EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 June 2020
EMA/PDCO/341309/2020
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 23-26 June 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

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. 17 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

Due to restricted involvement, the Chair Koenraad Norga was replaced by the Vice-Chair Sabine Scherer for the discussion on agenda topic 3.1.47 and 3.3.35.

1.2. Adoption of agenda

PDCO agenda for 23-26 June 2020

The agenda of the PDCO meeting 23-26 June 2020 was adopted.

1.3. Adoption of the minutes

PDCO minutes for 26-29 May 2020

The minutes of the May 2020 PDCO meeting were adopted and will be published on the EMA website.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Lonapegsomatropin - EMEA-002692-PIP01-19

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO adopted a positive Opinion for the pegylated growth hormone lonapegsomatropin for the treatment of growth hormone deficiency, during its plenary on 26 June 2020.
The PDCO adopted a positive Opinion for the paediatric development of hormone lonapegsomatropin for the treatment of growth hormone deficiency:
A waiver has been agreed by the PDCO in paediatric patients with growth hormone deficiency in a subset of the paediatric population, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2.1.2. Cilofexor - Orphan - EMEA-002554-PIP02-19

Gilead Sciences International Ltd.; Treatment of primary sclerosing cholangitis

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant’s response to the D90 issues was considered acceptable. A positive opinion with a deferral was adopted for a subset of children in the treatment of primary sclerosing cholangitis. A waiver on the grounds lack of significant therapeutic benefit was adopted for a subset of children.

2.1.3. Guselkumab - EMEA-001523-PIP05-19

Janssen-Cilag International N.V.; Treatment of Crohn's Disease

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant’s response to the D90 issue was considered acceptable and a positive opinion and a deferral was adopted for guselkumab in the treatment of a subset of children with Crohn’s disease. A waiver was also granted on the grounds lack of significant benefit for a subset of children.

2.1.4. Marstacimab - Orphan - EMEA-002285-PIP02-19

Pfizer Europe MA EEIG; Congenital haemophilia A, congenital haemophilia B

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO discussed the company's response to the outstanding for this monoclonal antibody against Tissue Factor Pathway Inhibitor (marstacimab) for haemophilia A and B with and without inhibitors during its plenary in June 2020
A waiver has been agreed by the PDCO in paediatric patients with haemophilia A and B in a subset of the paediatric patient population, on the grounds that the specific medicinal product is likely to be unsafe.
A deferral was granted by the PDCO for some studies of the paediatric development.

2.1.5. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19

Principia Biopharma, Inc.; Treatment of immune thrombocytopenia

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO's views expressed at D90 were endorsed and the opinion finalised taking into account the clarifications/details received by the applicant on the draft opinion.
In conclusion, the PDCO adopted a positive opinion on the agreement of a paediatric investigation plan and a deferral with a waiver for the age subset for rilzabrutinib for the treatment of immune thrombocytopenia.

2.1.6. Darunavir / Ritonavir - EMEA-002537-PIP02-19

PharOS - Pharmaceutical Oriented Services Ltd; Treatment of human immunodeficiency virus (HIV-1) infection

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

Following the Day 90 discussion an improved proposal for a modelling & simulation study was provided by the applicant on 16 June 2020, which was further discussed and adjusted by the PDCO.
Thus, based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed a PIP for darunavir/ritonavir for the treatment of human immunodeficiency virus (HIV-1) infection, with a waiver in a subset of children based on lack of safety. The PIP is due to complete in September 2023.

2.1.7. (R)-1-(1-acryloylpiperidin-3-yl)-4-amino-3-(4-phenoxyphenyl)-1H-imidazo[4,5-c] pyridin-2(3H)-one - EMEA-002566-PIP01-19

Genzyme Europe B.V.; Treatment of multiple sclerosis

Day 120 opinion

Neurology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including the new information received since Day 90, the PDCO agrees with the applicant's most recent adjusted proposal for the PIP.
The PDCO adopted a positive opinion endorsing the PIP.

2.1.8. Soticlestat - EMEA-002572-PIP02-19

Takeda Pharma A/S; Treatment of Dravet Syndrome / Treatment of Lennox-Gastaut Syndrome

Day 120 opinion

Neurology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including the new information received since Day 90, the PDCO agrees with the applicant's most recent adjusted proposal for the PIP.
The PDCO adopted a positive opinion endorsing the PIP.

2.1.9. Resamirigene bilparvovec - Orphan - EMEA-002571-PIP01-19

Audentes Therapeutics, Inc.; X-linked myotubular myopathy

Day 120 opinion

Other

Summary of committee discussion:

Thus, based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed a PIP for resamirigene bilparvovec for the treatment of X-linked myotubular myopathy (XLMTM). PIP completion is expected in September 2023.

2.1.10. Fasinumab - EMEA-002059-PIP02-19

Regeneron Ireland D.A.C.; Treatment of chronic musculoskeletal pain / Treatment of chronic non-musculoskeletal pain

Day 120 opinion

Pain

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee including the new information received since Day 90, the PDCO agrees with the applicant's most recent adjusted proposal for the PIP.
The PDCO adopted a positive opinion endorsing the PIP.

2.1.11. Pravastatin sodium / ezetimibe - EMEA-002805-PIP01-20

Laboratoires SMB S.A.; Treatment of hypercholesterolaemia / Prevention of cardiovascular events

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for ezetimibe / pravastatin sodium for all subsets of the paediatric population (from birth to less than 18 years of age) in the conditions of "treatment of hypercholesterolaemia" and "prevention of cardiovascular events" on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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. Sacubitril / valsartan - EMEA-000316-PIP03-20

Novartis Europharm Ltd.; Prevention of heart failure

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for sacubitril/valsartan for all subsets of the paediatric population (0 to 18 years of age) in the condition of Prevention of heart failure.
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.

2.1.13. Benralizumab - EMEA-001214-PIP06-20

AstraZeneca AB; Treatment of bullous pemphigoid

Day 60 opinion

Dermatology

Summary of committee discussion:

Based on the assessment of this application, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for benralizumab for all subsets of the

paediatric population (from birth to less than 18 years of age) in the condition of "treatment of bullous pemphigoid" on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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.

2.1.14. Dupilumab - EMEA-001501-PIP05-20

Regeneron Ireland DAC; Treatment of bullous pemphigoid

Day 60 opinion

Dermatology

Summary of committee discussion:

Based on the assessment of this application, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for dupilumab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of bullous pemphigoid" on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. 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.

2.1.15. Elafibranor - Orphan - EMEA-001857-PIP02-20

Genfit SA; Treatment of primary biliary cholangitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO's view expressed at D30 was re-discussed and endorsed.
Based on the assessment of this application and further discussions at the Paediatric Committee including contributions of external expert(s), the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for elafibranor for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of primary biliary cholangitis.

2.1.16. Balixafortide - EMEA-002718-PIP02-20

Polyphor Deutschland GmbH; Treatment of breast cancer

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the D30 discussion outcome. Overall the PDCO considered a product specific waiver for the condition of treatment of breast cancer acceptable. The committee agreed to the waiver ground of the disease not occurring in a subset of the paediatric population
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 Balixafortide for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of breast cancer.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.17. Half-life extended bispecific T-cell engager (BiTE) antibody construct that binds to prostate-specific membrane antigen and cluster of differentiation 3, with a single chain fragment crystallizable moiety - EMEA-002655-PIP02-20

Amgen Europe BV; Treatment of prostate cancer

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the D30 discussion outcome. 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 Half-life extended bispecific T-cell engager (BiTE) antibody construct that binds to prostate-specific membrane antigen and cluster of differentiation 3, with a single chain fragment crystallizable moiety for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of prostate cancer.
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.

2.1.18. Nivolumab - EMEA-001407-PIP03-20

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions in the category of malignant neoplasms (except haematopoietic and lymphoid tissue other than Hodgkin lymphoma).

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO took note of the applicant proposal submitted after D30
In conclusion, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for nivolumab solution for injection for subcutaneous use for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of all conditions in the category of malignant neoplasms (except central nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms other than Hodgkin lymphoma).
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.

2.1.19. Oportuzumab monatox - EMEA-002797-PIP01-20

Sesen Bio, Inc; Treatment of urothelial carcinoma

Day 60 opinion

Oncology

Summary of committee discussion:

This procedure was discussed at Day 60 during the June plenary meeting.
Thus, the Committee decided that the waiver should be agreed for the entire paediatric population, from birth to less than 18 years of age, on the grounds that the disease does not occur in the paediatric population.
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 oportuzumab monatox for all subsets of the paediatric population (0 to 18 years of age) for the 'treatment of urothelial carcinoma' on the grounds that the disease or condition occurs only in adult populations.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.20. Sasanlimab - EMEA-002777-PIP01-20

Pfizer Europe MA EEIG; Treatment of ureter and bladder carcinoma

Day 60 opinion

Oncology

Summary of committee discussion:

This procedure was discussed at Day 60 during the June plenary meeting.
The PDCO confirmed the conclusions reached at Day 30.
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 sasanlimab for all subsets of the paediatric population (0 to 18 years of

age) in the condition 'treatment of ureter and bladder carcinoma' on the grounds that the disease or condition occurs only in adult populations.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.2. 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. Eptinezumab - EMEA-C1-002243-PIP01-17

H. Lundbeck A/S; Prevention of migraine headaches

Day 60 letter

Neurology

Summary of committee discussion:

The PDCO noted the clarifications provided by the applicant on the completed study(ies) and considered that these are compliant with the latest Agency's Decision (P/0314/2019) of 11/9/2019.
The PDCO finalised this partially completed compliance procedure on 26/6/2020.

2.2.2. Empagliflozin - EMEA-C1-000828-PIP01-09

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The applicant is compliant on all key elements of the latest PIP EMEA-000828-PIP01-09-M07, as set out in the above stated Agency's Decision, and considers that there is no need for a scientific assessment by the PDCO as the presented data enable a clear conclusion by EMA.

2.2.3. Semaglutide - EMEA-C1-001441-PIP03-17-M01

Novo Nordisk A/S; Treatment of obesity

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Initiation of PIP Study is hereby confirmed to be compliant as set out in the EMA's Decision P/0326/2019 of 10/09/2019.

2.2.4. Risdiplam - EMEA-C1-002070-PIP01-16-M04

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 30 letter

Neurology

Summary of committee discussion:

All relevant PIP studies are deemed compliant with the latest PIP as set out in the above stated Agency's Decision, and considers that there is no need for a scientific assessment by the PDCO as the presented data enable a clear conclusion.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Crisaborole - EMEA-002065-PIP01-16-M03

Pfizer Europe MA EEIG; Atopic dermatitis

Day 60 opinion

Dermatology

Summary of committee discussion:

The Applicant provided further clarifications regarding initiation and study population as requested at Day 30. The clarifications were deemed acceptable by the PDCO. Therefore, a delay of completion date, including a change to the deferral, was accepted.
The Committee therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0162/2020 of 16 April 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. Efpeglenatide - EMEA-001903-PIP01-15-M01

Sanofi-aventis recherche et développement; Type 2 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

During its June 2020 plenary the PDCO adopted a positive Opinion on this PIP modification request for efpeglenatide (long acting glucagon-like-peptide-1 receptor agonist) for the treatment of adolescents from 10-18 years with type 2 diabetes.
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/0181/2019 of 15/05/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.3. Glucagon - EMEA-001657-PIP01-14-M01

Eli Lilly and Company; Treatment of severe hypogycaemia

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The PDCO confirmed the outcome of the Day 30 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/0184/2015 of 21/08/2015).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.4. Ladarixin - EMEA-002642-PIP01-19-M01

Dompé farmaceutici S.p.A; Treatment of type 1 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

During its plenary on 26 June 2020, the PDCO discussed the applicants' responses to the day 30 issues for the PIP modification request for ladarixin for the treatment of new-onset type 1 diabetes mellitus (T1D) with residual beta cell function.
The PDCO adopted a positive Opinion on this PIP modification of the agreed PIP as set in the Agency's latest decision (P/0124/2020 of 24/03/2020), in line with above discussion, i.e. only the proposed change in patient population (i.e. fasting C-peptide <0.205 nmol/l at baseline -> PIP Studies 3,4,5) and the timeline changes (-> PIP Studies 3,4,5,7) were deemed acceptable by the PDCO.

2.3.5. Recombinant human glutamic acid decarboxylase (rhGAD65) - EMEA-000609-PIP01-09-M02

Diamyd Medical AB; Treatment of type 1 diabetes mellitus

Day 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

The applicant's responses to the PDCO's comments at Day 30 were received.
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/0400/2017 of 19 December 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.6. Ustekinumab - EMEA-000311-PIP04-13-M03

Janssen-Cilag International NV; Treatment of Crohn's Disease

Day 60 opinion

Gastroenterology-Hepatology

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/0096/2019 issued on 22/03/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.7. Ustekinumab - EMEA-000311-PIP05-17-M01

Janssen-Cilag International NV; Treatment of Ulcerative Colitis

Day 60 opinion

Gastroenterology-Hepatology

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/0190/2018 issued on 17 July 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.8. Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-001855-PIP01-15-M02

Genzyme Europe B.V.; Treatment of Haemophilia A, Treatment of Heamophilia B

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP for fitusiran (siRNA oligonucleotide directed against antithrombin mRNA) for the treatment of haemophilia A and B, with and without inhibitors, as set in the Agency's latest decision (P/0424/2019 of 04/12/2019), during its June 2020 meeting.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.9. Belatacept - EMEA-000157-PIP01-07-M05

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

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO re-discussed the proposed modification considering the clarifications provided by the applicant after D30.
All pending issues identified at D30 were therefore considered solved and a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0260/2019 of 16 July 2019) was adopted by the PDCO.
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.10. Brincidofovir - Orphan - EMEA-001904-PIP03-18-M01

Chimerix IRL Limited; Treatment of smallpox

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO's view expressed at D30 was re-discussed and endorsed.
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/0349/2018 of 16 November 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.11. Ceftobiprole medocaril (sodium) - EMEA-000205-PIP02-11-M04

Basilea Pharmaceutica International Ltd.; Treatment of pneumonia

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO, therefore, adopted a favourable Opinion, agreeing on some but not all proposed modifications of the agreed PIP as set in the Agency's latest decision (P/0406/2018 of 20 December 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.12. Dasabuvir sodium - EMEA-001439-PIP01-13-M03

AbbVie Ltd; Chronic Hepatitis C (HCV) 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/0174/2018 of 15/06/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M02

Merck Sharp & Dohme (Europe), Inc.; Treatment of gram-negative organisms

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO view at Day 30 was re-discussed and endorsed.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0173/2019 of 15 May 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.14. Rilpivirine (hydrochloride) - EMEA-000317-PIP01-08-M12

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

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The applicant provided the requested clarification.
For the remaining changes, 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/0205/2019 of 12 June 2019).

2.3.15. Ombitasvir / paritaprevir / ritonavir - EMEA-001440-PIP01-13-M03

AbbVie Ltd; Treatment of chronic hepatitis C

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/0179/2018 of 15 June 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.16. Lasmiditan - EMEA-002166-PIP01-17-M04

Eli Lilly and Company Limited; Treatment of migraine headaches

Day 60 opinion

Neurology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that 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/0291/2019 of 14/8/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.17. Olenasufligene relduparvovec - Orphan - EMEA-002122-PIP02-17-M01

LYSOGENE; Treatment of mucopolysaccharidosis type IIIA

Day 60 opinion

Neurology

Summary of committee discussion:

This procedure was discussed at Day 60 during the June plenary meeting.
The applicant requests several minor changes to the key binding elements of the Opinion, including modification of completion dates for all studies. The requested changes will generally result in relatively short delays that do not affect the present deferral state of any study and in most cases have already taken place.
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/0078/2018 of 16 March 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.18. Perampanel - EMEA-000467-PIP01-08-M14

Eisai Europe Limited; Treatment of treatment-resistant epilepsies

Day 60 opinion

Neurology

Summary of committee discussion:

The PDCO's view expressed at D30 was re-discussed and endorsed.
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/0072/2020 of 18 March 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.19. Pitolisant - Orphan - EMEA-001176-PIP01-11-M05

Bioprojet PHARMA; Treatment of narcolepsy

Day 60 opinion

Neurology

Summary of committee discussion:

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

2.3.20. Concentrate of proteolytic enzyme in bromelain - Orphan - EMEA-000142-PIP02-09-M09

MediWound Germany GmbH; Treatment of burns

Day 60 opinion

Other

Summary of committee discussion:

The PDCO re-discussed this modification request in line with the D30 discussion outcome and considered the requested changes to the patient numbers acceptable.
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 (decision P/0240/2019 of 16/07/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.21. Fosnetupitant / palonosetron / - EMEA-001198-PIP03-17-M04

Helsinn Birex Pharmaceuticals Limited; Prevention of chemotherapy-induced nausea and vomiting

Day 60 opinion

Other

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome discussion from D30.
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/0025/2020 of 07/01/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.22. Dermatophagoides pteronyssinus / Dermatophagoides farinae - EMEA-001258-PIP01-11-M06

ALK-Abelló A/S; Treatment of asthma and treatment of allergic rhinitis

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO view at Day 30 was re-discussed and endorsed.
The PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0328/2019 of 11 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.23. Beclometasone dipropionate / formoterol fumarate dihydrate / glycopyrronium bromide (CHF 5993) - EMEA-001875-PIP02-18-M02

Chiesi Farmaceutici S.p.A.; Treatment of asthma

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the 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/0365/2019 issued on 7 November 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.24. Reslizumab - EMEA-001202-PIP02-13-M04

Teva Pharmaceuticals Europe; Treatment of asthma

Day 60 opinion

Pneumology - Allergology

Summary of committee discussion:

The PDCO view at Day 30 was re-discussed and endorsed.

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0284/2019 issued of 30 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.25. Derivative of 4H-pyrazolo[3,4-d]pyrimidin-4-one - EMEA-001742-PIP01-14-M01

Boehringer Ingelheim International GmbH; Treatment of schizophrenia

Day 60 opinion

Psychiatry

Summary of committee discussion:

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

2.3.26. Ebola Zaire Vaccine (rVSVΔG-ZEBOV-GP, live) - EMEA-001786-PIP01-15-M01

Merck Sharp & Dohme (Europe), Inc.; Prevention of Ebola disease

Day 60 opinion

Vaccines

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/0095/2017 of 11/04/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.27. Purified Rabies virus, WISTAR PM/WI 38-1503-3M strain (inactivated) - EMEA-002234-PIP01-17-M01

Sanofi Pasteur; Prevention of rabies viral infection

Day 60 opinion

Vaccines

Summary of committee discussion:

As requested at the Day 30 discussion, the applicant provided on 8 June 2020 a summary of study.
Thus, 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/0219/2018 of 17 July 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.4. Opinions on Re-examinations

No items

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of opinions

No item

2.7. Partial Compliance Checks completed by EMA

The following partial compliance checks have concluded positively without PDCO discussion. The Committee has been informed in writing.

2.7.1. Asciminib - EMEA-C1-002347-PIP01-18

Novartis Europharm Limited; Treatment of chronic myeloid leukaemia

Day 30 letter

Oncology / Haematology-Hemostaseology

2.7.2. Abrocitinib - EMEA-C1-002312-PIP01-17-M01

Pfizer Europe MA EEIG; Treatment of atopic dermatitis

Day 30 letter

Dermatology

2.7.3. Vaxchora - EMEA-C1-001490-PIP01-13-M01

Emergent Netherlands B.V.; Treatment of cholera

Day 30 letter

Vaccines

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. Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19

CUTISS AG; Treatment of burns

Day 90 discussion

Dermatology

3.1.2. Lerodalcibep - EMEA-002720-PIP01-19

Treatment of elevated cholesterol / Treatment of elevated low-density lipoprotein cholesterol (LDL-C) in children from 6 to less than 18 years of age with heterozygous familial hypercholesterolaemia (HeFH) or with homozygous familial hypercholesterolaemia (HoFH).

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Rebisufligene etisparvovec - Orphan - EMEA-002206-PIP02-19

Abeona Therapeutics Inc.; Treatment of Mucopolysaccharidosis IIIA / Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome) in children from birth to less than 18 years of age

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Odevixibat - Orphan - EMEA-002054-PIP02-18

Albireo AB; Biliary atresia

Day 90 discussion

Gastroenterology-Hepatology

3.1.5. Relamorelin - EMEA-002323-PIP02-19

Gastroparesis

Day 90 discussion

Gastroenterology-Hepatology

3.1.6. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the *BCL11A* gene - Orphan - EMEA-002730-PIP01-19

Vertex Pharmaceuticals (Ireland) Limited; Treatment of beta-thalassemia intermedia and major / Treatment of transfusion-dependent beta-thalassemia

Day 90 discussion

Haematology-Hemostaseology

3.1.7. Efgartigimod alfa - Orphan - EMEA-002597-PIP02-19

argenx bv; Treatment of immune thrombocytopenia

Day 90 discussion

Haematology-Hemostaseology

3.1.8. Mitapivat - EMEA-002684-PIP01-19

Pyruvate Kinase Deficiency / Treatment of paediatric patients with Pyruvate Kinase Deficiency

Day 90 discussion

Haematology-Hemostaseology

3.1.9. Pegfilgrastim - EMEA-002671-PIP01-19

Treatment of chemotherapy-induced neutropenia and Prevention of chemotherapy-induced febrile neutropenia / Reduction in the duration of neutropenia and the incidence of febrile neutropenia in paediatric patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 90 discussion

Haematology-Hemostaseology

3.1.10. Recombinant adeno-associated viral vector serotype 6 encoding the B-domain-deleted human factor VIII - Orphan - EMEA-002724-PIP01-19

Pfizer Europe MA EEIG; Treatment of haemophilia A (congenital FVIII deficiency)

Day 90 discussion

Haematology-Hemostaseology

3.1.11. Artesunate - Orphan - EMEA-002710-PIP01-19

Amivas Ireland Ltd; Treatment of Malaria

Day 90 discussion

Infectious Diseases

3.1.12. Diroximel - EMEA-002685-PIP02-19

Treatment of multiple sclerosis

Day 90 discussion

Neurology

3.1.13. EMEA-002631-PIP01-19

Treatment of Acute Myeloid Leukemia

Day 90 discussion

Oncology

3.1.14. Nivolumab / relatlimab - EMEA-002727-PIP01-19

Treatment of melanoma / Relatlimab/nivolumab fixed dose combination for treatment of unresectable or metastatic melanoma in patients from 12 to 18 years

Day 90 discussion

Oncology

3.1.15. Emixustat - EMEA-002581-PIP01-19

Stargardt disease

Day 90 discussion

Ophthalmology

3.1.16. Alpelisib - EMEA-002016-PIP03-19

PIK3CA related Overgrowth Spectrum

Day 90 discussion

Other

3.1.17. Anti-neonatal Fc receptor human monoclonal antibody - Orphan - EMEA-002559-PIP03-19

IBSA Farmaceutici Italia Srl; Autoimmune haemolytic anaemia

Day 90 discussion

Other

3.1.18. Levonorgestrel - EMEA-002767-PIP01-20

Prevention of pregnancy

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. Triheptanoin - Orphan - EMEA-001920-PIP04-19

Ultragenyx Germany GmbH; Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD) / Treatment of Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. EMEA-002773-PIP01-20

Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 discussion

Gastroenterology-Hepatology

3.1.21. EMEA-002757-PIP01-19

Treatment of ulcerative colitis

Day 60 discussion

Gastroenterology-Hepatology

3.1.22. Crovalimab - EMEA-002709-PIP01-19

Treatment of nocturnal hemoglobinuria (PNH)

Day 60 discussion

Haematology-Hemostaseology

3.1.23. Olinciguat - EMEA-002759-PIP01-19

Treatment of Sickle Cell Disease (SCD)

Day 60 discussion

Haematology-Hemostaseology

3.1.24. Voxelotor - Orphan - EMEA-002356-PIP02-20

Synteract GmbH; sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

3.1.25. Tacrolimus - EMEA-001642-PIP02-20

Solid organ transplant rejection / Prophylaxis of transplant rejection in liver, kidney or heart allograft recipients (children aged from birth to less than 18 years) / Treatment of allograft

rejection resistant to treatment with other immunosupressive medical products in children aged from birth to less than 18 years

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.26. Human anti-interleukin-15 (IL-15) monoclonal antibody - EMEA-002775-PIP01-20

Non-responsive coeliac disease (NRCD) with symptoms despite 12 months of following a gluten-free diet (GFD)

Day 60 discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.1.27. EMEA-002740-PIP01-19

Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Infectious Diseases

3.1.28. Sulbactam / durlobactam - EMEA-002807-PIP01-20

Treatment of bacterial infections / Treatment of infections caused by Acinetobacter baumannii-calcoaceticus complex

Day 60 discussion

Infectious Diseases

3.1.29. Rimegepant - EMEA-002812-PIP01-20

Prevention of migraine

Day 60 discussion

Neurology

3.1.30. EMEA-002779-PIP01-20

Treatment of Canavan disease / Treatment of Canavan disease in patients from birth to less than 18 years of age

Day 60 discussion

Neurology

3.1.31. 2-(hydroxymethyl)-2-(methoxymethyl)-1-azabicyclo[2,2,2]octan-3-one - Orphan - EMEA-002621-PIP01-19

Aprea Therapeutics Inc; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of solid malignant tumours

Day 60 discussion

Oncology / Haematology-Hemostaseology

3.1.32. Benzimidazole - EMEA-002394-PIP02-20

Treatment of cystic fibrosis to improve lung function and reduce pulmonary exacerbations for patients with CF in conjunction with standard therapies

Day 60 discussion

Pneumology - Allergology

3.1.33. Seltorexant - EMEA-002746-PIP01-20

Major Depressive Disorder (MDD)

Day 60 discussion

Psychiatry

3.1.34. Sparsentan - Orphan - EMEA-001984-PIP02-20

Retrophin Europe Ltd.; Treatment of Focal segmental glomerular sclerosis (FSGS)

Day 60 discussion

Uro-nephrology

3.1.35. EMEA-002814-PIP01-20

Invasive disease caused by Neisseria meningitidis group A, B, C, W and Y from 2 months of age

Day 60 discussion

Vaccines

3.1.36. EMEA-002795-PIP01-20

Prevention of RSV-associated medically attended lower respiratory tract illness (MA-LRTI) and/or RSV-associated severe MA LRTI in neonates and infants by maternal immunisation / Prevention of RSV-associated medically attended lower respiratory tract illness (MA-LRTI) and/or RSV associated severe MA-LRTI in neonates and infants by active immunisation of pregnant adolescents

Day 60 discussion

Vaccines

3.1.37. EMEA-002771-PIP01-20

Prevention of influenza disease

Day 60 discussion

Vaccines / Infectious Diseases

3.1.38. EMEA-002735-PIP02-20

Heart transplantation

Day 30 discussion

Cardiovascular Diseases

3.1.39. Acetylsalicylic acid / Rosuvastatin - EMEA-002831-PIP01-20

Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

3.1.40. Bisoprolol / Ramipril - EMEA-002794-PIP01-20

Treatment of heart failure / Treatment of coronary artery disease / Treatment of hypertension

Day 30 discussion

Cardiovascular Diseases

3.1.41. Finerenone - EMEA-001623-PIP02-20

Treatment of Heart Failure / Treatment of paediatric patients with heart failure and reduced ejection fraction

Day 30 discussion

Cardiovascular Diseases

3.1.42. EMEA-002778-PIP01-20

Diagnosis by evaluation of any known or suspected clinical condition with contrast enhanced magnetic resonance imaging

Day 30 discussion

Diagnostic

3.1.43. A self-complementary adeno-associated virus [AAV] serotype 8 virus particle encoding the human ornithine transcarbamylase [OTC] gene sequence) - Orphan - EMEA-002830-PIP01-20

Ultragenyx Germany GmbH; Late-onset OTC deficiency

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.44. Recombinant adeno-associated viral vector serotype 9 containing the human N-α-acetylglucosaminidase gene - Orphan - EMEA-002764-PIP01-20

Abeona Therapeutics Inc.; Treatment of Mucopolysaccharidosis IIIB / Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome) in children from birth to less than 18 years of age

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.45. Firsocostat / cilofexor - EMEA-002828-PIP01-20

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

Day 30 discussion

Gastroenterology-Hepatology

3.1.46. Icosabutate - EMEA-002816-PIP01-20

Nonalcoholic fatty liver disease (NAFLD) with fibrosis or NASH

Day 30 discussion

Gastroenterology-Hepatology

3.1.47. Linerixibat - EMEA-002800-PIP01-20

Treatment of primary biliary cholangitis

Day 30 discussion

Gastroenterology-Hepatology

3.1.48. Rozibafusp alfa - EMEA-002815-PIP01-20

Systemic lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.49. Telitacicept - EMEA-002824-PIP01-20

Treatment of systemic lupus erythematosus

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.50. EMEA-002825-PIP01-20

Sicca syndrome (Sjögren's)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.51. 5'-cEtG-sp-cEt5MeU-sp-cEt5MeU-sp-dT-sp-dA-sp-dT-sp-dT-sp-dA-sp-dT-sp-dA-sp-dG-sp-dG-sp-dG-sp-cEt5MeC-sp-cEt5MeU-sp-cEt5MeU-3' - Orphan - EMEA-002609-PIP01-19

Dynacure S.A.S; Centronuclear myopathies

Day 30 discussion

Neurology

3.1.52. Fruquintinib - EMEA-002784-PIP01-20

Treatment of colorectal carcinoma

Day 30 discussion

Oncology

3.1.53. Prolgolimab - EMEA-002792-PIP01-20

Non-small cell neoplasms malignant of the respiratory tract cell-type specified / Treatment of metastatic non-squamous non-small cell lung cancer

Day 30 discussion

Oncology

3.1.54. EMEA-002798-PIP01-20

Treatment of squamous carcinoma of the anal canal (SCAC)

Day 30 discussion

Oncology

3.1.55. Surufatinib - EMEA-002750-PIP01-19

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

nervous system tumours, haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Oncology

3.1.56. Faricimab - EMEA-002817-PIP03-20

Diabetic Retinopathy

Day 30 discussion

Ophthalmology

3.1.57. Faricimab - EMEA-002817-PIP04-20

Retinal vein occlusion

Day 30 discussion

Ophthalmology

3.1.58. Linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV2/5-hRKp.RPGR) - Orphan - EMEA-002827-PIP01-20

MeiraGTx UK II Ltd; Retinitis pigmentosa / RPGR mutation-associated X-linked retinitis pigmentosa

Day 30 discussion

Ophthalmology

3.1.59. Ranibizumab - EMEA-002832-PIP01-20

Diabetic Retinopathy

Day 30 discussion

Ophthalmology

3.1.60. Dronabinol - EMEA-000643-PIP02-20

Treatment of spasticity

Day 30 discussion

Other / Neurology

3.1.61. Zilucoplan - EMEA-002747-PIP01-20

Treatment of Myasthenia Gravis

Day 30 discussion

Other / Neurology

3.1.62. Tezepelumab - EMEA-001613-PIP02-20

Chronic rhinosinusitis with nasal polyps

Day 30 discussion

Oto-rhino-laryngology

3.1.63. Sparsentan - EMEA-001984-PIP03-20

Treatment of IgA Nephropathy (IgAN)

Day 30 discussion

Uro-nephrology

3.1.64. Live attenuated poliovirus type 3 / Live attenuated poliovirus type 1 - EMEA-002799-PIP01-20

Acute poliomyelitis

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. Idarucizumab - EMEA-C-001438-PIP01-13-M01

Boehringer Ingelheim International GmbH; Prevention of dabigatran associated haemorrhage

Day 30 discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.2.2. Eftrenonacog alfa - EMEA-C-000914-PIP01-10-M05

Swedish Orphan Biovitrum AB (publ); Treatment of hereditary factor IX deficiency

Day 30 discussion

Haematology-Hemostaseology

3.2.3. Avapritinib - EMEA-C1-002358-PIP02-18-M01

Blueprint Medicines (Netherlands) B.V.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Oncology

3.2.4. Glucarpidase - EMEA-C-001391-PIP01-12

Protherics Medicines Development BV; Treatment of methotrexate toxicity

Day 30 discussion

Oncology

3.2.5. Pneumococcal polysaccharide serotype 1 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 4 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 5 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 6B – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 9V – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 14 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 18C – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 23F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate (15-valent pneumococcal polysaccharide conjugate vaccine) - EMEA-C1-002215-PIP01-17-M02

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by Streptococcus pneumoniae

Day 30 discussion

Vaccines

3.2.6. Pazopanib - EMEA-C-000601-PIP01-09-M06

Novartis Europharm Limited; Treatment of Ewing sarcoma family of tumours

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Tadalafil - EMEA-000452-PIP02-10-M06

Eli Lilly and Company Ltd; Benign prostatic hyperplasia (already approved in adults), Pulmonary arterial hypertension (already approved in adults) / Treatment of Persistent Pulmonary Hypertension of the Newborn, Treatment of Pulmonary Arterial Hypertension

Day 30 discussion

Cardiovascular Diseases

3.3.2. Exenatide - EMEA-000689-PIP01-09-M10

AstraZeneca AB; Non-insulin dependent diabetes mellitus (excluding treatment with thiazolidinediones), Non-Insulin dependent diabetes mellitus (treatment including thiazolidinediones), Non-insulin dependent diabetes mellitus - in combination with insulin (with or without oral antidiabetics) / Treatment of Type 2 diabetes mellitus

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Cenicriviroc - EMEA-001999-PIP02-17-M01

Allergan Pharmaceuticals International Limited; NASH with Stage 2-3 fibrosis

Day 30 discussion

Gastroenterology-Hepatology

3.3.4. Odevixibat - Orphan - EMEA-002054-PIP01-16-M02

Albireo AB; Progressive familial intrahepatic cholestasis (PFIC)

Day 30 discussion

Gastroenterology-Hepatology

3.3.5. Potassium chloride / sodium chloride / citric acid, anhydrous / sodium citrate / simeticone / sodium sulphate, anhydrous / macrogol 4000 - EMEA-001356-PIP02-12-M03

Alfasigma S.p.A.; Any clinical procedures requiring a clean bowel e.g. bowel endoscopy or radiology

Day 30 discussion

Gastroenterology-Hepatology

3.3.6. Tofacitinib - EMEA-000576-PIP03-12-M04

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.7. Vedolizumab - EMEA-000645-PIP01-09-M07

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

Day 30 discussion

Gastroenterology-Hepatology

3.3.8. Upadacitinib - EMEA-001741-PIP01-14-M03

AbbVie Ltd; Treatment of chronic idiopathic arthritis / Treatment of juvenile idiopathic arthritis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.9. Treosulfan - Orphan - EMEA-000883-PIP01-10-M05

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

Day 30 discussion

Immunology-Rheumatology-Transplantation / Oncology

3.3.10. Avibactam / ceftazidime - EMEA-001313-PIP01-12-M09

Pfizer Europe MA EEIG; Treatment of bacterial infections / For the treatment of complicated urinary tract infections / For the treatment of complicated intra-abdominal infections / For the treatment of pneumonia / For the treatment of infections due to aerobic Gram-negative organisms

Day 30 discussion

Infectious Diseases

3.3.11. Bedaquiline (fumarate) - Orphan - EMEA-000912-PIP01-10-M05

Janssen-Cilag International NV; Treatment of multi-drug resistant tuberculosis

Day 30 discussion

Infectious Diseases

3.3.12. Cabotegravir - EMEA-001418-PIP01-13-M02

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) infection / Treatment of human immunodeficiency virus (HIV-1) infection, in combination with other antirectoviral agents

Day 30 discussion

Infectious Diseases

3.3.13. Cefiderocol - EMEA-002133-PIP01-17-M01

Shionogi B.V.; Treatment of infections due to aerobic Gram-negative bacteria

Day 30 discussion

Infectious Diseases

3.3.14. Ibalizumab - EMEA-002311-PIP01-17-M01

Theratechnologies Europe Limited; Treatment of human immunodeficiency virus (HIV-1) infection / Ibalizumab, a CD4 domain 2-directed HIV-1 inhibitor, in combination with other antiretroviral(s), is indicated for the treatment of children and adolescents (aged 6 to less than 18 years) infected with HIV-1 resistant to at least 1 agent in 3 different classes

Day 30 discussion

Infectious Diseases

3.3.15. Lamivudine / Dolutegravir - EMEA-001940-PIP01-16-M03

ViiV Healthcare UK Limited; Treatment of Human Immunodeficiency virus (HIV - 1) Infection / Treatment of Human Immunodeficiency virus (HIV - 1) Infection

Day 30 discussion

Infectious Diseases

3.3.16. Maribavir - Orphan - EMEA-000353-PIP02-16-M01

Shire Pharmaceuticals Ireland Limited; Treatment of cytomegalovirus (CMV) infection / Treatment of CMV infection in paediatric patients who have undergone a haematopoietic stem cell transplant (HSCT) or solid organ transplant (SOT)

Day 30 discussion

Infectious Diseases

3.3.17. Oseltamivir phosphate - EMEA-000365-PIP01-08-M11

Roche Registration GmbH; Treatment and prevention of influenza / Treatment and prevention of influenza in healthy and immunocompromised patients from birth to less than 18 years of age

Day 30 discussion

Infectious Diseases

3.3.18. Pretomanid - Orphan - EMEA-002115-PIP01-17-M02

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

Day 30 discussion

Infectious Diseases

3.3.19. Rilpivirine (RPV) / Dolutegravir (DTG) - EMEA-001750-PIP01-15-M03

ViiV Healthcare UK Limited; Unspecified Human Immunodeficiency Virus (HIV) disease / Treatment of Human Immunodeficiency Virus type 1 (HIV-1) infection

Day 30 discussion

Infectious Diseases

3.3.20. Tenofovir alafenamide / emtricitabine / bictegravir - EMEA-001766-PIP01-15-M02

Gilead Sciences International Ltd.; Treatment of Human immunodeficiency virus [HIV] disease resulting in other conditions for the treatment of adults and paediatrics aged less than 2 years weighing more than 4 kg infected with human immunodeficiency virus-1 (HIV-1) without any known mutations associated with resistance to the individual components

Day 30 discussion

Infectious Diseases

3.3.21. Hydrocortisone - EMEA-002305-PIP01-17-M01

LABORATOIRE AGUETTANT; Bronchopulmonary dysplasia

Day 30 discussion

Neonatology - Paediatric Intensive Care

3.3.22. Dopamine hydrochloride - EMEA-001105-PIP01-10-M05

Brepco BioPharma; Treatment of hypotension in neonates including the extremely low gestational age newborn / Treatment of hypotension in infants and children / Treatment of hypotension in neonates including the extremely low gestational age newborn / Treatment of hypotension in infants and children

Day 30 discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

3.3.23. Cannabidiol - Orphan - EMEA-001964-PIP01-16-M02

GW Pharma (International) B.V.; Lennox Gastaut Syndrome / Tuberous Sclerosis Complex / Infantile Spasms, Dravet Syndrome / Treatment of Seizures

Day 30 discussion

Neurology

3.3.24. Lacosamide - EMEA-000402-PIP03-17-M04

UCB Pharma S.A.; Treatment of generalised epilepsy and epileptic syndromes

Day 30 discussion

Neurology

3.3.25. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M03

AveXis EU Limited; Treatment of spinal muscular atrophy / Treatment of spinal muscular atrophy Type 1

Day 30 discussion

Neurology

3.3.26. Ozanimod - EMEA-001710-PIP02-14-M05

Celgene Europe B.V.; Treatment of multiple sclerosis

Day 30 discussion

Neurology

3.3.27. Iodine (131-I) murine IgG1 monoclonal antibody against B7-H3 (131I-omburtamab) - Orphan - EMEA-002101-PIP02-18-M01

Y-mAbs Therapeutics A/S; Treatment of pediatric neuroblastoma patients with CNS relapse as evidenced by CNS/LM metastases

Day 30 discussion

Oncology

3.3.28. Ixazomib - Orphan - EMEA-001410-PIP02-17-M03

Takeda Pharm A/S; Treatment of lymphoid malignancies (excluding multiple myeloma), 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

3.3.29. Midostaurin - Orphan - EMEA-000780-PIP01-09-M05

Novartis Europharm Limited; Malignant mastocystosis / Mast cell leukemia / Acute myeloid leukemia / Treatment of pediatric patients with FLT3 mutated AML, newly diagnosed

Day 30 discussion

Oncology

3.3.30. Quizartinib - Orphan - EMEA-001821-PIP01-15-M04

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia / For the treatment of

paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed AML with FLT3-ITD mutations

Day 30 discussion

Oncology

3.3.31. Venetoclax - Orphan - EMEA-002018-PIP02-16-M03

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms, Treatment of solid tumour malignant neoplasms / Treatment of a specific paediatric hematopoietic and lymphoid malignant neoplasm or of a solid malignant neoplasm as agreed by PDCO, in patients from 1 month to 18 years of age

Day 30 discussion

Oncology / Haematology-Hemostaseology

3.3.32. Lenadogene nolparvovec - Orphan - EMEA-001992-PIP02-16-M01

GenSight-Biologics; Leber Hereditary Optic Neuropathy (LHON)

Day 30 discussion

Ophthalmology

3.3.33. Andexanet alfa - EMEA-001902-PIP01-15-M04

Portola Netherlands B.V.; Prevention of factor Xa inhibitor associated haemorrhage, Treatment of factor Xa inhibitor associated haemorrhage / For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients requiring urgent surgery / For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients experiencing an acute major bleeding event

Day 30 discussion

Other

3.3.34. Mometasone (furoate) / Indacaterol (acetate) - EMEA-001217-PIP01-11-M06

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3.35. Vilanterol / fluticasone furoate - EMEA-000431-PIP01-08-M11

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

Day 30 discussion

Pneumology - Allergology

3.3.36. Ferric citrate coordination complex - EMEA-001213-PIP02-12-M03

Akebia Therapeutics, Inc.; Treatment of hyperphosphataemia / Treatment of hyperphosphataemia in patients with chronic kidney disease (CKD)

Day 30 discussion

Uro-nephrology

3.3.37. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues - Orphan - EMEA-002493-PIP01-18-M01

Dicerna Ireland Limited; Primary hyperoxaluria

Day 30 discussion

Uro-nephrology

3.3.38. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-001715-PIP01-14-M04

Seqirus Netherlands B.V.; Influenza / Prevention of influenza

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 6th July 2020 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

Summary of committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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.

5.1. New Scientific Advice

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

5.2. Ongoing Scientific Advice

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

5.3. Final Scientific Advice (Reports and Scientific Advice letters)

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. Ranibizumab - EMEA-04-2020

Roche Products GmBH; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of diabetic macular oedema / Treatment of neo vascular age-related macular degeneration

Summary of committee discussion:

The applicability of the class waiver as referred to in the Agency's Decision CW/0001/2015 to the planned therapeutic indications was confirmed.

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

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

No item

8. Annual reports on deferrals

No item

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

No item

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 the final CHMP Opinions on 2 medicinal products with recommended paediatric indications adopted in May 2020 by CHMP. These include Sivextro (tedizolid phosphate) and Taltz (ixekizumab).

The list of procedures with paediatric indications to be evaluated by the CHMP, starting in May 2020, was presented to the PDCO members.

9.2.2. Committee on Herbal Medicinal Products (HMPC)

HMPC request for PDCO input

Summary of committee discussion:

The PDCO discussed a draft reply to the HMPC that will be circulated for comments to the members after the meeting and adoption of a final position at the July PDCO.

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Summary of committee discussion:

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

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Summary of committee discussion:

Relevant products for FWG discussion were identified.

9.3.3. Registry-based studies guideline - draft

Summary of committee discussion:

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA)

No item

9.5. Cooperation with International Regulators

No item

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

9.8.1. Business pipeline quarterly update report

The business pipeline quarterly update report was circulated for information.

10. Any other business

10.1.1. Covid-19 update

Summary of committee discussion:

The PDCO was updated on the latest developments in the state of the art of SARS-Cov2 infection and COVID19 disease, and on the therapeutics that will soon submit or require a PIP submission.

10.1.2. Debrief on the breakout session

Summary of committee discussion:

Briefing on the discussion in the working group on main issues with paediatric development of COVID vaccines, including waivers, deferrals, and the level of safety data required for starting studies in children.

10.1.3. Pharmaceutical strategy for Europe

Summary of committee discussion:

A presentation on the Pharmaceutical strategy for Europe was provided by the European Commission.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The group discussed how development agreed in a PIP relates to patients with relapsed/refractory malignant tumours

11.1.2. Neonatology

Summary of committee discussion:

The group discussed a PIP modification concerning neonates, and also discussed potential topics for an upcoming EMA-INC meeting.

The Chair thanked all 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 23-26 June 2020 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Koenraad Norga	Chair	Belgium	When chairing the meeting: To be replaced for discussions, final deliberations and voting on:	3.1.47. Linerixibat - EMEA-002800-PIP01-20 3.3.35. Vilanterol / fluticasone furoate - EMEA-000431-PIP01-08-M11
Karl-Heinz Huemer	Member	Austria	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Lucie Kravackova	Member	Czech Republic	No interests declared	
Petra Dominikova	Alternate	Czech Republic	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Jana Lass	Alternate	Estonia	No interests declared	
Ann Marie Totterman	Member	Finland	No interests declared	
Sylvie Benchetrit	Member	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Mountaki	Alternate	Greece	No interests declared	
Ágnes Gyurasics	Member (CHMP member)	Hungary	No interests declared	
Brian Aylward	Member	Ireland	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Carola de Beaufort	Member (CHMP alternate)	Luxembourg	No interests declared	
Herbert Lenicker	Alternate	Malta	No interests declared	
Roel Bolt	Member	Netherlands	No interests declared	
Maaike van Dartel	Alternate	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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	
Peter Sisovsky	Member	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Eva Agurell	Alternate	Sweden	No interests declared	
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	3.3.37. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues - Orphan - EMEA-002493-PIP01-18-M01 2.3.21. Pitolisant - Orphan - EMEA-001176-PIP01-11-M05
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Doina Plesca	Alternate	Healthcare Professionals' Representative	No interests declared	
Günter Karl-Heinz Auerswald	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Paola Baiardi	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Dimitrios Athanasiou	Member	Patients' Organisation Representative	No interests declared	
Viviana	Alternate	Patients'	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Giannuzzi		Organisation Representative	applicable to this meeting	
	EC Representative	European Commission	No interests declared	
Nanna Borup Johansen	expert	Denmark	No interests declared	
Maria Estela Moreno Martín	expert	AEMPS	No interests declared	
A representative from the European Commission attended the meeting				
Meeting run with support from relevant EMA staff				

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

13. Explanatory notes

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

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

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

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

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

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

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