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The same but better (Part 2) – Shire v European Medicines Agency (C-359/18 P)

The CJEU confirms that an examination of "all relevant factors" is required before concluding that a previously authorised product precludes orphan designation of a later product with the same active

Summary

In its judgment of 29th July 2019 (C-359/18 P), the CJEU confirmed that the European Medicines Agency (EMA) had failed to correctly verify the validity of Shire's application for orphan designation for its intrathecally administered formulation of the active ingredient idursulfase (Idursulfase-IT). It agreed with the General Court's decision in Shire v EMA (T-80/16) that an examination of "all relevant factors" is required before the EMA can conclude that a previously authorised medicinal product precludes issuance of a new orphan designation for a follow-on product with the same active substance. Accordingly, the Court dismissed the EMA's appeal.

The decision is further good news for innovator companies that would like to improve on existing orphan products.

Basis of the EMA's appeal

The EMA appealed the General Court's judgment on the ground that the provisions for examining the validity of an application for orphan designation merely required it to ascertain whether the medicinal product in question was identical in its active substance and indication to an already authorised medicinal product because these were the only criteria set out in Article 5(2)(b) and (c) of Orphan Regulation 141/2000. As its second ground of appeal, the EMA had argued that the General Court had relied on an incorrect interpretation of the concept of "medicinal product". According to the EMA, Article 1(2) of the Medicinal Products Directive 2001/83 focused the concept of "medicinal product" on the active substance and not on excipients or methods of administration.

The terms "medicinal product" and "active substance" cover two distinct concepts

According to the Court, it was apparent from a combined reading of Article 3(1) and Article 5(2) of the Orphan Regulation that if, for a given indication, there is already a first authorised medicinal product, an applicant seeking orphan designation for a second medicinal product must establish *inter alia* that the second product will be of significant benefit to patients in comparison to the first. In such a situation, the applicant also has to show that the second medicinal product is not identical to the first.

The Court found that the EMA had erred when limiting its assessment of identity to the question whether the first and second medicinal products had the same active substance and use. It agreed with the General Court, that the terms "medicinal product" and "active substance" cover two distinct concepts that should not be confused with each other. The Court held that the active substance was merely one component of a medicinal product, and it was therefore necessary to examine all other relevant factors to determine whether the first and second medicinal products were identical.

With regard to the EMA's second ground of appeal, the Court agreed with the General Court's finding that, in accordance with Article 1(3b) of the Medicinal Products Directive, the concept of "medicinal product" included excipients in addition to the active substance. According to the Court, the EMA had not put forward any arguments to show that the General Court had erred in its interpretation of the law in this respect. It held that the second ground of appeal was therefore substantially the same as the first one, which it had already rejected.

The COMP has to assess the criteria for orphan designation

The Court agreed with the EMA that the verification of the designation criteria in Article 3(1) of the Orphan Regulation did not fall within the scope of the EMA's review of the validity of the application for orphan designation. In accordance with Articles 4 and 5(5) to (7) of the Orphan Regulation, the responsibility for this review lies with the Committee for Orphan Medicinal Products (COMP) because of the technical and scientific nature of the criteria set out in Article 3(1), which "*entail complex and nuanced analyses*" in particular when assessing whether the product in question provides a significant benefit to patients in comparison to an already authorised product. The Court held that for the very same reasons, the exclusive competence of the COMP extended to verifying the identity of the medicinal product for which the applicant seeks orphan designation.

The EMA's role is to perform a formalities check of the orphan designation application

The Court therefore endorsed the General Court's finding that the EMA was required to check whether the application for orphan designation was submitted prior to the application for marketing authorisation as required by Article 5(1) of the Orphan Regulation and whether it was accompanied by the information and documents referred in Article 5(2). If the application complied with these requirements, the EMA was obliged to transmit it to the COMP for further assessment. The factual finding of the General Court that Shire's Idursulfase-IT product differed from its authorised Elaprase® product, which contains the same active substance, was not subject to the review of the Court of Justice.

Conclusion

The Court's endorsement of the General Court's decision in T-80/16 is good news for innovator companies who have invested in research to further improve existing treatments for rare diseases.

Authors & Experts: Jens Grabenstein

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