Contribution ID: 7c14a096-bcc3-4cdb-b6eb-1b4960c91c80
Date: 30/09/2024 10:13:55

# Submission of comments on Central Nervous System Working Party (CNSWP) Workplan 2025-2027

Fields marked with * are mandatory.

## Introduction to the survey on submission of comments on Central Nervous system Workplan 2025-2027

We would like to draw your attention to the new draft CNS workplan for 2025-2027, recently adopted by EMA's human medicines committee (CHMP). This workplan outlines the strategic direction and key priorities for the next three years of the working party.

Please click on the link below to download the draft WP Workplan 2025-2027.Please click on the link below to download the draft WP Workplan 2025-2027.

CNS_WP_work_plan_2025-2027.pdf

**The public consultation is launched on 29 July 2024 until 30 September 2024 11:59 pm, CET.**

Those participating in the consultation are asked to please submit comments via the EU Survey tool only, by using the specific table for each section.

If you respond on behalf of a company that is affiliated with an EU (trade) industry organisations, you are encouraged to share your comments to the respective affiliated EU (Trade) Industry organisation.

Before submission, a draft of the comments can be saved in the EU Survey tool. Once submitted, comments can be edited (by the deadline) by clicking on "Edit contribution" in the link https://ec.europa.eu/eusurvey/ and entering your ID contribution that can be found on the pdf copy of your submission sent via email.

You are invited to provide your organisation or name, country and email address below for the purpose of this consultation (for further information, please see EMA's Data Protection Statement below).

# Data Protection Statement

**EMA Privacy Statement**

All personal data provided within this survey questionnaire will be processed in accordance with Regulation (EU) 2018/1725 on the protection of individuals regarding the processing of personal data by the Union institutions and bodies on the free movement of such data.

This data protection statement provides details on how the Agency, in its capacity as data controller, will process the information that you have given in your questionnaire.

Internally, an 'Internal Controller' has been appointed to ensure the lawful conduct of this processing operation. The contact details of the Internal Controller are the following: Datacontroller.HumanMedicines@ema.europa.eu

Collection of data

EMA will collect all the personal data in this questionnaire, such as your name, organisation, your view on the topics subject to the survey, country of residence and your contact details. Please do not reveal any other personal data in the free text fields. EMA does not directly intend to collect personal data but to use the aggregated data for the purpose of this survey.

For the collection of data in this survey, EMA relies on the EU Survey external system. For more information on how EU Survey processes personal data, please see: https://ec.europa.eu/eusurvey/home/privacystatement

The EU Survey external system uses:

- Session "cookies" to ensure communication between the client and the server. Therefore, user's browser must be configured to accept "cookies". The cookies disappear once the session has been terminated.
- Local storage to save copies of the inputs of a participant to a survey to have a backup if the server is not available during submission or the user's computer is switched off accidentally or any other cause.
- The local storage contains the IDs of the questions and the draft answers.
- IP of every connection is saved for security reasons for every server request.
- Once a participant has submitted one's answers successfully to the server or has successfully saved a draft on the server, the data is removed from the local storage.

Your consent to the processing of your data

When you submit this questionnaire, you consent that EMA will process your personal data provided in the questionnaire as explained in this data protection statement. You may also withdraw your consent later at any time. However, this will not affect the lawfulness of any data processing carried out before your consent is withdrawn.

Start of data processing

EMA will start processing your personal data as soon as the questionnaire response is received.

Purpose of data processing

The purpose of the present data processing activity is to collect the views of stakeholders and/or concerned individuals in relation to the subject-matter of the survey. Your personal data may be used to contact you in relation to the feedback you have provided in response to the survey. No further processing of your

personal data for any other purposes outside the scope of this specific context is envisaged.

Location of data storage

All data is stored within a secure data centre at the EMA premises which is password protected and only available to EMA staff members.

Publication of data

The following data collected in this questionnaire will be published on the EMA website at the time of issuing the final guideline subject to this survey:

- organisation name (the entity on behalf you respond to this survey)
- or your name (only if you do not respond to the survey on behalf of an organisation)
- your view/comments on the topics concerned

Country information and your email address will not be published.

Retention period

If you complete and submit this survey, your personal data will be kept until the results have been completely analysed and utilised. Your personal data will be deleted by EMA at the latest 5 years after the questionnaire response was submitted. The file of the data as published will remain stored for archiving purposes beyond the maximum 5 years-retention time of the submitted questionnaire responses.

Your rights

You have the right to access and receive a copy of your personal data processed, as well as to request rectification or completion of these data. You may also request erasure of the data or restriction of the processing in accordance with the provisions of Regulation (EU) 2018/1725. You can exercise your rights by sending an e-mail to Datacontroller.HumanMedicines@ema.europa.eu.

Complaints

If you have any complaints or concerns about the processing of your personal data, you can contact EMA's Data Protection Officer at dataprotection@ema.europa.eu.

You may also lodge a complaint with the European Data Protection Supervisor: edps@edps.europa.eu.

* Please confirm that you have read and understood the Data Protection Statement above and that you consent to the processing of your personal data.
  - (●) Yes
  - ( ) No

* Please confirm that you consent to possibly be contacted by EMA in relation to your survey responses to support the finalisation of the document subject this EU Survey.
  - (●) Yes
  - ( ) No

* Please confirm that you consent to the publication of your organisation name, your name (only if you do not respond to the EU Survey on behalf of an organisation) and your survey responses on the EMA website at the time of issuing the final guideline subject to this survey.
  - (●) Yes

- [ ] No

Should you not want to give consent to publish, please send your objections to Datacontroller. HumanMedicines@ema.europa.eu.

Please be aware that the sender of the comments is responsible to not disclose any personal data of third parties in the comments.

When you have filled in the EU Survey, please use the submission button at the end of the form to submit the comments to the European Medicines Agency.

For additional information, please consult EMA's privacy statement.

## Contributor details

* 1. First name

Katarina

* 2. Last name

Nedog

* 3. Professional email address

katarina.nedog@efpia.eu

* 4. Professional affiliation

EFPIA

5. Are you commenting on behalf of an organisation or stakeholder group, then please indicate the name and email of the main contact point (if it differs from the provided above)

- [x] Yes
- [ ] No

5.1. Name main contact point

Katarina Nedog

5.2. Email address main contact point

katarina.nedog@efpia.eu

7. Please indicate the type of organisation you belong to

- [ ] Academia
- [x] Industry

- [ ] Healthcare professional
- [ ] Individual
- [ ] Patient and consumer

7.1. Other - please specify

## 1. General comments on the Workplan

## 1. General comments on the Workplan

| | Stakeholder name<br>*(to be repeated in all rows to facilitate extraction and identification of comments, thank you.)* | General comment |
|---|---|---|
| 1 | EFPIA | Welcome publication of the 2025-2027 workplan and note the progress made so far against the current plan. |
| 2 | EFPIA | We welcome the CNS WP and continued efforts of the EMA to advance the European regulatory framework for innovation in medical fields under its remit, which extend beyond neurosciences to include as well ophthalmology. Commitment across all stakeholders and collaboration on continued incremental progress is essential to promoting continued investment and ultimately access to novel treatments options, including breakthroughs. This may entail innovation and flexibility in development requirements fully leveraging state of the art experience and expertise in the field. |
| 3 | EFPIA | The EFPIA would like to request a revision of the Guideline on clinical investigation of medicinal products for the treatment of Multiple Sclerosis, EMA/CHMP/771815/2011, Rev. 2, dated 26 March 2015. This request is based on three major reasons: (1) the significant progress made over recent years in the understanding of the pathophysiological mechanisms of multiple sclerosis (MS) including their clinical correlates, (2) major evolutions of the treatment landscape, and (3) the change in medical needs of people with MS, including special populations like pediatric-onset MS, in an expanded landscape of EU approved effective immune therapies |
| | | As is the case in other areas of medical product development, enabling incremental progress in ophthalmology is also essential to promoting continued investment and availability of novel, improved treatments options. As the strategic goals of WP in this document suggest, disease progression and prevention strategies also represent an important facet of this aspiration for novel treatments. Flexibility in interpretation of regulatory standards and application of state-of-the-art experience and expertise can play an important role in shaping development expectations in this field. |

| | | |
|---|---|---|
| 4 | EFPIA | We believe that there is a critical need for advancement of innovation in the field of ophthalmology, mainly driven by the following aspects:<br>• Advances in ophthalmology research offer new possibilities for treatments even for common ophthalmic diseases (e.g. intermediate age-related macular degeneration, geographic atrophy, glaucoma and non-proliferative diabetic retinopathy) that affect millions of Europeans and which through progression of disease can ultimately lead to meaningful vision loss.<br>• There is a high unmet medical need with regards to treatment options for rare or inherited retinal diseases (such as Retinitis Pigmentosa, Stargardt Disease etc.).<br><br>Therefore, we applaud the goal of developing a guideline for retinopathies, within the next workplan period. Nevertheless, we strongly believe that the progression of the therapeutic environment in ophthalmology warrants higher priority in the European regulatory system including at the EMA, which is why we are concerned about the lack of specific, explicit goals in the working party plans with respect to ophthalmology To address this, we propose to further foster a common understanding among regulators, industry, academia, HTA bodies and patient associations.<br><br>We encourage the continued expansion of ophthalmology expertise within the EMA and European medicines regulatory network. In addition, we encourage greater public and patient interface to collaborate in advancing the field. We applaud the participation in symposia such as the US NEI/FDA (National Eye Institute/ Food and Drug Administration) Symposia on ophthalmology drug development, which provide a public venue for exchange of ideas across stakeholders. In this regard, workshops sponsored by EMA on ophthalmology have in our view also been successful in the past; given that the last such workshop occurred in 2011, we encourage the CNS Working Party to consider a similar format in the near future touching on important ophthalmology challenges today. We are willing to contribute expertise and resources to the proposed multistakeholder workshop. |

| | | |
|---|---|---|
| 5 | EFPIA | The current workplan (covering until end of 2024) includes the plans for releasing a draft guideline on treatment of migraine in Q3 2024 and on epilepsy in Q4 2024 – hence expected soon. The same goes for the guideline for epilepsy which is expected for public consultation in Q4 2024. Does these 2 draft guidelines remain for to be released in 2024 or is there any anticipated delay that would push this into the next workplan for 2025-2027? |
| 6 | EFPIA | We would appreciate the workplan to include plans for when the updated guideline on MDD will be released (not mentioned in current draft 2025-2027, but previously planned for 2024 and public consultation took place March 2024). |
| 7 | EFPIA | Considering that an increasing number of new drug approvals in CNS is within rare diseases (4 out of 11 in 2023), it appears warranted that the CNS WP workplan include plans for guidance, e.g. a reflection paper, about rare diseases in neurology and/or degenerative disorders. |
| 8 | EFPIA | The work plan could benefit from reflecting plans for an EMA guideline on extrapolation for paediatric indications next to ICH E11A. |
| 9 | EFPIA | In follow up from the EMA multistakeholder meeting on psychedelics held in 2024, and with the report mentioning that further EU guidance in this area will be considered, it is suggested at tat the CNS WP workplan reflect these considerations for 2025-2027 as relevant. |
| 10 | EFPIA | EMA should clearly address in their future guideline the expectation around efficacy endpoints and rates of lesion growth in clinical development programs for products treating prevalent diseases with high unmet need in Europe such as geographic atrophy and also glaucoma endpoints. These topics would also be informed by workshops prior to issuing the guideline. |
| 11 | | |
| 12 | | |
| 13 | | |

| | | |
|---|---|---|
| 14 | | |
| 15 | | |

## 2. Specific comments on content of the Workplan

### Section 1. Strategic Goals

**Please include your comments on the long term and short term strategic goals**

| | Line number(s) of the relevant text *(e.g. 20-23)* | Stakeholder name (to be repeated in all rows) | Comment and rationale | Proposed guidance text |
|---|---|---|---|---|
| 1 | 33-36 | EFPIA | It is concerning that Ophthalmology is not explicitly considered in the strategic section of the CNS working party workplan. | Adding a strategic goal regarding Ophthalmology along the lines of the general EFPIA comments above. |
| 2 | 39-40 | EFPIA | Patient-focused drug development is becoming increasingly important, and disease specific guidance documents also need to consider patients' input | Suggest to add "and patients input". |
| 3 | 47-49 | EFPIA | Especially support this strategic goal as several fields within CNS move and develop fast these years. | Ensure that the innovative methods of outcome measurement are adequately described in guidelines in the field of CNS (including gene-therapy, artificial intelligence based methods and novel methods to measure outcome in rare diseases) to increase patient-centricity. |

| | | | | |
|---|---|---|---|---|
| 4 | 54-56 | EFPIA | We very much welcome the reference to building knowledge of new methodologies to measure and define clinical endpoint within the strategic goals of the CNS Working Party. We believe this is fundamental to advancing the development of treatments for neurological and ophthalmological conditions, especially those that follow a chronic, slowly evolving course. We would propose that some specific steps for 2025-2027 are included to reflect this commitment and recommend that the activities listed under 8.2 Training and workshop activities are further strengthened to include a workshop focused on the current status of clinical research and development in neurological conditions, the current landscape, the gaps and challenges and some solutions on next steps to address this. | Build knowledge regarding new methodologies to measure and define clinical endpoints in the field (including endpoints collected in gene editing studies, artificial intelligence based methods, chronic neurological conditions and novel methods to measure outcome in rare diseases). |
| 5 | 69 | EFPIA | We welcome the continued efforts of the EMA to advance the therapeutic area guidelines and highly agree with the focus on updating the final guideline on clinical investigation of medicinal products for the treatment of Parkinson’s Disease. | |

| | | | | |
|---|---|---|---|---|
| 6 | 75-76 | EFPIA | We welcome EMA´s strategic goal to develop guidance on the clinical investigation of medicinal products for the treatment of retinopathies.<br><br>In this context, we propose to further foster a common understanding among regulators, academia, industry, HTA bodies and patient associations on clinical development to help solving methodological issues, such as the choice of outcome measures (including identification of acceptable functional outcomes measures other than best corrected visual acuity).<br><br>In EU, to date, existing anatomic surrogate endpoints (e.g. reduction of GA lesion size growth, and DRSS improvement in NPDR) are currently considered only clinically relevant by regulators and HTA bodies if they are proven to be valid surrogates of visual function, validated through long-term natural history studies. In clinical practice, such functional outcome measures bear challenges in large-scale reproducibility and require long term validation studies. The evidence for surrogate relationships is yet to be established and accepted and new technologies in Data Science may help in a collaborative effort in this direction. | Amend the workplan by supporting advancement of research and innovation in the field of Ophthalmology, especially in retinal diseases with still high medical needs such as Geographic Atrophy, progression to advanced AMD (including both GA and neovascular AMD), Non-proliferative Diabetic Retinopathy (NPDR), and rare or inherited retinal diseases (such as Retinitis pigmentosa). |
| 7 | | | | |
| 8 | | | | |

| | | | | |
|---|---|---|---|---|
| 9 | | | | |
| 10 | | | | |

## Section 2. Tactical Goals

**Section 2.1. Guidance activities**

| | Line number(s) of the relevant text *(e.g. 20-23)* | Stakeholder name | *(to be repeated in all rows)* Comment and rationale | Proposed guidance text |
|---|---|---|---|---|
| 1 | 81 | EFPIA | It would be helpful if Rapporteur's countries could be made public. This would help companies select the most relevant countries for national EU scientific advices. | |
| 2 | 96-99 | EFPIA | The guideline on Alzheimer's Disease should be revised. Especially the part around staging of Alzheimers Disease (based on both biomarkers and clinical) distinction between Alzheimer's Disease in the preclinical phase and the early symptomatic phase (MCI), around estimands, around diagnosis, prognostication and surrogacy related to different newly developed biomarkers, around recommendations for key clinical efficacy and safety assessments and Johnson & Johnson supports NN comment here and proposes minor expansion of comment around expectations for future active comparators/ combination approaches with DMTs once these become available. | Add: "Guideline on the clinical investigation of medicines for the treatment of Alzheimer's disease. CPMP/EWP/553/95 Rev.2" |

| | | | | |
|---|---|---|---|---|
| 3 | 98-102 | EFPIA | This request for the revision of a guidance coming at 10 year of effective implementation is based on three major reasons: (1) the significant progress made over recent years in the understanding of the pathophysiological mechanisms of multiple sclerosis (MS) including their clinical correlates, (2) major evolutions of the treatment landscape, and (3) the change in medical needs of people with MS, including special populations like pediatric-onset MS, in an expanded landscape of EU approved era of multiple approved effective immune therapies. | Addition of -<br>Guideline on clinical investigation of medicinal products for the treatment of Multiple Sclerosis; EMA/CHMP/771815 /2011, Rev. 2<br><br>Target date - TBD |
| 4 | 98-102 | EFPIA | This request for the revision of a guidance for Alzheimer's disease delineating the need for clear expectation in a scientific field where the science/environment is moving rapidly and differing regulatory decision-making frameworks arise globally | Addition of -<br>Guideline on the clinical investigation of medicines for the treatment of Alzheimer’s disease CPMP/EWP/553/95 Rev.2<br><br>Target date - TBD |

| | | | | |
|---|---|---|---|---|
| 5 | 105-106 | EFPIA | There is a critical need for the advancement of research and innovation in the field of ophthalmology. Therefore, we welcome the development of guidance on the clinical investigation of medicinal products for the treatment of retinopathies during the next workplan period 2025-2027. While this is a great step in the right direction, we believe that the progression of regulatory science is needed within even shorter timelines, to match the fast pace at which new molecular entities (NME) have entered clinical development. | Advance target date for concept paper and guideline development. |
| 6 | | | | |
| 7 | | | | |
| 8 | | | | |
| 9 | | | | |
| 10 | | | | |

**Section 2.2. Training and workshop activities**

| | Line number(s) of the relevant text<br>*(e.g. 20-23)* | Stakeholder name<br>*(to be repeated in all rows)* | Comment and rationale | Proposed guidance text |
|---|---|---|---|---|
| 1 | 102, 112 | EFPIA | A common understanding among regulators, academia, industry, HTA bodies and patient associations on ophthalmologic clinical development is needed.<br><br>A workshop sponsored by EMA on ophthalmology has in our view been successful in the past. Given that the last such workshop occurred in 2011, we would encourage the CNS Working Party to consider a similar format in the near future touching on important ophthalmology challenges today. This would offer a platform to address the above- mentioned medical needs and to progress innovation in ophthalmology in the interest of European patients.<br>We are willing to contribute expertise and resources to the proposed multi-stakeholder workshop and to foster the scientific progress in line with the EMA proposed development of a concept paper /guidance. | Conduct a multi-stakeholder Ophthalmology workshop in areas of high medical need such as Geographic Atrophy (and slowing progression to advanced AMD), Non-proliferative Diabetic Retinopathy (NPDR), neuroprotection in the field of glaucoma and rare or inherited retinal diseases<br>Continue to maintain awareness of issues arising in the ophthalmology field (via for example discussion with stakeholders and /or review of scientific advice provided by the EMA) in order to identify the need for review and update of guidelines and development of additional guidance documents. |

| | | | | |
|---|---|---|---|---|
| 2 | 116-118 | EFPIA | The status of clinical endpoints in neurology is not satisfactory, especially since the greatest experience is with tools which may not necessarily be suitable for assessing the progress of neurological disorders at the asymptomatic stage of a chronic disease. We would recommend some additional actions in 8.2 Training and workshop activities to include a workshop to evaluate the current status of clinical endpoints for regulatory (and broader stakeholder) decision-making in chronic neurological conditions, with the aim of reviewing the current landscape (high level) and identify clear next steps. | Maintain awareness of issues arising in the CNS field (via for example discussion with stakeholders and/or review of scientific advices provided by EMA) in order to identify the need for review and update of guidelines and development of additional guidance documents. Include conducting at least one multistakeholder workshop to discuss the current status of clinical endpoints for chronic neurological conditions. |
| 3 | 123 | EFPIA | Regular interactions with patients organisations would be an opportunity for members of the CNS WP to increase their understanding of the patients experiences and needs. | Suggest adding a second bullet point related to the cooperation with patients organisations |
| 4 | 131 | EFPIA | We would like to encourage the EMA and the global stakeholder participating at events noted in sections 8.3.1 & 8.3.2 to share with the research and drug development communities and stakeholder appropriate pre-competitive insights which can help further improve any drug development efficiency across regions | N/A |

| | | | | |
|---|---|---|---|---|
| 5 | 131 | EFPIA | We would like to encourage the CNS working party to amend the section 8.3.2 to include Ophthalmology in the areas of further dialogue with FDA | Participation and contribution to FDA workshops in neurology, psychiatry and ophthalmology |
| 6 | 131 | EFPIA | Suggest adding as a separate bullet "Participation in the US C-Path's neuroscience program, aimed to advance drug development and create better treatments for people living with Parkinson' s, Alzheimer's, and rare neurodegenerative diseases" | |
| 7 | 135 | EFPIA | We would encourage for more specific details for EMA plans on multistakeholder workshops in the CNS area, specifically with emphasis on the planned therapeutic area guidelines (e.g. final guideline on clinical investigation of medicinal products in the treatment of Parkinson's disease). In this regard, we would also propose that EMA considers convening a multi-stakeholder workshop in order to capture scientific progress in this disease area and to inform the planned update of the Parkinson's Disease guideline. | Specific plans for multistakeholder workshops |
| 8 | | | | |
| 9 | | | | |
| 10 | | | | |

**Section 2.3. Communication and stakeholder activities**

| | Line number(s) of the relevant text (e.g. 20-23) | Stakeholder name (to be repeated in all rows) | Comment and rationale | Proposed guidance text |
|---|---|---|---|---|
| 1 | 135 | EFPIA | We welcome the collaboration at an international level. We would encourage greater collaboration in certain areas. Of note, the launch of the Rare Disease Endpoint Advancement (RDEA) pilot program by the FDA in 2023 which aims to advance rare disease drug development programs by providing a mechanism for sponsors to collaborate with the regulatory agency throughout the efficacy endpoint development process, may be an area that would benefit from greater international collaboration. | Participation and contribution to FDA workshops and other initiatives in neurology and psychiatry. |
| 2 | 133-135 | EFPIA | Considering facilitation of global development plans and expectations across EMA and FDA, please could the CNS WP provide for transparency on the more specific plans for collaboration with the FDA e.g. including the FDA neuroscience CoE. | Participation and collaborative efforts across FDA and EMA |
| 3 | | | | |
| 4 | | | | |
| 5 | | | | |
| 6 | | | | |
| 7 | | | | |
| 8 | | | | |

| | | | | |
|---|---|---|---|---|
| 9 | | | | |
| 10 | | | | |

**Section 2.4. Multi-disciplinary collaboration**

| | Line number(s) of the relevant text (e.g. 20-23) | Stakeholder name (to be repeated in all rows) | Comment and rationale | Proposed guidance text |
|---|---|---|---|---|
| 1 | | | | |
| 2 | | | | |
| 3 | | | | |
| 4 | | | | |
| 5 | | | | |
| 6 | | | | |
| 7 | | | | |
| 8 | | | | |
| 9 | | | | |
| 10 | | | | |

## Section 3. Operational Goals

**Please include your comments about pre-submission activities, evaluation and supervision activities**

| | Line number(s) of the relevant text (e.g. 20-23) | Stakeholder name (to be repeated in all rows) | Comment and rationale | Proposed guidance text |
|---|---|---|---|---|
| 1 | | | | |
| 2 | | | | |
| 3 | | | | |
| 4 | | | | |
| 5 | | | | |
| 6 | | | | |
| 7 | | | | |
| 8 | | | | |
| 9 | | | | |
| 10 | | | | |

# Thank you

Thank you for your contribution.


**Contact**

Contact Form