20 March 2025
EMA/COMP/77527/2025
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Minutes for the meeting on 18-19 February 2025

Chair: Tim Leest – Vice-Chair: Frauke Naumann-Winter

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 COMP meeting reports once the procedures are finalised.

Of note, this agenda is a working document primarily designed for COMP 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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1. Introduction

1.1. Welcome and declarations of interest of members and experts

The Chair opened the meeting by welcoming all participants. The meeting was held remotely.

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 and experts and based on the topics in the agenda of the meeting, the Committee Secretariat announced the restricted involvement of some Committee members 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 interests or restrictions 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.

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 18-19 February 2025 was adopted without amendments.

1.3. Adoption of the minutes

The minutes for 21-23 January 2025 were adopted with no amendments and will be published on EMA website.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000230735

Treatment of Duchenne muscular dystrophy

As agreed during the previous meeting, a list of issues was sent to the sponsor for response. The sponsor formally withdrew the application for orphan designation, on 04 February 2025, prior to responding to the list of issues.

2.1.2. deucrictibant monohydrate - EMA/OD/0000232365

Pharvaris Netherlands B.V.; Treatment of bradykinin-mediated angioedema

COMP Rapporteur: Cécile Dop

As agreed during the previous meeting, a list of issues was sent to the sponsor for response. The sponsor was asked to clarify the following issues:

- Condition and Prevalence

The COMP agreed with the proposed condition of "Treatment of bradykinin-mediated angioedema", as per the angioedema (AE) classification in the latest angioedema guideline by the World Allergy Organization (WAO) and the European Academy of Allergy and Clinical Immunology (EAACI) (Maurer et al., 2022). This also includes the following forms of acquired angioedema: "angiotensin-converting enzyme inhibitor-induced angioedema (ACEI-AE)" and "other drug-induced angioedema".

Therefore, the sponsor was asked to revise their prevalence calculation and include also these forms of acquired angioedema.

In the written response, and during an oral explanation before the Committee on 18 February 2025, the sponsor proposed a prevalence range between 0.53 and 1.42 per 10,000 persons. This value is based on the originally proposed combined prevalence range of 0.13-0.42 per 10,000 persons which included hereditary angioedema with and without C1 inhibitor deficiency (HAE-C1 INH and HAE-nC1 INH) and acquired angioedema with C1 inhibitor deficiency (AAE C1 INH), as well as the newly proposed prevalence range of 0.4-1 per 10,000 persons for angioedema induced by angiotensin-converting enzyme inhibitors (ACEI-AE), (Rasmussen et al., 2019; Toh et al., 2012; Aygören-Pürsün et al., 2018; Mahmoudpour et al., 2016). Of note, for estimating the yearly incidence, the sponsor assumed that ACEI-AE was an acute event with a short duration that resolves within a year of its onset. For acute events, incidence estimates reasonably approximate prevalence estimates.

The WAO/EAACI treatment guideline notes that the subgroup of "other drug-induced angioedema" may be induced by angiotensin II receptor blockers (ARBs), gliptins, neprilysin inhibitors or tissue plasminogen activators (Maurer et al., 2022). However, the sponsor did not identify any population-based studies reporting prevalence or incidence estimates in this regard. Available clinical (trial) data did not confirm a significant risk of angioedema associated with ARBs or gliptins when used as single agents (Cicardi and Zuraw, 2018).

The COMP adopted a positive opinion and agreed on a prevalence estimate of approximately 1.4 in 10,000 persons.

The Committee agreed that the condition, bradykinin-mediated angioedema, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing deucrictibant monohydrate was considered justified based on clinical data in patients with hereditary and acquired angioedema showing reduced attack severity (on demand setting) and reduced attack rate (prophylaxis setting).

The condition is life-threatening and chronically debilitating due to recurrent attacks of oedema that may cause airway obstruction leading to asphyxia.

The condition was estimated to be affecting approximately 1.4 in 10,000 persons in the European Union, at the time the application was made.

In addition, although satisfactory methods of treatment of the condition exist in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing deucrictibant monohydrate will be of significant benefit to those affected by the condition to the population at risk of developing the condition. The sponsor has provided clinical data in a broad patient population including patients with hereditary and acquired angioedema showing reduced attack severity (on demand setting) and rate (prophylaxis setting) which cannot be expected from currently authorised therapies. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for deucrictibant monohydrate, for treatment of bradykinin-mediated angioedema, was adopted by consensus.

2.1.3. extract from cannabis flower, containing high levels of cannabidiolic acid and <0.3% of tetrahydrocannabinol, extraction solvent: olive oil, virgin - EMA/OD/0000233979

Granzer Regulatory Consulting & Services GmbH; Treatment of Rett syndrome

COMP Rapporteur: Ingeborg Barisic

As agreed during the previous meeting, a list of issues was sent to the sponsor for response. The sponsor was asked to clarify the following issues:

- Intention to diagnose, prevent or treat

To establish correctly if there exists a scientific rationale for the development of the proposed product for treatment of Rett syndrome the sponsor was requested to further elaborate on the design of the study, considering the single-arm design of the study and the absence of a control/placebo group the results obtained in the study were not considered satisfactory to justify the medical plausibility, and the results obtained in the clinical study. Some improvements, while statistically significant, showed modest effect sizes and variable response rates across different domains. In addition, the results were based on a small number of patients with Rett syndrome who have been tested after a short treatment period of 12 weeks based on the assessment of clinicians and caregivers. No direct measurement of neurological or physiological changes have been conducted.

In the written response, and during an oral explanation before the Committee on 19 February 2025, the sponsor provided updated data from the open label study which suggested potential improvements in clinical global impression scale including improvements in eye contact, communication skills and mental alertness. Many patients continued treatment beyond the 20-week study period, with some having approached 52 weeks. In addition, parents reported increased awareness, responsiveness, and engagement in their children.

The COMP discussed the updated preliminary clinical data and also consider bibliographic data (Cosentino et al., 2024; Sep, Vigli et al., 2021; Desnous et al., 2024) which support that cannabidiol receptors modulate function in in vivo models of Rett syndrome. Based on the totality of evidence the COMP considered that the medical plausibility was justified.

The Committee agreed that the condition, Rett syndrome, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing extract from cannabis flower, containing high levels of cannabidiolic acid and <0.3% of tetrahydrocannabinol, extraction solvent: olive oil, virgin was considered justified based on the totality of available evidence, including preliminary clinical findings suggesting potential improvements in clinical global impression scale.

The condition is chronically debilitating and life-threatening and due to severe locomotor disability, sleep disturbances, seizures, respiratory complications and development of arrhythmias resulting in decreased life-expectancy.

The condition was estimated to be affecting approximately 1 in 10,000 persons in the European Union, at the time the application was made.

The sponsor has also established that there exists no satisfactory method of treatment in the European Union for patients affected by the condition.

A positive opinion for extract from cannabis flower, containing high levels of cannabidiolic acid and <0.3% of tetrahydrocannabinol, extraction solvent: olive oil, virgin, for treatment of Rett syndrome, was adopted by majority (26 out of 28 votes). The Norwegian COMP member agreed with the above-mentioned recommendation of the COMP.

The divergent positions (Brigitte Schwarzer-Daum, Olimpia Neagu) were appended to this opinion.

2.2. For discussion / preparation for an opinion

2.2.1. ianalumab - EMA/OD/0000224127

Novartis Europharm Limited; Treatment of immune thrombocytopenia

COMP Rapporteur: Karri Penttila

The Committee agreed that the condition, immune thrombocytopenia, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing ianalumab was considered justified based on preliminary clinical data showing that 50% of patients had a confirmed response, defined as a platelet count of ≥50,000/µl at two (or more) consecutive assessments.

The condition is life-threatening and chronically debilitating due to bleeding, which may occur without an obvious precipitating event and can involve the skin, oral cavity and gastrointestinal tract, as well as manifest with intracranial haemorrhage.

The condition was estimated to be affecting approximately 2.7 in 10,000 persons in the European Union, at the time the application was made.

In addition, although satisfactory methods of treatment of the condition have been authorised in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing ianalumab will be of significant benefit to those affected by the condition. The sponsor has provided preliminary clinical data that demonstrate that an additional improvement in platelet count is noted when ianalumab is given on top of standard of care. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for ianalumab, for treatment of immune thrombocytopenia, was adopted by consensus.

2.2.2. *Ixodes ricinus* contact phase inhibitor - EMA/OD/0000227245

Bioxodes; Treatment of non-traumatic spontaneous intracerebral haemorrhage

COMP Rapporteur: Darius Matusevicius

The Committee agreed that the condition, non-traumatic spontaneous intracerebral haemorrhage, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing *Ixodes ricinus* contact phase inhibitor was considered justified based on in vivo data in a valid model of the condition showing improvement in neurological outcomes.

The condition is life-threatening due to a high mortality which reaches approximately 50% within the first 3 months and most survivors are left with severe neurological disabilities.

The condition was estimated to be affecting approximately 3 in 10,000 persons in the European Union, at the time the application was made.

The sponsor has also established that there exists no satisfactory method of treatment in the European Union for patients affected by the condition.

A positive opinion for *Ixodes ricinus* contact phase inhibitor, for treatment of non-traumatic spontaneous intracerebral haemorrhage, was adopted by consensus.

2.2.3. adeno-associated virus vector serotype 8 containing the human *F9* gene - EMA/OD/0000228898

Regeneron Ireland Designated Activity Company; Treatment of haemophilia B

COMP Rapporteur: Enrico Costa

The Committee agreed that the condition, haemophilia B, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing adeno-associated virus vector serotype 8 containing the human *F9* gene was considered justified based on non-clinical data in models of the condition showing the rescuing of haemostasis and restoration of intrinsic coagulation function.

The condition is life-threatening and chronically debilitating due to spontaneous bleeding episodes and substantially prolonged bleeding upon injury. Bleeding starts early in life and can include life-threatening haemorrhages, such as intracranial and gastrointestinal haemorrhages. Adult patients are at risk for cerebral- or gastric haemorrhage, which can be life-threatening.

The condition was estimated to be affecting approximately 0.2 in 10,000 persons in the European Union, at the time the application was made.

In addition, although satisfactory methods of treatment of the condition exist in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing adeno-associated virus vector serotype 8 containing the human *F9* gene will be of significant benefit to those affected by the condition. The sponsor

has provided non-clinical data that demonstrate a durable and stable human factor IX expression in models of the condition that could represent a patient population currently not covered by authorised treatments. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for adeno-associated virus vector serotype 8 containing the human *F9* gene, for treatment of haemophilia B, was adopted by consensus.

2.2.4. single guide RNA containing a sequence complementary to human *ALB* locus gene, intron 1, target region, ziclumeran - EMA/OD/0000229932

Regeneron Ireland Designated Activity Company; Treatment of haemophilia B

COMP Rapporteur: Enrico Costa

The Committee agreed that the condition, haemophilia B, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing single guide RNA containing a sequence complementary to human *ALB* locus gene, intron 1, target region, ziclumeran was considered justified based on non-clinical data in models of the condition showing the rescuing of haemostasis and restoration of intrinsic coagulation function.

The condition is life-threatening and chronically debilitating due to spontaneous bleeding episodes and substantially prolonged bleeding upon injury. Bleeding starts early in life and can include life-threatening haemorrhages, such as intracranial and gastrointestinal haemorrhages. Adult patients are at risk for cerebral- or gastric haemorrhage, which can be life-threatening.

The condition was estimated to be affecting approximately 0.2 in 10,000 persons in the European Union, at the time the application was made.

In addition, although satisfactory methods of treatment of the condition exist in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing single guide RNA containing a sequence complementary to human *ALB* locus gene, intron 1, target region, ziclumeran will be of significant benefit to those affected by the condition. The sponsor has provided non-clinical data that demonstrate a durable and stable human factor IX expression in models of the condition that could represent a patient population currently not covered by authorised treatments. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for single guide RNA containing a sequence complementary to human *ALB* locus gene, intron 1, target region, ziclumeran, for treatment of haemophilia B, was adopted by consensus.

2.2.5. allopurinol - EMA/OD/0000232820

Consorcio Centro De Investigacion Biomedica En Red; Treatment of Marfan syndrome

COMP Rapporteur: Cécile Dop

The Committee agreed that the condition, Marfan syndrome, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing allopurinol was considered justified based on non-clinical data in valid disease models which have shown a reduction in aortic root diameter (aortic aneurysm), prevent aortic dissection and increase survival.

The condition is considered life-threatening due to the risk of acute aortic dissection and chronically debilitating due to pain, impaired respiratory function and impaired vision.

The condition was estimated to be affecting approximately 0.7 in 10,000 persons in the European Union, at the time the application was made.

The sponsor has also established that there exists no satisfactory method of treatment in the European Union for patients affected by the condition.

A positive opinion for allopurinol, for treatment of Marfan syndrome, was adopted by consensus.

2.2.6. - EMA/OD/0000235476

Treatment of non-infectious uveitis

The COMP adopted a list of issues that will be sent to the sponsor. The sponsor will be invited to an oral explanation before the Committee at the March meeting.

2.2.7. - EMA/OD/0000236424

Treatment of primary IgA nephropathy

The COMP adopted a list of issues that will be sent to the sponsor. The sponsor will be invited to an oral explanation before the Committee at the March meeting.

2.2.8. - EMA/OD/0000236836

Treatment of small cell lung cancer

The COMP adopted a list of issues that will be sent to the sponsor. The sponsor will be invited to an oral explanation before the Committee at the March meeting.

2.2.9. povetacicept - EMA/OD/0000237565

Vertex Pharmaceuticals (Ireland) Limited; Treatment of primary IgA nephropathy

COMP Rapporteur: Zsofia Gyulai

The Committee agreed that the condition, primary IgA nephropathy, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing povetacicept was considered justified based on preliminary clinical data showing an improvement in proteinuria.

The condition is life-threatening and chronically debilitating due to progressive loss of kidney function leading to kidney failure requiring dialysis and transplantation.

The condition was estimated to be affecting approximately 4 in 10,000 persons in the European Union, at the time the application was made.

In addition, although satisfactory methods of treatment of the condition exist in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing povetacicept will be of significant benefit to those affected by the condition. The sponsor has provided preliminary clinical data that demonstrate an added reduction in proteinuria and stable renal function which other authorised medicines have not shown. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for povetacicept, for treatment of primary IgA nephropathy, was adopted by consensus.

2.2.10. bexmarilimab - EMA/OD/0000237791

Faron Pharmaceuticals Oy; Treatment of myelodysplastic syndromes (MDS)

COMP Rapporteur: Karri Penttila

The Committee agreed that the condition, myelodysplastic syndromes (MDS), is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing bexmarilimab was considered justified based on preliminary clinical data showing response in patients with high risk, myelodysplastic syndromes who had failed prior treatment with hypomethylating agent.

The condition is life-threatening and chronically debilitating in particular due to anaemia, thrombocytopenia and neutropenia, as well as transformation into acute myeloid leukaemia.

The condition was estimated to be affecting approximately 2 in 10,000 persons in the European Union, at the time the application was made.

In addition, although satisfactory methods of treatment of the condition exist in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing bexmarilimab will be of significant benefit to those affected by the condition. The sponsor has provided preliminary clinical data that demonstrate that patients with high risk, myelodysplastic syndromes who had failed prior treatment with hypomethylating agent responded to the treatment with the proposed product in combination with standard of care. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for bexmarilimab, for treatment of myelodysplastic syndromes (MDS), was adopted by consensus.

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

None

2.6. Nominations

2.6.1. New applications for orphan medicinal product designation - Appointment of COMP rapporteurs

COMP rapporteurs were appointed for 16 applications submitted and upcoming applications.

2.7. Evaluation on-going

8 applications for orphan designation will not be discussed as evaluation is ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of erythropoietic protoporphyria

The Committee was briefed on the significant benefit issues. The COMP adopted the proposed answers on the significant benefit issues.

3.1.2. -

Diagnosis of marginal zone lymphoma

The Committee was briefed on the significant benefit issues in preparation of the March 2025 meeting.

3.1.3. -

Treatment of tuberculosis

The Committee was briefed on the significant benefit issues. The COMP adopted the proposed answers on the significant benefit issues.

3.1.4. -

Treatment of Berardinelli-Seip syndrome (congenital generalised lipodystrophy) / Treatment of Lawrence syndrome (acquired generalised lipodystrophy)

The Committee was briefed on the significant benefit issues. The COMP adopted the proposed answers on the significant benefit issues.

4. Review of orphan designation for orphan medicinal products at time of initial marketing authorisation

4.1. Orphan designated products for which CHMP opinions have been adopted

None

4.2. Orphan designated products for discussion prior to adoption of CHMP opinion

4.2.1. Kizfizo - temozolomide - EMEA/H/C/006169, EU/3/19/2188, EMA/OD/0000178760

Orphelia Pharma S.A.S.; Treatment of neuroblastoma

The status of the procedure at CHMP was noted.

4.2.2. Fabhalta - iptacopan hydrochloride - EMEA/H/C/005764/II/0001, EU/3/18/2104, EMA/OD/0000224423

Novartis Europharm Limited; Treatment of C3 glomerulopathy

COMP Rapporteur: Elisabeth Johanne Rook; COMP Co-Rapporteur: Vallo Tillmann; CHMP Rapporteur: Janet Koenig

An opinion recommending not to remove Fabhalta, iptacopan hydrochloride, EU/3/18/2104 from the EC Register of Orphan Medicinal Products was adopted by consensus.

The orphan maintenance assessment report will be publicly available on the EMA website.

[Post-meeting note: The COMP adopted the opinion by written procedure following its February meeting.]

4.2.3. Vyjuvek - beremagene geperpavec - EMEA/H/C/006330, EU/3/18/2012, EMA/OD/0000233504

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

COMP Rapporteur: Cécile Dop; COMP Co-Rapporteur: Gloria Maria Palomo Carrasco

An opinion recommending not to remove Vyjuvek, beremagene geperpavec, EU/3/18/2012 from the EC Register of Orphan Medicinal Products was adopted by consensus.

The orphan maintenance assessment report will be publicly available on the EMA website.

[Post-meeting note: The COMP adopted the opinion by written procedure following its February meeting.]

4.3. Appeal

None

4.4. On-going procedures

COMP co-ordinators were appointed for 1 application.

4.5. Orphan Maintenance Reports

Documents were tabled for information.

5. Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Columvi - glofitamab - EMEA/H/C/005751/II/0005, EU/3/21/2497, EMA/OD/0000225358

Roche Registration GmbH; Treatment of diffuse large B-cell lymphoma

CHMP Rapporteur: Boje Kvorning Pires Ehmsen

The COMP adopted a list of issues that will be sent to the sponsor. The sponsor will be invited to an oral explanation before the Committee at the March meeting.

5.3. Appeal

None

5.4. On-going procedures

None

6. Application of Article 8(2) of the Orphan Regulation

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

7.1.1. COMP membership

The Chair welcomed Ivica Brnčić, as the new member for Croatia.

The Chair welcomed Alexandru Mihail Simion, as the new member for Belgium.

7.1.2. Vote by proxy

Ioannis Kkolos gave a proxy to Evangelia Giannaki to vote on behalf of Ioannis Kkolos during the entire meeting.

Joao Rocha gave a proxy to Enrico Costa to vote on behalf of Joao Rocha during part of the meeting.

Bozenna Dembowska-Baginska gave a proxy to Vallo Tillmann to vote on behalf of Bozenna Dembowska-Baginska during part of the meeting.

7.1.3. Strategic Review & Learning meetings

The COMP noted the preliminary information for the meeting to be held face-to-face on 29-30 April 2025 in Warsaw, Poland.

7.1.4. Protocol Assistance Working Group (PAWG)

The working group on Protocol Assistance met remotely on 14 February 2025.

7.1.5. COMP Decisions Database

The COMP acknowledged the importance of adding further topics to the database.

7.1.6. Committee Meeting Dates for 2027-2028

The Committee was informed on proposed committee meeting dates for the period of 2027-2028.

7.1.7. COMP Agenda in IRIS

The Committee was informed on the upcoming change and impact of the agenda in IRIS and received a demo of the IRIS Network Portal features.

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

None

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

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

None

7.3.2. Innovation Task Force (ITF) meetings

The COMP noted the upcoming ITF meetings and the presentation of an overview of ITF activities in 2024.

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

None

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

7.5.3. Therapeutic Goods Administration (TGA), Australia

None

7.5.4. Health Canada

None

7.6. Contacts of the COMP with external parties and interaction with the Interested Parties to the Committee

None

7.7. COMP work plan

None

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2025

An updated list of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2025 were circulated.

7.8.2. Overview of orphan marketing authorisations/applications

An updated overview of orphan applications for Marketing Authorisation was circulated.

8. Any other business

8.1. EURORDIS activities and rare disease day campaign

The COMP noted the presentation on the newly project EURORDIS is involved in (IHI RealiseD) and rare disease day campaign 2025.

9. List of participants

List of participants including any restrictions with respect to involvement of members / experts following evaluation of declared interests for the 18-19 February 2025 COMP meeting, which was held remotely.

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Tim Leest	Chair	Belgium	No interests declared	
Brigitte Schwarzer-Daum	Member	Austria	No restrictions applicable to this meeting	
Alexandru Mihail Simion	Member	Belgium	No interests declared	
Ivica Brnčić	Member	Croatia	No interests declared	
Jana Mazelova	Member	Czechia	No interests declared	
Sine Buhl Naess-Schmidt	Member	Denmark	No restrictions applicable to this meeting	
Vallo Tillmann	Member	Estonia	No interests declared	
Karri Penttila	Member	Finland	No interests declared	
Cecile Dop	Member	France	No interests declared	
Frauke Naumann-Winter	Member (Vice-Chair)	Germany	No interests declared	
Evangelia Giannaki	Member	Greece	No restrictions applicable to this meeting	
Zsofia Gyulai	Member	Hungary	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Enrico Costa	Member	Italy	No interests declared	
Irena Rogovska	Member	Latvia	No restrictions applicable to this meeting	
Ruta Mameniskiene	Member	Lithuania	No interests declared	
Michel Hoffmann	Member	Luxembourg	No interests declared	
Luana Mifsud Buhagiar	Member	Malta	No interests declared	
Elisabeth Johanne Rook	Member	Netherlands	No interests declared	
Maria Elisabeth Kalland	Member	Norway	No interests declared	
Bożenna Dembowska-Baginska	Member	Poland	No restrictions applicable to this meeting	
Joao Rocha	Member	Portugal	No restrictions applicable to this meeting	
Olimpia Neagu	Member	Romania	No interests declared	
Jana Schweigertova	Member	Slovakia	No interests declared	
Gloria Maria Palomo Carrasco	Member	Spain	No interests declared	
Darius Matusevicius	Member	Sweden	No restrictions applicable to this meeting	
Julian Isla	Member	Patients' Organisation Representative	No interests declared	
Ines Alves	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Mariette Driessens	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Fernando Mendez Hermida	Member	Expert recommended by EMA	No interests declared	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Ingeborg Barisic	Member	Expert recommended by EMA	No interests declared	
Maria Judit Molnar	Member	Expert recommended by EMA	No participation in discussion, final deliberations and voting on:	2.1.1.- EMA/OD/0000230735
Maria Cavaller Bellaubi	Expert	Patients' Organisation Representative	No restrictions applicable to this meeting	
Meeting run with support from relevant EMA staff				

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

10. Explanatory notes

The notes below give a brief explanation of the main sections and headings in the COMP agenda and should be read in conjunction with the agenda or the minutes.

Abbreviations / Acronyms

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

EC: European Commission

OD: Orphan Designation

PA: Protocol Assistance

PDCO: Paediatric Committee

PRAC: Pharmacovigilance and Risk Assessment Committee

SA: Scientific Advice

SAWP: Scientific Advice Working Party

Orphan Designation *(section 2 Applications for orphan medicinal product designation)*

The orphan designation is the appellation given to certain medicinal products under development that are intended to diagnose, prevent or treat rare conditions when they meet a pre-defined set of criteria foreseen in the legislation. Medicinal products which get the orphan status benefit from several incentives (fee reductions for regulatory procedures (including protocol assistance), national incentives for research and development, 10-year market exclusivity) aiming at stimulating the development and availability of treatments for patients suffering from rare diseases.

Orphan Designations are granted by Decisions of the European Commission based on opinions from the COMP. Orphan designated medicinal products are entered in the Community Register of Orphan Medicinal Products.

Protocol Assistance *(section 3 Requests for protocol assistance with significant benefit question)*

The protocol assistance is the help provided by the Agency to the sponsor of an orphan medicinal product, on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of the medicinal product in view of the submission of an application for marketing authorisation.

Sponsor

Any legal or physical person, established in the Community, seeking to obtain or having obtained the designation of a medicinal product as an orphan medicinal product.

Maintenance of Orphan Designation *(section 4 Review of orphan designation for orphan medicinal products for marketing authorisation).*

At the time of marketing authorisation, the COMP will check if all criteria for orphan designation are still met. The designated orphan medicinal product should be removed from the Community Register of Orphan Medicinal Products if it is established that the criteria laid down in the legislation are no longer met.

For a list of acronyms and abbreviations, see:

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

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