21 July 2017
EMA/PDCO/460837/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Minutes of the meeting on 18 – 21 July 2017

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

18 July 2017, 14:00 - 19:00, room 3A

19 July 2017, 08:30 - 19:00, room 3A

20 July 2017, 08:30 - 19:00, room 3A

21 July 2017, 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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Table of contents

1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and based on the topics in the agenda of the current meeting, the Committee Secretariat announced the restricted involvement of some meeting participants in upcoming discussions as included in the pre-meeting list of participants and restrictions.

Participants in this meeting were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure and as included in the list of participants. All decisions taken at this meeting were made in the presence of a quorum of members (i.e. 23 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

1.2. Adoption of agenda

The agenda was adopted with amendments.

1.3. Adoption of the minutes

The minutes of the June 2017 PDCO plenary meeting 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. Selonsertib - EMEA-001868-PIP03-16

Gilead Sciences International Ltd.; K75.8 Other specified inflammatory liver diseases (non-alcoholic steatohepatitis [NASH]) / Treatment of Non-Alcoholic Steatohepatitis (NASH) with moderate to severe fibrosis (F2-F4) in paediatric subjects, 8 to < 18 years of age

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO discussed this procedure on D120. A positive opinion was adopted.

2.1.2. Atacicept - EMEA-002004-PIP01-16

Merck KGaA; Treatment of systemic lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO confirmed the outcome of the Day 90 discussion. PDCO adopted a positive opinion for atacicept for the treatment of systemic lupus erythematosus

2.1.3. Lefamulin - EMEA-002075-PIP01-16

Nabriva Therapeutics AG; Treatment of community-acquired pneumonia

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

All remaining issues at Day 90 were resolved satisfactorily.
The PDCO adopted a positive opinion, including a deferral.

2.1.4. fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16

Zogenix International Ltd; Dravet syndrome / The adjunctive treatment of seizures in paediatric patients at least 1 year of age with Dravet syndrome

Day 120 opinion

Neurology

Summary of committee discussion:

The PDCO has re-discussed the details of the remaining problematic points PDCO adopted a positive opinion on this PIP.

2.1.5. Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor - Orphan - EMEA-001862-PIP01-15

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at the July 2017 plenary. The PDCO adopted a positive Opinion at Day 120.

2.1.6. Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor - Orphan - EMEA-002010-PIP01-16

Kite Pharma EU B.V.; Treatment of B-cell neoplasm

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at the July 2017 plenary. The PDCO adopted a positive Opinion at Day 120.

2.1.7. daratumumab - Orphan - EMEA-002152-PIP02-17

Janssen-Cilag International N.V.; Natural Killer-cell Neoplasms, Mature B-cell Neoplasms

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO adopted a positive Opinion.

2.1.8. Burosumab - EMEA-001659-PIP02-16

Ultragenyx Pharmaceutical Inc.; Tumor-induced osteomalacia

Day 60 opinion

Other

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee at their July 2017 meeting, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Burosumab for all subsets of the paediatric population (0 to 18 years of age) in the condition of tumour-induced osteomalacia.

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.

2.1.9. Litoxetine (benzoate) - EMEA-002151-PIP01-17

Ixaltis SA; Bladder and urethral symptoms / Treatment of Mixed Urinary Incontinence (women), Treatment of Urinary Incontinence post prostatectomy (men)

Day 60 opinion

Uro-nephrology

Summary of committee discussion:
Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for Litoxetine (benzoate) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of bladder and urethral symptoms (including incontinence).

2.1.10. Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19 - Orphan - EMEA-001654-PIP02-17

Novartis Europharm Limited; Mature B-cell neoplasm / Treatment of paediatric patients with relapsed or refractory mature B-cell non-Hodgkin's lymphoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at the July 2017 plenary.
The PDCO requested a few clarifications to the applicant at day 30. The Committee assessed the information provided and found them agreeable.
The PDCO adopted a positive Opinion at Day 60.

2.1.11. Human normal immunoglobulin for intravenous use - EMEA-002163-PIP01-17

Kedrion S.p.A.; Replacement therapy: D80-D84 Primary Immunodeficiency Syndromes with failure of antibody production. Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed. Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation. Hypogammaglobulinaemia in patients after allogeneic haematopoietic stem cell transplantation (HSCT). / Primary Immunodeficiency Syndromes with failure of antibody production.

Day 60 opinion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO did not support the applicant's request for a PIP. The PDCO recommended granting a waiver on own motion for Human normal immunoglobulin for intravenous use for all subsets of the paediatric population (0 to 18 years of age) in the condition of Treatment of primary immunodeficiency, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The granting of a waiver does not prevent the applicant from applying for a Marketing Authorisation Application in children.
The PDCO emphasises that the granting of a waiver for the condition mentioned above

should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.12. Human normal immunoglobulin - EMEA-002084-PIP01-16

ProMetic BioTherapeutics Ltd; Primary Immunodeficiency Diseases

Day 90 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO agreed that paediatric needs in terms of intravenous immunoglobulins for primary immunodeficiency were already covered and that the product would not provide any significant therapeutic benefit over already available alternatives. Therefore, the waiver request was accepted and the proposed grounds supported.
Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for human normal immunoglobulin for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of primary immunodeficiency.

2.1.13. Human Normal Immunoglobulin for Intravenous Administration (IVIg) - EMEA-002092-PIP01-16

Biotest AG; Treatment of primary immunodeficiency (PID), Treatment of idiopathic thrombocytopenic purpura (ITP) / Primary immunodeficiency syndromes with impaired antibody production, Primary immune thrombocytopenia (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count

Day 90 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO concluded its procedure at their July 2017 meeting adopting a waiver for all subset of the paediatric population based on grounds of lack of therapeutic benefit.
The PDCO agreed that paediatric needs in terms of intravenous immunoglobulins for primary immunodeficiency were already covered and that the product would not provide any significant therapeutic benefit over already available alternatives. Therefore, the waiver request was accepted and the proposed grounds supported.

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for human normal immunoglobulin for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of primary immunodeficiency.

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. Burosumab - EMEA-C2-001659-PIP01-15-M02

Ultragenyx Pharmacetical Inc.; X-linked Hypophosphatemia

Day 30 letter

Other

Summary of committee discussion:

The completed study was checked for compliance.
The PDCO discussed the completed study and considered that this is compliant with the latest Agency's Decision (P/0149/2017) of 07/06/2017.
The PDCO finalised on 21 July 2017 this partially completed compliance procedure.

2.2.2. Ivacaftor - EMEA-C1-001640-PIP01-14-M01

Vertex Pharmaceuticals (Europe) Ltd; Treatment of cystic fibrosis

Day 30 letter

Other / Pneumology - Allergology

Summary of committee discussion:

The PDCO discussed the completed studies and considered that these are compliant with the latest Agency's Decision (P/0193/2016) of 15 July 2016.
The PDCO finalised on 21 July 2017 this partially completed compliance procedure and confirmed the compliance of all those studies contained in the agreed paediatric investigation plan that were to be completed until this date.

2.2.3. vigabatrin - EMEA-C-000717-PIP02-13-M02

ORPHELIA Pharma SA; Treatment of epilepsy

Day 30 opinion

Neurology

Summary of committee discussion:

The PDCO adopted on 21 July 2017 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0083/2017) of 16 March 2017.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Tilmanocept - EMEA-001255-PIP01-11-M02

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

Day 60 opinion

Diagnostic / Oncology

Summary of committee discussion:

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

2.3.2. exenatide - EMEA-000689-PIP01-09-M07

AstraZeneca AB; Non insulin dependent diabetes mellitus (treatment including thiazolidinediones), Non insulin dependent diabetes mellitus (excluding treatment with thiazolidinediones), Non insulin dependent diabetes mellitus - in combination with insulin (with or without oral antidiabetics)

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO discussed this glucagon-like peptide-1 (GLP-1) receptor agonist (exenatide) for the treatment of type 2 diabetes for the second time (day 60) during its plenary on 21 July 2017.

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.

2.3.3. Human Fibrinogen - EMEA-001208-PIP01-11-M03

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

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO's views expressed at the D30 was re-discussed and endorsed.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be

accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0119/2015 of 17/04/15)
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. cobicistat / darunavir - EMEA-001280-PIP01-12-M01

Janssen-Cilag International NV; Treatment of HIV-1 infection / Treatment of HIV-1 infection in pediatric patients from 3 to less than 18 years.

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO discussed during the July meeting the responses provided by the applicant to the D30 minutes and the clarifications requested on some issues,

The PDCO adopted an opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0036/2013 of 27 February 2013).

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

2.3.5. elbasvir / grazoprevir - EMEA-001604-PIP01-13-M03

Merck Sharp & Dohme (Europe), Inc.; Treatment of chronic hepatitis C genotype 1 infection with the combination regimen in children and adolescents from 3 years to less than 18 years of age who are previously untreated or who have failed previous Peg-Interferon/Interferon therapy with ribavirin.

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The applicant provided a response to the PDCO's questions raised at Day 30.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0025/2017 of 31 January 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Fidaxomicin - EMEA-000636-PIP01-09-M06

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

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed

paediatric investigation plan, the PDCO confirmed the outcome of the Day 30 discussion and 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/0058/2017 of 17 March 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M01

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (HIV-1) infection / Treatment of HIV-1 infection in paediatric subjects weighing 25 kg or more above 6 years of age

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO discussed at the July meeting the responses provided by the applicant to the D30 minutes and the clarifications requested on some issues.
The PDCO adopted an opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0036/2013 of 27 February 2013).

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

2.3.8. Tenofovir disoproxil (as fumarate) - EMEA-000533-PIP01-08-M07

Gilead Sciences International Ltd; Treatment of human immunodeficiency virus (HIV-1) infection, Treatment of chronic viral hepatitis B / For treatment of chronic hepatitis B in paediatric patients from 2 years of age with compensated liver disease., In combination with other antiretroviral medicinal products for the treatment of HIV-1 infection in antiretroviral treatment experienced paediatric patients.

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0192/2015 of 4 September 2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M12

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

Day 60 opinion

Neurology

Summary of committee discussion:

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

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

2.3.10. Autologous T cells transduced with lentivIral vector containing a chimeric antigen receptor directed against CD19 - Orphan - EMEA-001654-PIP01-14-M02

Novartis Europharm Limited; B cell acute lymphoblastic leukaemia (ALL) / Treatment of CD19+ B cell acute lymphoblastic leukaemia (ALL) in paediatric patients whose disease is refractory to a standard chemotherapy regimen, relapsed after stem cell transplantation (SCT) or are ineligible for allogenic SCT.

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at the July 2017 plenary.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0337/2016 of 2 December 2016).

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

2.3.11. Dabrafenib (dabrafenib mesilate) - EMEA-001147-PIP01-11-M05

Novartis Europharm Limited; Treatment of solid malignant tumours (excluding melanoma), Treatment of melanoma / Treatment of paediatric patients with solid malignant tumours containing BRAF V600 mutations, Treatment of adolescent patients with melanoma with a BRAF V600 mutation

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed the clarification received from the applicant after D30.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be

accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0022/2016 of 11/12/15).

2.3.12. Lipegfilgrastim - EMEA-001019-PIP01-10-M04

UAB "Sicor Biotech"; Treatment of chemotherapy-induced neutropenia, Prevention of chemotherapy-induced febrile neutropenia / Treatment of neutropenia and reduction in the incidence of febrile neutropenia in patients treated with chemotherapy for malignancy

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO's view expressed at D30 was confirmed and 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/0303/2015 of 21/12/2015).

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

2.3.13. Sunitinib malate - EMEA-000342-PIP01-08-M06

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 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this procedure at the July 2017 plenary.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision P/0321/2016 of 2 December 2016).

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

2.3.14. Trametinib (trametinib dimethyl sulfoxide) - EMEA-001177-PIP01-11-M04

Novartis Europharm Limited; Treatment of melanoma, Treatment of solid malignant tumours (excluding melanoma) / Treatment of paediatric patients with solid malignant tumours containing BRAF V600 mutations, Treatment of adolescent patients with melanoma with a BRAF V600 mutation

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed the clarification received from the applicant after D30.

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0024/2016 of 11/12/15).

2.3.15. lurasidone hydrochloride - EMEA-001230-PIP01-11-M03

Sunovion Pharmaceuticals Ltd.; Schizophrenia

Day 60 opinion

Psychiatry

Summary of committee discussion:

Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could not be accepted.
The PDCO therefore adopted a negative Opinion on the modification of the agreed PIP.

2.3.16. mepolizumab - Orphan - EMEA-000069-PIP02-10-M08

GlaxoSmithKline Trading Services; Treatment of asthma / add-on treatment for severe refractory eosinophilic asthma

Day 30 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO discussed the modification request on 19 July 2017.
Based on the review of the rationale submitted by the applicant for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed change could be accepted.
The PDCO therefore adopted at Day 30 a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0047/2017 of 17 March 2017).

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

2.4. Opinions on Re-examinations

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

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

Day 30 opinion

Ophthalmology

Summary of committee discussion:

The PDCO discussed this re-examination of the opinion. A positive opinion was adopted.

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. Recombinant human monoclonal antibody to GM-CSF - EMEA-001882-PIP02-16

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis) / Treatment of active systemic juvenile idiopathic arthritis (sJIA) in patients 1 year of age and older, Treatment of active polyarticular juvenile idiopathic arthritis (pJIA) in patients 2 years of age and older

Day 90 discussion

Immunology-Rheumatology-Transplantation

3.1.2. 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). , Treatment of complicated urinary tract infections (cUTI).

Day 90 discussion

Infectious Diseases

3.1.3. EMEA-002057-PIP01-16

Treatment of ischemic stroke to improve recovery

Day 90 discussion

Neurology

3.1.4. Pyridopyrimidione SMN2 Splicing Modifier - EMEA-002070-PIP01-16

Treatment of spinal muscular atrophy

Day 90 discussion

Neurology

3.1.5. EMEA-002072-PIP01-16

Treatment of select unresectable or metastatic solid tumours with epacadostat in combination with pembrolizumab in paediatric patients between the ages of 6 months and 18 years of age

Day 90 discussion

Oncology

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

Treatment of B-lymphoblastic leukemia/lymphoma, Treatment of mature B-cell neoplasms / Treatment of pediatric patients with relapsed or refractory CD19+ B-cell acute lymphoblastic leukemia, Treatment of pediatric patients with relapsed or refractory CD19+ B-NHL, including diffuse large B-cell lymphoma, Burkitt lymphoma, and primary mediastinal large B-cell lymphoma

Day 90 discussion

Oncology

3.1.7. Enasidenib - Orphan - EMEA-001798-PIP02-16

Celgene Europe Ltd; Treatment of Acute Myeloid Leukaemia / Treatment of patients aged 2 to 21 years old with relapsed or refractory IDH2- mutated AML after at least 2 prior induction attempts

Day 90 discussion

Oncology

3.1.8. Entospletinib - EMEA-002058-PIP01-16

Treatment of Acute myeloid leukemia

Day 90 discussion

Oncology

3.1.9. Angiotensin II - EMEA-001912-PIP02-16

Catecholamine-resistant hypotension associated with distributive shock

Day 90 discussion

Other

3.1.10. EMEA-002082-PIP01-16

Treatment of cystic fibrosis / indicated to improve lung function and reduce pulmonary

exacerbations for patients in all age groups with cystic fibrosis in conjunction with standard therapies.

Day 90 discussion

Pneumology - Allergology

3.1.11. tralokinumab - EMEA-001900-PIP02-17

Treatment of Atopic Dermatitis

Day 60 discussion

Dermatology

3.1.12. EMEA-002162-PIP01-17

Type 2 diabetes mellitus

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.13. Maralixibat Chloride - Orphan - EMEA-001475-PIP03-17

Shire Pharmaceuticals Ireland Limited; Treatment of Progressive Familial Intrahepatic Cholestasis

Day 60 discussion

Gastroenterology-Hepatology

3.1.14. Non-Pathogenic Bacterial Lysate of Escherichia coli (DSM 17252) and Enterococcus faecalis (DSM 16440) - EMEA-002155-PIP01-17

Irritable bowel syndrome (IBS)

Day 60 discussion

Gastroenterology-Hepatology

3.1.15. Hydroxycarbamide - EMEA-002156-PIP01-17

Sickle Cell Syndrome

Day 60 discussion

Haematology-Hemostaseology

3.1.16. Risankizumab - EMEA-001776-PIP02-17

Chronic Idiopathic Arthritis

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.17. EMEA-002080-PIP01-16

Treatment of influenza

Day 60 discussion

Infectious Diseases

3.1.18. Obiltoxaximab - EMEA-002144-PIP01-17

Treatment of bacillary infection, Prevention of bacillary infection / Treatment of inhalation anthrax following exposure to Bacillus anthracis in combination with appropriate antibacterial drugs, Post-exposure prophylaxis of inhalation anthrax when alternative therapies are not available or are not appropriate, Prophylaxis of inhalation anthrax when alternative therapies are not available or are not appropriate

Day 60 discussion

Infectious Diseases

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

Treatment of intestinal malabsorption in preterm infants

Day 60 discussion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.20. Insulin human - Orphan - EMEA-002116-PIP02-17

Nutrinia, Ltd.; Short bowel syndrome / Treatment of infants with Short Bowel Syndrome following surgical resection to improve intestinal absorption of nutrients and fluids

Day 60 discussion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.21. anetumab ravtansine - Orphan - EMEA-002123-PIP01-17

Bayer AG; Treatment of acute myeloid leukaemia, Treatment of mesothelioma / Treatment of patients from 2 to less than 18 years of age with relapsed and/or refractory mesothelin-positive acute myeloid leukaemia

Day 60 discussion

Oncology

3.1.22. daratumumab - Orphan - EMEA-002152-PIP01-17

Janssen-Cilag International N.V.; Acute Lymphoblastic Leukemia / Daratumumab in combination with standard chemotherapy is indicated for the treatment of pediatric patients aged 1 month to 18 years with acute lymphoblastic leukemia.

Day 60 discussion

Oncology

3.1.23. carotuximab - Orphan - EMEA-002138-PIP01-17

TRACON Pharma Limited; Treatment of angiosarcoma

Day 60 discussion

Oncology

3.1.24. Fully human monoclonal antibody (mAb) directed against the human PD-1 receptor - EMEA-002007-PIP02-17

Treatment of Solid Tumours / Treatment of newly diagnosed diffuse intrinsic pontine gliomas (DIPG) and recurrent high-grade gliomas (HGG)

Day 60 discussion

Oncology

3.1.25. Talacotuzumab - EMEA-002158-PIP01-17

Acute myeloid leukaemia / Talacotuzumab, in combination with anti-cancer therapy is indicated for the treatment of pediatric patients, 28 days to 18 years of age with acute myeloid leukaemia

Day 60 discussion

Oncology / Haematology-Hemostaseology

3.1.26. Fluticasone propionate - EMEA-002140-PIP01-17

Treatment of asthma (mild, moderate, and severe) / Prophylactic management in children who require prophylactic medication, including patients not controlled on currently available prophylactic medication

Day 60 discussion

Pneumology - Allergology

3.1.27. Salmeterol xinafoate / Fluticasone propionate - EMEA-002177-PIP01-17

Treatment of asthma (mild, moderate and severe) / Regular treatment of asthma where use of a combination product (long-acting β2 agonist and inhaled corticosteroid) is appropriate:

• patients not adequately controlled with inhaled corticosteroids and “as needed” inhaled short-acting β2 agonist or • patients already adequately controlled on both inhaled corticosteroid and long-acting β2 agonist

Day 60 discussion

Pneumology - Allergology

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

ICD-10 J45.5x severe persistent asthma

Day 60 discussion

Pneumology - Allergology

3.1.29. Amlodipine / Perindopril arginine / Bisoprolol fumarate - EMEA-002173-PIP01-17

Treatment of vascular hypertensive disorders, Treatment of ischaemic coronary artery disorders

Day 30 discussion

Cardiovascular Diseases

3.1.30. Amlodipine besylate / hydrochlorothiazide / candesartan cilexetil - EMEA-002174-PIP01-17

Treatment of essential hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.31. Fluoromisonidazole (18F) - EMEA-001977-PIP04-17

Visualisation of tissue hypoxia in solid tumours for diagnostic purposes / Gliomas, Renal Cell Carcinoma, Sarcomas

Day 30 discussion

Diagnostic / Oncology

3.1.32. EMEA-002109-PIP01-16

K75.8 Other specified inflammatory liver diseases (non-alcoholic steatohepatitis [NASH]) / Treatment of Non-Alcoholic Steatohepatitis (NASH) with mild to severe fibrosis (F1-F4) in paediatric subjects, 8 to < 18 years of age

Day 30 discussion

Gastroenterology-Hepatology

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

Novartis Europharm Limited; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.1.34. Filgotinib - EMEA-001619-PIP04-17

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis, and juvenile idiopathic arthritis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.35. Influenza virus surface antigens (haemagglutinin) of strain B / Influenza virus surface antigens (haemagglutinin) of strain A / Influenza virus surface antigens (haemagglutinin) of strain A / Influenza virus surface antigens (haemagglutinin) of strain B) - EMEA-002027-PIP02-17

Prevention of Influenza infection

Day 30 discussion

Infectious Diseases

3.1.36. EMEA-002184-PIP01-17

Treatment of excessive daytime sleepiness / Treatment of excessive daytime sleepiness in narcolepsy patients

Day 30 discussion

Neurology

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

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

Day 30 discussion

Neurology

3.1.38. Survival Motor Neuron Gene by Self-Complementary Adeno Associated Virus Serotype 9 (AAV9) - Orphan - EMEA-002168-PIP01-17

AveXis EU Limited; Spinal Muscular Atrophy

Day 30 discussion

Neurology

3.1.39. 16-base single-stranded PNA oligonucleotide linked to a 7 aminoacid peptide C214H290N114O57 - Orphan - EMEA-002119-PIP01-17

BIOGENERA SPA; Treatment of Neuroblastoma (NB) with MYCN expression/amplification

Day 30 discussion

Oncology

3.1.40. durvalumab - EMEA-002028-PIP01-16

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

Day 30 discussion

Oncology

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

Takeda Pharm A/S; Treatment of Acute Lymphoblastic Leukaemia (ALL), Treatment of Multiple Myeloma (MM) / Treatment of adult patients with Newly Diagnosed Multiple Myeloma (NDMM), Treatment of paediatric patients diagnosed with relapsed precursor B-ALL or T-ALL

Day 30 discussion

Oncology

3.1.42. Lenalidomide - Orphan - EMEA-000371-PIP04-16

Celgene Europe Limited; treatment of mature b-cell neoplasms

Day 30 discussion

Oncology

3.1.43. palbociclib - EMEA-002146-PIP01-17

Treatment of rhabdomyosarcoma, Treatment of Ewing sarcoma / treatment of refractory or recurrent rhabdomyosarcoma, treatment of refractory or recurrent Ewing sarcoma

Day 30 discussion

Oncology

3.1.44. tremelimumab - EMEA-002029-PIP01-16

Treatment of all conditions included in the category of malignant neoplasms (except central

nervous system, haematopoietic and lymphoid tissue), Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of paediatric patients from birth to less than 18 years old with solid tumours, Treatment of paediatric patients from birth to less than 18 years old with haematological malignancies

Day 30 discussion

Oncology

3.1.45. EMEA-002147-PIP02-17

Platinum-induced ototoxic hearing loss / Reducing ototoxicity in patients > 1 month and <18 years of age receiving cisplatin chemotherapy for standard risk hepatoblastoma

Day 30 discussion

Oncology / Oto-rhino-laryngology

3.1.46. Latanoprost / Netarsudil - EMEA-002175-PIP01-17

Treatment of Glaucoma

Day 30 discussion

Ophthalmology

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

Sensorion SA; Prevention of cisplatin-Induced otoxicity

Day 30 discussion

Oto-rhino-laryngology

3.1.48. benralizumab - EMEA-001214-PIP02-17

Chronic rhinosinusitis with nasal polyposis

Day 30 discussion

Oto-rhino-laryngology

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 coagulation factor X - EMEA-C-000971-PIP01-10-M02

Bio Products Laboratory Ltd; Treatment of hereditary factor X deficiency

Day 30 discussion

Haematology-Hemostaseology

3.2.2. doravirine - EMEA-C1-001676-PIP01-14-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.2.3. lamivudine / tenofovir disoproxil funarate / doravirine - EMEA-C1-001695-PIP01-14-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.2.4. depatuxizumab mafodotin - EMEA-C1-001732-PIP02-15

AbbVie Ltd; Treatment of high-grade glioma

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Alirocumab - EMEA-001169-PIP01-11-M03

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

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.2. Vestronidase alfa - Orphan - EMEA-001540-PIP01-13-M02

Ultragenyx Germany GmbH; ICD-10: E76.2, Mucopolysaccharidosis type VII (MPS VII) / Treatment of Mucopolysaccharidosis type VII (MPS VII)

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Eluxadoline - EMEA-001579-PIP01-13-M01

Allergan Limited; Irritable bowel syndrome with diarrhoea

Day 30 discussion

Gastroenterology-Hepatology

3.3.4. Naloxegol (as naloxegol oxalate) - EMEA-001146-PIP01-11-M03

Kyowa Kirin Pharmaceutical Development Limited; Treatment of opioid-induced constipation

Day 30 discussion

Gastroenterology-Hepatology

3.3.5. Eltrombopag (eltrombopag olamine) - EMEA-000170-PIP03-13-M03

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 30 discussion

Haematology-Hemostaseology

3.3.6. Human thrombin / Human fibrinogen - EMEA-001340-PIP01-12-M03

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

Day 30 discussion

Haematology-Hemostaseology

3.3.7. roxadustat - EMEA-001557-PIP01-13-M01

Astellas Pharma Europe B.V.; treatment of anaemia due to chronic disorders

Day 30 discussion

Haematology-Hemostaseology

3.3.8. Tenofovir alafenamide (as fumarate) - EMEA-001584-PIP01-13-M02

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

Day 30 discussion

Infectious Diseases

3.3.9. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M03

Vanda Pharmaceuticals; ICD-10 G47.24 Circadian rhythm sleep disorder, free-running type (Non-24) / Non24-Hour Sleep-Wake Disorder (Non-24) in the totally blind

Day 30 discussion

Neurology

3.3.10. L-asparaginase encapsulated in erythrocytes - Orphan - EMEA-000341-PIP02-09-M03

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

Day 30 discussion

Oncology

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 12 September 2017 for Nomination of Rapporteur and Peer reviewer

Summary of committee discussion:

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:

PDCO approved the lists of Rapporteurs and Peer Reviewers.

4.3. Nominations for other activities

4.3.1. Nomination of PDCO member to attend the '2nd Human Challenge Trials in Vaccine Development' to be held in Maryland, US on 28-30 September 2017

Summary of committee discussion:

There will be no official PDCO representative at the conference. However, individual experts may participate.

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. Olaparib - EMEA-11-2017

AstraZeneca AB; Treatment of breast carcinoma/monotherapy treatment for patients with metastatic gBRCAm HER2 negative breast cancer who are suitable for single agent chemotherapy when hormonal therapy is considered inappropriate

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.

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. Olaparib - EMEA-12-2017

AstraZeneca AB; Treatment of adenocarcinoma of the pancreas/monotherapy maintenance treatment for gBRCAm metastatic pancreatic cancer patients whose disease has not progressed on first line platinum based chemotherapy

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.

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

7.1.1. Ponesimod - EMEA-000798-PIP01-09

Actelion Pharmaceuticals Ltd.; Multiple Sclerosis/ Relapsing remitting forms of multiple sclerosis

Proposed indication: Add-on treatment for patients with relapsing forms of multiple sclerosis that are active despite treatment with dimethyl fumarate

Summary of committee discussion:

The PDCO agreed that the proposed indication is covered by the condition listed in the latest PIP Decision: 'Treatment of Multiple Sclerosis'.

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

None

9.2. Coordination with EMA Scientific Committees or CMDh-v

9.2.1. Committee for Medicinal Products for Human Use (CHMP)

Summary of committee discussion:

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

The members were also informed about 4 medicinal products, Imraldi, Harvoni, Kaletra and Mimpara for which the CHMP adopted a positive opinion recommending a paediatric indication during their meeting in June 2017.

A new pharmaceutical form (1 mg, 2.5 mg and 5 mg hard capsules) for Mimpara was approved to include paediatric use in the approved indication.

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen Van Malderen

Summary of committee discussion:

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

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

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

9.3.3. Minutes PCWP/HCPWP joint meeting held at EMA on 15 March 2017

Summary of committee discussion:

Minutes of the PCWP/HCPWP joint meeting were shared with the PDCO for information.

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

PDCO members: Maria Fernandez Cortizo, Irja Lutsar

Summary of committee discussion:

PDCO members discussed the wording of the revised guideline

9.3.5. Guideline on good pharmacovigilance practices (GVP), 'Product- or Population-Specific Considerations IV: Paediatric population'

PDCO Chair: Dirk Mentzer

Summary of committee discussion:

PDCO was informed that the GVP Module IV has been adopted by PRAC.

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA): Presentation of the European Cystic Fibrosis Clinical Trials Network

Summary of committee discussion:

Representatives of the European Cystic Fibrosis Clinical Trial Network (ECFS-CTN) discussed with PDCO how dialogue between the committee and the network could be improved to inform each other about challenges related to the conduct of paediatric studies and to discuss how to best address them. The ECFS-CTN representatives expressed their concern that it will be increasingly difficult to conduct clinical studies in light of the large new drug pipeline, which will not only result in high competition for patients to enrol into clinical studies but also raises the question how to best design those studies. The network representatives agreed that at least in the field of cystic fibrosis an age-staggered approach for development of new medicines is appropriate; and that for the development of CFTR modulators a waiver for conducting clinical studies in neonates may be acceptable, as it usually takes 4-6 weeks to confirm the diagnosis of CF following newborn screening.

The network offered to the PDCO the possibility to provide a consolidated network expert opinion on general scientific questions (as opposed to individual expert opinion) within a 2-3 week timeframe.

9.4.2. The 2017 Commission Report on the Paediatric Regulation

Summary of committee discussion:

The European Commission presented the state of play of the 10-year report on the Paediatric Regulation. The committee was informed that the replies received during the public consultation period have been published. The final report is planned to be published by the end of this year.

9.5. Cooperation with International Regulators

None

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

None

9.7. PDCO work plan

None

9.8. Planning and reporting

9.8.1. Draft Agenda of the Strategic Review and Learning Meeting to be held in Estonia on 4-6 October 2017

Summary of committee discussion:

The PDCO finalised the draft agenda of the Strategic Review and Learning Meeting to be held in Estonia on 4-6 October 2017.

10. Any other business

None

11. Breakout sessions

11.1. Paediatric oncology

Summary of committee discussion:

The participants discussed about PIPs for CAR-T cells and on the change of paediatric development (including change of agreed PIPs) for a condition in case of a new product becoming licensed.

11.2. Neonatology

Summary of committee discussion:

The breakout session focused on the neonatal guideline revision and neonatology topics for the upcoming SRL Meeting.

11.3. Inventory

Summary of committee discussion:

The group discussed different ways to collect and collate data on unmet needs for every procedure discussed at the PDCO plenary meetings.

12. List of participants

List of participants including any restrictions with respect to involvement of members/alternates/ experts following evaluation of declared interests for the 18 – 21 July 2017 meeting.

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dirk Mentzer	Chair	Germany	No interests declared	
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Koenraad Norga	Member (Vice-Chair)	Belgium	When chairing the meeting: To be replaced for discussions, final deliberations and voting When not chairing the meeting: No participation in final deliberations and voting	EMEA-001882-PIP02-16 EMEA-000069-PIP02-10-M08 EMEA-002153-PIP01-17
Karen Van Malderen	Alternate	Belgium	No interests declared	
Adriana Andrić	Member	Croatia	No interests declared	
Suzana Mimica Matanovic	Alternate	Croatia	No interests declared	
Jaroslav Sterba	Member	Czech Republic	No interests declared	
Marianne Orholm	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Ann Marie Kaukonen	Member	Finland	No interests declared	
Maija Pihlajamaki	Alternate	Finland	No interests declared	
Sylvie Benchetrit	Member	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Immanuel Barth	Alternate	Germany	No interests declared	
Eleni Katsomiti	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	
Alessandro Jenkner	Alternate	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No interests declared	
Sigita Burokiene	Member	Lithuania	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
John-Joseph Borg	Member	Malta	No interests declared	
Maaike van Dartel	Member	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Marek Migdal	Member	Poland	No interests 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	
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Doina Plesca	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Paola Baiardi	Alternate	Patients' Organisation Representative	No participation in final deliberations and voting on:	EMEA-001882-PIP02-16 EMEA-000069-PIP02-10-M08 EMEA-002153-PIP01-17
Tsvetana Schyns-Liharska	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Catriona Elizabeth Baker	Expert - in person*	United Kingdom	No interests declared	
Tim Lee	Expert - in person*	EnprEMA	No restrictions applicable to this meeting	
Sara Homer	Expert - in person*	United Kingdom	No interests declared	

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

13. Explanatory notes

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

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

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

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

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

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

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