3

Orphan Medicine Development in the UK

History[1-3]

On 20 January 2020, the United Kingdom (UK) left the European Union (EU), and the transition period after Brexit came to an end in December 2020. The orphan guidance came into effect on 1 January 2021. The Medicines and Healthcare products Regulatory Agency (MHRA) will be responsible for reviewing applications for orphan designation at the time of a marketing authorization application. The Commission of Human Medicines (CHM), MHRA's advisory board, examines orphan designation applications. The rare disease prevalence in the UK must not be more than five in 10,000 people, or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development.

Benefits[4]

The MHRA offers the following incentives to sponsors of orphan medicines:

- Market exclusivity
- Full or partial refunds for marketing authorization fees
- Waiver from scientific advice fees will also be available for UK-based small and medium enterprises (SME)

Tools

The MHRA has made several tools available for sponsors to utilize during the different stages of drug development for orphan medicines. These tools help guide and expedite the drug development process.

Conditional Marketing Authorization Applications[5]

The MHRA has introduced a national conditional marketing authorization (CMA) scheme for new medicinal products in UK effective from 1 January 2021. The eligibility criteria for this scheme, as that of the EU scheme, is intended for medicinal products that fulfill an unmet medical need and for serious and life-threatening diseases where no satisfactory treatment methods are available, or where the product offers a major therapeutic advantage.

The MHRA determines eligibility for a CMA at the time of MAA assessment. The MHRA does not have a specific application route for a CMA. The sponsor needs to file the MAA dossier for a full marketing authorization. At the completion of the MAA dossier assessment, the MHRA will determine whether to approve the application and grant a conditional MA or whether the benefit-risk ratio is negative and reject the application. The CMA may be granted where comprehensive clinical data is not yet

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complete and available. The sponsor must provide justification for a CMA, including the ongoing clinical studies' status and timing of the availability of comprehensive clinical data. CMAs are valid for one year and can be renewed annually

Exceptional Circumstances Marketing Authorizations[6]

From 1 January 2021, the MHRA's existing scheme for applications under exceptional circumstances will continue to be available for medicines where a comprehensive data package cannot be provided because the condition to be treated is rare or because the collection of full information is not possible or is unethical. This scheme has the same eligibility criteria as the EU scheme (see **Chapter 2**). The designation of a product as being eligible for an exceptional circumstances scheme by EMA or another jurisdiction may be taken into account by the MHRA, but the final decision on eligibility of the product for the GB scheme will rest with MHRA.

National Scientific Advice[7]

From 1 January 2021, the MHRA continues to offer its national scientific advice service to developers of medicinal products. The sponsor can apply at any stage of development. Applications for scientific advice submitted by UK-based SMEs are exempt from the fee. Requests for advice that is purely regulatory in nature will remain free of charge.

Early Access to Medicines Scheme[8-11]

The Early Access to Medicines Scheme (EAMS), often referred to as compassionate use, aims to give patients with life-threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need. Unauthorized medicinal products may be available through an approved clinical trial protocol. This program should be availed if no satisfactory treatment option exists or a patient is not eligible for the clinical trial; through this scheme, the sponsor receives a scientific opinion from the MHRA, based on the data available when the EAMS submission was made. The opinion lasts for a year and can be renewed. The scheme is voluntary, and the opinion from the MHRA does not replace the normal licensing procedures for medicines. The scientific opinion will be provided after a two-step evaluation process.

Step 1: The Promising Innovative Medicine (PIM) Designation

The sponsor can apply with data from nonclinical studies and from early stages of clinical development, indicating that the medicinal product fulfills the designation criteria of demonstrating significant benefit for patients suffering from life-threatening or seriously debilitating conditions. An MHRA scientific designation meeting is conducted on the basis of nonclinical and clinical data available on the product in a defined disease area. Once the drug receives the designation, the sponsor needs to complete the clinical development program(s) within a reasonable time period in order to continue with an application under the EAMS (Step 2). The sponsor also can apply for a joint PIM designation/presubmission meeting by submitting a PIM designation application template and a presubmission meeting template at the time of the request. The MHRA conducts a meeting within four weeks of the request.

Step 2: Scientific Opinion

The sponsor needs to have a promising innovative medicine (PIM) designation to enter this step. The sponsors also should attend a presubmission meeting with the MHRA to ensure the product is suitable for an EAMS scientific opinion application and to discuss the format of the data that the sponsor need to submit to support the opinion. To apply for the scientific opinion, the following documents are mandatory:

- Completed scientific opinion form
- Cover letter including the proposed submission slot and EAMS number
- Summary of pharmacovigilance system master file
- Risk management plan

The scientific opinion will be given according to the Step 2 timetable (75 or 90 days), dependent

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on a positive or negative initial benefit-risk opinion. Medicines with a positive EAMS opinion could be made available to patients 12–18 months ahead of normal marketing authorization. The EAMS scientific opinion is valid for one year in the first instance and lapses at the time a marketing authorization is granted.

150-day Assessment for National Applications for Medicines[12]

A sponsor of a new active substance can apply for a "150-day assessment timeline" process. This process is applicable only to high-quality new marketing authorization applications (MAAs), aiming at accelerating the availability of medicines for patients in the UK. Under this process, the MHRA will evaluate the application and provide its opinion on approvability within 150 days of submission of a valid application. Sponsors can apply for a presubmission meeting and need to provide a short summary of the dossier to share their intentions for the assessment and to verify the new active substance status. The sponsor can request consideration for orphan MA, and/or conditional MA, and/or MA during the meeting under exceptional circumstance. The orphan status will be determined at the time of the MA grant. If the orphan status is not agreed upon, and the company wishes to appeal this decision, marketing authorization will only be possible when the appeal process is completed.

Rolling Review for Marketing Authorization Applications[13]

The rolling review provides the flexibility for sponsors to submit sections of the dossier incrementally for the MHRA's pre-assessment rather than as a full (standard) submission. The rolling review offers the sponsor periodic enhanced regulatory interaction and advice to reduce the risk of failure at the final phase and may be integrated with the target development profile (TDP) to provide a clearer pathway for the development of innovative medicines. The sponsor can request a presubmission meeting to discuss the product, its intended target populations, and the data in each module to be submitted. A presubmission meeting with the MHRA should be requested approximately 90 days in advance of the intended submission for the final marketing authorization. For the meeting, it is advisable to briefly summarize the dossier and raise any special issues, such as requests for consideration for orphan MA, conditional MA, or MA under exceptional circumstances.

Innovative Licensing and Access Pathway[14]

The Innovative Licensing and Access Pathway (ILAP) aims to accelerate the time to market, facilitating patient access to medicines (new chemical entities, biological medicines, new indications, and repurposed medicines). Both commercial and non-commercial developers of medicines (UK-based and/or global) are eligible to apply. The pathway comprises an Innovation Passport designation and a Target Development Profile (TDP) and provides sponsors with access to a tool kit to support all stages of the design, development, and approval process. The MHRA usually conducts meetings within four to six weeks following receipt of the application form. The decision will be communicated to the sponsor within four weeks. The ILAP does not replace the EAMS. The ILAP is broader in scope and is open to all innovative products.

Patient Engagement

Pilot Project on Patient Involvement in New Applications[15]

Recently, the MHRA launched a pilot program to ensure that pharmaceutical companies and research teams harness the power of the patient voice during various stages of drug development and clinical trials. During the pilot phase, providing proof of patient involvement will be voluntary and will not alter or hinder the outcome of applications. However, in the future, the outcome of this program will lead to patient involvement playing a greater role in the final assessment process, when clinical trials are approved, or medicines are licensed. Starting 23 March 2021, the sponsor needs to present evidence of patient engagement during drug development when submitting MAAs for new active substances and new indications.

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During the exploratory stage of the program, the MHRA will not mandate showing patient engagement in the clinical trials but will document any evidence of patient involvement in clinical trial applications in medical assessment reports to better understand the current extent of patient engagement activities.

Patient Group Consultative Forum[16]

Through the Patient Group Consultative Forum, interested patient groups and stakeholders can present their views and experiences. The forum acts as a means of bringing the "patient/public voice" into the MHRA, to assist in the development of policy or approaches to a specific regulatory or scientific research area. In the past, patients' views, experiences, and real-life examples were presented in agency meetings relating to the packaging of medicines, regenerative medicine pathways, raising awareness of the yellow card scheme, and the early access to medicines scheme. The MHRA holds up to four meetings a year, and forum participants also can attend subject-specific workshops, provide comments (from the patient/care giver perspective) on draft materials, and participate in surveys.

Licensing of Medicines[17,18]

The MHRA also involves patients during the licensing of medicines and makes amendments based on their input. For example, in 2019, the agency renewed the scientific opinion issued under the EAMS for Raxone to treat the decline of respiratory function in patients with Duchenne muscular dystrophy. The agency took the renewal decision based on the views presented by patients and their caregivers during a patient-focused meeting.

Orphan Medicine Designation, Orphan Medicine Development/ Marketing Authorization Application

In the UK, no premarketing authorization orphan designation process exists, and if a medicinal product has been designated an orphan in the EU under Regulation (EC) 141/2000, an MAA can be submitted for the orphan medicine designation under regulation 50G of the Human Medicines Regulation 2012 (as amended). An orphan medicine designation application and the associated MAA (submitted together) in the UK can only be considered in the absence of an active EU orphan medicine designation.

The orphan designation criteria mirror those in the EU. Medicines need to fulfill the following criteria to qualify for orphan designation in an orphan condition:

- 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 the UK must not be more than five 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 the UK, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.
- Satisfactory methods may include authorized medicinal products, medical devices, or other methods of diagnosis, prevention, or treatment used in the UK.

To obtain the designation, the sponsor needs to submit an orphan medicine designation application form along with the MAA. The MHRA makes the decision on orphan status at the time it decides whether to approve the marketing application. This approach differs from EMA, which includes a process for granting the orphan medicine designation in advance of the MAA submission and offers orphan fee incentives and other benefits for sponsors of orphan medicines during development. The MHRA's advisory committee, the Commission on Human Medicines (CHM), will examine the application for orphan designation concurrently with the MAA under review. Medicines with an orphan marketing authorization will be listed on the UK Orphan Register.[19]

In the UK, no orphan designation is issued separately from the MA. Therefore, if a change of

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ownership application is submitted, the orphan designation will automatically transfer to the new marketing authorization holder.

It is assumed that the MAA requirements for approval will be similar to EU requirements. There might be a risk if the MHRA requirements differ, and additional work or evaluation of a new parameter during development is requested. Also, there may be difficulties navigating differences between MHRA and EMA opinions on protocol design or development plans.

Scope of Orphan Medicine Exclusive Approval[20]

Once a medicinal product receives MA with orphan designation in the UK, it benefits from 10 years of market exclusivity. The market exclusivity period begins on the date of the first approval of the product.

The UK also will recognize remaining market exclusivity for centrally authorized medicines (granted prior to 1 January 2021) in the EU that are converted to UK marketing authorizations. Unlike the EU, it is not necessary to submit orphan maintenance reports to the MHRA, but they can be submitted as additional information.

Market Access

The following three Health Technology Assessment (HTA) agencies have adopted special assessment criteria for orphan medicinal products (OMPs) in the UK:

1. The National Institute for Health and Care Excellence (NICE) includes a program for ultra-orphan medicine (highly specialized technologies [HST]).
2. The Scottish Medicine Consortium (SMC) includes orphan and ultra-orphan modifier criteria.
3. The Welsh agency, All Wales Medicines Strategy Group (AWMSG), includes additional criteria to consider the severity and unmet need.

A nominative prescription of the OMP from a National Health Service (NHS) doctor automatically provides the right to reimbursement to the patient.[21]

Effective April 2013, NICE is responsible for coordinating the evaluation of expensive ultra-rare orphan medicines. An interim method that builds on the framework used by Advisory Group for National Specialized Services (AGNSS) has been developed for the evaluation of highly specialized drugs. The HST program considers only drugs for very rare conditions, and these evaluations are recommendations on the use of new and existing highly specialized medicines and treatments within the NHS in England.[22,23]

The HST program takes the following criteria into consideration during the evaluation:[24]

- Nature of the condition (including morbidity or clinical disability with current standards of care; effect on caregivers' quality of life; current treatment options)
- Impact of the new technology (clinical effectiveness; magnitude of health benefits for patients, and caregivers when appropriate)
- Cost to the NHS and personal social services (PSS) (including budget impact; robustness of costing and budget impact information; patient access agreements)
- Value for money (benefit compared with current treatment; other resources needed to use the technology; impact on budget available)
- Impact beyond direct health benefits (are there any such benefits, are costs or savings incurred outside of the NHS and PSS)
- Impact on delivery of the specialized service (staffing and infrastructure requirements, such as training, planning for expertise)

In Scotland, pharmaceutical companies will be asked to state in their SMC submissions whether the medicine is in one of the following three categories (end-of-life medicine, orphan medicine, or ultra-orphan medicine) and to provide supporting evidence and rationale.

End-of-Life and Orphan Medicines

A submission for an end-of-life or orphan medicine will be made using the same submission form as before. The medicine will be evaluated

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by the New Drugs Committee (NDC) in the usual way. If the advice for the medicine is "not recommended" following NDC review, the pharmaceutical company can choose to request that SMC convenes a Patient and Clinician Engagement (PACE) meeting.

Ultra-Orphan Medicines

To meet the definition for an ultra-orphan medicine in Scotland, the following criteria must be met:

- The condition has a prevalence of 1 in 50,000 or less in Scotland
- The medicine has an EMA orphan designation for the condition, and this is maintained at time of marketing authorization
- The condition is chronic and severely disabling, and requires highly specialized management

A PACE meeting is not convened during the initial ultra-orphan assessment, as no decision will be made on the medicine at that time. Following the data collection period, and subsequent submission to SMC, the medicine will be evaluated by the NDC. If the advice for the medicine is 'not recommended' following NDC review, the pharmaceutical company can choose to request that SMC convenes a PACE meeting.[25] To assess ultra-orphan medicines, SMC will use a framework of explicit decision-making criteria, including the nature of the condition, impact of the medicine, impact of the technology beyond direct health benefits and on specialist services, costs to the NHS and PSS, and value for money. A cost-effectiveness ratio will still be requested as part of the company submission, but there may be circumstances where the choice of economic appraisal methodology has to be more flexible, given the available data and nature of the condition.[26]

Patient Access Schemes

If the NDC's advice for an end-of-life, orphan, or ultra-orphan medicine is "not recommended," the sponsor also will have the option to offer a new or revised patient access scheme aimed at making their product a better value for the NHS in Scotland.[27]

In Wales, Orphan Medicine is defined as a medicine with orphan status (or a medicine without orphan status with a prevalence of ≤ 1 in 2,000 people in Wales [or the UK] for the full licensed population and meets the criteria for European Commission orphan status). Ultra-Orphan is defined as a subset of orphan medicines that have a prevalence of ≤ 1 in 50,000 people in Wales (or the UK) for the full licensed population (or a medicine without orphan status and a prevalence of ≤1 in 50,000 people in Wales (or the UK) for the full licensed population and meets the criteria for European Commission orphan status).

All Wales Medicines Strategy Group (AWMSG) considers how the incremental cost-effectiveness of the medicine being appraised relates to other medicines or treatments currently being used in the NHS to treat a disease, including those that AWMSG or NICE have appraised. AWMSG's appraisal process for medicines for rare diseases aligns with NICE's technology appraisal and highly specialized technologies (HST) programs.[28] However, there is an additional stage to further assess the benefits of the medicine from the perspective of clinicians and patients through the Clinician and Patient Involvement Group (CAPIG). A CAPIG meeting may be convened if a medicine for a rare disease receives a negative recommendation from the New Medicines Group (NMG), or if a positive recommendation from the NMG is followed by a negative recommendation from AWMSG. The timelines for appraising a medicine for a rare disease are the same as for other medicines unless a CAPIG meeting is convened. A CAPIG meeting may add up to 12 weeks to the overall time for the appraisal by AWMSG.

Lastly, per the EU withdrawal agreement, "Goods placed on the market in the European Union or the United Kingdom before the end of the transition period may continue to circulate between these two markets from 1 January 2021."[29] This agreement avoids the delay of access of life-saving medicines (like orphan medicines) to patients.

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Conclusion

Effective 1 January 2021, MHRA became the standalone medicines and medical devices regulator in the UK, after Brexit. MHRA's criteria, tools, and regulatory standards for rare disease largely aligns with the EMA, with small but significant differences. MHRA has a flexible approach and provides multiple tools for sponsors to efficiently develop a drug for an orphan disease.

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4

Orphan Drug Development in Japan

History

Since 1972, the Japanese national health system has been active in implementing measures to promote drug development for the treatment of rare diseases. Initially, rare diseases were known as "intractable diseases (Nan-byo)." Policies to promote the research and development of orphan drugs were adopted in 1993, and a notification was issued by the Ministry of Health, Labor and Welfare (MHLW) concerning designation criteria and measures to promote research, e.g., the revised *Orphan Drug Regulations*, amendment to the *Pharmaceutical Affairs Act*, and an act establishing a fund for the relief of adverse drug reactions and promotion of research.[1-2] **Table 4-1** presents the timeline of rare disease regulations/initiatives in Japan, such as orphan drug regulations, subsidization of specific medical expenses related to intractable/rare diseases, and research on medical care for intractable/rare diseases

Benefits[3-4]

The following is a discussion of the benefits sponsors receive once a drug receives an orphan designation:

Subsidy Payment

Sponsors can receive subsidies, including funds for tests and research, through the National Institute of Biomedical Innovation (NIBIO). This subsidy is intended to help reduce the overall financial burden of orphan drug development. Companies receiving the subsidy are expected to pay a part of the profits obtained from orphan drugs to the NIBIO. The NIBIO will allocate payments received from companies for the development and promotion of orphan drugs.

Guidance and Consultation

Sponsors receive guidance and consultation from the MHLW, the Pharmaceuticals and Medical Devices Agency (PMDA), and NIBIO on research and development activities related to orphan drugs. The PMDA has established a priority consultation system with lower user fee categories for designated orphan drugs/medical devices.

Preferential Tax Treatment

Sponsors can report 12% of study expenses incurred during the NIBIO subsidy payment period (not including subsidies granted by NIBIO) for orphan drugs; these expenses can be reported as a tax credit.

Priority Review

During marketing authorization application, the designated orphan drug will be provided priority review for faster access to the market (the review period is nine months compared to the standard

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