Guidance

How the MHRA will manage orphan medicinal products from 1 January 2021 in Great Britain (GB)

The MHRA will review applications for orphan designation at the time of a marketing authorisation (MA) or variation application.

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From:
Medicines and Healthcare products Regulatory Agency (https://www.gov.uk/government/organisations/medicines-and-healthcare-products-regulatory-agency)

New rules for January 2021

The UK has left the EU, and the transition period after Brexit comes to an end this year.

This page tells you what you'll need to do from 1 January 2021. It will be updated if anything changes.

For current information, read: Apply for a licence to market a medicine in the UK (https://www.gov.uk/guidance/apply-for-a-licence-to-market-a-medicine-in-the-uk)

You can also read about the transition period (https://www.gov.uk/transition).

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The MHRA will offer incentives in the form of market exclusivity and full or partial refunds for marketing authorisation fees to encourage the development of medicines in rare diseases. Waiver from scientific advice fees will also be available for UK based SMEs.

See the UK Rare diseases strategy (http://www.gov.uk/government/publications/rare-diseases-strategy) for other supportive activities.

Application process

The MHRA will be responsible for reviewing applications from companies for orphan designation at the time of a marketing authorisation application (MAA). There is no pre-marketing authorisation orphan designation. To qualify for orphan designation in an orphan condition, a medicine must meet the following criteria:

- it must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating
- the prevalence of the condition in Great Britain (GB) must not be more than 5 in 10,000, or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development
- no satisfactory method of diagnosis, prevention or treatment of the condition concerned exists in GB, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition
    - Satisfactory methods may include authorised medicinal products, medical devices or other methods of diagnosis, prevention or treatment which are used in GB.

How to apply

Marketing Authorisation Applicants should submit the Great Britain Orphan Drug Designation Application Form (https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/913535/GB_Orphan_Drug_Application_Form.docx) (MS Word Document, 831KB) with their MAA in module 1.2 of the eCTD, specifically indicating in the cover letter their intention to seek an orphan designation.

Applications for orphan designation will be examined by the MHRA's advisory committee, the Commission on Human Medicines (CHM) (https://www.gov.uk/government/organisations/commission-on-human-medicines). A decision on fulfilment of the orphan criteria runs in parallel with the assessment of the marketing authorisation procedure.

Following the validation of the MAA, a decision on orphan status will be made at the time of the decision on approval of the marketing authorisation (See guidance on new assessment procedures (https://www.gov.uk/guidance/guidance-note-on-new-assessment-routes-in-a-no-deal-scenario)). Any questions concerning the fulfilment of the orphan designation criteria will be raised with the company during the evaluation of the MAA.

If the MHRA concludes that the criteria for orphan designation are not met, there will be an opportunity to appeal the decision to the CHM before the Marketing Authorisation (MA) is granted. The applicant should inform the MHRA of the intention to appeal as soon as possible.

Market exclusivity period

On grant of a marketing authorisation with orphan status, the medicinal product will benefit from up to 10 years of market exclusivity from similar products in the approved orphan indication.

The start of this market exclusivity period will be set from the date of first approval of the product in GB or EU/EEA.

Market exclusivity periods for centrally authorised orphan medicine marketing authorisations that are converted to UK marketing authorisations will continue to apply.

Paediatric indications

Orphan medicines authorised in GB with the results of studies from a paediatric investigation plan (PIP) included in the product information are eligible for an additional 2 years of market exclusivity.

Variation applications (section 4.1 of the Summary of Products Characteristics)

Marketing authorisation holders with orphan status are required to submit the with their variation MA application for new or extensions to orphan therapeutic indications. The orphan criteria will be assessed in parallel to the approval of the new indication.

A new period of market exclusivity is only given if the applied for therapeutic indication falls within a new orphan condition.

For non-orphan indications, a new marketing authorisation application is required.

Fees

There are no additional fees to apply for orphan designation. Information on fee waivers and refunds for orphan medicines is provided in the amended MHRA application fees (https://www.gov.uk/government/publications/mhra-fees/current-mhra-fees).

SME status

Companies who have, or intend to seek, SME status should ensure that they have the relevant documentation in place if an SME fee refund is to be applied for.

Orphan register

All medicines that gain a GB orphan marketing authorisation will be listed on the GB Orphan Register (active and then withdrawn, suspended or expired).

This guidance will apply from 1 January 2021 in line with the Human Medicines Regulations (Amendment etc.) (EU Exit) Regulations 2019 (http://www.legislation.gov.uk/uksi/2019/775/contents/made).

Contacts

For specific queries on orphan medicines and orphan designation please contact orphan@mhra.gov.uk.

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Transition period

Find out what it means for you (https://www.gov.uk/transition)

Related content

- Rare diseases strategy (https://www.gov.uk/government/publications/rare-diseases-strategy)
- UK strategy for rare diseases: implementation plan for England (https://www.gov.uk/government/publications/uk-strategy-for-rare-diseases-implementation-plan-for-england)
- UK rare disease policy board: second progress report (https://www.gov.uk/government/publications/uk-rare-disease-policy-board-second-progress-report)
- UK strategy for rare diseases: 2019 update to the implementation plan for England (https://www.gov.uk/government/publications/uk-strategy-for-rare-diseases-2019-update-to-the-implementation-plan-for-england)
- Supplying medicines to Northern Ireland from 1 January 2021

(https://www.gov.uk/guidance/supplying-medicines-to-northern-ireland-from-1-january-2021)

Collection

- MHRA post-transition period information (https://www.gov.uk/government/collections/mhra-post-transition-period-information)

Explore the topic

- Marketing authorisations, variations and licensing guidance (https://www.gov.uk/topic/medicines-medical-devices-blood/marketing-authorisations-variations-licensing)