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Sudden Price Spikes in Off-Patent Drugs: Perspectives from the Front Lines
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Madam Chair and Members of the Committee, thank you for the opportunity to address the impact of recent changes in drug pricing for pediatric and adult health care. For the past 21 years I have been a pediatric infectious diseases physician at the University of Alabama at Birmingham and Children's of Alabama, where I serve as Co-Director of the Pediatric Infectious Diseases Division and as Vice Chair of Clinical and Translational Research for the Department of Pediatrics. UAB is one of the top academic medical centers in the country, ranking in the top 25 of all institutions and the top 10 of public institutions in NIH funding. The UAB Department of Pediatrics practices in Children's of Alabama, which is the third largest children's hospital in the country in physical size and is consistently among the top pediatric programs nationally. I am the immediate-past president of the Pediatric Infectious Diseases Society, which is dedicated to the treatment and control of infectious diseases affecting children. I also am the editor of the American Academy of Pediatrics Red Book, which is often referred to as the 'bible' describing infectious diseases for pediatricians across the country and throughout the world. The views expressed in this testimony are my own.

I personally treat and provide advice to physicians caring for babies and immunocompromised patients who are infected with *Toxoplasma gondii*. This is a parasitic infection that causes life-threatening disease in patients whose immune systems are not strong. Specifically, when pregnant women acquire toxoplasma infections they can transmit the parasite to their fetus, resulting in brain damage, blindness, deafness, and even death. The toxoplasma organism is carried by cats, and this is the reason that pregnant women are not supposed to change the litter box. Up to 4,000 babies are born each year in the United States with congenital toxoplasmosis. *Toxoplasma gondii* also can cause life-threatening brain and vision-threatening eye infections in

children and adults with weakened immune systems, including cancer patients and people with HIV. The good news is that the infection can be successfully treated with a combination of two very old and well understood drugs, pyrimethamine and sulfadiazine. However, recently the price of pyrimethamine has increased more than 5000%, and restrictions have been placed on where physicians can obtain it for their patients. I am very concerned that these changes will directly put the lives of patients with this very severe infection at risk.

I first became aware of the sale of pyrimethamine to Turing Pharmaceuticals in late August. A pregnant woman at my institution had just been diagnosed with toxoplasmosis. Knowing that the baby would be delivered in early September, my team and I began seeking access to pyrimethamine and sulfadiazine for the baby. The barriers that we were facing, though, were two-fold: 1) the massively increased cost of the drug following Turing's purchase; and 2) the fact that a liquid compounded pyrimethamine could not be acquired in the outpatient setting through Turing's distribution system using a specialty pharmacy.

The reason that the pharmacy issue was a challenge is because babies cannot swallow pills, but pyrimethamine is only available in 25 mg tablets. In order to get the medicine into a liquid formulation, the tablets must be compounded in a pharmacy. Prior to Turing's purchase of pyrimethamine, the outpatient community pharmacy that we use in Birmingham could acquire the drug from the previous manufacturer. However, our pharmacy cannot acquire the drug from the distribution system set up by Turing due to restrictions in the sale of medications from one pharmacy to another, which threatened to block our access to a liquid formulation that we would need. When we contacted the specialty pharmacy, we had concerns about its experience in doing

such compounding with pyrimethamine, so we were facing a situation where we might not be able to acquire the drug in a form that could be taken by a baby.

The other challenge that we faced was the price of pyrimethamine. Initially my patient required four tablets to make a one month supply of pyrimethamine. Prior to Turing's purchase of the drug, this would cost approximately $54 per month. After Turing's purchase of pyrimethamine, the cost is no less than $3000 per month and probably more. Babies with congenital toxoplasmosis need to be treated for 12 months and the dose of the drug increases as the baby grows, so the total treatment cost before the Turing purchase was approximately $1,200 but now is estimated to be no less than $69,000 and probably significantly more. Looked at from another angle, the total 12 month cost before the Turing purchase now would buy less than two weeks of treatment at the new price. For HIV-infected adults with toxoplasma brain or eye disease, who require 2 or 3 tablets per day, the total costs now would approach no less than $500,000 whereas in mid-summer before the price increase it would have been approximately $8,500. The key issue for this Committee, from my perspective, is the order of magnitude of this change.

As we explored our options for the treatment of this little baby, we had a fortunate break develop. The pharmacist who works with me went to the outpatient community pharmacy in person and found a supply of pyrimethamine already on their shelves that had been purchased prior to the price increase. As a result, I was able to start my patient on pyrimethamine and sulfadiazine within the first two weeks of her life. The monthly cost so far has been $64.26 per month, and my patient is doing well.

On behalf of the babies being devastated by this infection, their mothers and families, I thank you for your consideration of these challenges. Babies' lives literally hang in the balance here, and it is encouraging to me to see the Senate take up this important issue.