Testimony of

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Drug Shortages: Examining Supply Challenges, Impacts, and Policy Solutions from a Federal Health Program Perspective

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Introduction

Chairman Wyden, Ranking Member Crapo, and members of the Committee, it is my pleasure to appear before you to discuss the ongoing drug shortage crisis facing patients today. I am Dr. Jason Westin, Professor of Medicine, Director of Lymphoma Clinical Research and Section Chief at M.D. Anderson Cancer Center in Houston, Texas. Today, I am speaking on behalf of the Association for Clinical Oncology (ASCO), the leading oncology professional organization representing nearly 50,000 oncology professionals, including physicians, researchers, and other healthcare providers dedicated to improving cancer care. We appreciate the Committee's bipartisan dedication to addressing the root causes of drug shortages.

Today, I aim to provide a firsthand account of the challenges faced by cancer patients and their healthcare providers amid some of the worst oncology drug shortages to date. This crisis is impacting whether patients receive lifesaving and life-prolonging oncology drugs on schedule and in the established doses or whether we're left to use sub-optimal alternatives, reduce doses, delay treatments, and in the worst situations, unable to provide any of the necessary therapies. Many of my colleagues have been forced to make impossible choices, including to choose which patients will be prioritized to receive potentially curative therapy. Patients and their families look to their providers as a trusted source, and we're left with no explanation.

In the summer of 2022, I started to feel the impact of a potential shortage of a drug called fludarabine, a crucial component of CAR T-cell therapy – an innovative, life-saving technology that teaches a patient's immune system to combat cancer. Fludarabine is a cheap and generic drug, initially approved over 30 years ago, and it is an essential component of CAR T-cell therapy. CAR T is a lifesaving, cutting-edge, "almost science fiction-like technology" that weaponizes the patient's own immune cells to fight their cancer by seeing the cancer cells, the wolf in sheep's clothing hiding in plain sight, but its

efficacy is dependent upon being given with fludarabine. Unfortunately, fludarabine has no known effective substitutes.

My patients with rapidly progressing, aggressive blood cancers, oftentimes only get one chance at CAR T treatment because they may not be well enough to try treatment again. Due to the shortages, I don't know if CAR T will work without fludarabine, and we can't wait to try again when fludarabine is back in stock. Moreover, CAR T is a one-time treatment, and because it is expensive, insurance plans won't cover it twice. In other words, the absence of a generic and cheap drug like fludarabine can mean the difference between life and death.

I recently treated a young mother of three who was battling an aggressive refractory cancer that grew despite multiple chemotherapy lines. Contemplating hospice care, she joined my CAR T clinical trial and is now in a long-term remission, offering her the potential for decades of life and her children the security of having their mom alive and well. Her story—and others like it—would not be possible without common, affordable drugs currently in short supply nationwide.

A colleague in Ames, Iowa, is treating a 21-year-old with testicular cancer. Cisplatin is essential for curing testicular cancer. When he first saw the patient in May of this year, he was able to treat him with Cisplatin, but by August, he had no drug and was forced to withhold care. It's not a situation where we don't know how to treat your cancer, it's that we can't get the drug because it's not being made. We have drugs that are lifesaving and shortages that are life threatening.

Shortages force impossible choices. The oncology care team is forced to work outside of the recommended practice guidelines or must choose how to allocate scarce resources. When physicians must use treatments that may not be standard of care, prior authorization—already an untenable burden—becomes even more intrusive. This added stress to patients and their families, is unacceptable.

The United States needs a more reliable generic drug supply chain to avert future shortages of lifesaving and life-prolonging medications. Most oncology drugs in shortage are old, generic injectables

that sell for anywhere from $1 to $8 per dose, leaving these drugs with slim profit margins, sometimes to the point of production costs exceeding the selling price[1]. Many of these drugs do not have alternatives. There are few manufacturers of these sterile injectables, and the ones that remain in the market face significant costs to remain in business. The leading cause of drug shortages is manufacturing quality issues, which are largely driven by economic factors. Often, any disruptions from quality issues leave the manufacturer unable to ramp up production for several months and at significant expense, that is, if they even choose to resume production. When one experiences quality issues, it has an impact on the entire supply chain. Some manufacturers decide to leave the market completely, while others take weeks or months to make expensive repairs, or they shift production to other more profitable drugs. There is little incentive for companies to enter the market, knowing they may be unable to make any profit on these lifesaving drugs.

Fundamentally, current drug payment policies compound quality issues. Purchasers have limited information – typically only price data - and do not have access to quality or supply information. This creates adverse market incentives for manufacturers to prioritize cost-cutting over quality improvements or capital investments. These are particularly challenging for oncology drugs in shortage, as generic manufacturers often operate on a slim or negative profit margin compared to brand drugs.

The current Medicare payment system bases drug reimbursement on average sales prices (ASP) plus six percent (ASP+6). These amounts are updated using data from previous quarters. Multiple-source drugs can experience artificially low reimbursement because of delays in updating ASP. This creates a barrier to entry for new manufacturers of multiple-source drugs, for increasing production, and potentially for correcting quality issues. Congress should consider alternative payment methodologies

[1] https://accessiblemeds.org/resources/blog/2022-savings-report#:~:text=91%25%3A%20Portion%20of%20U.S.,country%27s%20spending%20on%20prescription%20drugs

that would provide immediate relief from artificially low rates and encourage a more reliable supply of drugs.

While CMS is constrained by statute in how it pays for drugs, it could use its authority to investigate innovative reimbursement structures for sterile generic injectable drugs under the Center for Medicare & Medicaid Innovation's (CMMI's) current authority. For example, CMMI could develop and test demonstration projects that set a reimbursement floor on critical drugs that have been in and out of shortage; investigate novel methods of tying increased reimbursement to guaranteed supply by the manufacturer; or link increased reimbursement to the expansion of quality management maturity pilots already underway, such as the FDA's Center for Drug Evaluation and Research pilot program to promote quality manufacturing and minimize risks to reliable drug supply. At least one public-private utility has already shown proof of concept that purchasers will be willing to pay above spot market prices in return for guaranteed buffer supply.

Additionally, policymakers could incentivize changes to the drug supply chain in several areas: 1.) Encourage the adoption of advanced manufacturing technology and the development of continuous manufacturing for critical drugs and active pharmaceutical ingredients (APIs). Incentives could include tax credits or government contracts for domestic manufacturing. 2.) Consider coupling enforcement mechanisms to the existing requirement that manufacturers of certain drugs develop risk management plans. 3.) Incentivize purchasers to realign contracts with manufacturers with reliable supply. This will require additional transparency in the drug supply chain.

The Department of Health and Human Services (HHS) could incentivize the creation of private-sector reserves of essential medicines, medical devices, and supplies. HHS recently proposed consideration of additional payments to hospitals that acquire and maintain a buffer supply of certain drugs. While such proposals are worthy of consideration, they should be implemented in a manner that does not promote hoarding or create additional shortages or supply chain challenges. They must also

include independent and private practices, with consideration of their different needs. Any incentive programs should be enough to cover the cost of participation, focusing on improved reliability and quality, and should not be budget neutral.

The proposed solutions are immediate steps toward a comprehensive solution. We recognize concerns around increased costs to the health care system. But we will pay a greater long-term cost in the form of delayed or denied care if we do not address underlying economic forces driving shortages of generic drugs.

The shortage of critical cancer drugs is an urgent crisis. My patients, and their families, deserve to know that they will get the care they need without delay. Providers shouldn't have to make impossible choices about patient care.

Thank you for the opportunity to testify on this timely issue. We at ASCO appreciate the Committee's continued efforts to enhance the pharmaceutical and medical supply chain to protect our nation's most vulnerable patients. This is an urgent crisis, and we stand ready to collaborate with you to advance comprehensive solutions that ensure individuals with cancer receive the lifesaving and life-prolonging treatments they require.