



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 18-21 July 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

18 July 2023, 14:00- 19:00, Virtual meeting

19 July 2023, 08:30- 19:00, Virtual meeting

20 July 2023, 08:30- 19:00, Virtual meeting

21 July 2023, 08:30- 13:00, Virtual meeting

Disclaimers

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

Note on access to documents

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



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1. Introductions

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

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 18-21 July 2023. See July 2023 PDCO minutes (to be published post September 2023 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 18-21 July 2023.

1.3. Adoption of the minutes

PDCO minutes for 20-23 June 2023.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Dexmedetomidine - EMEA-003283-PIP01-22

Sedation

Day 120 opinion

Action: For adoption

Anaesthesiology

2.1.2. Isotretinoin - Orphan - EMEA-003303-PIP01-22

Timber Pharmaceuticals LLC; Treatment of congenital ichthyosis

Day 120 opinion

Action: For adoption

Dermatology

Note: withdrawal request received on 14 July 2023

2.1.3. Upadacitinib - EMEA-001741-PIP07-22

Treatment of vitiligo

Day 120 opinion

Action: For adoption

Dermatology

2.1.4. Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 - Orphan - EMEA-003253-PIP01-22

Sanofi B.V.; Treatment of achondroplasia

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.5. Recombinant human tissue nonspecific alkaline phosphatase (TNSALP) fragment crystallizable (Fc) deca aspartate fusion protein - EMEA-003343-PIP01-22

Treatment of hypophosphatasia

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.6. Govorestat - Orphan - EMEA-003365-PIP01-22

Applied Therapeutics, Inc; Treatment of galactosaemia

Day 120 opinion

Action: For adoption. Oral explanation to be held on Wednesday, 19 July 2023 at 14:00

Endocrinology-Gynaecology-Fertility-Metabolism / Ophthalmology / Neurology

2.1.7. EMEA-003002-PIP03-22

Treatment of portal hypertension

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.8. Obinutuzumab - Orphan - EMEA-001207-PIP05-22

Roche Registration GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.9. EMEA-003326-PIP01-22

Treatment of infections caused by gram-negative organisms / Complicated urinary tract infections (cUTI) / Hospital associated pneumonia or ventilator associated pneumonia

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.10. Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP02-22

Treatment of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Neonatology - Paediatric Intensive Care / Infectious Diseases

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

Calico Life Sciences LLC; Treatment of vanishing white matter disease

Day 120 opinion

Action: For adoption

Neurology

2.1.12. Vesleteplirsen - EMEA-003305-PIP01-22

Treatment of Duchenne muscular dystrophy

Day 120 opinion

Action: For adoption

Neurology

2.1.13. Retatrutide - EMEA-003258-PIP01-22

Treatment of obesity

Day 120 opinion

Action: For adoption

Other

Note: Withdrawal request received on 11 July 2023

2.1.14. [An acellular tubular graft composed of human collagen types I and III and other extracellular matrix proteins, including fibronectin and vitronectin - EMEA-003345-PIP01-22](#)

Treatment of vascular injuries

Day 120 opinion

Action: For adoption

Other

2.1.15. [Dexpramipexole - EMEA-003328-PIP01-22](#)

Treatment of asthma

Day 120 opinion

Action: For adoption. Oral explanation to be held on Thursday, 20 July 2023 at 14:00

Pneumology - Allergology

2.1.16. [EMEA-003319-PIP02-22](#)

Treatment of major depressive disorder (MDD)

Day 120 opinion

Action: For adoption

Psychiatry

2.1.17. [EMEA-003319-PIP03-22](#)

Treatment of post-traumatic stress disorder (PTSD)

Day 120 opinion

Action: For adoption

Psychiatry

2.1.18. [Obicetrapib - EMEA-003438-PIP01-23](#)

Treatment of mixed dyslipidaemia

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.19. Ramipril / nebivolol - EMEA-003430-PIP01-23

Treatment of hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.20. Pemafibrate - EMEA-001573-PIP03-23

Treatment of primary biliary cholangitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.21. Abemaciclib - EMEA-002342-PIP05-23

Treatment of prostate cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.22. Binimetinib - EMEA-001454-PIP06-23

Treatment of lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.23. Encorafenib - EMEA-001588-PIP04-23

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.24. Enfortomab vedotin - EMEA-002299-PIP02-23

Treatment of head and neck epithelial malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.25. Ivonescimab - EMEA-003378-PIP01-23

Treatment of lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.26. Lerociclib - EMEA-003436-PIP01-23

Treatment of endometrial cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.27. Modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens - EMEA-003434-PIP01-23

Treatment of melanoma / Treatment of lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.28. Allogeneic faecal microbiota, pooled - EMEA-003435-PIP01-23

Treatment in allogeneic haematopoietic stem cell transplantation

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.1.29. Clotrimazole - EMEA-003431-PIP01-23

Treatment of fungal otitis externa (otomycosis)

Day 60 opinion

Action: For adoption

Oto-rhino-laryngology

2.1.30. EMEA-002990-PIP02-23

Treatment of major depressive disorder

Day 60 opinion

Action: For adoption

Psychiatry

2.1.31. Ravulizumab - EMEA-001943-PIP06-23

Prevention of death, kidney replacement therapy, or sustained reduction in kidney function in high-risk adult patients with stage 3 or 4 chronic kidney disease undergoing cardiopulmonary bypass for coronary artery bypass graft, valve replacement or repair, or combined procedure

Day 60 opinion

Action: For adoption

Uro-nephrology

2.1.32. Ezetimibe / rosuvastatin - EMEA-003444-PIP01-23

Prevention of cardiovascular events in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS)

Day 30 opinion

Action: For adoption

Cardiovascular Diseases

2.1.33. Concentrate of proteolytic enzymes enriched in bromelain - Orphan - EMEA-000142-PIP03-23

MediWound Germany GmbH; Treatment of venous leg ulcers (VLUs) and diabetic foot ulcers (DFU)

Day 30 opinion

Action: For adoption

Other

2.1.34. EMEA-003447-PIP01-23

Treatment of malignant prostatic neoplasms / Treatment of malignant breast neoplasms

Day 30 opinion

Action: For adoption

Oncology

2.1.35. Teprotumumab - EMEA-001973-PIP02-23

Treatment of thyroid eye disease

Day 30 opinion

Action: For adoption

Ophthalmology

2.2. Opinions on Compliance Check

2.2.1. Birch bark extract - EMEA-C-001299-PIP03-17-M02

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 30 opinion

Action: For adoption

Dermatology

2.2.2. Saxagliptin - EMEA-C-000200-PIP01-08-M10

AstraZeneca AB; Treatment of type 2 diabetes

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.3. Dupilumab - EMEA-C-001501-PIP04-19-M02

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.2.4. Ex vivo expanded autologous human corneal epithelial cells containing stem cells - EMEA-C-001082-PIP02-11-M03

Holostem Terapie Avanzate S.r.l.; Treatment of limbal stem cell deficiency due to ocular burns

Day 60 opinion

Action: For adoption

Ophthalmology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Apixaban - EMEA-000183-PIP02-12-M04

Bristol-Myers Squibb / Pfizer EEIG; Treatment of venous thromboembolism

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19-M01

CUTISS AG; Treatment of burns

Day 60 opinion

Action: For adoption

Dermatology

2.3.3. Bimekizumab - EMEA-002189-PIP01-17-M04

UCB Biopharma SRL; Treatment of psoriasis

Day 60 opinion

Action: For adoption

Dermatology

2.3.4. Norucholic acid - Orphan - EMEA-002485-PIP01-18-M01

Dr. Falk Pharma GmbH; Treatment of autoimmune sclerosing cholangitis / Treatment of primary sclerosing cholangitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.5. Risankizumab - EMEA-001776-PIP03-17-M01

AbbVie Ltd; Treatment of Crohn's disease

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.6. Ustekinumab - EMEA-000311-PIP04-13-M06

Janssen-Cilag International NV; Treatment of Crohn's disease

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.7. Concizumab - Orphan - EMEA-002326-PIP04-20-M01

Novo Nordisk A/S; Treatment of congenital haemophilia A and B

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.8. Danicopan - Orphan - EMEA-002310-PIP01-17-M01

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.9. Etranacogene dezaparvovec - Orphan - EMEA-002722-PIP01-19-M03

CSL Behring GmbH; Treatment of haemophilia B

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.10. Ravulizumab (ALXN1210) - Orphan - EMEA-002077-PIP01-16-M06

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.11. Avacopan - Orphan - EMEA-002023-PIP01-16-M07

ChemoCentryx Ireland Ltd.; Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.12. Guselkumab - EMEA-001523-PIP03-18-M03

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.13. Baloxavir marboxil - EMEA-002440-PIP01-18-M05

Roche Registration GmbH; Treatment of influenza / Prevention of influenza

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Fostemsavir (tromethamine) - EMEA-001687-PIP01-14-M07

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Tedizolid phosphate - EMEA-001379-PIP01-12-M08

Merck Sharp & Dohme (Europe), Inc.; Treatment of acute bacterial skin and skin structure infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Tenofovir alafenamide - EMEA-001584-PIP01-13-M07

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Fremanezumab - EMEA-001877-PIP01-15-M04

Teva GmbH; Prevention of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.18. Rozanolixizumab - Orphan - EMEA-002681-PIP01-19-M02

UCB Pharma S.A; Treatment of myasthenia gravis

Day 60 opinion

Action: For adoption

Neurology

2.3.19. Bosutinib - EMEA-000727-PIP01-09-M07

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.20. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M03

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.21. Nivolumab - EMEA-001407-PIP02-15-M06

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of the central nervous system / Treatment of malignant neoplasms of lymphoid tissue

Day 60 opinion

Action: For adoption

Oncology

2.3.22. [Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 - Orphan - EMEA-003232-PIP01-22-M01](#)

Inozyme Pharma Ireland Limited; Treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency

Day 60 opinion

Action: For adoption

Other / Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

2.3.23. [Ravulizumab \(ALXN1210\) - Orphan - EMEA-001943-PIP01-16-M08](#)

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.24. [Davesomeran / imelasomeran / elasomeran - EMEA-002893-PIP01-20-M04](#)

Moderna Biotech Spain, S.L.; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Action: For adoption

Vaccines

2.3.25. [Repotrectinib - EMEA-002635-PIP02-21-M02](#)

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

Day 30 opinion

Action: For adoption

Oncology

2.3.26. [Semaglutide - EMEA-001441-PIP07-21-M01](#)

Novo Nordisk A/S; Treatment of obesity

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.27. [Guselkumab - EMEA-001523-PIP02-14-M03](#)

Janssen-Cilag International NV; Treatment of psoriasis

Day 30 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.28. BI 425809 - EMEA-002653-PIP01-19-M01

Boehringer Ingelheim International GmbH; Treatment of schizophrenia

Day 30 opinion

Action: For adoption

Psychiatry

2.3.29. Botaretigene sparoparvovec - Orphan - EMEA-002827-PIP01-20-M02

Janssen-Cilag International NV; Treatment of retinitis pigmentosa

Day 30 opinion

Action: For adoption

Ophthalmology

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. Erenumab - EMEA-C3-001664-PIP02-15-M05

Novartis Europharm Limited; Prevention of migraine headaches

Day 30 letter

Action: For information

Pain / Neurology

2.7.2. Odronextamab - EMEA-C1-003149-PIP01-21-M01

Regeneron Ireland DAC; Treatment of mature B-cell malignancies

Day 30 letter

Action: For information

Oncology

2.7.3. Iptacopan - EMEA-C1-002705-PIP01-19-M01

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Day 30 letter

Action: For information

Other / Uro-nephrology

2.7.4. Brivaracetam - EMEA-C1-000332-PIP02-17-M04

UCB Pharma S.A.; Treatment of neonatal seizures

Day 30 letter

Action: For information

Neurology

2.7.5. Cariprazine hydrochloride - EMEA-C1-001652-PIP01-14-M05

Gedeon Richter Plc.; Treatment of schizophrenia

Day 30 letter

Action: For information

Psychiatry

2.7.6. Marstacimab - EMEA-C2-002285-PIP02-19-M02

Pfizer Europe MA EEIG; Treatment of congenital haemophilia A / Treatment of congenital haemophilia B

Day 30 letter

Action: For information

Haematology-Hemostaseology

2.7.7. Garadacimab - EMEA-C1-002726-PIP01-19-M03

CSL Behring GmbH; Prevention of hereditary angioedema attacks

Day 30 letter

Action: For information

Immunology-Rheumatology-Transplantation

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

Ultragenyx Germany GmbH; Treatment of ornithine transcarbamylase deficiency

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. [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 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. [Fazirsiran - Orphan - EMEA-003355-PIP01-22](#)

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

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.4. [Inhibitor of receptor-interacting protein kinase 1 - EMEA-003356-PIP01-22](#)

Treatment of ulcerative colitis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.5. Tezepelumab - EMEA-001613-PIP03-21

Treatment of eosinophilic esophagitis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.6. EMEA-003090-PIP02-22

Treatment of hereditary angioedema

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.7. Ciraparantag - EMEA-003321-PIP01-22

Treatment of FXa inhibitor-associated haemorrhage / Prevention of FXa inhibitor-associated haemorrhage

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

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

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

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.9. Enpatoran - EMEA-003342-PIP02-22

Treatment of systemic lupus erythematosus

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.10. 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 - Orphan - EMEA-003025-PIP03-23

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

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Oncology / Haematology-Hemostaseology

3.1.11. EMEA-003192-PIP01-22

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.12. Ruzotolimod - EMEA-003363-PIP01-22

Treatment of chronic hepatitis B

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.13. Xalnesiran - EMEA-003362-PIP01-22

Treatment of chronic hepatitis B

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.14. Rozanolixizumab - EMEA-002681-PIP03-21

Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 90 discussion

Action: For discussion

Neurology

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

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

Day 90 discussion

Action: For discussion

Oncology

3.1.16. Pembrolizumab / vibostolimab - EMEA-003063-PIP03-22

Treatment of melanoma

Day 90 discussion

Action: For discussion

Oncology

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

GlycoMimetics, Inc.; Treatment of acute myeloid leukaemia

Day 90 discussion

Action: For discussion

Oncology

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

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

Day 90 discussion

Action: For discussion

Other

3.1.19. EMEA-003347-PIP01-22

Treatment of glomerulonephritis and nephrotic syndrome

Day 90 discussion

Action: For discussion

Uro-nephrology

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

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

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.21. 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* / recombinant *Neisseria meningitidis* group B fHbp 2-3-1.13NB fusion protein / recombinant *Neisseria meningitidis* group B protein 961c / recombinant *Neisseria meningitidis* group B protein 287-953 / recombinant *Neisseria meningitidis* group B protein 936-741 - EMEA-003359-PIP01-22

Prevention of meningococcal disease

Day 90 discussion

Action: For discussion

Vaccines

3.1.22. *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

Prevention of meningococcal disease

Day 90 discussion

Action: For discussion

Vaccines

3.1.23. Obicetrapib - EMEA-003438-PIP02-23

Treatment of elevated cholesterol

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.24. [Modified mRNA encoding human methylmalonyl-coenzyme A mutase containing a polymorphism at position 671 - Orphan - EMEA-003437-PIP01-23](#)

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

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. [Navepegritide - Orphan - EMEA-002689-PIP02-23](#)

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.26. [Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-003420-PIP01-23](#)

Arrowhead Pharmaceuticals, Inc.; Treatment of familial chylomicronemia syndrome (FCS)

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.27. [Dupilumab - EMEA-001501-PIP12-23](#)

Treatment of eosinophilic gastroenteritis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.28. [Tarperprumig - EMEA-003432-PIP01-23](#)

Treatment of patients with sickle cell disease (SCD)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.29. Amiodarone - EMEA-003428-PIP01-23

Treatment of supraventricular tachyarrhythmias in children from birth to less than 18 years of age

Day 60 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

3.1.30. Ganaxolone - Orphan - EMEA-002341-PIP02-23

Marinus Pharmaceuticals Inc.; Treatment of tuberous sclerosis complex

Day 60 discussion

Action: For discussion

Neurology

3.1.31. Tralesinidase alfa - Orphan - EMEA-003433-PIP01-23

Labcorp Clinical Development Limited; Treatment of mucopolysaccharidosis type III B (Sanfilippo B syndrome)

Day 60 discussion

Action: For discussion

Neurology

3.1.32. Alpelisib - Orphan - EMEA-002016-PIP05-23

Novartis Europharm Limited; Treatment of lymphatic malformations associated with a PIK3CA mutation

Day 60 discussion

Action: For discussion

Other

3.1.33. Ferric citrate coordination complex (FCCC) - EMEA-001213-PIP03-23

Treatment of anaemias due to chronic kidney disorders

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.34. Amlodipine / rosuvastatin - EMEA-003446-PIP01-23

Treatment of angina and dyslipidaemia / Treatment of essential hypertension in patients who are estimated to have a high risk for a first cardiovascular event / Treatment of concomitant hypertension and dyslipidaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

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

Treatment of myocardial infarction

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

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

Treatment of hypercholesterolemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.37. Upadacitinib - EMEA-001741-PIP10-23

Treatment of alopecia areata

Day 30 discussion

Action: For discussion

Dermatology

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

Treatment of obesity

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.39. Plecanatide - EMEA-003441-PIP01-23

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.40. EMEA-003456-PIP01-23

Treatment of myelofibrosis

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.41. Hydroxycarbamide - EMEA-003388-PIP01-23

Prevention of sickle cell disease crises

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.42. Ianalumab - EMEA-002338-PIP04-23

Treatment of autoimmune haemolytic anaemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.43. 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 30 discussion

Action: For discussion

Infectious Diseases

3.1.44. Ganaxolone - EMEA-002341-PIP03-23

Treatment of refractory status epilepticus

Day 30 discussion

Action: For discussion

Neurology

3.1.45. [Recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA - Orphan - EMEA-003459-PIP01-23](#)

Myrtelle,Inc.; Treatment of Canavan disease

Day 30 discussion

Action: For discussion

Neurology

3.1.46. [EMEA-003455-PIP01-23](#)

Treatment of lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.47. [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 30 discussion

Action: For discussion

Oncology

3.1.48. [Belrestotug - EMEA-003452-PIP01-23](#)

Treatment of lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.49. [Bezuclastinib - EMEA-003445-PIP01-23](#)

Treatment of mastocytosis

Day 30 discussion

Action: For discussion

Oncology

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

Treatment of HER2 expressing tumours / Treatment of solid tumours

Day 30 discussion

Action: For discussion

Oncology

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

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 30 discussion

Action: For discussion

Oncology

3.1.52. 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 30 discussion

Action: For discussion

Oncology

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

Autolus GmbH; Treatment of non-Hodgkin lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.54. Tiragolumab - EMEA-002721-PIP04-23

Treatment of hepatocellular carcinoma

Day 30 discussion

Action: For discussion

Oncology

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

PTC Therapeutics International; Treatment of soft tissue sarcoma

Day 30 discussion

Action: For discussion

Oncology

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

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

Day 30 discussion

Action: For discussion

Oncology

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

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

Day 30 discussion

Action: For discussion

Oncology

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

MaaT Pharma; Treatment of graft-versus-host disease

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.59. Clobetasol - EMEA-003458-PIP01-23

Treatment of inflammation and pain associated with ocular surgery

Day 30 discussion

Action: For discussion

Ophthalmology

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

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

Day 30 discussion

Action: For discussion

Ophthalmology

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

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

Day 30 discussion

Action: For discussion

Other

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

Fulcrum Therapeutics, Inc.; Treatment of facioscapulohumeral muscular dystrophy

Day 30 discussion

Action: For discussion

Other

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

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

Day 30 discussion

Action: For discussion

Other

3.1.64. Sisunatovir - EMEA-002529-PIP02-23

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

Day 30 discussion

Action: For discussion

Other

3.1.65. 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 30 discussion

Action: For discussion

Other / Uro-nephrology

3.1.66. [Mometasone - EMEA-003454-PIP01-23](#)

Treatment of chronic rhinosinusitis (CRS)

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.67. [EMEA-003451-PIP01-23](#)

Treatment of bronchiectasis

Day 30 discussion

Action: For discussion

Pneumology – Allergology

3.1.68. [Borrelia outer surface protein A \(OspA\) serotypes \(ST1-6\) lipidated, fusion protein vaccine - EMEA-003130-PIP02-23](#)

Prevention of Lyme disease

Day 30 discussion

Action: For discussion

Vaccines

3.1.69. [Pertussis filamentous haemagglutinin \(FHA\) / Genetically detoxified pertussis toxin \(PTgen\) - EMEA-003442-PIP01-23](#)

Prevention of pertussis disease

Day 30 discussion

Action: For discussion

Vaccines

3.2. **Discussions on Compliance Check**

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

3.2.1. [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 30 discussion

Action: For discussion

Infectious Diseases

3.2.2. [Cabotegravir / cabotegravir sodium - EMEA-C1-001418-PIP01-13-M05](#)

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.3. [Isavuconazonium \(sulfate\) - EMEA-C-001301-PIP02-12-M04](#)

Basilea Pharmaceutica Deutschland GmbH; Treatment of invasive aspergillosis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.4. [Leriglitzone - EMEA-C-002106-PIP01-16-M02](#)

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 30 discussion

Action: For discussion

Neurology

3.2.5. [Molgramostim - EMEA-C1-002282-PIP01-17-M01](#)

Savara ApS; Treatment of pulmonary alveolar proteinosis

Day 30 discussion

Action: For discussion

Pneumology – Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

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

Sanofi Winthrop Industrie; Treatment of asthma

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Vedolizumab - EMEA-000645-PIP04-20-M02

Takeda Pharma A/S; Treatment of pouchitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.3. Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-001855-PIP01-15-M05

Genzyme Europe B.V.; Treatment of haemophilia A / Treatment of haemophilia B

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

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

Baxalta Innovations GmbH; Treatment of Von Willebrand disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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

PAION Deutschland GmbH; Treatment of complicated intra-abdominal infection

Day 30 discussion

Action: For discussion

Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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

H. Lundbeck A/S; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

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

Janssen-Cilag International NV; Treatment of myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

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

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

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

Biogen Idec Ltd; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.12. Carfilzomib - Orphan - EMEA-001806-PIP04-19-M02

Amgen Europe B.V; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.13. 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 30 discussion

Action: For discussion

Oncology

3.3.14. Repotrectinib - EMEA-002635-PIP02-21-M02

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

Day 30 discussion

Action: For discussion

Oncology

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

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

Day 30 discussion

Action: For discussion

Oncology

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

Novartis Europharm Limited; Treatment of IgA nephropathy

Day 30 discussion

Action: For discussion

Other

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

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

Action: For discussion

Other

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

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

Day 30 discussion

Action: For discussion

Other

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

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Other

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

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

Day 30 discussion

Action: For discussion

Psychiatry

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

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

Day 30 discussion

Action: For discussion

Vaccines

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

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

Day 30 discussion

Action: For discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 14 August 2023 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

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

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

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

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

6. Discussion on the applicability of class waivers

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

6.1. Discussions on the applicability of class waiver for products

6.1.1. EMEA-07-2023

Boehringer Ingelheim; The class of pyrimidine- and pyrimidine analogue-containing medicinal products for treatment of lung malignant neoplasms / Treatment of non-small cell lung cancer

Action: For adoption

6.1.2. Pegylated interferon alfa-2a (Pegasys) - EMEA-06-2023

zr pharma& GmbH; The class of immunomodulatory cytokine medicinal products for myeloproliferative neoplasms / Treatment of polycythaemia vera and treatment of essential thrombocythaemia

Action: For adoption

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

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

7.1.1. Bempedoic acid – EMEA-001872-PIP01-15-M02; Bempedoic acid / ezetimibe - EMEA-002200-PIP01-17

Daiichi Sankyo Europe GmbH; Treatment of elevated cholesterol

Proposed indication: Cardiovascular risk reduction

Action: For adoption

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

Action: For information

9.1.2. Vote by Proxy

Action: For information

9.1.3. Strategic Review and Learning Meeting (SRLM)

No item

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

PDCO member: Brian Aylward (*ad interim*)

Action: For information

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

No item

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Action: For information

9.4. Cooperation within the EU regulatory network

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

No item

9.4.2. PDCO / HTA interaction

Action: For information

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Action: For information

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

No item

9.7. PDCO work plan

No Item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

No item

10. Any other business

10.1. Paediatric health threats update

Action: For information

10.2. PDCO requirement for modifying allergen PIPs

Action: For adoption

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

PDCO member: Anette Solli Karlsen

Action: For information

11. Breakout sessions

11.1. Internal PDCO Operations

Action: For discussion on Tuesday, 12:00 - 13:00

11.2. Neonatology

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

12. Explanatory notes

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

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

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

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

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

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

The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.

In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

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

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

Annual reports on deferrals (*section 8*)

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

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