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SCIENCE MEDICINES HEALTH

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Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Minutes for the meeting on 12-14 March 2024

Chair: Violeta Stoyanova-Beninska – Vice-Chair: Armando Magrelli

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 set of minutes 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 that no restriction in the involvement of meeting participants in upcoming discussions was identified.

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 12-14 March 2024 was adopted with no amendments.

1.3. Adoption of the minutes

The minutes for 13-15 February 2024 were adopted with no amendments and will be published on the EMA website.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. autologous adipose-derived mesenchymal stem cells embedded in an extracellular matrix with hydroxyapatite/beta-tricalcium phosphate particles – EMA/OD/0000158981

Novadip Biosciences; Treatment of congenital pseudarthrosis of long bones

COMP Rapporteur: Ines Alves

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:

- Number of people affected

The sponsor was asked to further justify the use of 18 years as appropriate disease duration, considering that the underlying condition and the sequelae thereof can still reach well into adulthood.

The prevalence estimate should be recalculated based on the updated disease duration.

For the estimation and presentation of the prevalence estimate the sponsor was advised to refer to the ["Points to Consider on the Estimation and Reporting of a Prevalence of a Condition for Orphan Designation"](#).

In the written response, the sponsor clarified that if it is assumed that patients are not healed at skeletal maturity then the prevalence can be estimated based on life expectancy of 80.7 years (Eurostat). Assuming an EU population of 454,304,293 at January 1st 2023 (Eurostat) the prevalence of congenital pseudarthrosis (CP) of the long bones is estimated as 0.9 per 10,000 population. The COMP agreed with the sponsors revised prevalence calculation and estimate of 0.9 per 10,000 persons, considering that CP of the long bones is a lifelong chronic condition. The COMP adopted a positive opinion during its March 2024 meeting.

The Committee agreed that the condition, congenital pseudarthrosis of long bones, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing autologous adipose-derived mesenchymal stem cells embedded in an extracellular matrix with hydroxyapatite/beta-tricalcium phosphate particles was considered justified based on preliminary clinical data in paediatric patients with severe congenital pseudarthrosis of the tibia demonstrating bone union and no re-fracturing during follow-up.

The condition is chronically debilitating due to the high risk of non-union after fracture, associated with impaired mobility and persistent pain. Furthermore, the condition is associated with a high re-fracture rate and can lead to limb amputation.

The condition was estimated to be affecting approximately 0.9 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 autologous adipose-derived mesenchymal stem cells embedded in an extracellular matrix with hydroxyapatite/beta-tricalcium phosphate particles, for treatment of congenital pseudarthrosis of long bones, was adopted by consensus.

2.1.2. autologous adipose-derived stem cells – EMA/OD/0000155985

Regenera GmbH; Treatment of traumatic spinal cord injury

COMP Rapporteur: Robert Nistico

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 studies to justify the assumption of significant benefit over authorised medicinal products for the proposed orphan condition. The sponsor was requested to elaborate in particular on longer-term data regarding locomotor function.

In the written response, and during an oral explanation before the Committee on 12 March 2024, the sponsor defended their position.

The COMP was of the opinion that it was still unclear how the arginine-glycine-aspartic acid (RGD) – synthesis of extracellular matrix (ECM) hydrogel supplemented with human mesenchymal stromal cells (hMSCs) in the Papa et al., 2018 study relates to the ATMP presented in the current application, which consists of an agarose carbomer hydrogel (spinoSave) containing autologous living mesenchymal stem cells from the stromal vascular fraction of adipose tissue (ADSCs).

The only results obtained with the product applied for are published (Veglianese, 2023). Unfortunately, these results have been measured only up to 14 days post treatment.

Two studies were described using the contusion spinal cord injury (SCI) model using AC hydrogel (spinoSave) loaded with human stem cells and positioned in epidural/epilesional position for transdural delivery of stem cell secretome into the spinal cord lesion.

In conclusion, the sponsor did not present new data on longer term recovery to substantiate the long-term effects of ADSCs in support of the significant benefit over MPSS.

The COMP considered that the use of MPSS as a “comparator” to establish significant benefit at this stage could be questionable as (i) current guidelines suggest insufficient evidence to make a recommendation for using MPSS (Picetti et al., 2024) and (ii) a recent metanalysis concludes that methylprednisone (MP) does not offer a benefit in ameliorating the functional outcome after treating acute SCI (Geisler et al., 2023).

Taking these points into consideration the COMP considered they could recommend granting the orphan designation.

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

The intention to treat the condition with the medicinal product containing autologous adipose-derived stem cells was considered justified based on non-clinical in vivo data showing a recovery of locomotor function.

The condition is chronically debilitating and life-threatening due to sensory and motor loss of function in the limbs and reduced life expectancy.

The condition was estimated to be affecting approximately 4.2 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 autologous adipose-derived stem cells will be of significant benefit to those affected by the condition. The sponsor has provided non-clinical in vivo data

that demonstrated an improvement in locomotor function which has not been adequately achieved with the currently authorised product. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for autologous adipose-derived stem cells, for treatment of spinal cord injury, was adopted by consensus.

2.1.3. acetylleucine – EMA/OD/0000159738

IntraBio Ireland Limited; Treatment of ataxia-oculomotor apraxia

COMP Rapporteur: Darius Matusevicius

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

The COMP considered that the valid orphan condition is the broader disease entity of treatment of ataxia-oculomotor apraxia.

Note that this is for the purposes of orphan medicinal product designation. The sponsor's attention was drawn to the orphan regulations and relevant guidelines (especially section A of [2022/C 440/02](#)).

- Number of people affected

The prevalence calculation and estimate needs to be aligned with the final orphan condition.

As a general remark, the sponsor was reminded that it needs to be clearly explained how the estimated prevalence has been calculated, indicating the methods and results from the primary epidemiological data sources.

For the estimation and presentation of the prevalence estimate the sponsor was advised to refer to the ["Points to Consider on the Estimation and Reporting of a Prevalence of a Condition for Orphan Designation"](#).

In the written response, the sponsor agreed to the overarching condition of ataxia-oculomotor apraxia (AOA) and provided a re-calculation of the prevalence for the broader disease entity accordingly. The COMP agreed with a prevalence estimate of AOA of less than 0.1 per 10,000 persons in the EU. The COMP considered that the written responses were sufficient to resolve the issue and adopted a positive opinion. The oral hearing was cancelled.

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

The intention to treat the condition with the medicinal product containing acetylleucine was considered justified based on clinical case reports from a few patients with of ataxia-oculomotor apraxia type 4 (AOA4) which suggests that treatment might improve gait, speech and fine motor skills.

The condition is chronically debilitating due to progressive loss of coordination of gait, arms and hands eventually leading to the inability to live independently. Symptoms also include dysarthria and difficulty swallowing. Most patients become wheelchair-bound in the later stages of the disease.

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.

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 acetylleucine, for treatment of ataxia-oculomotor apraxia, was adopted by consensus.

2.1.4. sevasekten – EMA/OD/0000158137

FGK Representative Service GmbH; Treatment of Duchenne muscular dystrophy (DMD)

COMP Rapporteur: Armando Magrelli

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 the new 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 the non-clinical studies to justify the assumption of significant benefit over authorised medicinal products in particular vamorolone for the proposed orphan condition.

Furthermore, it would be useful to obtain more information on the ongoing study development.

In the written response, the sponsor defended their position. Currently the standard of care in Duchenne muscular dystrophy is the use of corticosteroids. These medicinal products are approved in multiple member states, and in particular vamorolone recently received centralised authorisation in the European Union.

In support of the significant benefit claims, the sponsor elaborated on the non-clinical studies with the proposed products demonstrating efficacy in preservation of microscopy of intact muscle, protection from force decline after repeated contraction, and prevention of calcium influx after contraction. In the model used, ongoing contraction-induced injury continues even in the presence of corticosteroids.

However, the addition of the proposed product ameliorated force drop and maintained muscle strength. Contraction in the muscle also resulted in extracellular calcium influx and increases in associated resting tension but was improved in the group treated with the proposed product. These results would indicate that the proposed product would act directly on the contraction-induced injury that occurs when dystrophin is deficient or absent, in contrast to authorised treatments that decrease the inflammatory-associated response to muscle damage, but do not address the fundamental cause of disease progression.

In conclusion, at this point in time the responses from the sponsor were considered satisfactory by the COMP to support the assumption of significant benefit, and a positive opinion was adopted prior to the oral explanation. The sponsor was advised to request protocol assistance for the next steps in the development especially considering the clinical development will be started in the near future.

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 sevasemten was considered justified based on non-clinical data in models of the condition showing an improvement in biomarkers of muscle damage in combination with 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 0.5 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 sevasemten will be of significant benefit to those affected by the condition. The sponsor has provided non-clinical data which showed the protective effect in muscle strength of the proposed product when used as an add-on treatment to corticosteroids treatment. This data indicates that the product could be used in a broader patient population than the authorised medicinal products. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for sevasemten, for treatment of Duchenne muscular dystrophy, was adopted by consensus.

2.2. For discussion / preparation for an opinion

2.2.1. – EMA/OD/0000133472

Treatment of tuberculosis

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.

2.2.2. – EMA/OD/0000156967

Treatment of familial chylomicronemia syndrome (FCS)

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.

2.2.3. annamycin – EMA/OD/0000157122

Moleculin Amsterdam B.V.; Treatment of acute myeloid leukaemia

COMP Rapporteur: Karri Penttila

The Committee agreed that the condition, acute myeloid leukaemia, is a distinct medical entity and meets the criteria for orphan designation.

The intention to treat the condition with the medicinal product containing annamycin was considered justified based on preliminary clinical data showing responses in patients with relapsed/refractory acute myeloid leukaemia.

The condition is life-threatening and chronically debilitating due to the consequences of bone marrow dysfunction, such as intracranial or gastro-intestinal haemorrhagic episodes, disseminated intravascular coagulation, and the risk of severe infections. The condition progresses rapidly and is fatal within days to weeks or a few months if left untreated.

The condition was estimated to be affecting approximately 1.3 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 annamycin will be of significant benefit to those affected by the condition/to the population at risk of developing the condition. The sponsor has provided preliminary clinical data that showed improved responses in patients with relapsed/refractory acute myeloid leukaemia compared to standard of care. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for annamycin, for treatment of acute myeloid leukaemia, was adopted by consensus.

2.2.4. – EMA/OD/0000158039

Treatment of heparin-induced thrombocytopenia

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. humanised IgG1 (K322A) monoclonal antibody against disialoganglioside GD2 – EMA/OD/0000160213

Somerville Development Partners B.V.; Treatment of neuroblastoma

COMP Rapporteur: Bozena Dembowska-Baginska

The Committee agreed that the condition, neuroblastoma, 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 (K322A) monoclonal antibody against disialoganglioside GD2 was considered justified based on preliminary clinical data showing responses in patients with newly diagnosed and relapsed/refractory neuroblastoma.

The condition is life-threatening and chronically debilitating due to its aggressive nature and frequency of metastatic disease. It accounts for almost 15% of childhood cancer fatalities.

The condition was estimated to be affecting approximately 1.1 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 exist in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing humanised IgG1 (K322A) monoclonal antibody against disialoganglioside GD2 will be of significant benefit to those affected by the condition. The

sponsor has provided preliminary clinical data which showed improved efficacy in patients with newly diagnosed neuroblastoma compared to standard of care. The Committee considered that this constitutes a clinically relevant advantage.

A positive opinion for humanised IgG1 (K322A) monoclonal antibody against disialoganglioside GD2, for treatment of neuroblastoma, was adopted by consensus.

2.2.6. – EMA/OD/0000161033

Treatment of hypophosphatasia

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.

2.3. 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 24 applications.

2.7. Evaluation on-going

The Committee noted that evaluation was on-going for 16 applications for orphan designation.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. –

Treatment of Niemann-Pick disease, type C

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

3.1.2. –

Treatment of neurofibromatosis type 1

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

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

4.1.1. Voydeya – danicopan – EMEA/H/C/005517, EU/3/17/1946, EMA/OD/0000136076

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

COMP Rapporteur: Elisabeth Johanne Rook; COMP Co-Rapporteur: Karri Penttila

A list of issues was adopted on 15 February 2024.

An oral explanation was held on 13 March 2024.

An opinion recommending not to remove Voydeya, danicopan, EU/3/17/1946 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.

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

4.2.1. Fabhalta – iptacopan - EMEA/H/C/005764, EU/3/20/2281, EMA/OD/0000141229

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria

COMP Rapporteur: Karri Penttila; COMP Co-Rapporteur: Elisabeth Johanne Rook

An opinion recommending not to remove Fabhalta, iptacopan, EU/3/20/2281 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 March 2024 meeting.]

4.2.2. – efanesoctocog alfa - EMEA/H/C/005968, EU/3/19/2176, EMA/OD/0000160184

Swedish Orphan Biovitrum AB (publ); Treatment of haemophilia A

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.

4.3. Appeal

None

4.4. On-going procedures

COMP co-ordinators were appointed for 3 applications.

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. Reblozyl – luspatercept - EMEA/H/C/004444/II/0021, EU/3/14/1331, EMA/OD/0000134295

Bristol-Myers Squibb Pharma EEIG; Treatment of myelodysplastic syndromes

COMP Rapporteur: Karri Penttila; COMP Co-Rapporteur: Bozenna Dembowska-Baginska;
CHMP Rapporteur: Daniela Philadelphia; CHMP Co-Rapporteur: Ewa Balkowiec Iskra

An opinion recommending not to remove Reblozyl, luspatercept, EU/3/14/1331 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

5.2.1. Onivyde pegylated liposomal – irinotecan - EMEA/H/C/004125/II/0034, EU/3/11/933, EMA/OD/0000144740

Les Laboratoires Servier; Treatment of pancreatic cancer

COMP Rapporteur: Brigitte Schwarzer-Daum; COMP Co-Rapporteur: Frauke Naumann-Winter; CHMP Rapporteur: Filip Josephson

An opinion recommending not to remove Onivyde pegylated liposomal, irinotecan, EU/3/11/933 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 March 2024 meeting.]

5.3. Appeal

None

5.4. On-going procedures

COMP co-ordinators were appointed for 1 application.

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 thanked Eva Malikova for her contribution as a member for Slovakia.

7.1.2. Vote by proxy

None

7.1.3. Strategic Review & Learning meetings

None

7.1.4. Protocol Assistance Working Group (PAWG)

The working group on Protocol Assistance met remotely on 11 March 2024.

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

None

7.3.2. Upcoming 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. EU Network Training Centre (NTC): training webinar on the regulatory/HTA interface under the HTA Regulation

An EU NTC training webinar on the regulatory/HTA interface under the HTA Regulation will be held on 2 May 2024. EMA would like to raise awareness within the wider regulatory network, whilst extending the target audience also to HTA colleagues, given the mutual learnings in view of the future collaborations. The agenda will cover an overview of the new HTA Regulation, outline of collaboration between regulators and HTAs under the new legal framework, followed by mutual learning about the respective assessment scopes. The webinar will be delivered by colleagues from the EC, HTAs and regulatory network and EMA respectively.

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 2024

An updated list of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2024 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. Update on Real World Evidence, including DARWIN EU®

The Committee received an update on the progress of DARWIN EU including the most recently onboarded additional 10 data partners. The progress of a study to compare direct and indirect methods to estimate prevalence of chronic diseases using real-world data was also presented and comments provided during the plenary discussion were duly noted. Finally an update was given on recent and upcoming events, including the launch of the HMA-EMA catalogues of real-world data sources and studies, the pharmacoepidemiology and real-world evidence training curriculum (available via EU NTC), the introduction of a new knowledge sharing event series (called real-world academy) and the recent joint HMA-EMA [multistakeholder workshop on patient registries](#), which took place on 12 and 13 February 2024. The COMP welcomed the update.

8.2. Collaborare project: call for suggestions of conditions

The COMP noted the call for suggestions of conditions to start the pilot with.

8.3. EMA business Pipeline activity

Documents were tabled for information.

9. List of participants

List of participants including any restrictions with respect to involvement of members / experts following evaluation of declared interests for the 12-14 March 2024 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
Violeta Stoyanova-Beninska	Chair	Netherlands	No interests declared	
Armando Magrelli	Vice-Chair	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
Brigitte Schwarzer-Daum	Member	Austria	No restrictions applicable to this meeting	
Tim Leest	Member	Belgium	No interests declared	
Ioannis Kkolos	Member	Cyprus	No interests declared	
Jana Mazelova	Member	Czechia	No interests declared	
Elisabeth Penninga	Member	Denmark	No interests declared	
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	Germany	No interests declared	
Evangelia Yannaki	Member	Greece	No restrictions applicable to this meeting	
Zsafia Gyulai	Member	Hungary	No interests declared	
Emma Fagan	Member	Ireland	No interests declared	
Enrico Costa	Member	Italy	No interests declared	
Irena Rogovska	Member	Latvia	No restrictions applicable to this meeting	
Michel Hoffmann	Member	Luxembourg	No interests declared	
Robert Nistico	Member	Malta	No restrictions applicable to this meeting	
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	
Gloria Maria Palomo Carrasco	Member	Spain	No interests declared	
Darius Matusевичius	Member	Sweden	No restrictions applicable to this meeting	
Pauline Evers	Member	Patients' Organisation Representative	No interests declared	
Julian Isla	Member	Patients' Organisation Representative	No interests declared	
Ines Alves	Member	Patients' Organisation Representative	No restrictions applicable to this meeting	
Ingeborg Barisic	Member	Expert recommended by EMA	No restrictions applicable to this meeting	
Judit Molnar	Member	Expert recommended by EMA	No restrictions applicable to this meeting	
Maria Cavaller Bellaubi	Expert	Patients' Organisation Representative	No restrictions applicable to this meeting	
Meeting run with support from relevant EMA staff				

Experts 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/