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Strict interpretation of the "significant benefit" condition for the designation of an orphan medicinal product

Judgement of the General Court of 16 May 2019, Case T-733/17, GMP-Orphan v European Commission

Background

One of the available ways to obtain orphan drug designation for a medicinal product is to prove that such medicinal product will be of "significant benefit" to the patients. This "significant benefit" shall mean either (a) a clinically relevant advantage, or (b) a "major contribution" to patient care; and must be proved by means of a comparative analysis between the new medicinal product and the reference product already authorized. In 2003, a Communication from the Commission made clear that a "major contribution" may exist if the new medicinal product is available in all Member States and the reference product is only authorized in a limited number of Member States. In 2016, a new Communication followed a different approach: as a general rule, an EU wide marketing authorization was no longer sufficient to maintain an assumption of "major contribution".

In this case, the medicinal product was designated as orphan in 2015 on the grounds that it would be available at EU-level, while the reference product was only authorized in the UK. The designation was revoked in 2017 because it was considered that the "major contribution" condition was not satisfied.

Position of the Court

The Court accepts that the decision regarding the product cannot be made on the basis of the 2016 Communication. However, the Court rejects the appeal because of the following main grounds.

In the first place, the Court considers that under applicable regulations (Regulation 141/2000 and 847/2000) having an EU-wide marketing authorization is not, per se, sufficient to maintain an assumption of "major contribution". Regarding this matter, the Court gives limited value to the 2003 Communication, and points out that although such Communication acknowledges that a potential EU marketing authorization may constitute a significant benefit, the use of the word "may" implies that the existence of a "significant benefit" is just a possibility that must be substantiated by concrete evidence on a case-by-case basis. The Court also states that in the EU, a product that is not authorised in a Member State can be used in other Member States through special proceedings (as it is the case in Spain with Royal Decree 1015/2019). In view of the foregoing, the Court concludes that it was not proven that the patient needs were unmet or that the EU wide authorization of the new product would result in a "major contribution" to patients care.

Likewise, the Court points out that the authorisation of the new product through a centralised procedure does not guarantee its availability in all Member States, since a national health system may decide not to reimburse such product. This judgement shows the high degree of uncertainty that innovative industry faces. An uncertainty that is the result of the existence of many texts (with doubtful legal force) that, despite their purpose is to clarify concepts, they open the door to surprising interpretations of the applicable regulations and principles.