Children's Hospital Association Statement for the Record

U.S. House Committee on Energy and Commerce Hearing "Legislative Proposals to Prevent and Respond to Generic Drug Shortages."

Sept. 14, 2023

On behalf of the nation's children's hospitals and the children and families we serve, thank you for holding today's hearing, "Legislative Proposals to Prevent and Respond to Generic Drug Shortages." We applaud your efforts to ensure the U.S. is better prepared to respond to future drug shortages and encourage you to prioritize the unique needs of children, who represent 25% of the total U.S. population. We strongly support bipartisan efforts to strengthen and stabilize the nation's supply chain to forestall drug shortages—particularly those that impact children's health—to help ensure that our nation's most vulnerable pediatric patients have access to safe and effective health care.

We ask Congress to place a strong emphasis on identifying and leveraging pediatric-specific data to inform research and development, procurement strategies and guidance that can provide timely access to sufficient pediatric-appropriate medications and the supplies needed to administer them. It is critical that Congress works with manufacturers, suppliers, industry associations and pediatric providers, including children's hospitals, to mitigate the impacts of drug shortages on child patients and to guarantee that all available supply is shared, as appropriate, with children's hospitals to provide high-quality care.

Drug supply chain disruption implications for pediatric care

Children's hospitals have long faced shortages of critical drugs that their patients rely on for treatment and recovery. A drug shortage is particularly challenging for children because pediatric care requires specialized medications, therapeutics and equipment created with children's growing bodies and smaller size in mind. However, there are fewer manufacturers of pediatric-appropriate drugs and related supplies, which means the pediatric pharmaceutical supply chain is easily disrupted. In fact, a report[1] finds that **more than half of pediatric drugs had only one to two manufacturers currently supplying the dosage form most used by children's hospitals**.

As a result, children's hospitals are disproportionately impacted by drug shortages compared to non-pediatric hospitals. Children's hospitals spend more hours managing shortages than non-children's hospitals—**51 hours compared to 36 hours per drug shortage**. This additional time is often dedicated to compounding replacement products into safe pediatric dosage forms and as a result, children's hospitals are more than twice as likely to hire additional staff. Children's hospitals report that, on average, the cost to manage one drug shortage from onset to correction is **approximately $50,000**, in addition to the actual cost of the drug and routine care delivery.

Pediatric essential drugs—such as antineoplastics, Total Parenteral Nutrition (TPN) components and plasma products used to treat critical conditions including sepsis, cancer and immune deficiencies—are more likely to be impacted by shortages. Ongoing drug shortages also affect lifesaving electrolytes, including calcium chloride, magnesium, potassium, sodium and certain acids, which are essential for maintaining the health of

[1] Pediatric Drug Shortage Trends and Best Practices for Mitigation Strategies. Children's Hospital Association and Vizient. 2020

children's nerves and muscles, including those in the brain, heart, lungs, and other vital organs. Neonates have weight-based caloric requirements that are higher than they will be at any point in life, have a larger need for electrolytes and need specific pH to optimize protein and necessary electrolytes.

Shortages impact care protocols when drugs or supplies must be changed to alternatives, or worse yet, delayed or canceled all together because an appropriate substitute specifically geared for pediatric patients is not as readily available. For example, there is no alternative treatment for pediatric cancer patients who use the oncology drug methotrexate. Sometimes the location of care must abruptly change—care intended to be provided in a community setting close to a child's home may have to be moved to a setting further away, requiring parents and caregivers to take off work and children to miss school. Drug shortages can lead to adverse clinical outcomes for pediatric patients, such as increased rates of drug errors and possible disease relapse, with potentially detrimental impacts on their long-term health and well-being.

Congressional Action Needed

The nation's drug supply chain infrastructure must be strengthened to prevent avoidable shortages of vital pediatric drugs with the goal of providing high-quality products to deliver optimal child health. Thank you for considering the bipartisan H.R. 3008, *Drug Shortage Prevention Act*, introduced by Reps. Jacobs (D-Calif.) and Mills (R-Fla.) in response to the shortage of children's Tylenol. Requiring manufacturers to notify the Food and Drug Administration (FDA) if they experience consecutive weeks of an increase in product demand will minimize drug shortages and improve the FDA's predictive ability and responsiveness.

Several key opportunities for bipartisan congressional action to further prevent and mitigate pediatric drug shortages are highlighted below. In particular, we focus on aspects of the discussion draft, *Stop Drug Shortages Act*, which are relevant to children's access to needed drugs.

Require manufacturer and distributor transparency. We urge Congress to ensure manufacturer and distributor transparency throughout the supply chain, including the location of production and a clear timeline on product availability. The absence of this timely information hinders proactive steps that pediatric providers, policymakers and suppliers can take to collaboratively prepare for and mitigate possible shortages affecting pediatric drugs. Transparency on the availability of pediatric drugs and supplies is vital for providing life-saving treatments to patients.

To that end, we urge Congress to consider ways to address diversion, such as authorizing the FDA to provide certain Drug Supply Chain Security Act (DSCSA) exceptions to allow for streamlined sharing of information on drug product data among manufacturers, wholesalers, providers and pharmacies during shortages. For example, under impending DSCSA regulations, hospitals can share a product only when linked to a specific prescription. Children's hospitals are at a disadvantage when a specific drug becomes short as compared with adult hospitals, which are more likely to be consolidated and can easily trade products.

Therefore, an exception that allows providers to share product based on anticipatory needs during a shortage, rather than solely based on patient-specific needs, would be especially impactful in addressing pediatric drug shortages.

In addition, we recommend that members of the supply chain receive civil penalties for selling drugs to the gray market. When companies operate outside distribution channels not authorized by drug manufacturers, medications are diverted out of the legitimate supply chain. For example, the pediatric cancer drug—methotrexate—has been sold to the gray market and is experiencing a shortage. Diversion of this essential pediatric drug causes delays in chemotherapy infusions and risks child patients' long-term health outcomes.

Strengthen the FDA's authority to prevent avoidable shortages of vital pediatric drugs and related supplies. It is critical that Congress enhance the FDA's authority to work with manufacturers, suppliers and pediatric providers—including children's hospitals—to specifically address and mitigate pediatric drug and related supply shortages through proactive mitigation plans. The FDA plays an important role in protecting the pediatric drug supply chain through assuring access to innovative, safe and effective products.

- **Shelf-life Extension**
  We urge Congress to work with the FDA on exploring safe and effective pathways to allow pediatric providers to use expired pediatric drugs to avoid and mitigate shortages. Expiration dates guarantee a certain length of stability, but many medications can have much longer shelf lives than labeled. For example, an expired supply of the pediatric cancer drug, methotrexate, could still be safe to use as long as providers have accurate and appropriate information about its shelf life.
- **Lag Period for Outsourcing Facilities to Compound and Distribute Drugs**
  In addition, we urge Congress to require that the FDA provide an extended period for 503B outsourcing facilities to distribute drugs after they appear on the drug shortage list. We recommend extending that lag period to at least a year to allow more time for these facilities to recoup their expenses and for hospitals to recover from the shortage impact. A partnership among hospitals and compounding facilities proved crucial when ensuring a stable supply chain of albuterol for pediatric patients after a recent uptick in off-season RSV, flu and COVID-19.
- **Generic Drug Active Pharmaceutical Ingredients (API)**
  We also ask Congress to have the FDA require generic drug application holders to include more information on API manufacturers to better monitor the supply chain and mitigate potential triggers that could lead to pediatric drug shortages. It is important to consider the formulation components and the potential for any adverse effects in a vulnerable age group, especially for children.
- **FDA Drug Shortages List**
  Furthermore, we ask Congress to ensure that an appropriate accounting of pediatric drugs is included on the FDA drug shortages list. Specifically, Congress should require the FDA to adjust its drug shortages list to provide a timely and accurate accounting of pediatric populations and pediatric drug formulations, including the potential for regional shortages.

Ensure access to drugs subject to penny pricing. Children are largely insured by Medicaid and the Children's Health Insurance Program, but children's hospitals qualify for 340B because a significant shortfall exists between the cost of care and Medicaid payment. Children's hospitals depend on the 340B program to provide vulnerable patients with access to life-saving medications. Penny pricing is an important tool to ensure that manufacturers do not raise prices of 340B drugs above inflation.

We urge Congress to establish guardrails to prevent problematic actions that could impact access to 340B drugs subject to penny pricing related outcomes. For example, the producer of a leukemia drug that was successfully used in pediatrics for many years was recently subject to penny pricing. The manufacturer later announced the removal of the pediatric indication—without clinical justification—for a more profitable drug. These types of unwarranted changes in pediatric drug availability coming from manufacturers' inappropriate reactions to penny pricing can lead to uncertainty in the supply chain for our most vulnerable populations.

In conclusion, efforts to address drug shortages throughout government must be aligned, coordinated, strengthened and adequately funded to support the needs of pediatric patients. Developing a more efficient and reliable pediatric drug supply chain will help children by allowing children's hospitals to identify and leverage data to reduce supply spend, improve organizational effectiveness, and strengthen educational opportunities for clinical staff so pediatric patients received the highest quality products in a timely manner.

Thank you again for your commitment to ensuring the needs of children are met when addressing drug shortages. Children's hospitals stand ready to partner with you to advance policies that will make measurable improvements in the lives of our nation's children.