15 April 2025
EMA/COMP/146311/2025
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Minutes for the meeting on 18-20 March 2025

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

Health and safety information

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

Disclaimers

Some of the information contained in this set of minutes is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the 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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000
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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 in-person.

In accordance with the Agency's policy on handling of declarations of interests of scientific Committees' members and experts, based on the declarations of interest submitted by the Committee members 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 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 18-20 March 2025 was adopted with no amendment.

1.3. Adoption of the minutes

The minutes for 18-19 February 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/0000236836

Treatment of small cell lung cancer

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 3 March 2025, prior to responding to the list of issues.

2.1.2. vamikibart - EMA/OD/0000235476

Roche Registration GmbH; Treatment of non-infectious uveitis

COMP Rapporteur: Elisabeth Johanne Rook

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:

- Significant benefit

To further substantiate the significant benefit claims, explorative subgroup analyses were requested from Part 4 of the DOVETAIL study regarding efficacy in treatment-naïve participants, treatment-experienced patients and those with anterior uveitis as leading cause for uveitic macular oedema (UME).

In the written response, the sponsor provided exploratory subgroup efficacy analyses from their Phase I study in patients with non-infectious uveitis (NIU) who are treatment-naïve, treatment-experienced and those with anterior uveitis as leading cause for UME.

The sponsor has presented efficacy assessments based on functional outcomes (best corrected visual acuity evaluations), anatomical outcomes (including retinal thickness and macular fluid (intraretinal and subretinal fluid) to evaluate resolution of UME) evaluations by optical coherence tomography and uveitis disease activity (intraocular inflammation grades, per standardised uveitis nomenclature criteria) for the requested subgroups. These data support the positive trends observed in the general NIU population.

In addition, a subgroup analysis of aqueous humour (AH) interleukin-6 (IL-6) levels provided. In each subgroup, there was evidence of significant upregulation of AH IL-6 at baseline and observation of rapid IL-6 suppression following treatment with vamikibart, maintained for approximately 8 weeks after the last dose.

The COMP concluded that the additional data from the subgroup analyses support the use of vamikibart in a broader target population as compared to currently authorised therapies. The data were sufficient to establish significant benefit for the purpose of orphan designation. The COMP adopted a positive opinion and cancelled the oral hearing.

The Committee agreed that the condition, non-infectious uveitis, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing vamikibart was considered justified based on clinical data showing resolution of uveitic macular oedema and improvement in visual acuity.

The condition is chronically debilitating due to pain, redness of the eye and photophobia, and uveitic macular oedema, leading to significant visual impairment or blindness.

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 vamikibart will be of significant benefit to those affected by the condition. The sponsor has provided clinical data that show that vamikibart could benefit a broader patient population with non-infectious uveitis of all anatomical subtypes and including treatment-naïve as well as treatment-experienced patients, as compared to the

currently authorised medicines. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for vamikibart, for treatment of non-infectious uveitis, was adopted by consensus.

2.1.3. adeno-associated viral vector serotype LK03 containing the human *CFI* gene - EMA/OD/0000236424

Purespring Therapeutics Ireland Limited; Treatment of primary IgA nephropathy

COMP Rapporteur: Elisabeth Johanne Rook

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:

- Significant benefit

The arguments on significant benefit are based on an alternative mechanism of action and the potential improved efficacy in the condition.

The sponsor was requested to further discuss the arguments provided for significant benefit and to elaborate on the results from their non-clinical in vivo study to justify the assumption of significant benefit over authorised medicinal products for the proposed orphan condition. Further elaboration regarding the target patient population with current treatment recommendations for authorised medicines should be discussed.

In the written response, and during an oral explanation before the Committee on 19 March 2025, the sponsor noted that neither sparsentan nor budesonide could reduce the loss of chronic renal function and hence there is a need for additional medicines. Following 9 months of budesonide treatment, the addition of the proposed product showed results indicating that it is likely to work as an add-on therapy to sparsentan, for a subset (approximately 22% based on the PROTECT trial partial responder analysis) of patients not achieving proteinuria <1.0 g/day and thus have a high life-time risk of renal failure, despite the best currently available care.

Data derived from the non-clinical studies in the grouped ddY model of the condition was provided to show that the product could reduce fibrosis, the loss of podocytes as well as reduce the activation of complement in the nephron. Proteinuria is difficult to measure in rodents according to the sponsor as it is technically challenging to collect the urine, instead the sponsor thinks fibrosis is better in the non-clinical model.

The COMP accepted the argumentation proposed by the sponsor and considered they could recommend granting the orphan designation.

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 adeno-associated viral vector serotype LK03 containing the human *CFI* gene was considered justified based on non-clinical in vivo data showing a reduction in proteinuria and loss of podocytes.

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 4.5 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 viral vector serotype LK03 containing the human *CFI* gene will be of significant benefit to those affected by the condition. The sponsor has provided non-clinical in vivo data that demonstrate a reduction in glomerular fibrosis and proteinuria as well as the loss of podocytes which could offer an additional benefit to currently authorised medicines where progressive decline in renal function has been reported. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for adeno-associated viral vector serotype LK03 containing the human *CFI* gene, for treatment of primary IgA nephropathy, was adopted by consensus.

2.2. For discussion / preparation for an opinion

2.2.1. nipocalimab - EMA/OD/0000166069

Janssen Cilag International; Prevention of foetal and neonatal alloimmune thrombocytopenia

COMP Rapporteur: Elisabeth Johanne Rook

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

The intention to treat the condition with the medicinal product containing nipocalimab was considered justified based on a non-clinical in vivo pregnancy model and clinical data from a related maternal-foetal incompatibility disorder, demonstrating that the drug prevents exposure to the causative maternal alloantibodies in the foetus and neonate.

The condition is chronically debilitating and life-threatening due to the occurrence of bleeding including intracranial haemorrhage of the foetus or neonate, which can lead to miscarriage, still birth or neonatal death or to permanent neurological damage.

The condition was estimated to be affecting approximately 2.3 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 nipocalimab, for treatment of foetal and neonatal alloimmune thrombocytopenia, was adopted by consensus.

2.2.2. fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer - EMA/OD/0000236117

Pharma Gateway AB; Treatment of Duchenne muscular dystrophy

COMP Rapporteur: Darius Matusevicius

The Committee agreed that the condition, Duchenne muscular dystrophy, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer was considered justified based on non-clinical data showing improved skeletal and cardiac muscle function, and preliminary clinical data which showed a positive effect in functional endpoints upon treatment with the proposed product.

The condition is life-threatening and chronically debilitating due to progressive muscle weakness eventually affecting all voluntary muscles, this is followed by dilated cardiomyopathy and cardiac output decrease, leading to terminal respiratory or cardiac failure.

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 fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer will be of significant benefit to those affected by the condition. The sponsor has provided preliminary clinical data that demonstrate a positive effect in functional endpoints with the proposed product when used as an add-on treatment to corticosteroid treatment. This data indicates that the product could provide additional benefit for patients already treated with the authorised medicinal products. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer, for treatment of Duchenne muscular dystrophy, was adopted by consensus.

2.2.3. humanised IgG1 monoclonal antibody against muscle specific kinase - EMA/OD/0000236884

Argenx; Treatment of spinal muscular atrophy

COMP Rapporteur: Julian Isla

The Committee agreed that the condition, spinal muscular atrophy, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing humanised IgG1 monoclonal antibody against muscle specific kinase was considered justified based on non-clinical in vivo data using a valid model of the condition showing improvement in voluntary locomotion and muscle force.

The condition is life-threatening due to respiratory failure and chronically debilitating due to muscle wasting, weakness, failure to thrive, and orthopaedic complications.

The condition was estimated to be affecting less than 0.4 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 humanised IgG1 monoclonal antibody against muscle specific kinase will be of significant benefit to those affected by the condition. The sponsor has provided non-clinical in vivo data that showed functional improvements regarding voluntary

locomotion and muscle force following the use in combination with a precursor of an authorised product. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for humanised IgG1 monoclonal antibody against muscle specific kinase, for treatment of spinal muscular atrophy, was adopted by consensus.

2.2.4. - EMA/OD/0000239285

Diagnosis of ATTR amyloidosis

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 April meeting.

2.2.5. small-interfering RNA against soluble fms-like tyrosine kinase-1 e15a mRNA, small-interfering RNA against soluble fms-like tyrosine kinase-1 i13 mRNA - EMA/OD/0000239625

Comanche Biopharma (Europe) Limited; Treatment of pre-eclampsia

COMP Rapporteur: Fernando Mendez Hermida

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

The intention to treat the condition with the medicinal product containing small-interfering RNA against soluble feline McDonough sarcoma (fms)-like tyrosine kinase-1 e15a mRNA, small-interfering RNA against soluble fms-like tyrosine kinase-1 i13 mRNA was considered justified based on non-clinical in vivo models showing a reduction in blood pressure and proteinuria.

The condition is life-threatening due to seizures and risk of maternal and foetal death.

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 small-interfering RNA against soluble fms-like tyrosine kinase-1 e15a mRNA, small-interfering RNA against soluble fms-like tyrosine kinase-1 i13 mRNA will be of significant benefit to those affected by the condition. The sponsor has provided non-clinical in vivo data that demonstrate an effect on proteinuria which currently authorised medicines do not adequately treat. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for small-interfering RNA against soluble fms-like tyrosine kinase-1 e15a mRNA, small-interfering RNA against soluble fms-like tyrosine kinase-1 i13 mRNA, for treatment of pre-eclampsia, was adopted by consensus.

2.2.6. sirolimus - EMA/OD/0000240898

Scendea (NL) B.V.; Treatment of familial adenomatous polyposis

COMP Rapporteur: Cécile Dop

The Committee agreed that the condition, familial adenomatous polyposis, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing sirolimus was considered justified based on non-clinical data showing reduced polyp counts and improved survival, in combination with clinical data showing both a reduction in polyp burden and an increased proportion of patients classified as non-progressors.

The condition is chronically debilitating and life-threatening due to the high risk of developing colorectal cancer as well as extra colonic manifestations which include polyps of the gastric fundus and duodenum, desmoids, gastric and duodenal carcinoma, follicular or papillary thyroid cancer, and central nervous system tumours.

The condition was estimated to be occurring in approximately 0.5 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 for the population at risk of developing the condition.

A positive opinion for sirolimus, for treatment of familial adenomatous polyposis, was adopted by consensus.

2.2.7. - EMA/OD/0000241887

Treatment of Duchenne muscular dystrophy and Becker muscular dystrophy

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 April meeting.

2.2.8. parahydroxybenzoic acid - EMA/OD/0000242100

Universidad De Granada; Treatment of primary ubiquinone deficiency

COMP Rapporteur: Ingeborg Barisic

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

The intention to treat the condition with the medicinal product containing parahydroxybenzoic acid was considered justified based on preclinical data in a severe model of the disease showing rescue of perinatal lethality, developmental delays, brain and cardiovascular abnormalities. Withdrawal of the sponsor’s product postnatally led to decreased survival and the appearance of an encephalopathic phenotype.

The condition is life-threatening and chronically debilitating due to the progressive nature of the disorder causing encephalopathy, cardiac and renal failure.

The condition was estimated to be affecting less than 0.1 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 parahydroxybenzoic acid will be of significant benefit to those affected by the condition. The sponsor has provided non-clinical

comparative data that demonstrate better efficacy than current standard of care in terms of rescuing perinatal lethality/survival and suppression of the appearance of motor deficits suggestive of an encephalopathic phenotype. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for parahydroxybenzoic acid, for treatment of primary ubiquinone deficiency, 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 19 upcoming applications.

2.7. Evaluation on-going

The Committee noted that evaluation was on-going for 15 applications for orphan designation.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of beta-thalassaemia intermedia and major

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

3.1.2. -

Treatment of sickle cell disease

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

3.1.3. -

Treatment of graft-versus-host disease

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 pancreatic cancer

The Committee was briefed on the clarification request received on the significant benefit.

3.1.5. -

Diagnosis of marginal zone lymphoma

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. - zanidatamab - EMEA/H/C/006380, EU/3/21/2458, EMA/OD/0000241913

Jazz Pharmaceuticals Ireland Limited; Treatment of biliary tract 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 April meeting.

4.2.2. - givinostat - EMEA/H/C/006079, EU/3/12/1009, EMA/OD/0000178186

Italfarmaco S.p.A.; Treatment of Duchenne muscular dystrophy

The status of the procedure at CHMP was noted.

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

5.1.1. 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

COMP Rapporteur: Frauke Naumann-Winter; COMP Co-Rapporteur: Maria Elisabeth Kalland; CHMP Rapporteur: Boje Kvorning Pires Ehmsen

The status of the procedure at CHMP was noted. The sponsor withdrew the orphan status with the European Commission, prior to a final COMP opinion.

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

5.1.2. Kaftrio - ivacaftor / tezacaftor / elexacaftor - EMEA/H/C/005269/II/0042/G, EU/3/18/2116, EMA/OD/0000160021

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

COMP Rapporteur: Cécile Dop; COMP Co-Rapporteur: Enrico Costa; CHMP Rapporteur: Peter Mol

An opinion recommending not to remove Kaftrio (EU/3/18/2116) 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.

5.2. Prior to adoption of CHMP opinion

None

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

None

7.1.2. Vote by proxy

None

7.1.3. Strategic Review & Learning meetings

The COMP noted the topics and draft agenda 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 March 2025.

7.1.5. COMP Decisions Database

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

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

Documents were tabled for information.

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

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

7.3.1.1. Updates to Rules of Procedure and mandates for PCWP and HCPWP

Proposal to update the PCWP and HCPWP mandates and Rules of Procedure in order to reflect the enlargement of PCWP/HCPWP from 22 to 25 member organisations and the inclusion of a new responsibility to oversee EMA fora for information and communication with patients, healthcare professionals and their organisations was presented and endorsed by COMP.

7.3.1.2. Agenda and Minutes

The meeting summary of the PCWP/HCPWP and all eligible organisations meeting held on 20 November 2024 and the draft agenda of the PCWP-HCPWP meeting to be held on 1-2 April 2025 were tabled for information.

7.3.2. Innovation Task Force (ITF) meetings

The COMP noted the upcoming ITF meetings.

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.4.2. Feedback from the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP) Plenary

Feedback from the ENCePP Plenary was shared with the Committee and documents were tabled for information.

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. EMA rules of reimbursement

An overview of EMA rules of reimbursement was presented to the Committee members.

8.2. Revision of EMA policy 0044 on handling of competing interests

The main changes in the revision of policy 0044, the updated declaration of interests form and the next steps for experts were presented to the Committee. Committee members and all experts will be requested to submit an updated declaration of interests by 1 May 2025.

8.3. Revisions made to the reflection paper on real-world evidence (RWE)

The COMP noted the presentation of the main changes introduced to the reflection paper on use of real-world data (RWD) in non-interventional studies (NIS) to generate RWE for regulatory purposes after the public consultation.

8.4. Draft reflection paper on Patient Experience Data (PED) for internal consultation

The draft reflection paper on Patient Experience Data (PED) was presented to the Committee.

9. List of participants

List of participants including any restrictions with respect to involvement of members / experts following evaluation of declared interests for the 18-20 March 2025 COMP meeting, which was held in-person.

An asterisk (*) after the role, in the second column, signals that the member attended remotely. Additional experts participated in (part of) the meeting, either in person or 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	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Ivica Brnčić	Member	Croatia	No interests declared	
Jana Mazelova	Member	Czechia	No interests declared	
Sine Buhl Naess-Schmidt	Member	Denmark	No participation in discussion, final deliberations and voting on:	3.1.2.
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 participation in discussion, final deliberations and voting on:	3.1.1.
Zsofia Gyulai	Member	Hungary	No interests declared	
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	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
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	
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:	2.2.2. fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer - EMA/OD/0000236117 2.2.7. - EMA/OD/0000241887 4.2.2. - givinostat - EMEA/H/C/006079, EU/3/12/1009, EMA/OD/0000178186
Maria Cavaller Bellaubi	Expert	Patients' Organisation Representative	No restrictions applicable to this meeting	

Name	Role	Member State or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Olaf Klungel	Expert	Netherlands	No interests declared	
Xavier Kurz	Expert	Belgium	No interests declared	
Mencia De Lemus Belmonta	Expert	Germany	No interests declared	
A representative from the European Commission attended the 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/