



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

25 June 2019
EMA/PDCO/309721/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 25-28 June 2019

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

25 June 2019, 14:00- 19:00, room 2D

26 June 2019, 08:30- 19:00, room 2D

27 June 2019, 08:30- 19:00, room 2D

28 June 2019, 08:30- 13:00, room 2D

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In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

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Note on access to documents

Some documents mentioned in the agenda 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 ongoing procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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Table of contents

1.	Introductions	8
1.1.	Welcome and declarations of interest of members, alternates and experts.....	8
1.2.	Adoption of agenda	8
1.3.	Adoption of the minutes	8
2.	Opinions	8
2.1.	Opinions on Products.....	8
2.1.1.	EMEA-002350-PIP01-18	8
2.1.2.	EMEA-002464-PIP01-18	8
2.1.3.	Oxalobacter formigenes strain HC-1 - Orphan - EMEA-000370-PIP02-18	9
2.1.4.	Humanized anti-CD19, Fc engineered, monoclonal antibody - Orphan - EMEA-002414-PIP01-18	9
2.1.5.	Vedolizumab - EMEA-000645-PIP03-18	9
2.1.6.	Bulevirtide - Orphan - EMEA-002399-PIP01-18.....	9
2.1.7.	Recombinant adeno-associated viral vector serotype 2 carrying the gene for the human aromatic L-amino acid decarboxylase protein - Orphan - EMEA-002435-PIP01-18.....	9
2.1.8.	Botulinum toxin type A - EMEA-002521-PIP01-18.....	10
2.1.9.	Recombinant humanized anti-blood dendritic cell antigen 2 (BDCA2) monoclonal antibody - EMEA-002555-PIP01-19	10
2.1.10.	EMEA-002577-PIP01-19	10
2.1.11.	Human chorionic gonadotrophin - EMEA-002547-PIP01-19	10
2.1.12.	Mifepristone - EMEA-001437-PIP02-19	10
2.1.13.	Istradefylline - EMEA-002540-PIP01-18.....	10
2.1.14.	EMEA-002575-PIP01-19	11
2.1.15.	EMEA-002573-PIP01-19	11
2.1.16.	Humanised Fc engineered monoclonal antibody against CD19 for the treatment of diffuse large B-cell lymphoma - Orphan - EMEA-002499-PIP02-19	11
2.1.17.	Ciclosporin - EMEA-002491-PIP02-19.....	11
2.1.18.	EMEA-002559-PIP01-19	11
2.2.	Opinions on Compliance Check	12
2.3.	Opinions on Modification of an Agreed Paediatric Investigation Plan	12
2.3.1.	Apixaban - EMEA-000183-PIP01-08-M07	12
2.3.2.	Fluciclovine (¹⁸ F) - Orphan - EMEA-001644-PIP02-14-M01	12
2.3.3.	Saxagliptin - EMEA-000200-PIP01-08-M08	12
2.3.4.	Belatacept - EMEA-000157-PIP01-07-M04	12
2.3.5.	Ixekizumab - EMEA-001050-PIP02-18-M01.....	13
2.3.6.	Recombinant human monoclonal antibody to GM-CSF - EMEA-001882-PIP02-16-M01	13
2.3.7.	Delamanid - Orphan - EMEA-001113-PIP01-10-M06.....	13

2.3.8.	Eslicarbazepine acetate - EMEA-000696-PIP02-10-M06	13
2.3.9.	Eteplirsén - Orphan - EMEA-001722-PIP01-14-M02.....	14
2.3.10.	Lasmiditan - EMEA-002166-PIP01-17-M02.....	14
2.3.11.	Recombinant human tripeptidyl peptidase 1 (rhTPP1) - Orphan - EMEA-001362-PIP01-12-M04	14
2.3.12.	Bosutinib - EMEA-000727-PIP01-09-M03	14
2.3.13.	Daunorubicin (liposomal formulation) / cytarabine (liposomal formulation) - Orphan - EMEA-001858-PIP02-16-M03	14
2.3.14.	Ixazomib - Orphan - EMEA-001410-PIP02-17-M02.....	15
2.3.15.	Selumetinib - Orphan - EMEA-001585-PIP01-13-M03.....	15
2.3.16.	Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M02	15
2.3.17.	Octenidine (dihydrochloride) - EMEA-001514-PIP01-13-M01	15
2.3.18.	Reslizumab - EMEA-001202-PIP02-13-M03	16
2.3.19.	Tezepelumab - EMEA-001613-PIP01-14-M03	16
2.3.20.	Mirabegron - EMEA-000597-PIP02-10-M07	16
2.4.	Opinions on Re-examinations	16
2.4.1.	Genetically modified replication-incompetent herpes simplex virus-1 expressing collagen VII - Orphan - EMEA-002472-PIP02-19.....	16
2.5.	Opinions on Review of Granted Waivers	16
2.6.	Finalisation and adoption of Opinions.....	17
2.7.	Partial Compliance Checks completed by EMA	17
2.7.1.	Ponesimod - EMEA-C1-000798-PIP01-09-M01	17
2.7.2.	Peanut (arachis hypogaea) allergen powder (previously known as Peanut flour) - EMEA-C1-001734-PIP01-14-M04	17

3. Discussion of applications 17

3.1.	Discussions on Products D90-D60-D30.....	17
3.1.1.	Birch bark extract - Orphan - EMEA-001299-PIP03-17	17
3.1.2.	Trifarotene cream HE1 - EMEA-001492-PIP02-18	18
3.1.3.	Levonorgestrel - EMEA-002474-PIP02-18	18
3.1.4.	Tirzepatide - EMEA-002360-PIP01-18.....	18
3.1.5.	EMEA-002448-PIP01-18	18
3.1.6.	Bimekizumab - EMEA-002189-PIP02-18	18
3.1.7.	Liposomal ciclosporin A (L-CsA) - Orphan - EMEA-002344-PIP02-18	19
3.1.8.	Baloxavir marboxil - EMEA-002440-PIP01-18	19
3.1.9.	Equine immunoglobulin F(ab') ₂ fragments targeting Shiga toxin - Orphan - EMEA-002444-PIP02-18	19
3.1.10.	Eptinezumab - EMEA-002243-PIP01-17	19
3.1.11.	Fosmetpantotenate - Orphan - EMEA-002036-PIP01-16	19
3.1.12.	Niraparib (as tosylate monohydrate) - Orphan - EMEA-002268-PIP02-18.....	20
3.1.13.	Aldesleukin - EMEA-002492-PIP01-18	20

3.1.14.	Pegvorhalyuronidase alfa - Orphan - EMEA-001883-PIP03-17	20
3.1.15.	Anti PD-1 monoclonal antibody - EMEA-002463-PIP01-18.....	20
3.1.16.	A fully human, IgG2 mAb - EMEA-002433-PIP01-18	21
3.1.17.	Remimazolam - EMEA-001880-PIP02-19	21
3.1.18.	EMEA-002568-PIP01-19	21
3.1.19.	Hydrogen peroxide - EMEA-001884-PIP03-18	21
3.1.20.	Norethisterone acetate / estradiol / relugolix - EMEA-002428-PIP02-18.....	21
3.1.21.	Dupilumab - EMEA-001501-PIP04-19	22
3.1.22.	CD34+enriched cells from patients with Fanconi anemia subtype A (FA-A) transduced ex vivo with lentiviral vector carrying the FANCA gene - Orphan - EMEA-002578-PIP01-19.....	22
3.1.23.	Probenecid / sulopenem etzadroxil - EMEA-002602-PIP01-19.....	22
3.1.24.	Sulopenem - EMEA-002478-PIP01-18	22
3.1.25.	Pracinostat - Orphan - EMEA-002567-PIP01-19	22
3.1.26.	Budesonide - Orphan - EMEA-002500-PIP01-18	23
3.1.27.	Nebivolol / zofenopril - EMEA-002593-PIP01-19	23
3.1.28.	Ramipril / rosuvastatin - EMEA-002569-PIP01-19	23
3.1.29.	Dihomo- γ -linolenic acid (DGLA) - EMEA-002364-PIP03-19.....	23
3.1.30.	Lebrikizumab - EMEA-002536-PIP01-18	23
3.1.31.	Deoxycytidine - Orphan - EMEA-002513-PIP01-18.....	23
3.1.32.	Deoxythymidine - Orphan - EMEA-002624-PIP01-19.....	24
3.1.33.	Pegcetacoplan - Orphan - EMEA-002600-PIP01-19.....	24
3.1.34.	Rivipansel sodium - Orphan - EMEA-002596-PIP01-19	24
3.1.35.	Baricitinib - EMEA-001220-PIP05-19	24
3.1.36.	Mavorixafor - EMEA-002490-PIP01-18.....	24
3.1.37.	Polymyxin B - EMEA-002595-PIP01-19.....	25
3.1.38.	Zoliflodacin - EMEA-002599-PIP01-19	25
3.1.39.	EMEA-002572-PIP01-19	25
3.1.40.	Belantamab mafodotin - Orphan - EMEA-002468-PIP04-19.....	25
3.1.41.	Infigratinib - EMEA-002594-PIP01-19.....	25
3.1.42.	Adeno-associated viral vector serotype 8 containing the human RPGR gene - Orphan - EMEA-002601-PIP01-19	26
3.1.43.	Difelikefalin - EMEA-002565-PIP01-19.....	26
3.1.44.	Obidoxime chloride / atropine sulfate - EMEA-002570-PIP01-19	26
3.1.45.	Fexapotide trifluate - EMEA-002598-PIP01-19.....	26
3.1.46.	Recombinant hepatitis B vaccine - EMEA-002157-PIP01-17.....	26
3.2.	Discussions on Compliance Check.....	27
3.2.1.	Etravirine - EMEA-C-000222-PIP01-08-M09	27
3.3.	Discussions on Modification of an Agreed Paediatric Investigation Plan.....	27
3.3.1.	Sacubitril / valsartan - EMEA-000316-PIP02-11-M04.....	27

3.3.2.	Ticagrelor - EMEA-000480-PIP01-08-M12	27
3.3.3.	Liquid ethanolic extract 30 per cent (w/w) of <i>Allium cepa</i> L. (fresh bulb) and <i>Citrus limon</i> (L.) Burm. f. (fresh fruit), <i>Paullinia cupana</i> Kunth, <i>Theobroma cacao</i> L. - EMEA-001835-PIP01-15- M04	27
3.3.4.	2-hydroxypropyl- β -cyclodextrin (HP- β -CD) - Orphan - EMEA-001866-PIP01-15-M04.....	28
3.3.5.	Empagliflozin - EMEA-000828-PIP04-16-M03	28
3.3.6.	Semaglutide - EMEA-001441-PIP03-17-M01	28
3.3.7.	Etrolizumab - EMEA-001434-PIP01-13-M02	28
3.3.8.	Linaclotide - EMEA-000927-PIP01-10-M04	28
3.3.9.	Odevixibat - Orphan - EMEA-002054-PIP01-16-M01	28
3.3.10.	Ferric maltol - EMEA-001195-PIP01-11-M04	29
3.3.11.	Lonoctocog alfa - EMEA-001215-PIP01-11-M07	29
3.3.12.	Upadacitinib - EMEA-001741-PIP01-14-M02.....	29
3.3.13.	Ustekinumab - EMEA-000311-PIP03-11-M05.....	29
3.3.14.	Lamivudine (3TC) / dolutegravir (DTG) - EMEA-001940-PIP01-16-M02	29
3.3.15.	Nirsevimab - anti-respiratory syncytial virus human IgG1k monoclonal antibody - EMEA- 001784-PIP01-15-M01	30
3.3.16.	Pretomanid - Orphan - EMEA-002115-PIP01-17-M01	30
3.3.17.	Arimoclomol citrate - Orphan - EMEA-001748-PIP01-15-M02	30
3.3.18.	Ataluren - Orphan - EMEA-000115-PIP01-07-M10.....	30
3.3.19.	Brivaracetam - Orphan - EMEA-000332-PIP01-08-M14.....	30
3.3.20.	Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M02	31
3.3.21.	Risdiplam - Orphan - EMEA-002070-PIP01-16-M03.....	31
3.3.22.	Cabozantinib - Orphan - EMEA-001143-PIP01-11-M02	31
3.3.23.	Cobimetinib - EMEA-001425-PIP01-13-M04	31
3.3.24.	Larotrectinib - Orphan - EMEA-001971-PIP02-16-M02.....	31
3.3.25.	Pazopanib - EMEA-000601-PIP01-09-M06.....	32
3.3.26.	Tisagenlecleucel - Orphan - EMEA-001654-PIP02-17-M01.....	32
3.3.27.	Human thrombin / human fibrinogen - EMEA-001149-PIP01-11-M05	32
3.3.28.	Lomitapide - EMEA-001124-PIP01-10-M04.....	32
3.3.29.	<i>Dermatophagoides farinae</i> / <i>dermatophagoides pteronyssinus</i> - EMEA-001258-PIP01-11-M05	32
3.3.30.	Fevipirant - EMEA-001315-PIP02-16-M02	33
3.3.31.	Finerenone - EMEA-001623-PIP01-14-M03	33
3.3.32.	Influenza virus surface antigens (haemagglutinin) of strain A (H1N1) / influenza virus surface antigens (haemagglutinin) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin) of strain B (Yamagata lineage) - EMEA-002027-PIP02-17-M01	33
3.3.33.	Outer membrane vesicles (OMV) from <i>neisseria meningitidis</i> serogroup B strain NZ98/254 measured as amount of total protein containing the PorA P1.4 / Recombinant <i>neisseria</i> <i>meningitidis</i> serogroup B fHbp fusion protein / Recombinant <i>neisseria meningitidis</i> serogroup B	

	NadA protein / Recombinant neisseria meningitidis serogroup B NHBA fusion protein - EMEA-000139-PIP01-07-M03	33
3.3.34.	Prepandrix: A/Indonesia/05/2005 (H5N1) / Adjupanrix: split influenza virus, inactivated, containing antigen: A/VietNam/1194/2004 (H5N1) - EMEA-000160-PIP01-07-M05	34

4. Nominations 34

4.1.	List of letters of intent received for submission of applications with start of procedure 20 August 2019 for Nomination of Rapporteur and Peer reviewer	34
4.2.	Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver.	34
4.3.	Nominations for other activities	34

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction 34

5.1.	New Scientific Advice	34
5.2.	Ongoing Scientific Advice	34
5.3.	Final Scientific Advice (Reports and Scientific Advice letters)	35

6. Discussion on the applicability of class waivers 35

6.1.	Discussions on the applicability of class waiver for products.....	35
6.1.1.	NTHi-Mcat vaccine - EMEA-05-2019	35
6.1.2.	Pertuzumab/trastuzumab fixed dose combination - EMEA-08-2019	35
6.1.3.	Acumapimod - EMEA-09-2019	35

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver 36

7.1.	Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver	36
7.1.1.	Icosapent ethyl - EMEA-001300-PIP01-12	36
7.1.2.	Fc- and CDR-modified humanized monoclonal antibody against C5 - EMEA-001943-PIP01-16-M01	36
7.1.3.	Ticagrelor - EMEA-000480-PIP01-08-M11	36

8. Annual reports on deferrals 36

9. Organisational, regulatory and methodological matters 37

9.1.	Mandate and organisation of the PDCO.....	37
9.2.	Coordination with EMA Scientific Committees or CMDh-v	37
9.2.1.	Committee for Medicinal Products for Human Use (CHMP)	37
9.2.2.	EMA Scientific Committees – Timing for Chair elections	37
9.3.	Coordination with EMA Working Parties/Working Groups/Drafting Groups	37
9.3.1.	Non-clinical Working Group: Draft manuscript on the non-clinical CNS project.....	37
9.3.2.	Formulation Working Group	37
9.4.	Cooperation within the EU regulatory network.....	37

9.4.1.	CTFG: Recommendation Paper on the Initiation and Conduct of Complex Clinical Trials	37
9.5.	Cooperation with International Regulators.....	37
9.5.1.	Report from the Paediatric Cluster Teleconference	37
9.6.	Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee.....	38
9.7.	PDCO work plan.....	38
9.8.	Planning and reporting	38
9.8.1.	Marketing authorisation applications (MAA) forecast for 2019 – planning update dated Q2 2019	38
9.8.2.	A first perspective on the impact of the review of the class waiver list in July 2015	38
9.8.3.	Strategic Review and Learning Meeting (SRLM) under the Romanian Presidency held in Malta on 13-14 June 2019 - Report to the Committee.....	38
10.	Any other business	38
11.	Breakout sessions	38
11.1.1.	Paediatric oncology	38
11.1.2.	Neonatology	38
11.1.3.	Inventory	38
12.	Explanatory notes	39

1. Introductions

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

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 25-28 June 2019. See 25-28 June 2019 PDCO minutes (to be published post 23-26 July 2019 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 25-28 June 2019

1.3. Adoption of the minutes

PDCO minutes for 27-29 May 2019

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. EMEA-002350-PIP01-18

Treatment of psoriasis / Treatment of moderate to severe plaque psoriasis in paediatric patients 6 years of age and older

Day 120 Opinion

Action: For adoption

Dermatology

2.1.2. EMEA-002464-PIP01-18

Treatment of atopic dermatitis / Treatment of patients with moderate-to-severe atopic dermatitis.

Day 120 Opinion

Action: For adoption

Dermatology

2.1.3. Oxalobacter formigenes strain HC-1 - Orphan - EMEA-000370-PIP02-18

OxThera AB; ICD10-E72.53 (Hyperoxaluria) / Treatment of primary hyperoxaluria

Day 120 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

2.1.4. Humanized anti-CD19, Fc engineered, monoclonal antibody - Orphan - EMEA-002414-PIP01-18

Xencor, Inc.; Immunoglobulin G4-related disease / Treatment of adults, adolescents and children (> 23 months of age) with immunoglobulin G4-related disease

Day 120 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.5. Vedolizumab - EMEA-000645-PIP03-18

ICD-9-CM 279.51 / ICD-10-CM D89.810 - Other disorders involving the immune mechanism, not elsewhere classified: acute graft-versus-host disease

Day 120 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

2.1.6. Bulevirtide - Orphan - EMEA-002399-PIP01-18

MYR GmbH; Chronic hepatitis D infection

Day 120 Opinion

Action: For adoption

Infectious Diseases

2.1.7. Recombinant adeno-associated viral vector serotype 2 carrying the gene for the human aromatic L-amino acid decarboxylase protein - Orphan - EMEA-002435-PIP01-18

PTC Therapeutic International Limited; Aromatic L-amino acid decarboxylase (AADC) deficiency / Treatment of aromatic L-amino acid decarboxylase (AADC) deficiency

Day 120 Opinion

Action: For adoption

Neurology

2.1.8. Botulinum toxin type A - EMEA-002521-PIP01-18

Muscle-induced wrinkles

Day 60 Opinion

Action: For adoption

Dermatology

2.1.9. Recombinant humanized anti-blood dendritic cell antigen 2 (BDCA2) monoclonal antibody - EMEA-002555-PIP01-19

Cutaneous lupus erythematosus

Day 60 Opinion

Action: For adoption

Dermatology

2.1.10. EMEA-002577-PIP01-19

Prostate-specific membrane antigen (PSMA)-expressing metastatic prostate cancer

Day 60 Opinion

Action: For adoption

Diagnostic / Oncology

2.1.11. Human chorionic gonadotrophin - EMEA-002547-PIP01-19

Infertility / Assisted reproductive technology (ART) program such as in vitro fertilisation, anovulatory or oligo-ovulatory women

Day 60 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.12. Mifepristone - EMEA-001437-PIP02-19

Endometriosis

Day 60 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.13. Istradefylline - EMEA-002540-PIP01-18

Parkinson's disease

Day 60 Opinion

Action: For adoption

Neurology

2.1.14. [EMEA-002575-PIP01-19](#)

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 60 Opinion

Action: For adoption

Oncology

2.1.15. [EMEA-002573-PIP01-19](#)

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 60 Opinion

Action: For adoption

Oncology

2.1.16. [Humanised Fc engineered monoclonal antibody against CD19 for the treatment of diffuse large B-cell lymphoma - Orphan - EMEA-002499-PIP02-19](#)

MorphoSys AG; Diffuse large B-Cell lymphoma

Day 60 Opinion

Action: For adoption

Oncology

2.1.17. [Ciclosporin - EMEA-002491-PIP02-19](#)

Treatment of dry eye disease

Day 60 Opinion

Action: For adoption

Ophthalmology

2.1.18. [EMEA-002559-PIP01-19](#)

Haemolytic disease of the foetus and newborn (HDFN)

Day 60 Opinion

Action: For adoption

Other

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

No items.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Apixaban - EMEA-000183-PIP01-08-M07

Bristol-Myers Squibb / Pfizer EEIG; Prevention of arterial thromboembolism (TE) / Prevention of venous thromboembolism / Prevention of TE in paediatric patients with cardiac disease / Prevention of venous thromboembolism (VTE) in paediatric subjects with a newly diagnosed acute lymphoblastic leukemia (ALL) or lymphoma (T or B cell), a functioning central venous access device (CVAD) and receiving asparaginase during chemotherapy induction

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Fluciclovine (¹⁸F) - Orphan - EMEA-001644-PIP02-14-M01

Blue Earth Diagnostics Ireland Ltd; Diagnosis of amino acid metabolism in solid malignant tumours / Diagnosis of primary and recurrent brain tumours

Day 60 Opinion

Action: For adoption

Diagnostic / Oncology

2.3.3. Saxagliptin - EMEA-000200-PIP01-08-M08

AstraZeneca AB; E11 type 2 Diabetes / Treatment of type 2 Diabetes

Day 60 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Belatacept - EMEA-000157-PIP01-07-M04

Bristol-Myers Squibb Pharma EEIG; Prevention of rejection of transplanted kidney / in combination with corticosteroids and a mycophenolic acid (MPA), is indicated for prophylaxis of graft rejection in pediatric patients at least 12 years of age and with a stable renal transplant for at least 6 months, who convert to a calcineurin inhibitor (CNI) -free maintenance immunosuppressive regimen

Day 60 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.5. Ixekizumab - EMEA-001050-PIP02-18-M01

Eli Lilly Nederland B.V.; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis) / Children with juvenile idiopathic arthritis subtypes of enthesitis-related arthritis (including juvenile onset axial spondyloarthritis (JoAS)) and juvenile psoriatic arthritis.

Day 60 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.6. Recombinant human monoclonal antibody to GM-CSF - EMEA-001882-PIP02-16-M01

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

Day 60 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.7. Delamanid - Orphan - EMEA-001113-PIP01-10-M06

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

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.3.8. Eslicarbazepine acetate - EMEA-000696-PIP02-10-M06

BIAL - Portela & Ca, SA; Treatment of epilepsy with partial onset seizures / Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as adjunctive therapy. Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as monotherapy

Day 60 Opinion

Action: For adoption

Neurology

2.3.9. Eteplirsen - Orphan - EMEA-001722-PIP01-14-M02

Sarepta Therapeutics Ireland Limited; Duchenne muscular dystrophy

Day 60 Opinion

Action: For adoption

Neurology

2.3.10. Lasmiditan - EMEA-002166-PIP01-17-M02

Eli Lilly and Company Limited; Migraine with and without aura

Day 60 Opinion

Action: For adoption

Neurology

2.3.11. Recombinant human tripeptidyl peptidase 1 (rhTPP1) - Orphan - EMEA-001362-PIP01-12-M04

BioMarin International Limited; Neuronal ceroid lipofuscinosis type 2 (CLN2) disease / Treatment of neuronal ceroid lipofuscinosis Type 2 (NCL2)

Day 60 Opinion

Action: For adoption

Neurology

2.3.12. Bosutinib - EMEA-000727-PIP01-09-M03

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukemia (CML) / Treatment of chronic, accelerated or blast phase CML with resistance or intolerance to prior TKI therapy / Treatment of newly-diagnosed chronic phase of Philadelphia chromosome-positive chronic myeloid leukemia (Ph+ CML)

Day 60 Opinion

Action: For adoption

Oncology

2.3.13. Daunorubicin (liposomal formulation) / cytarabine (liposomal formulation) - Orphan - EMEA-001858-PIP02-16-M03

Jazz Pharmaceuticals Ireland Limited; Acute myeloid leukaemia / Treatment of acute myeloid leukaemia

Day 60 Opinion

Action: For adoption

Oncology

2.3.14. Ixazomib - Orphan - EMEA-001410-PIP02-17-M02

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-acute lymphoblastic leukemia (ALL) or T-ALL

Day 60 Opinion

Action: For adoption

Oncology

2.3.15. Selumetinib - Orphan - EMEA-001585-PIP01-13-M03

AstraZeneca AB; Treatment of thyroid cancer / Treatment of neurofibromatosis type 1 (NF1) / Selumetinib in combination with adjuvant radioactive iodine therapy is indicated for the treatment of adolescents newly diagnosed with differentiated thyroid cancer who are at high risk of primary treatment failure / Selumetinib is indicated for the treatment of inoperable NF1 related plexiform neurofibroma in children and adolescents

Day 60 Opinion

Action: For adoption

Oncology

2.3.16. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M02

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

Day 60 Opinion

Action: For adoption

Other

2.3.17. Octenidine (dihydrochloride) - EMEA-001514-PIP01-13-M01

Cassella-med GmbH & Co. KG; Treatment of upper respiratory tract infections / Treatment of sore-throat due to infectious pharyngitis

Day 60 Opinion

Action: For adoption

Oto-rhino-laryngology

2.3.18. Reslizumab - EMEA-001202-PIP02-13-M03

Teva Pharmaceuticals Europe; Treatment of asthma / Add-on treatment to reduce exacerbations, relieve symptoms and improve lung function in paediatric patients from 6 to less than 18 years of age with inadequately controlled severe asthma who have a blood eosinophil count greater than or equal to 300 μ L

Day 60 Opinion

Action: For adoption

Pneumology - Allergology

2.3.19. Tezepelumab - EMEA-001613-PIP01-14-M03

AstraZeneca AB; Treatment of asthma / Tezepelumab is indicated as add-on maintenance treatment of patients with severe asthma aged 5 years and older.

Day 60 Opinion

Action: For adoption

Pneumology - Allergology

2.3.20. Mirabegron - EMEA-000597-PIP02-10-M07

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

Day 60 Opinion

Action: For adoption

Uro-nephrology

2.4. Opinions on Re-examinations

2.4.1. Genetically modified replication-incompetent herpes simplex virus-1 expressing collagen VII - Orphan - EMEA-002472-PIP02-19

Krystal Biotech, Inc.; Dystrophic epidermolysis bullosa

Day 30 Opinion

Action: For adoption

Dermatology

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

For the following partial compliance checks, no need to refer them to PDCO Committee for discussion, were identified by the PME coordinator and PDCO Rapporteur. The PDCO has been informed in writing.

No items

2.7.1. Ponesimod - EMEA-C1-000798-PIP01-09-M01

Janssen-Cilag International NV; Treatment of multiple sclerosis

Day 1 letter

Action: For information

Other / Neurology

2.7.2. Peanut (*arachis hypogaea*) allergen powder (previously known as Peanut flour) - EMEA-C1-001734-PIP01-14-M04

Aimmune Therapeutics Netherlands B.V.; Treatment of peanut allergy

Day 1 letter

Action: For information

Pneumology - Allergology

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

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. Trifarotene cream HE1 - EMEA-001492-PIP02-18

Lamellar ichthyosis / Treatment of lamellar ichthyosis

Day 90 discussion

Action: For discussion

Dermatology

3.1.3. Levonorgestrel - EMEA-002474-PIP02-18

Contraception

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Tirzepatide - EMEA-002360-PIP01-18

Type 2 diabetes mellitus / Treatment of Type 2 diabetes mellitus

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. EMEA-002448-PIP01-18

Treatment of Non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.6. Bimekizumab - EMEA-002189-PIP02-18

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis) / Treatment of JIA (enthesitis-related arthritis (ERA) and juvenile psoriatic arthritis (JPsA)) in patients from ≥ 2 years to < 18 years of age

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.7. Liposomal ciclosporin A (L-CsA) - Orphan - EMEA-002344-PIP02-18

Breath Therapeutics GmbH; Treatment of bronchiolitis obliterans syndrome (BOS)

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.8. Baloxavir marboxil - EMEA-002440-PIP01-18

Prevention of influenza /Treatment of influenza / Treatment of influenza type A/B in otherwise healthy, high risk and hospitalised patients / Prevention (post-exposure prophylaxis) of influenza type A/B. Reduction of transmission of influenza type A/B.

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.9. Equine immunoglobulin F(ab')₂ fragments targeting Shiga toxin - Orphan - EMEA-002444-PIP02-18

Chemo Research, S.L.; Prevention of Shiga-toxin producing escherichia coli haemolytic uremic syndrome

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.10. Eptinezumab - EMEA-002243-PIP01-17

Prevention of migraine headaches / Prophylaxis of migraine

Day 90 discussion

Action: For discussion

Neurology

3.1.11. Fosmetpantotenate - Orphan - EMEA-002036-PIP01-16

Retrophin Europe Limited; Treatment of pantothenate kinase associated neurodegeneration (PKAN)

Day 90 discussion

Action: For discussion

Neurology

3.1.12. Niraparib (as tosylate monohydrate) - Orphan - EMEA-002268-PIP02-18

Tesaro UK Ltd; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / For the treatment of patients ≥ 6 months to < 18 years of age who have been diagnosed with recurrent solid tumours that exhibit a breast cancer susceptibility gene (BRCA)ness mutational signature (mutational signature 3)

Day 90 discussion

Action: For discussion

Oncology

3.1.13. Aldesleukin - EMEA-002492-PIP01-18

Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue) / Treatment of a relapsed or refractory paediatric malignant solid tumour in paediatric patients less than 18 years old / Treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old

Day 90 discussion

Action: For discussion

Oncology

3.1.14. Pegvorhialuronidase alfa - Orphan - EMEA-001883-PIP03-17

Halozyme Inc; Treatment of solid malignant tumours (except central nervous system tumours, haematopoietic and lymphoid tissue tumours) / Pegvorhialuronidase alfa is indicated in combination with cytotoxic cancer therapies for the treatment of paediatric patients aged from birth to less than 18 years with relapsed or refractory solid tumours that accumulate high levels of hyaluronan

Day 90 discussion

Action: For discussion

Oncology

3.1.15. Anti PD-1 monoclonal antibody - EMEA-002463-PIP01-18

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / For the treatment of patients ≥ 6 months to < 18 years of age who have been diagnosed with recurrent solid tumours that exhibit a breast cancer susceptibility gene (BRCA)ness mutational signature (mutational signature 3)

Day 90 discussion

Action: For discussion

Oncology

3.1.16. A fully human, IgG2 mAb - EMEA-002433-PIP01-18

Asthma / Treatment of severe asthma in patients 6 year-olds and above as an add-on therapy of standard of care

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.17. Remimazolam - EMEA-001880-PIP02-19

Sedation during medical procedures / General anaesthesia and post-operative sedation up to 24h, sedation in the intensive care unit (ICU) / Sedation for short procedures

Day 60 discussion

Action: For discussion

Anaesthesiology

3.1.18. EMEA-002568-PIP01-19

Psoriasis / Treatment of moderate to severe chronic plaque-type psoriasis who are candidates for systemic therapy

Day 60 discussion

Action: For discussion

Dermatology

3.1.19. Hydrogen peroxide - EMEA-001884-PIP03-18

Treatment of common warts (verrucae vulgaris) / Topical treatment to remove common warts (verrucae vulgaris)

Day 60 discussion

Action: For discussion

Dermatology

3.1.20. Norethisterone acetate / estradiol / relugolix - EMEA-002428-PIP02-18

Endometriosis / Treatment of symptoms associated with endometriosis

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.21. Dupilumab - EMEA-001501-PIP04-19

Treatment of eosinophilic esophagitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.22. CD34+enriched cells from patients with Fanconi anemia subtype A (FA-A) transduced ex vivo with lentiviral vector carrying the FANCA gene - Orphan - EMEA-002578-PIP01-19

Rocket Pharmaceuticals, Inc.; Treatment of Fanconi anaemia subtype A

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.23. Probenecid / sulopenem etzadroxil - EMEA-002602-PIP01-19

Urinary tract infections / Abdominal and gastrointestinal infections / Uncomplicated urinary tract infections / Complicated intra-abdominal infections / Complicated urinary tract infections

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.24. Sulopenem - EMEA-002478-PIP01-18

Urinary tract Infections / Abdominal and gastrointestinal infections / Uncomplicated urinary tract infections / Complicated intra-abdominal infections / Complicated urinary tract infections

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.25. Pracinostat - Orphan - EMEA-002567-PIP01-19

Day 60 discussion

Action: For discussion

Oncology

3.1.26. Budesonide - Orphan - EMEA-002500-PIP01-18

Calliditas Therapeutics AB; Primary IgA nephropathy

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.27. Nebivolol / zofenopril - EMEA-002593-PIP01-19

Treatment of essential hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.28. Ramipril / rosuvastatin - EMEA-002569-PIP01-19

Treatment of cardiovascular disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.29. Dihomo- γ -linolenic acid (DGLA) - EMEA-002364-PIP03-19

Treatment of atopic dermatitis / Treatment of moderate to severe atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.1.30. Lebrikizumab - EMEA-002536-PIP01-18

Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.1.31. Deoxycytidine - Orphan - EMEA-002513-PIP01-18

Modis Therapeutics, Inc.; Treatment of thymidine kinase 2 deficiency

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.32. Deoxythymidine - Orphan - EMEA-002624-PIP01-19

Modis Therapeutics, Inc.; Treatment of thymidine kinase 2 deficiency

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.34. Rivipansel sodium - Orphan - EMEA-002596-PIP01-19

Pfizer Europe MA EEIG; Treatment of sickle cell disease / Treatment of vaso-occlusive crisis

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.35. Baricitinib - EMEA-001220-PIP05-19

Treatment of systemic lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.36. Mavorixafor - EMEA-002490-PIP01-18

Treatment of warts, hypogammaglobulinemia, infections, and myelokathexis (WHIM) syndrome / Treatment of WHIM syndrome in paediatric patients aged 6 years and above.

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.37. Polymyxin B - EMEA-002595-PIP01-19

Treatment of infections due to aerobic Gram-negative bacteria

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.38. Zoliflodacin - EMEA-002599-PIP01-19

Treatment of gonococcal infection / Treatment of uncomplicated gonorrhoea

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.39. EMEA-002572-PIP01-19

Chromosome 15q duplication syndrome / cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder / Treatment of seizures associated with chromosome 15q duplication syndrome / Treatment of seizures associated with CDKL5 deficiency disorder

Day 30 discussion

Action: For discussion

Neurology

3.1.40. Belantamab mafodotin - Orphan - EMEA-002468-PIP04-19

GlaxoSmithKline Trading Services; Treatment of multiple myeloma

Day 30 discussion

Action: For discussion

Oncology

3.1.41. Infigratinib - EMEA-002594-PIP01-19

Cholangiocarcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.42. Adeno-associated viral vector serotype 8 containing the human RPGR gene - Orphan - EMEA-002601-PIP01-19

Nightstar Europa Limited; Treatment of X-linked retinitis pigmentosa

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.43. Difelikefalin - EMEA-002565-PIP01-19

Chronic kidney disease (CKD)-associated pruritus

Day 30 discussion

Action: For discussion

Other

3.1.44. Obidoxime chloride / atropine sulfate - EMEA-002570-PIP01-19

Organophosphate nerve agent poisoning

Day 30 discussion

Action: For discussion

Other

3.1.45. Fexapotide trifluate - EMEA-002598-PIP01-19

Treatment of patients with benign prostatic hyperplasia (BPH)

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.46. Recombinant hepatitis B vaccine - EMEA-002157-PIP01-17

Prevention of infection of hepatitis B virus / Secondary immunization of non-responder (anti-HBs levels < 10 mIU/mL) or hypo-responder (anti-HBs levels 10-99 mIU/mL) children age 2-18 years old to prior immunization against hepatitis B virus (HBV) infection / Primary active immunization of children from birth to 18 years old for the prevention of hepatitis B infection

Day 30 discussion

Action: For 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. Etravirine - EMEA-C-000222-PIP01-08-M09

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Sacubitril / valsartan - EMEA-000316-PIP02-11-M04

Novartis Europharm Ltd.; Heart Failure / Treatment of heart failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Ticagrelor - EMEA-000480-PIP01-08-M12

AstraZeneca AB; Thromboembolic events (children), acute coronary syndrome, history of myocardial infarction / Reduction in occurrence of vaso-occlusive crises in paediatric patients with sickle cell disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.3. Liquid ethanolic extract 30 per cent (w/w) of Allium cepa L. (fresh bulb) and Citrus limon (L.) Burm. f. (fresh fruit), Paullinia cupana Kunth, Theobroma cacao L. - EMEA-001835-PIP01-15-M04

LEGACY HEALTHCARE; Treatment of alopecia

Day 30 discussion

Action: For discussion

Dermatology

3.3.4. 2-hydroxypropyl-β-cyclodextrin (HP-β-CD) - Orphan - EMEA-001866-PIP01-15-M04

Mallinckrodt Pharmaceuticals Ireland Ltd; Treatment of Niemann-Pick disease, type C

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Empagliflozin - EMEA-000828-PIP04-16-M03

Boehringer Ingelheim International GmbH; Treatment of type 1 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Semaglutide - EMEA-001441-PIP03-17-M01

Novo Nordisk A/S; Treatment of obesity

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Etrolizumab - EMEA-001434-PIP01-13-M02

Roche Registration GmbH; Treatment of ulcerative colitis/ Treatment of Crohn's disease / Treatment of moderately to severely active Crohn's disease / Treatment of moderately to severely active ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.8. Linaclotide - EMEA-000927-PIP01-10-M04

Allergan Pharmaceuticals International Limited; Functional constipation in children

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.9. Odevixibat - Orphan - EMEA-002054-PIP01-16-M01

Albireo AB; Progressive familial intrahepatic cholestasis (PFIC)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.10. Ferric maltol - EMEA-001195-PIP01-11-M04

Norgine BV; Iron deficiency anaemia / Treatment for iron deficiency anaemia (IDA)

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.11. Lonococog alfa - EMEA-001215-PIP01-11-M07

CSL Behring GmbH; Haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.12. Upadacitinib - EMEA-001741-PIP01-14-M02

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.13. Ustekinumab - EMEA-000311-PIP03-11-M05

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis (PsA) and juvenile idiopathic arthritis (JIA)) / Treatment of juvenile idiopathic arthritis (juvenile psoriatic arthritis (jPsA))

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.14. Lamivudine (3TC) / dolutegravir (DTG) - EMEA-001940-PIP01-16-M02

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.15. Nirsevimab - anti-respiratory syncytial virus human IgG1k monoclonal antibody - EMEA-001784-PIP01-15-M01

AstraZeneca AB; Prevention of respiratory syncytial viral infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.16. Pretomanid - Orphan - EMEA-002115-PIP01-17-M01

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.17. Arimoclomol citrate - Orphan - EMEA-001748-PIP01-15-M02

Orphazyme A/S; Treatment of Niemann-Pick disease, Type C

Day 30 discussion

Action: For discussion

Neurology

3.3.18. Ataluren - Orphan - EMEA-000115-PIP01-07-M10

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

Day 30 discussion

Action: For discussion

Neurology

3.3.19. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M14

UCB Pharma S.A.; Treatment of neonatal seizures / Treatment of epilepsy with partial onset seizures / Treatment of paediatric patients with partial onset seizures / Treatment of neonatal seizures with adjunctive administration of brivaracetam

Day 30 discussion

Action: For discussion

Neurology

3.3.20. [Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M02](#)

AveXis Netherlands B.V.; Treatment of spinal muscular atrophy / Treatment of spinal muscular atrophy Type 1

Day 30 discussion

Action: For discussion

Neurology

3.3.21. [Risdiplam - Orphan - EMEA-002070-PIP01-16-M03](#)

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.22. [Cabozantinib - Orphan - EMEA-001143-PIP01-11-M02](#)

Ipsen Pharma; Treatment of malignant solid tumours

Day 30 discussion

Action: For discussion

Oncology

3.3.23. [Cobimetinib - EMEA-001425-PIP01-13-M04](#)

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) with Ras, Raf or MEK pathway activation / Treatment of children with a paediatric solid malignant tumour with known or expected Ras, Raf or MEK pathway activation, at first relapse or refractory to initial treatment

Day 30 discussion

Action: For discussion

Oncology

3.3.24. [Larotrectinib - Orphan - EMEA-001971-PIP02-16-M02](#)

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with advanced solid tumours harbouring an NTRK fusion

Day 30 discussion

Action: For discussion

Oncology

3.3.25. Pazopanib - EMEA-000601-PIP01-09-M06

Novartis Europharm Limited; Ewing sarcoma family of tumours /Rhabdomyosarcoma / Non-rhabdomyosarcoma soft tissue sarcoma / Treatment of paediatric patients with rhabdomyosarcoma / Treatment of paediatric patients with Ewing sarcoma family of tumours / Treatment of paediatric patients with non-rhabdomyosarcoma soft tissue sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3.26. Tisagenlecleucel - Orphan - EMEA-001654-PIP02-17-M01

Novartis Europharm Limited; Mature B-cell neoplasm / Treatment of paediatric patients with CD19+ relapsed or refractory mature B-cell non-Hodgkin's lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.3.27. Human thrombin / human fibrinogen - EMEA-001149-PIP01-11-M05

Omrix Biopharmaceuticals N.V.; Treatment of cerebrospinal fluid leakage resulting from a surgical procedure / Treatment of haemorrhage resulting from a surgical procedure / Indicated for suture line sealing in dura mater closure / Indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis / Indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis.

Day 30 discussion

Action: For discussion

Other

3.3.28. Lomitapide - EMEA-001124-PIP01-10-M04

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia / Treatment of homozygous familial hypercholesterolaemia, Treatment of heterozygous familial hypercholesterolemia

Day 30 discussion

Action: For discussion

Other

3.3.29. Dermatophagoides farinae / dermatophagoides pteronyssinus - EMEA-001258-PIP01-11-M05

ALK-Abelló A/S; Treatment of allergic rhinitis / Treatment of asthma / Indicated in house

dust mite allergic asthma / Indicated in house dust mite allergic rhinitis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.30. Fevipiprant - EMEA-001315-PIP02-16-M02

Novartis EuroPharm Limited; Asthma / Treatment of uncontrolled persistent asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.31. Finerenone - EMEA-001623-PIP01-14-M03

Bayer AG; Chronic kidney disease / Treatment of chronic kidney disease associated with proteinuria in addition to a therapy with angiotensin converting enzyme inhibitor (ACEi) or angiotensin receptor blocker (ARB)

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.32. Influenza virus surface antigens (haemagglutinin) of strain A (H1N1) / influenza virus surface antigens (haemagglutinin) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin) of strain B (Yamagata lineage) - EMEA-002027-PIP02-17-M01

Adimmune Corporation; Prevention of influenza infection

Day 30 discussion

Action: For discussion

Vaccines

3.3.33. Outer membrane vesicles (OMV) from neisseria meningitidis serogroup B strain NZ98/254 measured as amount of total protein containing the PorA P1.4 / Recombinant neisseria meningitidis serogroup B fHbp fusion protein / Recombinant neisseria meningitidis serogroup B NadA protein / Recombinant neisseria meningitidis serogroup B NHBA fusion protein - EMEA-000139-PIP01-07-M03

GSK Vaccines S.r.l.; Prevention of meningococcal meningitis

Day 30 discussion

Action: For discussion

Vaccines

- 3.3.34. Prepandrix: A/Indonesia/05/2005 (H5N1) like strain used / Adjupanrix: split influenza virus, inactivated, containing antigen: A/VietNam/1194/2004 (H5N1) like strain used - EMEA-000160-PIP01-07-M05
-

GlaxoSmithKline Biologicals SA; Prevention of influenza infection

Day 30 discussion

Action: For 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 20 August 2019 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver.

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

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. NTHi-Mcat vaccine - EMEA-05-2019

GlaxoSmithKline Biologicals SA; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft-versus-host disease after [bone-marrow] transplantation) / Reduction of exacerbations of chronic obstructive pulmonary disease (COPD) in patients with a history of exacerbations

Action: For adoption

6.1.2. Pertuzumab/trastuzumab fixed dose combination - EMEA-08-2019

Roche Registration GmbH; The class of HER - / Epidermal growth factor-receptor antibody medicinal products for treatment of breast malignant neoplasms. Early breast cancer: neoadjuvant treatment of adult patients with HER2-positive, locally advanced, inflammatory or early stage breast cancer at high risk of recurrence in combination with chemotherapy / Adjuvant treatment of adult patients with HER2-positive early breast cancer at high risk of recurrence in combination with chemotherapy. Metastatic breast cancer: treatment of adult patients with HER2-positive metastatic or locally recurrent unresectable breast cancer, who have not received previous anti-HER2 therapy or chemotherapy for their metastatic disease, for use in combination with docetaxel

Action: For adoption

6.1.3. Acumapimod - EMEA-09-2019

Mereo BioPharma Ireland Limited; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD)(excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft versus host disease after [bone-marrow] transplantation) / Treatment of acute exacerbations of COPD

Action: For adoption

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

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

7.1.1. Icosapent ethyl - EMEA-001300-PIP01-12

NDA Regulatory Science Ltd; Treatment of hypertriglyceridemia

Proposed indication: to reduce the risk of cardiovascular death, myocardial infarction, stroke, coronary revascularization, and unstable angina requiring hospitalization as an adjunct to statin therapy in adult patients with elevated triglyceride levels (TG \geq 135 mg/dL) and other risk factors for cardiovascular disease

Action: For adoption

Cardiovascular disease

7.1.2. Fc- and CDR-modified humanized monoclonal antibody against C5 - EMEA-001943-PIP01-16-M01

Alexion Pharma GmbH; Treatment of atypical haemolytic uremic syndrome

Proposed indication: Treatment of adult patients with complement-mediated thrombotic microangiopathy (TMA)

Action: For adoption

7.1.3. Ticagrelor - EMEA-000480-PIP01-08-M11

AstraZeneca AB; Prevention of thromboembolic events

Proposed indication: Ticagrelor is indicated for the prevention of stroke and death in patients with acute ischaemic stroke or transient ischaemic attack

Action: For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

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

PDCO member(s)/alternate(s) mandate status

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

9.2.2. EMA Scientific Committees – Timing for Chair elections

Action: For information

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

9.3.1. Non-clinical Working Group: Draft manuscript on the non-clinical CNS project

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Action: For information

9.4. Cooperation within the EU regulatory network

9.4.1. CTFG: Recommendation Paper on the Initiation and Conduct of Complex Clinical Trials

CTFG Chair: Ann Marie Janson Lang; Elke Stahl

Action: For information

9.5. Cooperation with International Regulators

9.5.1. Report from the Paediatric Cluster Teleconference

Action: For information

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

No items

9.7. PDCO work plan

No items

9.8. Planning and reporting

9.8.1. Marketing authorisation applications (MAA) forecast for 2019 – planning update dated Q2 2019

Action: For information

9.8.2. A first perspective on the impact of the review of the class waiver list in July 2015

Action: For information

9.8.3. Strategic Review and Learning Meeting (SRLM) under the Romanian Presidency held in Malta on 13-14 June 2019 - Report to the Committee

PDCO topic leaders: Dirk Mentzer, Dana Gabriela Marin, Herbert Lenicker

Action: For information

10. Any other business

No items

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 14:00 - 15:00, room 0-E

11.1.2. Neonatology

Action: For discussion on Thursday, 14:00 - 15:00, room 0-D

11.1.3. Inventory

Action: For discussion on Thursday, 14:00 - 15:00, room 2-D

12. 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/