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Scientists are testing pricey rare-disease drugs as Covid-19 treatments. What if one works?

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Some of the existing drugs scientists are testing as Covid-19 treatments have a special status for rare disease treatments, and the price tags to match — prompting early warnings from academics and drug pricing reform advocates that if one is effective, access could be an issue.

At issue are so-called "orphan drugs," which get special exclusivity perks from the Food and Drug Administration because they treat a rare disease. Drugs get to keep that designation — and the perks — even if they're later approved for a

broader category of patients. That exclusivity often translates into higher prices, especially for rare disease drugs where demand is expected to be low.

Demand for any Covid-19 treatment, however, will be high. That alone has prompted calls from drug pricing advocates for the government to ensure drug makers keep any treatment or vaccine affordable. And now some are calling into question whether drug makers should maintain the perks of the orphan drug status, too, if any of the 17 orphan drugs being tested as Covid-19 treatments proves effective. Already a similar backlash inspired Gilead, which makes the high-profile medicine remdesivir that's been proven effective as a Covid-19 treatment, to rescind its application for an orphan designation for that drug, giving up exclusivity it would have otherwise enjoyed.

"The nightmare situation really is [a] breakthrough, extremely effective Covid-19 treatment that's priced so high that hospitals and patients can't access it," said Kao-Ping Chua, a pediatrics researcher at the University of Michigan who co-authored a study published in Health Affairs that examined the orphan drugs that scientists are testing as Covid-19 therapies.

Tom Wilbur, a spokesman for the trade group PhRMA, said in a statement that the industry is dedicated to affordable pricing for any Covid-19 treatments.

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Scientists are testing hundreds of existing medicines as potential Covid-19 treatments, hoping to alight on one that will alleviate the symptoms associated with the novel coronavirus or hasten the body's natural immune response to the disease.

Existing medicines also already have set prices — and for some, designated status as an "orphan drug" meant to treat a rare disease. The designation, established by Congress, is intended to encourage drug makers to research medicines they might not otherwise have financial incentives to research. The FDA grants benefits

including an extra two years of exclusivity over non-orphan drugs, tax breaks for development, and clinical research subsidies.

Drug makers often bestow orphan drugs with high price tags, arguing that the smaller market for them justifies a higher price. One study from insurance companies, who are vocal opponents of high drug prices, showed that 88% of drugs with an orphan designation cost each patient who takes them more than $100,000 annually.

And that's true even when the drug is later approved for a broader population, several researchers told STAT. Most American drug makers charge the same price for a drug no matter what it's treating, according to Chua.

"If you're approved for an orphan drug, and you have very little competition, and … no other drugs can be approved for that indication, you basically can charge whatever you want," said Kenneth Kaitin, director of the Tufts Center for the Study of Drug Development. "It seems like it's unfair, then, that if you're now using that same drug for non-orphan use, you should charge the same amount, and yet, it's most likely that that's what companies will do. They're not going to suddenly lower their price."

Seventeen orphan drugs are being tested as Covid-19 treatments, according to the Health Affairs study. They range in price from $553 for a 4-milliliter monthly intravenous dose of Actemra, a drug used in rheumatoid arthritis patients, to over $16,000 for a month's supply of Jakafi, a bone marrow cancer treatment.

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Already, patient advocates are worried that those price tags and the other special perks associated with orphan drug status shouldn't transfer to a Covid-19 treatment, since it would be used widely. The federal government has already received or purchased about 620,000 treatment courses of remdesivir, the only treatment for Covid-19 that has been shown to reduce recovery time, for example.

“It’s one thing if they are developing drugs for a very rare disease, and the only way they can break even or make money on it is to charge a lot of money. But it’s another thing when, in many cases [with] these drugs, the costs could be recouped in a year … and the companies are actually making an enormous profit,” said Diana Zuckerman, president of the National Center for Health Research, a consumer advocacy group.

She said Congress should close loopholes that allow orphan drugs that have more common uses or treat larger populations than originally intended to keep the perks associated with orphan drug designation.

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Both Zuckerman and Kaitin also said they hoped public scrutiny and pressure on drug companies might encourage companies to keep Covid-19 therapies reasonably priced, the way Gilead rescinded its application for orphan status.

But Chua, the University of Michigan researcher, was skeptical.

“These are profit-driven entities, so there’s not a lot of motivation [to lower prices] other than goodwill, and I guess maybe these PR effects,” he said. “I don’t know that you can rely on that.”

A spokesperson for the National Organization for Rare Disorders, which was instrumental in passing the Orphan Drug Act, emphasized that most of the 17 drugs being tested for Covid-19 therapies have even longer-lasting exclusivity perks because they are biologics, or drugs derived from living organisms. Biologic drugs, he said, “typically have patents and other forms of marketing exclusivity that permit extended periods of monopoly pricing, well beyond those provided under the Orphan Drug Act.”

He added that NORD disapproves of any abuse of the Orphan Drug Act during the pandemic, including Gilead’s attempt to utilize it for remdesivir.

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Ultimately, though, most experts argued that the main culprit isn't the perks, but the price, and called for broader drug pricing reform. "Orphan drugs and specialty drugs have become the poster child for pricing exorbitantly," said Rena Conti, a professor at Boston University and the other author of the Health Affairs study.

Kaitin agreed. "We have a much bigger discussion that needs to happen in this country about drug pricing," he said. "[Orphan drug designations are] just one small part of a health care system that enables pricing that sometimes makes it impossible for people to afford their medications."

About the Author Reprints

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