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11th Circ. Hands Catalyst Win In 'Orphan Drug' Case

By **Nathan Hale**

Law360 (September 30, 2021, 9:54 PM EDT) -- Florida-based Catalyst Pharmaceuticals Inc. won a decision on Thursday in the Eleventh Circuit, which found that the U.S. Food and Drug Administration unlawfully infringed on an exclusivity period it awarded the company for a drug that treats a rare autoimmune disease when it approved a lower-cost version.

The appeals court reversed a Florida district court's decision from almost exactly a year ago, in which the lower court dismissed Catalyst's suit after concluding that language in the Orphan Drug Act, which incentivizes the development of drugs for rare diseases, was ambiguous and that it should thus defer to the agency's decision under the Chevron doctrine. The doctrine gives deference to federal agencies' interpretations of ambiguous laws.

The three-judge appellate panel determined that, while not defined in the statute, the act's reference to the "same disease or condition," in defining the exclusivity right, is not ambiguous. It added that based on the undisputed facts in the case, Catalyst should have been granted summary judgment and that the FDA's approval of rival Jacobus Pharmaceutical Company Inc.'s drug Ruzurgi should be set aside.

"The ruling … adopts the 'plain meaning' arguments that we put forward and concludes that the statute means what it says: FDA cannot approve the same drug for the same disease for the duration of our client's exclusivity, even for a different patient population," Catalyst counsel Philip J. Perry of Latham & Watkins LLP told Law360 on Thursday.

The FDA granted approval in 2018 under the Orphan Drug Act for the use of Catalyst's drug Firdapse to treat Lambert-Eaton myasthenic syndrome, or LEMS, an extremely rare autoimmune disease that causes muscle weakness and difficulty walking. According to the opinion, there are estimated to be fewer than 1,300 diagnosed cases in the United States.

Under the statute, the manufacturer of an orphan drug is granted a seven-year exclusivity period following approval, during which the FDA is not permitted to approve another application for "the same drug for the same disease or condition."

But in 2019, the FDA approved New Jersey-based Jacobus' drug Ruzurgi for treatment of LEMS in patients 6 to 17 years old. The FDA determined that the approval of Ruzurgi, which like Firdapse features the active ingredient amifampridine, did not violate Catalyst's exclusivity because its approval for pediatric use represented a different "indication or use," according to the opinion.

Catalyst filed suit, arguing that the Ruzurgi approval violated the "same drug for the same disease or condition" restriction and that Ruzurgi's labeling violated FDA regulations by indicating that it can be used by adults, which facilitates illegal "off-label" marketing by Jacobus for adult patients.

During arguments before the district court, Perry said that almost 99% of LEMS patients are adults and that Jacobus has been selling Ruzurgi — which goes for $175,000 per year compared to Firdapse's $375,000-per-year cost — mostly to adults because the disease is the same.

The parties did not dispute that Firdapse and Ruzurgi qualify as the "same drug" under the Orphan Drug Act, but U.S. District Judge Beth Bloom adopted U.S. Magistrate Judge Lauren Louis' finding that the statute's reference to "the same disease or condition" is ambiguous, saying it is unclear if it refers to the use the drug is approved for after it submits its new drug application — in this case, LEMS for adults — or the disease or condition for which it received its orphan drug designation — LEMS for all patients.

But the Eleventh Circuit said it found the meaning to be clear based both on the ordinary and plain meaning of the words, as well as the context of the statute.

"A statute is not ambiguous merely because it contains a term without a statutory definition," the Eleventh Circuit said, quoting from case law. "As we have recognized, '[w]e interpret words that are not defined in a statute with their ordinary and plain meaning because we assume that Congress uses words in a statute as they are commonly understood.'"

The panel said that word "same" is used in the sense of "being the one under discussion or already referred to," and that the only "disease or condition" already referred to in that section of the law is the "rare disease or condition" for which the first drug was designated under the Orphan Drug Act.

The panel said that because it agrees with Catalyst that the "same disease and condition" language is not ambiguous, it did not need to address the district court's finding that the FDA's interpretation of the law was reasonable or the questions about the Ruzurgi labeling.

An FDA spokesperson said the agency "is reviewing the decision and considering next steps."

Counsel for Jacobus did not immediately respond to a request for comment late Thursday.

Circuit Judges Barbara Lagoa, R. Lanier Anderson III and Stanley Marcus sat on the panel for the Eleventh Circuit.

Catalyst is represented by Michael C. Marsh and Ryan Roman of Akerman LLP and Philip J. Perry, John R. Manthei, Andrew D. Prins, Ryan S. Baasch, Cherish A. Drain, Brent Murphy and Monica C. Groat of Latham & Watkins LLP.

Jacobus is represented by Marisa C. Maleck, Jarred Lee Reiling and Gabriel Krimm of King & Spalding LLP.

The FDA is represented in-house by Stacy C. Amin, Annamarie Kempic and Barbara Alkalay, and by Robert P. Charrow of the U.S. Department of Health and Human Services, and Scott R. McIntosh and Brian J. Springer of the U.S. Department of Justice's Civil Division.

The case is Catalyst Pharmaceuticals Inc. v. Becerra et al., case number 20-13922, in the U.S. Court of Appeals for the Eleventh Circuit.

--Additional reporting by Carolina Bolado, Adam Lidgett and Christopher Cole. Editing by Steven Edelstone.
