Pediatric Oncology Drug Shortages: A Multifaceted Problem

The consensus statement by Unguru et al1 concerning national shortages of therapies used to treat children with cancer accomplishes 2 important tasks: it highlights a serious public health threat and proposes steps to address the problem. The working group (WG) has outlined 6 recommendations. All are constructive approaches and, in the case of the recommendations that are within US Food and Drug Administration’s (FDA’s) purview, the FDA has already taken steps to implement actions.

The first recommendation (Support Current and Develop Innovative Measures to Prevent Drug Shortages) might be perceived as primarily in the FDA’s purview. Addressing the complex issue of drug shortages continues to be a high priority for the FDA. On October 31, 2013, the FDA released a strategic plan to improve the agency’s response to imminent or existing shortages and to identify longer-term approaches for addressing the underlying causes of drug shortages.2 This plan also highlights opportunities for drug manufacturers and others to prevent drug shortages by promoting and sustaining quality manufacturing. One critical element that limits the FDA’s responses, however, is the reality that the agency does not have authority to force the manufacture and distribution of a drug product. Companies that make these products are private businesses and the FDA’s influence is therefore limited in some of the arenas identified in the WG’s action items under their first recommendation. Even so, the FDA has an important role in implementing some of these action items.

For example, the action item to “implement a proposed FDA quality metric to incentivize and reward high-quality manufacturing practices” is something that the FDA is considering as part of its strategic plan to address manufacturing and quality issues, such as “batch failures” or quality transgressions, that are most often the root cause of shortages. The FDA will be working with industry and other stakeholders on these efforts. Another action item that the FDA has implemented involves rapid access to international suppliers of drugs or ingredients during a shortage. Early notification from manufacturers about possible shortages has enabled the FDA to work with manufacturers to restore production of many lifesaving therapies. Once notified of a shortage, the agency works with the manufacturer to investigate the issue leading to the manufacturing disruption and identifies other manufacturers who can make up the shortfall. When manufacturers of the FDA-approved drug are not able to immediately resolve a shortage of a medically necessary drug, the FDA will take appropriate actions to address the drug shortage. These actions may include, for example, not objecting to the shipment of alternative versions of the product with the same active ingredient, when appropriate, until the shortage of the FDA-approved version is resolved.

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KEY WORDS
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ABBREVIATIONS
FDA—Food and Drug Administration
WG—working group

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The FDA evaluates the drug for quality to ensure there are no significant risks for US patients. Information about the alternate supply of the drug is posted on the FDA Drug Shortage Web site, along with the “Dear Healthcare Professional” letter from the company distributing the drug. At present, there are 8 shortage products where this approach is being used.

Some of the action items are not in the FDA’s remand (Amend Laws to Allow Greater Price Increases for Generic Oncology Drugs on the Critical Drug List; Create a National Stockpile of Critical Drugs) or are best addressed by those caring for the affected patients (Create a Critical Drug and Shortage List). The FDA is actively working on the WG’s fourth action item (Create an Improved Information Source for Drug Shortages). In addition to enhancing the FDA Drug Shortage Web site, the FDA is working to improve communications, such as launching a new mobile app, so that individuals can instantaneously access drug shortage information via their smartphones.

There are few more heart-wrenching moments in life than to hear of a child, whether or not he or she is enrolled in a clinical trial, who requires a specific product for the treatment of cancer and to know that a critical component of that therapy is not available. The FDA is composed of scientists and other professionals, many of whom are parents and grandparents, who are truly dedicated to decreasing the frequency with which drug shortages occur. Drug shortages are, however, a complex issue requiring multiple solutions. The Consensus WG has provided useful insights and recommendations for these solutions. The FDA is committed to being an active partner in this activity, and we look forward to continuing progress in this area.

REFERENCES


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