



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

22 March 2022
EMA/PDCO/128811/2022
Human Medicines Division

Paediatric Committee (PDCO)

Agenda for the meeting on 22-25 March 2022

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

22 March 2022, 14:00- 19:00, Virtual meeting

23 March 2022, 08:30- 19:00, Virtual meeting

24 March 2022, 08:30- 19:00, Virtual meeting

25 March 2022, 08:30- 13:00, Virtual meeting

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	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.	Ibutamoren mesylate - Orphan - EMEA-003032-PIP01-21	8
2.1.2.	Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP02-20	8
2.1.3.	Zinc gluconate / alisitol / retinyl palmitate - Orphan - EMEA-002198-PIP01-21.....	9
2.1.4.	Mitapivat - Orphan - EMEA-002684-PIP02-21.....	9
2.1.5.	Deucravacitinib - EMEA-002350-PIP02-20	9
2.1.6.	Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase - Orphan - EMEA-003020-PIP01-21	9
2.1.7.	Lorlatinib - EMEA-002669-PIP03-21	9
2.1.8.	Recombinant SARS-CoV-2 spike (S)-protein virus-like particle - EMEA-003008-PIP01-21 .	10
2.1.9.	SARS-CoV-2 virus, beta-propiolactone inactivated - EMEA-003077-PIP01-21	10
2.1.10.	Amlodipine / indapamide / perindopril arginine / atorvastatin - EMEA-003173-PIP01-21...	10
2.1.11.	Dupilumab - EMEA-001501-PIP11-21	10
2.1.12.	Adeno-associated virus vector serotype 1 containing the human GRN gene - Orphan - EMEA-003167-PIP01-21	10
2.1.13.	Anti-CD40L humanized monoclonal antibody - EMEA-002945-PIP02-21	10
2.1.14.	Izaflortaucipir (¹⁸ F) - EMEA-003040-PIP02-21	11
2.1.15.	Latozinemab - Orphan - EMEA-002997-PIP02-22.....	11
2.1.16.	EMEA-003169-PIP01-21	11
2.1.17.	Human IgG4-based bispecific antibody binding to both B-cell maturation antigen (BCMA) and cluster of differentiation 3 (CD3) - EMEA-003175-PIP01-21	11
2.1.18.	Infigratinib - Orphan - EMEA-002594-PIP03-21	11
2.1.19.	Sacituzumab govitecan - Orphan - EMEA-002645-PIP03-21	12
2.1.20.	Zandelisib - EMEA-003158-PIP01-21	12
2.1.21.	Triazolopyrimidine derivative - EMEA-003095-PIP01-21.....	12
2.1.22.	EMEA-003159-PIP01-21	12
2.1.23.	Pseudoephedrine / bilastine - EMEA-003164-PIP01-21	12
2.2.	Opinions on Compliance Check	12
2.2.1.	Treosulfan - EMEA-C-000883-PIP01-10-M05	12
2.2.2.	Oritavancin - EMEA-C2-001270-PIP01-12-M04	13
2.2.3.	Dabrafenib - EMEA-C3-001147-PIP01-11-M07.....	13

2.2.4.	Trametinib - EMEA-C2-001177-PIP01-11-M06	13
2.3.	Opinions on Modification of an Agreed Paediatric Investigation Plan	13
2.3.1.	Regdanvimab - EMEA-002961-PIP01-21-M01	13
2.3.2.	Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20-M01	14
2.3.3.	Remibrutinib - EMEA-002582-PIP01-19-M01	14
2.3.4.	Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19-M02	14
2.3.5.	Dapagliflozin - EMEA-000694-PIP02-14-M03	14
2.3.6.	Tirzepatide - EMEA-002360-PIP01-18-M02	14
2.3.7.	Tolvaptan - EMEA-001231-PIP02-13-M09	14
2.3.8.	Odevixibat - Orphan - EMEA-002054-PIP01-16-M03	15
2.3.9.	Potassium sulphate / magnesium sulphate heptahydrate / sodium sulphate anhydrous - EMEA-000816-PIP02-10-M03	15
2.3.10.	Betibeglogene autotemcel - Orphan - EMEA-001665-PIP01-14-M06	15
2.3.11.	Luspatercept - Orphan - EMEA-001521-PIP01-13-M06	15
2.3.12.	Brincidofovir - Orphan - EMEA-001904-PIP03-18-M02	15
2.3.13.	Cefiderocol - EMEA-002133-PIP01-17-M02	16
2.3.14.	Eslicarbazepine acetate - EMEA-000696-PIP02-10-M08	16
2.3.15.	Cemiplimab - EMEA-002007-PIP02-17-M02	16
2.3.16.	Lenvatinib - EMEA-001119-PIP03-19-M02	16
2.3.17.	Venetoclax - Orphan - EMEA-002018-PIP02-16-M05	16
2.3.18.	Sonlicromanol - Orphan - EMEA-002113-PIP01-16-M01.....	17
2.3.19.	Ketamine / sufentanil - EMEA-001739-PIP02-16-M01.....	17
2.4.	Opinions on Re-examinations	17
2.5.	Opinions on Review of Granted Waivers	17
2.6.	Finalisation and adoption of Opinions.....	17
2.7.	Partial Compliance Checks completed by EMA	17
3.	Discussion of applications	17
3.1.	Discussions on Products D90-D60-D30.....	17
3.1.1.	Dersimelagon - EMEA-002850-PIP02-21	17
3.1.2.	Humanised KLB/FGFR1c monoclonal antibody - EMEA-003058-PIP01-21	18
3.1.3.	Apraglutide - Orphan - EMEA-003016-PIP01-21.....	18
3.1.4.	Recombinant adeno-associated viral (rAAV) vector expressing the human ornithine transcarbamylase (hOTC) gene - EMEA-002983-PIP01-21	18
3.1.5.	Sirolimus - EMEA-002982-PIP01-21	18
3.1.6.	Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate (ECT-001-CB) - Orphan - EMEA-003025-PIP02-21	18
3.1.7.	Reparixin - Orphan - EMEA-001693-PIP03-21	19

3.1.8.	Omaveloxolone - Orphan - EMEA-002487-PIP01-18	19
3.1.9.	Cedazuridine: (4R)-1-[(2R,4R,5R)-3,3-difluoro-4-hydroxy-5-(hydroxymethyl)oxolan-2-yl]-4-hydroxy-1,3-diazinan-2-one / decitabine: 4-amino-1-[(2R,4S,5R)-4-hydroxy-5-(hydroxymethyl)oxolan-2-yl]-1,3,5-triazin-2(1H)-one - Orphan - EMEA-003071-PIP01-21	19
3.1.10.	Whole-cell heat-inactivated bacterial strains of <i>Escherichia coli</i> , <i>klebsiella pneumoniae</i> , <i>proteus vulgaris</i> and <i>enterococcus faecalis</i> - EMEA-003026-PIP02-21	19
3.1.11.	Cedirogant - EMEA-003142-PIP02-21	19
3.1.12.	Sirolimus - Orphan - EMEA-003168-PIP01-21	20
3.1.13.	Manganese chloride tetrahydrate - EMEA-003035-PIP02-21	20
3.1.14.	Avexitide acetate - Orphan - EMEA-003125-PIP02-21	20
3.1.15.	Glucagon analogue linked to a human immunoglobulin Fc fragment - Orphan - EMEA-003170-PIP01-21	20
3.1.16.	Insulin lispro - EMEA-003166-PIP01-21	20
3.1.17.	Semaglutide - EMEA-001441-PIP07-21	21
3.1.18.	Efavaleukin alfa - EMEA-003156-PIP01-21	21
3.1.19.	HIV-1 maturation inhibitor - EMEA-003153-PIP01-21	21
3.1.20.	HIV-1 maturation inhibitor / dolutegravir - EMEA-003152-PIP01-21	21
3.1.21.	Interferon beta-1a - EMEA-003056-PIP01-22	21
3.1.22.	Batoclimab - EMEA-003162-PIP01-21	21
3.1.23.	Pridopidine hydrochloride - Orphan - EMEA-003174-PIP01-21	22
3.1.24.	Satralizumab - Orphan - EMEA-001625-PIP03-21	22
3.1.25.	Camidanlumab tesirine - EMEA-003160-PIP01-21	22
3.1.26.	Emactuzumab - EMEA-003172-PIP01-21	22
3.1.27.	Obecabtagene autoleucel - EMEA-003171-PIP01-21	22
3.1.28.	Freeze-dried allergen extract of <i>Betula pendula</i> pollen - EMEA-003117-PIP02-21	23
3.1.29.	Cannabidiol - EMEA-003176-PIP01-21	23
3.1.30.	Atrasentan - Orphan - EMEA-001666-PIP02-21	23
3.1.31.	EMEA-003165-PIP01-21	23
3.1.32.	Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc - EMEA-003157-PIP01-21	23
3.1.33.	Repagermanium - Orphan - EMEA-003154-PIP01-21	23
3.1.34.	Pneumococcal polysaccharide serotype 35B – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 31 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 24F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 23B – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 23A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 16F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 15C – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 15A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 20 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 17F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 12F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 11A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 10A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 9N – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 8 – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide	

	serotype 22F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate / Pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate - EMEA-003155-PIP01-21	24
3.1.35.	Yellow fever virus, strain vYF-247 - EMEA-003030-PIP02-21	24
3.1.36.	Indapamide / perindopril arginine - EMEA-003186-PIP01-22	24
3.1.37.	A 2'-MOE antisense oligonucleotide targeting apoC-III - EMEA-003177-PIP01-21	25
3.1.38.	Treprostinil - EMEA-003182-PIP01-22	25
3.1.39.	Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22	25
3.1.40.	Lenzilumab - EMEA-003188-PIP01-22	25
3.1.41.	Flortaucipir F18 - EMEA-003187-PIP01-22	25
3.1.42.	Methylphenidate hydrochloride - EMEA-003189-PIP01-22	25
3.1.43.	Tolebrutinib - EMEA-002566-PIP02-22	26
3.1.44.	Anti-TGFbeta fully human monoclonal antibody - EMEA-003178-PIP01-21	26
3.1.45.	Sugemalimab - EMEA-003179-PIP01-22	26
3.1.46.	Enzastaurin hydrochloride - EMEA-003096-PIP02-22	26
3.1.47.	COVID-19 Vaccine (recombinant, adjuvanted) – EMEA-003191-PIP01-22	26
3.2.	Discussions on Compliance Check.....	27
3.2.1.	Landiolol hydrochloride - EMEA-C2-001150-PIP02-13-M04.....	27
3.2.2.	Alirocumab - EMEA-C1-001169-PIP01-11-M05	27
3.2.3.	Elosulfase alfa - EMEA-C2-000973-PIP01-10-M03	27
3.2.4.	Lumacaftor / ivacaftor - EMEA-C6-001582-PIP01-13-M10	27
3.2.5.	Ad26.RSV.preF is a monovalent, recombinant, replication-incompetent human adenovirus serotype 26-vectored vaccine encoding the pre-fusion conformation-stabilised F protein derived from the RSV A2 strain - EMEA-C1-002172-PIP02-17-M01	27
3.3.	Discussions on Modification of an Agreed Paediatric Investigation Plan.....	28
3.3.1.	Nemolizumab - EMEA-001624-PIP01-14-M05	28
3.3.2.	Canagliflozin (1s)-1,5-anhydro-1-[3-[[5-(4-fluorophenyl)-2-thienyl]methyl]-4-methylphenyl]-D-glucitol hemihydrate - EMEA-001030-PIP01-10-M10	28
3.3.3.	Aztreonam / avibactam - EMEA-002283-PIP01-17-M03	28
3.3.4.	Posaconazole - EMEA-000468-PIP02-12-M07	28
3.3.5.	Efinaconazole - EMEA-001627-PIP01-14-M02	28
3.3.6.	Dopamine hydrochloride - EMEA-001105-PIP01-10-M06	29
3.3.7.	Inebilizumab - Orphan - EMEA-001911-PIP01-15-M04	29
3.3.8.	Erdafitinib - EMEA-002042-PIP02-20-M01.....	29
3.3.9.	Tisagenlecleucel - Orphan - EMEA-001654-PIP01-14-M04.....	29
3.3.10.	Zanubrutinib - EMEA-002354-PIP02-18-M01	29
3.3.11.	Lanadelumab - Orphan - EMEA-001864-PIP01-15-M07	30
3.3.12.	Sodium chloride solution 4.2% (w/v) / 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide - Orphan - EMEA-002935-PIP01-20-M01.....	30

3.3.13.	Loxapine - EMEA-001115-PIP01-10-M08	30
3.3.14.	Mirabegron - EMEA-000597-PIP03-15-M05	30
3.3.15.	Ravulizumab (ALXN1210) - Orphan - EMEA-001943-PIP01-16-M07.....	30
3.3.16.	Ravulizumab (ALXN1210) - Orphan - EMEA-002077-PIP01-16-M05.....	30
3.3.17.	Ravulizumab (ALXN1210) - EMEA-001943-PIP02-20-M01	31

4. Nominations 31

4.1.	List of submissions of applications with start of procedure 21 March 2022 for Nomination of Rapporteur and Peer reviewer.....	31
4.2.	Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver	31
4.3.	Nominations for other activities	31

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction 31

6. Discussion on the applicability of class waivers 31

6.1.	Discussions on the applicability of class waiver for products.....	32
-------------	---	-----------

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver 32

7.1.	Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver	32
-------------	--	-----------

8. Annual reports on deferrals 32

9. Organisational, regulatory and methodological matters 32

9.1.	Mandate and organisation of the PDCO.....	32
9.1.1.	PDCO membership.....	32
9.1.2.	Vote by Proxy	32
9.1.3.	Strategic Review & Learning meeting (SRLM)	32
9.2.	Coordination with EMA Scientific Committees or CMDh-v	32
9.2.1.	Committee for Medicinal Products for Human Use (CHMP)	32
9.3.	Coordination with EMA Working Parties/Working Groups/Drafting Groups	33
9.3.1.	Non-clinical Working Group: D30 Products identified	33
9.3.2.	Formulation Working Group	33
9.3.3.	Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)	33
9.3.4.	EMA Emergency task force (ETF) – PDCO nominations	33
9.4.	Cooperation within the EU regulatory network.....	33
9.4.1.	European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA).....	33
9.5.	Cooperation with International Regulators.....	33
9.6.	Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee	33
9.7.	PDCO work plan.....	33

9.8.	Planning and reporting	34
9.8.1.	EMA Business Pipeline activity and Horizon scanning	34
10.	Any other business	34
10.1.	COVID-19 update.....	34
10.2.	Introducing DARWIN EU® Coordination Centre and next steps for RWE	34
10.3.	Data protection notice – processing of scientific Committees (CxMP) members/alternates’ contact details.....	34
10.4.	Compliance check training.....	34
11.	Breakout sessions	34
11.1.	Neonatology	34
11.2.	Paediatric oncology	34
11.3.	Vaccines	34
12.	Explanatory notes	35

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 22-25 March 2022. See (current) March 2022 PDCO minutes (to be published post April 2022 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 22-25 March 2022.

1.3. Adoption of the minutes

PDCO minutes for 22-25 February 2022.

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. Ibutamoren mesylate - Orphan - EMEA-003032-PIP01-21

Lumos Pharma, Inc.; Treatment of growth hormone deficiency

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP02-20

Treatment of obesity

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

[2.1.3. Zinc gluconate / alisitol / retinyl palmitate - Orphan - EMEA-002198-PIP01-21](#)

Vanessa Research Magyarorszag Kft/Vanessa Research Hungary ltd; Treatment of microvillus inclusion disease

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

[2.1.4. Mitapivat - Orphan - EMEA-002684-PIP02-21](#)

Agios Netherlands B.V.; Treatment of thalassaemia

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

[2.1.5. Deucravacitinib - EMEA-002350-PIP02-20](#)

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

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

[2.1.6. Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase - Orphan - EMEA-003020-PIP01-21](#)

Lysogene; Treatment of GM1 gangliosidosis

Day 120 opinion

Action: For adoption

Neurology

[2.1.7. Lorlatinib - EMEA-002669-PIP03-21](#)

Treatment of ALK-aberrant neuroblastoma

Day 120 opinion

Action: For adoption - Oral explanation to be held on Wednesday, 23 March 2022

Oncology

2.1.8. [Recombinant SARS-CoV-2 spike \(S\)-protein virus-like particle - EMEA-003008-PIP01-21](#)

Prevention of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Vaccines

2.1.9. [SARS-CoV-2 virus, beta-propiolactone inactivated - EMEA-003077-PIP01-21](#)

Prevention of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Vaccines

2.1.10. [Amlodipine / indapamide / perindopril arginine / atorvastatin - EMEA-003173-PIP01-21](#)

Treatment of hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.11. [Dupilumab - EMEA-001501-PIP11-21](#)

Treatment of chronic pruritus of unknown origin

Day 60 opinion

Action: For adoption

Dermatology

2.1.12. [Adeno-associated virus vector serotype 1 containing the human GRN gene - Orphan - EMEA-003167-PIP01-21](#)

Passage Bio, Inc.; Treatment of frontotemporal dementia

Day 60 opinion

Action: For adoption

Neurology

2.1.13. [Anti-CD40L humanized monoclonal antibody - EMEA-002945-PIP02-21](#)

Treatment of multiple sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.14. Izaflortaucipir (¹⁸F) - EMEA-003040-PIP02-21

Diagnosis of Alzheimer's disease

Day 60 opinion

Action: For adoption

Neurology

2.1.15. Latozinemab - Orphan - EMEA-002997-PIP02-22

Alector, Inc.; Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.16. EMEA-003169-PIP01-21

Treatment of gastrointestinal stromal tumours

Day 60 opinion

Action: For adoption

Oncology

2.1.17. Human IgG4-based bispecific antibody binding to both B-cell maturation antigen (BCMA) and cluster of differentiation 3 (CD3) - EMEA-003175-PIP01-21

Treatment of multiple myeloma

Day 60 opinion

Action: For adoption

Oncology

2.1.18. Infigratinib - Orphan - EMEA-002594-PIP03-21

Helsinn Birex Pharmaceuticals Ltd.; Treatment of urothelial carcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.19. Sacituzumab govitecan - Orphan - EMEA-002645-PIP03-21

Gilead Sciences International Ltd.; Treatment of lung carcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.20. Zandelisib - EMEA-003158-PIP01-21

Treatment of mature B cell neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.21. Triazolopyrimidine derivative - EMEA-003095-PIP01-21

Treatment of diabetic retinopathy / diabetic retinopathy

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.22. EMEA-003159-PIP01-21

Treatment of idiopathic pulmonary fibrosis (IPF)

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.1.23. Pseudoephedrine / bilastine - EMEA-003164-PIP01-21

Treatment of allergic rhinitis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.2. Opinions on Compliance Check

2.2.1. Treosulfan - EMEA-C-000883-PIP01-10-M05

medac Gesellschaft für klinische Spezialpräparate mbH; Conditioning treatment prior to

haematopoietic progenitor cell transplantation

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.2.2. Oritavancin - EMEA-C2-001270-PIP01-12-M04

Menarini International Operations Luxembourg S.A.; Treatment of acute bacterial skin and skin structure infections

Day 60 letter

Action: For adoption

Infectious Diseases

2.2.3. Dabrafenib - EMEA-C3-001147-PIP01-11-M07

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

Day 60 letter

Action: For adoption

Oncology

2.2.4. Trametinib - EMEA-C2-001177-PIP01-11-M06

Novartis Europharm Limited; Treatment of melanoma

Day 60 letter

Action: For adoption

Oncology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Regdanvimab - EMEA-002961-PIP01-21-M01

Celltrion Healthcare Hungary Kft.; Treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Action: For adoption

Vaccines

2.3.2. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20-M01

Amgen Europe B.V.; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.3. Remibrutinib - EMEA-002582-PIP01-19-M01

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 60 opinion

Action: For adoption

Dermatology

2.3.4. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19-M02

Alexion Europe S.A.S.; Treatment of Wilson disease

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Dapagliflozin - EMEA-000694-PIP02-14-M03

AstraZeneca AB; Treatment of type 1 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Tirzepatide - EMEA-002360-PIP01-18-M02

Eli Lilly and Company Ltd; Treatment type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Tolvaptan - EMEA-001231-PIP02-13-M09

Otsuka Pharmaceutical Netherlands B.V.; Treatment of polycystic kidney disease (PKD)

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

2.3.8. Odevixibat - Orphan - EMEA-002054-PIP01-16-M03

Albireo AB; Treatment of progressive familial intrahepatic cholestasis (PFIC)

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.9. Potassium sulphate / magnesium sulphate heptahydrate / sodium sulphate anhydrous - EMEA-000816-PIP02-10-M03

IPSEN Consumer Healthcare; Diagnostic of organic and/or functional bowel diseases

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.10. Betibeglogene autotemcel - Orphan - EMEA-001665-PIP01-14-M06

bluebird bio (Netherlands) B.V.; Treatment of beta-thalassaemia

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.11. Luspatercept - Orphan - EMEA-001521-PIP01-13-M06

Bristol-Myers Squibb Pharma EEIG; Treatment of beta-thalassaemia

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.12. Brincidofovir - Orphan - EMEA-001904-PIP03-18-M02

Chimerix IRL Limited; Treatment of smallpox

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Cefiderocol - EMEA-002133-PIP01-17-M02

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Eslicarbazepine acetate - EMEA-000696-PIP02-10-M08

BIAL - Portela & Ca, SA; Treatment of epilepsy with partial onset seizures

Day 60 opinion

Action: For adoption

Neurology

2.3.15. Cemiplimab - EMEA-002007-PIP02-17-M02

Regeneron Ireland DAC; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 60 opinion

Action: For adoption

Oncology

2.3.16. Lenvatinib - EMEA-001119-PIP03-19-M02

Eisai GmbH; Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue neoplasms, papillary thyroid cancer, follicular thyroid cancer and osteosarcoma

Day 60 opinion

Action: For adoption

Oncology

2.3.17. Venetoclax - Orphan - EMEA-002018-PIP02-16-M05

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

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.3.18. Sonlicromanol - Orphan - EMEA-002113-PIP01-16-M01

Khondrion BV; Treatment of mitochondrial respiratory chain/oxidative phosphorylation defects

Day 60 opinion

Action: For adoption

Other

2.3.19. Ketamine / sufentanil - EMEA-001739-PIP02-16-M01

Cessatech A/S; Treatment of acute pain

Day 60 opinion

Action: For adoption

Pain

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.

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. Dersimelagon - EMEA-002850-PIP02-21

Treatment of X-linked protoporphyria / Treatment of erythropoietic protoporphyria

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. Humanised KLB/FGFR1c monoclonal antibody - EMEA-003058-PIP01-21

Treatment of non-alcoholic steatohepatitis

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Apraglutide - Orphan - EMEA-003016-PIP01-21

VectivBio AG; Treatment of short bowel syndrome

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.4. Recombinant adeno-associated viral (rAAV) vector expressing the human ornithine transcarbamylase (hOTC) gene - EMEA-002983-PIP01-21

Treatment of ornithine transcarbamylase deficiency

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.5. Sirolimus - EMEA-002982-PIP01-21

Treatment of ornithine transcarbamylase deficiency

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.6. Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate (ECT-001-CB) - Orphan - EMEA-003025-PIP02-21

ExCellThera; Allogeneic haematopoietic stem cell transplantation

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Oncology / Haematology-Hemostaseology

3.1.7. [Reparixin - Orphan - EMEA-001693-PIP03-21](#)

Dompé farmaceutici S.p.A.; Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.8. [Omaveloxolone - Orphan - EMEA-002487-PIP01-18](#)

Reata Ireland Limited; Treatment of Friedreich's ataxia

Day 90 discussion

Action: For discussion

Neurology

3.1.9. [Cedazuridine: \(4R\)-1-\[\(2R,4R,5R\)-3,3-difluoro-4-hydroxy-5-\(hydroxymethyl\)oxolan-2-yl\]-4-hydroxy-1,3-diazinan-2-one / decitabine: 4-amino-1-\[\(2R,4S,5R\)-4-hydroxy-5-\(hydroxymethyl\)oxolan-2-yl\]-1,3,5-triazin-2\(1H\)-one - Orphan - EMEA-003071-PIP01-21](#)

Otsuka Pharmaceutical Netherlands B.V.; Treatment of acute myeloid leukaemia

Day 90 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.10. [Whole-cell heat-inactivated bacterial strains of *Escherichia coli*, *klebsiella pneumoniae*, *proteus vulgaris* and *enterococcus faecalis* - EMEA-003026-PIP02-21](#)

Prevention of recurrent urinary tract infections

Day 90 discussion

Action: For discussion

Vaccines / Infectious Diseases / Uro-nephrology

3.1.11. [Cedirogant - EMEA-003142-PIP02-21](#)

Treatment of moderate to severe psoriasis

Day 60 discussion

Action: For discussion

Dermatology

[3.1.12. Sirolimus - Orphan - EMEA-003168-PIP01-21](#)

Desitin Arzneimittel GmbH; Treatment of tuberous sclerosis

Day 60 discussion

Action: For discussion

Dermatology

[3.1.13. Manganese chloride tetrahydrate - EMEA-003035-PIP02-21](#)

Diagnostic evaluation of liver lesions by magnetic resonance imaging (MRI)

Day 60 discussion

Action: For discussion

Diagnostic

[3.1.14. Avexitide acetate - Orphan - EMEA-003125-PIP02-21](#)

EigerBio Europe Limited; Treatment of congenital hyperinsulinism

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

[3.1.15. Glucagon analogue linked to a human immunoglobulin Fc fragment - Orphan - EMEA-003170-PIP01-21](#)

Hanmi Pharm. Co., Ltd.; Treatment of congenital hyperinsulinism

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

[3.1.16. Insulin lispro - EMEA-003166-PIP01-21](#)

Treatment of diabetes mellitus type 1 / Treatment of diabetes mellitus type 2

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.17. Semaglutide - EMEA-001441-PIP07-21

Treatment of obesity

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. Efavaleukin alfa - EMEA-003156-PIP01-21

Treatment of systemic lupus erythematosus (SLE)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.19. HIV-1 maturation inhibitor - EMEA-003153-PIP01-21

Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.20. HIV-1 maturation inhibitor / dolutegravir - EMEA-003152-PIP01-21

Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.21. Interferon beta-1a - EMEA-003056-PIP01-22

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.22. Batoclimab - EMEA-003162-PIP01-21

Treatment of generalized myasthenia gravis

Day 60 discussion

Action: For discussion

Neurology

3.1.23. Pridopidine hydrochloride - Orphan - EMEA-003174-PIP01-21

Prilenia Therapeutics B.V.; Treatment of Huntington disease (HD)

Day 60 discussion

Action: For discussion

Neurology

3.1.24. Satralizumab - Orphan - EMEA-001625-PIP03-21

Roche Registration GmbH; Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 60 discussion

Action: For discussion

Neurology

3.1.25. Camidanlumab tesirine - EMEA-003160-PIP01-21

Treatment of Hodgkin lymphoma

Day 60 discussion

Action: For discussion

Oncology

3.1.26. Emactuzumab - EMEA-003172-PIP01-21

Treatment of tenosynovial giant cell tumour, local and diffuse type

Day 60 discussion

Action: For discussion

Oncology

3.1.27. Obecabtagene autoleucel - EMEA-003171-PIP01-21

Treatment of acute lymphoblastic leukaemia

Day 60 discussion

Action: For discussion

Oncology

3.1.28. Freeze-dried allergen extract of *Betula pendula* pollen - EMEA-003117-PIP02-21

Diagnosis of IgE mediated allergy to tree pollen of the birch group

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.29. Cannabidiol - EMEA-003176-PIP01-21

Treatment of fragile X syndrome (FXS)

Day 60 discussion

Action: For discussion

Psychiatry

3.1.30. Atrasentan - Orphan - EMEA-001666-PIP02-21

Chinook Therapeutics, Inc.; Treatment of primary IgA nephropathy

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.31. EMEA-003165-PIP01-21

Treatment of chronic kidney disease

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.32. Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc - EMEA-003157-PIP01-21

Treatment of focal segmental glomerulosclerosis (FSGS)

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.33. Repagermanium - Orphan - EMEA-003154-PIP01-21

Dimerix Bioscience Pty Ltd; Treatment of focal segmental glomerulosclerosis (FSGS)

Day 60 discussion

Action: For discussion

Uro-nephrology

- 3.1.34. Pneumococcal polysaccharide serotype 35B – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 31 – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 24F – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 23B – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 23A – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 16F – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 15C – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 15A – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 20 – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 17F – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 12F – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 11A – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 10A – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 9N – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 8 – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate /
Pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate - EMEA-003155-PIP01-21
-

Prevention of disease caused by *Streptococcus pneumoniae*

Day 60 discussion

Action: For discussion

Vaccines

- 3.1.35. Yellow fever virus, strain vYF-247 - EMEA-003030-PIP02-21
-

Prevention of yellow fever disease

Day 60 discussion

Action: For discussion

Vaccines

- 3.1.36. Indapamide / perindopril arginine - EMEA-003186-PIP01-22
-

Treatment of (essential) primary hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

[3.1.37. A 2'-MOE antisense oligonucleotide targeting apoC-III - EMEA-003177-PIP01-21](#)

Treatment of severe and extreme hypertriglyceridaemia / Treatment of familial chylomicronaemia syndrome

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

[3.1.38. Treprostinil - EMEA-003182-PIP01-22](#)

Treatment of pulmonary arterial hypertension (PAH) group 1

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

[3.1.39. Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22](#)

Krystal Biotech, Inc.; Treatment of dystrophic epidermolysis bullosa

Day 30 discussion

Action: For discussion

Dermatology

[3.1.40. Lenzilumab - EMEA-003188-PIP01-22](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

[3.1.41. Flortaucipir F18 - EMEA-003187-PIP01-22](#)

Diagnosis of Alzheimer's disease

Day 30 discussion

Action: For discussion

Neurology

[3.1.42. Methylphenidate hydrochloride - EMEA-003189-PIP01-22](#)

Treatment of attention-deficit hyperactivity disorder

Day 30 discussion

Action: For discussion

Neurology

3.1.43. Tolebrutinib - EMEA-002566-PIP02-22

Treatment of myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.1.44. Anti-TGFbeta fully human monoclonal antibody - EMEA-003178-PIP01-21

Treatment of metastatic pancreatic ductal adenocarcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.45. Sugemalimab - EMEA-003179-PIP01-22

Treatment of non-small cell lung cancer (NSCLC)

Day 30 discussion

Action: For discussion

Oncology

3.1.46. Enzastaurin hydrochloride - EMEA-003096-PIP02-22

Treatment of vascular Ehlers-Danlos syndrome

Day 30 discussion

Action: For discussion

Other

3.1.47. COVID-19 Vaccine (recombinant, adjuvanted) – EMEA-003191-PIP01-22

HIPRA Human Health S.L.U.; Prevention of COVID-19

Day 30 discussion

Action: For discussion

Infectious Diseases, 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. Landiolol hydrochloride - EMEA-C2-001150-PIP02-13-M04

Orpha-Devel Handels und Vertriebs GmbH; Treatment of supraventricular arrhythmias

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.2.2. Alirocumab - EMEA-C1-001169-PIP01-11-M05

sanofi-aventis recherche & développement; Treatment of elevated cholesterol

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Elosulfase alfa - EMEA-C2-000973-PIP01-10-M03

BioMarin International Limited; Treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.4. Lumacaftor / ivacaftor - EMEA-C6-001582-PIP01-13-M10

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

Action: For discussion

Other

3.2.5. Ad26.RSV.preF is a monovalent, recombinant, replication-incompetent human adenovirus serotype 26-vectored vaccine encoding the pre-fusion conformation-stabilised F protein derived from the RSV A2 strain - EMEA-C1-002172-PIP02-17-M01

Janssen-Cilag International NV; Prevention of lower respiratory tract disease caused by respiratory syncytial virus

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Nemolizumab - EMEA-001624-PIP01-14-M05

Galderma International S.A.S; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Canagliflozin (1s)-1,5-anhydro-1-[3-[[5-(4-fluorophenyl)-2-thienyl]methyl]-4-methylphenyl]-D-glucitol hemihydrate - EMEA-001030-PIP01-10-M10

Janssen-Cilag International NV; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Aztreonam / avibactam - EMEA-002283-PIP01-17-M03

Pfizer Europe MA EEIG; Treatment of infections caused by aerobic gram-negative bacteria

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.4. Posaconazole - EMEA-000468-PIP02-12-M07

Merck Sharp & Dohme (Europe), Inc.; Treatment of invasive fungal infections / Prevention of invasive fungal infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.5. Efinaconazole - EMEA-001627-PIP01-14-M02

Almirall, S.A.; Treatment of onychomycosis (tinea unguium)

Day 30 discussion

Action: For discussion

3.3.6. Dopamine hydrochloride - EMEA-001105-PIP01-10-M06

Breco Biopharma Limited; Treatment of hypotension in neonates including the extremely low gestational age newborn / Treatment of hypotension in infants and children

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

3.3.7. Inebilizumab - Orphan - EMEA-001911-PIP01-15-M04

Horizon Therapeutics Ireland DAC; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

Action: For discussion

Neurology

3.3.8. Erdafitinib - EMEA-002042-PIP02-20-M01

Janssen-Cilag International N.V.; Treatment of all conditions included in the category of malignant neoplasms (except urothelial carcinoma, haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Action: For discussion

Oncology

3.3.9. Tisagenlecleucel - Orphan - EMEA-001654-PIP01-14-M04

Novartis Europharm Limited; Treatment of B cell acute lymphoblastic leukaemia/lymphoblastic lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.3.10. Zanubrutinib - EMEA-002354-PIP02-18-M01

BeiGene Ireland Ltd.; Treatment of lymphoplasmacytic lymphoma/ Treatment of mature B cell neoplasms (excluding lymphoplasmacytic lymphoma)

Day 30 discussion

Action: For discussion

Oncology

3.3.11. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M07

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of hereditary angioedema

Day 30 discussion

Action: For discussion

Other

3.3.12. Sodium chloride solution 4.2% (w/v) / 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide - Orphan - EMEA-002935-PIP01-20-M01

Parion Sciences, Inc.; Treatment of primary ciliary dyskinesia (PCD)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.13. Loxapine - EMEA-001115-PIP01-10-M08

Ferrer Internacional, S.A.; Treatment of bipolar disorder / schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.3.14. Mirabegron - EMEA-000597-PIP03-15-M05

Astellas Pharma Europe B.V.; Treatment of neurogenic detrusor overactivity

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.15. Ravulizumab (ALXN1210) - Orphan - EMEA-001943-PIP01-16-M07

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.16. Ravulizumab (ALXN1210) - Orphan - EMEA-002077-PIP01-16-M05

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.17. Ravulizumab (ALXN1210) - EMEA-001943-PIP02-20-M01

Alexion Europe SAS; Treatment of patients with haematopoietic stem cell transplant associated thrombotic microangiopathy (HSCT-TMA)

Day 30 discussion

Action: For discussion

Uro-nephrology / Haematology-Hemostaseology

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 21 March 2022 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

No item

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 & Learning meeting (SRLM)

Strategic Review & Learning meeting – joint COMP/PDCO meeting under the French Presidency of the Council of the EU, to be held virtually on 31 March 2022

PDCO member: Sylvie Benchetrit

Action: For information

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Action: For information

9.3.3. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

Nomination of a representative (and alternate) for PCWP/HCPWP for a new three-year mandate (June 2022 to May 2025).

PDCO member: Koenraad Norga

Action: For information

9.3.4. EMA Emergency task force (ETF) – PDCO nominations

PDCO member: Koenraad Norga

Action: For information

9.4. Cooperation within the EU regulatory network

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

Action: For information

9.5. Cooperation with International Regulators

No item

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q1/2022 Update of the Business Pipeline report for the human scientific committees

Action: For information

10. Any other business

10.1. COVID-19 update

Action: For information

10.2. Introducing DARWIN EU® Coordination Centre and next steps for RWE

Action: For information

10.3. Data protection notice – processing of scientific Committees (CxMP) members/alternates' contact details

Action: For information

10.4. Compliance check training

Action: For information

11. Breakout sessions

11.1. Neonatology

Action: For discussion on Tuesday, 13:00-14:00

11.2. Paediatric oncology

Action: For discussion on Wednesday, 13:00-14:00

11.3. Vaccines

Action: For discussion on Thursday 13:00-14:00

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/