trading Services Limited; Treatment of transthyretin amyloidosis (ATTR)

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

A positive opinion was adopted on D60.
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 dezamizumab and miridesap for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of transthyretin amyloidosis (ATTR).

2.1.13. Ezetimibe / Rosuvastatin (calcium) - EMEA-002131-PIP01-17

Errekappa Euroterapici S.p.A.; Treatment of hypercholesterolaemia

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO's views expressed at Day 30 was re-discussed and endorsed.
Based on the assessment of this application and further discussions at the Paediatric

Committee including contributions of external expert(s), the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for rosuvastatin (calcium) / ezetimibe for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of elevated cholesterol.

2.1.14. Ezetimibe / Rosuvastatin (calcium) - EMEA-002135-PIP01-17

BENEDETTI & Co. S.r.l.; Treatment of hypercholesterolaemia

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO's views expressed at Day 30 was re-discussed and endorsed
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 (calcium) / ezetimibe for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of elevated cholesterol.

2.1.15. miridesap - Orphan - EMEA-002111-PIP02-17

GlaxoSmithKline Trading Services Limited; Treatment of transthyretin amyloidosis (ATTR)

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

A positive opinion was adopted on D60.
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 miridesap for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of transthyretin amyloidosis (ATTR).

2.1.16. Benzydamine hydrochloride / Econazole nitrate - EMEA-002143-PIP01-17

Aziende Chimiche Riunite Angelini Francesco A.C.R.A.F. S.p.A.; Treatment of vulvovaginal candidosis (VVC)

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

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 econazole (nitrate) / benzydamine (hydrochloride) for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of vulvovaginal candidiasis.

2.1.17. dezamizumab - Orphan - EMEA-002110-PIP01-17

GlaxoSmithKline Trading Services Limited; Systemic AL amyloidosis

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

A positive opinion was adopted on D60.
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 dezamizumab for all subsets of the paediatric population (0 to 18 years of age) in the condition of Systemic AL amyloidosis.

2.1.18. miridesap - Orphan - EMEA-002111-PIP01-17

GlaxoSmithKline Trading Services Limited; Systemic AL amyloidosis

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

A positive opinion was adopted on D60.
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 miridesap for all subsets of the paediatric population (0 to 18 years of age) in the condition of Systemic AL amyloidosis.

2.1.19. efavirenz / lamivudine / abacavir - EMEA-002114-PIP01-16

Lek Pharmaceuticals d.d.; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

In conclusion, based on the assessment of this application and further discussions at the Paediatric Committee including contributions of the experts, the PDCO agrees with the applicant's request for a full product-specific waiver. The PDCO recommends granting a waiver for lamivudine / efavirenz / abacavir for all subsets of the paediatric population (0 to less than 18 years of age) in the condition of Treatment of human immunodeficiency virus (HIV-1) infection.

2.1.20. pseudoephedrine HCl / ibuprofen - EMEA-002102-PIP01-16

FARMALIDER, S.A; J06.9/ For the relief of symptoms of cold and flu with associated pain, fever, congestion and runny nose

Day 60 opinion

Infectious Diseases / Oto-rhino-laryngology

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 all subsets of the paediatric 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.

2.1.21. Radium Ra 223 dichloride - EMEA-001986-PIP01-16

Bayer AG; C00 - C70, C73 - C80, C97: Treatment of all conditions contained in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms), C90: Treatment of Multiple Myeloma

Day 60 opinion

Oncology

Summary of committee discussion:

Further clarifications and justifications for the proposed full product-specific waiver were provided by the applicant on 5 May 2017.
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 Radium Ra223 dichloride for all subsets of the paediatric population (0 to less than 18 years of age) in the conditions of 'Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms)' and 'Treatment of multiple myeloma'.
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. 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.

2.2. 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. cinacalcet - EMEA-C-000078-PIP01-07-M08

Amgen Europe B.V; treatment of secondary hyperparathyroidism in patients with end-stage renal disease

Day 30 discussion

Uro-nephrology

Summary of committee discussion:

The PDCO discussed the compliance request on D0 and concluded that the studies are in compliance with the agreed PIP. This new compliance opinion supersedes the opinion for EMEA-C-000078-PIP01-07-M07.

2.2.2. emicizumab - EMEA-C1-001839-PIP01-15

Roche Registration Limited; Treatment of Hereditary FVIII Deficiency

Day 60 letter

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (Decision P/0196/2016) of 15 July 2016.
The PDCO finalised on 19 May 2017 this partially completed compliance procedure.

2.2.3. mepolizumab - EMEA-C1-000069-PIP04-13-M01

GSK Trading Services Limited; Treatment of vasculitides

Day 60 letter

Pneumology - Allergology

Summary of committee discussion:

The following completed study(ies)was/were checked for compliance.

The PDCO finalised on 19th May 2017 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. mirabegron - EMEA-C2-000597-PIP02-10-M05

Astellas Pharma Europe B.V.; Treatment of Idiopathic overactive bladder

Day 60 letter

Uro-nephrology

Summary of committee discussion:

The applicant provided the missing information regarding the potential issues identified at Day 30. The committee found the information sufficient.
The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0287/2016) of 4 November 2016.
The PDCO finalised on 19 May 2017 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 by this date.

2.2.5. mirabegron - EMEA-C2-000597-PIP03-15-M03

Astellas Pharma Europe B.V.; Treatment of neurogenic detrusor overactivity

Day 60 letter

Uro-nephrology

Summary of committee discussion:

The applicant provided the missing information regarding the potential issues identified at Day 30. The committee found the information sufficient.
The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0056/2017) of 16 March 2017.
The PDCO finalised on 19 May 2017 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.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. lacosamide - EMEA-000402-PIP02-11-M04

UCB Pharma S.A.; Treatment of Epilepsy - Partial-onset seizures [G40.0 - G40.1 - G40.2] / Adjunctive therapy in the treatment of partial-onset seizures with or without secondary generalization in pediatric patients with epilepsy (birth to <16 years), Monotherapy in the treatment of partial-onset seizures with or without secondary generalization in pediatric patients (1 month to <18 years)

Day 30 opinion

Neurology

Summary of committee discussion:

The splitting of the PIP to two separate PIPs per condition has been endorsed by the PDCO. A positive opinion was adopted at Day 30.

2.3.2. ferric maltol - EMEA-001195-PIP01-11-M03

Shield TX (UK) Limited; Iron deficiency anaemia / Treatment for iron deficiency anaemia (IDA)

Day 30 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/0102/2017 of 11/04/2017).

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

2.3.3. Dobutamine - EMEA-001262-PIP01-12-M03

Proveca Limited; Circulatory impairment / haemodynamic insufficiency

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan and in line with the discussion during the April 2017 plenary , the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0331/2016 of 02/12/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. ticagrelor - EMEA-000480-PIP01-08-M10

AstraZeneca AB; thromboembolic events (children), acute coronary syndrome, history of myocardial infarction / reduction in occurrence of vaso-occlusive crises in paediatric patients with sickle cell disease, waiver

Day 60 opinion

Cardiovascular Diseases / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed this procedure at the May 2017 plenary.
The Committee considered the clarifications provided by the applicant
The PDCO also agreed with the other changes requested.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0298/2015 of 21 December 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Lonoctocog alfa - EMEA-001215-PIP01-11-M05

CSL Behring GmbH; Haemophilia A

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO re-discussed this modification during the May 2017 plenary.
The Committee assessed the answers the applicant provided to the questions asked during the day 30 plenary the PDCO did not agree with modifying the study.

2.3.6. Apremilast - Orphan - EMEA-000715-PIP05-13-M01

Celgene Europe Limited; Treatment of Behcets Disease / Treatment of patients with active oral ulcers (with or without genital ulcers) associated with Behcets Disease, who are candidates for systemic therapy

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO's view expressed at Day 30 was re-discussed.
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/0217/2014 of 18 July 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Certolizumab pegol - EMEA-001071-PIP02-12-M02

UCB Pharma S.A.; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed this modification request Therefore, the PDCO adopted an Opinion on the refusal of a modification of the agreed PIP and granted a product-specific waiver in all paediatric age groups on own motion based on the lack of a significant therapeutic benefit. This new PIP Opinion (i.e. full waiver) will cover the conditions/indications treatment of chronic idiopathic arthritis, including rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis and juvenile idiopathic arthritis as well as juvenile idiopathic arthritis associated uveitis).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Aciclovir - EMEA-001066-PIP02-11-M02

ONXEO; Treatment of herpes simplex labialis / Treatment of recurrent herpes simplex virus infections of the lips in immunocompetent children aged 10 to less than 18 years

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The committee re-discussed its view expressed at day 30, taking into account the applicant's supplementary information
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as

set in the Agency's latest decision (P/0312/2013 of 19 December 2013).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of HIV-1 infection / indicated in combination with other ARV medicinal products for the treatment of HIV-1 infected adults and adolescents from 12 years of age without known mutations associated with resistance to atazanavir.

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO discussed the modification request on 16 May 2017.
The PDCO adopted a favourable Opinion on some of the requested changes in the current modification of the agreed PIP as set in the Agency's latest decision (P/0090/2014 of 4 April 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Colistimethate sodium - Orphan - EMEA-000176-PIP01-07-M05

TEVA B.V.; Cystic fibrosis with pulmonary manifestations ICD-10 (version 2007) E84.0 / Treatment of Pseudomonas aeruginosa pulmonary infection in patients with cystic fibrosis aged 6 years and over.

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO re-discussed its views expressed at day 30 in light of the applicant's supplementary information.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0181/2013 of 31 July 2013).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Velpatasvir / Sofosbuvir - EMEA-001646-PIP01-14-M01

Gilead Sciences International Ltd.; Treatment of Chronic Hepatitis C / Treatment of chronic Hepatitis C in adolescents and children 3 years of age and older

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

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

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0099/2015 of 8 May 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Dimethyl fumarate - EMEA-000832-PIP01-10-M04

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

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 and taking the comments of the assessors into account, the PDCO considered that the proposed changes could be accepted.
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. ozanimod - EMEA-001710-PIP02-14-M01

Celgene Europe Limited; Treatment of multiple sclerosis / Treatment of relapsing remitting forms of multiple sclerosis

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/0198/2015 of 4 September 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Teriflunomide - EMEA-001094-PIP01-10-M04

Genzyme Europe B.V. / Sanofi-Aventis groupe; Multiple Sclerosis / Treatment of children and adolescents from 10 to less than 18 years of age with relapsing forms of Multiple Sclerosis

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 and taking the comments of the assessors into account, the PDCO considered that the proposed changes could be accepted.

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.

2.3.15. pazopanib - EMEA-000601-PIP01-09-M04

Novartis Europharm Limited; Treatment of pediatric patients with rhabdomyosarcoma, Treatment of pediatric patients with Ewing sarcoma family of tumours, Treatment of pediatric patients with non-rhabdomyosarcoma soft tissue sarcoma

Day 60 opinion

Oncology / Uro-nephrology

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/0061/2016 of 18/03/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. CYSTEAMINE HYDROCHLORIDE - Orphan - EMEA-000322-PIP01-08-M05

ORPHAN EUROPE SARL; CYSTINOSIS / Treatment of corneal cystine crystal deposits in cystinosis

Day 60 opinion

Ophthalmology

Summary of committee discussion:

The PDCO discussed this modification procedure on D60. The applicant's responses were acknowledged.
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/0322/2013).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human ARSA cDNA sequence - Orphan - EMEA-001765-PIP02-15-M01

GlaxoSmithKline Trading Services Limited; Metachromatic leukodystrophy (MLD)

Day 60 opinion

Other

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/0222/2016 of 12 August 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. methoxyflurane - EMEA-000334-PIP01-08-M06

Medical Developments UK Ltd; treatment of acute pain / 1. 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 60 opinion

Pain

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan and taking the comments of the assessors into account, the PDCO considered that the proposed changes could be accepted.
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.

2.3.19. Ivacaftor - EMEA-001640-PIP01-14-M02

Vertex Pharamceuticals (Europe) lTd; Cystic Fibrosis / Treatment of Cystic Fibrosis

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO's view expressed at day 30 was re-discussed and endorsed, taking into account the applicant's additional information and request.
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/0193/2016 of 15 July 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.20. Fluticasone furoate / vilanterol - EMEA-000431-PIP01-08-M10

Glaxo Group Limited; Mixed Asthma / Treatment of asthma where use of a combination product (long acting beta agonist and inhaled corticosteroid) is appropriate

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, and the supplementary information received the PDCO considered that the proposed changes of the PIP for fluticasone furoate / vilanterol for the condition treatment of asthma 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/0276/2016 of 10/10/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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. Fc- and CDR-modified humanised monoclonal antibody against C5 - Orphan - EMEA-002077-PIP01-16

Alexion Europe SAS; Paroxysmal Nocturnal Haemoglobinuria / Treatment of Paroxysmal Nocturnal Haemoglobinuria

Day 90 discussion

Haematology-Hemostaseology

3.1.2. Daunorubicin (liposomal combination) / Cytarabine (liposomal combination) - Orphan - EMEA-001858-PIP02-16

Jazz Pharmaceuticals Ireland Limited; Acute myeloid leukemia

Day 90 discussion

Oncology

3.1.3. Venetoclax - Orphan - EMEA-002018-PIP02-16

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms, Treatment of solid tumour malignant neoplasms / As monotherapy, or in combination with chemotherapy, for the treatment of relapsed or refractory NHL patients < 18 years of age, who have progressed following autologous stem cell transplantation or who are ineligible for

transplantation, As monotherapy or in combination for the treatment of patients with relapsed or refractory neuroblastoma < 18 years of age, As monotherapy, or in combination with chemotherapy, for the treatment of relapsed or refractory ALL in the third line setting in patients < 18 years of age, As monotherapy, or in combination with chemotherapy, for the treatment of relapsed or refractory AML in patients < 18 years of age

Day 90 discussion

Oncology / Haematology-Hemostaseology

3.1.4. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-002068-PIP01-16

Influenza / Prevention of influenza

Day 90 discussion

Vaccines

3.1.5. EMEA-001749-PIP02-16

Treatment of Atopic Dermatitis

Day 60 discussion

Dermatology

3.1.6. recombinant humanised IgG4 monoclonal antibody against MSRV-Envelope protein - EMEA-002127-PIP01-17

Treatment of Multiple Sclerosis (RRMS), Treatment of Multiple Sclerosis (PMS), Treatment of patients from 10 to less than 18 years old with relapsing-remitting multiple sclerosis

Day 60 discussion

Neurology

3.1.7. trazodone hydrochloride - EMEA-002142-PIP01-17

Treatment of insomnia

Day 60 discussion

Neurology

3.1.8. Sulindac / Eflornithine - Orphan - EMEA-001518-PIP02-16

Cancer Prevention Pharma Ltd.; Treatment of Familial Adenomatous Polyposis

Day 60 discussion

Oncology

3.1.9. Lactobacillus reuteri - Orphan - EMEA-001895-PIP01-15

Infant Bacterial Therapeutics AB; Prevention of necrotising enterocolitis

Day 60 discussion

Other / Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.10. EMEA-002148-PIP01-17

Treatment of venous and mixed (venous/arterial) leg ulcers

Day 30 discussion

Cardiovascular Diseases

3.1.11. macitentan - Orphan - EMEA-001032-PIP02-17

Actelion Registration Ltd.; Chronic Thromboembolic Pulmonary Hypertension (CTEPH)

Day 30 discussion

Cardiovascular Diseases

3.1.12. Methacholine Chloride - EMEA-002120-PIP01-17

Diagnosis of asthma

Day 30 discussion

Diagnostic

3.1.13. Empagliflozin - EMEA-000828-PIP05-17

Prevention of cardiovascular events in patients with chronic heart failure

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.1.14. Recombinant protein derived from the saliva of Ornithodoros moubata tick - EMEA-002100-PIP01-16

Atypical haemolytic uraemic syndrome

Day 30 discussion

Haematology-Hemostaseology

3.1.15. Recombinant protein derived from the saliva of Ornithodoros moubata tick - Orphan - EMEA-002100-PIP02-16

Akari Therapeutics plc; Paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Haematology-Hemostaseology

3.1.16. tocilizumab - EMEA-000309-PIP04-17

Treatment of SSc (ICD 10-M34)/scleroderma and associated disorders (MedDRA). / Treatment of juvenile Systemic Sclerosis (jSSc) in children 5 years of age and older.

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.17. Cefiderocol - EMEA-002133-PIP01-17

Treatment of Gram-negative bacterial infections

Day 30 discussion

Infectious Diseases

3.1.18. 5-[4-[2-(5-(1-hydroxyethyl)-2-pyridinyl)ethoxy]benzyl]-2,4-thiazolidinedione hydrochloride - Orphan - EMEA-002106-PIP01-16

Minoryx Therapeutics SL; Treatment of adrenoleukodystrophy / Treatment of X-linked adrenoleukodystrophy

Day 30 discussion

Neurology

3.1.19. Adeno-associated viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA - Orphan - EMEA-002122-PIP02-17

LYSOGENE; Mucopolysaccharidosis type IIIA

Day 30 discussion

Neurology

3.1.20. H-Tyr-Gly-Arg-Lys-Lys-Arg-Arg-Gln-Arg-Arg-Arg-Lys-Leu-Ser-Ser-Ile-Glu-Ser-Asp-Val-OH (YGRKKRRQRRRKLSSIESDV) - EMEA-002108-PIP01-16

Acute Ischemic Stroke (AIS) in adult subjects with a large intracranial arterial occlusion, a small ischemic core, and good collaterals.

Day 30 discussion

Neurology

3.1.21. Recombinant humanized anti-alpha-synuclein IgG1 monoclonal antibody - EMEA-002137-PIP01-17

treatment of Parkinson's disease (in adults)

Day 30 discussion

Neurology

3.1.22. Entrectinib - Orphan - EMEA-002096-PIP01-16

Ignyta, Inc.; 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 primary brain tumours with NTRK1/2/3, ROS1 or ALK gene fusions, Treatment of extracranial solid tumours with NTRK1/2/3, ROS1 or ALK gene fusions

Day 30 discussion

Oncology

3.1.23. OSIMERTINIB MESYLATE - EMEA-002125-PIP01-17

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 30 discussion

Oncology

3.1.24. Pevonedistat - EMEA-002117-PIP01-17

Acute Myeloid Leukemia (AML), Myelodysplastic Syndromes (MDS) / The treatment of paediatric patients with relapsed or refractory (R/R) MDS (including juvenile myelomonocytic leukemia), The treatment of paediatric patients with relapsed or refractory (R/R) AML.

Day 30 discussion

Oncology

3.1.25. Pexastimogene devacirepvec - Orphan - EMEA-002124-PIP01-17

Transgene S.A.; Treatment of hepatocellular carcinoma. (MedDra PT: 10073071)

Day 30 discussion

Oncology

3.1.26. daxibotulinumtoxinA - EMEA-002149-PIP01-17

Treatment for temporary improvement in the appearance of moderate to severe glabellar lines with corrugator and/or procerus muscle activity in adults.

Day 30 discussion

Other

3.1.27. Diclofenac sodium - EMEA-002132-PIP01-17

Symptomatic relief of pain associated with osteoarthritis, Symptomatic relief of mild to moderate pain and inflammation / Indicated for the symptomatic relief of pain associated with osteoarthritis in superficial joints, including the knee. For the local symptomatic relief of mild to moderate pain and inflammation following acute blunt trauma of small and medium-sized joints and periarticular structures, such as trauma of the tendons, ligaments, muscles and joints e.g. due to sprains and strains.

Day 30 discussion

Pain

3.1.28. EMEA-002121-PIP01-17

Treatment of insomnia / Treatment of attention deficit hyperactivity disorder (ADHD)-related insomnia

Day 30 discussion

Psychiatry

3.1.29. Recombinant Clostridium difficile Toxoid B / Recombinant Clostridium difficile Toxoid A - EMEA-002112-PIP01-16

Prevention of Clostridium difficile infection (CDI) / Active immunization for the prevention of primary Clostridium difficile infection in children and adolescents 2 to 18 years of age

Day 30 discussion

Vaccines

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. Brentuximab vedotin - EMEA-C2-000980-PIP01-10-M04

Takeda Pharma A/S; Treatment of anaplastic large cell lymphoma

Day 12 letter

Oncology

Summary of committee discussion:

The PDCO discussed the completed study and considered that it is compliant with the latest Agency's Decision (P/0211/2016) of 12 August 2016.

The PDCO finalised 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.

3.2.2. Clostridium Botulinum neurotoxin type A (150 kD), free from complexing proteins - EMEA-C1-001039-PIP01-10-M02

Merz Pharmaceuticals GmbH; Treatment of muscle spasticity

Day 30 letter

Neurology

Summary of committee discussion:

The PDCO discussed the completed study and considered that this is compliant with the latest Agency's Decision (P/0157/2016) of 15 June 2016.
The PDCO finalised this partially completed compliance procedure at Day 30 and confirmed the compliance of the study.

3.2.3. Tofacitinib - EMEA-C1-000576-PIP03-12

Pfizer Limited; Treatment of Ulcerative Colitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.2.4. Dolutegravir (DTG) / Rilpivirine (RPV) - EMEA-C1-001750-PIP01-15-M01

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

Day 30 letter

Infectious Diseases

Summary of committee discussion:

The following 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/0028/2017) of 10 February 2017.
The PDCO finalised on 19 May 2017 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.

3.2.5. Human normal immunoglobulin - EMEA-C1-001797-PIP01-15

Octapharma Pharamzeutika Produktionsges.m.b.H; Treatment of primary immunodeficiency

Day 30 discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.2.6. Ibrutinib - EMEA-C1-001397-PIP03-14-M02

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

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. apixaban - EMEA-000183-PIP01-08-M05

Bristol-Myers Squibb / Pfizer EEIG; Prevention of arterial thromboembolism, Prevention of venous thromboembolism / Prevention of venous thromboembolism (VTE) in paediatric subjects (1 to <18 years old) with a newly diagnosed acute lymphoblastic leukemia (ALL) or lymphoma (T or B cell), a functioning central venous access device (CVAD) and receiving PEG L-asparaginase during chemotherapy induction. Prevention of TE in paediatric patients (birth to below 18 years old) with cardiac disease.

Day 30 discussion

Cardiovascular Diseases

3.3.2. Allantoin - Orphan - EMEA-001590-PIP01-13-M04

Scioderm, Inc.; Treatment of epidermolysis bullosa

Day 30 discussion

Dermatology

3.3.3. Asfotase alfa - Orphan - EMEA-000987-PIP01-10-M03

Alexion Europe SAS; Hypophosphatasia (E83.38) / Treatment of hypophosphatasia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Canagliflozin - EMEA-001030-PIP01-10-M07

Janssen-Cilag International NV; Type 2 Diabetes Mellitus / Treatment of Type 2 Diabetes Mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Liraglutide - EMEA-000128-PIP01-07-M08

Novo Nordisk A/S; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. semaglutide - EMEA-001441-PIP02-15-M01

Novo Nordisk; Type 2 Diabetes Mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M02

AstraZeneca AB; Treatment of Hyperkalaemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.8. sotagliflozin - EMEA-001517-PIP01-13-M01

sanofi-aventis R&D; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.9. sotagliflozin - EMEA-001517-PIP02-14-M01

sanofi-aventis R&D; Treatment of type 1 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.10. Obeticholic Acid (6 alpha-ethylchenodeoxycholic acid) - Orphan - EMEA-001304-PIP02-13-M03

Intercept Pharma Ltd.; Primary Biliary Cirrhosis (PBC) / Biliary Atresia

Day 30 discussion

Gastroenterology-Hepatology

3.3.11. Coagulation Factor VIIa (Recombinant) - EMEA-001203-PIP02-14-M02

LFB SA; Treatment of congenital coagulation disorders, Treatment of acquired haemophilia / Treatment of bleeding and prevention of bleeding in those undergoing surgery or invasive

procedures in patients with haemophilia A or B with inhibitors to Factors VIII or IX, Treatment of bleeding and prevention of bleeding in those undergoing surgery or invasive procedures in patients with acquired haemophilia

Day 30 discussion

Haematology-Hemostaseology

3.3.12. Damoctocog alfa pegol - Orphan - EMEA-001229-PIP01-11-M03

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

Day 30 discussion

Haematology-Hemostaseology

3.3.13. Luspatercept - Orphan - EMEA-001521-PIP01-13-M01

Celgene Europe Ltd; Treatment of myelodysplastic syndromes, Treatment of beta-thalassaemia, Treatment of anaemia in patients with b-thalassaemia intermedia and major

Day 30 discussion

Haematology-Hemostaseology

3.3.14. Recombinant fusion protein linking coagulation factor IX with albumin - Orphan - EMEA-001107-PIP01-10-M03

CSL Behring GmbH; Treatment of hereditary factor IX deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3.15. Recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP) - Orphan - EMEA-001886-PIP01-15-M01

CSL Behring GmbH; Treatment of Haemophilia B, Treatment of Haemophilia A / Treatment of Haemophilia B with Inhibitors, Treatment of Haemophilia A with Inhibitors

Day 30 discussion

Haematology-Hemostaseology

3.3.16. Recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP;) - Orphan - EMEA-001886-PIP02-15-M01

CSL Behring GmbH; Treatment of congenital Factor VII Deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3.17. Romiplostim - Orphan - EMEA-000653-PIP01-09-M05

Amgen Europe B.V.; Treatment of disease-related thrombocytopenia in myelodysplastic syndrome, Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura) / Treatment of chronic immune thrombocytopenia (idiopathic thrombocytopenic purpura; ITP) in paediatric patients who are refractory or intolerant to other treatments (e.g., glucocorticosteroids, immunoglobulins, splenectomy)

Day 30 discussion

Haematology-Hemostaseology

3.3.18. Treosulfan - Orphan - EMEA-000883-PIP01-10-M04

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

Day 30 discussion

Immunology-Rheumatology-Transplantation / Oncology

3.3.19. Lamivudine (3TC) / Abacavir (ABC) / Dolutegravir (DTG) - EMEA-001219-PIP01-11-M03

ViiV Healthcare UK Limited; Treatment of Human Immunodeficiency Virus (HIV-1) infection / Treatment Human Immunodeficiency Virus (HIV-1) infection in paediatric population

Day 30 discussion

Infectious Diseases

3.3.20. Tenofovir Alafenamide / Emtricitabine - EMEA-001577-PIP02-14-M02

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

Day 30 discussion

Infectious Diseases

3.3.21. Tenofovir Alafenamide / Emtricitabine / Cobicistat / Elvitegravir - EMEA-001460-PIP01-13-M02

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection / indicated for the treatment of HIV-1 infection in paediatric patients from 6 years to less than 18 years.

Day 30 discussion

Infectious Diseases

3.3.22. zanamivir - EMEA-001318-PIP01-12-M02

GlaxoSmithKline Trading Services Limited; Treatment of influenza, Prevention of influenza / Treatment of influenza A and B virus infection, Prevention of influenza A and B virus infection

Day 30 discussion

Infectious Diseases

3.3.23. Bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein - EMEA-001793-PIP01-15-M02

Bristol-Myers Squibb International Corporation; Treatment of Duchenne Muscular Dystrophy / Treatment of Duchenne Muscular Dystrophy in patients from 2 to less than 18 years of age

Day 30 discussion

Neurology

3.3.24. Brentuximab vedotin - Orphan - EMEA-000980-PIP01-10-M05

Takeda Pharma A/S; Treatment of Hodgkin lymphoma / Treatment of paediatric patients with newly diagnosed, relapsed or refractory Hodgkin lymphoma (from 5 years of age)

Day 30 discussion

Oncology

3.3.25. decitabine - Orphan - EMEA-000555-PIP01-09-M06

Janssen-Cilag International NV; Treatment of acute myeloid leukemia / Treatment of paediatric patients with acute myeloid leukaemia who have high-risk cytogenetics, or are refractory to, or have a relapse after first-line treatment

Day 30 discussion

Oncology

3.3.26. Isopropyl Alcohol / Chlorhexidine Gluconate - EMEA-000989-PIP01-10-M02

3M Health Care Limited; Prevention of infection

Day 30 discussion

Other

3.3.27. ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M06

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 discussion

Other

3.3.28. Recombinant Varicella Zoster Virus (VZV) glycoprotein E - EMEA-001426-PIP01-13-M01

GlaxoSmithKline Biologicals SA; Prevention of Varicella Zoster Virus reactivation / Prevention of herpes zoster in immunocompromised subjects aged 1 to 17 years

Day 30 discussion

Vaccines

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 18 July 2017 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

4.3.1. Call for volunteers for the Paediatric addendum to CHMP guidelines on the clinical investigations of Venous thromboembolism (VTE)

Summary of committee discussion:

The PDCO nominated 2 members to contribute to the Paediatric addendum to CHMP guidelines on the clinical investigation of venous thromboembolism (VTE), Angeliki Siapkara and Jaroslav Sterba.

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

None

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. rilpivirine (as hydrochloride) - EMEA-000317-PIP01-08-M09

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

Proposed indication: Maintenance of virologic suppression in HIV-1-infected patients without evidence of prior virologic failure on ARV therapy, in combination with cabotegravir long acting

Summary of committee discussion:

The planned new indication is considered to fall within the scope of the PIP.

However, any new pharmaceutical form or route of administration would not be covered by the current PIP 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

None

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 PDCO members were presented the list of procedures with paediatric indications to be evaluated by the CHMP, starting in April 2017.

The members were also informed about 4 medicinal products, Brineura, Cuprior, Erelzi and Celsentri for which the CHMP adopted a positive opinion recommending a paediatric indication during their meeting in April 2017. A new pharmaceutical form (20 mg/ml oral solution) and 2 new strenghts of film-coated tablets (25 mg and 75 mg) for Celsentri were approved to include paediatric use from 2 to less than 18 years of age.

Joint CHMP/PDCO session

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

9.3.1. Non-clinical Working Group: D30 Products identified and Election of the Non-clinical Working Group Chair

Summary of committee discussion:

The PDCO elected Karen Van Malderen as new chairperson for their Non-clinical Working Group by consensus.

The main task of the Non-clinical Working Group is to advise the PDCO on pre-clinical issues and requirements in PIPs.

9.3.2. 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.

9.3.3. Paediatric Addendum to the guideline on the evaluation of medicinal products indicated for treatment of bacterial infections

PDCO member: Maria Fernandez Cortizo

Summary of committee discussion:

An updated proposal of the paediatric addendum to the guideline on antibacterial agents was presented to the committee.

The committee discussed that the collection of safety data post-authorizations should be encouraged and concrete proposals discussed when granting the paediatric marketing

authorisation. It was also stressed that paediatric antibiotic studies are global studies. Therefore requirements for paediatric development should be discussed with FDA.

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:

On 16 May 2017 PDCO members were invited to attend the annual workshop of Enpr-EMA.

9.5. Cooperation with International Regulators

9.5.1. Gaucher disease - A strategic collaborative approach from EMA and FDA

PDCO member: Sylvie Benchetrit

Summary of committee discussion:

The discussion on the paper was postponed.

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

9.8.1. Report from the Strategic Review and Learning Meeting held in Malta on 11-12 April 2017

PDCO Chair: Dirk Mentzer; PDCO member: Herbert Lenicker

Summary of committee discussion:

Brief report from the Strategic Review and learning Meeting was presented to the PDCO.

10. Any other business

10.1.1. Preparedness of the system and capacity increase

Summary of committee discussion:

PDCO noted a presentation on the preparedness of the system and the capacity increase.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The break-out session was not held.

11.1.2. Neonatology

Summary of committee discussion:

The break-out session focused on Guidelines.

11.1.3. Inventory

Summary of committee discussion:

The inventory group convened to progress on the development of a new methodology and focused on how to define unmet needs and how to prioritise them.

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 16-19 May 2017 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 on: When not chairing the meeting: No participation in final deliberations and voting on:	EMEA-001426-PIP01-13-M01 EMEA-001749-PIP02-16 EMEA-001318-PIP01-12-M02 EMEA-001765-PIP02-15-M01 EMEA-C1-000069-PIP04-13-M01 EMEA-002111-PIP01-17 EMEA-002110-PIP01-17 EMEA-002111-PIP02-17 EMEA-002110-PIP02-17 EMEA-000431-PIP01-08-M10
Karen Van Malderen	Alternate	Belgium	No interests declared	
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	
Jaroslav Sterba	Member	Czech Republic	No interests declared	
Marianne Orholm	Member	Denmark	No interests	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
			declared	
Irja Lutsar	Member	Estonia	No interests declared	
Ann Marie Kaukonen	Member	Finland	No interests declared	
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	
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Alessandro Jenkner	Alternate	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	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	
Marek Migdal	Member	Poland	No interests	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
			declared	
Helena Fonseca	Member	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	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	
Eva Agurell	Alternate	Sweden	No restrictions applicable to this meeting	
Angeliki Siapkara	Member	United Kingdom	No interests declared	
Martina Riegl	Alternate	United Kingdom	No interests declared	
Riccardo Riccardi	Member	Healthcare Professionals' Representative	No participation in discussion, final deliberations and voting on:	EMEA-001094-PIP01-10-M04
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	
Antje Neubert	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
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	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Paola Baiardi	Alternate	Patients' Organisation Representative	No participation in final deliberations and voting on:	EMEA-001426-PIP01-13-M01 EMEA-001749-PIP02-16 EMEA-001318-PIP01-12-M02 EMEA-001765-PIP02-15-M01 EMEA-C1-000069-PIP04-13-M01 EMEA-002111-PIP01-17 EMEA-002110-PIP01-17 EMEA-002111-PIP02-17 EMEA-002110-PIP02-17 EMEA-000431-PIP01-08-M10
Tsvetana Schyns-Liharska	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Juliana Min	Expert - in person*	United Kingdom	No interests declared	
Kristine Moll Harboe	Expert - in person*	Denmark	No interests declared	
Jacqueline Carleer	Expert - in person*	Belgium	No interests declared	
Sara Homer	Expert - in person*	United Kingdom	No interests declared	
Eleni Gaki	Expert - in person*	United Kingdom	No interests declared	
Meeting run with support from relevant EMA staff				

* Experts were only evaluated against the product(s) they have been invited to talk about.

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/