30 July 2020
EMA/PDCO/541950/2020
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 21-24 July 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 2.1.19, 2.3.35, 3.1.11, 3.1.55, 3.3.7, 3.3.9, 3.1.57.

1.2. Adoption of agenda

PDCO agenda for 21-24 July 2020

The agenda of the PDCO meeting 21-24 July 2020 was adopted.

1.3. Adoption of the minutes

PDCO minutes for extraordinary PDCO virtual meeting 14 May 2020 on redemsivir.

PDCO minutes for extraordinary PDCO virtual meeting 9 July 2020 on tocilizumab.

The minutes of the PDCO extraordinary virtual meeting of 14 May, 9 July and 21-24 July 2020 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. Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19

CUTISS AG; Treatment of burns

Day 120 opinion

Dermatology

Summary of committee discussion:

The applicant addressed all outstanding issues satisfactorily between Day 90 and Day 120. Regarding the waiver on the grounds of lack of significant therapeutic benefit over existing treatments, the committee clarified that it should only apply to a subset of newborn infants. The committee agreed on a PIP for the condition "treatment of burns", which is expected to be completed by July 2024. The PIP contains three clinical studies and a deferral.

2.1.2. Lerodalcibep - EMEA-002720-PIP01-19

LIB Therapeutics, LLC; Treatment of hypercholesterolaemia

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

An oral explanation took place on 22nd July 2020 to provide additional background on these outstanding issues.
Moreover, the PDCO agreed with the applicant's initial request for a waiver. The PDCO recommends granting a waiver for lerodalcibep for a subset of the paediatric population in the condition of treatment of hypercholesterolaemia 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. The PDCO identified limited non-invasive treatment options in HoFH (homozygous familial hypercholesterolemia) as an unmet 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.

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

Abeona Therapeutics Inc.; Treatment of mucopolysaccharidosis IIIA

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

This procedure was discussed at Day 120 during the July 2020 plenary meeting.
The PDCO took into consideration the further discussion on the topics discussed at Day 90 and

changes to some wording of the Opinion and adopted a positive Opinion on a Paediatric Investigation Plan for the treatment of mucopolysaccharidosis IIIA and on the granting of a deferral.

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

Albireo AB; Treatment of biliary atresia

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO discussed the responses to the D90 issues on D120.
The responses regarding the quality issues were considered acceptable.
A positive opinion for a PIP and a deferral was adopted for the development of odevixibat in children from birth to less than 18 years with biliary atresia.

2.1.5. Relamorelin - EMEA-002323-PIP02-19

Allergan Pharmaceuticals International Limited; Treatment of gastroparesis

Day 120 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO refused the waiver and accepted the latest proposal from the applicant, notwithstanding a proposed completion date. A positive opinion on a PIP with a deferral was adopted.

2.1.6. Giroctocogene fitelparvovec - Orphan - EMEA-002724-PIP01-19

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

In conclusion, the PDCO adopted a positive opinion for giroctocogene fitelparvovec for the treatment of haemophilia A across the entire paediatric population from birth to less than 18 years of age and a deferral.

2.1.7. Mitapivat - EMEA-002684-PIP01-19

Agios Pharmaceuticals, Inc.; Treatment of pyruvate kinase deficiency

Day 120 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO views expressed at D90 were endorsed and the opinion finalised taking also into account the comments received from 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 in children and a deferral for mitapivat for the treatment of pyruvate kinase deficiency.

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

Amivas Ireland Ltd; Treatment of malaria

Day 120 opinion

Infectious Diseases

Summary of committee discussion:

Based on the responses of the applicant after day 90, the PDCO accepted the proposal of the applicant for an oral explanation.

During the oral explanation that took place on 23rd July 2020, the applicant further described their plans. A positive opinion was granted to the applicant for a PIP in the condition 'treatment of malaria'.

2.1.9. Diroximel fumarate (BIIB098) - EMEA-002685-PIP02-19

Biogen Netherlands B.V.; Treatment of multiple sclerosis

Day 120 opinion

Neurology

Summary of committee discussion:

Following the final discussion of the proposed PIP during the July PDCO plenary meeting taking into account the information sent by the applicant following the PDCO discussion in June 2020 the PDCO came to an agreement with the applicant on the paediatric medicinal product development for treatment of multiple sclerosis in paediatrics.
The PDCO adopted a positive opinion on the paediatric investigation plan, with a deferral for diroximel fumarate for the treatment of relapsing form of multiple sclerosis and a waiver for the age subset in children on the ground that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

2.1.10. (4S,7aR,9aR,10S,11E,14S,15R)-6'-chloro-10-methoxy-14,15-dimethyl-3',4',7a,8,9,9a,10,13,14,15-decahydro-2'H,7H-spiro[1,19-ethenocyclobuta[i][1,4]oxazepino[3,4-f][1,2,7]thiadiazacyclohexadecine-4,1'-naphthalen]-18(17H)-one 16,16-dioxide (AMG 176)- EMEA-002631-PIP01-19

Amgen Europe BV; Treatment of acute myeloid leukemia

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion from the D90 discussion.
A positive Opinion was therefore agreed on this PIP for development of (4S,7aR,9aR,10S,11E,14S,15R)-6'-chloro-10-methoxy-14,15-dimethyl-3',4',7a,8,9,9a,10,13,14,15-decahydro-2'H,7H-spiro[1,19-ethenocyclobuta[i][1,4]oxazepino[3,4f][1,2,7]thiadiazacyclohexadecine-4,1'-naphthalen]-18(17H)-one 16,16-dioxide in patients with relapsed/ refractory AML, including a deferral and waiver for patients based on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

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

Bristol-Myers Squibb International Corporation; Treatment of melanoma

Day 120 opinion

Oncology

Summary of committee discussion:

The PDCO discussed the proposed plan for the relatlimab/ nivolumab fixed dose combination considering the responses received by the applicant after D90, their comments on the draft opinion and the input received by CHMP member(s)/assessors
In summary, the PDCO concluded that the proposed plan for the PIP, was acceptable, acknowledging that the adequacy of the plan will need to be assessed in the context of the totality of data and of the results that will be observed in the adult studies.
In conclusion, the PDCO adopted a positive opinion on the agreement of a paediatric investigation plan and a waiver for an age subset for relatlimab/ nivolumab for the treatment of melanoma.

2.1.12. Emixustat (hydrochloride) - EMEA-002581-PIP01-19

Kubota Pharmaceutical Holdings Co. Limited; Treatment of Stargardt disease

Day 120 opinion

Ophthalmology

Summary of committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO recommends granting a paediatric investigation plan for emixustat with a deferral for children from 6 years to less than 18 years and waiver for children from birth to less than 6 years in the condition of Stargardt disease.

2.1.13. Alpelisib - EMEA-002016-PIP03-19

Novartis Europharm; Treatment of PIK3CA related overgrowth spectrum

Day 120 opinion

Other

Summary of committee discussion:

The applicant provided the remaining clarifications to the points raised at D90
Therefore the PDCO adopted a positive opinion for alpelisib, for treatment of PIK3CA (Phosphatidylinositol-4,5-Bisphosphate 3-Kinase Catalytic Subunit Alpha) related overgrowth spectrum with a waiver for a subset of the paediatric population - on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit - and a deferral.

2.1.14. Anti-neonatal Fc receptor human monoclonal antibody (M281) - Orphan - EMEA-002559-PIP03-19

Momenta Pharmaceuticals, Inc.; Treatment of autoimmune haemolytic aneamia

Day 120 opinion

Other

Summary of committee discussion:

In conclusion, the PDCO adopted a positive opinion for anti-neonatal Fc receptor human monoclonal antibody (M281) for treatment of autoimmune haemolytic anaemia with a deferral and a waiver for a subset of children on the ground that the specific medicinal product is likely to be unsafe.

2.1.15. Acetylsalicylic acid / rosuvastatin (calcium) - EMEA-002831-PIP01-20

IBSA Farmaceutici Italia s.r.l.; Prevention of cardiovascular events

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 acetylsalicylic acid / rosuvastatin calcium for all subsets of the paediatric population (0 to 18 years of age) in the condition of 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.

2.1.16. Bisoprolol (fumarate) / Ramipril - EMEA-002794-PIP01-20

Adamed Pharma S.A.; Treatment of heart failure / Treatment of coronary artery disease / Treatment of hypertension

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 ramipril / bisoprolol (fumarate) for all subsets of the paediatric population (0 to less than 18 years of age) in the conditions of treatment of heart failure, treatment of coronary artery disease and treatment of hypertension.
The PDCO emphasises that the granting of a waiver for the conditions mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need.

2.1.17. Linerixibat - EMEA-002800-PIP01-20

GlaxoSmithKline (Ireland) Ltd; Treatment of primary biliary cholangitis

Day 60 opinion

Gastroenterology-Hepatology

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 linerixibat for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of primary biliary cholangitis.

2.1.18. VIB4920 - EMEA-002825-PIP01-20

Viela Bio Inc; Treatment of Sjögren's syndrome

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

This procedure was discussed at Day 60 during the July plenary meeting.
The PDCO agreed with all 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 Cluster of differentiation 40 (CD40) ligand antagonist comprising two identical Tn3 modules fused to human serum albumin, with each Tn3 module being an engineered form of the third fibronectin type III protein domain of human Tenascin C (VIB4920) for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of Sjögren's syndrome.
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. Fruquintinib - EMEA-002784-PIP01-20

Hutchison MediPharma Ltd; Treatment of colorectal carcinoma

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusion from the D30 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 Fruquintinib for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of colorectal carcinoma based on the grounds that the disease for which the specific medicinal product is intended does not occur in the paediatric population.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.20. Prolgolimab - EMEA-002792-PIP01-20

JSC "BIOCAD"; Treatment of non-small cell lung cancer

Day 60 opinion

Oncology

Summary of committee discussion:

This procedure was discussed at Day 60 during the July plenary meeting.
The PDCO confirmed all points raised 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 prolgolimab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of non-small cell lung 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.

2.1.21. Retifanlimab - EMEA-002798-PIP01-20

Incyte Biosciences Distribution B.V.; Treatment of squamous carcinoma of the anal canal

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions at D30.
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 retifanlimab for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of squamous carcinoma of the anal canal (SCAC) based on the ground that the disease for which the specific medicinal product is intended does not occur in the paediatric population.
The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.22. Faricimab - EMEA-002817-PIP03-20

Roche Registration GmbH; Treatment of diabetic retinopathy

Day 60 opinion

Ophthalmology

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 faricimab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of diabetic retinopathy" 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. The PDCO identified treatment of retinopathy of prematurity as an unmet 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.

2.1.23. Faricimab - EMEA-002817-PIP04-20

Roche Registration GmbH; Treatment of retinal vein occlusion

Day 60 opinion

Ophthalmology

Summary of committee discussion:

Based on the assessment of this application and further clarifications by the applicant, the PDCO agrees with the applicant's request for a waiver. The PDCO recommends granting a waiver for faricimab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of retinal vein occlusion" on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible due to the rarity of this condition in the paediatric population.

The PDCO emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. The PDCO identified treatment of retinopathy of prematurity as an unmet 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.

2.1.24. Ranibizumab - EMEA-002832-PIP01-20

Roche Registration GmbH; Treatment of diabetic retinopathy

Day 60 opinion

Ophthalmology

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 ranibizumab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of "treatment of diabetic retinopathy" 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.

2.1.25. Dronabinol - EMEA-000643-PIP02-20

Bionorica Ethics GmbH; Treatment of spasticity

Day 60 opinion

Other / Neurology

Summary of committee discussion:

The PDCO recommends granted a waiver for dronabinol for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'Treatment of spasticity' on the grounds that the specific medicinal product is likely to be unsafe.

2.1.26. Tezepelumab - EMEA-001613-PIP02-20

AstraZeneca AB; Treatment of nasal polyposis

Day 60 opinion

Oto-rhino-laryngology

Summary of committee discussion:

Based on the assessment of this application the PDCO agrees with the applicant's request for a waiver. The PDCO granted a waiver for tezepelumab for all subsets of the paediatric

population (0 to 18 years of age) in the condition of 'Treatment of nasal polyposis'.

2.1.27. Icosabutate - EMEA-002816-PIP01-20

Treatment of non-alcoholic fatty liver disease including non-alcoholic steatohepatitis

Day 60 discussion

Gastroenterology-Hepatology

Summary of committee discussion:

During its plenary on 24 July 2020, the PDCO adopt an Opinion on the refusal of a PIP and adoption of a full waiver for all paediatric age subsets from birth to less than 18 years on the grounds of likely lack of efficacy, on own motion, for the PIP proposal for icosabutate (NST-4016), a derivative of omega-3 fatty acid (n-3 FA) eicosapentaenoic acid (EPA), for the treatment of Nonalcoholic fatty liver disease (NAFLD) with fibrosis or NASH.

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

Prevention of poliomyelitis viral infection

Day 60 discussion

Vaccines

Summary of committee discussion:

Based on the assessment of this application and the reflection of discussions at the Paediatric Committee during the June 2020 plenary the Paediatric Committee recommends during the July 2020 plenary meeting granting a waiver for Live attenuated poliovirus type 1 / Live attenuated poliovirus type 3 for all subsets of the paediatric population (0 to 18 years of age) in the condition of prevention of poliomyelitis viral infection on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as the needs are already covered.
The Paediatric Committee emphasises that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a marketing authorisation application in the paediatric population.

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

Boehringer Ingelheim International GmbH; Prevention of dabigatran associated haemorrhage / Treatment of dabigatran associated haemorrhage

Day 60 opinion

Cardiovascular Diseases / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO adopted on 24 July 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0151/2019) of 17 April 2019.

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

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

Day 60 opinion

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-000914-PIP01-10-M02

The PDCO adopted on 24 July 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0071/2020) of 18 March 2020.

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

Protherics Medicines Development BV; Treatment of methotrexate toxicity

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO discussed the compliance request again at its July 2020 plenary.
The PDCO adopted on 24 July 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision P/0176/2013 of 30 July 2013.

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

Novartis Europharm Limited; Treatment of rhabdomyosarcoma / Treatment of non-rhabdomyosarcoma soft tissue sarcoma / Treatment of Ewing sarcoma family of tumours

Day 60 opinion

Oncology

Summary of committee discussion:

This procedure was discussed at Day 30 during the July 2020 plenary meeting.
The PDCO took into consideration the additional information provided by the applicant after the D30 discussion.
The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-000601-PIP01-09
- EMEA-C3-000601-PIP01-09-M01
- EMEA-C4-000601-PIP01-09-M03

The PDCO adopted on 24 July 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision P/0333/2019 of 11/09/2019.

2.2.5. Clostridium Botulinum neurotoxin type A (150 kD), free of complexing proteins - EMEA-C-001039-PIP02-12-M04

Merz Pharmaceuticals GmbH; Treatment of sialorrhea

Day 30 Opinion

Neurology

Summary of committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:

- partial compliance procedure EMEA-C1-001039-PIP02-12-M02

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision P/0125/2020 of 30 March 2020.

2.2.6. Delamanid - EMEA-C-001113-PIP01-10-M06

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of multi-drug-resistant-tuberculosis

Day 30 Opinion

Infectious Diseases

Summary of committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedure:

- EMEA-C1-001113-PIP01-10-M06

The PDCO adopted on 24 July 2020 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0271/2019) of 14 August 2019.

2.2.7. Spheroids of human autologous matrix-associated chondrocytes - EMEA-C-001264-PIP01-12-M02

CO.DON AG; Treatment of cartilage defects

Day 30 Opinion

Other

Summary of committee discussion:

The PDCO adopted an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0161/2018) of 15 June 2018.

2.2.8. Pegcetacoplan - EMEA-C1-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal Nocturnal Haemoglobinuria

Day 30 letter

Haematology-Hemostaseology

Summary of committee discussion:

The PDCO noted that the initiation date of study 2, as listed in part B of the report is confirmed to be compliant as set out in the EMA's Decision P/0149/2020 of 17 April 2020.

2.2.9. 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 letter

Oncology

Summary of committee discussion:

The PDCO re-discussed the compliance check request for Study 4 taking into account the clarifications provided by the applicant after D30.

The PDCO therefore concluded that the study was completed in compliance with the latest Agency's Decision P/0007/2020 of 6 January 2020.
The PDCO finalised this partially completed compliance procedure on 24 July 2020.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

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

Eli Lilly and Company Ltd; Treatment of pulmonary arterial hypertension / Treatment of benign prostatic hyperplasia

Day 60 opinion

Cardiovascular Diseases

Summary of committee discussion:

The PDCO re-discussed this application in line with the outcome conclusions from the D30 discussion.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency’s latest decision (P/0395/2018 of 7 November 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.2. 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 60 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could 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/0017/2020 of 06/01/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Allergan Pharmaceuticals International Limited; Treatment of non-alcoholic steatohepatitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant's response to the D30 issues was considered 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 (P/0392/2018 of 7 December 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Albireo AB; Treatment of progressive familial intrahepatic cholestasis

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/0316/2019 of 11 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.5. Potassium chloride / sodium chloride / citric acid (as citric acid anhydrous) / sodium citrate / simeticone / sodium sulfate (as sodium sulfate anhydrous) / macrogol 4000 - EMEA-001356-PIP02-12-M03

Alfasigma S.p.A.; Bowel cleansing prior to clinical procedures

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The PDCO confirmed the outcome of the discussion at Day 30.
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/0223/2018 of 17/07/2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Pfizer Europe MA EEIG; 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 some of the proposed changes could be accepted.
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/0165/2020 of 24 March 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Takeda Pharma A/S; Treatment of Crohn's Disease / Treatment of ulcerative colitis

Day 60 opinion

Gastroenterology-Hepatology

Summary of committee discussion:

The applicant's responses to the issues raised on D30 were considered 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 (P/0109/2018 of 11 April 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

AbbVie Ltd; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, spondyloarthritis and juvenile idiopathic arthritis)

Day 60 opinion

Immunology-Rheumatology-Transplantation

Summary of committee discussion:

The PDCO discussed the clarification received by the applicant.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0322/2019 of 11 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

Day 60 opinion

Immunology-Rheumatology-Transplantation / Oncology

Summary of committee discussion:

The PDCO's views expressed at D30 were endorsed.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0197/2017 of 14 July 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

This procedure was discussed at Day 60 during the July plenary meeting.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0403/2018 of 19 December 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO discussed the proposed modification considering the clarifications provided by the applicant after the D30 discussion.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0312/2017 of 30 October 2017).

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

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

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/0266/2018 of 14 August 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.13. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M03

ViiV Healthcare UK Limited; Treatment of Human Immunodeficiency virus type 1 (HIV-1) infection

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO in July 2020 noted the responses of the applicant on the issues raised at D30.
Therefore, PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0319/2019 of 11/9/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Shire Pharmaceuticals Ireland Limited; Treatment of cytomegalovirus infection

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO discussed this application in line with the assessors' comments.
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/0297/2019 of 12 August 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.15. Oseltamivir (phosphate) - EMEA-000365-PIP01-08-M11

Roche Registration GmbH; Treatment and prevention of influenza

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO's views expressed at D30 were endorsed.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0251/2018 of 14 August 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

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, and the discussions in the plenary, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0337/2019 of 10/09/2019).

2.3.17. Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M03

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

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO in July 2020 noted the responses of the applicant on the issues raised at D30.
Therefore, PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0319/2019 of 11/9/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

infection

Day 60 opinion

Infectious Diseases

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0339/2016 of 2/12/2016).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

LABORATOIRE AGUETTANT; Bronchopulmonary dysplasia.

Day 60 opinion

Neonatology - Paediatric Intensive Care

Summary of committee discussion:

The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency' s latest decision (P/0407/2019 of 4 December 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

Day 60 opinion

Neurology

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/0047/2020 of 29 January 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

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, and the responses to the comments raised at D30 the PDCO

considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0059/2019 of 28/2/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

AveXis EU Limited; Treatment of spinal muscular atrophy

Day 60 opinion

Neurology

Summary of committee discussion:

This procedure was discussed at Day 60 during the July plenary meeting.
Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0315/2019 of 11 September 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.23. Ozanimod (hydrochloride) - EMEA-001710-PIP02-14-M05

Celgene Europe B.V.; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of committee discussion:

The Applicant's response was discussed by the PDCO at Day 60.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0345/2017 of 23 November 2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Y-mAbs Therapeutics A/S; Treatment of neuroblastoma

Day 60 opinion

Oncology

Summary of committee discussion:

During its plenary on 24 July 2020, the PDCO discussed the applicant's responses to the day 30 outstanding issues.
In conclusion, all modification proposals from the applicant could be supported by the PDCO.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0405/2019 of 04/12/2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

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

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed this application, taking into consideration the additional information received after the D30 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/0261/2019 of 16 July 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 60 opinion

Oncology

Summary of committee discussion:

The PDCO re-discussed the proposed modification taking into account the clarifications provided by the applicant after D30 and their comments on the draft opinion.
In summary, the proposed changes for study 4 were considered acceptable.
In conclusion, the PDCO adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0163/2019 of 26 April 2019).

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

AbbVie Ltd; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of solid malignant tumours

Day 60 opinion

Oncology / Haematology-Hemostaseology

Summary of committee discussion:

The PDCO re-discussed this application taking into consideration the additional information received after the D30 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/0246/2019 of 17 July 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

GenSight-Biologics; Treatment of Leber hereditary optic neuropathy

Day 60 opinion

Ophthalmology

Summary of committee discussion:

The PDCO confirmed the outcome of the discussion at Day 30. 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/0166/2020 of 24/04/2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

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

Portola Netherlands B.V.; Treatment of factor Xa inhibitor associated haemorrhage / Prevention of factor Xa inhibitor associated haemorrhage

Day 60 opinion

Other

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/0243/2018 of 15 August 2018).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.30. Vilanterol / fluticasone (furoate) - EMEA-000431-PIP01-08-M11

Glaxo Group Limited; 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/0157/2017 of 9/6/2017).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.31. 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 (DCR-PHXC) - Orphan - EMEA-002493-PIP01-18-M01

Dicerna Ireland Limited; Treatment of primary hyperoxaluria

Day 60 opinion

Uro-nephrology

Summary of committee discussion:

The applicant's responses to the questions raised at Day 30 were considered acceptable by the committee. 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/0426/2019 of 6 December 2019).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.32. 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 60 opinion

Vaccines

Summary of committee discussion:

The PDCO' s views expressed at the D30 were endorsed. The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency' s latest decision (P/0037/2020 of 29 January 2020).
The new PDCO Opinion on the modified agreed PIP supersedes the previous PDCO Opinion.

2.3.33. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M15

UCB Pharma S.A.; Treatment of neonatal seizures / Treatment of epilepsy with partial onset seizures

Day 30 opinion

Neurology

Summary of committee discussion:

The PDCO discussed this application in line with the assessors' comments.
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 with an Opinion agreed at D30.
The PDCO therefore adopted a favourable Opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0297/2019 of 12 August 2019).

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 items

2.6. Finalisation and adoption of opinions

No items

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. Pegcetacoplan - EMEA-C1-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 30 letter

Haematology-Hemostaseology

2.7.2. Etrolizumab - EMEA-C1-001434-PIP01-13-M03

Roche Registration GmbH; Treatment of Crohn’s disease

Day 30 letter

Gastroenterology-Hepatology

2.7.3. Ixekizumab - EMEA-C3-001050-PIP01-10-M05

Eli Lilly and Company Limited; Treatment of Psoriasis

Day 30 letter

Dermatology

2.7.4. Avalglucosidase alfa - EMEA-C1-001945-PIP01-16-M02

Genzyme Europe B.V.; Treatment of Pompe disease

Day 30 letter

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.5. Maralixibat chloride - EMEA-C1-001475-PIP03-17-M02

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 letter

Gastroenterology-Hepatology

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. Glycopyrronium - EMEA-002383-PIP01-18

Hyperhidrosis / Treatment of primary axillary hyperhidrosis

Day 90 discussion

Dermatology

3.1.2. Ruxolitinib - EMEA-002618-PIP01-19

Vitiligo

Day 90 discussion

Dermatology

3.1.3. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the *BCL11A* gene - Orphan - EMEA-002730-PIP02-19

Vertex Pharmaceuticals (Ireland) Limited; Treatment of sickle cell disease

Day 90 discussion

Haematology-Hemostaseology

3.1.4. Danicopan - Orphan - EMEA-002310-PIP01-17

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria / Treatment of paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Haematology-Hemostaseology

3.1.5. Plasma kallikrein inhibitor - EMEA-002723-PIP01-19

Treatment of Hereditary Angioedema

Day 90 discussion

Haematology-Hemostaseology

3.1.6. Doravirine / islatravir - EMEA-002707-PIP01-19

Treatment of human immunodeficiency virus-1 (HIV-1) infection / Doravirine/Islatravir is indicated alone or in combination with other antiretroviral medicinal products for the treatment of HIV-1 infection in paediatric patients from 28 days to less than 18 years of age.

Day 90 discussion

Infectious Diseases

3.1.7. Mixture of 2 synthetic double-stranded N-acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against the hepatitis B virus - EMEA-002694-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 90 discussion

Infectious Diseases

3.1.8. EMEA-002693-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 90 discussion

Infectious Diseases

3.1.9. The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood (Hau-UCB-mnc) - Orphan - EMEA-001799-PIP03-19

BrainRepair UG (haftungsbeschränkt); Periventricular leukomalacia (PVL)

Day 90 discussion

Neonatology - Paediatric Intensive Care

3.1.10. RAAVrh74.MHCK7. microdystrophin - Orphan - EMEA-002677-PIP01-19

Sarepta Therapeutics Ireland; Duchenne Muscular Dystrophy

Day 90 discussion

Neurology

3.1.11. Autologous CD4 and CD8 T-cells that have been transduced with a self-inactivating lentiviral vector expressing an affinity enhanced NY-ESO-1 specific T-Cell Receptor - Orphan - EMEA-002476-PIP02-19

GlaxoSmithKline (Ireland) Limited; Soft tissue sarcoma

Day 90 discussion

Oncology

3.1.12. Efbemalenograstim alfa - EMEA-002507-PIP02-19

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 patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 90 discussion

Oncology

3.1.13. 17-mer, 2'-O-methyl modified phosphorothioate RNA oligonucleotide - Orphan - EMEA-002717-PIP01-19

ProQR Therapeutics; Treatment of inherited retinal disorders / Treatment of Leber's congenital amaurosis due to the p.Cys998X mutation (C2991 +1655A>G) in the CEP290 gene

Day 90 discussion

Ophthalmology

3.1.14. 4-{(2S,4S)-4-Ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl} benzoic acid hydrochloride(1/1) - Orphan - EMEA-002705-PIP01-19

Novartis Europharm Limited; C3 Glomerulopathy

Day 90 discussion

Other

3.1.15. EMEA-002705-PIP02-19

IgA Nephropathy

Day 90 discussion

Other

3.1.16. Finerenone - EMEA-001623-PIP02-20

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

Day 60 discussion

Cardiovascular Diseases

3.1.17. EMEA-002778-PIP01-20

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

Day 60 discussion

Diagnostic

3.1.18. 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 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. 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 (ICD-10 E76.2) / Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome) in children from birth to less than 18 years of age

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. 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 60 discussion

Gastroenterology-Hepatology

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

Systemic lupus erythematosus

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.22. Telitacicept - EMEA-002824-PIP01-20

Treatment of systemic lupus erythematosus

Day 60 discussion

Immunology-Rheumatology-Transplantation

3.1.23. 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 60 discussion

Neurology

3.1.24. 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 60 discussion

Oncology

3.1.25. 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 60 discussion

Ophthalmology

3.1.26. Zilucoplan - EMEA-002747-PIP01-20

Treatment of Myasthenia Gravis

Day 60 discussion

Other / Neurology

3.1.27. Sparsentan - EMEA-001984-PIP03-20

Treatment of IgA Nephropathy (IgAN)

Day 60 discussion

Uro-nephrology

3.1.28. Colchicine - EMEA-002837-PIP01-20

Secondary prevention of atherothrombotic events in patients with coronary artery disease by

reducing local plaque inflammation and subsequent plaque erosion and/or rupture

Day 30 discussion

Cardiovascular Diseases

3.1.29. Fenofibrate / Ezetimibe / Pravastatin - EMEA-002835-PIP01-20

Treatment of mixed hyperlipidaemia

Day 30 discussion

Cardiovascular Diseases

3.1.30. Dupilumab - EMEA-001501-PIP06-20

Prurigo Nodularis

Day 30 discussion

Dermatology

3.1.31. Dupilumab - EMEA-001501-PIP07-20

Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

3.1.32. LM-030 - Orphan - EMEA-002770-PIP02-20

LifeMax Laboratories, Inc.; Netherton Syndrome

Day 30 discussion

Dermatology

3.1.33. Adeno-associated virus serotype 5- (AAV5-) based vector that contains the human phenylalanine hydroxylase (*hPAH)* gene - Orphan - EMEA-002833-PIP01-20

BioMarin International Limited; Treatment of phenylalanine hydroxylase deficiency / Treatment of patients with Phenylketonuria

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.34. Dasiglucagon - Orphan - EMEA-002233-PIP02-20

Zealand Pharma; Congenital Hyperinsulinism

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. Hydroxypropyl-β-cyclodextrin (HPβCD) - Orphan - EMEA-002839-PIP01-20

Cyclo Therapeutics Inc; Niemann Pick disease type C / Treatment of Niemann Pick type C1 disease in children, adolescents and adults

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.36. Liposomal annamycin - EMEA-002810-PIP01-20

Treatment of acute myeloid leukaemia / Treatment of relapsed acute myeloid leukaemia

Day 30 discussion

Haematology-Hemostaseology

3.1.37. EMEA-002350-PIP02-20

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis) / Treatment of juvenile psoriatic arthritis (JPsA) in paediatric patients 6 years of age and older

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.38. Fenebrutinib - EMEA-002349-PIP02-20

Chronic idiopathic arthritis (including RA, axial spondyloarthritis, PsA, and JIA)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.39. Human plasma derived c1-inhibitor - EMEA-002818-PIP01-20

Pre-procedure prevention of acute hereditary angioedema (HAE) / Treatment of hereditary angioedema (HAE)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.40. Dabrafenib - EMEA-001147-PIP02-20

Treatment of glioma / Treatment of paediatric patients with glioma with a BRAF V600 mutation in combination with trametinib

Day 30 discussion

Oncology

3.1.41. Bimekizumab - EMEA-002189-PIP04-20

Treatment of hidradenitis suppurativa / Treatment of moderate to severe hidradenitis suppurativa in adolescents from 12 years of age

Day 30 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.42. Risankizumab - EMEA-001776-PIP05-20

Hidradenitis suppurativa

Day 30 discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.43. 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting glial fibrillary acidic protein pre-mRNA - Orphan - EMEA-002822-PIP01-20

Ionis Pharmaceuticals; Alexander disease

Day 30 discussion

Neurology

3.1.44. Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains - EMEA-002823-PIP01-20

Treatment of multiple myeloma / Treatment of patients with relapsed and/or refractory multiple myeloma (RRMM) who have received at least one proteasome inhibitor (PI), one immunomodulatory agent (IMiD), and one anti-CD38 monoclonal antibody (mAb). The patients shall have received at least 3 prior lines of therapy for MM.

Day 30 discussion

Oncology

3.1.45. Erdafitinib - EMEA-002042-PIP02-20

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of locally advanced or metastatic solid tumors, including primary CNS tumors, harboring susceptible FGFR alterations in patients who have progressed following prior therapies and those who have no acceptable standard therapies

Day 30 discussion

Oncology

3.1.46. Imetelstat - Orphan - EMEA-001910-PIP03-20

Geron Corporation; Treatment of Acute Myeloid Leukemia (AML) / Treatment of Myelodysplastic Syndromes (MDS), including Juvenile Myelomonocytic Leukemia (JMML) / Treatment of paediatric patients with relapsed or refractory AML or MDS, including JMML, from 1 year to less than 18 years of age

Day 30 discussion

Oncology

3.1.47. Sacituzumab govitecan - EMEA-002645-PIP02-20

Treatment of urothelial carcinoma

Day 30 discussion

Oncology

3.1.48. Tiragolumab - EMEA-002721-PIP02-20

Treatment of cervical cancer

Day 30 discussion

Oncology

3.1.49. Tiragolumab - EMEA-002721-PIP03-20

Treatment of esophageal carcinoma

Day 30 discussion

Oncology

3.1.50. Trametinib - EMEA-001177-PIP02-20

Treatment of glioma / Treatment of paediatric patients with glioma with a BRAF V600 mutation in combination with dabrafenib

Day 30 discussion

Oncology

3.1.51. Alpha-R-lipoic acid choline ester tosilate - EMEA-002811-PIP01-20

Treatment of Presbyopia

Day 30 discussion

Ophthalmology

3.1.52. Brolucizumab - EMEA-002691-PIP02-20

Diabetic retinopathy

Day 30 discussion

Ophthalmology

3.1.53. Recombinant humanised monoclonal antibody (IgG1, Kappa) to IL-5 - EMEA-002836-PIP01-20

Treatment of Asthma / Add-on treatment for patients (adolescents aged 12 years and older) with severe asthma and an eosinophilic phenotype.

Day 30 discussion

Pneumology - Allergology

3.1.54. Conditionally replication-defective human cytomegalovirus (CMV) - EMEA-002838-PIP01-20

Congenital CMV infection and disease / Prevention of congenital CMV infection and disease

Day 30 discussion

Vaccines

3.1.55. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002821-PIP01-20

Prevention of RSV- associated lower respiratory tract illness through maternal immunization / Active immunization of pregnant women during the second and third trimester of pregnancy to prevent respiratory syncytial virus (RSV) -associated lower respiratory tract illness (LRTI) in infants by transfer of maternal antibodies.

Day 30 discussion

Vaccines

3.1.56. Tocilizumab - EMEA-000309-PIP05-20 (discussed at the extraordinary PDCO plenary meeting held on the 9th July 2020)

Treatment of coronavirus disease 19 (COVID19)

Scope: discussion

Infectious diseases

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. Evinacumab - EMEA-C1-002298-PIP01-17-M01

Regeneron Ireland DAC; Treatment of elevated cholesterol

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.2.2. Elivaldogene autotemcel - EMEA-C-001244-PIP01-11-M02

bluebird bio (Netherlands) B.V.; Treatment of adrenoleukodystrophy

Day 30 discussion

Neurology

3.2.3. Ad26.ZEBOV (recombinant, replication-incompetent) - EMEA-C2-002307-PIP01-17-M01

Janssen-Cilag International NV; Prevention of Ebola Virus Disease

Day 30 discussion

Vaccines

3.2.4. MVA-BN-Filo (recombinant, non-replicating) - EMEA-C2-002308-PIP01-17-M01

Janssen-Cilag International NV; Prevention of Ebola Virus Disease

Day 30 discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Etripamil - EMEA-002303-PIP01-17-M02

Milestone Pharmaceuticals, Inc.; Treatment of supraventricular tachychardia / Treatment of acute paroxysmal supraventricular tachycardia (PSVT)

Day 30 discussion

Cardiovascular Diseases

3.3.2. Edoxaban tosylate - EMEA-000788-PIP02-11-M10

Daiichi Sankyo Europe GmbH; Prevention of arterial thromboembolism, Prevention of venous thromboembolism, Treatment of venous thromboembolism / Prevention of arterial thromboembolism in paediatric cardiac patients at risk of thrombotic events, Acute treatment & secondary prevention of symptomatic recurrent venous thrombotic events (VTE) in paediatric patients at risk

Day 30 discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.3. Birch bark extract - Orphan - EMEA-001299-PIP03-17-M01

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 30 discussion

Dermatology

3.3.4. Velmanase alfa - Orphan - EMEA-001056-PIP02-12-M01

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

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Ozanimod hydrochloride - EMEA-001710-PIP03-17-M02

Celgene Europe B.V.; Treatment of ulcerative colitis / Treatment of moderate to severely active ulcerative colitis

Day 30 discussion

Gastroenterology-Hepatology

3.3.6. Pegylated-fibroblast growth factor 21 - EMEA-002448-PIP01-18-M01

Bristol-Myers Squibb International Corporation; Treatment of non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis

Day 30 discussion

Gastroenterology-Hepatology

3.3.7. Mepolizumab - Orphan - EMEA-000069-PIP01-07-M07

GSK Trading Services Limited; Treatment of Hypereosinophilic Syndrome [HES]

Day 30 discussion

Haematology-Hemostaseology

3.3.8. Vadadustat - EMEA-001944-PIP01-16-M02

Akebia Therapeutics, Inc.; Treatment of anaemia due to chronic disorders / Treatment of anaemia secondary to chronic kidney disease

Day 30 discussion

Haematology-Hemostaseology

3.3.9. Zanamivir - EMEA-001318-PIP01-12-M04

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

Day 30 discussion

Infectious Diseases

3.3.10. Eculizumab - Orphan - EMEA-000876-PIP05-15-M04

Alexion Europe SAS; Treatment of myasthenia gravis / Treatment of refractory acetylcholine receptor antibody (AChR-Ab) - positive generalised myasthenia gravis

Day 30 discussion

Neurology

3.3.11. Copanlisib dihydrochloride - Orphan - EMEA-001757-PIP02-15-M02

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of mature B-cell neoplasms / Treatment of children with a relapsed or refractory neuroblastoma, Ewing sarcoma, osteosarcoma or rhabdomyosarcoma including at first relapse, in combination with chemotherapy

Day 30 discussion

Oncology

3.3.12. Dabrafenib - EMEA-001147-PIP01-11-M07

Novartis Europharm Limited; Treatment of solid malignant tumours (excluding melanoma and glioma), 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 30 discussion

Oncology

3.3.13. Daratumumab - Orphan - EMEA-002152-PIP01-17-M02

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with a lymphoid malignancy (except mature B-cell neoplasms)

Day 30 discussion

Oncology

3.3.14. Lenvatinib - EMEA-001119-PIP02-12-M07

Eisai GmbH; Treatment of osteosarcoma, Treatment of papillary thyroid carcinoma, Treatment

of follicular carcinoma / Treatment of refractory or relapsed osteosarcoma in children and adolescents, Treatment of progressive, radioiodine-refractory differentiated thyroid cancer in children and adolescents

Day 30 discussion

Oncology

3.3.15. Trametinib - EMEA-001177-PIP01-11-M06

Novartis Europharm Limited; Treatment of all conditions included in the category of malignant neoplasms (except melanoma, glioma, haematopoietic and lymphoid tissue), Treatment of melanoma / Treatment of paediatric patients with a solid malignant tumour with known or expected RAS, RAF or MEK pathway activation, Treatment of adolescent patients with melanoma containing BRAF V600 activating mutations

Day 30 discussion

Oncology

3.3.16. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M01

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

Other / Pneumology - Allergology

3.3.17. Dupilumab - EMEA-001501-PIP02-13-M06

sanofi-aventis recherche & développement; Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3.18. Peanut Allergen Extract - EMEA-001481-PIP01-13-M04

DBV Technologies S.A.; peanut allergy

Day 30 discussion

Pneumology - Allergology

3.3.19. Ravulizumab - Orphan - EMEA-001943-PIP01-16-M05

Alexion Europe SAS; Treatment of atypical Haemolytic Uremic Syndrome

Day 30 discussion

Uro-nephrology / Haematology-Hemostaseology

3.3.20. Ravulizumab - Orphan - EMEA-002077-PIP01-16-M03

Alexion Europe SAS; Treatment of Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Uro-nephrology / Haematology-Hemostaseology

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 submission of applications with start of procedure 18 August 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

No item

4.3. Nominations for other activities

Summary of committee discussion:

The PDCO approved the nomination of Dr. Javier de Cristobal, non-clinical assessor at the Spanish Agency (AEMPS), to the NcWG.

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

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. Selective estrogen receptor degrader - EMEA-06-2020

Sanofi-Aventis Recherche & Développement; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing of inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant neoplasm/ Treatment of estrogen receptor (ER)-positive, human epidermal growth factor receptor 2 (HER2)-negative breast cancer

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 indication 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

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

Denmark: Kirstine Moll Harboe – resigned membership on the 1st July 2020

Denmark: Nanna – membership mandate started on 1st July 2020

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 3 medicinal products with recommended paediatric indications adopted in June 2020 by CHMP. These include Kaftrio (elexacaftor / tezacaftor / ivacaftor), Cosentyx (secukinumab) and Epclusa (sofosbuvir / velpatasvir).

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

9.2.2. Committee for Advanced Therapies (CAT)

Improving CAT-PDCO interactions

Summary of committee discussion:

There is the intention to improve the collaboration between CAT and PDCO with the aim of maximising the benefit of each Committee's expertise. A brainstorming meeting will be held in September 2020, involving the chair and vice chair from CAT and PDCO and two additional members from each Committee.

9.2.3. Committee on Herbal Medicinal Products (HMPC)

HMPC request for PDCO input

Summary of committee discussion:

Following the discussion in June 2020 the PDCO noted the comments received and agreed an updated response to the HMPC.

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.

9.4. Cooperation within the EU regulatory network

No item

9.5. Cooperation with International Regulators

9.5.1. Report from the Paediatric Cluster Teleconference

Summary of committee discussion:

Minutes of the FDA cluster T-conference will be included in the post-mail.

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. Strategic Review and Learning Meeting (SRLM) under the German Presidency to be held virtually on 22nd October 2020

Summary of committee discussion:

The next SRLM meeting under the German presidency will host three committees: PDCO, CAT and COMP. The draft agenda for the joint session was presented and a call for topics for the PDCO only session was made.

10. Any other business

10.1.1. EMA Working Parties review

Summary of committee discussion:

The members were informed of the ongoing exercise in the review of the working parties. It was decided to include a separate session at the next PDCO meeting in order to consolidate the views of the PDCO members.

10.1.2. Covid-19 update

Summary of committee discussion:

The PDCO was updated on the most recent developments on treatments and vaccines, as well as on aspects of COVID19 disease particularly relevant to the paediatric population.

11. Breakout sessions

11.1.1. Paediatric oncology

Summary of committee discussion:

The breakout session was cancelled.

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 21-24 July 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:	**EMEA-000069-PIP01-07-M07** Mepolizumab **EMEA-002476-PIP02-19** Autologous CD4 and CD8 T-cells that have been transduced with a self-inactivating lentiviral vector expressing an affinity enhanced NY-ESO-1 specific T-Cell Receptor **EMEA-002836-PIP01-20** Recombinant humanised monoclonal antibody (IgG1, Kappa) to IL-5 **EMEA-001318-PIP01-12-M04** Zanamivir **EMEA-002821-PIP01-20** Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein **EMEA-002800-PIP01-20** Linerixibat **EMEA-000431-PIP01-08-M11** Vilanterol / fluticasone furoate
Karl-Heinz Huemer	Member	Austria	No interests declared	
Johanna Wernsperger	Alternate	Austria	No interests declared	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable for this meeting	
Petra Dominikova	Alternate	Czech Republic	No interests declared	
Nanna Borup Johansen	Member	Denmark	No interests declared	
Irja Lutsar	Member	Estonia	No interests declared	
Ann Marie Totterman	Member	Finland	No interests declared	
Sylvie Benchetrit	Member	France	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Dominique Ploin	Alternate	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	
Dina Apele-Freimane	Member	Latvia	No restrictions applicable for this meeting	
Sigita Burokiene	Member	Lithuania	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	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No interests declared	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Eva Agurell	Alternate	Sweden	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Fernando Cabanas	Member	Healthcare Professionals' Representative	No restrictions applicable for this meeting	
Jorrit Gerritsen	Alternate	Healthcare Professionals' Representative	No interests declared	
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to meetings	
Catherine Cornu	Alternate	Healthcare Professionals' Representative	No participation in final deliberations and voting on: the following product:	**EMEA-002493-PIP01-18-M01** 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 (DCR-PHXC)
Johannes Taminiau	Member	Healthcare Professionals' Representative	No interests declared	
Günter Karl-Heinz Auerswald	Member	Patients' Organisation Representative	No restrictions applicable for this meeting	
Paola Baiardi	Alternate	Patients' Organisation Representative	No restrictions applicable for this meeting	
Michal Odermarsky	Member	Patients' Organisation Representative	No restrictions applicable for this meeting	
Viviana Giannuzzi	Alternate	Patients' Organisation Representative	No restrictions applicable for this meeting	
Maria Estela Moreno Martín	expert	AEMPS	No interests declared	
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/