

## IMPLICATIONS OF THE U.S. DISTRICT COURT OPINION VACATING THE 340B PROGRAM'S "ORPHAN DRUG" FINAL RULE AND REGULATIONS

### EXECUTIVE SUMMARY

On May 23, 2014, the United States District Court for the District of Columbia issued an opinion invalidating a final agency rule issued on July 23, 2013 ("Final Rule") by the Health Resources and Services Administration ("HRSA"). The Final Rule had served to clarify the scope of the 340B Program's "Orphan Drug" exclusion. Our article detailing the scope of this Final Rule can be found [here](#).

As a result of this ruling, both manufacturers and 340B Program participating entities ("Covered Entities") will need to assess how they intend to interpret the applicable statutory provisions implementing the Orphan Drug exclusion. These stakeholders will also want to monitor ongoing developments with the District Court case as well as related HRSA guidance to determine next steps.

### DISCUSSION

An Orphan Drug is a drug that is designated by the Food and Drug Administration as being for the treatment of a rare disease or condition. Covered Entities subject to the Orphan Drug exclusion include freestanding cancer hospitals, critical access hospitals, rural referral centers and sole community hospitals ("Orphan-Excluded Hospitals"). The Orphan Drug exclusion is a statutorily mandated general restriction on the use of Orphan Drugs by these Orphan-Excluded Hospitals. The statute does not directly address key implementation questions, which is what the Final Rule had attempted to do.

At a high level, the Final Rule had confirmed that access to 340B Program pricing for an Orphan Drug is unavailable only where such an Orphan Drug is used by an Orphan-Excluded Hospital for the rare disease or condition for which orphan status was granted. The Final Rule was significant since it served to limit the applicability of the Orphan Drug exclusion to conditions for which an orphan indication was granted by the FDA. This meant that Orphan-Excluded Hospitals were clearly permitted to purchase Orphan Drugs using a 340B Program discount, provided the use of that drug was not related to the disease or condition for which orphan status was granted.

The District Court's ruling is notable in that it serves, for now, to invalidate the Final Rule on the grounds that HRSA did not have the authority to issue regulations governing this topic. Notably, however, the District Court did not rule that HRSA's interpretation of the underlying statute was incorrect. Rather, the District Court held that HRSA's authority to promulgate regulations did not extend to the statutory provisions governing the scope of 340B discounts for Orphan Drugs.

Thus, the District Court did not make a determination as to: i) whether the 340B Program's Orphan Drug exclusion applies to only drugs used for an orphan indication; or ii) whether the exclusion applies to any use of an Orphan Drug once orphan status has been obtained.

Importantly, the language in the District Court's opinion leaves open the possibility that HRSA could reissue the key components of the Final Rule in the form of interpretive guidance. It is also possible that further procedural actions, including but not limited to an appeal by HRSA, will occur within the next 60 days.

We also note that the language of the District Court's opinion calls into question the publication of the pending 340B Program "Mega Regulation" that had been slated for this summer by questioning the scope of HRSA's authority to issue 340B Program regulations that address anything other than a few specific topics.

### PRACTICAL TAKEAWAY

Interested stakeholders will want to closely monitor further developments related to this case as subsequent rulings, procedural actions and HRSA guidance could impact both the Orphan Drug exclusion as well as the pending Mega Regulation.

If you have any questions, would like additional information about this topic or need help preparing and submitting comments, please contact Todd Nova at 414-721-0464 or [tnova@hallrender.com](mailto:tnova@hallrender.com).

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