8 December 2020
EMA/2677/01 Rev.3[1]
Human Medicines

Procedural advice on appeal procedure for orphan medicinal product designation or review of orphan designation criteria at the time of marketing authorisation

Legal basis

In accordance with Article 5(7), Regulation (EC) No 141/2000 of 16 December 1999, where the opinion of the Committee for Orphan Medicinal Products (COMP) is that an application does not satisfy the criteria for orphan medicinal product designation, the European Medicines Agency (EMA) shall forthwith inform the sponsor. Within 90 days of the receipt of the opinion, the sponsor may submit detailed grounds for appeal, which EMA shall refer to the COMP. The Committee shall consider whether its opinion should be revised at the following meeting.

Appeal procedure

- Upon adoption of an opinion on orphan medicinal product designation or on the review of orphan designation criteria at the time of Marketing Authorisation (MA), EMA will communicate the opinion to the sponsor together with a copy of the COMP summary report.
- The sponsor may inform EMA of any intent to appeal, without delay after receipt of the opinion, by giving written notice to EMA.
- The COMP will appoint a new COMP rapporteur for the appeal procedure. If necessary, the COMP may appoint additional experts.
- Detailed grounds for appeal must be submitted by the sponsor within 90 days of receipt of the COMP opinion, and preferably 14 days before the COMP plenary meeting at which the appeal will be discussed. The grounds for appeal should be submitted to the EMA via the IRIS portal.
- EMA will refer the grounds for appeal to the COMP immediately after receipt.
- The sponsor will be invited to an oral explanation before the COMP at the meeting following the receipt of the grounds.
- An ad-hoc expert meeting may be convened, as necessary.

[1] Eudralink was replaced by the IRIS portal (submission, upload of documents and communication with EMA). Publication of OMAR was added to the review of orphan designation procedure.

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

- The EMA co-ordinator, in association with the COMP rapporteur, will update the COMP summary report. The revised summary report will be available to COMP members and appointed by EMA expert(s) for comments.
- The COMP, at the first meeting following the sponsor's submission of the grounds for appeal, having reviewed the detailed grounds for appeal and having heard the oral explanation of the sponsor, will consider whether the opinion should be revised and will adopt a final COMP opinion. Where possible the expert(s) involved in the procedure will be invited to attend the COMP discussion.

Post-appeal opinion

- EMA will communicate the final opinion to the European Commission (EC) and the sponsor.
- The following steps conclude the procedure:

Orphan designation	Review of orphan designation criteria at the time of marketing authorisation
• A decision will be adopted by the EC, within 30 days of its receipt of the final opinion. • Upon a favourable decision the designated medicinal product shall be entered in the EU Register of Orphan Medicinal Products.	• In case of a final negative opinion the designated orphan medicinal product shall be removed from the EU Register of Orphan Medicinal Products in accordance with article 5(12)(b) of Regulation (EC) No 141/2000 of 16 December 1999.
• Following the EC decision a public summary of opinion will be published on the EMA website.	• Following the sponsor's review, an orphan maintenance assessment report (OMAR) will be published on the EMA website.