Changes on European regulations on orphan drugs

Commission Regulation (EU) 2018/781 amending Regulation (EC) 847/2000 as regards the definition of the term "similar medicinal product"

Introduction

The precise and unambiguous definitions of "active substance" and "similar active substance" is essential in the field of medicinal products for rare diseases: the so-called "orphan medicinal products".

Regulation (EC) 847/2000, where the definitions of "active substance" and "similar active substance" are contemplated, was recently amended by Regulation (EU) 2018/781, effective as of 19 June. Changes introduced by this new Regulation (mainly referring to the definition of "similar active substance" as opposed to "same active substance") are theoretically limited to the field of rare diseases. However, it is to be noted that the content of such changes may be useful in other fields in which a calm reflection on the terms and scope of the terms "active substance" and "similar active substance" is also necessary. This is the case, for example, of the new reference price system for medicines of the National Health System announced by the MoH earlier this year.

Regulation (EU) 2018/781

Two are the main amendments introduced by the new European Regulation which deserve special attention. The first of them is the deletion of the "active substance" definition contained in Regulation (EC) 847/2000 which differed from the same term defined in Directive 2001/83/EC. Such deletion is due to the fact that the Commission was not empowered to modify, through implementing regulations (such as Regulation (EC) 847/2000), the legal term of "active substance" already defined in the above-mentioned Directive. The second relevant amendment is the development and clarification of the term "similar active substance". In this regard, the new Regulation accurately determines what "principal molecular structural features" should mean, which has been the only criterion used so far for the similarity assessment between two active substances.

Additionally, the new Regulation incorporates an additional criterion to assess similarity between certain kinds of medicines such as advanced therapy medicinal products. This criterion refers to the evaluation of the active substances to be compared on the basis of their biological and functional characteristics.

New rules regarding the reference price system

Several considerations arise from the new Regulation, which may be useful in connection with the announced amendment of the reference price system in Spain. First, we consider that changes must be addressed carefully to prevent them from altering the current definition of "active substance" which, as this new Regulation reminds us, is a well-established definition of in the Community legislation. Second, the precise definition provided by Regulation (EU) 2018/781 of "similar active substance", as opposed to "identical active substance", is something that, in our opinion, should be considered by Spanish authorities when defining the term of "same active substance" in the context of the Spanish reference price system.