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Help children with rare diseases get the new medicines they need

By Nicole Boice

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Having a seriously ill child is always difficult and painful. It's even more so when a child's illness is one of the 7,000 rare diseases for which cures and treatments are hard to find.

Rare diseases affect more than 15 million children in the United States. A disease is considered rare if it affects less than 200,000 people; however the majority of rare diseases are considered 'ultra rare' meaning that they affect less than 6,500 patients. Diseases such as Cystic Fibrosis, Muscular Dystrophy, Huntington's disease and pediatric cancers are some of the better known rare diseases that strike children and adults. But there are thousands more that touch only a few hundred lives and are virtually unknown to the broader public.

Because these diseases are rare, support networks are hard to find and information can be difficult to obtain. Even getting a diagnosis can be a challenge because doctors may not know what they're looking for. And when the diagnosis comes, it is often followed by the frustrating news that no cure exists and treatment options are minimal.

But there's great hope in the rare-disease community these days because new advances, including our growing understanding of the human genome, are leading to the development of exciting and potentially lifesaving treatments. Currently 460 new medicines are in clinical trials or under review by the Food and Drug Administration (FDA) for rare diseases. It's essential that we get them to patients as quickly as possible.

Under a law called the Prescription Drug User Fee Act (PDUFA), pharmaceutical companies pay a mandatory fee to help provide needed resources to ensure the FDA has more expert staff to review applications for new drugs in a timely fashion. PDUFA established a goal that all drug applications be reviewed within 10 months; the goal is six months for priority review medicines, which are those that may offer a major advance in treatment or medicines that offer treatment where none currently exists, as is often the case for medicines to treat rare diseases. To meet that goal, PDUFA provided the FDA with a new revenue stream -- in addition to congressional appropriations -- by creating a system of user fees paid by pharmaceutical companies who are seeking review of new medicines. This combination of public and private funding has allowed the FDA to hire more than 1,500 additional reviewers and ensure that the process is efficient. While companies don't pay fees for the review of drugs intended to treat rare diseases, the user fee program supports the review of all new drug applications.

Generally, PDUFA has worked. After the law was passed in 1992, review times for new drugs fell by about 60 percent. Safety continues to be the top priority. In 2007, industry and FDA agreed to dedicate some of the private sector user fee funding to safety activities—to better monitor medications after they are approved for patient use.

PDUFA needs congressional reauthorization every five years, and we're coming up on another reauthorization next year. It's a long process, as hearings begin this week in the U.S. House. The FDA has already issued its proposed language to the Office of Management and Budget for regulatory review. As members of the Administration and Congress move forward on reauthorization, I urge them to ensure the law meets its original objective – the efficient and safe review of new drugs.

Recently, the length of time it takes new drugs to get through the review and approval process has been climbing. A key reason for the missed deadlines is the congressional mandates which were introduced as part of the Food and Drug Administration Amendments Act in 2007 (the last reauthorization of PDUFA legislation). While these were well-intentioned, they have caused unnecessary delays for patients desperately waiting for new treatment options.

One such requirement is for an outside Advisory Committee of experts to discuss most drug applications and provide their counsel. The law also imposed more limits on who could serve on the committees. While valuable in

theory, in reality the process for scheduling such time-intensive meetings often slows the review process. In fact, as of last April, one-third of Advisory Committee positions were vacant. When dealing with treatments for rare diseases, it can be difficult to find medical professionals who have enough knowledge of a particular disease and also have never worked with the pharmaceutical companies developing new treatments. Their prior work to bring about cures can make them ineligible to participate on Advisory Committees.

On the whole, PDUFA works well and the FDA, along with pharmaceutical companies, is bringing hope and needed relief to millions of patients and their families, in the form of new treatment options. As PDUFA gets reauthorized, it's important to learn from past experience. The FDA should have the resources it needs to ensure that safe, effective medicines reach patients as quickly as possible, and that medicines that don't meet the FDA standards don't reach the market. The best way to do that is to make the review process consistent and transparent, with clear communication between regulators and drug makers. Recent revisions to PDUFA should be assessed to determine whether they make the process stronger or simply add time.

The 460 potential treatments for rare diseases that are currently in the clinical trial and review process could be vital lifelines for young patients. To turn hope into reality, let's make sure PDUFA stays true to its mission: a public-private funding model advancing health in America.

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