

Removing Barriers to New Treatments

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Life is full of trade-offs. No one understands that better than a person with a chronic disease or disability.

For example, alpha-1 antitrypsin deficiency is an inherited condition that results in chronic lung and liver disease at various ages. Some treatment options can cost an average of more than \$130,000 a year, and there is no cure. People diagnosed with alpha-1 antitrypsin deficiency want treatments with lower out-of-pocket costs today. They also seek more research to find better treatments.

A frequently discussed trade-off for the patient community at large is affordable versus improved treatments. Economists have viewed it as a trade-off between more intensive price competition and incentives for new product development.¹

Out-of-pocket medical costs have risen more than 25% over the past 5 years.² Should there be a push to quickly bring cheaper treatments to market? Or should the emphasis be on developing new or improved treatments? In focus groups conducted by the National Health Council, people with chronic diseases and disabilities have told us that they want affordable medicines. But, they also seek better treatments with the hope that their children and grandchildren will not face the same limited treatment options if given the same diagnosis.

Currently, companies are granted a 20-year patent for the discovery of new compounds and new treatment methods. After an application is filed and the patent granted, the company conducts lengthy clinical trials to prove efficacy and safety to receive US Food and Drug Administration (FDA) approval to take the product to market. The goal is to complete the trials, receive FDA approval, and begin mass production long before the life of the patent expires.

The only way a new drug or treatment is commercially viable is if it has *both* a patent and regulatory approval.

For example, drugs known to kill cancer cells were patented in the 1950s, but the treatment process could not control toxicity levels in the patient. Today we have treatment methods approved by the FDA that could make utilization of these drugs safer, but the patents on the compounds have expired. Consequently, the pioneer company

will not pursue development of a new treatment regimen if the drug has no patent.

On the other hand, companies will abandon a patented drug if the process to win FDA approval is so onerous that the patent will expire before that goal is achieved. Because of the long clinical trial time and regulatory review period, much of the 20-year patent life is lost prior to final FDA approval.¹

Now is the time for Congress to further the creation of new and better treatments, which in turn will lead to lower-cost generic drugs of the future.

As many as 5.3 million Americans are living with Alzheimer's disease.³ Nearly 18 million people have been diagnosed with diabetes.⁴ Yet drugs that may arrest the progression of these diseases have not been brought to market for the very simple reason that the clinical trials necessary to prove the drugs' ability to slow or stop the disease progression take longer than the life of the drug patent. The result often is discontinuation of the research; in some instances, the research phase is not begun.

Our country has used federal policy to strike the right balance between affordable access to medications and encouraging companies to create new breakthrough treatments. For example, the Hatch-Waxman Act provides for partial restoration of some of the patent time lost during the clinical and regulatory processes. Under this provision, the pioneering company receives an extended term on the patent to compensate for the time lost in regulatory review. The Hatch-Waxman Act also provides incentives for development of lower-cost generic drugs following expiration of the patent protection period.⁵

Generic drugs are scientifically equivalent to the pioneer drug. To bring their product to market, a generic

company is not required to conduct a clinical trial to prove its safety and efficacy. Instead, the generic company depends on the clinical trial data previously submitted by the pioneer drug company and made available at the end of its “data exclusivity” period. This is a set period of time after which a generic company may rely on the innovator’s data to pursue regulatory approval of the generic drug. As a result, generic drugs are priced on the manufacturing costs—not the development costs—which dramatically reduce the purchase price of the products. Generic drugs, which typically cost less than pioneer medications, now account for more than 50% of all US prescriptions.¹

The 1983 Orphan Drug Act has been successful in increasing the number of new drugs approved to treat rare diseases, defined as conditions affecting fewer than 200,000 patients. Since 1983, the Orphan Drug Act has resulted in the development of more than 250 new treatments.⁶

As part of healthcare reform, Congress adopted language that creates a pathway for the development of biosimilars. These complex medications can provide life-altering benefits for some patients. Similar to generic drugs, biosimilars are intended to be a lower cost alternative to the original biologic.

In the past, Congress has demonstrated its willingness to create a means to direct and encourage the development of new treatments so desperately needed by people living with chronic diseases, balanced by policies that have helped to lower patient out-of-pocket costs.

People with chronic conditions face the greatest loss

when new treatments are not brought to market. The potential return of investing in new treatments is immeasurable. Just ask a person diagnosed with Parkinson’s disease or the spouse of someone with Alzheimer’s disease. These individuals face a life without a cure for themselves or a loved one.

Now is the time for Congress to further the creation of new and better treatments, which in turn will lead to lower-cost generic drugs of the future.

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