12 October 2016
EMA/PDCO/612683/2016
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes for the meeting on 14-16 September 2016

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

14 September 2016, 08:30- 19:00, room 3A

15 September 2016, 08:30- 19:00, room 3A

16 September 2016, 08:30- 13:00, room 3A

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. Introduction

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 post-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 were adopted with amendments 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. Ascorbic Acid / Sodium Ascorbate / Potassium Chloride / Sodium Chloride / Sodium Sulfate / Macrogol 3350 - EMEA-001705-PIP02-15

Norgine Ltd.; Diagnosis of large intestine disorders / For bowel cleansing prior to any clinical procedures requiring a clean bowel e.g. bowel endoscopy or radiology

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

It was agreed that the condition of the PIP should be worded "bowel cleansing prior to clinical procedures". The PDCO confirmed the outcome of the Day 90 discussion and adopted a

positive opinion.

2.1.2. Monoclonal IgG1 anti-influenza A antibody - EMEA-001831-PIP01-15

Roche Products Limited; Treatment of influenza / Treatment of patients hospitalised with severe influenza A virus infection

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

In conclusion the PDCO recommended granting a paediatric investigation plan for all subsets of the paediatric population (from birth to less than 18 years of age) and a deferral for the condition 'treatment of influenza'.

2.1.3. Fremanezumab - EMEA-001877-PIP01-15

Teva Pharma GmbH; Episodic Migraine, Chronic Migraine / Prophylaxis of headache in children aged 12 to 18 years with chronic migraine, Prophylaxis of headache in children aged 6 to 18 years with episodic migraine

Day 120 opinion

Neurology

Summary of committee discussion:

The PDCO agrees with the applicant's request for a waiver. The PDCO adopted a positive opinion on PIP with deferral and waiver.

2.1.4. Humanised chimeric antibody with a humanised H chain and a chimeric (mouse V-domain, human C-domain) L chain against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F - Orphan - EMEA-001732-PIP02-15

AbbVie Ltd; Treatment of high-grade glioma / Treatment of high-grade glioma.

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO discussed on 14 September 2016 the paediatric investigation plan proposed for ABT-414 for high-grade glioma, taking into account comments by the PDCO non-clinical working group experts on the non-clinical development and comments by the applicant on drafts of the Opinion.
The PDCO agreed a PIP for ABT-414.

2.1.5. Birch pollen extract (Betula verrucosa) - EMEA-001879-PIP01-15

ALK Abelló A/S; Treatment of allergic rhinitis / rhino-conjunctivitis / Treatment of tree pollen allergic rhinitis and / or conjunctivitis

Day 120 opinion

Pneumology - Allergology

Summary of committee discussion:

The committee's view expressed on day 90 was re-discussed and endorsed.
Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO adopted a positive opinion for the Birch pollen extract (Betula verrucosa) in the condition of Treatment of allergic rhinitis / rhino-conjunctivitis.

2.1.6. 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-001894-PIP01-15

NDA Regulatory Service GmbH; Prevention of influenza

Day 120 opinion

Vaccines

Summary of committee discussion:

The PDCO re-discussed the application for Seqirus quadrivalent vaccine taking also into account the additional clarifications and the comments on the draft opinion provided by the applicant after the D90 discussion.
In conclusion the PDCO recommended granting a paediatric investigation plan for Seqirus quadrivalent vaccine and waiver for the age subset from birth to less than 6 months of age and a deferral in the condition 'prevention of influenza'.

2.1.7. Amlodipine / Candesartan - EMEA-002014-PIP01-16

CIPROS S.r.l.; Hypertension

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 and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO adopted a positive opinion granting a waiver for Amlodipine / Candesartan for all subsets of the paediatric population (0 to less than 18 years of age) in the condition "treatment of hypertension".

2.1.8. Amlodipine / Perindopril - EMEA-001968-PIP01-16

ERREKAPPA EUROTERAPICI S.p.A.; Hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO deemed the request for waiver acceptable.

2.1.9. Hydrochlorothiazide / Valsartan / Amlodipine - EMEA-002006-PIP01-16

ELPEN Pharmaceutical Co. Inc; Essential hypertension / Treatment of essential hypertension as substitution therapy in adult patients whose blood pressure is adequately controlled on the combination of amlodipine, valsartan and hydrochlorothiazide (HCT), taken either as three single-component formulations or as a dual-component and a single-component formulation

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 and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO adopted a positive opinion granting a waiver for Amlodipine / Hydrochlorothiazide / Valsartan for all subsets of the paediatric population (0 to less than 18 years of age) in the condition of treatment of hypertension.

2.1.10. Atorvastatin / Amlodipine - EMEA-002005-PIP01-16

ELPEN Pharmaceutical Co. Inc; Treatment of concomitant angina and dyslipidaemia, Prevention of cardiovascular events in hypertensive patients and diabetes mellitus type 2 patients with multiple risk factors for cardiovascular disease, Treatment of concomitant hypertension and dyslipidaemia / Substitution therapy in patients already taking concomitantly amlodipine and atorvastatin mono-products for the management of: Concomitant hypertension and dyslipidaemia

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 the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for amlodipine / atorvastatin for all subsets of the paediatric population (0 to 18 years of age) in the conditions of:

- Prevention of cardiovascular events in hypertension and diabetes mellitus type 2
- Treatment of concomitant angina and dyslipidaemia
- Treatment of concomitant hypertension and dyslipidaemia

2.1.11. Sirukumab - EMEA-001043-PIP02-16

Janssen-Cilag International N.V.; Adults: Giant Cell Arteritis, Children: Paediatric vasculitides / N.A., Treatment of vasculitides

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

Based on the assessment of this application the PDCO at their September meeting agreed with the applicant's request for a waiver. The PDCO recommends granting a waiver for sirukumab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of vasculitides.
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. Ciclosporin - EMEA-001998-PIP01-16

Drug Delivery Solutions ApS; Dry eye disease/Keratoconjuntivitis Sicca

Day 60 opinion

Ophthalmology

Summary of committee discussion:

A positive opinion was adopted.
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 Ciclosporin for all subsets of the paediatric population (0 to 18 years of age) in the condition of Dry eye disease.

2.1.13. Allogeneic Mesenchymal Precursor Cells (Rexlemestrocel-L) - EMEA-001140-PIP02-15

Mesoblast UK Limited; Disc degeneration disease

Day 60 opinion

Other

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Allogeneic Mesenchymal Precursor Cells (Rexlemestrocel-L) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of intervertebral disc disorder.
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.

2.2. 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. ertugliflozin - EMEA-C1-001533-PIP01-13

MSD (Europe) Inc.; Treatment of type II diabetes mellitus

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO considered that the completed PIP study 1 is compliant with the latest Agency's Decision (P/0214/2014) of 01/09/2014.
The PDCO finalised on 16 September 2016 this partially completed compliance procedure.

2.2.2. exenatide - EMEA-C1-000689-PIP01-09-M06

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO considered that the completed PIP studies are compliant with the latest Agency's Decision (P/0130/2016) of 20/05/2016.
The PDCO finalised on 16 September 2016 this partially completed compliance procedure.

2.2.3. Sofosbuvir / ledipasvir - EMEA-C1-001411-PIP01-12-M03

Gilead Sciences International Ltd.; Treatment of chronic hepatitis C

Day 30 opinion

Infectious Diseases

Summary of committee discussion:

At D60, the PDCO discussed the completed study and considered that this is compliant with the latest Agency's Decision (P/0174/2016) of 30 June 2016.
In conclusion, the following completed studies were checked for compliance:
The PDCO considered that these are compliant with the latest Agency's Decision (P/0174/2016) of 30 June 2016.
The PDCO finalised on 16 September 2016 this partially completed compliance procedure.

2.2.4. rufinamide - EMEA-C-000709-PIP01-09-M05

Eisai Limited; Treatment of Lennox-Gastaut Syndrome

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO took note of preceding procedure and report on partially completed compliance (EMEA-C3-000709-PIP01-09-M05).

The PDCO adopted on 16 September 2016 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0116/2016) of 15 April 2016.

2.2.5. Secukinumab - EMEA-C2-000380-PIP02-09-M03

Novartis Europharm Ltd; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed the completed Study, and considered that it is compliant with the latest Agency's Decision (P/0168/2016) of 17 June 2016.
The PDCO finalised on 16 September 2016 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.

2.2.6. Tralokinumab (INN) - EMEA-C1-000782-PIP01-09-M03

MedImmune Ltd; Treatment of asthma

Day 30 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO considered that the completed PIP study is compliant with the latest Agency's Decision (P/0099/2016) of 15/04/2016.
The PDCO finalised on 16 September 2016 this partially completed compliance procedure.

2.2.7. Recombinant Human TriPeptidyl Peptidase 1 (rhTPP1) - Orphan - EMEA-C3-001362-PIP01-12-M03

BioMarin International Limited; Neuronal Ceroid Lipofuscinosis Type 2 (NCL2) / Treatment of Neuronal Ceroid Lipofuscinosis Type 2 (NCL2)

Action: For information; compliance report adopted via written procedure on 8 September 2016

Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Alipogene Tiparvovec - Orphan - EMEA-000292-PIP01-08-M03

uniQure biopharma B.V.; Hyperchylomicronaemia

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/205/2013 of 3 September 2013).
The new PDCO Opinion on the acceptance of a modification of an agreed PIP and on the granting of a product-specific waiver supersedes the previous PDCO Opinion.

2.3.2. riociguat - Orphan - EMEA-000718-PIP01-09-M06

Bayer Pharma AG; I27.2 Other secondary pulmonary hypertension, I27.0 Primary pulmonary hypertension / Treatment of drug and toxin-induced pulmonary arterial hypertension, Treatment of pulmonary hypertension with unclear multifactorial mechanisms, Treatment of pulmonary veno-occlusive disease (PVOD) and/or pulmonary capillary hemangiomatosis (PCH), Treatment of pulmonary hypertension due to lung disease and /or hypoxia, Treatment of chronic thromboembolic pulmonary hypertension (CTEPH), Treatment of pulmonary hypertension owing to left heart diseases, Treatment of pulmonary arterial hypertension (PAH)

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0036/2015 of 06/03/2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. serelaxin - EMEA-001168-PIP01-11-M03

Novartis Europharm Limited; Treatment of Acute Heart Failure / Treatment of acute heart failure following surgical repair of a congenital heart defect

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0333/2014 of 22 December 2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. dabigatran etexilate mesilate - EMEA-000081-PIP01-07-M09

Boehringer Ingelheim International GmbH; Treatment of thromboembolic events, Prevention

of thomboembolic events /Treatment of venous thromboembolic events in paediatric patients (secondary venous thrombotic event prevention)

Day 60 opinion

Cardiovascular Diseases / Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0057/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M01

ZS Pharma, Inc; Hyperkalaemia / Treatment of Hyperkalaemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO agreed with the applicant's response.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0210/2014 of 12/08/2014).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Tolvaptan - EMEA-001231-PIP02-13-M04

Otsuka Pharmaceutical Europe Ltd.; Polycystic Kidney Disease (PKD), Dilutional hyponatraemia / Treatment of chronic (>48 hours) dilutional hyponatraemia resistant to fluid restriction (i.e., euvolemic and hypervolemic hyponatremia) associated with heart failure, cirrhosis or SIADH, Treatment of progression of ADPKD, Treatment of progression of ARPKD

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could not be accepted.
The PDCO therefore adopted a negative Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0161/2016 of 15 June 2016).

2.3.7. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene - Orphan - EMEA-001665-PIP01-14-M01

bluebird bio France; β-thalassaemia

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0257/2015 of 30/10/2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Human normal immunoglobulin for subcutaneous use - EMEA-000454-PIP01-08-M07

Kedrion S.p.A.; D80-D90 Certain disorders involving the immune mechanism. Primary Immunodeficiency Syndromes / Treatment of Primary Immunodeficiency Syndromes

Day 60 opinion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

Summary of committee discussion:

At their September 2016 meeting based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted and supported a waiver for the all subsets of the paediatric population.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0113/2015 of 7 June 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. ataluren - Orphan - EMEA-000115-PIP01-07-M08

PTC Therapeutics International, Limited; Treatment of dystrophinopathy ICD-10: G71.0 Muscular dystrophy [of Duchenne and Becker] / Treatment of nonsense-mutation dystrophinopathy

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO's view expressed at day 30 was re-discussed taking into account the applicant's clarifications.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the some, but not all 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/0122/2016 of 29 April 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. eteplirsen - Orphan - EMEA-001722-PIP01-14-M01

Sarepta International C.V.; Duchenne muscular dystrophy

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that 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/0029/2016 of 29 January 2016)
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Olaratumab - Orphan - EMEA-001760-PIP01-15-M01

Eli Lilly and Company Limited; Treatment of Soft Tissue Sarcoma, Treatment of Osteosarcoma / Treatment of recurrent rhabdomyosarcoma in children aged from birth to less than 18 years in combination with a standard-of-care chemotherapy regimen, First-line treatment of osteosarcoma in children aged from 5 to 18 years in combination with a standard-of-care chemotherapy regimen.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed on 15 September 2016 the request for modification of the PIP agreed for olaratumab, targeting a paediatric use for treatment of rhabdomyosarcoma (in all age ranges) and osteosarcoma.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision. Drafts of the Opinion had been shared with the applicant.

2.3.12. Cenegermin - Orphan - EMEA-001729-PIP01-14-M01

Dompé farmaceutici S.p.A.; Neurotrophic Keratitis

Day 60 opinion

Ophthalmology

Summary of committee discussion:

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

2.3.13. Xylitol / Procaine hydrochloride / Magnesium sulphate heptahydrate / Potassium chloride - EMEA-001171-PIP01-11-M01

MIT Gesundheit GmbH; Cardioplegia / Induction of immediate and prolonged diastolic cardiac arrest in open heart surgery

Day 60 opinion

Other

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Human thrombin / Human fibrinogen - EMEA-001340-PIP01-12-M02

Mallinckrodt Pharmaceuticals; Treatment of haemorrhage resulting from a surgical procedure / Supportive treatment where standard surgical techniques are insufficient for improvement of haemostasis

Day 60 opinion

Other / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO adopted a positive opinion on the modification of the agreed PIP.

2.3.15. ataluren - Orphan - EMEA-000115-PIP02-09-M03

PTC Therapeutics International, Limited; Cystic Fibrosis ICD10: E84.9 Cystic fibrosis, unspecified / Treatment of cystic fibrosis

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the some, but not all 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/0133/2015 of 12 June 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. mepolizumab - Orphan - EMEA-000069-PIP04-13-M01

GSK Trading Services Limited; Vasculitides / Treatment of paediatric patients aged 6 to 17 years with eosinophilic granulomatosis with polyangiitis (EGPA) using corticosteroid therapy with or without concomitant immunosuppressant therapy.

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0326/2014) of 22 December 2014.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. mirabegron - EMEA-000597-PIP02-10-M05

Astellas Pharma Europe B.V.; Treatment of idiopathic overactive bladder / Symptomatic treatment of urge incontinence and/or increased urinary frequency and urgency as may occur in patients with overactive bladder syndrome

Day 60 opinion

Uro-nephrology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0116/2015 of 5 June 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. mirabegron - EMEA-000597-PIP03-15-M02

Astellas Pharma Europe B.V.; Treatment of neurogenic detrusor overactivity / Treatment of detrusor overactivity in children and adolescents with neurogenic bladder dysfunction

Day 60 opinion

Uro-nephrology

Summary of committee discussion:

The PDCO confirmed the outcome of the Day 30 discussion and adopted a positive opinion.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0269/2015 of 27 November 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.4. Opinions on Re-examinations

2.4.1. Linaclotide - EMEA-000927-PIP01-10-M03

Allergan Pharmaceuticals International Limited; Functional Constipation / in children

Day 30 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO discussed this re-examination procedure. The clarifications and proposals from the applicant were considered acceptable and a positive opinion was adopted.

2.4.2. Angiotensin II - EMEA-001912-PIP01-15

La Jolla Pharmaceutical Company, Inc.; Treatment of Catecholamine-resistant hypotension associated with distributive shock.

Day 30 opinion

Other

Summary of committee discussion:

The re-examination request was discussed at the PDCO plenary meeting on 14 September. The PDCO reviewed and discussed the detailed grounds submitted by the applicant, along with the assessors' comments. The previous PDCO opinion is being maintained, thus the proposed PIP is refused.

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. alvimopan - EMEA-001922-PIP01-15

Prevention of postoperative ileus

Day 90 discussion

Gastroenterology-Hepatology

3.1.2. Naldemedine Tosylate - EMEA-001893-PIP01-15

Opioid-induced constipation (OIC)

Day 90 discussion

Gastroenterology-Hepatology

3.1.3. Antithrombin alfa - EMEA-001154-PIP02-15

Treatment of congenital antithrombin deficiency, Treatment of acquired antithrombin deficiency (Preeclampsia), Treatment of acquired antithrombin deficiency (ECMO) / Prophylaxis of peri-partum thromboembolic events in congenital antithrombin deficient patients., Antithrombin supplementation during ECMO procedure, Treatment of pregnant women less than 30 weeks GA with preeclampsia to prolong gestation and decrease foetal and neonatal morbidity and mortality.

Day 90 discussion

Haematology-Hemostaseology

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

CSL Behring GmbH; Treatment of congenital Haemophilia A or B

Day 90 discussion

Haematology-Hemostaseology

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

CSL Behring GmbH; Treatment of congenital Factor VII Deficiency

Day 90 discussion

Haematology-Hemostaseology

3.1.6. Galcanezumab - EMEA-001860-PIP03-16

Prophylactic treatment of migraine headache

Day 90 discussion

Neurology

3.1.7. inebilizumab - EMEA-001911-PIP01-15

Treatment of Neuromyelitis Optica (NMO) and Neuromyelitis Optica Spectrum Disorders (NMOSD)

Day 90 discussion

Neurology

3.1.8. 3,6-diamino-2,5-bis{N-[(1R)-1-carboxy-2-hydroxyethyl]carbamoyl}pyrazine - EMEA-001983-PIP01-16

Monitoring of renal function

Day 60 discussion

Diagnostic / Uro-nephrology

3.1.9. triheptanoin - Orphan - EMEA-001920-PIP02-16

Ultragenyx Pharmaceutical Inc.; Mitochondrial trifunctional protein (TFP) deficiency, Long-chain 3 hydroxyacyl-CoA dehydrogenase (LCHAD) deficiency, Carnitine palmitoyl transferase 2 (CPT-II) deficiency, Very long-chain acyl-CoA dehydrogenase (VLCAD) deficiency

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.10. Amiselimod - EMEA-001991-PIP01-16

Ulcerative colitis / Treatment of moderately to severely active ulcerative colitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.11. Cenicriviroc mesylate - EMEA-001999-PIP01-16

Treatment of non-alcoholic steatohepatitis (NASH) in subjects with liver fibrosis.

Day 60 discussion

Gastroenterology-Hepatology

3.1.12. Human fibrinogen concentrate - EMEA-001931-PIP01-16

Treatment of congenital fibrinogen deficiency

Day 60 discussion

Haematology-Hemostaseology

3.1.13. tazobactam / ceftolozane - EMEA-001142-PIP02-16

Treatment of abdominal and gastrointestinal infections, Treatment of urinary tract infections, Treatment of pneumonia / Treatment of nosocomial pneumonia, Treatment of complicated intra-abdominal infections (cIAI). Please refer to EMA decision (P/0126/2014) in relation to procedure EMEA-001142-PIP-01-11-M01., Treatment of complicated urinary tract infections (cUTI). Please refer to EMA decision (P/0126/2014) in relation to procedure EMEA-001142-PIP-01-11-M01.

Day 60 discussion

Infectious Diseases

3.1.14. acalabrutinib - Orphan - EMEA-001796-PIP03-16

ACERTA PHARMA, BV; Treatment of mature B cell neoplasms / Treatment of children from 1 to < 18 years of age with relapsed/refractory mature B-cell neoplasms (eg, diffuse large B-cell lymphoma [DLBCL], Burkitt lymphoma [BL] and primary mediastinal B-cell lymphoma [PMBCL]).

Day 60 discussion

Oncology

3.1.15. triheptanoin - Orphan - EMEA-001920-PIP01-15

Ultragenyx Pharmaceutical Inc.; glucose transporter type-1 deficiency syndrome

Day 60 discussion

Other

3.1.16. Orphan - EMEA-001984-PIP01-16

Retrophin Europe Limited; Treatment of Focal Segmental Glomerulosclerosis (FSGS) / Treatment of Focal Segmental Glomerulosclerosis (FSGS)

Day 60 discussion

Uro-nephrology

3.1.17. EMEA-001978-PIP01-16

Hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.18. Isopropyl Alcohol / Chlorhexidine Gluconate - EMEA-002011-PIP01-16

Prevention of infections associated with transcutaneous procedures

Day 30 discussion

Dermatology

3.1.19. Macimorelin - EMEA-001988-PIP01-16

Growth hormone deficiency / Diagnosis of growth hormone deficiency

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic

3.1.20. Orphan - EMEA-002023-PIP01-16

ChemoCentryx, Ltd.; Treatment of ANCA-associated vasculitis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.21. Atacicept - EMEA-002004-PIP01-16

Treatment of systemic lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.22. Recombinant humanised monoclonal antibody against human complement component C5a - EMEA-002009-PIP01-16

Treatment of acute Graft-versus-Host Disease

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.23. Synthetic double-stranded siRNA oligonucleotide directed against p53 mRNA - Orphan - EMEA-001993-PIP01-16

Quark Pharmaceuticals Inc.; Prevention of delayed graft function (DGF) after kidney transplantation / Prevention of DGF after transplantation of kidneys from deceased donors ≥ 45 years old

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.24. Terguride hydrogenmaleate - Orphan - EMEA-002015-PIP01-16

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of Systemic scleroderma

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.25. Orphan - EMEA-001960-PIP02-16

Catabasis Pharmaceuticals Inc.; Duchenne Muscular Dystrophy / Treatment of Duchenne Muscular Dystrophy

Day 30 discussion

Neurology

3.1.26. Fremanezumab - EMEA-001877-PIP02-16

Cluster Headache / Not applicable

Day 30 discussion

Neurology

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

Celator (UK) Ltd; Acute myeloid leukemia / Treatment

Day 30 discussion

Oncology

3.1.28. lenadogene nolparvovec - Orphan - EMEA-001992-PIP02-16

GENSIGHT-BIOLOGICS; Leber Hereditary Optic Neuropathy (LHON)

Day 30 discussion

Ophthalmology

3.1.29. Teprotumumab - EMEA-001973-PIP01-16

Active thyroid eye disease

Day 30 discussion

Ophthalmology

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

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

Day 30 discussion

Other

3.1.31. EMEA-001742-PIP02-16

prevention of psychosis / prevention of first episode of psychosis (FEP) in individuals with attenuated psychotic syndrome (APS)

Day 30 discussion

Psychiatry

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. dupilumab - EMEA-C1-001501-PIP01-13-M03

Regeneron Pharmaceuticals, Inc.; Treatment of atopic dermatitis

Day 30 discussion

Dermatology

3.2.2. Adalimumab - EMEA-C-000366-PIP05-12-M02

AbbVie Ltd; Treatment of non-infectious uveitis

Day 30 discussion

Immunology-Rheumatology-Transplantation / Ophthalmology / Dermatology / Gastroenterology-Hepatology

3.2.3. Ivacaftor - EMEA-C2-001582-PIP01-13

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 discussion

Other

3.2.4. lisdexamfetamine (dimesylate) - EMEA-C-000553-PIP01-09-M04

Shire Pharmaceutical Contracts Ltd; Treatment of attention Deficit Hyperactivity Disorder

(ADHD)

Day 30 discussion

Psychiatry

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. ambrisentan - Orphan - EMEA-000434-PIP01-08-M04

Glaxo Group Limited; Treatment of Pulmonary Arterial Hypertension / Idiopathic (IPAH) and Familial (FPAH) Pulmonary Hypertension; Associated Pulmonary Hypertension (APAH)

Day 30 discussion

Cardiovascular Diseases

3.3.2. Dobutamine - EMEA-001262-PIP01-12-M02

Proveca Limited; Circulatory impairment / haemodynamic insufficiency

Day 30 discussion

Cardiovascular Diseases

3.3.3. Empagliflozin - EMEA-000828-PIP01-09-M05

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. linagliptin (as base) - EMEA-000498-PIP01-08-M06

Boehringer Ingelheim International GmbH; Type 2 Diabetes Mellitus / Type 2 Diabetes Mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. migalstat hydrochloride - Orphan - EMEA-001194-PIP01-11-M02

Amicus Therapeutics UK Ltd; Fabry disease

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Semaglutide - EMEA-001441-PIP01-13-M01

Novo Nordisk A/S; Diabetes Mellitus type 2 / Treatment of Diabetes Mellitus type 2

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M02

Basilea Pharmaceutica International Ltd.; J15: Bacterial pneumoniae no elsewhere classified, J13: Pneumonia due to Streptococcus pneumoniae, J14: Pneumonia due to Hemophilus influenzae / Treatment of nosocomial pneumonia, Treatment of community acquired pneumonia

Day 30 discussion

Infectious Diseases

3.3.8. daclatasvir - EMEA-001191-PIP01-11-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of chronic viral hepatitis C / Daklinza is indicated in combination with sofosbuvir (SOF) for the treatment of CHC in children 3 years of age and older, and adolescents.

Day 30 discussion

Infectious Diseases

3.3.9. Eravacycline - EMEA-001555-PIP01-13-M02

Tetraphase Pharmaceuticals, Inc.; Complicated Intra-Abdominal Infection, Complicated Urinary Tract Infection / Complicated Intra-Abdominal Infection, Urinary Tract Infection

Day 30 discussion

Infectious Diseases

3.3.10. Telavancin hydrochloride - EMEA-000239-PIP01-08-M02

Clinigen Healthcare Ltd; Nosocomial Pneumonia (NP), Complicated skin and soft tissue infections (cSSTI) / Waiver, Nosocomial Pneumonia (NP)

Day 30 discussion

Infectious Diseases

3.3.11. Tenofovir alafenamide / Emtricitabine / Bictegravir - EMEA-001766-PIP01-15-M01

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

Day 30 discussion

Infectious Diseases

3.3.12. tenofovir disoproxil / emtricitabine / cobicistat / elvitegravir - EMEA-000970-PIP01-10-M01

Gilead Sciences International Ltd; B23 Human immunodeficiency virus disease [HIV] resulting in other conditions / Stribild indicated for the treatment of HIV-1 infection in paediatric patients aged 12 years and over.

Day 30 discussion

Infectious Diseases

3.3.13. Autologous haematopoietic stem cells transduced with lentiviral vector Lenti-D encoding the human ATP-binding cassette, sub-family D (ALD), member 1 (ABCD1) from cDNA - Orphan - EMEA-001244-PIP01-11-M01

bluebird bio France; Treatment of adrenoleukodystrophy

Day 30 discussion

Neurology

3.3.14. Delta-9-tetrahydrocannabinol / Cannabidiol - EMEA-000181-PIP01-08-M03

GW Pharma Ltd; Spasticity / Intractable spasticity due to cerebral palsy or traumatic CNS injury

Day 30 discussion

Neurology

3.3.15. Perampanel - EMEA-000467-PIP01-08-M08

Eisai Europe Limited; Treatment of treatment-resistant epilepsies / Adjunctive therapy in patients with other paediatric epilepsies, Adjunctive therapy in patients with refractory partial onset seizures including secondarily generalised seizures

Day 30 discussion

Neurology

3.3.16. Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19 - Orphan - EMEA-001654-PIP01-14-M01

Novartis Europharm Limited; B cell acute lymphoblastic leukaemia (ALL) / Treatment of B cell acute lymphoblastic leukaemia (ALL) in paediatric patients who have relapsed after at least two prior regimens or are refractory

Day 30 discussion

Oncology

3.3.17. Bosutinib - Orphan - EMEA-000727-PIP01-09-M02

Pfizer Limited; CML / Treatment of CML in children and adolescents (from 1 to <18 years of age) with resistance or intolerance to prior TKI therapy.

Day 30 discussion

Oncology

3.3.18. Eribulin - EMEA-001261-PIP01-11-M03

Eisai Europe Ltd; Soft Tissue Sarcoma / Treatment of non-Rhabdomyosarcoma soft tissue sarcoma, Treatment of Rhabdomyosarcoma

Day 30 discussion

Oncology

3.3.19. pixantrone - EMEA-000713-PIP02-10-M04

CTI Life Sciences Limited; ICD-09. C83 Diffuse Non-Hodgkin's Lymphoma (including C83.7 Burkitt Lymphoma, C83.5 Lymphoblastic Lymphoma, C83.3 Large-cell Lymphoma) / Treatment of Non-Hodgkin's Lymphoma

Day 30 discussion

Oncology

3.3.20. Sunitinib - EMEA-000342-PIP01-08-M05

Pfizer Limited; CD10 code C49.4 malignant neoplasms of connective and soft tissue of abdomen - gastro-intestinal stromal tumours (GIST) / Treatment of gastro-intestinal stromal tumour in paediatric patients aged 6 to less than 18

Day 30 discussion

Oncology

3.3.21. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M01

Pfizer Limited; Treatment of Acute Lympohoblastic Leukaemia / For the treatment of relapsed or refractory B cell precursor Acute Lympohoblastic Leukaemia

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.3.22. Bupropion HCl / Naltrexone HCl - EMEA-001373-PIP01-12-M02

Orexigen Therapeutics Ireland Limited; Treatment of obesity / Treatment of obesity

Day 30 discussion

Other

3.3.23. fentanyl hydrocholoride - EMEA-001509-PIP01-13-M01

Incline Therapeutics Europe Ltd. (a wholly owned subsidiary of The Medicines Company); Treatment of acute pain

Day 30 discussion

Pain

3.3.24. methoxyflurane - EMEA-000334-PIP01-08-M05

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 30 discussion

Pain

3.3.25. Tapentadol - EMEA-000018-PIP01-07-M12

Grünenthal GmbH; Acute pain / Treatment of acute pain

Day 30 discussion

Pain

3.3.26. Tapentadol - EMEA-000325-PIP01-08-M06

Grünenthal GmbH; Chronic pain / Treatment of chronic pain

Day 30 discussion

Pain

3.3.27. Loxapine - EMEA-001115-PIP01-10-M05

Ferrer Internacional, S.A.; Bipolar disorder, Schizophrenia / For rapid control of agitation in patients with schizophrenia, For rapid control of agitation in patients with bipolar disorder

Day 30 discussion

Psychiatry

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 29 November 2016 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. Bevacizumab - EMEA-27-2016 (EMEA-000056-PIP01-07-M02, EMEA-000056-PIP03-10-M02)

Roche Registration Limited; Treatment of mesothelioma / Bevacizumab in combination with pemetrexed and cisplatin is indicated for the first-line treatment of adult patients with unresectable malignant pleural mesothelioma

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.

Note: in case of removal from the list of class waivers listed in the Agency's Decision CW/1/2011, the requirements set out in Article 7 and 8 of Regulation (EC) No 1901/2006 of the Agency's Decision CW/0001/2015 shall apply after 36 months from the date of the removal from the list of class waivers.

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

None

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

9.1.1. Elections of PDCO Chair

Summary of committee discussion:

The PDCO was reminded of the 'Procedure for the election of the PDCO Chair, according to which the election was conducted.

The PDCO noted the candidature of Dirk Mentzer who had the opportunity to express in a short statement the motivation for which he was standing. Then, the PDCO proceeded with the election by secret ballot.

Dirk Mentzer was elected as Chair.

9.2. Coordination with EMA Scientific Committees or CMDh-v

None

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Jacqueline Carleer

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. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

Relevant products for FWG discussion were identified.

9.3.3. Inventory of paediatric therapeutic needs - Respiratory

Summary of committee discussion:

The final 'Inventory of paediatric therapeutic needs – Respiratory' was adopted for publication following the review of comments received during the public consultation phase. The Committee also adopted for publication a response document summarising the PDCO's review of the public comments.

9.3.4. Concept paper on the revision of the Guideline on the role of pharmacokinetics in the development of medicinal products in the paediatric population

Summary of committee discussion:

The Committee noted the concept paper which will be distributed to PDCO members for comments in the post-mail.

9.3.5. Report on the responses from the Vaccines Working Party (VWP) on Dengue vaccine PIP

PDCO member: Marta Granstrom

Summary of committee discussion:

The Committee noted the report.

9.3.6. Report of a joint EMA workshop with patient and healthcare professional representatives about communication on medicines held on 8 March 2016

Summary of committee discussion:

The PDCO noted the tabled document.

9.3.7. Minutes of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting held on 9 March 2016

Summary of committee discussion:

The PDCO noted the tabled document.

9.3.8. Agenda of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting – Workshop on social media held on 19 September 2016

Summary of committee discussion:

The PDCO noted the tabled document.

9.3.9. Draft Agenda of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting to be held on 20 September 2016

Summary of committee discussion:

The PDCO noted the tabled document.

9.3.10. Agenda and Minutes of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) meeting held on 14 June 2016

Action: Documents tabled for information

9.3.11. Agenda of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) 10th Anniversary meeting held on 14 June 2016

Summary of committee discussion:

The PDCO noted the tabled document.

9.3.12. Agenda and Minutes of the EMA Human Scientific Committees' Working Parties with Healthcare Professionals' Organisations (HCPWP) meeting held on 15 June 2016

Summary of committee discussion:

The PDCO noted the tabled document.

9.4. Cooperation within the EU regulatory network

9.4.1. European Commission (EC) launches call for expressions of interest for the EMA PDCO Committee: civil society representatives

Summary of committee discussion:

The Committee noted the published EC call for expressions of interest for the EMA PDCO Committee: civil society representatives (news item on EC website).

9.5. Cooperation with International Regulators

9.5.1. Addendum (R1) to International Council for Harmonisation (ICH E11) Guideline 'Clinical Investigation of Medicinal Products in Paediatric Population'

PDCO Chair: Dirk Mentzer

Summary of committee discussion:

The updated sections of the guideline are ethical considerations, age classification and paediatric subgroups and paediatric formulations. The new sections are commonality of scientific approach; approaches to optimize paediatric development (extrapolation + modelling and simulation); practicalities in the design and execution of pediatric clinical trials (feasibility, outcome assessments, long-term clinical aspects). The purpose of the new section of Commonality of scientific approach is to align Health Authorities regulatory processes, to reduce substantial differences among regions for the acceptance of data generated in pediatric global drug development programs and to ensure timely access to medicines for children. EMA and FDA have now harmonised their scientific approach to extrapolation. The global approach towards extrapolation is in line with the EMA strategy and activities developed over the past 2 years.

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

9.6.1. International Neonatal Consortium

Summary of committee discussion:

Following the Second Scientific Workshop of the International Neonatal Consortium (INC) hosted at the EMA on 12-13 September 2016 the Critical Path Institute presented the approaches as a private-public partnership to design consortia to support development of medicines in the paediatric population specifically using the examples of INC as a “pre-competitive” space and the Pediatric Trial Consortium (PTC). INC is a multi-stakeholder consortium including families/parents, nurses, neonatologists, researchers, industry and regulatory bodies to advance development of neonates by working on specific deliverables in areas of highest unmet need. Deliverables are being worked on in specific work groups and face to face workshops. The PTC was presented and discussed also in view of ongoing European activities.

9.7. PDCO work plan

9.7.1. PDCO Work Plan 2017

Summary of committee discussion:

Postponed to PDCO October 2016 to allow the drafting group to fine-tune activity areas in the draft document.

9.8. Planning and reporting

None

9.9. PDCO ORGAM

None

10. Any other business

10.1.1. EMA – internal organisational adjustments

Summary of committee discussion:

The PDCO noted the changes in EMA organisation.

10.1.2. Business Pipeline Report Q3 2016

Summary of committee discussion:

The PDCO noted the tabled document.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The participants discussed reports on recent public meetings and work on guidances concerning paediatric oncology.

11.1.2. Neonatology

Summary of committee discussion:

The participants discussed product related neonatal issues.

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 14-16 September 2016 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dirk Mentzer	Member	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:	EMEA-000434-PIP01-08-M04
Jacqueline Carleer	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Adriana Andrić	Member	Croatia	No interests declared	
Georgios Savva	Member	Cyprus	No interests declared	
Marianne Orholm	Member	Denmark	No interests declared	
Marta Granström	Alternate	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	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	
Immanuel Barth	Expert - in person*	Germany	No interests declared	
Sabine Scherer	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	
Dina Apele-Freimane	Member	Latvia	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg		
John-Joseph Borg	Member	Malta	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	
Ine Skottheim Rusten	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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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 restrictions applicable to this meeting	
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	
Paolo Paolucci	Alternate	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Günther Auerswald	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Michal Odermarsky	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Tsvetana Schyns-Liharska	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Lynn Hudson	Expert - in person*	Critical Path Institute	No restrictions applicable to this meeting	
Martha Brumfield	Expert - in person*	Critical Path Institute	No restrictions applicable to this meeting	
Ann Robbins	Expert - in person*	Critical Path Institute	No restrictions applicable to this meeting	
Graham Higson	Expert - in person*	Critical Path Institute	No restrictions applicable to this meeting	
John Davis	Expert - in person*	International Neonatal Consortium	No restrictions applicable to this meeting	
Ron Portman	Expert - in person*	International Neonatal Consortium	No restrictions applicable to this meeting	
Bob Ward	Expert - in	International	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
	person*	Neonatal Consortium	applicable to this meeting	
Mark Turner	Expert - in person*	International Neonatal Consortium	No restrictions applicable to this meeting	
Gerri Baer	Expert - in person*	Food and Drug Administration	No restrictions applicable to this 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/