21 August 2018
EMA/527101/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the written procedure 21-24 August 2018

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

Disclaimers

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

Note on access to documents

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

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Table of contents

1. Introductions

1.1. Adoption of agenda

PDCO agenda for 21-24 August 2018

1.2. Adoption of the minutes

PDCO minutes for 24-27 July 2018

2. Opinions

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

2.1. Opinions on Products

2.1.1. Indapamide / Perindopril arginine / Atorvastatin calcium trihydrate - EMEA-002395-PIP01-18

Treatment of Essential Hypertension, Treatment of Cardiovascular diseases, Prevention of Cardiovascular diseases, Treatment of Elevated Cholesterol

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. EMEA-002363-PIP01-18

Treatment of transthyretin-mediated amyloidosis (ATTR amyloidosis) - cardiomyopathy amyloidosis, Treatment of transthyretin-mediated amyloidosis (ATTR amyloidosis) - polyneuropathy amyloidosis

Day 60 opinion

Action: For adoption

Cardiovascular Diseases / Neurology

2.1.3. Cell-free solution of lysed Escherichia coli culture, strain Laves - EMEA-002393-PIP01-18

Treatment of irritable bowel syndrome, treatment of colitis (excluding infective)

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.4. Ianalumab - EMEA-002338-PIP02-18

Primary Sjögren's Syndrome (pSS)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.5. Sarilumab - EMEA-001045-PIP02-18

Vasculitides

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.6. A synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 (SOD1) messenger ribonucleic acid - Orphan - EMEA-002403-PIP01-18

Biogen Idec Ltd; Treatment of Amyotrophic lateral sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.7. Autologous dendritic cells pulsed with allogeneic tumour cell lysate - Orphan - EMEA-002381-PIP01-18

Amphera BV; Treatment of malignant mesothelioma

Day 60 opinion

Action: For adoption

Oncology

2.1.8. Brentuximab vedotin - Orphan - EMEA-000980-PIP04-18

Takeda Pharma A/S; Treatment of Mature T and NK neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.9. Pamiparib - EMEA-002389-PIP01-18

Gastric Neoplasms Malignant

Day 60 opinion

Action: For adoption

Oncology

2.1.10. Diphtheria Toxin Interleukin-3 Fusion Protein; Orphan - EMEA-002244-PIP02-18

Stemline Therapeutics, Inc.; Treatment of blastic plasmacytoid dendritic cell neoplasm

Day 60 opinion

Action: For adoption

Oncology

2.1.11. Telisotuzumab vedotin - EMEA-002361-PIP01-18

Lung carcinoma (small cell and non-small cell carcinoma)

Day 60 opinion

Action: For adoption

Oncology

2.1.12. Eflapegrastim - EMEA-002385-PIP01-18

Treatment of Chemotherapy- Induced Neutropenia / Decrease the incidence of infection, as manifested by febrile neutropenia, in patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs associated with a clinically significant incidence of febrile neutropenia.

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.2. Opinions on Compliance Check

The members of the PDCO have been invited to comment on issues of possible non-compliance

2.2.1. Lamivudine (3TC) / Dolutegravir (DTG) - EMEA-C1-001940-PIP01-16-M01

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

Day 60 letter

Action: For adoption

Infectious Diseases

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

No items.

2.4. Opinions on Re-examinations

No items.

2.5. Opinions on Review of Granted Waivers

No items.

2.6. Finalisation and adoption of opinions

No items.

2.7. Partial Compliance Checks completed by EMA

For the following partial compliance checks, no need to refer them to PDCO Committee for discussion, were identified by the PME coordinator and PDCO Rapporteur. The PDCO has been informed in writing.

No items.

3. Comments on Products

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

3.1. Comments on Products D60-D30

3.1.1. EMEA-002378-PIP01-18

Treatment of acute heart failure

Request for Modification (RfM) – Day 60

Action: For adoption

Cardiovascular Diseases

3.1.2. Dihomo-γ-linolenic acid (DGLA) - EMEA-002364-PIP01-18

Treatment of atopic dermatitis / Treatment of moderate to severe atopic dermatitis

Request for Modification (RfM) – Day 60

Action: For adoption

Dermatology

3.1.3. Dihomo-γ-linolenic acid (DGLA) - EMEA-002364-PIP02-18

Treatment of atopic dermatitis / Treatment of pruritus associated with mild to moderate atopic dermatitis, Treatment of mild to moderate atopic dermatitis

Request for Modification (RfM) – Day 60

Action: For adoption

Dermatology

3.1.4. Pyridoxal 5'-phosphate monohydrate - Orphan - EMEA-002404-PIP01-18

Medicure Pharma Europe Limited; Treatment of pyridox(am)ine 5'-phosphate oxidase (PNPO) deficiency

Request for Modification (RfM) – Day 60

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. Tirzepatide - EMEA-002360-PIP01-18

Treatment of Type 2 diabetes mellitus

Request for Modification (RfM) – Day 60

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.6. Iclaprim mesylate - EMEA-002391-PIP01-18

Infection with Gram-positive bacteria / Treatment of acute bacterial skin and skin structure infections caused by susceptible strains of Gram-positive bacteria

Request for Modification (RfM) – Day 60

Action: For adoption

Infectious Diseases

3.1.7. Oteseconazole - EMEA-002392-PIP01-18

Treatment of vulvovaginal candidiasis

Request for Modification (RfM) – Day 60

Action: For adoption

Infectious Diseases

3.1.8. Ofatumumab - EMEA-002397-PIP01-18

Treatment of Multiple Sclerosis / Treatment of relapsing remitting multiple sclerosis

Request for Modification (RfM) – Day 60

Action: For adoption

Neurology

3.1.9. Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signaling domains - Orphan - EMEA-002369-PIP01-18

Celgene Europe Limited; Treatment of mature B-cell neoplasms / Treatment of pediatric BCMA+ relapsed or refractory B non-Hodgkin lymphoma

Request for Modification (RfM) – Day 60

Action: For adoption

Oncology

3.1.10. Vinorelbine Tartrate (liposomal) - EMEA-002365-PIP01-18

treatment of osteosarcomas, treatment of rhabdomyosarcoma, treatment of Ewing's sarcoma, treatment of non-rhabdomyosarcoma soft-tissue sarcomas / treatment of relapsed or refractory Ewing's sarcoma, treatment of relapsed or refractory rhabdomyosarcoma, treatment of relapsed or refractory osteosarcomas, treatment of relapsed or refractory non-rhabdomyosarcoma soft-tissue sarcomas

Request for Modification (RfM) – Day 60

Action: For adoption

Oncology

3.1.11. Benzimidazole-containing ENaC inhibitor - EMEA-002394-PIP01-18

Treatment of cystic fibrosis / indicated to improve lung function and reduce pulmonary exacerbations for patients with CF in conjunction with standard therapies.

Request for Modification (RfM) – Day 60

Action: For adoption

Pneumology - Allergology

3.1.12. EMEA-002398-PIP01-18

Cystic Fibrosis / Treatment of cystic fibrosis in individuals with cystic fibrosis who are homozygous for the F508del mutation and are receiving treatment with a CFTR modulator

Request for Modification (RfM) – Day 60

Action: For adoption

Pneumology - Allergology

3.1.13. Nintedanib - Orphan - EMEA-001006-PIP05-18

Boehringer Ingelheim International GmbH; Treatment of fibrosing Interstitial Lung Diseases (ILD) in paediatric patients

Request for Modification (RfM) – Day 60

Action: For adoption

Pneumology - Allergology / Oncology

3.1.14. EMEA-002373-PIP01-18

Schizophrenia

Request for Modification (RfM) – Day 60

Action: For adoption

Psychiatry

3.2. Comments on Compliance Check

The members of the PDCO have been invited to comment on issues of possible non-compliance

3.2.1. Ticagrelor - EMEA-C2-000480-PIP01-08-M11

AstraZeneca AB; Prevention of thromboembolic events

Day 30 adoption

Action: For adoption

Cardiovascular Diseases

3.2.2. Glycerol Phenylbutyrate - EMEA-C-000297-PIP02-12-M02

Horizon Pharma Ireland Limited; Treatment of Urea Cycle Disorders

Day 30 discussion

Action: For comments (adoption in September).

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Luspatercept - EMEA-C1-001521-PIP01-13-M02

Celgene Europe Ltd; Treatment of beta-thalassaemia

Day 30 adoption

Action: For adoption

Haematology-Hemostaseology

3.2.4. Human normal immunoglobulin - EMEA-C-001797-PIP01-15-M01

Octapharma Pharmazeutika Produktionsges.m.b.H; Treatment of primary immunodeficiency

Day 30 discussion

Action: For comments

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.2.5. Bedaquiline (fumarate) - EMEA-C2-000912-PIP01-10-M03

Janssen-Cilag International NV; Treatment of multi-drug resistant tuberculosis

Day 30 adoption

Action: For adoption

Infectious Diseases

3.2.6. Ataluren - EMEA-C3-000115-PIP01-07-M09

PTC Therapeutics International, Limited; Duchenne/Becker Muscular Dystrophy

Day 30 adoption

Action: For adoption

Neurology

3.2.7. Larotrectinib - EMEA-C1-001971-PIP02-16-M01

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haemtopoietic and lymphoid tissue neoplasms).

Day 30 adoption

Action: For adoption

Oncology

3.2.8. Ranibizumab - EMEA-C1-000527-PIP04-13-M01

Novartis Europharm Limited; Treatment of retinopathy of prematurity

Day 30 discussion

Action: For comments

Ophthalmology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

No items.

4. Nominations

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

4.1. List of letters of intent received for submission of applications with start of procedure 16 October 2018 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

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6. Discussion on the applicability of class waivers

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Discussions on the applicability of class waiver for products

No items.

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

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

No items

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

No items.

9.2. Coordination with EMA Scientific Committees or CMDh-v

No items.

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

No items

9.4. Cooperation within the EU regulatory network

No items.

9.5. Cooperation with International Regulators

No items.

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

No items.

9.7. PDCO work plan

No items.

9.8. Planning and reporting

No items.

10. Any other business

No items.

11. Breakout sessions

No items.

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/