



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

21 June 2024
EMA/PDCO/267948/2024
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 25-28 June 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

25 June 2024, 14:00 - 19:00, room 2C

26 June 2024, 08:30 - 19:00, room 2C

27 June 2024, 08:30 - 19:00, room 2C

28 June 2024, 08:30 - 13:00, room 2C

Health and safety information

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.

Disclaimers

Some of the information contained in this agenda 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).

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 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).



Table of contents

1.	Introductions	9
1.1.	Welcome and declarations of interest of members, alternates and experts	9
1.2.	Adoption of agenda	9
1.3.	Adoption of the minutes	9
2.	Opinions	9
2.1.	Opinions on Products	9
2.1.1.	Transglutaminase 2 inhibitor - EMEA-003513-PIP01-23	9
2.1.2.	Mezagitamab - EMEA-003502-PIP01-23.....	9
2.1.3.	Ganaxolone - EMEA-002341-PIP03-23.....	9
2.1.4.	Vosoritide - EMEA-002033-PIP02-23	10
2.1.5.	A/H1N1, A/H3N2, and B/Victoria and single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A/H1N1, A/H3N2, and B/Victoria and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein - EMEA003521-PIP01-23	10
2.1.6.	Tadalafil / ambrisentan - EMEA-003617-PIP01-24.....	10
2.1.7.	Tadalafil / ambrisentan - EMEA-003621-PIP01-24.....	10
2.1.8.	Tarumase - EMEA-003616-PIP01-24	10
2.1.9.	Copper (⁶⁴ Cu) oxodotreotide - Orphan - EMEA-003610-PIP01-24.....	11
2.1.10.	Alvelestat - EMEA-003605-PIP01-24	11
2.1.11.	Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - EMEA-003420-PIP02-24	11
2.1.12.	Inobrodib - EMEA-003622-PIP01-24.....	11
2.1.13.	Acasunlimab - EMEA-003606-PIP01-24	11
2.1.14.	Buparlisib - EMEA-003607-PIP01-24	12
2.1.15.	Ifinatamab deruxtecan - Orphan - EMEA-003613-PIP01-24.....	12
2.1.16.	Iloperidone - EMEA-000995-PIP02-24	12
2.1.17.	Brukinsa - EMEA-002354-PIP03-24	12
2.2.	Opinions on Compliance Check	12
2.2.1.	Recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (RIV3) - EMEA-C-003640-PIP01-24.....	12
2.2.2.	Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage - EMEA-C-003641-PIP01-24 .	13
2.2.3.	Estetrol monohydrate / drospirenone - EMEA-C-001332-PIP01-12-M06	13
2.2.4.	Liraglutide - EMEA-C-000128-PIP02-09-M05	13
2.3.	Opinions on Modification of an Agreed Paediatric Investigation Plan	13
2.3.1.	Gadopiclenol - EMEA-001949-PIP01-16-M07	13
2.3.2.	Gadopiclenol - EMEA-001949-PIP02-18-M05	13

2.3.3.	Crinecerfont - Orphan - EMEA-002700-PIP01-19-M02	14
2.3.4.	Humanised monoclonal antibody Fab fragment - Orphan - EMEA-003253-PIP01-22-M01 ..	14
2.3.5.	Navepegritide - Orphan - EMEA-002689-PIP02-23-M01	14
2.3.6.	Teplizumab - EMEA-000524-PIP01-08-M03	14
2.3.7.	Survodutide - EMEA-002942-PIP02-20-M02	14
2.3.8.	Tezepelumab - EMEA-001613-PIP03-21-M01	14
2.3.9.	Vonoprazan - EMEA-002703-PIP01-19-M01	15
2.3.10.	Fitusiran - Orphan - EMEA-001855-PIP01-15-M06	15
2.3.11.	Osivelotor - EMEA-003241-PIP01-22-M01.....	15
2.3.12.	Narsoplimab - Orphan - EMEA-002479-PIP01-18-M02	15
2.3.13.	Vadadustat - EMEA-001944-PIP01-16-M05	15
2.3.14.	Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19-M03	16
2.3.15.	Human normal immunoglobulin - EMEA-001290-PIP01-12-M01	16
2.3.16.	Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M07	16
2.3.17.	Lenacapavir sodium - EMEA-002740-PIP01-19-M01	16
2.3.18.	Pretomanid - Orphan - EMEA-002115-PIP01-17-M06	16
2.3.19.	Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M07	17
2.3.20.	Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M0517	
2.3.21.	Cenobamate - EMEA-002563-PIP02-19-M03	17
2.3.22.	Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19-M01	17
2.3.23.	Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M02.....	17
2.3.24.	Sodium ({{(2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2- yl}oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) - Orphan - EMEA-003344-PIP01-22-M01.....	18
2.3.25.	Midostaurin - Orphan - EMEA-000780-PIP01-09-M08	18
2.3.26.	Venetoclax - Orphan - EMEA-002018-PIP02-16-M06	18
2.3.27.	Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M06.....	18
2.3.28.	Bupropion hydrochloride / naltrexone hydrochloride - EMEA-001373-PIP01-12-M06.....	18
2.3.29.	Ketamine / sufentanil - EMEA-001739-PIP02-16-M03.....	19
2.3.30.	Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M05	19
2.3.31.	Benralizumab - EMEA-001214-PIP04-19-M01	19
2.3.32.	Donidalorsen - Orphan - EMEA-003112-PIP01-21-M01	19
2.3.33.	COVID-19 vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M04	19
2.3.34.	<i>Neisseria meningitidis</i> serogroup B protein-based active substance / recombinant <i>Neisseria meningitidis</i> serogroup B protein 3 / recombinant <i>Neisseria meningitidis</i> serogroup B protein 2 / recombinant <i>Neisseria meningitidis</i> serogroup B protein 1 / <i>Neisseria meningitidis</i> group Y polysaccharide conjugated to tetanus toxoid carrier protein / <i>Neisseria meningitidis</i> group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / <i>Neisseria meningitidis</i> group C polysaccharide conjugated to tetanus toxoid carrier protein / <i>Neisseria meningitidis</i> group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-003379-PIP01-22-M0119	

2.3.35.	Osilodrostat - Orphan - EMEA-000315-PIP02-15-M04.....	20
2.4.	Opinions on Re-examinations	20
2.5.	Opinions on Review of Granted Waivers	20
2.6.	Finalisation and adoption of Opinions.....	20
2.7.	Partial Compliance Checks completed by EMA	20
2.7.1.	Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-C1-002780-PIP02-20-M01	20
2.7.2.	Selumetinib - EMEA-C2-001585-PIP01-13-M06	20
2.7.3.	Olezarsen - EMEA-C1-003177-PIP01-21	21
2.7.4.	Finerenone - EMEA-C1-001623-PIP03-20-M01	21
2.7.5.	Glucagon - EMEA-C2-001657-PIP01-14-M01	21

3. Discussion of applications 21

3.1.	Discussions on Products D90-D60-D30.....	21
3.1.1.	Seralutinib - Orphan - EMEA-002972-PIP02-23	21
3.1.2.	Pegtibatinase - Orphan - EMEA-003518-PIP01-23.....	22
3.1.3.	Efinopegdutide - EMEA-003549-PIP01-23	22
3.1.4.	Hydroxycarbamide - EMEA-003388-PIP01-23.....	22
3.1.5.	Recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody - EMEA-003510-PIP01-23	22
3.1.6.	Belumosudil - Orphan - EMEA-003425-PIP02-23.....	22
3.1.7.	Iptacopan - EMEA-002705-PIP06-23	22
3.1.8.	Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003524-PIP01-23.....	23
3.1.9.	Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23	23
3.1.10.	Retatrutide - EMEA-003258-PIP02-23	23
3.1.11.	Riloncept - Orphan - EMEA-003571-PIP01-23	23
3.1.12.	Luliconazole - EMEA-003604-PIP01-24	23
3.1.13.	2-Isopropyl-3H-naphtho[1,2-d]imidazole-4,5-dione - Orphan - EMEA-003618-PIP01-24... 24	24
3.1.14.	Diazoxide choline - Orphan - EMEA-003614-PIP01-24	24
3.1.15.	Pegozafermin - EMEA-003619-PIP01-24	24
3.1.16.	Telitacicept - EMEA-002824-PIP02-24	24
3.1.17.	Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding human Wiskott-Aldrich syndrome protein - EMEA-003620-PIP01-24	24
3.1.18.	EMEA-003609-PIP01-24	24
3.1.19.	EMEA-003306-PIP02-24	25
3.1.20.	EMEA-003615-PIP01-24	25
3.1.21.	Frexalimab - EMEA-002945-PIP04-24.....	25
3.1.22.	Gedatolisib - EMEA-003612-PIP01-24.....	25
3.1.23.	Letetresgene autoleucel - Orphan - EMEA-002476-PIP03-24	25

3.1.24.	Rocatinlimab - EMEA-002886-PIP03-24	26
3.1.25.	Sargramostim - EMEA-003568-PIP02-24	26
3.1.26.	Adeno-associated virus serotype 9 vector containing the human LAMP2 isoform B transgene - Orphan - EMEA-003639-PIP01-24.....	26
3.1.27.	Zerlasiran - EMEA-003627-PIP01-24	26
3.1.28.	EMEA-003632-PIP01-24	26
3.1.29.	EMEA-003633-PIP01-24	26
3.1.30.	Rememulgene arelactibac - EMEA-003638-PIP01-24	27
3.1.31.	Dasiglucagon - Orphan - EMEA-002233-PIP03-24.....	27
3.1.32.	Etavopivat - Orphan - EMEA-002924-PIP03-24.....	27
3.1.33.	Sonelokimab - EMEA-002568-PIP03-24	27
3.1.34.	Heterologous intestinal microbiota from healthy donors - EMEA-003635-PIP01-24.....	27
3.1.35.	EMEA-003602-PIP01-24	28
3.1.36.	Adeno-associated viral vector serotype 9 containing the human MECP2 gene - Orphan - EMEA-003634-PIP01-24	28
3.1.37.	Aldesleukin - Orphan - EMEA-001556-PIP02-24.....	28
3.1.38.	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O ₂)-NH ₂ -DOTA-225-Actinium - Orphan - EMEA-003630-PIP01-24	28
3.1.39.	EMEA-003631-PIP01-24	28
3.1.40.	Anitocabtagene autoleucl - EMEA-003628-PIP01-24	29
3.1.41.	Golcadomide - Orphan - EMEA-003161-PIP02-24.....	29
3.1.42.	Tafasitamab - Orphan - EMEA-002499-PIP03-24	29
3.1.43.	Esonadogene imvoparvovec - Orphan - EMEA-003626-PIP01-24.....	29
3.1.44.	Tacrolimus - EMEA-003625-PIP01-24.....	29
3.1.45.	Rupatadine / montelukast - EMEA-003629-PIP01-24.....	29
3.1.46.	Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24.....	30
3.1.47.	mRNA-based vaccine consisting of 3 mRNAs encoding the VP1 capsid protein from NoV genotypes, GII.4, GII.3 and GI.3 - EMEA-003637-PIP01-24.....	30
3.2.	Discussions on Compliance Check.....	30
3.2.1.	Sebetralstat - EMEA-C1-002723-PIP01-19-M02	30
3.2.2.	Emtricitabine / tenofovir alafenamide - EMEA-C1-001577-PIP02-14-M05.....	30
3.2.3.	Palbociclib - EMEA-C-002146-PIP01-17-M04	31
3.3.	Discussions on Modification of an Agreed Paediatric Investigation Plan.....	31
3.3.1.	Fenebrutinib - EMEA-002349-PIP03-20-M01	31
3.3.2.	Ambrisentan - Orphan - EMEA-000434-PIP01-08-M10.....	31
3.3.3.	Spesolimab - EMEA-002475-PIP03-22-M01.....	31
3.3.4.	Macimorelin acetate - EMEA-001988-PIP01-16-M02.....	31
3.3.5.	Apremilast - EMEA-000715-PIP02-11-M08.....	31
3.3.6.	Apremilast - EMEA-000715-PIP05-13-M06.....	32

3.3.7.	Deucravacitinib - EMEA-002350-PIP02-20-M01	32
3.3.8.	Human plasma derived C1-inhibitor (OCTA-C1-INH) - EMEA-002818-PIP01-20-M01	32
3.3.9.	Cefiderocol - EMEA-002133-PIP01-17-M04	32
3.3.10.	Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M03.....	32
3.3.11.	Sotrovimab - EMEA-002899-PIP01-20-M03.....	33
3.3.12.	Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19-M03	33
3.3.13.	Lasmiditan - EMEA-002166-PIP01-17-M08.....	33
3.3.14.	Rimegepant - EMEA-002812-PIP02-20-M03	33
3.3.15.	Tasimelteon - Orphan - EMEA-001531-PIP01-13-M06	33
3.3.16.	Vamorolone - Orphan - EMEA-001794-PIP02-16-M08.....	34
3.3.17.	Vesleleplirsen - EMEA-003305-PIP01-22-M01	34
3.3.18.	Avelumab - EMEA-001849-PIP02-15-M05	34
3.3.19.	Daratumumab - Orphan - EMEA-002152-PIP01-17-M04	34
3.3.20.	Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M03	34
3.3.21.	Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21-M01.....	34
3.3.22.	Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04.....	35
3.3.23.	Iptacopan - Orphan - EMEA-002705-PIP03-20-M01	35
3.3.24.	Ebola Zaire vaccine (rVSVΔG-ZEBOV-GP, live) - EMEA-001786-PIP01-15-M04	35
3.3.25.	Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M02	35
3.3.26.	Recombinant varicella zoster virus glycoprotein E - EMEA-001426-PIP01-13-M03	35

4. Nominations 36

4.1.	List of submissions of applications with start of procedure 08 July 2024 for Nomination of Rapporteur and Peer reviewer.....	36
4.2.	Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver	36
4.3.	Nominations for other activities	36

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction 36

6. Discussion on the applicability of class waivers 36

6.1.	Discussions on the applicability of class waiver for products.....	36
6.1.1.	Depemokimab - EMEA-06-2024.....	36
6.1.2.	Leuprorelin - EMEA-07-2024	36

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver 37

7.1.	Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver	37
-------------	--	-----------

8.	Annual reports on deferrals	37
9.	Organisational, regulatory and methodological matters	37
9.1.	Mandate and organisation of the PDCO.....	37
9.1.1.	PDCO membership.....	37
9.1.2.	Vote by Proxy	37
9.1.3.	Strategic Review and Learning Meeting (SRLM)	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.	Pharmacovigilance Risk Assessment Committee (PRAC)	38
9.2.3.	Joint resolution on the principles of conduct within EMA Scientific Committees, WPs and CMDx	38
9.3.	Coordination with EMA Working Parties/Working Groups/Drafting Groups	38
9.3.1.	Non-clinical Working Party: D30 Products identified	38
9.3.2.	Paediatric Formulation Operational Expert Group (PFOEG)	38
9.3.3.	Patients and Consumers Working Party (PCWP)/Healthcare Professionals Working Party (HCPWP)	38
9.3.4.	Upcoming Innovation Task Force (ITF) meetings	38
9.3.5.	Cardiovascular Working Party (CVS WP)	38
9.4.	Cooperation within the EU regulatory network.....	38
9.4.1.	European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA).....	38
9.4.2.	Feedback and status update from the PaedForm Project (EDQM)	39
9.5.	Cooperation with International Regulators.....	39
9.5.1.	Paediatric Cluster Teleconference	39
9.6.	Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee.....	39
9.7.	PDCO work plan.....	39
9.8.	Planning and reporting	39
9.8.1.	EMA Business Pipeline activity and Horizon scanning	39
10.	Any other business	39
10.1.	Health Technology Assessment body (HTAb) / EMA workshop on uncertainty management	39
10.2.	Potential pitfalls from uncritical use of artificial intelligence (AI)	39
10.3.	Practical aspects of IRIS for PDCO Members	39
10.4.	Presenting efforts/work ongoing on a facilitation framework	40
10.5.	Real World Evidence, including DARWIN EU®	40
11.	Breakout sessions	40
11.1.	Paediatric oncology	40
11.2.	Neonatology	40
11.3.	HIV	40

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 2024. See June 2024 PDCO minutes (to be published post July PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 25-28 June 2024.

1.3. Adoption of the minutes

PDCO minutes for 28-31 May 2024.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Transglutaminase 2 inhibitor - EMEA-003513-PIP01-23

Treatment of coeliac disease

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.2. Mezagitamab - EMEA-003502-PIP01-23

Treatment of immune thrombocytopenia

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.3. Ganaxolone - EMEA-002341-PIP03-23

Treatment of refractory status epilepticus

Day 120 opinion

Action: For adoption

Neurology

2.1.4. Vosoritide - EMEA-002033-PIP02-23

Treatment of hypochondroplasia

Day 120 opinion

Action: For adoption

Other

2.1.5. A/H1N1, A/H3N2, and B/Victoria and single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A/H1N1, A/H3N2, and B/Victoria and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein - EMEA-003521-PIP01-23

Prevention of influenza and coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Vaccines

2.1.6. Tadalafil / ambrisentan - EMEA-003617-PIP01-24

Treatment of pulmonary arterial hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.7. Tadalafil / ambrisentan - EMEA-003621-PIP01-24

Treatment of pulmonary arterial hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.8. Tarumase - EMEA-003616-PIP01-24

Treatment of chronic wounds

Day 60 opinion

Action: For adoption

Dermatology

2.1.9. Copper (⁶⁴Cu) oxodotreotide - Orphan - EMEA-003610-PIP01-24

Curium Pet France; Diagnosis of neuroendocrine neoplasms

Day 60 opinion

Action: For adoption

Diagnostic

2.1.10. Alvelestat - EMEA-003605-PIP01-24

Treatment of alpha-1 antitrypsin deficiency

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.11. Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - EMEA-003420-PIP02-24

Treatment of severe hypertriglyceridaemia (SHTG)

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 18 June 2024

2.1.12. Inobrodib - EMEA-003622-PIP01-24

Treatment of multiple myeloma

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.13. Acasunlimab - EMEA-003606-PIP01-24

Treatment of lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.14. Buparlisib - EMEA-003607-PIP01-24

Treatment of head and neck epithelial malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.15. Ifinatamab deruxtecan - Orphan - EMEA-003613-PIP01-24

Daiichi Sankyo Europe GmbH; Treatment of lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.16. Iloperidone - EMEA-000995-PIP02-24

Treatment of bipolar disorder

Day 60 opinion

Action: For adoption

Psychiatry

Note: Withdrawal request received on 19 June 2024

2.1.17. Brukinsa - EMEA-002354-PIP03-24

Treatment of lymphoplasmacytic lymphoma / Treatment of mature B-cell neoplasms (excluding lymphocytic lymphoma)

Day 30 opinion

Action: For adoption

Oncology

2.2. Opinions on Compliance Check

2.2.1. Recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (RIV3) - EMEA-C-003640-PIP01-24

Sanofi Winthrop Industrie; Prevention of influenza disease

Day 30 opinion

Action: For adoption

Vaccines

2.2.2. Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage - EMEA-C-003641-PIP01-24

GlaxoSmithKline Biologicals S.A.; Prevention of influenza infection

Day 30 opinion

Action: For adoption

Vaccines

2.2.3. Estetrol monohydrate / drospirenone - EMEA-C-001332-PIP01-12-M06

Estetra SRL; Prevention of pregnancy

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.4. Liraglutide - EMEA-C-000128-PIP02-09-M05

Novo Nordisk A/S; Treatment of obesity

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Gadopiclenol - EMEA-001949-PIP01-16-M07

Guerbet; Detection and visualisation of areas with disruption of the blood brain barrier and/or abnormal vascularity for diagnostic purposes

Day 60 opinion

Action: For adoption

Diagnostic

2.3.2. Gadopiclenol - EMEA-001949-PIP02-18-M05

Guerbet; Detection and visualisation of disorders or lesions with suspected abnormal vascularity in various body regions for diagnostic purposes

Day 60 opinion

Action: For adoption

Diagnostic

2.3.3. Crinecerfont - Orphan - EMEA-002700-PIP01-19-M02

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Humanised monoclonal antibody Fab fragment - Orphan - EMEA-003253-PIP01-22-M01

Sanofi B.V; Treatment of achondroplasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Navepegritide - Orphan - EMEA-002689-PIP02-23-M01

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Teplizumab - EMEA-000524-PIP01-08-M03

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Survodutide - EMEA-002942-PIP02-20-M02

Boehringer Ingelheim International GmbH; Treatment of obesity

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. Tezepelumab - EMEA-001613-PIP03-21-M01

AstraZeneca AB; Treatment of eosinophilic esophagitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.9. [Vonoprazan - EMEA-002703-PIP01-19-M01](#)

Phathom Pharmaceuticals, Inc.; Treatment of gastroesophageal reflux disease (GORD) / Treatment of *Helicobacter pylori* infection

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.10. [Fitusiran - Orphan - EMEA-001855-PIP01-15-M06](#)

Sanofi B.V.; Treatment of haemophilia B / Treatment of haemophilia A

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.11. [Osivelotor - EMEA-003241-PIP01-22-M01](#)

Pfizer Europe MA EEIG; Treatment of sickle cell disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.12. [Narsoplimab - Orphan - EMEA-002479-PIP01-18-M02](#)

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.13. [Vadadustat - EMEA-001944-PIP01-16-M05](#)

Medice Arzneimittel Pütter GmbH & Co KG; Treatment of anaemia due to chronic disorders

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.14. Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19-M03

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of acute graft-versus-host-disease

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.15. Human normal immunoglobulin - EMEA-001290-PIP01-12-M01

LFB Biotechnologies; Treatment of primary immunodeficiency (PID)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.16. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M07

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Lenacapavir sodium - EMEA-002740-PIP01-19-M01

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.18. Pretomanid - Orphan - EMEA-002115-PIP01-17-M06

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.19. Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M07

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.20. Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M05

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.21. Cenobamate - EMEA-002563-PIP02-19-M03

Angelini Pharma S.p.A; Treatment of focal onset seizures (FOS) / Treatment of primary generalised tonic clonic seizures (PGTC)

Day 60 opinion

Action: For adoption

Neurology

2.3.22. Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19-M01

Quince Therapeutics, S.p.A.; Treatment of ataxia telangiectasia

Day 60 opinion

Action: For adoption

Neurology

2.3.23. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M02

argenx BV; Treatment of generalised myasthenia gravis

Day 60 opinion

Action: For adoption

Neurology

2.3.24. Sodium ({{(2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2-yl}oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) - Orphan - EMEA-003344-PIP01-22-M01

AbbVie Limited; Treatment of vanishing white matter disease

Day 60 opinion

Action: For adoption

Neurology

2.3.25. Midostaurin - Orphan - EMEA-000780-PIP01-09-M08

Novartis Europharm Limited; Treatment of acute myeloid leukaemia / Treatment of malignant mastocytosis / Treatment of mast cell leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.26. Venetoclax - Orphan - EMEA-002018-PIP02-16-M06

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms / Treatment of solid tumour malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

Note: Withdrawal request received on 14 June 2024

2.3.27. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M06

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.3.28. Bupropion hydrochloride / naltrexone hydrochloride - EMEA-001373-PIP01-12-M06

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 60 opinion

Action: For adoption

Other

2.3.29. Ketamine / sufentanil - EMEA-001739-PIP02-16-M03

Cessatech A/S; Treatment of acute pain

Day 60 opinion

Action: For adoption

Pain

2.3.30. Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M05

Heron Therapeutics B.V.; Treatment of acute postoperative pain

Day 60 opinion

Action: For adoption

Pain

2.3.31. Benralizumab - EMEA-001214-PIP04-19-M01

AstraZeneca AB; Treatment of hypereosinophilic syndrome

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.32. Donidalorsen - Orphan - EMEA-003112-PIP01-21-M01

Otsuka Pharmaceutical Netherlands B.V.; Treatment of hereditary angioedema

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.33. COVID-19 vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M04

AstraZeneca AB; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Action: For adoption

Vaccines

2.3.34. *Neisseria meningitidis* serogroup B protein-based active substance / recombinant *Neisseria meningitidis* serogroup B protein 3 / recombinant *Neisseria meningitidis* serogroup B protein 2 / recombinant *Neisseria meningitidis* serogroup B protein 1 / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* Group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to

tetanus toxoid carrier protein / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-003379-PIP01-22-M01

Sanofi Pasteur; Prevention of meningococcal disease (serogroups A, B, C, W and Y)

Day 60 opinion

Action: For adoption

Vaccines

2.3.35. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M04

Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

The following partial compliance checks have been identified by the PME coordinator and PDCO rapporteur as not needing to be referred to the PDCO for discussion. The PDCO has been informed in writing.

2.7.1. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-C1-002780-PIP02-20-M01

Sanofi Pasteur; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 letter

Action: For information

Vaccines

2.7.2. Selumetinib - EMEA-C2-001585-PIP01-13-M06

AstraZeneca AB; Treatment of neurofibromatosis type 1

Day 30 letter

Action: For information

Oncology

2.7.3. Olezarsen - EMEA-C1-003177-PIP01-21

Ionis Ireland Limited; Treatment of familial chylomicronaemia syndrome

Day 30 letter

Action: For information

Cardiovascular Diseases

2.7.4. Finerenone - EMEA-C1-001623-PIP03-20-M01

Bayer AG; Treatment of heart failure

Day 30 letter

Action: For information

Cardiovascular Diseases

2.7.5. Glucagon - EMEA-C2-001657-PIP01-14-M01

Amphastar France Pharmaceuticals; Treatment of hypoglycaemia

Day 30 letter

Action: For information

Endocrinology-Gynaecology-Fertility-Metabolism

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. Seralutinib - Orphan - EMEA-002972-PIP02-23

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.2. Pegtibatase - Orphan - EMEA-003518-PIP01-23

Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Efinopegdutide - EMEA-003549-PIP01-23

Treatment of non-alcoholic steatohepatitis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.4. Hydroxycarbamide - EMEA-003388-PIP01-23

Treatment of sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.5. Recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody - EMEA-003510-PIP01-23

Prevention of hereditary angioedema attacks

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.6. Belumosudil - Orphan - EMEA-003425-PIP02-23

Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.7. Iptacopan - EMEA-002705-PIP06-23

Treatment of myasthenia gravis

Day 90 discussion

Action: For discussion

Neurology

3.1.8. [Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003524-PIP01-23](#)

Sensorion SA; Treatment of otoferlin gene-mediated hearing loss

Day 90 discussion

Action: For discussion

Other

3.1.9. [Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23](#)

Glycomine Inc; Treatment of phosphomannomutase 2-congenital disorder of glycosylation

Day 90 discussion

Action: For discussion

Other

3.1.10. [Retatrutide - EMEA-003258-PIP02-23](#)

Treatment of obesity

Day 90 discussion

Action: For discussion

Other

3.1.11. [Riloncept - Orphan - EMEA-003571-PIP01-23](#)

Kiniksa Pharmaceuticals (UK), Ltd.; Treatment of idiopathic pericarditis

Day 90 discussion

Action: For discussion

Other / Cardiovascular Diseases

3.1.12. [Luliconazole - EMEA-003604-PIP01-24](#)

Treatment of onychomycosis

Day 60 discussion

Action: For discussion

Dermatology

3.1.13. [2-Isopropyl-3H-naphtho\[1,2-d\]imidazole-4,5-dione - Orphan - EMEA-003618-PIP01-24](#)

Abliva AB; Treatment of primary mitochondrial disease

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.14. [Diazoxide choline - Orphan - EMEA-003614-PIP01-24](#)

Soleno Therapeutics Europe Ltd.; Treatment of Prader-Willi syndrome

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Psychiatry / Neurology

3.1.15. [Pegozafermin - EMEA-003619-PIP01-24](#)

Treatment of metabolic dysfunction-associated steatohepatitis (MASH)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.16. [Telitacicept - EMEA-002824-PIP02-24](#)

Treatment of myasthenia gravis

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.17. [Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding human Wiskott-Aldrich syndrome protein - EMEA-003620-PIP01-24](#)

Treatment of Wiskott-Aldrich syndrome

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.18. [EMEA-003609-PIP01-24](#)

Treatment of respiratory tract disease caused by human respiratory syncytial virus

Day 60 discussion

Action: For discussion

Infectious Diseases

[3.1.19. EMEA-003306-PIP02-24](#)

Treatment of human respiratory syncytial virus (RSV)

Day 60 discussion

Action: For discussion

Infectious Diseases

[3.1.20. EMEA-003615-PIP01-24](#)

Treatment of Prader Willi syndrome

Day 60 discussion

Action: For discussion

Neurology

[3.1.21. Frexalimab - EMEA-002945-PIP04-24](#)

Treatment of multiple sclerosis

Day 60 discussion

Action: For discussion

Neurology

[3.1.22. Gedatolisib - EMEA-003612-PIP01-24](#)

Treatment of children with solid malignancies / Treatment of haematologic malignancies /
Treatment of central nervous system malignancies

Day 60 discussion

Action: For discussion

Oncology

[3.1.23. Letetresgene autoleucel - Orphan - EMEA-002476-PIP03-24](#)

Adaptimmune B. V.; Treatment of soft tissue sarcoma

Day 60 discussion

Action: For discussion

Oncology

3.1.24. Rocatinlimab - EMEA-002886-PIP03-24

Treatment of asthma

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.25. Sargramostim - EMEA-003568-PIP02-24

Treatment of autoimmune pulmonary alveolar proteinosis (aPAP)

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.26. Adeno-associated virus serotype 9 vector containing the human LAMP2 isoform B transgene - Orphan - EMEA-003639-PIP01-24

Rocket Pharmaceuticals, B.V.; Treatment of Danon disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.27. Zerlasiran - EMEA-003627-PIP01-24

Atherosclerotic cardiovascular disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.28. EMEA-003632-PIP01-24

Treatment of symptomatic heart failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.29. EMEA-003633-PIP01-24

Treatment of psoriasis

Day 30 discussion

Action: For discussion

Dermatology

3.1.30. Rememulgene arelactibac - EMEA-003638-PIP01-24

Treatment of diabetic foot ulcer / Treatment of venous leg ulcer

Day 30 discussion

Action: For discussion

Dermatology

3.1.31. Dasiglucagon - Orphan - EMEA-002233-PIP03-24

Zealand Pharma A/S; Treatment of congenital hyperinsulinism

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.32. Etavopivat - Orphan - EMEA-002924-PIP03-24

Novo Nordisk A/S; Treatment of thalassemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.33. Sonelokimab - EMEA-002568-PIP03-24

Treatment of active psoriatic arthritis / Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.34. Heterologous intestinal microbiota from healthy donors - EMEA-003635-PIP01-24

Treatment of *Clostridioides difficile* infection (CDI)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.35. EMEA-003602-PIP01-24

Treatment of tuberculosis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.36. Adeno-associated viral vector serotype 9 containing the human MECP2 gene - Orphan - EMEA-003634-PIP01-24

Taysha Gene Therapies, Inc.; Treatment of Rett syndrome

Day 30 discussion

Action: For discussion

Neurology

3.1.37. Aldesleukin - Orphan - EMEA-001556-PIP02-24

ILTOO Pharma SAS; Treatment of amyotrophic lateral sclerosis (ALS)

Day 30 discussion

Action: For discussion

Neurology

3.1.38. H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O₂)-NH₂-DOTA-225-Actinium - Orphan - EMEA-003630-PIP01-24

NovaCurie GmbH; Treatment of low-grade glioma

Day 30 discussion

Action: For discussion

Neurology

3.1.39. EMEA-003631-PIP01-24

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) whose treatment may include DNA-damaging agents

Day 30 discussion

Action: For discussion

Oncology

3.1.40. Anitocabtagene autoleucel - EMEA-003628-PIP01-24

Treatment of multiple myeloma

Day 30 discussion

Action: For discussion

Oncology

3.1.41. Golcadomide - Orphan - EMEA-003161-PIP02-24

Bristol-Myers Squibb Pharma EEIG; Treatment of mature B cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.42. Tafasitamab - Orphan - EMEA-002499-PIP03-24

Incyte Biosciences Distribution B.V; Treatment of mature B-cell neoplasms [excluding diffuse large B-cell lymphoma (DLBCL)]

Day 30 discussion

Action: For discussion

Oncology

3.1.43. Esonadogene imvoparvovec - Orphan - EMEA-003626-PIP01-24

Neurophth Therapeutics, Inc.; Treatment of Leber's hereditary optic neuropathy

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.44. Tacrolimus - EMEA-003625-PIP01-24

Treatment of vernal keratoconjunctivitis

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.45. Rupatadine / montelukast - EMEA-003629-PIP01-24

Treatment of allergic rhinitis

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.46. [Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24](#)

Prevention of lower respiratory tract disease caused by respiratory syncytial virus or human metapneumovirus

Day 30 discussion

Action: For discussion

Vaccines

3.1.47. [mRNA-based vaccine consisting of 3 mRNAs encoding the VP1 capsid protein from NoV genotypes, GII.4, GII.3 and GI.3 - EMEA-003637-PIP01-24](#)

Prevention of norovirus acute gastroenteritis

Day 30 discussion

Action: For discussion

Vaccines

3.2. **Discussions on Compliance Check**

The following compliance checks have been identified for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance.

3.2.1. [Sebetralstat - EMEA-C1-002723-PIP01-19-M02](#)

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.2. [Emtricitabine / tenofovir alafenamide - EMEA-C1-001577-PIP02-14-M05](#)

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.3. Palbociclib - EMEA-C-002146-PIP01-17-M04

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Fenebrutinib - EMEA-002349-PIP03-20-M01

Roche Registration GmbH; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

3.3.2. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M10

GlaxoSmithKline (Ireland) Limited; Treatment pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. Spesolimab - EMEA-002475-PIP03-22-M01

Boehringer Ingelheim International GmbH; Treatment of Netherton syndrome

Day 30 discussion

Action: For discussion

Dermatology

3.3.4. Macimorelin acetate - EMEA-001988-PIP01-16-M02

Atnahs Pharma Netherlands B. V.; Diagnosis of growth hormone deficiency

Day 30 discussion

Action: For discussion

Diagnostic

3.3.5. Apremilast - EMEA-000715-PIP02-11-M08

Amgen Europe B.V.; Treatment of juvenile idiopathic arthritis (JIA) / Treatment of juvenile psoriatic arthritis (JPsA)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.6. Apremilast - EMEA-000715-PIP05-13-M06

Amgen Europe.B.V; Treatment of Behçet's disease

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.7. Deucravacitinib - EMEA-002350-PIP02-20-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.8. Human plasma derived C1-inhibitor (OCTA-C1-INH) - EMEA-002818-PIP01-20-M01

Octapharma Pharmazeutika Produktionsges. m.b.H; Treatment of hereditary angioedema (HAE)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.9. Cefiderocol - EMEA-002133-PIP01-17-M04

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M03

Mundipharma GmbH; Treatment of invasive candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Sotrovimab - EMEA-002899-PIP01-20-M03

GlaxoSmithKline Trading Services Ltd; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19-M03

SIGA Technologies, Inc.; Treatment of the following viral infections in adults and children with body weight at least 13 kg: smallpox, monkeypox, cowpox. Also indicated to treat complications due to replication of vaccinia virus following vaccination against smallpox in adults and children with body weight at least 13 kg.

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. Lasmiditan - EMEA-002166-PIP01-17-M08

Eli Lilly and Company Ltd; Treatment of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.14. Rimegepant - EMEA-002812-PIP02-20-M03

Pfizer Europe MA EEIG; Treatment of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.15. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M06

Vanda Pharmaceuticals Netherlands B.V.; Treatment of non-24-hour sleep-wake disorder in the totally blind

Day 30 discussion

Action: For discussion

Neurology

3.3.16. Vamorolone - Orphan - EMEA-001794-PIP02-16-M08

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

Note: Withdrawal request received on 13 June 2024

3.3.17. Vesleteplirsen - EMEA-003305-PIP01-22-M01

Sarepta Therapeutics Ireland; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.18. Avelumab - EMEA-001849-PIP02-15-M05

Merck Healthcare KGaA; Treatment of malignant neoplasms of the central nervous system

Day 30 discussion

Action: For discussion

Oncology

3.3.19. Daratumumab - Orphan - EMEA-002152-PIP01-17-M04

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell neoplasms)

Day 30 discussion

Action: For discussion

Oncology

3.3.20. Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M03

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.21. Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21-M01

Miltenyi Biomedicine GmbH; Treatment of mature B cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.3.22. [Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04](#)

Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

Day 30 discussion

Action: For discussion

Other

3.3.23. [Iptacopan - Orphan - EMEA-002705-PIP03-20-M01](#)

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Day 30 discussion

Action: For discussion

Other / Haematology-Hemostaseology

3.3.24. [Ebola Zaire vaccine \(rVSVΔG-ZEBOV-GP, live\) - EMEA-001786-PIP01-15-M04](#)

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

Day 30 discussion

Action: For discussion

Vaccines

3.3.25. [Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M02](#)

Sanofi Pasteur; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Action: For discussion

Vaccines

3.3.26. [Recombinant varicella zoster virus glycoprotein E - EMEA-001426-PIP01-13-M03](#)

GlaxoSmithKline Biologicals; Prevention of varicella zoster virus (VZV) reactivation

Day 30 discussion

Action: For discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 08 July 2024 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

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

6. Discussion on the applicability of class waivers

Some information related to this section cannot be disclosed 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. Depemokimab - EMEA-06-2024

GlaxoSmithKline Trading Services 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 (bone-marrow) transplantation.

Add-on to inhaled corticosteroid-based maintenance therapy for COPD patients.

Action: For adoption

6.1.2. Leuprorelin - EMEA-07-2024

Recordati Industria Chimica e Farmaceutica S.p.A.; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their

releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of prostate malignant neoplasms.

Treatment of hormone dependent advanced prostate cancer and for the treatment of high-risk localised and locally advanced hormone dependent prostate cancer in combination with radiotherapy.

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

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

Action: For information

9.1.2. Vote by Proxy

Action: For information

9.1.3. Strategic Review and Learning Meeting (SRLM)

No item

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. Pharmacovigilance Risk Assessment Committee (PRAC)

Scope: Committee consultation of the PRAC-approved final revision of GVP Module XVI on risk minimisation measures post-public consultation

Action: For information

9.2.3. Joint resolution on the principles of conduct within EMA Scientific Committees, WPs and CMDx

Action: For information

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Paediatric Formulation Operational Expert Group (PFOEG)

PDCO member: Brian Aylward (*ad interim*)

Action: For information

9.3.3. Patients and Consumers Working Party (PCWP)/Healthcare Professionals Working Party (HCPWP)

Action: For information

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Action: For information

9.3.5. Cardiovascular Working Party (CVS WP)

Scope: Under the supervision of the CVS WP a temporary drafting group (DG) has been established. The DG is working on the revision of the Paediatric Addendum on pulmonary arterial hypertension. Two PDCO representatives have been nominated to contribute to the DG.

Action: For information

9.4. Cooperation within the EU regulatory network

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

No item

9.4.2. Feedback and status update from the PaedForm Project (EDQM)

Scope: Feedback from the Paediatric Formulary meeting held in Strasbourg (FR) on 5-6 March 2024 and current status

PDCO member: Siri Wang

Action: For information

9.5. Cooperation with International Regulators

9.5.1. 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 item

9.7. PDCO work plan

No Item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q2/2024 Update of the Business Pipeline report for the human scientific committees

Action: For information

10. Any other business

10.1. Health Technology Assessment body (HTAb) / EMA workshop on uncertainty management

Scope: To inform the Committee on an upcoming HTAb / EMA workshop series on uncertainty management

Action: For information

10.2. Potential pitfalls from uncritical use of artificial intelligence (AI)

Action: For information

10.3. Practical aspects of IRIS for PDCO Members

Scope: The PDCO will be informed about the practical aspects associated with the onboarding of paediatric procedures in IRIS

Action: For information

10.4. Presenting efforts/work ongoing on a facilitation framework

Scope: The PDCO will be informed about ongoing considerations towards building a paediatric drug development facilitation framework

Action: For information

10.5. Real World Evidence, including DARWIN EU®

Action: For information

11. Breakout sessions

11.1. Paediatric oncology

Action: For discussion on Tuesday, 13:00 - 14:00, room 2C

11.2. Neonatology

Action: For discussion on Wednesday, 13:00 - 14:00, room 2C

11.3. HIV

Action: For discussion on Thursday, 13:00 - 14:00, room 2C

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.

For a list of acronyms and abbreviations, see:

[Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA's regulatory activities](#)

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