



May 3, 2012

2012

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The Honorable Henry Waxman
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Dear Chairman Upton and Ranking Member Waxman:

The American Society of Hematology (ASH) appreciates the opportunity to submit comments regarding the drug shortage provisions included in the House Committee on Energy and Commerce's draft Prescription Drug User Fee Act (PDUFA) and Medical Device User Fee and Modernization Act (MDUFMA) legislation. The increasing problem of drug shortages has become a crisis taking a serious, sometimes life-threatening toll, on hematology patients and negatively impacting the practice of hematology and the clinical research our members conduct. ASH commends the Committee for developing a bipartisan draft designed to address shortages of drugs that are critical for hematology treatment and research.

ASH represents approximately 14,000 clinicians and scientists committed to the study and treatment of blood and blood-related diseases, including blood cancers such as leukemia, lymphoma, and myeloma and nonmalignant illnesses such as anemias, thrombosis, and bleeding disorders. The increasing number of drug shortages has significantly affected the practice of hematology because the standard therapies frequently used to treat many hematologic conditions are older, sterile injectable products that are particularly vulnerable to production, marketing, and other business factors that lead to shortage. Fewer firms manufacture these products, the products require complex manufacturing processes, companies may be tempted to redirect resources to more profitable products, and financial return may not justify corrective action when problems occur.

Consequently, the patients hematologists treat have been especially adversely affected by recent shortages. For example, last year there was a national shortage of cytarabine, an irreplaceable chemotherapy drug essential to the cure of acute myeloid leukemia (AML). Cytarabine cures forty percent of patients with AML; without cytarabine, the cure rate is zero percent. This shortage affected all AML patients who needed to be treated in the approximately 6-month period when cytarabine was out of stock. Earlier this year, the country experienced critical shortages of additional drugs for the treatment of leukemia, including methotrexate injection and daunorubicin. In the meantime, scores of other leukemia, lymphoma and myeloma patients also faced shortages of other life-saving treatments and our sickle cell patients have struggled with a lack of access to hydroxyurea, the only approved drug used to treat this disease.

In addition, these drug shortages have affected current and future hematology clinical trials. The recent spate of drug shortages has resulted in the disruption of multiple clinical trials, halting the accrual of new patients, and delaying future advances in hematology.

ASH supports many of the drug shortage provisions included in the draft and believes this is a helpful first step toward ensuring that patients have access to the medications they need while not compromising safety and quality of those medications. The Society strongly supports the provisions regarding early notification, broad parameters to define drugs in shortage, development of a drug shortage list, distribution of information about drug shortages to patient and provider organizations; and expedited review of manufacturing changes. However, the Society has the following recommendations to further strengthen the draft:

Inclusion of Radio Pharmaceuticals

ASH is particularly concerned that the reporting requirement needs to include radio pharmaceutical drug products. Many of radio pharmaceutical drugs are critical to the treatment of patients with hematologic conditions and they are at risk for being in short supply. Examples of hematologic products that fall into these categories, which have already experienced severe shortages, include: intravenous immunoglobulin (IVIG) used as part of the treatment regimen for allogeneic bone marrow transplant, chronic lymphocytic leukemia and idiopathic thrombocytopenic purpura and antithymocyte globulin used in the treatment of aplastic anemia. ASH urges the Committee to include radio pharmaceutical drug products within the defined parameters for reporting in the final legislation.

Enforcement

ASH believes simply publicly disclosing the names of manufacturers who fail to comply with the reporting requirements on the FDA's website will not serve as an effective enforcement mechanism. Given the importance and magnitude of drug shortages on treatment and research, ASH recommends that an enforcement mechanism be included in the final legislation that instructs the Secretary to promulgate regulations establishing a schedule of civil monetary penalties for failure to submit a required notification.

Communication to Physicians and Patients

As noted, ASH supports the provisions in the legislation that address communication to stakeholders. ASH believes the timely dissemination of information on drug shortages and discontinuations is of paramount importance to hematologists and other health care providers. While the Society acknowledges that legislation may not be able to prevent all future drug shortages, what has become increasingly important to our hematologist members and the patients they treat is that when a shortage occurs, they are provided in a timely fashion with information about the shortage as well as information about how to obtain the drug for critical patients. Therefore, ASH recommends that the legislation also require the Secretary to develop a plan on how the FDA will improve its communication and distribution of information about drug shortages to physician and patient organizations.

ASH believes that public notification of timely and accurate information is essential so that physicians can adequately plan for potential disruptions in patient care caused by a drug shortage. The Society urges the Committee to add provisions that would require FDA to increase its communications with medical practitioners and patients by developing specialty-specific list-serves and other means of targeted communications.

Study on Drug Shortages and Annual Report on Drug Shortages

The Society recommends that the Committee add a provision to the study that requires the Comptroller

General to examine the impact of drug shortages on federally-sponsored clinical trials. Additionally, ASH requests that the Committee add a provision to the Annual Report on Drug Shortages that describes the impact that drug shortages are having on federally-sponsored clinical trials, specifically including a list of federally-sponsored hematology clinical trials that were delayed, interrupted, modified, or halted due to drug shortages as well as the strategies that have been undertaken to avoid the disruption of clinical research.

Economic Incentives

As discussed in recent congressional hearings about drug shortages, ASH believes economics clearly contribute to the shortage problem. Many shortages have occurred because manufacturers are having a difficult time maintaining a profit margin for lower cost generic drugs. Consequently, the Society supports looking at how to provide economic incentives to manufacturers to prevent shortages. ASH urges the Committee to examine options for economic incentives and recommends considering an option modeled on the Orphan Drug Program to incentivize manufacturers' production of specific low cost critical drugs.

Study Regarding National Contingency Plan

ASH recommends that the Committee consider adding a provision that directs the Secretary to coordinate a study on the feasibility of developing a national contingency plan for drug shortages.

Increased Resources for FDA

Once drugs are created, it is critical that they continue to be available. ASH asks the Committee to consider a special set aside for the FDA's Drug Shortage Program to provide adequate funding to carry out the activities authorized by this legislation.

Thank you for your attention to these important issues and your consideration of ASH's comments and recommendations. ASH believes the Committee's draft is a good first step to addressing the problem of drug shortages and that the Committee has included helpful and thoughtful provisions to combat the crisis. The Society hopes to continue to work with you. Please contact ASH Government Relations Manager, Stephanie Kaplan (skaplan@hematology.org or 202-776-0544), if the Society can provide additional information or expertise.

Sincerely yours,



Armand Keating, MD
President