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The CJEU Rules that Companies Can Seek Orphan Status for a New Product Containing the Same Active Substance as an Existing Orphan Product

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Life Sciences

On July 29, 2019, the Court of Justice of the European Union ("CJEU") issued its judgment in Case C-359/18 P, Shire Pharmaceuticals Ireland v. EMA, dismissing the European Medicines Agency's ("EMA") appeal and reconfirming the position set by the General Court. Covington represented Shire Pharmaceuticals Ireland ("Shire"), which was recently acquired by Takeda.

The judgment presents an important victory for the innovative pharmaceutical industry. The CJEU agreed with the General Court that new medicines containing the same active substance as existing orphan medicinal products should be entitled to an additional period of orphan exclusivity if they meet the criteria for orphan designation under Article 3(1) of Regulation No 141/2000 (*i.e.*, the prevalence or return on investment criterion and also provide a significant benefit for patients over existing medicinal products).

The Commission's and EMA's position had historically been that companies are only entitled to one period of orphan exclusivity per active substance per orphan condition. This ruling therefore reflects a sea change and presents significant opportunities for companies developing and marketing orphan medicinal products.

The EMA's main ground for appeal was that the General Court erred in failing to read Article 5(1) and (2) of Regulation No 141/2000. Article 5(1) places on the Agency an obligation to check whether an application for designation as an orphan medicinal product is already the subject of an earlier application for a marketing authorization. Article 5(2) of that Regulation sets out a list of documents to be submitted in the application for orphan designation. Article 5(2) provides that the application for designation includes only the following:

> "*(a) name or corporate name and permanent address of the sponsor;*
>
> *(b)* ***active ingredients*** *of the medicinal product;*
>
> *(c) proposed therapeutic indication;*
>
> *(d) justification that the criteria laid down in Article 3(1) are met and a description of the stage of development, including the indications expected.*" (emphasis added)

The EMA and Commission argued that Article 5(1) must therefore be read to require rejection of applications where the sponsor has already submitted an application for a marketing authorization for the medicinal product containing the same active ingredient.

The CJEU disagreed. It said that the correct approach was that Shire (now Takeda) and the General Court had advocated, *i.e.*, that the EMA must read Article 5(2) in conjunction with Article 3(1). The Agency must validate the application if it includes the elements set out in Article 5(2) and the second medicinal product is not identical to the existing product. It is then for the Committee for Orphan Medicinal Products ("COMP") to assess whether the second product fulfils the prevalence and significant benefit criteria.

The CJEU also rejected the arguments that the General Court had erred in relying on the definitions of "medicinal product" and "active substance" in Article 1(2) of Directive 2001/83/EC as either unfounded or inadmissible.

Interestingly, the CJEU seems to have gone further than the General Court on the extent to which the first and second product need to be "different." As the CJEU summarizes in paragraph 40 of its judgment, the General Court considered the differences in the composition, method of administration and therapeutic effects between the Shire products in question and concluded that the second product was not the same as the first. At paragraph 31 of its judgment, the CJEU suggested that all that is required is for the sponsor to show that the second medicinal product is "not identical" to the first medicinal product. Obviously, minor inconsequential differences are unlikely to result in the necessary clinical benefit.

The judgment is significant as it reaffirms the possibility that companies in the orphan drug space may be eligible for separate periods of orphan exclusivity when they develop new products containing the same active substance, provided they offer a significant benefit (meaning 'a clinically relevant advantage or a major contribution to patient care').

If you have any questions concerning the material discussed in this client alert, please contact the following members of our Life Sciences practice:

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