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HEALTH

Monkeypox Pill Puts Drugmaker on the Map

SIGA has received $60 million in orders for Tpoxx, which was developed to treat smallpox in the event of an attack.

By Joseph Walker | Photographs by Michelle Gustafson for The Wall Street Journal

In the aftermath of 9/11, SIGA Technologies Inc. started work on a drug it hoped would never be used. Now, Tpoxx is playing a surprising role as one of the few available treatments thought to be effective against monkeypox.

SIGA's employees spent nearly two decades developing Tpoxx to treat smallpox in case of a bioterrorism attack. Monkeypox is a related virus that is mainly infecting men in the U.S. and other countries who have sex with men.

Like Bavarian Nordic AS, the only maker of a vaccine licensed for monkeypox, SIGA is largely alone on one front of the response to an epidemic that has caused more than

13,500 cases in the U.S.

The company said it is juggling calls from governments around the world, leading to $60 million in Tpoxx orders from places including Europe and Canada. SIGA's market value has more than doubled this year to $1.54 billion. SIGA Chief Executive Officer Phil Gomez compared the experience to being a reserve player on a team that has made it to the championships.

"All of a sudden you have to be ready to get on the court," he said.

U.S. officials have made Tpoxx available for seriously ill monkeypox patients, drawing on its stockpile of 1.7 million treatment courses that it acquired in the event of a smallpox outbreak.The Biden administration said it would make available 50,000 treatment courses to local health departments this week in addition to 20,000 courses sent previously.

SIGA relies on partners including Catalent Inc. to make the pills and Packaging Coordinators Inc. to bottle and box them. Tpoxx was approved in the U.S. to treat smallpox in 2018. Because Tpoxx hasn't been approved to treat monkeypox, it isn't always easy for infected patients to get it.

Under special protocols created by the Centers for Disease Control and Prevention, Tpoxx can be used to treat patients with severe symptoms, which can include bleeding and excruciatingly painful sores on the genitals. Doctors and support staff have to submit paperwork on each patient they treat and conduct patient follow-up visits.

Tpoxx never underwent human testing because smallpox was eradicated decades ago. Instead, the drug was tested in animals against viruses from the same genus as smallpox, including in monkeys with monkeypox.

The data showed that monkeys given a lethal dose of monkeypox were saved when given Tpoxx, said SIGA Chief Scientific Officer Dennis Hruby, who works from his home in Bend, Ore. Most of SIGA's employees work at the company's research-and-development facilities in Corvallis, Ore., near Oregon State University, where Dr. Hruby previously served as chairman of the microbiology department.

SIGA, which has its corporate headquarters in New York City, was founded in 1995 to develop a strep-throat vaccine using technology created by founding scientists Vincent Fischetti at Rockefeller University in New York and Dr. Hruby.

By 2001, SIGA shares were faltering, and the company was largely relying on grants from

the National Institutes of Health, Dr. Hruby said. After Sept. 11, the federal government made billions of dollars in new research funding available for biodefense research. SIGA shifted its focus to drugs and vaccines that could be used in the event of a bioterrorism attack. SIGA was awarded more than $1 billion in government contracts to develop a smallpox drug and manufacture it for the U.S. stockpile.

SIGA acquired the rights to Tpoxx in 2004. The drug appeared to be effective against smallpox in laboratory testing, but animal studies looked less promising, said Dr. Hruby. The company bet that it might bring the drug to market if it could figure out dosing, he said.

"We were developing a drug for a disease that doesn't exist," said Dr. Hruby. "It took a long time to figure out."

SIGA's phones began ringing soon after the monkeypox virus was detected in the U.K. in May. Doctors outside the U.S. asked how to obtain Tpoxx. U.S. doctors had questions about administering the drug, which typically requires six pills daily over two weeks. To boost the drug's effectiveness, patients take the pills after a high-fat meal.

Dr. Gomez said he fielded a Saturday-night phone call in May from a European public-health official eager to obtain Tpoxx doses as cases mounted in the official's country. It took two months of wrangling with the country's contracting officials to secure the order.

Tpoxx appears to be safe, and the animal data supports its use as an antiviral against monkeypox, doctors said. Doctors reported in the Journal of the American Medical Association on Monday that among 25 patients treated with Tpoxx from early June to mid-August, 40% had their lesions healed after one week and 92% had their lesions healed and were without pain within three weeks. The study's authors said large-scale studies are needed to determine whether patients recover because of the drug or the natural course of disease.

Because the drug hasn't been proven to work in humans, the CDC requires that doctors collect and submit data from patients and have at least two follow-up visits with patients who have been prescribed the drug, even if they are feeling better.

The U.K. approved Tpoxx to treat monkeypox in July, and the European Medicines Agency approved it in January for smallpox, monkeypox and cowpox. The U.S. is unlikely to authorize Tpoxx formally for monkeypox before it sees human study data, which could start coming in by the end of this year, SIGA executives said. A U.S. study is being planned by the National Institute of Allergy and Infectious Diseases, a spokeswoman said. Oxford University researchers in the U.K. began a study this month.

“It is really important to know whether or not this drug works,” said Jason Zucker, an assistant professor of medicine at Columbia University Irving Medical Center, which has prescribed Tpoxx to more than 100 patients.

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