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Orphan Drug Designation For Remdesivir Is 'Consistent' With Precedent, Former FDA Lawyer Says

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by Brenda Sandburg | @brendasandburg | Brenda.Sandburg@informa.com

Executive Summary

Attorney who drafted Orphan Drug Act implementing regs says companies need incentive to develop a treatment that may not be available until crisis passes; KEI is mulling citizen petition to request revocation of the designation for Gilead's investigational COVID-19 product.

GILEAD'S REMDESIVIR RECEIVES ORPHAN DRUG DESIGNATION FOR COVID-19

The US Food and Drug Administration's decision to grant orphan drug designation to Gilead Sciences Inc.'s remdesivir for COVID-19 has raised questions about whether the award should be given when the treatment population is expected to surpass the orphan limit of 200,000 in the US.

But Frank Sasinowski, former deputy director of health policy staff at FDA who drafted the Orphan Drug Act's implementing regulations, says the designation for remdesivir is consistent with FDA precedent, with the implementing regs, and with what the agency did during the AIDS crisis.

The intent of the implementing regs was to "take a snapshot the moment the request" for orphan drug designation comes in and see if 200,000 people need the treatment at that moment, Sasinowski, now a director at Hyman, Phelps & McNamara, told the Pink Sheet. People ask of the remdesivir orphan designation, "isn't that crazy?" Sasinowski said. "No, no, not at all."

"We want to incentivize companies to develop a treatment," he said. "By the time anyone does the research, makes a submission, gets approval, manufactures and supplies it, we might be beyond the current crisis and we don't know if it will come back."

Sasinowski compared the COVID-19 pandemic to that of the AIDS crisis. Although AIDS was not a pandemic, it was a massive public health crisis. Glaxo Wellcome Inc. obtained orphan designation for AZT in July 1985, at which time everyone suspected that the number of people with AIDS would increase beyond 200,000.

Remdesivir Will Be 'Accessible And Affordable'

The FDA posted a notice on its website that it granted orphan designation to remdesivir for treatment of COVID-19 on 23 March. The designation provides seven years of marketing exclusivity once the product is approved and tax credits for qualified clinical testing.

It is unclear what patent protections remdesivir will have and how essential the exclusivity is to Gilead.

As of 23 March, there were 44,183 cases of COVID-19 in the US and 544 deaths, according to the US Centers for Disease Control and Prevention.

In addition to its clinical trials, Gilead has provided the investigational drug, which is administered intravenously, to several hundred patients with COVID-19 on a compassionate use basis for emergency treatment outside of ongoing clinical studies. The company announced 22 March that due to the overwhelming demand for the drug, it was transitioning from individual compassionate use requests to expanded access programs. (Also see "Gilead's Expanded Access Shift Offers New Data Collection Opportunities For Remdesivir" - Pink Sheet, 23 Mar, 2020.)

Asked about its rationale for seeking orphan drug designation, Gilead replied, "In response to this global health emergency, Gilead has been making significant at-risk investments and is working around the clock to study the safety and efficacy of remdesivir as a potential COVID-19 treatment, to responsibly provide emergency access to remdesivir while these studies are ongoing, and to scale up to supply as much as possible."

"If remdesivir is proven to be safe and effective to treat COVID-19, we are committed to making the medicine both accessible and affordable to governments and patients around the world," the company said.

Hurdles For Pandemic Products

Alexander Varond, an attorney at Goodwin Procter, said a major hurdle for pandemic products is that many, such as vaccines, would be for prevention and likely would not qualify for orphan drug designation because of the large number who would be vaccinated.

Products for the treatment of COVID-19 will have an easier time, at least initially, to get an orphan drug designation, he said, as prevalence is defined as the number of persons in the US who have been diagnosed as having the disease or condition at the time the request is submitted.

Varond noted that a number of products to treat outbreaks that had the potential to be pandemics have received orphan drug designation, including those for Ebola and small pox. Remdesivir received orphan drug designation for treatment of Ebola in 2015. Aphidicolin, pyronaridine tetraphosphate, quinacrine, and ranpimase also received an orphan drug designation for Ebola.

Limitations To Orphan Drug Designation

Dan Orr, former FDA regulatory counsel and partner at Womble Bond Dickinson, noted that there are limitations to orphan designation. For example, if a sponsor is unable to provide sufficient quantity of a drug to treat all patients, that could be a basis for precluding exclusivity and giving other sponsors an opportunity to provide the drug.

In addition, Orr said the scope of exclusivity is narrower than other forms of exclusivity. Sponsors seeking approval for a follow-on drug for the same rare disease or condition as the first designated product must be the "same drug" with the same active ingredients. Also, a sponsor of a follow-on product may obtain approval if it is deemed to be "clinically superior" to the prior product. Orr said there are a number of factors to determine if a follow-on is clinically superior, including whether it significantly contributes to patient care.

He said this has led to theorizing that if a follow-on is significantly less expensive so it can be accessed by a greater number of people, that could potentially be a basis for orphan exclusivity not having preclusive effect.

As for whether remdesivir could lose orphan drug designation if the number of people in the US infected with COVID-19 surpasses 200,000, Orr said that is unlikely. He noted that a number of blockbuster drugs got their start as orphan designated products and the patient population subsequently exceeded the orphan limit.

Groups Seek Revocation

Some patient advocacy groups object to the orphan drug designation and want FDA to revoke it. Patients Over Pharma, a project of Accountable.US., which says it is focused on exposing the ties between the Trump Administration and the pharmaceutical industry, called for an immediate reversal of the decision.

"There is no doubt that the number of people who will suffer from COVID-19 will surpass 200,000 very quickly, so for Gilead to scramble to apply for orphan drug status before that happens is craven even by Big Pharma's standards, and for their request to be granted by the FDA is absurd," the group's spokesperson Eli Zupnick said in a release.

Knowledge Ecology International Director James Love said his group might try to make a case that there was insufficient evidence for FDA to make the orphan drug determination. He said KEI may first file a citizen petition requesting revocation of the designation.

Democratic presidential candidate Bernie Sanders, I-VT, issued a release calling on President Trump to rescind the designation.

"It is truly outrageous that after taxpayerrs put tens of millions of dollars in developing remdesivir Trump's FDA is exploiting a law reserved for rare diseases to privatize a drug to treat a pandemic virus," Sanders stated. "When Jonas Salk developed the polio vaccine 65 years ago, he understood the tremendous value it would have for all of humanity and he refused to patent it. Right now, we must put human life above corporate profit. We cannot give pharmaceutical corporations a monopoly on treatments that could save millions of people during this crisis."

[*Editor's note: A new Coronavirus Analytic Solution including comprehensive integrated data on clinical trials, pipeline, market events and insights from across Pharma Intelligence's suite of products, updated daily, is now available. Contact Duncan Emerton, PhD; Director, Custom Intelligence & Analytics for details (separate*

purchase required).]