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Senate Homeland Security and Government Affairs Committee  
**Hearing on Drug Shortage Health and National Security Risks: Underlying Causes  
and Needed Reforms**

March 28, 2023

Mr. Chairman, Ranking Member, and other Members of the Committee,

Thank you for holding this important hearing on our nation's drug shortages and the impact on national security. I am the Chief Executive Officer of Children's Cancer Cause.

The Children's Cancer Cause is a leading national advocacy organization working to achieve access to less toxic and more effective pediatric cancer therapies; to expand resources for research and specialized care; and to address the unique needs and challenges of childhood cancer survivors and their families. Children's Cancer Cause leads efforts to ensure that these needs and perspectives of children with cancer are integrated into the highest level deliberations on health care and policy.

Each year in the U.S. approximately 16,000 children are diagnosed with cancer. Today, thanks to major treatment advances and participation in clinical trials, the 5-year survival rate is 85% for children and 86% for teens. Between 1970 and 2020, the number of deaths from cancer in children and teens decreased by more than 50% due to advances in treatment. Unfortunately, cancer remains the most common cause of death by disease for children in America.

The excellent statistics are dependent on an adequate and stable supply of life-saving drugs. We must continue to support the uninterrupted treatment of children with cancer who need these drugs to eradicate their disease. To this effect, we must guarantee adequate supplies of drugs for children in the United States.

Our community knows firsthand the impact of drug shortages which are too common in pediatric oncology. In a survey done by Children's Cancer Cause in 2017, a survivor shared that her treatment regimen was altered due to a shortage, and she was given alternative drugs. They only worked for a short time, and she suffered a relapse within months.

A mother of a child with cancer shared: "The chemo drug was no longer available and my daughter had a life-threatening allergy to the only available alternative. We had no choice but to start the alternate drug in the ICU at a low rate in hopes of breaking through the sensitivities and reactions." One family told us that a shortage delayed the

start of their child's clinical trial by a full three months with untold consequences to his effective cancer treatment. Some families reported having to travel significant distances—further from home and support networks—to find a treatment center with the available chemotherapy drug.

Children are uniquely vulnerable to drug shortages and data suggests they have an impact on survival for patients with cancer. In the last five years, 75% of the 20 most essential pediatric cancer drugs have been in shortage, according to the advocacy group Angels For Change. Not only are pediatric oncology drugs more likely to go into shortage than adult essential therapies, but those shortages last about one-third longer than adult shortages.

During the hearing, an oncologist, Dr. Shuman from the University of Michigan Medical School, testified of a critical shortage of etoposide, a drug that is often used to treat children. He reported having to weigh the burden as a clinician to decide which patients, young and old, with lung, brain and testicular cancer, should receive the limited number of available doses. He described the systemic issue adding: "Our pharmacists should not be desperately trying to squeeze out a few last drops when a life may be on the line."

In 2019, there was a national shortage of vincristine—the single most widely used chemotherapy agent in treating childhood cancers. As the New York Times reported the vincristine shortage was just the latest of dozens of drug shortages in recent years, The persistent problem of shortages prompted the Food and Drug Administration (FDA) to review the shortage of sterile injectables.

A 2019 FDA report (updated in February 2020) found that in the ten-year period from 2009-2019, nine of the eleven drugs used to treat acute lymphoblastic leukemia were in and out of shortage. The report went on: "Despite recent evidence that adding nelarabine to children's treatment regimens improves survival rates and is thus becoming the new standard of care, nelarabine has been in shortage recently, causing much anguish and grief for patients, parents, and clinicians."

We acknowledge that the drug shortage issue is complicated and involves multiple factors, such as aging manufacturing facilities overseas and a consolidation of supplier. More than Europe, most experts believe the US is not impacted by shortages and payment policies are the root of many of the shortages. While shortage problems will not be solved by a single solution, we must ensure that families in the future are not faced with devastating shortages of drugs to treat their children's cancer

The above examples frame the history and issues our community has faced and will continue to encounter in the future. They serve as notice that we must act now and put steps in place to mitigate drug shortages.



We wish to re-iterate and emphasize the earlier recommendations made by the Children's Oncology Group during the 2019 drug shortage. As proposed in 2019, solutions that could be enacted in a reasonable time frame for today's children include but are not limited to: (1) establishment and maintenance of a national stockpile of key cancer drugs used for the treatment of children with cancer, and (2) US government purchasing contracts that provide a guaranteed buyer and may help stabilize a fragile market.

There undoubtedly are other ideas to consider, and we are committed to working with lawmakers and the childhood cancer community in galvanizing these efforts. We hope to work with you and the Committee on solutions to this difficult problem. Thank you for your efforts to bring attention to the drug shortages issue.