



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

17 May 2023  
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Human Medicines Division

## Paediatric Committee (PDCO)

### Draft Agenda for the meeting on 23-26 May 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

23 May 2023, 14:00 - 19:00, Virtual meeting

24 May 2023, 08:30 - 19:00, Virtual meeting

25 May 2023, 08:30 - 19:00, Virtual meeting

26 May 2023, 08:30 - 13:00, Virtual 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 ongoing procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).



# Table of contents

<b>1.</b>	<b>Introductions</b>	<b>9</b>
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
<b>2.</b>	<b>Opinions</b>	<b>9</b>
<b>2.1.</b>	<b>Opinions on Products.....</b>	<b>9</b>
2.1.1.	Adapalene, micronised / benzoyl peroxide, hydrous / clindamycin - EMEA-003263-PIP01-229	
2.1.2.	Spesolimab - EMEA-002475-PIP03-22.....	9
2.1.3.	EMEA-003019-PIP01-21 .....	10
2.1.4.	Izokibep - EMEA-003325-PIP01-22.....	10
2.1.5.	Lenacapavir / bictegravir - EMEA-003324-PIP01-22 .....	10
2.1.6.	Posoleucel - Orphan - EMEA-002908-PIP02-22.....	10
2.1.7.	1-(4-(6-chloropyridazin-3-yl)piperazin-1-yl)-2-(4-cyclopropyl-3-fluorophenyl)ethan-1-one - Orphan - EMEA-003268-PIP01-22 .....	10
2.1.8.	Immunoglobulin G4 [228-proline], anti-( <i>Betula alleghaniensis</i> allergen Bet v 1) (human monoclonal $\gamma$ 4-chain), disulphide with human monoclonal $\kappa$ -chain, dimer / immunoglobulin G4 [227-proline], anti-( <i>Betula alleghaniensis</i> allergen Bet v 1) (human monoclonal $\gamma$ 4-chain), disulphide with human monoclonal $\kappa$ -chain, dimer / immunoglobulin G4 [224-proline], anti-( <i>Betula alleghaniensis</i> allergen Bet v 1) (human monoclonal $\gamma$ 4-chain), disulphide with human monoclonal $\kappa$ -chain, dimer - EMEA-003270-PIP01-22.....	10
2.1.9.	EMEA-003276-PIP01-22 .....	11
2.1.10.	Phuket modRNA / Darwin modRNA / Austria modRNA / Wisconsin modRNA - EMEA-003318-PIP01-22 .....	11
2.1.11.	Zapomeran - EMEA-003349-PIP01-22.....	11
2.1.12.	Acetylsalicylic acid / rosuvastatin - EMEA-003410-PIP01-23 .....	11
2.1.13.	Rivaroxaban / amlodipine - EMEA-003412-PIP01-23 .....	12
2.1.14.	Deucravacitinib - EMEA-002350-PIP05-23 .....	12
2.1.15.	Efzofitimod - Orphan - EMEA-003352-PIP02-23.....	12
2.1.16.	Secukinumab - EMEA-000380-PIP11-23 .....	12
2.1.17.	Reparixin - Orphan - EMEA-001693-PIP05-23 .....	12
2.1.18.	Opicapone - EMEA-003406-PIP01-23 .....	12
2.1.19.	EMEA-003409-PIP01-23 .....	13
2.1.20.	Therapeutic DNA plasmid vaccine targeting HPV16 E6 and E7 proteins - EMEA-003403-PIP01-23 .....	13
2.1.21.	Fuzuloparib - EMEA-003422-PIP01-23 .....	13
2.1.22.	Nizaracianine triflutate - EMEA-003367-PIP01-22 .....	13
<b>2.2.</b>	<b>Opinions on Compliance Check .....</b>	<b>13</b>
2.2.1.	Lenvatinib - EMEA-C-001119-PIP02-12-M08 .....	13

2.2.2.	Crisantaspase - EMEA-C-002934-PIP01-20 .....	14
2.2.3.	Baloxavir marboxil - EMEA-C3-002440-PIP01-18-M04 .....	14
<b>2.3.</b>	<b>Opinions on Modification of an Agreed Paediatric Investigation Plan .....</b>	<b>14</b>
2.3.1.	Azilsartan medoxomil - EMEA-000237-PIP01-08-M11 .....	14
2.3.2.	Macitentan - Orphan - EMEA-001032-PIP01-10-M06 .....	14
2.3.3.	Mavacamten - EMEA-002231-PIP01-17-M01 .....	14
2.3.4.	Nemolizumab - EMEA-001624-PIP01-14-M06.....	15
2.3.5.	Rocatinlimab - EMEA-002886-PIP01-20-M03.....	15
2.3.6.	Recombinant parathyroid hormone: rhPTH (1-84) - Orphan - EMEA-001526-PIP01-13-M0615	
2.3.7.	Potassium chloride / sodium chloride / ascorbic acid / sodium sulfate / sodium ascorbate / polyethylene glycol 3350 - EMEA-001705-PIP02-15-M04 .....	15
2.3.8.	Efgartigimod alfa - Orphan - EMEA-002597-PIP04-21-M01.....	15
2.3.9.	Ixekizumab - EMEA-001050-PIP02-18-M02 .....	16
2.3.10.	Tenofovir alafenamide / emtricitabine / bictegravir - EMEA-001766-PIP01-15-M05 .....	16
2.3.11.	Soticlestat - Orphan - EMEA-002572-PIP02-19-M03.....	16
2.3.12.	(4S,7aR,9aR,10S,11E,14S,15R)-6'-chloro-10-methoxy-14,15-dimethyl-3',4',7a,8,9,9a,10,13,14,15-decahydro-2'H,7H-spiro[1,19-ethenocyclobuta[i][1,4]oxazepino[3,4f][1,2,7]thiadiazacyclohexadecine-4,1'-naphthalen]-18(17H)-one 16,16-dioxide (AMG 176) - EMEA-002631-PIP01-19-M02.....	16
2.3.13.	Magrolimab - Orphan - EMEA-002819-PIP01-20-M01 .....	16
2.3.14.	Talimogene laherparepvec - EMEA-001251-PIP01-11-M06 .....	17
2.3.15.	Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M05.....	17
2.3.16.	Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M04.....	17
2.3.17.	Pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed) - EMEA-002330-PIP01-18-M03 .....	17
2.3.18.	Modified vaccinia Ankara - Bavarian Nordic virus (smallpox) - EMEA-001161-PIP02-11-M0317	
2.3.19.	NVX-CoV2373 - EMEA-002941-PIP01-20-M03 .....	17
2.3.20.	Recombinant COVID-19 subunit nanoparticle (adjuvanted with AS03) (GBP510) - EMEA-003115-PIP01-21-M01 .....	18
<b>2.4.</b>	<b>Opinions on Re-examinations .....</b>	<b>18</b>
2.4.1.	Adtralza - EMEA-001900-PIP02-17-M07 .....	18
2.4.2.	Posaconazole - EMEA-000468-PIP02-12-M08 .....	18
<b>2.5.</b>	<b>Opinions on Review of Granted Waivers .....</b>	<b>18</b>
<b>2.6.</b>	<b>Finalisation and adoption of Opinions.....</b>	<b>18</b>
<b>2.7.</b>	<b>Partial Compliance Checks completed by EMA .....</b>	<b>18</b>
2.7.1.	Selexipag - EMEA-C2-000997-PIP01-10-M06 .....	19
2.7.2.	Evinacumab - EMEA-C2-002298-PIP01-17-M05 .....	19
2.7.3.	(1R,3S,5R)-2-(2-(3-acetyl-5-(2-methylpyrimidin-5-yl)-1H-indazol-1-yl)acetyl)-N-(6-bromo-3-methylpyridin-2-yl)-5-methyl-2-azabicyclo[3.1.0]hexane-3-carboxamide (ALXN2050, vemircopan) - EMEA-C1-002863-PIP01-20 .....	19
2.7.4.	Etranacogene dezaparovec - EMEA-C1-002722-PIP01-19-M02 .....	19

2.7.5.	Delgocitinib - EMEA-C1-002329-PIP02-20-M02 .....	19
2.7.6.	Eliglustat - EMEA-C2-000461-PIP02-11-M05.....	20

### **3. Discussion of applications** **20**

<b>3.1.</b>	<b>Discussions on Products D90-D60-D30.....</b>	<b>20</b>
3.1.1.	Furosemide - EMEA-003316-PIP01-22 .....	20
3.1.2.	Milvexian - EMEA-003220-PIP01-22 .....	20
3.1.3.	EMEA-003301-PIP01-22 .....	20
3.1.4.	Povorcitinib - EMEA-003313-PIP01-22 .....	20
3.1.5.	Ritlecitinib - EMEA-002451-PIP03-22 .....	21
3.1.6.	Encalret - Orphan - EMEA-003348-PIP01-22 .....	21
3.1.7.	Dirloctocogene samoparvovec - Orphan - EMEA-003290-PIP01-22 .....	21
3.1.8.	Mocravimod - Orphan - EMEA-003304-PIP01-22 .....	21
3.1.9.	Recombinant human arylsulfatase A - Orphan - EMEA-002050-PIP02-22.....	21
3.1.10.	EMEA-003271-PIP01-22 .....	22
3.1.11.	EMEA-003274-PIP01-22 .....	22
3.1.12.	Dersimelagon - EMEA-002850-PIP03-22 .....	22
3.1.13.	mRNA encoding modified human ornithine transcarbamylase - Orphan - EMEA-003315-PIP01-22 .....	22
3.1.14.	Pamrevlumab - EMEA-002979-PIP04-22 .....	22
3.1.15.	Repagermanium - Orphan - EMEA-003154-PIP01-21.....	22
3.1.16.	Influenza recombinant H7 haemagglutinin - EMEA-003314-PIP01-22.....	23
3.1.17.	Single-stranded 5' capped mRNA encoding the HAs of the influenza virus - EMEA-003346-PIP01-22.....	23
3.1.18.	MVA-BN-RSV - EMEA-003185-PIP01-22 .....	23
3.1.19.	Influenza virus type B, whole virion, inactivated / influenza virus type A H3N2, whole virion, inactivated / influenza virus type A H1N1, whole virion, inactivated - EMEA-003267-PIP02-23 .....	23
3.1.20.	Semaglutide - EMEA-003402-PIP01-23 .....	23
3.1.21.	Tetrahydrouridine / decitabine - Orphan - EMEA-003404-PIP01-23 .....	24
3.1.22.	EMEA-003350-PIP01-23 .....	24
3.1.23.	Belimumab - EMEA-000520-PIP03-23 .....	24
3.1.24.	Daxdilimab - EMEA-003411-PIP01-23 .....	24
3.1.25.	Nipocalimab - Orphan - EMEA-002559-PIP08-23 .....	24
3.1.26.	Niclosamide ethanolamine - EMEA-003407-PIP01-23 .....	24
3.1.27.	Povidone-iodine - EMEA-003413-PIP01-23 .....	25
3.1.28.	Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP01-23 .....	25
3.1.29.	Vemircopan - Orphan - EMEA-002863-PIP02-23.....	25
3.1.30.	mRNA encoding CMV gB / mRNA encoding the gH protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL131A protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL130 protein in the CMV glycoprotein complex pentamer / mRNA	

	encoding the gL protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL128 protein in the CMV glycoprotein complex pentamer - EMEA-003405-PIP01-23.....	25
3.1.31.	Remibrutinib - EMEA-002582-PIP03-23.....	25
3.1.32.	Spesolimab - EMEA-002475-PIP04-23.....	26
3.1.33.	EMEA-003414-PIP01-23 .....	26
3.1.34.	EMEA-003414-PIP02-23 .....	26
3.1.35.	Frexalimab - EMEA-002945-PIP03-23 .....	26
3.1.36.	Mavodelpar - Orphan - EMEA-003331-PIP02-23 .....	26
3.1.37.	Modified messenger ribonucleic acid encoding human propionyl-coenzyme a carboxylase alpha and beta subunits encapsulated into lipid nanoparticles - Orphan - EMEA-003419-PIP01-2327	
3.1.38.	Semaglutide - EMEA-001441-PIP08-23 .....	27
3.1.39.	Semaglutide / cagrilintide - EMEA-003059-PIP02-23 .....	27
3.1.40.	Sepiapterin - Orphan - EMEA-003027-PIP02-23 .....	27
3.1.41.	Cobitolimod - Orphan - EMEA-001589-PIP02-23.....	27
3.1.42.	Autologous CD34+ cells transduced with a lentiviral vector containing a codon-optimised RPS19 gene - Orphan - EMEA-003074-PIP02-23 .....	28
3.1.43.	Rilzabrutinib - Orphan - EMEA-002438-PIP04-23 .....	28
3.1.44.	Belumosudil - Orphan - EMEA-003425-PIP01-23 .....	28
3.1.45.	Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP03-23 .....	28
3.1.46.	Sotuletinib - Orphan - EMEA-003415-PIP01-23 .....	28
3.1.47.	Abemaciclib - EMEA-002342-PIP04-23 .....	28
3.1.48.	Atezolizumab / tiragolumab - EMEA-003418-PIP01-23 .....	29
3.1.49.	Autologous dendritic cells pulsed with allogeneic tumour cell lysate - Orphan - EMEA-002381-PIP02-23 .....	29
3.1.50.	Blinatumomab - Orphan - EMEA-000574-PIP03-23 .....	29
3.1.51.	Domvanalimab - EMEA-003429-PIP01-23.....	29
3.1.52.	Domvanalimab - EMEA-003429-PIP02-23.....	29
3.1.53.	Irinotecan - Orphan - EMEA-003416-PIP01-23 .....	30
3.1.54.	Olutasidenib - Orphan - EMEA-003421-PIP01-23 .....	30
3.1.55.	Tazemetostat - Orphan - EMEA-003055-PIP02-23 .....	30
3.1.56.	Volrustomig - EMEA-003423-PIP01-23.....	30
3.1.57.	Zimberelimab - EMEA-003427-PIP01-23 .....	30
3.1.58.	Zimberelimab - EMEA-003427-PIP02-23 .....	30
3.1.59.	Cedazuridine / decitabine - Orphan - EMEA-003071-PIP02-23 .....	31
3.1.60.	Satralizumab - Orphan - EMEA-001625-PIP05-23 .....	31
3.1.61.	Iptacopan - EMEA-002705-PIP05-23.....	31
3.1.62.	Autologous cell product composed of CD34+ enriched haematopoietic stem cells (HSCs) that have been genetically modified to express the functional $\beta$ -glucocerebrosidase (GCase) protein - Orphan - EMEA-003424-PIP01-23 .....	31
3.1.63.	EMEA-003417-PIP01-23 .....	31

3.1.64.	mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 - EMEA-003426-PIP01-23 .....	32
<b>3.2.</b>	<b>Discussions on Compliance Check.....</b>	<b>32</b>
3.2.1.	Clascoterone - EMEA-C-003330-PIP01-22 .....	32
3.2.2.	Marstacimab - EMEA-C1-002285-PIP02-19-M02 .....	32
3.2.3.	Rilpivirine (hydrochloride) - EMEA-C-000317-PIP01-08-M13.....	32
3.2.4.	Eladocagene exuparvovec - EMEA-C-002435-PIP01-18-M03.....	33
3.2.5.	Teriflunomide - EMEA-C-001094-PIP01-10-M04 .....	33
3.2.6.	Imetelstat - EMEA-C1-001910-PIP03-20-M01 .....	33
3.2.7.	Peanut ( <i>Arachis hypogaea</i> ) allergen powder (previously known as peanut flour) - EMEA-C-001734-PIP01-14-M06 .....	33
3.2.8.	Influenza virus surface antigens - A/turkey/Turkey/1/05 (H5N1) - EMEA-C-000599-PIP01-09-M07.....	33
<b>3.3.</b>	<b>Discussions on Modification of an Agreed Paediatric Investigation Plan.....</b>	<b>34</b>
3.3.1.	Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22-M01 .....	34
3.3.2.	Birch bark extract - Orphan - EMEA-001299-PIP03-17-M02 .....	34
3.3.3.	Dupilumab - EMEA-001501-PIP07-20-M01.....	34
3.3.4.	Inclisiran - EMEA-002214-PIP01-17-M02.....	34
3.3.5.	Obeticholic acid - Orphan - EMEA-001304-PIP02-13-M07 .....	34
3.3.6.	PF-06865571 - EMEA-002773-PIP01-20-M01 .....	35
3.3.7.	Iron as ferric maltol - EMEA-001195-PIP01-11-M06 .....	35
3.3.8.	Ibrutinib - EMEA-001397-PIP04-17-M02.....	35
3.3.9.	Tofacitinib citrate - EMEA-000576-PIP01-09-M15 .....	35
3.3.10.	Cobicistat / darunavir - EMEA-001280-PIP01-12-M06.....	35
3.3.11.	Delandistrogene moxeparvovec - Orphan - EMEA-002677-PIP01-19-M03.....	35
3.3.12.	Erenumab - EMEA-001664-PIP02-15-M06 .....	36
3.3.13.	Givinostat - Orphan - EMEA-000551-PIP04-21-M02 .....	36
3.3.14.	Lacosamide - EMEA-000402-PIP03-17-M07 .....	36
3.3.15.	Abemaciclib - EMEA-002342-PIP01-18-M03 .....	36
3.3.16.	Abemaciclib - EMEA-002342-PIP02-18-M02 .....	36
3.3.17.	Avapritinib - Orphan - EMEA-002358-PIP02-18-M03 .....	37
3.3.18.	Cabozantinib - Orphan - EMEA-001143-PIP01-11-M06 .....	37
3.3.19.	Olaparib - EMEA-002269-PIP01-17-M02.....	37
3.3.20.	Quizartinib - Orphan - EMEA-001821-PIP01-15-M07 .....	37
3.3.21.	Bupropion (hydrochloride) / naltrexone (hydrochloride) - EMEA-001373-PIP01-12-M05 ...	37
3.3.22.	Tirzepatide - EMEA-002360-PIP02-22-M01 .....	37
3.3.23.	Ketamine / sufentanil - EMEA-001739-PIP02-16-M02.....	38

<b>4.</b>	<b>Nominations</b>	<b>38</b>
4.1.	List of submissions of applications with start of procedure 22 May 2023 for Nomination of Rapporteur and Peer reviewer.....	38
4.2.	Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver .....	38
4.3.	Nominations for other activities .....	38
<b>5.</b>	<b>Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction</b>	<b>38</b>
<b>6.</b>	<b>Discussion on the applicability of class waivers</b>	<b>38</b>
6.1.	Discussions on the applicability of class waiver for products.....	39
6.1.1.	Bepranemab - EMEA-02-2023.....	39
6.1.2.	Ixoberogene soroparvovec - EMEA-03-2023 .....	39
<b>7.</b>	<b>Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver</b>	<b>39</b>
7.1.	Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver.....	39
7.1.1.	Ziltivekimab - EMEA-002840-PIP01-20 .....	39
<b>8.</b>	<b>Annual reports on deferrals</b>	<b>39</b>
<b>9.</b>	<b>Organisational, regulatory and methodological matters</b>	<b>39</b>
9.1.	Mandate and organisation of the PDCO .....	39
9.1.1.	PDCO membership.....	39
9.1.2.	Vote by Proxy .....	40
9.1.3.	Strategic Review and Learning Meeting (SRLM) - Uppsala, 7-8 June 2023 .....	40
9.2.	Coordination with EMA Scientific Committees or CMDh-v .....	40
9.2.1.	Committee for Medicinal Products for Human Use (CHMP).....	40
9.3.	Coordination with EMA Working Parties/Working Groups/Drafting Groups .....	40
9.3.1.	Non-clinical Working Party: D30 Products identified .....	40
9.3.2.	Formulation Working Group.....	40
9.3.3.	Patients and Consumers Working Party (PCWP)/Healthcare Professionals Working Party (HCPWP) .....	40
9.4.	Cooperation within the EU regulatory network.....	41
9.4.1.	European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA) .....	41
9.5.	Cooperation with International Regulators.....	41
9.5.1.	Paediatric Cluster Teleconference .....	41
9.6.	Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee .....	41
9.7.	PDCO work plan.....	41
9.8.	Planning and reporting .....	41
9.8.1.	EMA Business Pipeline activity and Horizon scanning .....	41

9.8.2.	Marketing Authorisation Applications (MAAs) 3-year forecast report (March 2023 to December 2025) .....	41
--------	--	----

<b>10.</b>	<b>Any other business</b>	<b>41</b>
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10.1.	COVID-19 Update .....	41
10.2.	Feedback on the discussion during the internal operations BOS on the External Data Safety Monitoring Board (eDSMBs).....	41
10.3.	Guideline on allergen products development in moderate to low-sized study populations.....	42
10.4.	Report on experience with RWE studies to support EMA scientific committees ....	42
10.5.	Request for feedback of requirements for development in children related to the revision of the orally inhaled products (OIP) guideline .....	42
10.6.	Workshop on ICH E6 (R3) in July 2023.....	42
10.7.	Upcoming Innovation Task Force (ITF) meetings .....	42

<b>11.</b>	<b>Breakout sessions</b>	<b>42</b>
------------	--------------------------	-----------

11.1.	Internal PDCO Operations .....	42
11.2.	Paediatric oncology .....	42
11.3.	Neonatology .....	42
11.4.	HIV .....	42
11.5.	Vaccines .....	42

<b>12.</b>	<b>Explanatory notes</b>	<b>44</b>
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## 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 23-26 May 2023. See May 2023 PDCO minutes (to be published post June 2023 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 23-26 May 2023.

### 1.3. Adoption of the minutes

PDCO minutes for 24-26 April 2023.

## 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. Adapalene, micronised / benzoyl peroxide, hydrous / clindamycin - EMEA-003263-PIP01-22

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Treatment of acne vulgaris

Day 120 opinion

**Action:** For adoption

Dermatology

#### 2.1.2. Spesolimab - EMEA-002475-PIP03-22

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Treatment of Netherton syndrome

Day 120 opinion

**Action:** For adoption

Dermatology

### 2.1.3. EMEA-003019-PIP01-21

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Treatment of inborn errors of amino acid metabolism

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.1.4. Izokibep - EMEA-003325-PIP01-22

---

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

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.5. Lenacapavir / bictegravir - EMEA-003324-PIP01-22

---

Treatment of human immunodeficiency virus (HIV-1) infection

Day 120 opinion

**Action:** For adoption

Infectious Diseases

### 2.1.6. Posoleucel - Orphan - EMEA-002908-PIP02-22

---

AlloVir International DAC; Prevention of viral disease in haematopoietic stem cell transplantation (HCT)

Day 120 opinion

**Action:** For adoption

Infectious Diseases

### 2.1.7. 1-(4-(6-chloropyridazin-3-yl)piperazin-1-yl)-2-(4-cyclopropyl-3-fluorophenyl)ethan-1-one - Orphan - EMEA-003268-PIP01-22

---

Bridge Bio Europe B.V.; Treatment of pantothenate kinase-associated neurodegeneration

Day 120 opinion

**Action:** For adoption

Other

### 2.1.8. Immunoglobulin G4 [228-proline], anti-(*Betula alleghaniensis* allergen Bet v 1) (human monoclonal $\gamma$ 4-chain), disulphide with human monoclonal $\kappa$ -chain, dimer /

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immunoglobulin G4 [227-proline], anti-*(Betula alleghaniensis* allergen Bet v 1) (human monoclonal  $\gamma$ 4-chain), disulphide with human monoclonal  $\kappa$ -chain, dimer / immunoglobulin G4 [224-proline], anti-*(Betula alleghaniensis* allergen Bet v 1) (human monoclonal  $\gamma$ 4-chain), disulphide with human monoclonal  $\kappa$ -chain, dimer - EMEA-003270-PIP01-22

---

Treatment of allergic rhinitis with or without conjunctivitis in birch tree pollen allergic patients

Day 120 opinion

**Action:** For adoption

Pneumology - Allergology

#### 2.1.9. EMEA-003276-PIP01-22

---

Treatment of post-traumatic stress disorder

Day 120 opinion

**Action:** For adoption

Psychiatry

#### 2.1.10. Phuket modRNA / Darwin modRNA / Austria modRNA / Wisconsin modRNA - EMEA-003318-PIP01-22

---

Prevention of influenza disease

Day 120 opinion

**Action:** For adoption

Vaccines

#### 2.1.11. Zapomeran - EMEA-003349-PIP01-22

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 opinion

**Action:** For adoption

Vaccines

#### 2.1.12. Acetylsalicylic acid / rosuvastatin - EMEA-003410-PIP01-23

---

Prevention of cardiovascular events

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.13. Rivaroxaban / amlodipine - EMEA-003412-PIP01-23

---

Treatment of hypertension / Prevention of thromboembolic events

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.14. Deucravacitinib - EMEA-002350-PIP05-23

---

Treatment of Sjögren's syndrome

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.1.15. Efgofitmod - Orphan - EMEA-003352-PIP02-23

---

FGK Representative Service GmbH; Treatment of systemic sclerosis

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.1.16. Secukinumab - EMEA-000380-PIP11-23

---

Treatment of rotator cuff tendinopathy

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.1.17. Reparixin - Orphan - EMEA-001693-PIP05-23

---

Dompé farmaceutici S.p.A.; Treatment of acute respiratory distress syndrome (ARDS)

Day 60 opinion

**Action:** For adoption

Infectious Diseases

*Note: Withdrawal request received on 11 May 2023*

#### 2.1.18. Opicapone - EMEA-003406-PIP01-23

---

Treatment of Parkinson's disease

---

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.1.19. EMEA-003409-PIP01-23

---

Treatment of solid malignancies

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.20. Therapeutic DNA plasmid vaccine targeting HPV16 E6 and E7 proteins - EMEA-003403-PIP01-23

---

Treatment of head and neck cancer / Treatment of cervical cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.21. Fuzuloparib - EMEA-003422-PIP01-23

---

Treatment of prostate cancer

Day 30 opinion

**Action:** For adoption

Oncology

#### 2.1.22. Nizaracianine triflutate - EMEA-003367-PIP01-22

---

Visualisation of ureter

Day 90 opinion

**Action:** For adoption

Diagnostic

## 2.2. Opinions on Compliance Check

#### 2.2.1. Lenvatinib - EMEA-C-001119-PIP02-12-M08

---

Eisai GmbH; Treatment of osteosarcoma

Day 60 opinion

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**Action:** For adoption

Oncology

### 2.2.2. Crisantaspase - EMEA-C-002934-PIP01-20

---

Jazz Pharmaceuticals Ireland Limited; Treatment of lymphoblastic lymphoma

Day 30 opinion

**Action:** For adoption

Oncology

### 2.2.3. Baloxavir marboxil - EMEA-C3-002440-PIP01-18-M04

---

Roche Registration GmbH; Treatment of influenza

Day 60 letter

**Action:** For adoption

Infectious Diseases

## 2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

### 2.3.1. Azilsartan medoxomil - EMEA-000237-PIP01-08-M11

---

Takeda Development Centre Europe Ltd; Treatment of hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

### 2.3.2. Macitentan - Orphan - EMEA-001032-PIP01-10-M06

---

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension / Treatment of idiopathic pulmonary fibrosis / Treatment of systemic sclerosis

Day 60 opinion

**Action:** For adoption. Oral explanation to be held on Wednesday, 24 May 2023 at 11:00

Cardiovascular Diseases

### 2.3.3. Mavacamten - EMEA-002231-PIP01-17-M01

---

Bristol-Myers Squibb Pharma EEIG; Treatment of hypertrophic cardiomyopathy

Day 60 opinion

**Action:** For adoption

#### 2.3.4. [Nemolizumab - EMEA-001624-PIP01-14-M06](#)

---

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

Day 60 opinion

**Action:** For adoption

Dermatology

#### 2.3.5. [Rocatinlimab - EMEA-002886-PIP01-20-M03](#)

---

Amgen Europe B.V; Treatment of atopic dermatitis

Day 60 opinion

**Action:** For adoption

Dermatology

#### 2.3.6. [Recombinant parathyroid hormone: rhPTH \(1-84\) - Orphan - EMEA-001526-PIP01-13-M06](#)

---

Takeda Pharmaceuticals International AG Ireland Branch; Hypoparathyroidism

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

#### 2.3.7. [Potassium chloride / sodium chloride / ascorbic acid / sodium sulfate / sodium ascorbate / polyethylene glycol 3350 - EMEA-001705-PIP02-15-M04](#)

---

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

*Note: Withdrawal request received on 16 May 2023*

#### 2.3.8. [Efgartigimod alfa - Orphan - EMEA-002597-PIP04-21-M01](#)

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argenx BV; Treatment of immune thrombocytopenia

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.9. Ixekizumab - EMEA-001050-PIP02-18-M02

---

Eli Lilly and Company (Ireland) Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondyloarthritis and juvenile idiopathic arthritis)

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.10. Tenofovir alafenamide / emtricitabine / bictegravir - EMEA-001766-PIP01-15-M05

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.11. Soticlestat - Orphan - EMEA-002572-PIP02-19-M03

---

Takeda Pharma A/S; Dravet syndrome / Lennox-Gastaut syndrome

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.12. (4S,7aR,9aR,10S,11E,14S,15R)-6'-chloro-10-methoxy-14,15-dimethyl-3',4',7a,8,9,9a,10,13,14,15-decahydro-2'H,7H-spiro[1,19-ethenocyclobuta[i][1,4]oxazepino[3,4f][1,2,7]thiadiazacyclohexadecine-4,1'-naphthalen]-18(17H)-one 16,16-dioxide (AMG 176) - EMEA-002631-PIP01-19-M02

---

Amgen Europe BV; Treatment of acute myeloid leukaemia

Day 60 opinion

**Action:** For adoption

Oncology

### 2.3.13. Magrolimab - Orphan - EMEA-002819-PIP01-20-M01

---

Gilead Sciences International Ltd; Treatment of myelodysplastic syndromes (including juvenile myelomonocytic leukaemia) / Treatment of acute myeloid leukaemia

Day 60 opinion

**Action:** For adoption

Oncology

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#### 2.3.14. Talimogene laherparepvec - EMEA-001251-PIP01-11-M06

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Amgen Europe B.V.; Treatment of melanoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.15. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M05

---

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

Day 60 opinion

**Action:** For adoption

Oncology / Haematology-Hemostaseology

#### 2.3.16. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M04

---

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 60 opinion

**Action:** For adoption

Other / Pneumology - Allergology

#### 2.3.17. Pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed) - EMEA-002330-PIP01-18-M03

---

Pfizer Europe MA EEIG; Prevention of disease caused by *Streptococcus pneumoniae*

Day 60 opinion

**Action:** For adoption

Vaccines

#### 2.3.18. Modified vaccinia Ankara - Bavarian Nordic virus (smallpox) - EMEA-001161-PIP02-11-M03

---

Bavarian Nordic A/S; Prevention of smallpox, mpox and related orthopoxvirus infection

Day 60 opinion

**Action:** For adoption

Vaccines

#### 2.3.19. NVX-CoV2373 - EMEA-002941-PIP01-20-M03

---

Novavax CZ, a.s.; Prevention of coronavirus disease 2019 (COVID-19)

---

Day 60 opinion

**Action:** For adoption

Vaccines

#### 2.3.20. Recombinant COVID-19 subunit nanoparticle (adjuvanted with AS03) (GBP510) - EMEA-003115-PIP01-21-M01

---

SK Chemicals GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 opinion

**Action:** For adoption

Vaccines

## 2.4. Opinions on Re-examinations

#### 2.4.1. Adtralza - EMEA-001900-PIP02-17-M07

---

LEO Pharma A/S; Treatment of atopic dermatitis

Final opinion

**Action:** For adoption

Dermatology

#### 2.4.2. Posaconazole - EMEA-000468-PIP02-12-M08

---

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

Final opinion

**Action:** For adoption

Infectious Diseases

## 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

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been informed in writing.

#### 2.7.1. Selexipag - EMEA-C2-000997-PIP01-10-M06

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 letter

**Action:** For information

Other

#### 2.7.2. Evinacumab - EMEA-C2-002298-PIP01-17-M05

Ultragenyx Germany GmbH; Treatment of elevated cholesterol

Day 30 letter

**Action:** For information

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

#### 2.7.3. (1R,3S,5R)-2-(2-(3-acetyl-5-(2-methylpyrimidin-5-yl)-1H-indazol-1-yl)acetyl)-N-(6-bromo-3-methylpyridin-2-yl)-5-methyl-2-azabicyclo[3.1.0]hexane-3-carboxamide (ALXN2050, vemircopan) - EMEA-C1-002863-PIP01-20

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 letter

**Action:** For information

Haematology-Hemostaseology

#### 2.7.4. Etranacogene dezaparovec - EMEA-C1-002722-PIP01-19-M02

CSL Behring GmbH; Treatment of haemophilia B

Day 30 letter

**Action:** For information

Haematology-Hemostaseology

#### 2.7.5. Delgocitinib - EMEA-C1-002329-PIP02-20-M02

LEO Pharma A/S; Treatment of chronic hand eczema

Day 30 letter

**Action:** For information

Dermatology

### 2.7.6. [Eliglustat - EMEA-C2-000461-PIP02-11-M05](#)

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Genzyme Europe B.V.; Treatment of Gaucher disease type 1 and type 3

Day 30 letter

**Action:** For information

Other

## 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. [Furosemide - EMEA-003316-PIP01-22](#)

---

Treatment of fluid retention

Day 90 discussion

**Action:** For discussion

Cardiovascular Diseases

#### 3.1.2. [Milvexian - EMEA-003220-PIP01-22](#)

---

Prevention of thromboembolism

Day 90 discussion

**Action:** For discussion

Cardiovascular Diseases

#### 3.1.3. [EMEA-003301-PIP01-22](#)

---

Treatment of psoriasis

Day 90 discussion

**Action:** For discussion

Dermatology

#### 3.1.4. [Povorcitinib - EMEA-003313-PIP01-22](#)

---

Treatment of hidradenitis suppurativa

Day 90 discussion

**Action:** For discussion

Dermatology

### 3.1.5. Ritlecitinib - EMEA-002451-PIP03-22

---

Treatment of vitiligo

Day 90 discussion

**Action:** For discussion

Dermatology

### 3.1.6. Encaleret - Orphan - EMEA-003348-PIP01-22

---

Calcilytix Therapeutics, Inc a BridgeBio Company; Treatment of hypoparathyroidism

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.7. Dirloctocogene samoparvovec - Orphan - EMEA-003290-PIP01-22

---

Spark Therapeutics Ireland Limited; Treatment of haemophilia A

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.8. Mocravimod - Orphan - EMEA-003304-PIP01-22

---

Priothera SAS; Treatment in haematopoietic stem cell transplantation (HSCT)

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.9. Recombinant human arylsulfatase A - Orphan - EMEA-002050-PIP02-22

---

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of metachromatic leukodystrophy

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.10. EMEA-003271-PIP01-22

---

Treatment of focal onset seizures

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.11. EMEA-003274-PIP01-22

---

Treatment of melanoma

Day 90 discussion

**Action:** For discussion

Oncology

### 3.1.12. Dersimelagon - EMEA-002850-PIP03-22

---

Treatment of systemic sclerosis

Day 90 discussion

**Action:** For discussion

Other

### 3.1.13. mRNA encoding modified human ornithine transcarbamylase - Orphan - EMEA-003315-PIP01-22

---

Arcturus Therapeutics Europe B.V.; Treatment of ornithine transcarbamylase deficiency

Day 90 discussion

**Action:** For discussion

Other

### 3.1.14. Pamrevlumab - EMEA-002979-PIP04-22

---

Treatment of interstitial lung diseases with fibrosis

Day 90 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.15. Repagermanium - Orphan - EMEA-003154-PIP01-21

---

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

Day 90 discussion

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**Action:** For discussion

Uro-nephrology

### 3.1.16. Influenza recombinant H7 haemagglutinin - EMEA-003314-PIP01-22

---

Prevention of influenza infection

Day 90 discussion

**Action:** For discussion

Vaccines

### 3.1.17. Single-stranded 5' capped mRNA encoding the HAs of the influenza virus - EMEA-003346-PIP01-22

---

Prevention of influenza disease

Day 90 discussion

**Action:** For discussion

Vaccines

### 3.1.18. MVA-BN-RSV - EMEA-003185-PIP01-22

---

Prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV)

Day 90 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.19. Influenza virus type B, whole virion, inactivated / influenza virus type A H3N2, whole virion, inactivated / influenza virus type A H1N1, whole virion, inactivated - EMEA-003267-PIP02-23

---

Prevention of influenza disease

Day 60 discussion

**Action:** For discussion

### 3.1.20. Semaglutide - EMEA-003402-PIP01-23

---

Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.21. [Tetrahydrouridine / decitabine - Orphan - EMEA-003404-PIP01-23](#)

---

Novo Nordisk A/S; Treatment of sickle cell disease (SCD)

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.22. [EMEA-003350-PIP01-23](#)

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.23. [Belimumab - EMEA-000520-PIP03-23](#)

---

Treatment of systemic sclerosis

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.24. [Daxdilimab - EMEA-003411-PIP01-23](#)

---

Treatment of systemic lupus erythematosus

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.25. [Nipocalimab - Orphan - EMEA-002559-PIP08-23](#)

---

Janssen-Cilag International NV; Treatment of idiopathic inflammatory myopathies

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.26. [Niclosamide ethanolamine - EMEA-003407-PIP01-23](#)

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion



**Action:** For discussion

Infectious Diseases

### 3.1.27. Povidone-iodine - EMEA-003413-PIP01-23

---

Treatment of common cold

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.28. Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP01-23

---

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy type 2E/R4

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.29. Vemircopan - Orphan - EMEA-002863-PIP02-23

---

Alexion Europe SAS; Treatment of generalised myasthenia gravis

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.30. mRNA encoding CMV gB / mRNA encoding the gH protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL131A protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL130 protein in the CMV glycoprotein complex pentamer / mRNA encoding the gL protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL128 protein in the CMV glycoprotein complex pentamer - EMEA-003405-PIP01-23

---

Prevention of cytomegalovirus infection

Day 60 discussion

**Action:** For discussion

Vaccines

### 3.1.31. Remibrutinib - EMEA-002582-PIP03-23

---

Treatment of chronic inducible urticaria

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.1.32. [Spesolimab - EMEA-002475-PIP04-23](#)

---

Treatment of hidradenitis suppurativa

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.1.33. [EMEA-003414-PIP01-23](#)

---

Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.34. [EMEA-003414-PIP02-23](#)

---

Treatment of obesity

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.35. [Frexalimab - EMEA-002945-PIP03-23](#)

---

Treatment of type 1 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.36. [Mavodelpar - Orphan - EMEA-003331-PIP02-23](#)

---

Reneo Pharmaceuticals Inc; Treatment of primary mitochondrial disorders

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.37. [Modified messenger ribonucleic acid encoding human propionyl-coenzyme a carboxylase alpha and beta subunits encapsulated into lipid nanoparticles - Orphan - EMEA-003419-PIP01-23](#)

---

Moderna Biotech Spain, S.L.; Treatment of propionic acidaemia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.38. [Semaglutide - EMEA-001441-PIP08-23](#)

---

Reduction in major adverse cardiac events / cardiovascular risk reduction in overweight and obese population

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.39. [Semaglutide / cagrilintide - EMEA-003059-PIP02-23](#)

---

Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.40. [Sepsiapterin - Orphan - EMEA-003027-PIP02-23](#)

---

PTC Therapeutics International; Treatment of hyperphenylalaninaemia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.41. [Cobitolimod - Orphan - EMEA-001589-PIP02-23](#)

---

InDex Pharmaceuticals AB; Treatment of ulcerative colitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

3.1.42. Autologous CD34+ cells transduced with a lentiviral vector containing a codon-optimised RPS19 gene - Orphan - EMEA-003074-PIP02-23

---

Apriligen LLC; Treatment of Diamond-Blackfan anaemia

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

3.1.43. Rilzabrutinib - Orphan - EMEA-002438-PIP04-23

---

Sanofi B.V.; Treatment of warm autoimmune haemolytic anaemia

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

3.1.44. Belumosudil - Orphan - EMEA-003425-PIP01-23

---

Sanofi Winthrop Industrie; Treatment of transplant complications

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

3.1.45. Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP03-23

---

Treatment of lower respiratory tract and lung infections

Day 30 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Pneumology - Allergology

3.1.46. Sotuletinib - Orphan - EMEA-003415-PIP01-23

---

Novartis Europharm Limited; Treatment of amyotrophic lateral sclerosis (ALS)

Day 30 discussion

**Action:** For discussion

Neurology

3.1.47. Abemaciclib - EMEA-002342-PIP04-23

---

Treatment of neuroblastoma

Day 30 discussion

---

**Action:** For discussion

Oncology

### 3.1.48. [Atezolizumab / tiragolumab - EMEA-003418-PIP01-23](#)

---

Treatment of lung carcinoma (non-small cell carcinoma)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.49. [Autologous dendritic cells pulsed with allogeneic tumour cell lysate - Orphan - EMEA-002381-PIP02-23](#)

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Amphera BV; Treatment of pancreatic cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.50. [Blinatumomab - Orphan - EMEA-000574-PIP03-23](#)

---

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.51. [Domvanalimab - EMEA-003429-PIP01-23](#)

---

Treatment of lung cancer (small-cell and non-small-cell cancers)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.52. [Domvanalimab - EMEA-003429-PIP02-23](#)

---

Treatment of gastric, gastroesophageal junction and oesophageal adenocarcinoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.53. Irinotecan - Orphan - EMEA-003416-PIP01-23

---

Les Laboratoires Servier; Treatment of pancreatic cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.54. Olutasidenib - Orphan - EMEA-003421-PIP01-23

---

Rigel Pharmaceuticals B.V.; Treatment of acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.55. Tazemetostat - Orphan - EMEA-003055-PIP02-23

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Ipsen Pharma; Treatment of follicular lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.56. Volrustomig - EMEA-003423-PIP01-23

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Treatment of lung cancer (small cell and non-small cell lung cancer) / Treatment of renal cell cancer / Treatment of cervical cancer / Treatment of malignant pleural mesothelioma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.57. Zimberelimab - EMEA-003427-PIP01-23

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Treatment of lung cancer (small-cell and non-small-cell cancers)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.58. Zimberelimab - EMEA-003427-PIP02-23

---

Treatment of gastric, gastroesophageal junction and oesophageal adenocarcinoma

---

Day 30 discussion

**Action:** For discussion

Oncology

---

### 3.1.59. [Cedazuridine / decitabine - Orphan - EMEA-003071-PIP02-23](#)

---

Otsuka Pharmaceutical Netherlands B.V.; Treatment of myelodysplastic syndromes

Day 30 discussion

**Action:** For discussion

Oncology / Haematology-Hemostaseology

---

### 3.1.60. [Satralizumab - Orphan - EMEA-001625-PIP05-23](#)

---

Roche Registration GmbH; Treatment of thyroid eye disease (TED)

Day 30 discussion

**Action:** For discussion

Ophthalmology / Neurology

---

### 3.1.61. [Iptacopan - EMEA-002705-PIP05-23](#)

---

Treatment of immune-complex mediated membranoproliferative glomerulonephritis (IC-MPGN)

Day 30 discussion

**Action:** For discussion

Other

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### 3.1.62. [Autologous cell product composed of CD34+ enriched haematopoietic stem cells \(HSCs\) that have been genetically modified to express the functional \$\beta\$ -glucocerebrosidase \(GCase\) protein - Orphan - EMEA-003424-PIP01-23](#)

---

AVROBIO, Inc.; Treatment of Gaucher disease type 1 / Treatment of Gaucher disease type 2 / Treatment of Gaucher disease type 3

Day 30 discussion

**Action:** For discussion

Other / Pain / Endocrinology-Gynaecology-Fertility-Metabolism / Pneumology - Allergology / Gastroenterology-Hepatology / Haematology-Hemostaseology / Neurology

---

### 3.1.63. [EMEA-003417-PIP01-23](#)

---

Treatment of patients in need of peritoneal dialysis

---

Day 30 discussion

**Action:** For discussion

Uro-nephrology

### 3.1.64. mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 - EMEA-003426-PIP01-23

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

## 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. Clascoterone - EMEA-C-003330-PIP01-22

---

Cassiopea S.p.A; Treatment of acne vulgaris

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.2.2. Marstacimab - EMEA-C1-002285-PIP02-19-M02

---

Pfizer Europe MA EEIG; Treatment of congenital haemophilia A

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.2.3. Rilpivirine (hydrochloride) - EMEA-C-000317-PIP01-08-M13

---

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases



### 3.2.4. Eladocagene exuparvovec - EMEA-C-002435-PIP01-18-M03

---

PTC Therapeutics International Limited; Treatment of aromatic L-amino acid decarboxylase deficiency

Day 30 discussion

**Action:** For discussion

Neurology

### 3.2.5. Teriflunomide - EMEA-C-001094-PIP01-10-M04

---

Sanofi Aventis Groupe; Treatment of multiple sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.2.6. Imetelstat - EMEA-C1-001910-PIP03-20-M01

---

Geron Corporation; Treatment of myelodysplastic syndromes (MDS), including juvenile myelomonocytic leukaemia (JMML)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.2.7. Peanut (*Arachis hypogaea*) allergen powder (previously known as peanut flour) - EMEA-C-001734-PIP01-14-M06

---

Aimmune Therapeutics Ireland Ltd; Treatment of peanut allergy

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.2.8. Influenza virus surface antigens - A/turkey/Turkey/1/05 (H5N1) - EMEA-C-000599-PIP01-09-M07

---

Seqirus S.r.l.; Prevention of influenza

Day 30 discussion

**Action:** For discussion

Vaccines

### 3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

#### 3.3.1. Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22-M01

---

Krystal Biotech, Inc.; Treatment of dystrophic epidermolysis bullosa

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.2. Birch bark extract - Orphan - EMEA-001299-PIP03-17-M02

---

Amryt Pharmaceuticals DAC; Treatment of epidermolysis bullosa

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.3. Dupilumab - EMEA-001501-PIP07-20-M01

---

Sanofi Winthrop Industrie; Treatment of chronic spontaneous urticaria

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.4. Inclisiran - EMEA-002214-PIP01-17-M02

---

Novartis Europharm Ltd.; Treatment of elevated cholesterol

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.3.5. Obeticholic acid - Orphan - EMEA-001304-PIP02-13-M07

---

Advanz Pharma Limited; Treatment of primary biliary cholangitis (PBC)

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.6. PF-06865571 - EMEA-002773-PIP01-20-M01

---

Pfizer Europe MA EEIG; Treatment of non-alcoholic steatohepatitis (NASH)

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.7. Iron as ferric maltol - EMEA-001195-PIP01-11-M06

---

Norgine BV; Treatment of iron deficiency anaemia (IDA)

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.8. Ibrutinib - EMEA-001397-PIP04-17-M02

---

Janssen-Cilag International NV; Treatment of chronic graft-versus-host disease

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.9. Tofacitinib citrate - EMEA-000576-PIP01-09-M15

---

Pfizer Europe MA 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

### 3.3.10. Cobicistat / darunavir - EMEA-001280-PIP01-12-M06

---

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.11. Delandistrogene moxeparvovec - Orphan - EMEA-002677-PIP01-19-M03

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Roche Registration GmbH; Treatment of Duchenne muscular dystrophy

Day 30 discussion

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**Action:** For discussion

Neurology

### **3.3.12. Erenumab - EMEA-001664-PIP02-15-M06**

---

Novartis Europharm Limited; Prevention of migraine headaches

Day 30 discussion

**Action:** For discussion

Neurology

### **3.3.13. Givinostat - Orphan - EMEA-000551-PIP04-21-M02**

---

Italfarmaco S.p.A.; Treatment of Duchenne muscular dystrophy

Day 30 discussion

**Action:** For discussion

Neurology

### **3.3.14. Lacosamide - EMEA-000402-PIP03-17-M07**

---

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

Day 30 discussion

**Action:** For discussion

Neurology

### **3.3.15. Abemaciclib - EMEA-002342-PIP01-18-M03**

---

Eli Lilly and Company Limited; Treatment of Ewing's sarcoma

Day 30 discussion

**Action:** For discussion

Oncology

### **3.3.16. Abemaciclib - EMEA-002342-PIP02-18-M02**

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Eli Lilly and Company Limited; Treatment of glioma / Treatment of neuroblastoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.17. Avapritinib - Orphan - EMEA-002358-PIP02-18-M03

---

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

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.18. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M06

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Ipsen Pharma; Treatment of malignant solid tumours

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.19. Olaparib - EMEA-002269-PIP01-17-M02

---

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.20. Quizartinib - Orphan - EMEA-001821-PIP01-15-M07

---

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.21. Bupropion (hydrochloride) / naltrexone (hydrochloride) - EMEA-001373-PIP01-12-M05

---

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 30 discussion

**Action:** For discussion

Other

### 3.3.22. Tirzepatide - EMEA-002360-PIP02-22-M01

---

Eli Lilly and Company; Treatment of obesity

---

Day 30 discussion

**Action:** For discussion

Other

### 3.3.23. Ketamine / sufentanil - EMEA-001739-PIP02-16-M02

---

Cessatech A/S; Treatment of acute pain

Day 30 discussion

**Action:** For discussion

Pain

## 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 22 May 2023 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. Bepranemab - EMEA-02-2023

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UCB Pharma S.A.; All classes of medicinal products for treatment of Alzheimer's disease / Treatment of Alzheimer's disease

**Action:** For adoption

### 6.1.2. Ixoberogene soroparvovec - EMEA-03-2023

---

Adverum Biotechnologies, Inc.; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema /Treatment of neovascular (wet) age-related macular degeneration in adult patients

**Action:** For adoption

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

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

#### 7.1.1. Ziltivekimab - EMEA-002840-PIP01-20

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Novo Nordisk A/S; Prevention of cardiovascular events in patients with atherosclerosis

**Action:** For adoption

## 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

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**Action:** For information

### 9.1.2. Vote by Proxy

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**Action:** For information

### 9.1.3. Strategic Review and Learning Meeting (SRLM) - Uppsala, 7-8 June 2023

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Update on the Strategic Review & Learning meeting under the Swedish Presidency of the Council of the EU, to be held on 7-8 June 2023

PDCO member: Sara Vennberg

**Action:** For information

## 9.2. Coordination with EMA Scientific Committees or CMDh-v

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

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**Action:** For information

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

### 9.3.1. Non-clinical Working Party: D30 Products identified

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PDCO member: Karen van Malderen

**Action:** For information

### 9.3.2. Formulation Working Group

---

PDCO member: Brian Aylward

**Action:** For information

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

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Agenda - HCPWP plenary meeting - 27 June 2023

Agenda - PCWP and HCPWP joint meeting - 28 June 2023

Agenda - PCWP plenary meeting - 27 June 2023

Meeting Summary PCWP HCPWP meeting - 3 March 2023

**Action:** For information



## 9.4. Cooperation within the EU regulatory network

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

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No item

## 9.5. Cooperation with International Regulators

### 9.5.1. Paediatric Cluster Teleconference

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**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

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No item

### 9.8.2. Marketing Authorisation Applications (MAAs) 3-year forecast report (March 2023 to December 2025)

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**Action:** For information

## 10. Any other business

### 10.1. COVID-19 Update

**Action:** For information

### 10.2. Feedback on the discussion during the internal operations BOS on the External Data Safety Monitoring Board (eDSMBs)

PDCO member: Sabine Scherer

**Action:** For information

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### **10.3. Guideline on allergen products development in moderate to low-sized study populations**

**Action:** For information

### **10.4. Report on experience with RWE studies to support EMA scientific committees**

**Action:** For information

### **10.5. Request for feedback of requirements for development in children related to the revision of the orally inhaled products (OIP) guideline**

**Action:** For information

### **10.6. Workshop on ICH E6 (R3) in July 2023**

To inform the committee on the upcoming [ACT EU PA04 - Multi-stakeholder Workshop on ICH E6 \(R3\) - Public Consultation](#) which will take place on 13-14 July 2023.

**Action:** For information

### **10.7. Upcoming Innovation Task Force (ITF) meetings**

**Action:** For information

## **11. Breakout sessions**

### **11.1. Internal PDCO Operations**

**Action:** For discussion on Tuesday, 11:00 - 12:00

### **11.2. Paediatric oncology**

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

### **11.3. Neonatology**

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

### **11.4. HIV**

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

### **11.5. 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/](http://www.ema.europa.eu/)