pbbr.a SOCIEDADE DE ADVOGADOS, SP, RL

Health 10-2023

Regulation on Exceptional Use Authorization

*The following regulations came into force on 15.09.2023, approved by Resolution no. 840/2023 of 31 August, of Infarmed's Board of Directors: Regulation on Exceptional Use Authorization ("**EUA**") and the Regulation on Authorization of Marketing Medicines without Valid Authorization or Registration in Portugal ("**WAR**") thus replacing the previous regulations on these matters.*

The new regulations set out the regime applicable to authorizations for the marketing of medicines, of an exceptional nature, as provided for in articles 92 and 93 of the Medicines Statute (approved by Decree-Law 176/2006 of 30 August in its current wording), which applies to medicines that do not have a marketing authorization ("MA") or registration, or that are not marketed in Portugal.

1. EUA's Regulation

With regard to the EUA´s Regulation, which deals with authorizations of an exceptional and temporary nature, its revision introduces different procedures and evaluation methods depending on the type of applicants and addressees of these authorizations. EUA's can be intended for specific patients, groups of patients or population groups.

a) EUAs for specific patients

EUAs for specific patients may be granted to entities authorized to purchase medicines directly (i.e. hospitals or other healthcare providers), or to pharmacies.
In any case, the granting of EUAs requires proof of the indispensability of the medicine in relation to the alternatives available, through individualized clinical justification.

• Entities authorized to purchase medicines directly

These authorizations can be for medicines that do not already have a MA in Portugal, or which, although they have a MA, are not subject to a prior evaluation agreement with a view to their funding by the National Health Service.

Applications are submitted on the SIATS platform, managed by Infarmed, I.P., and must comply with the requirements set out in the Regulation regarding the characterization of

the patient, the medicine, the pathology and the intended treatment.

For medicines that have a marketing authorization but are not covered by a health technologies assessment agreement, Infarmed, I.P. shall consider including them in Early Access Programs (EAP).

Entities that have direct purchase authorization have to report INFARMED, on a monthly basis, all data of the consumption of the medicines which exceptional use has been authorized.

• EUA by pharmacies
The purchase of medicines by a pharmacy is automatically considered to be covered by an EUA, without the need to submit an application to INFARMED, I.P., provided that the following requirements are met:
- the existence of a medical prescription accompanied by the respective clinical justification;
- there are no similar medicines authorized;
- medicines have a MA in one or more EEA countries;
- quantity of packages to be purchased is compatible with the prescribed treatment, and never exceeds the quantity needed for 12 months.
In the case of medicines purchased by pharmacies, the price for the patient is calculated exclusively on the basis of the cost price purchased by the pharmacy, and no margin is applicable.

The EUA regime for pharmacies does not apply to the purchase of medicines for exclusive hospital use, medicines which prescription and dispensing does not comply with the rules applicable in Portugal, medicines containing psychotropic substances, vaccines and medicines derived from human plasma.
Pharmacies have to report to INFARMED, by the 15th day of the 1st month of each calendar semester, the medicines purchased and dispensed, in the previous semester under these regulations.

b) EUAs for patient groups or population groups
These EUAs can be granted to entities with authorization for the direct purchase of medicines, MA holders, wholesale distributors and holders of authorization to manufacture medicines, and can be for medicines that do not have an MA, or that if they do, are not actually marketed in Portugal, or batches of medicines in a situation of supply shortage.

The conditions of the authorization and the period of validity of each authorization are defined in the authorization.

With regard to medicines that do not have a marketing authorization or which, if they do, are not marketed in Portugal, wholesale distributors and holders of authorization to manufacture medicines are obliged to inform Infarmed, I.P. of the date the medicine was received in their warehouse and the quantities, as well as the quantities of medicines they have supplied, naming the entities concerned and the respective date.

2. SAR regulation
With regard to the Regulation on the SAR provided for in Article 93, which has now been made autonomous from the EUAs, the changes introduced are essentially aimed at bringing greater clarity to the authorization process.

This authorization is essentially intended to ensure the availability of medicines considered essential for justified public health reasons.
The SAR can be granted at the request of MA holders, wholesale distributors or holders of manufacturing authorizations of medicinal products.
This authorization is only granted for medicines that have a valid MA in an EU Member State.

The application should be decided within 75 days of submission of a valid application.

The holder of a SAR is subject to the obligations arising from the law for the MA holder in terms of authorization, pharmacovigilance, advertising and recall. The holder of a SAR shall keep at INFARMED's disposal all data and information relating to specific batches of these medicines until the end of the second year after the expiry of the authorization and, in any case, at least until two years have elapsed after the expiry date of each specific batch.

The medicinal product marketed under a SAR is subject to the rules laid down in the National Health Technology Assessment System (SiNATS) with regard to prices, reimbursement or prior assessment.

In view of the number of applicants and the specific nature of the requirements, new forms and guidelines are available in the Management of the availability of medicines area.

You can also consult the updated list of medicines for EULA by Wholesale Distributors and Manufacturer from this link.

If you have any questions on this subject, please do not hesitate to contact the Health Law team at pbbr - Sociedade de Advogados, SP, RL.

Contact:
Rita Roque de Pinho - rita.pinho@pbbr.pt
Raquel Soares Lourenço - raquel.lourenco@pbbr.pt