Paediatric Committee (PDCO)
Draft Agenda for the meeting on 25-28 June 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

25 June 2024, 14:00 - 19:00, room 2C
26 June 2024, 08:30 - 19:00, room 2C
27 June 2024, 08:30 - 19:00, room 2C
28 June 2024, 08:30 - 13:00, room 2C

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

Disclaimers
Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Note on access to documents
Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to 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).

1 Correction for 3.3.1. Fenebrutinib - EMEA-002349-PIP03-20-M01 and 3.3.2. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M10
# Table of contents

## 1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts .................. 9
1.2. Adoption of agenda ................................................................................................ 9
1.3. Adoption of the minutes ......................................................................................... 9

## 2. Opinions

### 2.1. Opinions on Products

2.1.1. Transglutaminase 2 inhibitor - EMEA-003513-PIP01-23 .......................................................... 9
2.1.2. Mezagitamab - EMEA-003502-PIP01-23 ................................................................................. 9
2.1.3. Ganaxolone - EMEA-002341-PIP03-23 ................................................................................... 9
2.1.4. Vosoritide - EMEA-002033-PIP02-23 ...................................................................................... 10
2.1.5. A/H1N1, A/H3N2, and B/Victoria and single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A/H1N1, A/H3N2, and B/Victoria and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein - EMEA003521-PIP01-23 ................................................................. 10
2.1.6. Tadalafil / ambrisentan - EMEA-003617-PIP01-24 .............................................................. 10
2.1.7. Tadalafil / ambrisentan - EMEA-003621-PIP01-24 .............................................................. 10
2.1.8. Tarumase - EMEA-003616-PIP01-24 ..................................................................................... 10
2.1.9. Copper (64Cu) oxodotreotide - Orphan - EMEA-003610-PIP01-24 ........................................ 11
2.1.10. Alvelestat - EMEA-003605-PIP01-24 .................................................................................. 11
2.1.11. Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - EMEA-003420-PIP02-24 ........................................................................ 11
2.1.12. Inobroda - EMEA-003622-PIP01-24 ..................................................................................... 11
2.1.13. Acasunlimab - EMEA-003606-PIP01-24 ............................................................................. 11
2.1.15. Ifinatamab deruxtecan - Orphan - EMEA-003613-PIP01-24 .............................................. 12
2.1.16. Ilperidone - EMEA-000995-PIP02-24 .................................................................................. 12
2.1.17. Brukinsa - EMEA-002354-PIP03-24 ................................................................................... 12

### 2.2. Opinions on Compliance Check

2.2.1. Recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (RIV3) - EMEA-C-003640-PIP01-24 ......................................................... 12
2.2.2. Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage - EMEA-C-003641-PIP01-24 ......................... 13
2.2.3. Estetrol monohydrate / drospirenone - EMEA-C-001332-PIP01-12-M06 .......................... 13
2.2.4. Liraglutide - EMEA-C-000128-PIP02-09-M05 ..................................................................... 13

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

2.3.1. Gadopiclenol - EMEA-001949-PIP01-16-M07 .................................................................... 13
2.3.2. Gadopiclenol - EMEA-001949-PIP02-18-M05 .................................................................... 13
2.3.3. Crinecerfont - Orphan - EMEA-002700-PIP01-19-M02 .......................................................... 14
2.3.4. Humanised monoclonal antibody Fab fragment - Orphan - EMEA-003253-PIP01-22-M01. 14
2.3.5. Navepegritide - Orphan - EMEA-002689-PIP02-23-M01 .................................................. 14
2.3.6. Teplizumab - EMEA-000524-PIP01-08-M03 ................................................................. 14
2.3.7. Survodutide - EMEA-002942-PIP02-20-M02 ................................................................. 14
2.3.8. Tezepelumab - EMEA-001613-PIP03-21-M01 ................................................................. 14
2.3.9. Vonoprazan - EMEA-002703-PIP01-19-M01 ................................................................. 15
2.3.10. Fitusiran - Orphan - EMEA-001855-PIP01-15-M06 ...................................................... 15
2.3.11. Osivelotor - EMEA-003241-PIP01-22-M01 ................................................................. 15
2.3.12. Narsoplimab - Orphan - EMEA-002479-PIP01-18-M02 .................................................. 15
2.3.13. Vadadustat - EMEA-001944-PIP01-16-M05 ................................................................. 15
2.3.14. Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19-M03 .................................................................................... 16
2.3.15. Human normal immunoglobulin - EMEA-001290-PIP01-12-M01 .................................... 16
2.3.16. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M07 ........................................ 16
2.3.17. Lenacapavir sodium - EMEA-002740-PIP01-19-M01 ..................................................... 16
2.3.18. Pretomanid - Orphan - EMEA-002115-PIP01-17-M06 ................................................... 16
2.3.19. Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M07 ................................................ 17
2.3.20. Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M05 17
2.3.21. Cenobamate - EMEA-002563-PIP02-19-M03 ............................................................... 17
2.3.22. Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19-M01 .................................................................................... 17
2.3.23. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M02............................................. 17
2.3.24. Sodium ((2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2-yl)oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) - Orphan - EMEA-003344-PIP01-22-M01 ....................................................................... 18
2.3.25. Midostaurin - Orphan - EMEA-000780-PIP01-09-M08 ...................................................... 18
2.3.26. Venetoclax - Orphan - EMEA-002018-PIP02-16-M06 ...................................................... 18
2.3.27. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M06 .................................... 18
2.3.28. Bupropion hydrochloride / naltrexone hydrochloride - EMEA-001373-PIP01-12-M06 ......... 18
2.3.29. Ketamine / sufentanil - EMEA-001739-PIP02-16-M03 ................................................... 19
2.3.30. Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M05 ................................................ 19
2.3.31. Benralizumab - EMEA-001214-PIP04-19-M01 .............................................................. 19
2.3.32. Donidalorsen - Orphan - EMEA-003112-PIP01-21-M01 ................................................. 19
2.3.33. COVID-19 vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M04 ............. 19
2.3.34. *Neisseria meningitidis* serogroup B protein-based active substance / recombinant *Neisseria meningitidis* serogroup B protein 3 / recombinant *Neisseria meningitidis* serogroup B protein 2 / recombinant *Neisseria meningitidis* serogroup B protein 1 / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* Group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-003379-PIP01-22-M0119
2.3.35. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M04

2.4. Opinions on Re-examinations

2.5. Opinions on Review of Granted Waivers

2.6. Finalisation and adoption of Opinions

2.7. Partial Compliance Checks completed by EMA

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

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

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

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

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

3. Discussion of applications

3.1. Discussions on Products D90-D60-D30

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

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

3.1.3. Efinopegdutide - EMEA-003549-PIP01-23

3.1.4. Hydroxy carbamide - EMEA-003388-PIP01-23

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

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

3.1.7. Iptacopan - EMEA-002705-PIP06-23

3.1.8. Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003619-PIP01-24

3.1.9. Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23

3.1.10. Retatrutide - EMEA-003258-PIP02-23

3.1.11. Rilonacept - Orphan - EMEA-003571-PIP01-23

3.1.12. Luliconazole - EMEA-003604-PIP01-24

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


3.1.15. Pegozafermin - EMEA-003619-PIP01-24

3.1.16. Telitacicept - EMEA-002824-PIP02-24

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

3.1.18. EMEA-003609-PIP01-24

3.1.19. EMEA-003306-PIP02-24

3.1.20. EMEA-003615-PIP01-24

3.1.21. Frexalimab - EMEA-002945-PIP04-24

3.1.22. Gedatolisib - EMEA-003612-PIP01-24

3.1.23. Letetresgene autoleucel - Orphan - EMEA-002476-PIP03-24

3.1.24. Rocatinlimab - EMEA-002886-PIP03-24 .......................................................... 26
3.1.25. Sargramostim - EMEA-003568-PIP02-24 .......................................................... 26
3.1.26. Adeno-associated virus serotype 9 vector containing the human LAMP2 isoform B transgene - Orphan - EMEA-003639-PIP01-24 .......................................................... 26
3.1.27. Zeralisiran - EMEA-003627-PIP01-24 .......................................................... 26
3.1.28. EMEA-003632-PIP01-24 .................................................................................. 26
3.1.29. EMEA-003633-PIP01-24 .................................................................................. 26
3.1.30. Rememulgene arelactibac - EMEA-003638-PIP01-24 ........................................... 27
3.1.31. Dasiglucagon - Orphan - EMEA-002233-PIP03-24 .............................................. 27
3.1.32. Etavopivat - Orphan - EMEA-002924-PIP03-24 .................................................. 27
3.1.33. Sonelokimab - EMEA-002568-PIP03-24 .......................................................... 27
3.1.34. Heterologous intestinal microbiota from healthy donors - EMEA-003635-PIP01-24 .... 27
3.1.35. EMEA-003602-PIP01-24 .................................................................................. 28
3.1.36. Adeno-associated viral vector serotype 9 containing the human MECP2 gene - Orphan - EMEA-003634-PIP01-24 .............................................................................. 28
3.1.37. Aldesleukin - Orphan - EMEA-001556-PIP02-24 ............................................... 28
3.1.39. EMEA-003631-PIP01-24 .................................................................................. 28
3.1.40. Anitocabtagene autoleucel - EMEA-003628-PIP01-24 ........................................ 29
3.1.41. Golcadomide - Orphan - EMEA-003161-PIP02-24 .............................................. 29
3.1.42. Tafasitamab - Orphan - EMEA-003161-PIP02-24 .............................................. 29
3.1.43. Esonadogene imvoparvovec - Orphan - EMEA-003626-PIP01-24 ...................... 29
3.1.44. Tacrolimus - EMEA-003625-PIP01-24 .............................................................. 29
3.1.45. Rupatadine / montelukast - EMEA-003629-PIP01-24 ........................................ 29
3.1.46. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24 .......................................................... 30
3.1.47. mRNA-based vaccine consisting of 3 mRNAs encoding the VP1 capsid protein from NoV genotypes, GII.4, GII.3 and GI.3 - EMEA-003637-PIP01-24 ........................................... 30

3.2. Discussions on Compliance Check ................................................................... 30
3.2.1. Sebetralstat - EMEA-C1-002723-PIP01-19-M02 ................................................. 30
3.2.2. Emtricitabine / tenofovir alafenamide - EMEA-C1-001577-PIP02-14-M05 ............ 30
3.2.3. Palbociclib - EMEA-C-002146-PIP01-17-M04 .................................................. 31

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan .......... 31
3.3.1. Fenebrutinib - EMEA-002349-PIP03-20-M01 ..................................................... 31
3.3.2. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M10 ...................................... 31
3.3.3. Spesolimab - EMEA-002475-PIP03-22-M01 ..................................................... 31
3.3.4. Macimorelin acetate - EMEA-001988-PIP01-16-M02 ........................................ 31
3.3.5. Apremilast - EMEA-000715-PIP02-11-M08 ..................................................... 31
3.3.6. Apremilast - EMEA-000715-PIP05-13-M06 ..................................................... 32
3.3.7. Deucravacitinib - EMEA-002350-PIP02-20-M01 ........................................................... 32
3.3.8. Human plasma derived C1-inhibitor (OCTA-C1-INH) - EMEA-002818-PIP01-20-M01 ....... 32
3.3.9. Cefiderocol - EMEA-002133-PIP01-17-M04 ................................................................. 32
3.3.10. Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M03 ........................................ 32
3.3.11. Sotrovimab - EMEA-002899-PIP01-20-M03 .............................................................. 33
3.3.12. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19-M03 ............................ 33
3.3.13. Lasmiditan - EMEA-002166-PIP01-17-M08 ................................................................. 33
3.3.14. Rimegepant - EMEA-002812-PIP02-20-M03 ............................................................. 33
3.3.15. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M06 ................................................ 33
3.3.16. Vamorolone - Orphan - EMEA-001794-PIP02-16-M08 .................................................. 34
3.3.17. Vesletiplirsen - EMEA-003305-PIP01-22-M01 ............................................................ 34
3.3.18. Avelumab - EMEA-001849-PIP02-15-M05 ................................................................. 34
3.3.19. Daratumumab - Orphan - EMEA-002152-PIP01-17-M04 ............................................. 34
3.3.20. Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M03 ............................ 34
3.3.21. Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21-M01 .......................... 34
3.3.22. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04 ........................................ 35
3.3.23. Iptacopan - Orphan - EMEA-002705-PIP03-20-M01 .................................................... 35
3.3.25. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M02 ................................................................. 35

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

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

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

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver 37
7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver ................................................................. 37
<table>
<thead>
<tr>
<th>Section</th>
<th>Page</th>
</tr>
</thead>
<tbody>
<tr>
<td>8. Annual reports on deferrals</td>
<td>37</td>
</tr>
<tr>
<td>9. Organisational, regulatory and methodological matters</td>
<td>37</td>
</tr>
<tr>
<td>9.1. Mandate and organisation of the PDCO</td>
<td>37</td>
</tr>
<tr>
<td>9.1.1. PDCO membership</td>
<td>37</td>
</tr>
<tr>
<td>9.1.2. Vote by Proxy</td>
<td>37</td>
</tr>
<tr>
<td>9.1.3. Strategic Review and Learning Meeting (SRLM)</td>
<td>37</td>
</tr>
<tr>
<td>9.2. Coordination with EMA Scientific Committees or CMDh-v</td>
<td>37</td>
</tr>
<tr>
<td>9.2.1. Committee for Medicinal Products for Human Use (CHMP)</td>
<td>37</td>
</tr>
<tr>
<td>9.2.2. Pharmacovigilance Risk Assessment Committee (PRAC)</td>
<td>38</td>
</tr>
<tr>
<td>9.2.3. Joint resolution on the principles of conduct within EMA Scientific Committees, WPs and CMDx</td>
<td>38</td>
</tr>
<tr>
<td>9.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups</td>
<td>38</td>
</tr>
<tr>
<td>9.3.1. Non-clinical Working Party: D30 Products identified</td>
<td>38</td>
</tr>
<tr>
<td>9.3.2. Paediatric Formulation Operational Expert Group (PFOEG)</td>
<td>38</td>
</tr>
<tr>
<td>9.3.4. Upcoming Innovation Task Force (ITF) meetings</td>
<td>38</td>
</tr>
<tr>
<td>9.3.5. Cardiovascular Working Party (CVS WP)</td>
<td>38</td>
</tr>
<tr>
<td>9.4. Cooperation within the EU regulatory network</td>
<td>38</td>
</tr>
<tr>
<td>9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA)</td>
<td>38</td>
</tr>
<tr>
<td>9.4.2. Feedback and status update from the PaedForm Project (EDQM)</td>
<td>39</td>
</tr>
<tr>
<td>9.5. Cooperation with International Regulators</td>
<td>39</td>
</tr>
<tr>
<td>9.5.1. Paediatric Cluster Teleconference</td>
<td>39</td>
</tr>
<tr>
<td>9.6. Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee</td>
<td>39</td>
</tr>
<tr>
<td>9.7. PDCO work plan</td>
<td>39</td>
</tr>
<tr>
<td>9.8. Planning and reporting</td>
<td>39</td>
</tr>
<tr>
<td>9.8.1. EMA Business Pipeline activity and Horizon scanning</td>
<td>39</td>
</tr>
<tr>
<td>10. Any other business</td>
<td>39</td>
</tr>
<tr>
<td>10.1. Health Technology Assessment body (HTAb) / EMA workshop on uncertainty management</td>
<td>39</td>
</tr>
<tr>
<td>10.2. Potential pitfalls from uncritical use of artificial intelligence (AI)</td>
<td>39</td>
</tr>
<tr>
<td>10.3. Practical aspects of IRIS for PDCO Members</td>
<td>39</td>
</tr>
<tr>
<td>10.4. Presenting efforts/work ongoing on a facilitation framework</td>
<td>40</td>
</tr>
<tr>
<td>10.5. Real World Evidence, including DARWIN EU®</td>
<td>40</td>
</tr>
<tr>
<td>11. Breakout sessions</td>
<td>40</td>
</tr>
<tr>
<td>11.1. Paediatric oncology</td>
<td>40</td>
</tr>
<tr>
<td>11.2. Neonatology</td>
<td>40</td>
</tr>
<tr>
<td>11.3. HIV</td>
<td>40</td>
</tr>
</tbody>
</table>
1. **Introductions**

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

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 25-28 June 2024. See June 2024 PDCO minutes (to be published post July PDCO meeting).

1.2. **Adoption of agenda**

PDCO agenda for 25-28 June 2024.

1.3. **Adoption of the minutes**

PDCO minutes for 28-31 May 2024.

2. **Opinions**

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

2.1. **Opinions on Products**

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

Treatment of coeliac disease

Day 120 opinion

**Action**: For adoption

Gastroenterology-Hepatology

2.1.2. **Mezagitamab - EMEA-003502-PIP01-23**

Treatment of immune thrombocytopenia

Day 120 opinion

**Action**: For adoption

Haematology-Hemostaseology

2.1.3. **Ganaxolone - EMEA-002341-PIP03-23**

Treatment of refractory status epilepticus

Day 120 opinion
2.1.4. **Vosoritide - EMEA-002033-PIP02-23**

Treatment of hypochondroplasia

Day 120 opinion

**Action**: For adoption

Other

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

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

Day 120 opinion

**Action**: For adoption

Vaccines

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

Treatment of pulmonary arterial hypertension

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

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

Treatment of pulmonary arterial hypertension

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

2.1.8. **Tarumase - EMEA-003616-PIP01-24**

Treatment of chronic wounds

Day 60 opinion

**Action**: For adoption

Dermatology
2.1.9. **Copper ($^{64}$Cu) oxodotreotide - Orphan - EMEA-003610-PIP01-24**

Curium Pet France; Diagnosis of neuroendocrine neoplasms

**Day 60 opinion**

**Action**: For adoption

Diagnostic

2.1.10. **Alvelestat - EMEA-003605-PIP01-24**

Treatment of alpha-1 antitrypsin deficiency

**Day 60 opinion**

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

Treatment of severe hypertriglyceridaemia (SHTG)

**Day 60 opinion**

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

*Note: Withdrawal request received on 18 June 2024*

2.1.12. **Inobrodib - EMEA-003622-PIP01-24**

Treatment of multiple myeloma

**Day 60 opinion**

**Action**: For adoption

Haematology-Hemostaseology

2.1.13. **Acasunlimab - EMEA-003606-PIP01-24**

Treatment of lung cancer

**Day 60 opinion**

**Action**: For adoption

Oncology

Treatment of head and neck epithelial malignant neoplasms
Day 60 opinion
**Action**: For adoption
Oncology

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

Daiichi Sankyo Europe GmbH; Treatment of lung cancer
Day 60 opinion
**Action**: For adoption
Oncology

2.1.16.  Iloperidone - EMEA-000995-PIP02-24

Treatment of bipolar disorder
Day 60 opinion
**Action**: For adoption
Psychiatry
*Note: Withdrawal request received on 19 June 2024*

2.1.17.  Brukinsa - EMEA-002354-PIP03-24

Treatment of lymphoplasmacyticymphoma / Treatment of mature B-cell neoplasms (excluding lymphocytic lymphoma)
Day 30 opinion
**Action**: For adoption
Oncology

2.2.  Opinions on Compliance Check

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

Sanofi Winthrop Industrie; Prevention of influenza disease
Day 30 opinion
**Action**: For adoption
Vaccines
2.2.2. **Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage** - EMEA-C-003641-PIP01-24

GlaxoSmithKline Biologicals S.A.; Prevention of influenza infection

**Action:** For adoption

**Vaccines**

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

Estetra SRL; Prevention of pregnancy

**Action:** For adoption

**Endocrinology-Gynaecology-Fertility-Metabolism**

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

Novo Nordisk A/S; Treatment of obesity

**Action:** For adoption

**Endocrinology-Gynaecology-Fertility-Metabolism**

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

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

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

**Action:** For adoption

**Diagnostic**

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

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

**Action:** For adoption

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

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia
Day 60 opinion
Action: For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

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

Sanofi B.V; Treatment of achondroplasia
Day 60 opinion
Action: For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

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

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia
Day 60 opinion
Action: For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

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

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus
Day 60 opinion
Action: For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

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

Boehringer Ingelheim International GmbH; Treatment of obesity
Day 60 opinion
Action: For adoption
Gastroenterology-Hepatology

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

AstraZeneca AB; Treatment of eosinophilic esophagitis
Day 60 opinion
Action: For adoption
Gastroenterology-Hepatology

2.3.9. Vonoprazan - EMEA-002703-PIP01-19-M01
Phathom Pharmaceuticals, Inc.; Treatment of gastroesophageal reflux disease (GORD) / Treatment of *Helicobacter pylori* infection
Day 60 opinion
Action: For adoption
Gastroenterology-Hepatology

2.3.10. Fitusiran - Orphan - EMEA-001855-PIP01-15-M06
Sanofi B.V.; Treatment of haemophilia B / Treatment of haemophilia A
Day 60 opinion
Action: For adoption
Haematology-Hemostaseology

2.3.11. Osivelotor - EMEA-003241-PIP01-22-M01
Pfizer Europe MA EEIG; Treatment of sickle cell disease
Day 60 opinion
Action: For adoption
Haematology-Hemostaseology

2.3.12. Narsoplimab - Orphan - EMEA-002479-PIP01-18-M02
Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation
Day 60 opinion
Action: For adoption
Haematology-Hemostaseology

2.3.13. Vadadustat - EMEA-001944-PIP01-16-M05
Medice Arzneimittel Pütter GmbH & Co KG; Treatment of anaemia due to chronic disorders
Day 60 opinion
Action: For adoption
Haematology-Hemostaseology
2.3.14. **Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19-M03**

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

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

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

LFB Biotechnologies; Treatment of primary immunodeficiency (PID)

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

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

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 60 opinion

**Action:** For adoption

Infectious Diseases

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

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

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

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases
2.3.19. **Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M07**

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus type-1 (HIV-1) infection
Day 60 opinion
**Action:** For adoption
Infectious Diseases

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

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (HIV-1) infection
Day 60 opinion
**Action:** For adoption
Infectious Diseases

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

Angelini Pharma S.p.A; Treatment of focal onset seizures (FOS) / Treatment of primary generalised tonic clonic seizures (PGTC)
Day 60 opinion
**Action:** For adoption
Neurology

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

Quince Therapeutics, S.p.A.; Treatment of ataxia telangiectasia
Day 60 opinion
**Action:** For adoption
Neurology

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

argenx BV; Treatment of generalised myasthenia gravis
Day 60 opinion
**Action:** For adoption
Neurology
2.3.24. Sodium ((2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2-yl]oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) - Orphan - EMEA-003344-PIP01-22-M01

Abbvie Limited; Treatment of vanishing white matter disease

Day 60 opinion

**Action**: For adoption

Neurology

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

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

Day 60 opinion

**Action**: For adoption

Oncology

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

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

Day 60 opinion

**Action**: For adoption

Oncology

*Note: Withdrawal request received on 14 June 2024*

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

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

Day 60 opinion

**Action**: For adoption

Oncology / Haematology-Hemostaseology

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

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 60 opinion

**Action**: For adoption

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

Cessatech A/S; Treatment of acute pain
Day 60 opinion
**Action**: For adoption

Pain

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

Heron Therapeutics B.V.; Treatment of acute postoperative pain
Day 60 opinion
**Action**: For adoption

Pain

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

AstraZeneca AB; Treatment of hypereosinophilic syndrome
Day 60 opinion
**Action**: For adoption

Pneumology - Allergology

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

Otsuka Pharmaceutical Netherlands B.V.; Treatment of hereditary angioedema
Day 60 opinion
**Action**: For adoption

Pneumology - Allergology

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

AstraZeneca AB; Prevention of coronavirus disease 2019 (COVID-19)
Day 60 opinion
**Action**: For adoption

Vaccines

2.3.34. *Neisseria meningitidis* serogroup B protein-based active substance / recombinant *Neisseria meningitidis* serogroup B protein 3 / recombinant *Neisseria meningitidis* serogroup B protein 2 / recombinant *Neisseria meningitidis* serogroup B protein 1 / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* Group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to
tetanus toxoid carrier protein / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-003379-PIP01-22-M01

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

**Day 60 opinion**

**Action:** For adoption

**Vaccines**

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

Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

**Day 30 opinion**

**Action:** For adoption

**Endocrinology-Gynaecology-Fertility-Metabolism**

### 2.4. Opinions on Re-examinations

No item

### 2.5. Opinions on Review of Granted Waivers

No item

### 2.6. Finalisation and adoption of Opinions

No item

### 2.7. Partial Compliance Checks completed by EMA

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

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

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

**Day 30 letter**

**Action:** For information

**Vaccines**

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

AstraZeneca AB; Treatment of neurofibromatosis type 1
Day 30 letter
**Action**: For information

Oncology

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

Ionis Ireland Limited; Treatment of familial chylomicronaemia syndrome

Day 30 letter
**Action**: For information

Cardiovascular Diseases

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

Bayer AG; Treatment of heart failure

Day 30 letter
**Action**: For information

Cardiovascular Diseases

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

Amphastar France Pharmaceuticals; Treatment of hypoglycaemia

Day 30 letter
**Action**: For information

Endocrinology-Gynaecology-Fertility-Metabolism

### 3. Discussion of applications

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

### 3.1. Discussions on Products D90-D60-D30

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

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 90 discussion
**Action**: For discussion

Cardiovascular Diseases
3.1.2. Pegtibatinase - Orphan - EMEA-003518-PIP01-23
Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria
Day 90 discussion
**Action**: For discussion
Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Efinopegdutide - EMEA-003549-PIP01-23
Treatment of non-alcoholic steatohepatitis
Day 90 discussion
**Action**: For discussion
Gastroenterology-Hepatology

3.1.4. Hydroxycarbamide - EMEA-003388-PIP01-23
Treatment of sickle cell disease
Day 90 discussion
**Action**: For discussion
Haematology-Hemostaseology

3.1.5. Recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody - EMEA-003510-PIP01-23
Prevention of hereditary angioedema attacks
Day 90 discussion
**Action**: For discussion
Haematology-Hemostaseology

3.1.6. Belumosudil - Orphan - EMEA-003425-PIP02-23
Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction
Day 90 discussion
**Action**: For discussion
Immunology-Rheumatology-Transplantation

3.1.7. Iptacopan - EMEA-002705-PIP06-23
Treatment of myasthenia gravis
Day 90 discussion
3.1.8. Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003524-PIP01-23

Sensorion SA; Treatment of otoferlin gene-mediated hearing loss
Day 90 discussion
Action: For discussion
Other

3.1.9. Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23

Glycomine Inc; Treatment of phosphomannomutase 2-congenital disorder of glycosylation
Day 90 discussion
Action: For discussion
Other

3.1.10. Retatrutide - EMEA-003258-PIP02-23

Treatment of obesity
Day 90 discussion
Action: For discussion
Other

3.1.11. Rilonacept - Orphan - EMEA-003571-PIP01-23

Kiniksa Pharmaceuticals (UK), Ltd.; Treatment of idiopathic pericarditis
Day 90 discussion
Action: For discussion
Other / Cardiovascular Diseases

3.1.12. Luliconazole - EMEA-003604-PIP01-24

Treatment of onychomycosis
Day 60 discussion
Action: For discussion
Dermatology
3.1.13. **2-Isopropyl-3H-naphtho[1,2-d]imidazole-4,5-dione - Orphan - EMEA-003618-PIP01-24**

Abliva AB; Treatment of primary mitochondrial disease

*Action*: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.14. **Diazoxide choline - Orphan - EMEA-003614-PIP01-24**

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

*Action*: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Psychiatry / Neurology

3.1.15. **Pegozafermin - EMEA-003619-PIP01-24**

Treatment of metabolic dysfunction-associated steatohepatitis (MASH)

*Action*: For discussion

Gastroenterology-Hepatology

3.1.16. **Telitacicept - EMEA-002824-PIP02-24**

Treatment of myasthenia gravis

*Action*: For discussion

Immunology-Rheumatology-Transplantation

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

Treatment of Wiskott-Aldrich syndrome

*Action*: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.18. **EMEA-003609-PIP01-24**

Treatment of respiratory tract disease caused by human respiratory syncytial virus
Day 60 discussion

**Action**: For discussion

**Infectious Diseases**

3.1.19. EMEA-003306-PIP02-24

Treatment of human respiratory syncytial virus (RSV)

Day 60 discussion

**Action**: For discussion

**Infectious Diseases**

3.1.20. EMEA-003615-PIP01-24

Treatment of Prader Willi syndrome

Day 60 discussion

**Action**: For discussion

**Neurology**

3.1.21. Frexalimab - EMEA-002945-PIP04-24

Treatment of multiple sclerosis

Day 60 discussion

**Action**: For discussion

**Neurology**

3.1.22. Gedatolisib - EMEA-003612-PIP01-24

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

Day 60 discussion

**Action**: For discussion

**Oncology**

3.1.23. Letetresgene autoleucel - Orphan - EMEA-002476-PIP03-24

Adaptimmune B. V.; Treatment of soft tissue sarcoma

Day 60 discussion

**Action**: For discussion

**Oncology**
3.1.24. **Rocatinlimab - EMEA-002886-PIP03-24**

Treatment of asthma

Day 60 discussion

**Action**: For discussion

Pneumology - Allergology

3.1.25. **Sargramostim - EMEA-003568-PIP02-24**

Treatment of autoimmune pulmonary alveolar proteinosis (aPAP)

Day 60 discussion

**Action**: For discussion

Pneumology - Allergology

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

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

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

3.1.27. **Zerlasiran - EMEA-003627-PIP01-24**

Atherosclerotic cardiovascular disease

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

3.1.28. **EMEA-003632-PIP01-24**

Treatment of symptomatic heart failure

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

3.1.29. **EMEA-003633-PIP01-24**

Treatment of psoriasis

Day 30 discussion
**Action**: For discussion

**Dermatology**

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

Treatment of diabetic foot ulcer / Treatment of venous leg ulcer

Day 30 discussion

**Action**: For discussion

**Dermatology**

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

Zealand Pharma A/S; Treatment of congenital hyperinsulinism

Day 30 discussion

**Action**: For discussion

**Endocrinology-Gynaecology-Fertility-Metabolism**

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

Novo Nordisk A/S; Treatment of thalassemia

Day 30 discussion

**Action**: For discussion

**Haematology-Hemostaseology**

**3.1.33. Sonelokimab - EMEA-002568-PIP03-24**

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

Day 30 discussion

**Action**: For discussion

**Immunology-Rheumatology-Transplantation**

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

Treatment of *Clostridioides difficile* infection (CDI)

Day 30 discussion

**Action**: For discussion

**Infectious Diseases**
<table>
<thead>
<tr>
<th>Code</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>3.1.35.</td>
<td><strong>EMEA-003602-PIP01-24</strong>&lt;br&gt;Treatment of tuberculosis&lt;br&gt;Day 30 discussion&lt;br&gt;<strong>Action</strong>: For discussion&lt;br&gt;Infectious Diseases</td>
</tr>
<tr>
<td>3.1.36.</td>
<td><strong>Adeno-associated viral vector serotype 9 containing the human MECP2 gene - Orphan - EMEA-003634-PIP01-24</strong>&lt;br&gt;Taysha Gene Therapies, Inc.; Treatment of Rett syndrome&lt;br&gt;Day 30 discussion&lt;br&gt;<strong>Action</strong>: For discussion&lt;br&gt;Neurology</td>
</tr>
<tr>
<td>3.1.37.</td>
<td><strong>Aldesleukin - Orphan - EMEA-001556-PIP02-24</strong>&lt;br&gt;ILTOO Pharma SAS; Treatment of amyotrophic lateral sclerosis (ALS)&lt;br&gt;Day 30 discussion&lt;br&gt;<strong>Action</strong>: For discussion&lt;br&gt;Neurology</td>
</tr>
<tr>
<td>3.1.38.</td>
<td><strong>H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O2)-NH2-DOTA-225-Actinium - Orphan - EMEA-003630-PIP01-24</strong>&lt;br&gt;NovaCurie GmbH; Treatment of low-grade glioma&lt;br&gt;Day 30 discussion&lt;br&gt;<strong>Action</strong>: For discussion&lt;br&gt;Neurology</td>
</tr>
<tr>
<td>3.1.39.</td>
<td><strong>EMEA-003631-PIP01-24</strong>&lt;br&gt;Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) whose treatment may include DNA-damaging agents&lt;br&gt;Day 30 discussion&lt;br&gt;<strong>Action</strong>: For discussion&lt;br&gt;Oncology</td>
</tr>
</tbody>
</table>
3.1.40. **Anitocabtagene autoleucel - EMEA-003628-PIP01-24**

Treatment of multiple myeloma

Day 30 discussion

**Action**: For discussion

**Oncology**

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

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

Day 30 discussion

**Action**: For discussion

**Oncology**

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

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

Day 30 discussion

**Action**: For discussion

**Oncology**

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

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

Day 30 discussion

**Action**: For discussion

**Ophthalmology**

3.1.44. **Tacrolimus - EMEA-003625-PIP01-24**

Treatment of vernal keratoconjunctivitis

Day 30 discussion

**Action**: For discussion

**Ophthalmology**

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

Treatment of allergic rhinitis

Day 30 discussion
Action: For discussion  
Oto-rhino-laryngology

3.1.46. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle  - EMEA-003636-PIP01-24

Prevention of lower respiratory tract disease caused by respiratory syncytial virus or human metapneumovirus  
Day 30 discussion  
Action: For discussion  
Vaccines

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

Prevention of norovirus acute gastroenteritis  
Day 30 discussion  
Action: For discussion  
Vaccines

3.2. Discussions on Compliance Check

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

3.2.1. Sebetralstat - EMEA-C1-002723-PIP01-19-M02

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema  
Day 30 discussion  
Action: For discussion  
Haematology-Hemostaseology

3.2.2. Emtricitabine / tenofovir alafenamide - EMEA-C1-001577-PIP02-14-M05

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection  
Day 30 discussion  
Action: For discussion  
Infectious Diseases
3.2.3. **Palbociclib - EMEA-C-002146-PIP01-17-M04**

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma

Day 30 discussion

**Action**: For discussion

**Oncology**

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

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

Roche Registration GmbH; Treatment of multiple sclerosis

Day 30 discussion

**Action**: For discussion

**Neurology**

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

GlaxoSmithKline (Ireland) Limited; Treatment of pulmonary arterial hypertension

Day 30 discussion

**Action**: For discussion

**Cardiovascular Diseases**

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

Boehringer Ingelheim International GmbH; Treatment of Netherton syndrome

Day 30 discussion

**Action**: For discussion

**Dermatology**

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

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

Day 30 discussion

**Action**: For discussion

**Diagnostic**

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

Amgen Europe B.V.; Treatment of juvenile idiopathic arthritis (JIA) / Treatment of juvenile
psoriatic arthritis (JPsA)
Day 30 discussion
**Action:** For discussion
Immunology-Rheumatology-Transplantation

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

Amgen Europe B.V; Treatment of Behçet's disease
Day 30 discussion
**Action:** For discussion
Immunology-Rheumatology-Transplantation

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

Bristol-Myers Squibb Pharma EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)
Day 30 discussion
**Action:** For discussion
Immunology-Rheumatology-Transplantation

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

Octapharma Pharmazeutika Produktionsges. m.b.H; Treatment of hereditary angioedema (HAE)
Day 30 discussion
**Action:** For discussion
Immunology-Rheumatology-Transplantation

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

Shionogi B.V.; Treatment of infections due to aerobic gram-negative bacteria
Day 30 discussion
**Action:** For discussion
Infectious Diseases

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

Mundipharma GmbH; Treatment of invasive candidiasis
Day 30 discussion
3.3.11. Sotrovimab - EMEA-002899-PIP01-20-M03

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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

Eli Lilly and Company Ltd; Treatment of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

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

Pfizer Europe MA EEIG; Treatment of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

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

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

Day 30 discussion

Action: For discussion

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

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

Day 30 discussion

**Action:** For discussion

Neurology

*Note: Withdrawal request received on 13 June 2024*

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

Sarepta Therapeutics Ireland; Treatment of Duchenne muscular dystrophy

Day 30 discussion

**Action:** For discussion

Neurology

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

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

Day 30 discussion

**Action:** For discussion

Oncology

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

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

Day 30 discussion

**Action:** For discussion

Oncology

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

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

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

Miltenyi Biomedicine GmbH; Treatment of mature B cell neoplasms
Day 30 discussion

**Action**: For discussion

**Oncology**

### 3.3.22. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04

Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

Day 30 discussion

**Action**: For discussion

**Other**

### 3.3.23. Iptacopan - Orphan - EMEA-002705-PIP03-20-M01

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

Day 30 discussion

**Action**: For discussion

**Other / Haematology-Hemostaseology**

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

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

Day 30 discussion

**Action**: For discussion

**Vaccines**

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

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

Day 30 discussion

**Action**: For discussion

**Vaccines**

### 3.3.26. Recombinant varicella zoster virus glycoprotein E - EMEA-001426-PIP01-13-M03

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

Day 30 discussion

**Action**: For discussion

**Vaccines**
4. Nominations

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

4.1. List of submissions of applications with start of procedure 08 July 2024 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

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

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

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

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

6. Discussion on the applicability of class waivers

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

6.1. Discussions on the applicability of class waiver for products

6.1.1. Depemokimab - EMEA-06-2024

GlaxoSmithKline Trading Services Limited; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft-versus-host disease (bone-marrow) transplantation.

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

Action: For adoption

6.1.2. Leuprorelin - EMEA-07-2024

Recordati Industria Chimica e Farmaceutica S.p.A.; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their
releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of prostate malignant neoplasms.

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

**Action:** For adoption

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

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

No item

### 8. Annual reports on deferrals

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

### 9. Organisational, regulatory and methodological matters

#### 9.1. Mandate and organisation of the PDCO

##### 9.1.1. PDCO membership

**Action:** For information

##### 9.1.2. Vote by Proxy

**Action:** For information

##### 9.1.3. Strategic Review and Learning Meeting (SRLM)

No item

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

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

**Action:** For information
9.2.2. Pharmacovigilance Risk Assessment Committee (PRAC)

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

**Action:** For information

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

**Action:** For information

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

**Action:** For information

9.3.2. Paediatric Formulation Operational Expert Group (PFOEG)

PDCO member: Brian Aylward (*ad interim*)

**Action:** For information


**Action:** For information

9.3.4. Upcoming Innovation Task Force (ITF) meetings

**Action:** For information

9.3.5. Cardiovascular Working Party (CVS WP)

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

**Action:** For information

9.4. Cooperation within the EU regulatory network

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

No item
9.4.2. **Feedback and status update from the PaedForm Project (EDQM)**

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

PDCO member: Siri Wang

**Action:** For information

9.5. **Cooperation with International Regulators**

9.5.1. **Paediatric Cluster Teleconference**

**Action:** For information

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

No item

9.7. **PDCO work plan**

No Item

9.8. **Planning and reporting**

9.8.1. **EMA Business Pipeline activity and Horizon scanning**

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

**Action:** For information

10. **Any other business**

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

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

**Action:** For information

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

**Action:** For information

10.3. **Practical aspects of IRIS for PDCO Members**

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

10.4. Presenting efforts/work ongoing on a facilitation framework

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

Action: For information

10.5. Real World Evidence, including DARWIN EU®

Action: For information

11. Breakout sessions

11.1. Paediatric oncology

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

11.2. Neonatology

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

11.3. HIV

Action: For discussion on Thursday, 13:00 - 14:00, room 2C
12. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

**Paediatric investigation plan (PIP)** *(section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs)*

A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, when it is safe to do so, to support the authorisation of a medicine for children. Pharmaceutical companies submit proposals for PIPs to the European Medicines Agency's Paediatric Committee (PDCO). This Committee is responsible for agreeing or refusing the plan.

**Compliance checks** *(section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check)*

A compliance check may be necessary before any application for marketing authorisation (even for an adult indication) can be considered valid, if there was no deferral for at least one of the studies agreed in the PIP, or after the due date of initiation or completion of a study/measure. The same applies to some regulatory applications for authorised products, as described above.

**Modification of an Agreed Paediatric Investigation Plan** *(section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP)*

The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate. In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

**Class waiver** *(section 6 Discussion on the applicability of class waiver)*

As some diseases do not affect children (for example Parkinson's disease), the development of medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived. For more information on the classes of diseases subject to waivers, see class waivers.

**Annual reports on deferrals** *(section 8)*

If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

For a list of acronyms and abbreviations, see:

*Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA’s regulatory activities*

More detailed information on the above terms can be found on the EMA website: [www.ema.europa.eu/](https://www.ema.europa.eu/)