13 October 2023
EMA/PDCO/424082/2023
Human Medicines Division

Paediatric Committee (PDCO)

Minutes for the meeting on 05-08 September 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

Health and safety information

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

Disclaimers

Some of the information contained in this set of minutes 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).

Of note, this set of minutes is a working document primarily designed for PDCO members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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Table of contents

1. Introductions 10

1.1. Welcome and declarations of interest of members, alternates and experts 10

1.2. Adoption of agenda 10

1.3. Adoption of the minutes 10

2. Opinions 10

2.1. Opinions on Products 11

2.1.1. A self-complementary adeno-associated virus [AAV] serotype 8 virus particle encoding the human ornithine transcarbamylase [OTC] gene sequence (DTX301) - Orphan - EMEA-002830-PIP01-20 11

2.1.2. A nonreplicating, recombinant adeno-associated virus (AAV) serotype 9 (AAV9) gene transfer vector that contains a modified human ATP7B coding sequence - Orphan - EMEA-003131-PIP01-21 11

2.1.3. Fazirsiran - Orphan - EMEA-003355-PIP01-22 11

2.1.4. Inhibitor of receptor-interacting protein kinase 1 (ABBV-668) - EMEA-003356-PIP01-22 .. 12

2.1.5. Tezepelumab - EMEA-001613-PIP03-21 12

2.1.6. Deucrictibant - EMEA-003090-PIP02-22 12

2.1.7. Ciraparantag - EMEA-003321-PIP01-22 13

2.1.8. Luspatercept - Orphan - EMEA-001521-PIP03-22 13

2.1.9. Enpatoran - EMEA-003342-PIP02-22 13

2.1.10. Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate (ECT-001-CB) - Orphan - EMEA-003025-PIP0-23 14

2.1.11. Ensitrelvir - EMEA-003192-PIP01-22 14

2.1.12. Ruzotolimod - EMEA-003363-PIP01-22 14

2.1.13. Xalnesiran - EMEA-003362-PIP01-22 15

2.1.14. Rozanolixizumab - EMEA-002681-PIP03-21 15

2.1.15. Nemvaleukin alfa - EMEA-003357-PIP01-22 15

2.1.16. Vibostolimab / pembrolizumab - EMEA-003063-PIP03-22 16

2.1.17. Uproleselan - Orphan - EMEA-003307-PIP01-22 16

2.1.18. Pabinafusp alfa - Orphan - EMEA-003033-PIP02-22 17

2.1.19. Derivative of 6-(piperidine-1-carbonyl)pyridin-3-ol (BI 764198) - EMEA-003347-PIP01-22 . 17

2.1.20. Inaxaplin - Orphan - EMEA-003368-PIP01-22 17

2.1.21. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / outer membrane vesicles (OMV) from *N. meningitidis* strain NZ 98/254 / recombinant *Neisseria meningitidis* group B fHbp 2-3-1.13NB fusion protein / recombinant *Neisseria meningitis* group B protein 961c / recombinant

Neisseria meningitis group B protein 287-953 / recombinant *Neisseria meningitis* group B protein 936-741 - EMEA-003359-PIP01-22 18

2.1.22. *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 18

2.1.23. Rosuvastatin / amlodipine - EMEA-003446-PIP01-23 19

2.1.24. Modified, recombinant version of the human myeloid-derived growth factor - EMEA-003449-PIP01-23 19

2.1.25. 2-[3-(dimethyl-1H-1,2,3-triazol-5-yl)-5-[(S)-oxan-4-yl(phenyl)methyl]-5H-pyrido[3,2-b]indol-7-yl]propan-2-ol - EMEA-003456-PIP01-23 20

2.1.26. 2-((R)-3-(1-(1-((R)-1-(2,4-dichlorophenyl)ethyl)-3-(trifluoromethyl)-1H-pyrazolo[3,4-b]pyrazin-6-yl)azetidin-3-yl)piperidin-1-yl)ethan-1-ol, benzenesulfonate - EMEA-003455-PIP01-23 20

2.1.27. Belrestotug - EMEA-003452-PIP01-23 20

2.1.28. Bezuclastinib - EMEA-003445-PIP01-23 21

2.1.29. Humanised IgG1 monoclonal antibody against TROP2, conjugated to a topoisomerase I inhibitor belotecan-derivative - EMEA-003461-PIP01-23 21

2.1.30. Tiragolumab - EMEA-002721-PIP04-23 22

2.1.31. Valemetostat tosilate - Orphan - EMEA-003256-PIP02-23 22

2.1.32. Zanidatamab - Orphan - EMEA-003450-PIP01-23 23

2.1.33. Genetically detoxified pertussis toxin (PTgen) / pertussis filamentous haemagglutinin (FHA) - EMEA-003442-PIP01-23 23

2.1.34. Actinium chloride (non-carrier added) - EMEA-003482-PIP01-23 23

2.1.35. Ulviprubart - EMEA-003474-PIP01-23 24

2.2. Opinions on Compliance Check 24

2.2.1. Abacavir (ABC) / lamivudine (3TC) / dolutegravir (DTG) - EMEA-C-001219-PIP01-11-M06 24

2.2.2. Leriglitazone - EMEA-C2-002106-PIP01-16-M02 25

2.2.3. Molgramostim - EMEA-C1-002282-PIP01-17-M01 25

2.2.4. Bulevirtide - EMEA-C-002399-PIP01-18-M01 25

2.2.5. Cysteamine hydrochloride - EMEA-C-000322-PIP01-08-M06 25

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan 26

2.3.1. Dupilumab - EMEA-001501-PIP02-13-M08 26

2.3.2. Fitusiran - Orphan - EMEA-001855-PIP01-15-M05 26

2.3.3. Vonicog alfa - EMEA-001164-PIP01-11-M07 26

2.3.4. BNT162b2 / tozinameran / famtozinameran / riltozinameran - EMEA-002861-PIP02-20-M0627

2.3.5. Eravacycline - EMEA-001555-PIP01-13-M05 27

2.3.6. Remdesivir - EMEA-002826-PIP01-20-M04 27

2.3.7. Eptinezumab - EMEA-002243-PIP01-17-M04 28

2.3.8. Nipocalimab (anti-neonatal Fc receptor human monoclonal antibody) - EMEA-002559-PIP02-19-M01 28
2.3.9. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M05 28
2.3.10. Peginterferon beta-1a - EMEA-001129-PIP01-11-M06 28
2.3.11. Carfilzomab - Orphan - EMEA-001806-PIP04-19-M02 29
2.3.12. Cemiplimab - EMEA-002007-PIP02-17-M03 29
2.3.13. Tovorafenib - Orphan - EMEA-002763-PIP01-20-M01 29
2.3.14. Iptacopan - Orphan - EMEA-002705-PIP02-19-M01 30
2.3.15. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M05 30
2.3.16. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M05 30
2.3.17. Selexipag - EMEA-000997-PIP01-10-M07 31
2.3.18. Evenamide - EMEA-002519-PIP03-21-M01 31
2.3.19. NVX-CoV2373 - EMEA-002941-PIP01-20-M04 31
2.3.20. Recombinant COVID-19 subunit nanoparticle - EMEA-003115-PIP01-21-M02 32
2.3.21. Autologous tumour-infiltrating lymphocytes (LN-144/LN-145) - EMEA-002776-PIP01-20-M02 32
2.3.22. Angiotensin II acetate - EMEA-001912-PIP02-16-M03 32
2.3.23. Brivaracetam - EMEA-000332-PIP02-17-M05 33
2.3.24. Risankizumab - EMEA-001776-PIP04-17-M01 33
2.4. Opinions on Re-examinations 33
2.5. Opinions on Review of Granted Waivers 33
2.6. Finalisation and adoption of Opinions 33
2.7. Partial Compliance Checks completed by EMA 33
2.7.1. Beremagene geperpavec - EMEA-C1-002472-PIP03-22-M01 34
2.7.2. Gepotidacin - EMEA-C1-002443-PIP01-18-M02 34
2.7.3. Repotrectinib - EMEA-C1-002635-PIP02-21-M01 34
2.7.4. Sarilumab - EMEA-C1-001045-PIP01-10-M03 34
2.7.5. Remibrutinib - EMEA-C1-002582-PIP01-19-M02 34

3. Discussion of applications 34

3.1. Discussions on Products D90-D60-D30 35
3.1.1. Amlitelimab - EMEA-003233-PIP01-22 35
3.1.2. Upadacitinib - EMEA-001741-PIP08-22 35
3.1.3. Upadacitinib - EMEA-001741-PIP09-23 35
3.1.4. *Escherichia coli*, expressing high affinity phenylalanine transporter, modified phenylalanine ammonia lyase and L-amino acid deaminase - EMEA-003381-PIP01-22 35
3.1.5. Recombinant human monoclonal antibody to insulin receptor - Orphan - EMEA-002813-PIP01-23 35
3.1.6. Venglustat - Orphan - EMEA-001716-PIP07-22 35
3.1.7. Cemdisiran - Orphan - EMEA-003237-PIP02-22 36

3.1.8. Pozelimab - EMEA-003238-PIP02-22 36
3.1.9. Tetrahydrouridine / decitabine - Orphan - EMEA-003404-PIP01-23 36
3.1.10. Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP01-23 36
3.1.11. Inebilizumab - EMEA-001911-PIP02-22 36
3.1.12. Cobolimab - EMEA-003273-PIP02-22 36
3.1.13. Iptacopan - EMEA-002705-PIP05-23 36
3.1.14. Oral inhibitor of PCSK9 - EMEA-003453-PIP01-23 37
3.1.15. Upadacitinib - EMEA-001741-PIP10-23 37
3.1.16. GIPR antagonist/GLP-1R agonist - EMEA-003439-PIP02-23 37
3.1.17. Plecanatide - EMEA-003441-PIP01-23 37
3.1.18. Hydroxycarbamide - EMEA-003388-PIP01-23 37
3.1.19. Ianalumab - EMEA-002338-PIP04-23 37
3.1.20. Reparixin - Orphan - EMEA-001693-PIP06-23 38
3.1.21. Ganaxolone - EMEA-002341-PIP03-23 38
3.1.22. Recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA - Orphan - EMEA-003459-PIP01-23 38
3.1.23. Autologous patient-derived CD4+ and CD8+ T cells expressing a chimeric antigen receptor specific for claudin 6 - EMEA-003377-PIP01-23 38
3.1.24. Disitamab vedotin - EMEA-003443-PIP02-23 38
3.1.25. Liposome-formulated messenger ribonucleic-acid vaccine encoding the chimeric antigen-receptor target antigen claudin 6 - EMEA-003464-PIP01-23 38
3.1.26. Obecabtagene autoleucel - Orphan - EMEA-003171-PIP02-23 39
3.1.27. Unesbulin - Orphan - EMEA-003297-PIP02-23 39
3.1.28. Allogeneic faecal microbiota, pooled - Orphan - EMEA-003435-PIP02-23 39
3.1.29. Clobetasol - EMEA-003458-PIP01-23 39
3.1.30. Laruparetigene zovaparvovec - Orphan - EMEA-003457-PIP01-23 39
3.1.31. Garetosmab - Orphan - EMEA-002736-PIP02-23 39
3.1.32. Losmapimod - Orphan - EMEA-003448-PIP01-23 39
3.1.33. Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23 40
3.1.34. Sisunatovir - EMEA-002529-PIP02-23 40
3.1.35. Imlifidase - EMEA-002183-PIP02-23 40
3.1.36. Mometasone - EMEA-003454-PIP01-23 40
3.1.37. EMEA-003451-PIP01-23 40
3.1.38. *Borrelia* outer surface protein A (OspA) serotypes (ST1-6) lipidated, fusion protein vaccine - EMEA-003130-PIP02-23 40
3.1.39. Candesartan / atorvastatin / amlodipine - EMEA-003466-PIP01-23 41
3.1.40. Ezetimibe / rosuvastatin - EMEA-003472-PIP01-23 41
3.1.41. Fusion protein consisting of relaxin and Fc domain of IgG1 - EMEA-003476-PIP01-23 41
3.1.42. Glyceryl - EMEA-003479-PIP01-23 41
3.1.43. EMEA-003480-PIP01-23 41

3.1.44. Lutikizumab - EMEA-003481-PIP01-23 41
3.1.45. EMEA-003478-PIP01-23 41
3.1.46. Bitopertin - Orphan - EMEA-000439-PIP03-23 42
3.1.47. Etavopivat - Orphan - EMEA-002924-PIP02-23 42
3.1.48. Human alpha-1 proteinase inhibitor, modified - EMEA-003463-PIP01-23 42
3.1.49. Ianalumab - EMEA-002338-PIP05-23 42
3.1.50. EMEA-003002-PIP04-23 42
3.1.51. Iodine (^{131}I) apamistamab - Orphan - EMEA-003395-PIP02-23 42
3.1.52. Radiprodil - EMEA-003462-PIP01-23 43
3.1.53. EMEA-003477-PIP01-23 43
3.1.54. (3S,3'S,3a'S,10a'S)-6-chloro-3'-(3-chloro-2-fluorophenyl)-1' (cyclopropylmethyl)-6'-methyl-2-oxo-1,2,3',3a',10',10a'-hexahydro-1'H-spiro[indole- 3,2'-pyrrolo[2',3':4,5]pyrrolo[1,2-b]indazole]-7'- carboxylic acid - Orphan - EMEA-003260-PIP03-23 43
3.1.55. EMEA-003470-PIP01-23 43
3.1.56. Gotistobart - EMEA-003467-PIP01-23 43
3.1.57. Humanised IgG1 monoclonal antibody against integrin beta-6 conjugated to monomethyl auristatin E via a valine-citrulline linker - EMEA-003471-PIP01-23 43
3.1.58. Ilginatinib - EMEA-003468-PIP01-23 44
3.1.59. Relatlimab / nivolumab - EMEA-002727-PIP02-23 44
3.1.60. Relatlimab / nivolumab - EMEA-002727-PIP03-23 44
3.1.61. EMEA-003469-PIP01-23 44
3.1.62. Messenger RNA encoding Cas9, single guide RNA targeting the human KLKB1 gene - EMEA-003465-PIP01-23 44
3.1.63. Imlifidase - Orphan - EMEA-002183-PIP03-23 44
3.1.64. Naproxen / paracetamol - EMEA-003475-PIP01-23 45
3.1.65. Humanised IgG1 monoclonal antibody against pituitary adenylate cyclase-activating polypeptide - EMEA-003483-PIP01-23 45
3.1.66. Atacicept - EMEA-002004-PIP04-23 45
3.2. Discussions on Compliance Check 45
3.2.1. Macitentan - EMEA-C3-001032-PIP01-10-M05 45
3.2.2. Nirsevimab - EMEA-C-001784-PIP01-15-M04 45
3.2.3. Ataluren - EMEA-C-000115-PIP01-07-M13 45
3.2.4. Lenvatinib - EMEA-C-001119-PIP03-19-M03 46
3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan 46
3.3.1. Macitentan - Orphan - EMEA-001032-PIP01-10-M07 46
3.3.2. Treprostinil sodium - EMEA-003182-PIP01-22-M01 46
3.3.3. Abrocitinib - EMEA-002312-PIP01-17-M02 46
3.3.4. Remibrutinib - EMEA-002582-PIP01-19-M03 46
3.3.5. Liraglutide - EMEA-000128-PIP02-09-M05 47
3.3.6. Pegzilarginase - Orphan - EMEA-001925-PIP02-19-M01 47

3.3.7. Maralixibat chloride - Orphan - EMEA-001475-PIP03-17-M04 47
3.3.8. Denecimig - EMEA-002762-PIP02-20-M02 47
3.3.9. Human fibrinogen concentrate - EMEA-001931-PIP01-16-M03 47
3.3.10. Sebetralstat - Orphan - EMEA-002723-PIP01-19-M02 47
3.3.11. Risankizumab - EMEA-001776-PIP02-17-M02 47
3.3.12. Upadacitinib - EMEA-001741-PIP03-16-M04 48
3.3.13. Bemnifosbuvir - EMEA-002963-PIP01-21-M01 48
3.3.14. Cilgavimab (AZD1061) - EMEA-002925-PIP01-20-M02 48
3.3.15. Dalbavancin hydrochloride - EMEA-000016-PIP01-07-M09 48
3.3.16. Letermovir - Orphan - EMEA-001631-PIP01-14-M05 48
3.3.17. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M04 48
3.3.18. Ritonavir / nirmatrelvir - EMEA-003081-PIP01-21-M03 49
3.3.19. Tixagevimab (AZD8895) - EMEA-002900-PIP01-20-M02 49
3.3.20. Zanamivir - EMEA-001318-PIP01-12-M05 49
3.3.21. Alprazolam - EMEA-003043-PIP01-21-M01 49
3.3.22. Ravulizumab - EMEA-001943-PIP04-20-M01 49
3.3.23. Satralizumab - Orphan - EMEA-001625-PIP02-21-M02 49
3.3.24. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M04 49
3.3.25. Lisocabtagene maraleucel - EMEA-001995-PIP01-16-M04 50
3.3.26. Lutetium (^{177}Lu) oxodotreotide - Orphan - EMEA-002950-PIP01-20-M01 50
3.3.27. Tirzepatide - EMEA-002360-PIP02-22-M02 50
3.3.28. Tapentadol (hydrochloride) - EMEA-000325-PIP01-08-M11 50
3.3.29. Mometasone (furoate) / glycopyrronium bromide / indacaterol - EMEA-001812-PIP01-15-M02 50
3.3.30. Seltorexant - EMEA-002746-PIP01-20-M02 50

4. Nominations 51

4.1. List of submissions of applications with start of procedure 11 September 2023 for Nomination of Rapporteur and Peer reviewer 51

4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver 51

4.3. Nominations for other activities 51

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction 51

5.1. New Scientific Advice 51

5.2. Final Scientific Advice (Reports and Scientific Advice letters) 51

6. Discussion on the applicability of class waivers 51

6.1. Discussions on the applicability of class waiver for products 51

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver 52

7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver 52

7.1.1. Setmelanotide - Orphan - EMEA-002209-PIP01-17-M03 52

7.1.2. Finerenone - EMEA-001623-PIP01-14-M06 52

8. Annual reports on deferrals 52

9. Organisational, regulatory and methodological matters 52

9.1. Mandate and organisation of the PDCO 52

9.1.1. PDCO membership 52

9.1.2. Vote by Proxy 53

9.1.3. Strategic Review and Learning Meeting (SRLM) - Madrid, Spain 17-18 October 2023 53

9.2. Coordination with EMA Scientific Committees or CMDh-v 53

9.2.1. Committee for Medicinal Products for Human Use (CHMP) 53

9.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups 53

9.3.1. Non-clinical Working Party: D30 Products identified 53

9.3.2. Formulation Working Group 54

9.3.3. Patients and Consumers Working Party (PCWP)/Healthcare Professionals Working Party (HCPWP) 54

9.3.4. Upcoming Innovation Task Force (ITF) meetings 54

9.3.5. SAWP cases for PDCO 54

9.4. Cooperation within the EU regulatory network 54

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA) 54

9.5. Cooperation with International Regulators 55

9.5.1. Paediatric Cluster Teleconference 55

9.6. Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee 55

9.7. PDCO work plan 55

9.7.1. PDCO Work Plan 2023 - reporting on progress 55

9.8. Planning and reporting 55

9.8.1. EMA Business Pipeline activity and Horizon scanning 55

10. Any other business 55

10.1. ACT EU priority action (PA) 8 on methodology 55

10.2. Request for feedback on recommendation for inclusion of adolescents in adult trials from the Clinical Trials Coordination Group (CTCG) 55

11. Breakout sessions 56

11.1. Paediatric oncology 56

11.2. Neonatology 56

12. List of participants 57
13. Explanatory notes 60

1. Introductions

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

The Chair opened the meeting by welcoming all participants. The meeting was held in-person.

In accordance with the Agency's policy on handling of declarations of interests of scientific Committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and on the topics in the agenda of the meeting, the Committee Secretariat announced the restricted involvement of some Committee members, alternates and experts for concerned agenda topics.

Participants were asked to declare any changes, omissions or errors to their declared interests concerning the matters for discussion. No new or additional competing interests were declared. Restrictions applicable to this meeting are captured in the List of participants included in the minutes.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure. All decisions taken at this meeting were made in the presence of a quorum of members. All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

The Chair welcomed the new members and alternates and thanked the departing members/alternates for their contributions to the Committee.

The EMA Secretariat announced the names of the Committee members who delegated their vote via proxy and the Committee members who received such proxy.

1.2. Adoption of agenda

The agenda for 05-08 September 2023 meeting was adopted with amendments.

1.3. Adoption of the minutes

The minutes for 17-21 July 2023 meeting were adopted with amendments and will be published on the EMA website.

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. A self-complementary adeno-associated virus [AAV] serotype 8 virus particle encoding the human ornithine transcarbamylase [OTC] gene sequence (DTX301) - Orphan - EMEA-002830-PIP01-20

Ultragenyx Germany GmbH; Treatment of ornithine transcarbamylase deficiency

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Summary of Committee discussion:

During its plenary in September 2023, the PDCO discussed at Day 120 the paediatric investigation plan with a deferral proposal for DTX301 (a self-complementary adeno-associated virus [AAV] serotype 8 virus particle encoding the human ornithine transcarbamylase [OTC] gene sequence) for treatment of ornithine transcarbamylase (OTC) deficiency.
The Committee confirmed all conclusions reached at Day 90 and took into consideration further information provided by the applicant between Day 90 and Day 120.
The PDCO adopted a positive opinion on a paediatric investigation plan.

2.1.2. A nonreplicating, recombinant adeno-associated virus (AAV) serotype 9 (AAV9) gene transfer vector that contains a modified human ATP7B coding sequence - Orphan - EMEA-003131-PIP01-21

Ultragenyx Germany GmbH; Treatment of Wilson disease

Day 120 opinion

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 24 August 2023

2.1.3. Fazirsiran - Orphan - EMEA-003355-PIP01-22

Takeda Pharma A/S; Treatment of alpha-1 antitrypsin deficiency-associated liver disease

Day 120 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from 9 months to less than 18 months of age, in the condition treatment of congenital alpha-1 antitrypsin deficiency was adopted. The PDCO agreed on a waiver in a subset of children from birth to less than 9 months of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.4. Inhibitor of receptor-interacting protein kinase 1 (ABBV-668) - EMEA-003356-PIP01-22

AbbVie Ltd; Treatment of ulcerative colitis

Day 120 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for the paediatric population from birth to less than 2 years of age, in the condition treatment of ulcerative colitis was adopted. The PDCO agreed on a waiver in children from birth to less than 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.5. Tezepelumab - EMEA-001613-PIP03-21

AstraZeneca AB; Treatment of eosinophilic esophagitis

Day 120 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

The applicant addressed some of the issues raised by the Committee at Day 90 in their written response.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion on a PIP for tezepelumab has been agreed in children from 2 years to less than 18 years of age for treatment of eosinophilic esophagitis.
The Committee granted a waiver for the paediatric population from birth to less than 2 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.
The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.6. Deucrictibant - EMEA-003090-PIP02-22

Pharvaris Netherlands BV; Treatment of hereditary angioedema

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for the paediatric population from, in the condition treatment of hereditary angioedema with indication treatment of and prevention of hereditary angioedema (HAE) attacks in paediatric patients from 2 years to less than 18 years of age. The PIP includes one quality study, five clinical

studies and three modelling and simulation analyses to support an extrapolation plan. The PDCO agreed on a waiver in a subset of children on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.7. Ciraparantag - EMEA-003321-PIP01-22

NORGINE BV; Treatment of FXa inhibitor-associated haemorrhage / Prevention of FXa inhibitor-associated haemorrhage

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from birth to less than 18 years of age, in the condition of prevention of FXa inhibitor-associated haemorrhage, treatment of FXa inhibitor-associated haemorrhage was adopted. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.8. Luspatercept - Orphan - EMEA-001521-PIP03-22

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

Day 120 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed at Day 120, during the September 2023 plenary meeting a paediatric investigation plan for luspatercept for the treatment of alpha thalassaemia. The PDCO confirmed all conclusions reached at Day 90 and, based on the assessment of this application, adopted a positive opinion on a paediatric investigation plan for children from 6 years to less than 18 years, with a deferral for the treatment of alpha thalassaemia and a waiver for a subset of children from birth to less than 6 years of age on the grounds that the specific medicinal product is likely to be unsafe for these paediatric patients.

2.1.9. Enpatoran - EMEA-003342-PIP02-22

Merck Europe B.V.; Treatment of systemic lupus erythematosus

Day 120 opinion

Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

The PDCO adopted a positive opinion for this PIP for enpatoran (film-coated tablet) for the

treatment of systemic lupus erythematosus in the paediatric population from 5 years of age. The population below 5 years of age is covered by a waiver based on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). The PIP includes one quality-related study and two non-clinical studies. The clinical development consists in a double-blind, placebo-controlled, randomised withdrawal study to evaluate the clinical response, pharmacokinetics and safety of enpatoran in paediatric patients with systemic lupus erythematosus and a modelling and simulation study, which are part of an extrapolation plan covering the paediatric population from 5 years to less than 18 years of age. The completion of some studies in the agreed PIP is deferred.

2.1.10. Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate (ECT-001-CB) - Orphan - EMEA-003025-PIP03-23

ExCellThera; Treatment in haematopoietic stem cell transplantation in patients with acute myeloid leukaemia

Day 120 opinion

Immunology-Rheumatology-Transplantation / Oncology / Haematology-Hemostaseology

Summary of Committee discussion:

The PDCO re-discussed at Day 120, during the September 2023 plenary meeting a paediatric investigation plan for haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate (ECT-001-CB) for the treatment in allogeneic haematopoietic stem cell transplantation.
The PDCO confirmed all conclusions reached at Day 90 and assessed further clarification provided by the applicant between Day 90 and Day 120. Based on the assessment of this application, the PDCO, adopted a positive opinion at Day 120 on a paediatric investigation plan for children from birth to less than 18 years, with a deferral for the treatment in haematopoietic stem cell transplantation in patients with acute myeloid leukaemia.

2.1.11. Ensitrelvir - EMEA-003192-PIP01-22

Treatment of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Infectious Diseases

Note: Withdrawal request received on 07 September 2023

2.1.12. Ruzotolimod - EMEA-003363-PIP01-22

F. Hoffmann-La Roche Ltd.; Treatment of chronic hepatitis B

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO adopted a positive opinion for a PIP for ruzotolimod in children from 2 years of age to less than 18 years of age in the condition of treatment of chronic hepatitis B. The PIP includes a waiver below 2 years of age based on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset, and a deferral. The PIP consists of one clinical study and one modelling and simulation study.

2.1.13. Xalnesiran - EMEA-003362-PIP01-22

F. Hoffmann-La Roche Ltd.; Treatment of chronic hepatitis B

Day 120 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO adopted a positive opinion for a PIP for xalnesiran in children from 2 years of age to less than 18 years of age in the condition of treatment of chronic hepatitis B. The PIP includes a waiver below 2 years of age based on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset, and a deferral. The PIP consists of one clinical study and one modelling and simulation study.

2.1.14. Rozanolixizumab - EMEA-002681-PIP03-21

UCB Pharma S.A.; Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 120 opinion

Neurology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from 2 years to less than 18 years of age, in the condition of myelin oligodendrocyte glycoprotein antibody-associated disease was adopted. The PDCO agreed on a waiver in a subset of children on the grounds of lack of significant therapeutic benefit. The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.15. Nemvaleukin alfa - EMEA-003357-PIP01-22

Alkermes Pharma Ireland Limited; Treatment of malignant neoplasms of the lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue)

Day 120 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee.
Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for the age subset from 6 months to less than 18 years of age, in the condition of treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue). The PDCO agreed on a waiver in a subset of children on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.16. Vibostolimab / pembrolizumab - EMEA-003063-PIP03-22

Merck Sharp & Dohme (Europe) Inc.; Treatment of melanoma

Day 120 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 120, during the September 2023 plenary meeting, a paediatric investigation plan for vibostolimab / pembrolizumab for the treatment of melanoma.
Based on the assessment of this application and the additional information provided by the applicant, the PDCO adopted a positive opinion on a paediatric investigation plan for children from 12 years to less than 18 years for the treatment of melanoma and a waiver for a subset of children from birth to less than 12 years of age on the grounds that the disease for which the specific medicinal product is intended does not occur in the specified paediatric subset.

2.1.17. Uproleselan - Orphan - EMEA-003307-PIP01-22

GlycoMimetics, Inc.; Treatment of acute myeloid leukaemia

Day 120 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for birth to less than 28 days, in the condition of treatment of acute myeloid leukaemia was adopted. The Norwegian PDCO member was in agreement. The PDCO agreed on a waiver in a subset of children on the grounds that clinical studies with the specific medicinal product cannot be

expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). The PDCO granted a deferral for one or more measures contained in the paediatric investigation plan.

2.1.18. Pabinafusp alfa - Orphan - EMEA-003033-PIP02-22

JCR Pharmaceuticals Co., Ltd.; Treatment of mucopolysaccharidosis II (Hunter´s syndrome)

Day 120 opinion

Other

Summary of Committee discussion:

Based on the assessment of this application, the additional information provided by the applicant and further discussions at the Paediatric Committee, the PDCO adopted a positive opinion on Day 120 for pabinafusp alfa for paediatric patients from birth to 18 years of age for the treatment of mucopolysaccharidosis II (Hunter´s syndrome).
The PDCO granted a deferral for one or more measures contained in this PIP.

2.1.19. Derivative of 6-(piperidine-1-carbonyl)pyridin-3-ol (BI 764198) - EMEA-003347-PIP01-22

Boehringer Ingelheim International GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 120 opinion

Uro-nephrology

Summary of Committee discussion:

In the written response the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for derivative of 6-(piperidine-1-carbonyl)pyridin-3-ol (BI 764198), for the paediatric population from 1 year to less than 18 years of age in the condition treatment of glomerulonephritis and nephrotic syndrome was adopted.
The PDCO agreed on a waiver in the paediatric population from birth to less than 1 year of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO granted a deferral for some measures contained in this PIP.

2.1.20. Inaxaplin - Orphan - EMEA-003368-PIP01-22

Vertex Pharmaceuticals (Ireland) Limited; Treatment of apolipoprotein L1 (APOL1)-mediated kidney disease

Day 120 opinion

Uro-nephrology

Summary of Committee discussion:

The PDCO adopted a positive opinion on this PIP for inaxaplin for the treatment of APOL1-mediated kidney disease in paediatric patients from 12 years of age. A waiver was adopted for the population below 12 years of age on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible. The PIP includes a double-blind, randomised, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics of inaxaplin as add-on to standard of care in paediatric patients from 12 years to less than 18 years of age (and adults) with APOL1-mediated kidney disease, which is part of an extrapolation plan. The completion of the PIP is deferred.

2.1.21. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / outer membrane vesicles (OMV) from *N. meningitidis* strain NZ 98/254 / recombinant *Neisseria meningitidis* group B fHbp 2-3-1.13NB fusion protein / recombinant *Neisseria meningitis* group B protein 961c / recombinant *Neisseria meningitis* group B protein 287-953 / recombinant *Neisseria meningitis* group B protein 936-741 - EMEA-003359-PIP01-22

GlaxoSmithKline Biologicals SA; Prevention of meningococcal disease

Day 120 opinion

Vaccines

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from 2 months to less than 18 years of age, in the condition of prevention of meningococcal disease, was adopted. The PDCO agreed on a waiver from birth to less than 2 months of age on the grounds that the specific medicinal product is likely to be ineffective.

2.1.22. *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

Sanofi Pasteur; Prevention of meningococcal disease

Day 120 opinion

Vaccines

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and the additional information

provided by the applicant, a positive opinion for the PIP for the proposed medicine for children from 6 weeks to less than 18 years of age, in the condition of prevention of meningococcal disease, was adopted. The PDCO agreed on a waiver from birth to less than 6 weeks of age on the grounds that the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset. The PDCO granted a deferral for one or more measures contained in the paediatric investigational plan.

2.1.23. Rosuvastatin / amlodipine - EMEA-003446-PIP01-23

LANOVA FARMACEUTICI SRL; Prevention of cardiovascular events / Treatment of ischemic coronary artery disorders / Treatment of hypertension

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for rosuvastatin / amlodipine for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of hypertension, treatment of ischemic coronary artery disorders and prevention of cardiovascular events.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.24. Modified, recombinant version of the human myeloid-derived growth factor - EMEA-003449-PIP01-23

Boehringer Ingelheim International GmbH (BI); Treatment of ischaemic coronary artery disorders

Day 60 opinion

Cardiovascular Diseases

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for a modified recombinant version of the human myeloid-derived growth factor, for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of ischaemic coronary artery disorders.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.25. 2-[3-(dimethyl-1H-1,2,3-triazol-5-yl)-5-[(S)-oxan-4-yl(phenyl)methyl]-5H-pyrido[3,2-b]indol-7-yl]propan-2-ol - EMEA-003456-PIP01-23

Bristol-Myers Squibb Pharma EEIG; Treatment of myelofibrosis

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for 2-[3-(dimethyl-1H-1,2,3-triazol-5-yl)-5-[(S)-oxan-4-yl(phenyl)methyl]-5H-pyrido[3,2-b]indol-7-yl]propan-2-ol in the condition of treatment of myelofibrosis for all subsets of the paediatric population from birth to less than 18 years of age, all pharmaceutical forms, all routes of administration, on the grounds that the condition for which the specific medicinal product is intended does not occur in the paediatric population. The applicant agreed to extending the waiver to all pharmaceutical forms and routes of administration.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.26. 2-((R)-3-(1-(1-((R)-1-(2,4-dichlorophenyl)ethyl)-3-(trifluoromethyl)-1H-pyrazolo[3,4-b]pyrazin-6-yl)azetidin-3-yl)piperidin-1-yl)ethan-1-ol, benzenesulfonate - EMEA-003455-PIP01-23

RAPT Therapeutics Inc; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of lung cancer on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.27. Belrestotug - EMEA-003452-PIP01-23

GlaxoSmithKline Trading Services Limited; Treatment of lung cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of lung cancer on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.28. Bezuclastinib - EMEA-003445-PIP01-23

Cogent Biosciences, Inc; Treatment of mastocytosis

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver.
The PDCO recommended granting a waiver for bezuclastinib for all subsets of the paediatric population (0 to 18 years of age) in the condition of 'treatment of mastocytosis' on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2.1.29. Humanised IgG1 monoclonal antibody against TROP2, conjugated to a topoisomerase I inhibitor belotecan-derivative - EMEA-003461-PIP01-23

Merck Sharp & Dohme (Europe) Inc.; Treatment of lung cancer / Treatment of ovarian cancer / Treatment of cervical cancer / Treatment of gastric cancer / Treatment of breast cancer / Treatment of endometrial cancer

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for humanised IgG1 monoclonal antibody against TROP2, conjugated to a topoisomerase I inhibitor belotecan-derivative for all subsets of the paediatric population (from birth to 18 years of age) in the condition of treatment of gastric cancer, treatment of cervical cancer, treatment of lung cancer, treatment of breast cancer,

treatment of ovarian cancer, treatment of endometrial cancer on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.30. Tiragolumab - EMEA-002721-PIP04-23

Roche Registration GmbH; Treatment of hepatocellular carcinoma

Day 60 opinion

Oncology

Summary of Committee discussion:

The PDCO re-discussed at Day 60, during the September 2023 plenary meeting a request for a product-specific waiver for tiragolumab for the treatment of hepatocellular carcinoma on the grounds that the disease does not occur in children.
The PDCO confirmed all the conclusions reached at Day 30 and adopted a positive opinion at Day 60 for tiragolumab for the treatment of hepatocellular carcinoma on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.31. Valemetostat tosilate - Orphan - EMEA-003256-PIP02-23

Daiichi Sankyo Europe GmbH; Treatment of mature T-cell neoplasms

Day 60 opinion

Oncology

Summary of Committee discussion:

In the written response, the applicant addressed the remaining issues raised by the Committee. Based on the assessment of this application and discussions at the Paediatric Committee the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for the proposed medicine for all subsets of the paediatric population (from birth to less than 18 years of age) for the condition treatment of mature T-cell neoplasms on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). The Norwegian PDCO member was in agreement.

2.1.32. Zanidatamab - Orphan - EMEA-003450-PIP01-23

Jazz Pharmaceuticals Ireland Limited; Treatment of biliary tract cancer (BTC)

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for zanidatamab for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of treatment of biliary tract cancer, based on the ground that the diseases do not occur in children. Since the agreed waiver ground is disease not occurring, the possibility to apply this to all pharmaceutical forms and all routes of administrations was agreed to by the applicant.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.33. Genetically detoxified pertussis toxin (PTgen) / pertussis filamentous haemagglutinin (FHA) - EMEA-003442-PIP01-23

BIONET EUROPE; Prevention of pertussis disease

Day 60 opinion

Vaccines

Summary of Committee discussion:

The PDCO discussed at Day 60, during the September 2023 plenary meeting, an application for a waiver for genetically detoxified pertussis toxin (PTgen) / pertussis filamentous haemagglutinin (FHA) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of prevention of pertussis disease.
Based on the assessment of this application the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for genetically detoxified pertussis toxin (PTgen) / pertussis filamentous haemagglutinin (FHA) for all subsets of the paediatric population (from birth to less than 18 years of age) in the condition of prevention of pertussis disease.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.34. Actinium chloride (non-carrier added) - EMEA-003482-PIP01-23

ITM Medical Isotopes GmbH; Radiolabelling agent

Day 30 opinion

Oncology

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for actinium chloride (non-carrier added) for all subsets of the paediatric population (0 to 18 years of age) in the condition of radiolabelling agent.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.1.35. Ulviprubart - EMEA-003474-PIP01-23

Abcuro, Inc.; Treatment of inclusion body myositis

Day 30 opinion

Other / Immunology-Rheumatology-Transplantation

Summary of Committee discussion:

Based on the assessment of this application and further discussions at the Paediatric Committee, the PDCO agreed with the applicant's request for a waiver. The PDCO recommended granting a waiver for ulviprubart for all subsets of the paediatric population (0 to 18 years of age) in the condition of treatment of inclusion body myositis.
The PDCO emphasised that the granting of a waiver for the condition mentioned above should not prevent the applicant from considering a development in the paediatric population in indications where there is a paediatric need. In principle according to the Paediatric Regulation, incentives for the development for use in the paediatric population are available even if a waiver has been granted in another condition.

2.2. Opinions on Compliance Check

2.2.1. Abacavir (ABC) / lamivudine (3TC) / dolutegravir (DTG) - EMEA-C-001219-PIP01-11-M06

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

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO took note of outcomes of preceding partial compliance check procedures:

- EMEA-C1-001219-PIP01-11-M05

The PDCO adopted on 8 September 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision

(P/0038/2023) of 31 January 2023.

2.2.2. Leriglitazone - EMEA-C2-002106-PIP01-16-M02

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 60 letter

Neurology

Summary of Committee discussion:

The PDCO confirmed compliance of all key elements of Study 1 except its completion date; the completion and compliance of Study 4 will be checked in another procedure.

2.2.3. Molgramostim - EMEA-C1-002282-PIP01-17-M01

Savara ApS; Treatment of pulmonary alveolar proteinosis

Day 60 letter

Pneumology - Allergology

Summary of Committee discussion:

The PDCO discussed the completed studies, Study 1 and Study 2, and considered that these are compliant with the latest Agency's Decision (P/0509/2021) of 3 December 2021.
The PDCO finalised this partially completed compliance procedure on 8 September 2023.

2.2.4. Bulevirtide - EMEA-C-002399-PIP01-18-M01

Gilead Sciences Ireland UC; Treatment of chronic hepatitis D infection

Day 30 opinion

Infectious Diseases

Summary of Committee discussion:

The PDCO adopted on 8 September 2023 an opinion confirming the compliance of all studies in the agreed paediatric investigation plan as set out in the latest Agency's Decision (P/0270/2022) of 10 August 2022.

2.2.5. Cysteamine hydrochloride - EMEA-C-000322-PIP01-08-M06

Recordati Rare Diseases; Treatment of corneal cystine crystal deposits in cystinosis

Day 30 opinion

Ophthalmology

Summary of Committee discussion:
The PDCO discussed the compliance check request and considered Study 3 to be compliant. The remaining studies were already assessed in previous procedures. A positive opinion was issued on this full compliance check.

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Dupilumab - EMEA-001501-PIP02-13-M08

Sanofi Winthrop Industrie; Treatment of asthma

Day 60 opinion

Dermatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The main change was an update to the statistical analysis of Study 7 (randomised, double-blind, placebo controlled study to assess the efficacy and long term safety of dupilumab in children 2 years to less than 6 years old with uncontrolled asthma and/or recurrent severe asthmatic wheeze).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0039/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.2. Fitusiran - Orphan - EMEA-001855-PIP01-15-M05

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

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes to Studies 4, 5 and 6 including among others the number of patients, the definition of patient population, the treatment duration, the definition of endpoints, the analysis of the data, a delay in timelines and the addition of a new study to assess efficacy and safety of the revised dosing strategy in adults and adolescents could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0109/2023 of 14 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.3. Vonicog alfa - EMEA-001164-PIP01-11-M07

Baxalta Innovations GmbH; Treatment of Von Willebrand disease

Day 60 opinion

Haematology-Hemostaseology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes to the

number of patients on the on-demand arm could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0363/2022 of 7 September 2022)
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.4. BNT162b2 / tozinameran / famtozinameran / riltozinameran - EMEA-002861-PIP02-20-M06

BioNTech Manufacturing GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0466/2022 of 7 November 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.5. Eravacycline - EMEA-001555-PIP01-13-M05

PAION Deutschland GmbH; Treatment of complicated intra-abdominal infection

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted (main change related to the deletion of Study 5, a double-blind, randomised, active controlled trial in children with complicated intra-abdominal infections from 8 years to less than 18 years of age).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0255/2020 of 15 July 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.6. Remdesivir - EMEA-002826-PIP01-20-M04

Gilead Sciences International Ltd.; Treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Infectious Diseases

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be

accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0221/2022 of 24 June 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.7. Eptinezumab - EMEA-002243-PIP01-17-M04

H. Lundbeck A/S; Prevention of migraine headaches

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0341/2022 of 10 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.8. Nipocalimab (anti-neonatal Fc receptor human monoclonal antibody) - EMEA-002559-PIP02-19-M01

Janssen-Cilag International NV; Treatment of myasthenia gravis

Day 60 opinion

Neurology

Note: Withdrawal request received on 06 September 2023

2.3.9. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M05

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a positive opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0015/2022 of 31 January 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.10. Peginterferon beta-1a - EMEA-001129-PIP01-11-M06

Biogen Idec Ltd; Treatment of multiple sclerosis

Day 60 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted. The main changes concerned timelines and sample sizes.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0359/2022 of 22 August 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.11. Carfilzomab - Orphan - EMEA-001806-PIP04-19-M02

Amgen Europe B.V; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes on the timelines for Study 1 from January 2024 to October 2024, delaying the overall completion of the PIP, could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0107/2023 of 14 April 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.12. Cemiplimab - EMEA-002007-PIP02-17-M03

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

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0179/2022 of 13 May 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.13. Tovorafenib - Orphan - EMEA-002763-PIP01-20-M01

Day One Biopharmaceuticals, Inc; Treatment of paediatric low-grade glioma

Day 60 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0500/2020 of 22 December 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.14. Iptacopan - Orphan - EMEA-002705-PIP02-19-M01

Novartis Europharm Limited; Treatment of IgA nephropathy

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted (main modification: study timelines).
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0415/2020 of 23 October 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.15. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M05

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered safe to initiate the clinical study 10 (C-7) in children from 12 months of age to less than 2 years of age and therefore considered that the proposed change could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0265/2022 of 22 July 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.16. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M05

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

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0272/2020 of 13 July 2020).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.17. Selexipag - EMEA-000997-PIP01-10-M07

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 60 opinion

Other

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0088/2023 of 10 March 2023).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.18. Evenamide - EMEA-002519-PIP03-21-M01

Newron Pharmaceuticals S.p.A.; Treatment of schizophrenia

Day 60 opinion

Psychiatry

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that proposed changes to the timelines for Studies 1, 2, 3 and 4 delaying the overall completion date of the PIP from June 2026 to November 2026 could be accepted and that Studies 1 and 4 are to remain not deferred.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0517/2021 of 3 December 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.19. NVX-CoV2373 - EMEA-002941-PIP01-20-M04

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

Day 60 opinion

Vaccines

Summary of Committee discussion:

Based on the responses received by the applicant to the outstanding point at Day 30, and

the review of the overall rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0250/2022 of 8 July 2022).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.20. Recombinant COVID-19 subunit nanoparticle - EMEA-003115-PIP01-21-M02

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

Day 60 opinion

Vaccines

Note: Withdrawal request received on 01 September 2023

2.3.21. Autologous tumour-infiltrating lymphocytes (LN-144/LN-145) - EMEA-002776-PIP01-20-M02

Iovance Biotherapeutics, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 opinion

Oncology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0363/2021 of 8 September 2021).
The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.22. Angiotensin II acetate - EMEA-001912-PIP02-16-M03

PAION Deutschland GmbH; Treatment of hypotension associated with distributive or vasodilatory shock

Day 30 opinion

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0159/2019 of 15 April 2019). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.23. Brivaracetam - EMEA-000332-PIP02-17-M05

UCB Pharma S.A.; Treatment of paediatric epilepsy syndromes / Treatment of neonatal seizures

Day 30 opinion

Neurology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.
The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0093/2023 of 10 March 2023). The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

2.3.24. Risankizumab - EMEA-001776-PIP04-17-M01

AbbVie Ltd; Treatment of ulcerative colitis

Day 30 opinion

Gastroenterology-Hepatology

Summary of Committee discussion:

Based on the review of the rationale submitted by the application for modifying the agreed paediatric investigation plan, the PDCO considered that the proposed changes could be accepted.

The PDCO therefore adopted a favourable opinion on the modification of the agreed PIP as set in the Agency's latest decision (P/0231/2018 of 3 August 2018).

The new PDCO opinion on the modified agreed PIP supersedes the previous PDCO opinion.

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. Beremagene geperpavec - EMEA-C1-002472-PIP03-22-M01

Krystal Biotech Netherlands B.V.; Treatment of dystrophic epidermolysis bullosa

Day 30 letter

Dermatology

2.7.2. Gepotidacin - EMEA-C1-002443-PIP01-18-M02

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urinary tract infections

Day 30 letter

Infectious Diseases

2.7.3. Repotrectinib - EMEA-C1-002635-PIP02-21-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic neoplasms)

Day 30 letter

Oncology

2.7.4. Sarilumab - EMEA-C1-001045-PIP01-10-M03

sanofi-aventis recherche & développement; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 letter

Immunology-Rheumatology-Transplantation

2.7.5. Remibrutinib - EMEA-C1-002582-PIP01-19-M02

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 letter

Dermatology

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. Amlitelimab - EMEA-003233-PIP01-22

Treatment of atopic dermatitis

Day 90 discussion

Dermatology

3.1.2. Upadacitinib - EMEA-001741-PIP08-22

Treatment of hidradenitis suppurativa

Day 90 discussion

Dermatology

3.1.3. Upadacitinib - EMEA-001741-PIP09-23

Treatment of systemic lupus erythematosus

Day 90 discussion

Dermatology

3.1.4. *Escherichia coli*, expressing high affinity phenylalanine transporter, modified phenylalanine ammonia lyase and L-amino acid deaminase - EMEA-003381-PIP01-22

Treatment of hyperphenylalaninemia

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. Recombinant human monoclonal antibody to insulin receptor - Orphan - EMEA-002813-PIP01-23

Rezolute (Bio) Ireland Limited; Treatment of congenital hyperinsulinism

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.6. Venglustat - Orphan - EMEA-001716-PIP07-22

Sanofi B.V.; Treatment of Gaucher disease type 3 / Treatment of Gaucher disease

Day 90 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. Cemdisiran - Orphan - EMEA-003237-PIP02-22

Regeneron Ireland DAC; Treatment of paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Haematology-Hemostaseology

3.1.8. Pozelimab - EMEA-003238-PIP02-22

Treatment of paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Haematology-Hemostaseology

3.1.9. Tetrahydrouridine / decitabine - Orphan - EMEA-003404-PIP01-23

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

Day 90 discussion

Haematology-Hemostaseology

3.1.10. Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP01-23

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy

Day 90 discussion

Neurology

3.1.11. Inebilizumab - EMEA-001911-PIP02-22

Treatment of generalised myasthenia gravis

Day 90 discussion

Neurology

3.1.12. Cobolimab - EMEA-003273-PIP02-22

Treatment of all conditions included in the category of malignant neoplasms including lymphoma (except lung cancers and hematopoietic malignancies)

Day 90 discussion

Oncology

3.1.13. Iptacopan - EMEA-002705-PIP05-23

Treatment of immune-complex mediated membranoproliferative glomerulonephritis

Day 90 discussion

Other

3.1.14. Oral inhibitor of PCSK9 - EMEA-003453-PIP01-23

Treatment of hypercholesterolaemia

Day 60 discussion

Cardiovascular Diseases

3.1.15. Upadacitinib - EMEA-001741-PIP10-23

Treatment of alopecia areata

Day 60 discussion

Dermatology

3.1.16. GIPR antagonist/GLP-1R agonist - EMEA-003439-PIP02-23

Treatment of obesity

Day 60 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.17. Plecanatide - EMEA-003441-PIP01-23

Treatment of irritable bowel syndrome with constipation / Treatment of chronic idiopathic constipation

Day 60 discussion

Gastroenterology-Hepatology

3.1.18. Hydroxycarbamide - EMEA-003388-PIP01-23

Treatment of sickle cell disease

Day 60 discussion

Haematology-Hemostaseology

3.1.19. Ianalumab - EMEA-002338-PIP04-23

Treatment of autoimmune haemolytic anaemia

Day 60 discussion

Haematology-Haemostaseology

3.1.20. Reparixin - Orphan - EMEA-001693-PIP06-23

Dompé farmaceutici S.p.A.; Treatment of infectious pneumonia acquired in the community, excluding coronavirus disease 2019 (COVID-19)

Day 60 discussion

Infectious Diseases

3.1.21. Ganaxolone - EMEA-002341-PIP03-23

Treatment of refractory status epilepticus

Day 60 discussion

Neurology

3.1.22. Recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA - Orphan - EMEA-003459-PIP01-23

Myrtelle,Inc.; Treatment of Canavan disease

Day 60 discussion

Neurology

3.1.23. Autologous patient-derived CD4+ and CD8+ T cells expressing a chimeric antigen receptor specific for claudin 6 - EMEA-003377-PIP01-23

Treatment of malignant neoplasms of the central nervous system (CNS) / Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue neoplasms)

Day 60 discussion

Oncology

3.1.24. Disitamab vedotin - EMEA-003443-PIP02-23

Treatment of HER2 expressing tumours / Treatment of solid tumours

Day 60 discussion

Oncology

3.1.25. Liposome-formulated messenger ribonucleic-acid vaccine encoding the chimeric antigen-receptor target antigen claudin 6 - EMEA-003464-PIP01-23

Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue neoplasms) / Treatment of malignant neoplasms of the central nervous system (CNS)

Day 60 discussion

Oncology

3.1.26. Obecabtagene autoleucel - Orphan - EMEA-003171-PIP02-23

Autolus GmbH; Treatment of non-Hodgkin lymphoma

Day 60 discussion

Oncology

3.1.27. Unesbulin - Orphan - EMEA-003297-PIP02-23

PTC Therapeutics International; Treatment of soft tissue sarcoma

Day 60 discussion

Oncology

3.1.28. Allogeneic faecal microbiota, pooled - Orphan - EMEA-003435-PIP02-23

MaaT Pharma; Treatment of graft-versus-host disease

Day 60 discussion

Oncology / Haematology-Hemostaseology

3.1.29. Clobetasol - EMEA-003458-PIP01-23

Treatment of ocular infections, inflammations and associated manifestations

Day 60 discussion

Ophthalmology

3.1.30. Laruparetigene zovaparvovec - Orphan - EMEA-003457-PIP01-23

FGK Representative Service GMBH; Treatment of X-linked retinitis pigmentosa

Day 60 discussion

Ophthalmology

3.1.31. Garetosmab - Orphan - EMEA-002736-PIP02-23

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

Day 60 discussion

Other

3.1.32. Losmapimod - Orphan - EMEA-003448-PIP01-23

Fulcrum Therapeutics, Inc.; Treatment of facioscapulohumeral muscular dystrophy

Day 60 discussion

Other

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

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

Day 60 discussion

Other

3.1.34. Sisunatovir - EMEA-002529-PIP02-23

Treatment of respiratory tract disease caused by respiratory syncytial virus (RSV) infection

Day 60 discussion

Other

3.1.35. Imlifidase - EMEA-002183-PIP02-23

Treatment of patients with Duchenne muscular dystrophy (DMD) and pre-existing antibodies to the AAV vector to enable gene therapy

Day 60 discussion

Other / Uro-nephrology

3.1.36. Mometasone - EMEA-003454-PIP01-23

Treatment of chronic rhinosinusitis (CRS)

Day 60 discussion

Oto-rhino-laryngology

3.1.37. EMEA-003451-PIP01-23

Treatment of bronchiectasis

Day 60 discussion

Pneumology - Allergology

3.1.38. *Borrelia* outer surface protein A (OspA) serotypes (ST1-6) lipidated, fusion protein vaccine - EMEA-003130-PIP02-23

Prevention of Lyme disease

Day 60 discussion

Vaccines

3.1.39. Candesartan / atorvastatin / amlodipine - EMEA-003466-PIP01-23

Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

3.1.40. Ezetimibe / rosuvastatin - EMEA-003472-PIP01-23

Treatment of hypercholesterolaemia / Prevention of cardiovascular events

Day 30 discussion

Cardiovascular Diseases

3.1.41. Fusion protein consisting of relaxin and Fc domain of IgG1 - EMEA-003476-PIP01-23

Treatment of pulmonary hypertension due to left heart disease

Day 30 discussion

Cardiovascular Diseases

3.1.42. Glyceryl - EMEA-003479-PIP01-23

Treatment of ischaemic coronary artery disorders / Treatment of heart failure signs and symptoms / Prevention of cardiac and vascular procedural complications

Day 30 discussion

Cardiovascular Diseases

3.1.43. EMEA-003480-PIP01-23

Treatment of psoriasis

Day 30 discussion

Dermatology

3.1.44. Lutikizumab - EMEA-003481-PIP01-23

Treatment of hidradenitis suppurativa

Day 30 discussion

Dermatology

3.1.45. EMEA-003478-PIP01-23

Treatment of psoriasis

Day 30 discussion

Dermatology

3.1.46. Bitopertin - Orphan - EMEA-000439-PIP03-23

Disc Medicine B.V.; Treatment of X-linked protoporphyria / Treatment of erythropoietic protoporphyria

Day 30 discussion

Dermatology / Haematology-Hemostaseology

3.1.47. Etavopivat - Orphan - EMEA-002924-PIP02-23

Novo Nordisk A/S; Treatment of sickle cell disease

Day 30 discussion

Haematology-Hemostaseology

3.1.48. Human alpha-1 proteinase inhibitor, modified - EMEA-003463-PIP01-23

Treatment of haemophilia B

Day 30 discussion

Haematology-Hemostaseology

3.1.49. Ianalumab - EMEA-002338-PIP05-23

Treatment of immune thrombocytopenia (ITP)

Day 30 discussion

Haematology-Haemostaseology

3.1.50. EMEA-003002-PIP04-23

Treatment of systemic sclerosis (SSc)

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.1.51. Iodine (^{131}I) apamistamab - Orphan - EMEA-003395-PIP02-23

Immedica Pharma AB; Treatment in allogenic stem cell transplantation

Day 30 discussion

Immunology-Rheumatology-Transplantation / Oncology

3.1.52. Radiprodil - EMEA-003462-PIP01-23

Treatment of GRIN-related disorders

Day 30 discussion

Neurology

3.1.53. EMEA-003477-PIP01-23

Treatment of appetite and general nutrition disorders

Day 30 discussion

Nutrition

3.1.54. (3S,3'S,3a'S,10a'S)-6-chloro-3'-(3-chloro-2-fluorophenyl)-1' (cyclopropylmethyl)-6'-methyl-2- oxo-1,2,3',3a',10',10a'-hexahydro-1'H-spiro[indole- 3,2'-pyrrolo[2',3':4,5]pyrrolo[1,2-b]indazole]-7'- carboxylic acid - Orphan - EMEA-003260-PIP03-23

Boehringer Ingelheim International GmbH; Treatment of soft tissue sarcoma

Day 30 discussion

Oncology

3.1.55. EMEA-003470-PIP01-23

Treatment of breast malignant neoplasms

Day 30 discussion

Oncology

3.1.56. Gotistobart - EMEA-003467-PIP01-23

Treatment of non-small cell lung cancer

Day 30 discussion

Oncology

3.1.57. Humanised IgG1 monoclonal antibody against integrin beta-6 conjugated to monomethyl auristatin E via a valine-citrulline linker - EMEA-003471-PIP01-23

Treatment of solid tumours

Day 30 discussion

Oncology

3.1.58. Ilginatinib - EMEA-003468-PIP01-23

Treatment of myelofibrosis

Day 30 discussion

Oncology

3.1.59. Relatlimab / nivolumab - EMEA-002727-PIP02-23

Treatment of all conditions included in the category of malignant neoplasms (except nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms and melanoma)

Day 30 discussion

Oncology

3.1.60. Relatlimab / nivolumab - EMEA-002727-PIP03-23

Treatment of melanoma

Day 30 discussion

Oncology

3.1.61. EMEA-003469-PIP01-23

Treatment of lung cancer

Day 30 discussion

Other

3.1.62. Messenger RNA encoding Cas9, single guide RNA targeting the human KLKB1 gene - EMEA-003465-PIP01-23

Treatment of hereditary angioedema (HAE)

Day 30 discussion

Other

3.1.63. Imlifidase - Orphan - EMEA-002183-PIP03-23

Hansa Biopharma AB; Treatment of anti–glomerular basement membrane (anti-GBM) disease

Day 30 discussion

Other / Uro-nephrology

3.1.64. Naproxen / paracetamol - EMEA-003475-PIP01-23

Treatment of rheumatoid arthritis / Treatment of febrile disorders / Treatment of acute pain

Day 30 discussion

Pain

3.1.65. Humanised IgG1 monoclonal antibody against pituitary adenylate cyclase-activating polypeptide - EMEA-003483-PIP01-23

Prevention of migraine

Day 30 discussion

Pain / Neurology

3.1.66. Atacicept - EMEA-002004-PIP04-23

Treatment immunoglobulin A nephropathy

Day 30 discussion

Uro-nephrology

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. Macitentan - EMEA-C3-001032-PIP01-10-M05

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.2.2. Nirsevimab - EMEA-C-001784-PIP01-15-M04

AstraZeneca AB; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 30 discussion

Infectious Diseases

3.2.3. Ataluren - EMEA-C-000115-PIP01-07-M13

PTC Therapeutics International, Limited; Treatment of dystrophinopathy

Day 30 discussion

Neurology

3.2.4. Lenvatinib - EMEA-C-001119-PIP03-19-M03

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

Day 30 discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Macitentan - Orphan - EMEA-001032-PIP01-10-M07

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

Day 30 discussion

Cardiovascular Diseases

3.3.2. Treprostinil sodium - EMEA-003182-PIP01-22-M01

AOP Orphan Pharmaceuticals GmbH; Treatment of pulmonary arterial hypertension (PAH) group 1 / Treatment of pulmonary arterial hypertension

Day 30 discussion

Cardiovascular Diseases

3.3.3. Abrocitinib - EMEA-002312-PIP01-17-M02

Pfizer Europe MA EEIG; Treatment of moderate to severe atopic dermatitis

Day 30 discussion

Dermatology

3.3.4. Remibrutinib - EMEA-002582-PIP01-19-M03

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

Dermatology

3.3.5. Liraglutide - EMEA-000128-PIP02-09-M05

Novo Nordisk A/S; Treatment of obesity

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Pegzilarginase - Orphan - EMEA-001925-PIP02-19-M01

Immedica Pharma AB; Treatment of hyperargininaemia

Day 30 discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Maralixibat chloride - Orphan - EMEA-001475-PIP03-17-M04

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 discussion

Gastroenterology-Hepatology

3.3.8. Denecimig - EMEA-002762-PIP02-20-M02

Novo Nordisk A/S; Treatment of haemophilia A

Day 30 discussion

Haematology-Hemostaseology

3.3.9. Human fibrinogen concentrate - EMEA-001931-PIP01-16-M03

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 30 discussion

Haematology-Hemostaseology

3.3.10. Sebetralstat - Orphan - EMEA-002723-PIP01-19-M02

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 30 discussion

Haematology-Haemostaseology

3.3.11. Risankizumab - EMEA-001776-PIP02-17-M02

AbbVie Ltd; Treatment of chronic idiopathic arthritis

Day 30 discussion

Immunology-Rheumatology-Transplantation

3.3.12. Upadacitinib - EMEA-001741-PIP03-16-M04

AbbVie Ltd; Treatment of Crohn's disease

Day 30 discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.3.13. Bemnifosbuvir - EMEA-002963-PIP01-21-M01

Atea Pharmaceuticals, Inc; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.14. Cilgavimab (AZD1061) - EMEA-002925-PIP01-20-M02

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

Day 30 discussion

Infectious Diseases

3.3.15. Dalbavancin hydrochloride - EMEA-000016-PIP01-07-M09

AbbVie Ltd; Treatment of acute bacterial skin and skin structure infections (ABSSSI)

Day 30 discussion

Infectious Diseases

3.3.16. Letermovir - Orphan - EMEA-001631-PIP01-14-M05

Merck Sharp & Dohme (Europe), Inc.; Prevention of cytomegalovirus infection

Day 30 discussion

Infectious Diseases

3.3.17. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of gram-negative bacterial infections

Day 30 discussion

Infectious Diseases

3.3.18. Ritonavir / nirmatrelvir - EMEA-003081-PIP01-21-M03

Pfizer Europe MA EEIG; Treatment of coronavirus disease 2019 (COVID-19) / Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Infectious Diseases

3.3.19. Tixagevimab (AZD8895) - EMEA-002900-PIP01-20-M02

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

Day 30 discussion

Infectious Diseases

3.3.20. Zanamivir - EMEA-001318-PIP01-12-M05

GlaxoSmithKline Trading Services Limited; Treatment of influenza / Prevention of influenza

Day 30 discussion

Infectious Diseases

3.3.21. Alprazolam - EMEA-003043-PIP01-21-M01

UCB Pharma SA.; Treatment of epileptic seizures

Day 30 discussion

Neurology

3.3.22. Ravulizumab - EMEA-001943-PIP04-20-M01

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

Neurology

3.3.23. Satralizumab - Orphan - EMEA-001625-PIP02-21-M02

Roche Registration GmbH; Treatment of generalised myasthenia gravis

Day 30 discussion

Neurology

3.3.24. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M04

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

Day 30 discussion

Oncology

3.3.25. Lisocabtagene maraleucel - EMEA-001995-PIP01-16-M04

Bristol-Myers Squibb Pharma EEIG; Treatment of B-lymphoblastic leukaemia/lymphoma / Treatment of mature B-cell neoplasms

Day 30 discussion

Oncology

3.3.26. Lutetium (^{177}Lu) oxodotreotide - Orphan - EMEA-002950-PIP01-20-M01

Advanced Accelerator Applications; Treatment of gastroenteropancreatic neuroendocrine tumours

Day 30 discussion

Oncology

3.3.27. Tirzepatide - EMEA-002360-PIP02-22-M02

Eli Lilly and Company; Treatment of obesity

Day 30 discussion

Other

3.3.28. Tapentadol (hydrochloride) - EMEA-000325-PIP01-08-M11

Grünenthal GmbH; Treatment of chronic pain

Day 30 discussion

Pain

3.3.29. Mometasone (furoate) / glycopyrronium bromide / indacaterol - EMEA-001812-PIP01-15-M02

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

Pneumology - Allergology

3.3.30. Seltorexant - EMEA-002746-PIP01-20-M02

Janssen-Cilag International NV; Treatment of major depressive disorder

Day 30 discussion

Psychiatry

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 11 September 2023 for Nomination of Rapporteur and Peer reviewer

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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

No item

4.3. Nominations for other activities

Summary of Committee discussion:

The PDCO approved the lists of Rapporteurs and Peer Reviewers.

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.

5.1. New Scientific Advice

5.2. Final Scientific Advice (Reports and Scientific Advice letters)

No item

6. Discussion on the applicability of class waivers

6.1. Discussions on the applicability of class waiver for products

No item

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

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

7.1.1. Setmelanotide - Orphan - EMEA-002209-PIP01-17-M03

Rhythm Pharmaceuticals, Inc; Treatment of appetite and general nutrition disorders

Proposed indication: Treatment of hypothalamic obesity

Summary of Committee discussion:

The PDCO was of the view that the proposed indication 'treatment of hypothalamic obesity' falls under the scope of the Decision, as the indication is considered to be covered by the condition 'treatment of appetite and general nutrition disorders' listed in the Agency Decision.

7.1.2. Finerenone - EMEA-001623-PIP01-14-M06

Bayer AG; Treatment of chronic kidney disease

Proposed indication: Treatment of chronic kidney disease in adults with type 1 diabetes

Summary of Committee discussion:

The PDCO was of the view that the proposed indication "treatment of chronic kidney disease in adults with type 1 diabetes" falls under the scope of the Decision, as the indication is considered to be covered by the condition "treatment of chronic kidney disease" listed in the Agency Decision.

8. Annual reports on deferrals

The members of the PDCO took note of the products listed in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

The Chair welcomed Pavlina Chladova as the new alternate for Czech Republic.

The Chair thanked Milena Stevanovic as an alternate representing patients' organisations, Fabio Midulla as an alternate representing healthcare professionals, Tomas Boran as a member for Czech Republic and Dovile Zacharkiene as a member for Lithuania for their

contribution.

The Chair announced that Tereza Bazantova is the new member for Czech Republic, replacing Tomas Boran.

The following members/alternates have been appointed by the European Commission to the PDCO to represent healthcare professionals for a 3-year term from 01 August 2023:

- Member: Francesca Rocchi
- Alternate: Jose Ignacio Malagon
- Member: Fernando Cabanas
- Alternate: Doina Plesca
- Alternate: Johannes Taminiau

The following members/alternates have been appointed by the European Commission to the PDCO to represent patients' organisations for a 3-year term from 01 August 2023:

- Member: Tomasz Grybek
- Alternate: Jaroslav Sterba
- Member: Viviana Giannuzzi
- Alternate: Patricia Felgueiras Seabra Durao

9.1.2. Vote by Proxy

None

9.1.3. Strategic Review and Learning Meeting (SRLM) - Madrid, Spain 17-18 October 2023

PDCO member: Fernando de Andrés Trelles, Maria Jesús Fernández Cortizo

Summary of Committee discussion:

The Committee was updated about the next strategic review and learning meeting in Madrid.

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Summary of Committee discussion:

Feedback on the ongoing CHMP procedures was provided to the Committee by the nominated PDCO experts.

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

Summary of Committee discussion:

The Vice-Chair of the Non-clinical Working Party (NcWP) identified the products which will require NcWP evaluation and discussion.

9.3.2. Formulation Working Group

PDCO member: Brian Aylward (*ad interim*)

Summary of Committee discussion:

The Chair of the Formulation Working Group (FWG) identified the products which will require FWG evaluation and discussion.

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

Summary of Committee discussion:

The Chair informed the Committee of the upcoming nomination of a second representative for PCWP/HCPWP following the end of membership of Dimitrios Athanasiou.

In addition, the Draft Agenda - PCWP-HCPWP Joint meeting - 19 & 20 September 2023 and the Meeting Summary - PCWP HCPWP 27 and 28 June 2023 were shared for information.

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Summary of Committee discussion:

The three ITF briefing meeting planned for September were presented.

9.3.5. SAWP cases for PDCO

Scientific Advice cases with PDCO involvement in network portal

Summary of Committee discussion:

A demo was given to show how SAWP cases for PDCO view are now available in the IRIS network platform.

9.4. Cooperation within the EU regulatory network

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

Summary of Committee discussion:

The Committee was informed about the annual meeting and workshop of Enpr-EMA, which will take place on 09 and 10 October 2023.

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Summary of Committee discussion:

The Ad Hoc September 2023 paediatric agenda and minutes of the cluster were shared with the PDCO members 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

9.7.1. PDCO Work Plan 2023 - reporting on progress

PDCO Chair: Brian Aylward

Summary of Committee discussion:

PDCO members reported on the progress on topics included in the PDCO workplan.

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q3/2023 Update of the Business Pipeline report for the human scientific committees

Summary of Committee discussion:

The business pipeline report for Q3/2023 was provided for information.

10. Any other business

10.1. ACT EU priority action (PA) 8 on methodology

Summary of Committee discussion:

The Committee was informed about ACT EU (Accelerating Clinical Trials in the EU) Priority Action 8 on methodology guidance. Members were asked to express their interest in contributing to a methodology workshop planned for 22-23 November 2023.

10.2. Request for feedback on recommendation for inclusion of adolescents in adult trials from the Clinical Trials Coordination Group (CTCG)

PDCO member: Anette Solli Karlsen

Summary of Committee discussion:

PDCO discussed in more detail the written feedback to CTCG on the issue of inclusion of adolescents in adult studies.

11. Breakout sessions

11.1. Paediatric oncology

Summary of Committee discussion:

The group discussed ongoing paediatric procedures.

11.2. Neonatology

Summary of Committee discussion:

The group discussed topics for revision of the neonatal guideline.

The Chair thanked all participants and closed the meeting.

12. List of participants

List of participants including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 05-08 September 2023 PDCO meeting, which was held in-person.

An asterisk (*) after the role, in the second column, signals that the participant attended remotely.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Brian Aylward	Chair	Ireland	No interests declared	
Johanna Wernsperger	Member*	Austria	No interests declared	
Karen Van Malderen	Alternate	Belgium	No interests declared	
Dimitar Roussinov	Member	Bulgaria	No restrictions applicable to this meeting	
Zena Gunther	Member	Cyprus	No interests declared	
Tereza Bazantova	Member	Czechia	No interests declared	
Pavlina Chladová	Alternate*	Czechia	No interests declared	
Louisa Braun Exner	Member	Denmark	No interests declared	
Jana Lass	Alternate*	Estonia	No interests declared	
Pauliina Lehtolainen-Dalkilic	Member*	Finland	No interests declared	
Anne Paavola	Alternate*	Finland	No interests declared	
Sylvie Benchetrit	Member (Vice-Chair)	France	No interests declared	
Dominique Ploin	Alternate	France	No interests declared	
Sabine Scherer	Member	Germany	No interests declared	
Yuansheng Sun	Alternate	Germany	No interests declared	
Eleni Katsomiti	Member	Greece	No interests declared	
Anastasia Mountaki	Alternate*	Greece	No interests declared	
Adrienn Horváth	Member*	Hungary	No interests declared	
Sara Galluzzo	Member	Italy	No interests declared	
Cinzia Ciceroni	Alternate*	Italy	No interests declared	
Dina Apele-Freimane	Member	Latvia	No participation in discussion, final deliberations and voting on:	3.2.4. Nirsevimab - EMEA-C-001784-PIP01-15-M04
Carola de Beaufort	Member	Luxembourg	No restrictions applicable to this meeting	
Olivier Moes	Alternate*	Luxembourg	No interests declared	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
John-Joseph Borg	Member	Malta	No interests declared	
Herbert Lenicker	Alternate*	Malta	No interests declared	
Roderick Houwen	Member	Netherlands	No restrictions applicable to this meeting	
Maaike Van Dartel	Alternate	Netherlands	No interests declared	
Siri Wang	Member	Norway	No interests declared	
Anette Solli Karlsen	Alternate	Norway	No interests declared	
Marek Migdal	Member	Poland	No restrictions applicable to this meeting	
Helena Fonseca	Member	Portugal	No interests declared	
Hugo Tavares	Alternate	Portugal	No interests declared	
Dana Gabriela Marin	Member (CHMP alternate)	Romania	No interests declared	
Peter Sisovsky	Member	Slovakia	No interests declared	
Peter Szitanyi	Alternate	Slovakia	No interests declared	
Stefan Grosek	Member	Slovenia	No interests declared	
Fernando de Andrés Trelles	Member*	Spain	No interests declared	
Maria Jesus Fernández Cortizo	Alternate	Spain	No interests declared	
Sara Vennberg	Member	Sweden	No interests declared	
David Khan	Alternate	Sweden	No restrictions applicable to this meeting	
Johannes Taminiau	Alternate	Healthcare Professionals' Representative	No interests declared	
Fernando Cabanas	Member	Healthcare Professionals' Representative	No participation in final deliberations and voting on:	3.2.4. Nirsevimab - EMEA-C-001784-PIP01-15-M04
Francesca Rocchi	Member	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Jose Ignacio Malagon Calle	Alternate	Healthcare Professionals' Representative	No restrictions applicable to this meeting	
Tomasz Grybek	Member	Patients' Organisation Representative	No interests declared	
Jaroslav Sterba	Alternate	Patients' Organisation Representative	No restrictions applicable to this meeting	
Viviana	Member	Patients'	No restrictions	

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Giannuzzi		Organisation Representative	applicable to this meeting	
Patricia Felgueiras Seabra Durao	Alternate*	Patients' Organisation Representative	No restrictions applicable to this meeting	
Celine Chu	Expert*	France	No interests declared	
María Estela Moreno Martín	Expert*	Spain	No interests declared	
Olga Kholmanskikh	Expert*	Belgium	No interests declared	
Mette Linnert Jensen	Expert*	Denmark	No interests declared	
Ole Weis Bjerrum	Expert*	Denmark	No interests declared	
Meeting run with support from relevant EMA staff				

Experts were evaluated against the agenda topics or activities they participated in.

13. 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/