

Expert Opinion on Orphan Drugs: Author Guidelines for Original Research

1. Overview

Expert Opinion on Orphan Drugs is a monthly, peer reviewed journal providing the pharmaceutical and academic communities with comprehensive coverage of the emerging literature on, and experience with orphan drugs. The journal covers all aspects of rare diseases, including investigational, pre-launch and launched therapies for rare indications.

The following document details the requirements for 'Original Research' submissions.

1.1 Audience

The journal audience consists of research scientists and managers in the healthcare and pharmaceutical industries, academic pharmaceutical scientists and related professionals.

1.2 Scope

In addition to review papers, drug evaluations and editorials, Expert Opinion on Orphan Drugs is also a medium for the publication of original research on early-phase drugs; therapies on the market; therapies nearing the end of their clinical development; post-launch studies and HE/OR studies.

Contributions are welcomed in the form of:

- Full-length primary research papers reporting the original results of investigations on orphan drugs that are in Phase IIa, IIb, III and IV clinical trials, as well as post-marketing studies, health-economic studies and observational research on launched drugs.
- Studies on drugs that are under review for NDA or have been granted Orphan Drug Status.
- Studies with a strong link to clinical practice, both in primary and secondary (hospital) care settings, particularly those that have significant implication for clinical prescribing by primary care physicians and general practitioners, as well as hospital physicians and specialists.

Supplements are published and actively encouraged. These include both traditional free-standing and in-journal supplements based on symposia proceedings (or sponsored workshops and meetings), and collected articles on specific drugs or disease states. Enquiries from potential guest editors, meetings organizers and sponsors are welcomed.

In addition, the journal has a Correspondence section. We welcome the submission of any correspondence piece or letters regarding any article published in the journal since its launch. The Correspondence section of the journal presents an open arena for objective commentary, debate and lively discussion.

For more information regarding a Supplement or for the submission of a Correspondence piece, please send an email to Lizzie.howard@informa.com.

1.3 Rapid Publication

Publication is driven entirely by editorial considerations and independent authoritative peer review. As part of the journal's responsive approach to the publication of clinical evidence, we offer two prioritised modes of rapid publication and a third non-prioritised mode:

- **FastTrack:** This offers the most highly prioritised service, with a submission to online publication timeline of 5–7 weeks (subject to 1–2 week author revision following initial peer-review and prompt turnaround of proofs). There is a publication support fee for this, based on a charge of £550 / €625 / \$850 per published page. This charge supports the ultra-swift processing of material and 15 downloads of the article via access-tokens.
- **Rapid Track:** This prioritised service offers submission to online publication in 10–12 weeks and is subject to a publication support fee of £260 / €300 / \$400 per page; 15 downloads of the article via access-tokens are provided for this service.
- **StandardTrack:** This non-prioritised standard service provides submission to online publication up to 20 weeks; there are no publication support fees charged for this mode.

We welcome contact with the Editorial Offices preceding formal submission, and particularly encourage prior contact for FastTrack submissions where a particular publication deadline is desired.

2. Submitting a manuscript

All material should be prepared as detailed below. Please refer to the Submission Checklist at the end of this document and ensure that all criteria are met before submission. This will ensure timely publication of your article.

Submissions should conform to the latest version of the Uniform Requirements for Manuscripts Submitted to Biomedical Journals, prepared by the International Committee of Medical Journal Editors (ICMJE: <http://www.icmje.org/>). Although it is recommended that authors read the entire Uniform Requirements document, in particular, authors and contributors are referred to the following sections/paragraphs of this document: II A.1 and II A.2. – Notes defining and distinguishing authorship and contributorship.

II .D.1. – Peer review

II. E.1. – Patients and study participants

II.F – Protection of human subjects in research

IV.A.3 – Conflict of interest notification

Please submit manuscripts in Microsoft Word (double spaced, Times New Roman, 12 pt) using the Manuscript Central online submission system (<http://mc.manuscriptcentral.com/eood>). As the peer reviewers will be blinded to the identity of the authors, contributors MUST provide a copy of the manuscript containing no author names or affiliations. These details should be provided on a separate cover sheet. Pages must be numbered.

The declaration of interest statement must be returned to the Editor upon submission (see end of this document).

Authors sending incomplete submissions may be asked to rectify and resubmit the paper in case of any omissions or serious failures to follow the journal style.

Prior publication: the Journal will only consider work that has not been previously published in full. Abstract, poster or oral presentations do not constitute prior publication, but should be mentioned in the covering letter and details included as a footnote on the manuscript title page.

Trial registration: the Journal does not require trial registration as a condition for publication. However, as per the ICMJE requirements, if a study that is the subject of the manuscript has been registered, this should be mentioned in the covering letter and trial registration information included at the end of the Abstract; publication of a trial registry summary should also be mentioned on the manuscript title page and the details provided as a reference citation.

In the instance where preclinical research/work with animal subjects is included within the original research submission, contributors are required to follow the procedures in force in their countries which govern the ethics of work done with human or animal subjects. The Code of Ethics of the World Medical Association (Declaration of Helsinki) represents a minimal requirement. In particular:

When experimental animals are used, state the species, strain, number used, and other pertinent descriptive characteristics.

When describing surgical procedures on animals, identify the pre anaesthetic and anaesthetic agents used and state the amount of concentration and the route and frequency of administration for each. The use of paralytic agents, such as curare or succinylcholine, is not an acceptable substitute for anaesthetics. For other invasive procedures on animals, report the analgesic or tranquilizing drugs used; if none were used, provide justification for such exclusion.

When reporting studies on unanaesthetized animals or on humans, indicate that the procedures followed were in accordance with institutional guidelines.

3. Correspondence relating to the submission of papers

All manuscripts must be accompanied by a covering letter and an electronic version of the Disclosure Form signed by the principal author(s). The covering letter should include all of the following information:

- The name and contact details (telephone, fax, postal and email addresses) of the corresponding author who will deal with the comments from reviewers and approve final proofs.
- A statement that the contribution represents original work that has not been previously published or simultaneously submitted for publication elsewhere.
- A statement that the manuscript has been read and approved by all the authors, and that all the conditions as previously stated by the ICMJE have been met.
- The body providing explicit ethical approval of the work reported should also be stated.
- A statement of financial or other relationships of a declarable nature (i.e., commercial associations that might lead to a conflict of interest), including disclosure of sources of support in the form of sponsorship, grants, materials (drugs) or equipment, and editorial or manuscript support (see also Acknowledgements under Section 4.2). In case there are no disclosures of a declarable nature, or acknowledgements for financial or editorial support, this should be clearly stated.
- The authors' preference for publication with either English (UK) or US spellings.

Please note that manuscripts without a covering letter or a Disclosure Form will not be considered to be 'complete' submissions. All complete submissions will be acknowledged by the Editorial Office upon receipt.

For a detailed description of all the journal's editorial policies, including plagiarism, authorship, patient privacy, and redundant publication, authors are referred to the editorial policy document on the [journal's website](#).

4. Manuscript content

4.1 General

Manuscripts submitted should be in English and written for an international, general medicine readership. Where national or regional issues are discussed, the international context should also be considered. When a licensed drug or device is being discussed outside its licensed indication, this must be made clear to the reader.

Manuscripts should be double-spaced with numbered pages. Keep the layout as simple as possible – it will be set to house style during the production process.

4.2 Every article must contain:

- **Title page:** All articles should have a concise, informative title that contains no brand names. In addition to the title itself, the title page should also have the name(s) and initials of all the authors and their institutional affiliation(s). The name of the corresponding author and their mailing address should be given in full, including email, telephone and fax numbers. Should any of the material contained in the paper have been previously presented at a meeting, the full name, location and inclusive dates of the meeting should also appear on the title page. Authors are to avoid using Trade names in their titles, and are encouraged to have non-promotional titles for their articles.
- **Abstract:** The second page should contain a brief, structured abstract of the paper (no more than 200 words) summarizing the main facts, findings and principal conclusions. Suggested headings: Objective (including the reason for the study); Research design and methods (including study population and setting, study blinding, comparators, dosage, treatment regimens and durations, efficacy and safety issues); Main outcome measures; Results (both efficacy results and adverse events should be given); Conclusions (qualified by any key limitations). No references are to be cited in the Abstract. Please bear in mind that the Abstract needs to accurately reflect the content of the article and that the aim of the Abstract is to draw in the interested reader.
- **Keywords:** 4–6 keywords, listed in alphabetical order, are required to assist indexers in cross-referencing. The keywords will encompass the therapeutic area, mechanism(s) of action, key compounds and so on.

- **Body of the article:** This should include the following sections: introduction; patients and methods; results; discussion; conclusions (see section 5 for organization of content).
- **Acknowledgements:** This must include any declaration of interest by the authors (see also Section 3: Correspondence), including grants, fellowships or any commercial assistance or financial sponsorship received. It should also list any affiliation(s), organization(s) or entity(ies) that are relevant to the work reported. Some or all of this information may be published at the discretion of the Editor. Any contributions to the research, data analysis or assistance in manuscript preparation must also be acknowledged in this section. Finally, if any trial registration information and/or a trial registration number are available for the study, this should also be mentioned in the acknowledgements section.
- **References:** Full references to relevant material cited in the text should be provided (see Reference style in Section 9 below). References should be comprehensive, accurate and up-to-date: wherever possible, please use primary references, and as far as possible, avoid the use of unpublished references (such as 'Data on file' or 'Poster').

5. Organization of content

5.1 General

Keep all formatting to a minimum and avoid assigning 'styles' to headings, extracts or paragraphs. Make sure that the 'normal' style is used throughout the text. Turn off the automatic hyphenation feature. Please use double-line spacing throughout the manuscript. Headings and sub-headings should be used to provide structure to the text with numbers (Arabic numerals) to indicate a hierarchy of the headings/sub-headings (i.e., 1.0, 1.1, 2.0, 2.1, 2.1.1, 2.1.2 and so on).

5.2 Units and symbols

Use SI units where possible. Non-SI units should be accompanied by SI equivalents, for example, LDL-C < 70 mg/dl (1.8 mmol/l). To indicate atom positions in a molecule, use the convention C-1, C-2 and so on.

5.3 Spelling

The Journal will ensure that consistent British (The Concise Oxford Dictionary) or American (Webster's Dictionary) spellings are used. If you have a preference for one or other spelling, please clearly state this in your covering letter. All accepted manuscripts will be copyedited in house upon acceptance, but authors are advised to check their work for English spelling and grammar prior to submission. **The Editorial Office can arrange an English language edit prior to submission if required - please contact the editorial office for details of available services and costs.**

5.4 Abbreviations

Abbreviations should be defined on their first appearance both in the Abstract and in the text; commonly used abbreviations need not be defined. Authors are encouraged to submit a list of abbreviations used to the Editorial Office alongside the manuscript. Only underline or italicize words and letters that are required to appear in italics.

5.5 Introduction

This should state the clinical relevance and background to the study, its rationale and purpose.

5.6 Patients and methods

This should contain details of the study population and setting, subject selection (inclusion/exclusion criteria), methods of randomization and blinding, and efficacy and safety measures. The study design and statistical methodology should be described, with justification for the choice of analysis and sample size given; CONSORT guidelines (www.consort-statement.org) should be considered where appropriate. All materials should be identified precisely, with drugs referred to by their generic names (proprietary names, if required, should be given in parentheses along with the company name and country of the manufacturer), and with dose and routes of administration. The ethical approval procedure followed and the name of the ethics committee should be stated. Indicate how adverse events were determined (and by whom) and indicate if/how compliance was measured. (Authors are reminded to ensure that they follow the ICMJE requirements when dealing with privacy and informed consent from patients – see Section II.E.1. of the ICMJE requirements document <http://www.icmje.org/#privacy>).

5.7 Results

Use should be made of tables and figures to help with the clear presentation of results data. The sample size of each data point should be shown, with p-values and confidence intervals quoted for significant findings. Any data not included in the analysis (including patients withdrawn from the study) should be detailed. Details of data on efficacy and adverse events should be provided in a balanced fashion.

5.8 Discussion

This will essentially be a discussion of the results and experimental data collected. The section should include implications of the findings and their limitations, with reference to all other relevant studies and the possibilities these suggest for future research. In addition, the discussion affords authors the opportunity to discuss the developments that are likely to be important in the future and the avenues of research likely to become exciting as further studies yield more detailed results.

5.9 Conclusions

This section must summarize the paper, with a concise statement of the clinical implications of the study results. Ensure that extrapolations are reasonable and that conclusions are justified by the data presented. Indicate if the study design can be generalized to a broader patient population.

6. Tables

Each table (with a brief, comprehensive title/header) should be presented in cellular format (rather than a simple tabbed form) on a separate page, at the end of the text. Tables should be numbered with Arabic numerals (Table 1, Table 2....) and each table should be followed by a footnote where necessary. Define in the footnotes all abbreviations that are used in the table. Be sure that each table is cited in the text (but not within any section headings) and number tables consecutively in the order of their first citation in the text. In case data from another published or unpublished source is being used, please obtain permission and acknowledge the source(s).

7. Figures & Structures

Do include illustrations (figures/diagrams/structures) as appropriate. Please ensure that the following recommendations are adhered to as closely as possible:

- Provide editable, electronic copies if possible; otherwise, please ensure that the camera-ready copy is of the highest resolution available.
- Please submit figures as eps, illustrator, jpeg, ISISdraw or ChemDraw format. If these formats are not possible, figures can be submitted in PowerPoint or Word as a last resort.
- Figures should employ CMYK rather than RGB color scheme.
- Small figures should be 300 dpi and large figures should be 72 dpi.
- Please note that we cannot improve resolution beyond that of the file submitted.
- Figures and structures should be in separate files to the text. It is unnecessary to incorporate the figures into the body of the manuscript. If there are several figures, please submit these individually, rather than as one file (preferably the original source files).
- Ensure that titles and figure legends/captions are included on a fresh page at the end of the manuscript. Legends (and labeling) should be based on the 'stand-alone concept', so that looking at the tables and figures conveys an immediate, clear message without needing to refer to the text. Define in the legend(s) all abbreviations that are used in the figure(s).
- Figures should be numbered consecutively according to the order in which they have been first cited in the text.
- If a figure has been previously published, acknowledge the original source and please submit written permission from the copyright holder to reproduce the material.
- Colour figures will only be accepted if colour is deemed to be essential, at the discretion of the Editor-in-Chief and Publisher. Use of colour in the printed journal will entail a charge on a scale related to the additional costs of colour reproduction.
- Please submit chemical structures drawn in ISISDraw or Chemdraw and ensure that you submit these in their original, editable formats. Always indicate stereochemistry, and where necessary, use the wedge and hash bond convention for chiral centres and mark cis/trans bonds as such.

The Editorial Office can arrange for figure re-drawing and medical illustration - please contact us for details of available services and costs.

8. Companies and drug proprietary names

- Companies are treated as single entities requiring a verb in the third person singular (e.g., GSK is developing an AII antagonist).
- All medical substances should be referred to by their Recommended International Non-Proprietary Name (rINN). Proprietary or individual company trade names should only be mentioned where absolutely essential, for clarity, as in the case of combination products, or to avoid confusion, for example, between different formulations.
- Drug brand names should not appear in paper titles.
- In the body of an article, if it is necessary to use a brand name, when referring to a lead compound (or compounds claimed in patents) for the first time, please ensure that the ® or TM symbols are used as required, and that the name of the relevant company is also stated. Generic names always take a lower case first letter unless they are beginning a new sentence.

9. References

Articles should principally review recent primary literature and scientific meeting reports, rather than patents, although relevant patent information may be included where appropriate.

Websites of interest may also be referenced. Occasional 'historic' papers may be cited.

Ensure that all key work relevant to the topic under discussion is cited in the text and listed in the bibliography.

9.1 Numbering

References MUST be numbered consecutively, using Arabic numerals in square brackets, in the order in which they are first mentioned in the text. The reference list should appear in the *same sequence* as the numbers in the text.

9.2 Annotations

Papers or patents of particular interest should be identified using one or two asterisk symbols (• = of interest, ** = of considerable interest), and annotated with a brief sentence explaining why the reference is considered to be of interest.

9.3 Bibliography

References can be formatted using EndNote or Reference Manager according to the style of *Current Medical Research and Opinion*.

References use plain, unformatted text, as in the following examples:

Journals:

1. Weissman P, Goldstein BJ, Rosenstock J, et al. Effects of rosiglitazone added to submaximal doses of metformin compared with dose escalation of metformin in type 2 diabetes: the EMPIRE Study. *Curr Med Res Opin* 2004;21:2029-35

Books:

2. Gottman J. *Time Series Analysis*. Cambridge: CUP, 1981

Working party reports and similar:

3. Clinical Disputes Forum Working Party. *Pre-action protocol for the resolution of clinical disputes*. London: Clinical Disputes Forum, 1998

Pre-publication articles assigned DOI numbers:

4. de Lau LM, Koudstaal PJ, Hofman A, Breteler MM. Subjective complaints precede Parkinson disease: the Rotterdam study. *Arch Neurol* 2006; published online 9 January 2006, doi:10.1001/archneur.63.3.noc50312

Internet articles and website information:

5. Suicidality in adults being treated with antidepressant medications. FDA Public Health Advisory. Washington, DC: FDA/Center for Drug Evaluation and Research, 2005. Available at: www.fda.gov/cder/drug/advisory/SSRI200507.htm [Last accessed 3 January 2006]

Patents:

6. Basf AG. Means and methods for preventing and treating caries. WO2006027265 (2006)

Use the following formats for patent numbers issued by the World, US and European patent offices, respectively: WO0113324; US6803189; EP1549318

Note, for citations with four or fewer authors/assignees, cite all names; for citations with more than four authors, cite three author names plus et al.

Full reference details must be provided in the bibliography (for example, for journal citations, author surnames and initials, article title, journal name, year, volume, page range). Failure to do so may lead to a delay in publication or a return of the paper by the Editor to the author.

10. Copyright assignment and disclosure

Papers are published on the understanding that their copyright is assigned to the Publisher (Informa UK Ltd) once they are accepted for publication. If there are any problems with this, this should be raised with the Editor as soon as possible.

If any material used is subject to third-party copyright, copyright clearance is the sole responsibility of the authors and must be supplied in writing to the Publisher.

It is the responsibility of authors to disclose any affiliation with any organisation with a financial interest, direct or indirect, in the subject matter or materials discussed in the manuscript (such as consultancies, employment, expert testimony, honoraria, speakers bureaus, retainers, stock options or ownership) that may affect the conduct or reporting of the work submitted. If uncertain as to what might be considered a potential conflict of interest, authors should err on the side of full disclosure. Information about potential conflict of interest may be made available to reviewers and may be published with the manuscript at the discretion of the Editor.

Corresponding authors will be sent a Copyright Form (and a Disclosure Form, if this has not already been received) to sign on acceptance of their paper. The Disclosure Form (see end of this document) would serve as a signed guarantee that:

1. The manuscript submitted represents original work and has not been previously published or simultaneously submitted elsewhere for publication.
2. Conditions as previously stated by the ICMJE; <http://www.icmje.org> have been met, and the body providing explicit ethical approval of the work reported has been stated in the manuscript.
3. Trial registration information and a trial registration number (if available) for the study is mentioned in the Acknowledgements section.
3. The manuscript has been read and approved by all authors.
4. Any potential conflicts of interest and sources of funding are disclosed, ethical approval for the study has been obtained and that any sources of contributory support (bio statisticians, medical writers and so on) in the preparation of the manuscript are acknowledged.

10.1 NIH/Wellcome-funded research

Regarding articles that are funded by the [NIH](#) and the [Wellcome trust](#): we allow authors to deposit the POST-print manuscript (peer-reviewed and revised, formally-accepted version) on Pub Med Central ([PMC](#)). Note that the POST-print version is not the Publisher's final PDF.

Depositing the final, accepted manuscript on PMC will be the authors' responsibility, and will need to be done via the NIH Manuscript Submission System ([NIHMS](#)). Some steps on how to deposit your manuscript are listed under NIH's 'Method C' [here](#). Publicly funded articles can be deposited by authors on the PMC site immediately on receipt of formal acceptance of the manuscript. When prompted about the number of months after publication when the manuscript may become publicly available in PMC, select 12 months for NIH-funded work and 6 months for research funded by the Wellcome Trust.

11. Proofs and publication

Proofs in the form of Adobe Acrobat PDF files will be sent to the corresponding author via email for checking. If, because of delays, time is limited, the Publisher reserves the right to have proofs checked against original manuscripts by editorial staff or medical advisers. No major alterations to text will be accepted at proof stage. Electronic publication online will constitute formal publication of the article, with all subsequent critical changes or error correction being dealt with via publication of a formal Erratum or Corrigendum.

12. Submission checklist

Please use the checklist below to help prepare your submission or resubmission. It will help us to swiftly process your manuscript through production and avoid any unnecessary delays at a later stage.

If you have any queries, please feel free to contact:

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1. Manuscripts submitted online at <http://mc.manuscriptcentral.com/eood>
Include: i) covering letter uploaded as 'file not for review', ii) completed Disclosure Form, iii) attachment of the Original Research article (containing no author details) (in MS Word, double spaced, Times New Roman, 12 pt, pages numbered) and iv) any separately attached image files.
2. Cover letter includes:
 - a) Name and contact details of all authors, including corresponding author.
 - b) A statement that the contribution represents original work, has not been previously published or simultaneously submitted for publication, that the manuscript has been read and approved by all authors, and that all the conditions as previously stated by the ICMJE have been met.
 - c) A statement of financial or other relationships of a declarable nature.
3. A structured abstract is provided.
4. Four to six keywords are provided.
5. Section headings follow EOBT style and all articles contain: Introduction; Methods; Results; Discussion and Conclusion (see Author guidelines: Section 5).
6. No propriety drug names or brand names are used (or, these are used only when absolutely necessary, with a ® or TM sign and the company name included along with the first appearance of the trade name).
7. An Acknowledgment section includes any declaration of interest by the authors, including grants, fellowships, or any commercial assistance or financial sponsorship received or of any affiliation, organization or entity which is relevant to the work reported. Any contributions to the research, data analysis or assistance in manuscript preparation should also be acknowledged in this section.
8. References follow EOBT style (Author guidelines: Section 9). Reference annotations are present in the reference list.
9. All figures and tables cited in the text are supplied. No figures or tables appear without being cited in the text.
10. Tables are in cellular format, appearing at the end of the manuscript (following the references)
11. All figures are supplied as editable, separate files with the legends appearing on a fresh page at the end of the manuscript.

13. Editorial support services

The Editorial Office is available to advise authors throughout the writing and submission process. If required, we can also arrange for professional medical writing and illustration assistance, including:

- Pre-submission language editing
- Comprehensive re-formatting
- Detailed technical editing
- Figure redrawing
- Medical illustration

If you are interested in the above services, please contact the Editorial Office for further details and costs. Please note, these services are independent of the peer review process and undertaking such work does not guarantee final acceptance of the manuscript.