22 June 2016
EMA/PDCO/316468/2016
Procedure Management and Committees Support Division

Paediatric Committee (PDCO)

Minutes for the meeting on 25-27 May 2016

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

25 May 2016, 08:30- 19:00, room 3E

26 May 2016, 08:30- 19:00, room 3E

27 May 2016, 08:30- 13:00, room 3E

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 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. Eleclazine - EMEA-001697-PIP01-14

Gilead Sciences International Ltd; Treatment of congenital long QT syndromes /Indicated for the treatment of long QT syndrome type 2 (LQT2), Indicated for the treatment of long QT syndrome type 3 (LQT3)

Day 120 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO evaluated the answers that the applicant provided to the question on the open issues asked at day 90 and considered them satisfactory.
Taking the above and previous conclusions into consideration, the PDCO adopted a positive opinion at day 120.

2.1.2. Eleclazine - EMEA-001697-PIP02-14

Gilead Sciences International Ltd; Treatment of hypertrophic cardiomyopathy / indicated for the treatment of symptomatic hypertrophic cardiomyopathy (HCM)

Day 120 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO evaluated the answers that the applicant provided to the question on the open issues asked at day 90 and considered them satisfactory.
Taking the above and previous conclusions into consideration, the PDCO adopted a positive opinion at day 120.

2.1.3. Metreleptin - Orphan - EMEA-001701-PIP01-14

Aegerion Pharmaceuticals Ltd; Treatment of lipodystrophy

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The Committee assessed the answers that the applicant provided to the Committee's questions at day 90 and was pleased that the applicant agreed with the requests from the PDCO.
Taking the above and previous conclusions into consideration, the PDCO adopted a positive opinion at day 120.

2.1.4. Humanized monoclonal modified immunoglobulin G4 (IgG4) antibody with bispecific structure targeting factors IX, IXa, X and Xa (Emicizumab) - Orphan - EMEA-001839-PIP01-15

Roche Registration Limited; Treatment of Hereditary FVIII Deficiency / Indicated for the routine prophylaxis to reduce the frequency of or prevent bleeding episodes in paediatric patients with hemophilia A with FVIII inhibitors

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, a positive opinion was adopted on the agreement of a PIP and a deferral and a waiver. The PDCO adopted a waiver on its own motion.

2.1.5. Cadazolid - EMEA-001108-PIP02-15

Actelion Registration Ltd.; Enterocolitis due to Clostridium difficile / Treatment of Clostridium difficile-associated diarrhea (CDAD)

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

A PIP for children from birth to less than 18 years of age with Clostridium difficile associated diarrhoea was agreed. The applicant provided an acceptable justification for the proposed timelines.

2.1.6. Glycopyrronium bromide / Mometasone furoate / Indacaterol acetate - EMEA-001812-PIP01-15

Novartis Europharm Ltd.; Treatment of asthma

Day 120 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO discussed this application on D120. The responses following the D90 discussion were generally considered acceptable.
A positive opinion was adopted.

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-M07

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

Day 60 opinion

Uro-nephrology

Summary of committee discussion:

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0008/2016) of 29 January 2016.

2.2.2. Diphtheria toxoid-2IU / Tetanus toxoid-20IU / Bordetella pertussis antigen : Pertussis toxoid-8µg Filamentous Haemagglutinin-8µg Pertactin-2.5µg / Inactivated poliovirus: type 1 (Mahoney strain)-40D Inactivated poliovirus: type 2 (MEF-1 strain)-8D Inactivated poliovirus: type 3 (Saukett strain)-32D - EMEA-C-000500-PIP01-08-M03 – early adoption of opinion

GlaxoSmithKline Biologicals S.A; Prevention of infectious diseases caused by Corynebacterium diphtheriae / Clostridium tetani / Bordetella pertussis / Poliovirus types 1, 2 and 3

Day 30 opinion

Vaccines

Summary of committee discussion:

Compliance with all measures is confirmed.
The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0035/2015) of 06 March 2015.

2.2.3. Rufinamide- EMEA-C3-000709-PIP01-09-M05

Eisai Limited; Treatment of Lennox Gastaut Syndrome

Neurology

Summary of committee discussion:

The committee noted the adoption of the compliance report via written procedure on 23 May 2016.

2.2.4. Tiotropium bromide (monohydrate) - EMEA-C-000035-PIP02-09-M02 – early adoption of opinion

Boehringer Ingelheim International GmbH; Treatment of asthma

Day 30 opinion

Pneumology - Allergology

The PDCO discussed the compliance request.
The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0005/2015 of 30 January 2015).

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Landiolol hydrochloride - EMEA-001150-PIP02-13-M01

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrhythmias / Treatment of

sinus tachycardia or supraventricular tachyarrhythmias, including junctional ectopic tachycardia (JET), atrial flutter (AF), atrial fibrillation (AFL), focal atrial tachycardia (FAT), atrioventricular re-entrant tachycardia (AVRT), and atrioventricular nodal re-entrant tachycardia (AVNRT), peri-operatively (during an induction phase, intra-operatively, and during the weaning phase), or when in the phycisian's judgement control of the heart rate is required

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO reviewed the additional information received after Day 30 and re-discussed the proposal. In conclusion the modifications were considered acceptable.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO 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.

2.3.2. rdESAT-6 (recombinant dimer of 6 kD early secretory antigenic target) / rCFP-10 (recombinant 10 kD culture filtrate protein) - EMEA-001156-PIP01-11-M07

Statens Serum Institut; Diagnosis of tuberculosis / To diagnose individuals suspected to be infected with Mycobacterium tuberculosis from 28 days of age

Day 60 opinion

Diagnostic

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/0068/2016 of 18 March 2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. corifollitropin alfa - EMEA-000306-PIP01-08-M03

Merck Sharp & Dohme Limited; Inability to achieve pregnancy, female / hypogonadotropic hypogonadism

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP.

2.3.4. dulaglutide - EMEA-000783-PIP01-09-M04

Eli Lilly & Company; Type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO reviewed the clarifications provided by the applicant after D30 of this procedure and adopted a positive opinion on the PIP modification request. However, it should be noted that some modifications were not deemed acceptable.

2.3.5. Liraglutide - EMEA-000128-PIP01-07-M07

Novo Nordisk A/S; E11 Non-insulin-dependent diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP.

2.3.6. caplacizumab (anti-von Willebrand Factor Nanobody) - Orphan - EMEA-001157-PIP01-11-M01

Ablynx NV; Treatment of thrombotic thrombocytopenic purpura / Treatment of acquired thrombotic thrombocytopenic purpura

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the modification request of EMEA-001157-PIP01-11-M01 at D60.
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/0060/2012 of 26 March 2012).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Deferasirox - Orphan - EMEA-001103-PIP01-10-M03

Novartis Europharm Limited; Treatment of chronic overload requiring chelation therapy / Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is contraindicated or inadequate in patients with others anaemias, Treatment of chronic transfusional iron overload in patients with beta thalassemia major, Treatment of chronic iron overload in patients with non-transfusion-dependent thalassemia

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO agreed with the addition of a quality-related study for the development of granules.
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/0039/2015 of 06 March 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Eltrombopag - EMEA-000170-PIP03-13-M01

Novartis Europharm Limited; Bone Marrow Depression and Hypoplastic Anaemia / Treatment of cytopenias in paediatric patients with severe aplastic anaemia who are no receiving hematopoietic stem cell transplant

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the request to modify the PIP agreed for eltrombopag targeting a use to treat severe aplastic anaemia, noting the supplementary information provided by the applicant. It was noted that recently the indication to treat children with chronic immune-thrombocytopenia had been authorised (Commission Decision 20 April 2016), as flagged by the applicant.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that some of the proposed changes could be accepted as outlined above. 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.

2.3.9. baricitinib - EMEA-001220-PIP01-11-M01

Eli Lilly & Company Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis) / Treatment of juvenile idiopathic arthritis, Treatment of JIA-associated uveitis

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as

set in the Agency's latest decision (P/0069/2013 of 26 March 2013).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. belimumab - EMEA-000520-PIP01-08-M05

Glaxo Group Limited; Systemic lupus erythematosus / Treatment of systemic lupus erythematosus

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO adopted a favourable Opinion on the modification of the agreed PIP.

2.3.11. Eculizumab - Orphan - EMEA-000876-PIP05-15-M01

Alexion Europe SAS; Myasthenia Gravis / Treatment of Refractory Generalized Myasthenia Gravis

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0155/2015 of 10 July 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. sofosbuvir - EMEA-001411-PIP01-12-M03

Gilead Sciences International Ltd; Chronic Viral Hepatitis C infection / Chronic Viral Hepatitis C infection

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0003/2015 of 16 January 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M10

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

Day 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 including the clarification response the applicant submitted after D30 the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0242/2015 of 30 October 2015)
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Decitabine - Orphan - EMEA-000555-PIP01-09-M05

Janssen-Cilag International NV; Acute Myeloid Leukaemia / 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 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed in depth the modification request for decitabine, the paediatric development of which targets a use / indication to treat paediatric patients with an acute myeloid leukaemia who have high-risk cytogenetics, or are refractory to, or have a relapse after first-line treatment. The Committee took into account the supplementary information sent by the applicant, a discussion with the CHMP Rapporteur and information obtained from the external expert.
A draft Opinion had been shared with the applicant in which the requested modifications were refused under Article 22 of the paediatric regulation (EC) No 1901/2006.

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.

2.3.15. Regorafenib - EMEA-001178-PIP01-11-M02

Bayer Pharma; Treatment of all conditions contained in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of paediatric patients with a solid malignant tumour(s) integrated with anti-cancer therapy

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed the modification request concerning the PIP agreed for regorafenib, which is to target the development of regorafenib for the treatment of paediatric patients with a solid malignant tumour(s) integrated with anti-cancer therapy. The supplementary information was taken into account as far as possible, while a comprehensive review was impossible due to its amount. A draft Opinion had been provided to the applicant.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that some changes to the Opinion could be accepted while it was noted that other changes were not needed and that further work to support the proposed development plan is ongoing.
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.

2.3.16. Ivacaftor - Orphan - EMEA-001640-PIP01-14-M01

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. The committee also discussed the applicant's clarifications and considered them agreeable with one exception.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that almost all 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/0098/2015 of 8 May 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion

2.3.17. AGOMELATINE - EMEA-001181-PIP01-11-M03

Les Laboratoires Servier; Major Depressive Episodes / Major Depressive Episodes

Day 60 opinion

Psychiatry

Summary of committee discussion:

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. betrixaban - EMEA-001834-PIP01-15

Prevention of venous thromboembolism / adults and children

Day 90 discussion

Cardiovascular Diseases

3.1.2. Semaglutide - EMEA-001441-PIP02-15

Treatment of Type 2 Diabetes Mellitus / Treatment of Type 2 Diabetes Mellitus

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. EMEA-001843-PIP01-15

Chronic Idiopathic Arthritis / Treatment of Juvenile Idiopathic Arthritis

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.4. Eculizumab - Orphan - EMEA-000876-PIP06-15

Alexion Europe SAS; Prevention of graft rejection following solid organ transplantation / Prevention of acute antibody-mediated rejection in sensitized recipients after kidney transplantation

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.5. EMEA-001776-PIP01-15

Treatment of Active Psoriatic Arthritis, Treatment of Crohn's disease, Treatment of plaque psoriasis, Treatment of Ankylosing Spondylitis, Treatment of Asthma / not available at present, Treatment of moderate to severe plaque psoriasis in children and adolescents from the age of 6 years, who are candidates for systemic therapies

Day 90 discussion

Immunology-Rheumatology-Transplantation / Dermatology / Pneumology - Allergology / Gastroenterology-Hepatology

3.1.6. EMEA-001838-PIP01-15

Treatment of respiratory tract disease caused by human respiratory syncytial virus (RSV) / Treatment of respiratory tract disease caused by human RSV

Day 90 discussion

Infectious Diseases

3.1.7. Cabotegravir - EMEA-001418-PIP02-15

Prevention of human immunodeficiency virus (HIV-1) infection / Cabotegravir is to be indicated in combination with safer sex practices for PrEP to reduce the risk of HIV-1 acquisition in sexually active adolescents at high risk, from 12 to < 18 years of age

Day 90 discussion

Infectious Diseases

3.1.8. Peramivir - EMEA-001856-PIP01-15

Treatment of influenza / Treatment of influenza

Day 90 discussion

Infectious Diseases

3.1.9. Quizartinib - Orphan - EMEA-001821-PIP01-15

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

Day 90 discussion

Oncology

3.1.10. andexanet alfa - EMEA-001902-PIP01-15

Prevention of factor Xa inhibitor associated haemorrhage, Treatment of factor Xa inhibitor associated haemorrhage / (as above), For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients experiencing an acute major bleeding event or requiring urgent surgery.

Day 90 discussion

Other

3.1.11. 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

GlaxoSmithKline Trading Services Limited; Metachromatic leukodystrophy (MLD) / For the treatment of metachromatic leukodystrophy (MLD)

Day 90 discussion

Other

3.1.12. alvimopan - EMEA-001922-PIP01-15

Postoperative ileus

Day 60 discussion

Gastroenterology-Hepatology

3.1.13. Ascorbic Acid / Sodium Ascorbate / Potassium Chloride / Sodium Chloride / Sodium Sulfate / Macrogol 3350 - EMEA-001705-PIP02-15

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

Day 60 discussion

Gastroenterology-Hepatology

3.1.14. Susoctocog alfa - EMEA-000753-PIP02-16

Congenital haemophilia A with antibodies (inhibitors) to human factor VIII / Peri-operative management in patients with congenital haemophilia A with antibodies (inhibitors) to human FVIII, On-demand treatment and control of bleeding episodes in patients with congenital haemophilia A with antibodies (inhibitors) to human FVIII

Day 60 discussion

Haematology-Hemostaseology

3.1.15. Fc- and CDR-modified humanized monoclonal antibody against C5 - EMEA-001943-PIP01-16

Atypical Haemolytic Uremic Syndrome / Treatment of atypical Haemolytic Uremic Syndrome

Day 60 discussion

Uro-nephrology / Haematology-Hemostaseology

3.1.16. Allogeneic Human Adult Mesodermal Immunomodulatory Progenitor Cells - EMEA-001955-PIP01-16

Heart Failure

Day 30 discussion

Cardiovascular Diseases

3.1.17. Hydrochlorothiazide / Amlodipine / Ramipril - EMEA-001942-PIP01-16

Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.18. Rosuvastatin / Amlodipine - EMEA-001935-PIP01-16

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 30 discussion

Cardiovascular Diseases

3.1.19. pegvaliase - Orphan - EMEA-001951-PIP01-16

BioMarin International Limited; For the treatment of hyperphenylalaninaemia / For the treatment of hyperphenylalaninaemia in paediatric patients of all ages with phenylketonuria

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. EMEA-001929-PIP01-16

Crohn's disease, Ulcerative colitis / Treatment of children 4 to 17 years of age with moderately to severely active Crohn's disease who have had an inadequate response with, lost response to, or were intolerant to treatment with a tumour necrosis factor-alpha inhibitor; or immunomodulator, or had an inadequate response with, were intolerant to, or demonstrated dependence on corticosteroids., Treatment of children 4 to 17 years of age with moderately to severely active ulcerative colitis who have had an inadequate response with, lost response to, or were intolerant to treatment with a tumour necrosis factor-alpha inhibitor; or immunomodulator, or had an inadequate response with, were intolerant to, or demonstrated dependence on corticosteroids

Day 30 discussion

Gastroenterology-Hepatology

3.1.21. Autologous CD34+ cells transduced with lentiviral vector encoding the human beta-globin gene - EMEA-001933-PIP01-16

Beta-thalassemia major and intermedia / Treatment of Beta thalassemia major and intermedia

Day 30 discussion

Haematology-Hemostaseology

3.1.22. EMEA-001944-PIP01-16

Anaemia secondary to chronic kidney disease / Treatment of anaemia secondary to chronic kidney disease

Day 30 discussion

Haematology-Hemostaseology

3.1.23. Lusutrombopag - EMEA-001905-PIP01-15

Treatment of thrombocytopenia

Day 30 discussion

Haematology-Hemostaseology

3.1.24. EMEA-001923-PIP01-15

Chronic idiopathic arthritis, including rheumatoid arthritis, axial spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis (pJIA indication), Chronic idiopathic arthritis, including rheumatoid arthritis, axial spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis (sJIA indication) / Treatment of systemic Juvenile Idiopathic Arthritis (sJIA), Treatment of polyarticular-course Juvenile Idiopathic Arthritis (pJIA).

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.25. Dolutegravir (DTG) / Lamivudine (3TC) - EMEA-001940-PIP01-16

Treatment of human immunodeficiency virus (HIV-1) infection / Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.1.26. Anti-(human calcitonin gene-related peptide receptor) human monoclonal antibody - EMEA-001664-PIP02-15

Migraine headaches / Prophylaxis of migraine

Day 30 discussion

Neurology

3.1.27. Humanized monoclonal calcitonin gene-related peptide neutralizing antibody - EMEA-001860-PIP04-16

Prophylactic treatment of cluster headache

Day 30 discussion

Neurology

3.1.28. Lenalidomide - Orphan - EMEA-000371-PIP03-15

Celgene Europe Limited; Marginal zone lymphoma, Multiple Myeloma, Follicular Lymphoma, Diffuse Large B-cell Lymphoma, Myelodysplastic syndrome, Mantle Cell Lymphoma / not applicable - class waiver, not applicable - product specific waiver

Day 30 discussion

Oncology

3.1.29. Pembrolizumab - EMEA-001474-PIP02-16

Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)., Treatment of Hodgkin Lymphoma / Treatment of relapsed or refractory classical Hodgkin Lymphoma in children from 5 years to less than 18 years of age., Treatment of advanced, untreated or previously treated, malignant melanoma in children from 12 year old to less than 18 years of age. Treatment as monotherapy of a PD-L1 positive paediatric malignant solid tumor in children from 6 months to less than 18 years of age

Day 30 discussion

Oncology

3.1.30. Recombinant human monoclonal IgG1 antibody directed against Programmed Death Ligand-1 (anti-PD-L1) - Orphan - EMEA-001849-PIP02-15

Merck KGaA; The treatment of solid malignant neoplasms

Day 30 discussion

Oncology

3.1.31. Dexamethasone / Povidone-Iodine - EMEA-001936-PIP01-16

Treatment of Infectious conjunctivitis (adenoviral and bacterial)

Day 30 discussion

Ophthalmology

3.1.32. EMEA-001947-PIP01-16

Grass pollen-induced allergic rhinitis/rhinoconjunctivitis / Treatment of grass pollen-induced allergic rhinitis, with or without conjunctivitis (AR/C)

Day 30 discussion

Pneumology - Allergology / Oto-rhino-laryngology

3.1.33. Lisdexamfetamine dimesylate - EMEA-000553-PIP02-16

Binge Eating Disorder / Binge Eating Disorder in Adults

Day 30 discussion

Psychiatry

3.1.34. Recombinant E. coli serotype O25B antigen polysaccharide (EcoO25B) – EPA (E) conjugate / Recombinant E. coli serotype O6A antigen polysaccharide (EcoO6A) – EPA (E) conjugate / Recombinant E. coli serotype O2 antigen polysaccharide (EcoO2) – EPA (E) conjugate / Recombinant E. coli serotype O1A antigen polysaccharide (EcoO1A) – EPA (E) conjugate - EMEA-001937-PIP01-16

Prevention of Escherichia infections / Full waiver for pediatric use is being requested

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. Human Fibrinogen - EMEA-C1-001208-PIP01-11-M02

Octapharma Pharamzeutika Produktionsges.m.b.H; Treatment of congenital fibrinogen deficiency

Day 30 discussion

Haematology-Hemostaseology

3.2.2. Recombinant Human TriPeptidyl Peptidase 1 (rhTPP1) - EMEA-C-001362-PIP01-12-M02

BioMarin International Limited; Treatment of Neuronal Ceroid Lipofuscinosis Type 2 (CLN2)

Day 30 opinion

Neurology

Summary of committee discussion:

The PDCO discussed the compliance request. The PDCO took note of the preceding procedure and report on partially completed compliance, confirming that one study, is compliant with the Agency's Decision (P/0209/2015) of 18 September 2015.
The PDCO discussed the completed study and considered that this study was not undertaken in compliance with the Agency's Decision (P/0209/2015) of 18 September 2015The PDCO adopted on 27 May 2016 an opinion refusing the compliance of one or more studies in the agreed paediatric investigation plan as set out in the Agency's Decision (P/0209/2015) of 18 September 2015.

3.2.3. Recombinant human alpha-mannosidase; Lamazym; INN: velmanase alfa; - EMEA-C1-001056-PIP02-12

Chiesi Farmaceutici S.p.A.; Treatment of alpha-Mannosidosis

Day 30 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0122/2014) of 7 May 2014.
The PDCO finalised this partial compliance check procedure.

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Dobutamine - EMEA-001262-PIP01-12-M01

Proveca Limited; Circulatory impairment / haemodynamic insufficiency

Day 30 discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

3.3.2. Recombinant human beta-glucuronidase - Orphan - EMEA-001540-PIP01-13-M01

Ultragenyx UK Limited; ICD-10: E76.2, Mucopolysaccharidosis type 7 (MPS 7) / Treatment of Mucopolysaccharidosis 7 (MPS 7)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. elobixibat - EMEA-001484-PIP01-13-M01

Elobix AB; Constipation

Day 30 discussion

Gastroenterology-Hepatology

3.3.4. turoctocog alfa - EMEA-000428-PIP01-08-M03

Novo Nordisk A/S; Hereditary Factor VIII Deficiency / Treatment and prophylaxis of bleeding in patients with Haemophilia A (congenital Factor VIII deficiency)

Day 30 discussion

Haematology-Hemostaseology

3.3.5. Adalimumab - EMEA-000366-PIP05-12-M02

AbbVie Limited; Non-infectious uveitis

Day 30 discussion

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

3.3.6. (3-((4-Benzoyl-1-piperazinyl)(oxo)acetyl)-4-methoxy-7-(3-methyl-1H-1,2,4-triazol-1-yl)-1H-pyrrolo[2,3-c]pyridin-1-yl)methyl dihydrogen phosphate, 2-amino-2-(hydroxymethyl)-1,3-propanediol (1:1) - EMEA-001687-PIP01-14-M01

Bristol-Myers Squibb International Corporation; Treatment of human immunodeficiency virus [HIV-1] infection / Treatment of multi-drug resistant HIV-1 infection as part of a combination therapy in paediatric patients aged 2 years to <18 years, who have no more than 2 remaining available fully active antiretroviral therapies

Day 30 discussion

Infectious Diseases

3.3.7. Anidulafungin - EMEA-000469-PIP01-08-M06

Pfizer Limited; Treatment of invasive candidiasis

Day 30 discussion

Infectious Diseases

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

Janssen-Cilag International NV; Treatment of human immunodeficiency virus (HIV-1) infection / Rilpivirine is indicated in combination with other antiretroviral (ARV) medicinal products, for the treatment of human immunodeficiency virus (HIV-1) infection in ARV-naïve paediatric patients from 2 to less than 18 years with a baseline viral load below 100,000 HIV-1 RNA copies/mL.

Day 30 discussion

Infectious Diseases

3.3.9. Tenofovir alafenamide (as fumarate) - EMEA-001584-PIP01-13-M01

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B / <Tradename> is indicated for the treatment of chronic hepatitis B infection in paediatric patients aged 2 years and above.

Day 30 discussion

Infectious Diseases

3.3.10. Brentuximab vedotin - Orphan - EMEA-000980-PIP01-10-M04

Takeda Pharma A/S; Treatment of Hodgkin lymphoma, Treatment of anaplastic large cell lymphoma / Treatment of paediatric patients with newly diagnosed, relapsed or refractory Hodgkin lymphoma (from 5 years of age), Treatment of paediatric patients with first and subsequent relapse or refractory systemic anaplastic large cell lymphoma (from 2 years of age)

Day 30 discussion

Oncology

3.3.11. Dinutuximab - Orphan - EMEA-001285-PIP01-12-M02

United Therapeutics Europe Limited; Neuroblastoma / Treatment of patients with high-risk neuroblastoma following myeloablative therapy and autologous stem cell rescue in combination with GM-CSF, IL-2, and isotretinoin.

Day 30 discussion

Oncology

3.3.12. vemurafenib (propane-1-sulfonic acid {3-[5-(4-chlorophenyl)-1H-pyrrolo[2,3-b]pyridine-3-carbonyl]-2,4-difluorophenyl}-amide - EMEA-000978-PIP01-10-M01

Roche Registration Limited; Treatment of melanoma

Day 30 discussion

Oncology

3.3.13. ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M04

Vertex Pharmaceuticals (Europe) Limited; cystic fibrosis / Treatment of cystic fibrosis

Day 30 discussion

Other

3.3.14. Benralizumab - EMEA-001214-PIP01-11-M05

AstraZeneca AB; Asthma / Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3.15. budesonide - EMEA-001087-PIP02-12-M02

Vectura Limited; treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3.16. lurasidone hydrochloride - EMEA-001230-PIP01-11-M02

Sunovion Pharmaceuticals Ltd.; schizophrenia / schizophrenia

Day 30 discussion

Psychiatry

3.3.17. Pneumococcal polysaccharide serotype 23F conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 19F conjugated to diphtheria toxoid / Pneumococcal polysaccharide serotype 18C conjugated to tetanus toxoid / Pneumococcal polysaccharide serotype 14 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 9V conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 7F conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 6B conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 5 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 1 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 4 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein - EMEA-000673-PIP01-09-M09

GlaxoSmithKline Biologicals S.A.; Disease caused by Streptococcus pneumoniae, Acute Otitis Media caused by Non-typeable Haemophilus influenzae / Disease caused by Streptococcus pneumoniae, Acute Otitis Media caused by Non-typeable Haemophilus influenzae

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 19 July 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

4.3.1. Call for expression of interest to become PDCO representative in Enpr-EMA Coordinating Group

Scope: Replacement of Christoph Male

Summary of committee discussion:

Further to the resignation of a current representative, the PDCO members were invited to express their interest to be nominated as representative in the Enpr-EMA Coordinating Group. The nominated representative will be appointed at the PDCO June 2016 meeting.

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. Durvalumab, Tremelimumab - EMEA-15-2016

AstraZeneca AB; Treatment of ureter and bladder carcinoma; Durvalumab monotherapy for the treatment of patients with inoperable or metastatic urothelial cancer (UC) who express PD-L1 and have progressed during or after 1 prior line of therapy; Durvalumab monotherapy and durvalumab in combination with tremelimumab for first line treatment of

patients with unresectable stage IV urothelial bladder cancer

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 indications was confirmed.

Other potential paediatric interest of this medicine suggested by PDCO: paediatric solid tumours (e.g. sarcoma, neuroblastoma and glioblastoma multiforme).

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.

6.1.2. NGR-human Tumor Necrosis Factor alpha (NGR-hTNF) - EMEA-16-2016

MolMed S.p.A.; Treatment of mesothelioma/ Treatment of adult patients with advanced malignant pleural mesothelioma who have progressed within six months after a pemetrexed-based first-line therapy

Summary of committee discussion:

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

Other potential paediatric interest of this medicine suggested by PDCO: TNF-α has the potential to be highly specific with activity against several paediatric neoplasms as for example recurrent/refractory Wilm's tumour and ganglioblastoma.

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.

6.1.3. Pembrolizumab - EMEA-17-2016

Merck Sharp & Dohme (Europe), Inc.; Treatment of multiple myeloma; Treatment of patients with refractory or relapsed and refractory multiple myeloma; Treatment of patients with newly diagnosed and naïve multiple myeloma

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 indications was confirmed.

Other potential paediatric interest of this medicine suggested by PDCO: a paediatric investigation plan already agreed for the treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) (EMEA-001474-PIP01-13)

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

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

None

8. Annual reports on deferrals

The members of the PDCO took note of the products listed in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

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 informed about 5 products, Enzepi, Odefsey, HyQvia, Reyataz and Zinforo, for which the CHMP adopted positive opinions recommending paediatric indications during their meeting in April 2016. A new paediatric pharmaceutical form (oral powder 50 mg) for Reyataz was approved to enable administration in younger children.

9.2.2. Nomination of PDCO representative at the Working Party with Healthcare Professionals' Organisations (HCPWP) and Patients' and Consumers' Working Party (PCWP)

PDCO Chair: Dirk Mentzer

Summary of committee discussion:

The PDCO confirmed the current representatives at the Consumers' and Patients' Working Party (PCWP) and the following representatives at the Working Party with Healthcare Professionals' Organisations (HCPWP): Antje Neubert, Riccardo Riccardi, Johannes Taminiau, Doina Plesca.

9.2.3. Reflection paper on collecting and reporting information on off-label use in pharmacovigilance

Summary of committee discussion:

EMA presented the draft concept paper which is now open to public consultation. The PDCO considered some points that would need to be jointly elaborated in the new chapter of the GVP on paediatric pharmacovigilance and members were invited to provide comments in writing

9.2.4. GVP module VI on Management and reporting of adverse reactions to medicinal products - revision 2

Summary of committee discussion:

EMA presented a proposal to transform the current Revision of the Paediatric Pharmacovigilance Guidance in a new chapter of the Good Vigilance Practice. The proposal was endorsed.

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 NcWG identified the products which will require NcWG evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

Relevant products for FWG discussion were identified.

9.4. Cooperation within the EU regulatory network

9.4.1. Feedback on EU Network Training Centre (EU NTC) Paediatric Curriculum

Summary of committee discussion:

The following main constructive comments on the draft content of the EU NTC Paediatric Curriculum were made/discussed by the PDCO members:

The importance of including relevant information on establishing an adequate dose of a medicinal product for use in the paediatric population was emphasized. It has been confirmed that this topic will be covered by the foreseen lecture on *General considerations on*

conducting paediatric clinical trials. Furthermore, a lecture on *PBPK modelling (with focus on paediatric modelling) and sparse sampling* is foreseen.

A PDCO member offered to contribute the following additional lecture:

- Drug development in paediatric respiratory diseases

Furthermore the Committee members emphasized that lectures on the following topics would be good to have in addition:

- Drug development in paediatric cardiovascular diseases;
- Drug development in rare diseases relevant for the paediatric population (including metabolic diseases and neuromuscular diseases);
- Drug development in paediatric CNS diseases.

Relevant experts in the field will be contacted to see if such lectures can be generated and added to the curriculum.

It was clarified that training material generated through the Global Research in Paediatrics (GRiP) Masters program cannot be made available free of charge for the EU NTC Paediatric Curriculum.

The current timeline for completion of the currently drafted EU NTC Paediatric Curriculum is December 2016. The EU regulatory network should have access to all recorded lectures by beginning of 2017. However, the EU NTC Paediatric Curriculum can be complemented by additional lectures at any time in the future.

The PDCO members expressed again their full support for this initiative of an EU NTC Paediatric Curriculum. The importance for European regulators having access to such training material was emphasized.

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:

PDCO was updated on the progress of the E11(R) 1 drafting, most of the principles have been agreed and the drafting will be finalised during the ICH steering Committee in June. There is the need to revise the wording of the Ethical Considerations part in view to make it compatible with the EU Clinical Trial Regulation particulars on minors, and this has been reinforced by the wording of the “ETHICAL CONSIDERATIONS FOR CLINICAL TRIALS ON MEDICINAL PRODUCTS CONDUCTED WITH MINORS” of the Ad-hoc group on Clinical Trials.

9.5.2. Report from the workshop ‘Successes and Challenges of Performing Long-Term Paediatric Safety Studies’ organised by the Food and Drug Administration (FDA) on 13-14 April 2016

PDCO Chair: Dirk Mentzer

Summary of committee discussion:

Postponed to PDCO June 2016 meeting.

9.5.3. Report from the 'EMA public workshop on extrapolation of efficacy and safety in medicine development' held on 17 May 2016

Summary of committee discussion:

The Committee was informed of the successful outcome of the workshop. Meeting highlights will be circulated as soon as available to PDCO. Target timelines for CHMP and PDCO adoption of the Reflection Paper for public consultation is September 2016.

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

None

9.7. PDCO work plan

None

9.8. Planning and reporting

None

9.9. PDCO ORGAM

None

10. Any other business

10.1.1. Templates for the summaries of the PDCO opinions

Summary of committee discussion:

The topic was postponed to the PDCO June 2016 meeting.

10.1.2. Presentation of Business Pipeline activity

Summary of committee discussion:

Business Pipeline activity was presented highlighting main features: data acquisition, data management and reporting.

10.1.3. Simplification of members' access to EMA decisions including PDCO opinions and summary reports

Summary of committee discussion:

The committee was informed that the full text of each EMA Decision, which includes also the Opinion with its Annexes and the Summary Report, is now available to them in a single MMD folder, for easier retrieval. The Committee was also informed that it is planned to allow access to the full application documents, as sent by applicants, via an interface with the PedRA database; however the timeline for delivery of this improvement is not finalised yet.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The plans of networks to initiate a working group on paediatric oncology in the Enpr-EMA were discussed. An update on the forthcoming single-arm workshop was provided.

11.1.2. Neonatology

Summary of committee discussion:

Potential approaches to endpoints for clinical trials for Bronchopulmonary Dysplasia (BPD) were discussed, specifically taking into account short and long term objectives.

11.1.3. Inventory

Summary of committee discussion:

This session was cancelled.

The Chair thanked the participants and closed the meeting.

12. List of participants

List of participants including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 25-27 June 2016.

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:	EMEA-000520-PIP01-08-M05 EMEA-000673-PIP01-09-M09 EMEA-C-000500-PIP01-08-M03 EMEA-001765-PIP02-15
Jacqueline Carleer	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 participation in discussion, final deliberations and voting on:	EMEA-001839-PIP01-15 EMEA-000978-PIP01-10-M01
Georgios Savva	Member	Cyprus	No interests declared	
Jaroslav Sterba	Member	Czech Republic	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	Member	Germany	No interests declared	
Sabine Scherer	Alternate	Germany	No interests declared	
Grigorios Melas	Member	Greece	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Paolo Rossi	Member	Italy	No restrictions applicable to this meeting	
Francesca Rocchi	Alternate	Italy	No restrictions applicable to this meeting	
Dina Apele-Freimane	Member	Latvia	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No restrictions applicable to this meeting	
Herbert Lenicker	Alternate	Malta	No interests declared	
Hendrik van den Berg	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	
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	
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	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Antje Neubert	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Paolo Paolucci	Alternate	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No participation in discussion, final deliberations and voting on:	EMEA-C-000035-PIP02-09-M02
Paola Baiardi	Alternate	Patients' Organisation Representative	No interests declared	
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	
Sara Homer	Expert - in person*	United Kingdom	No interests declared	
Juliana Min	Expert - in person*	United Kingdom	No restrictions applicable to this meeting	
Shiva Ramroop	Expert - in person*	United Kingdom	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/