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Shire Poised to Gain on Genzyme's Troubles

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U.K.-based Shire plc stands to reap enormous benefits as its competitor, Cambridge-based Genzyme Corp., continues to struggle with manufacturing woes that sunk its 2009 fourth-quarter earnings by 73 percent.

Shire, with two facilities in Massachusetts, is very likely to receive U.S. Food and Drug Administration approval before the end of this month for a rival drug to Genzyme's blockbuster treatment for Gaucher disease, Cerezyme. News Wednesday that Genzyme will have to further delay the full production of both Cerezyme and Fabrazyme, its treatment for Fabry disease, is a further boon for Shire.

Analysts say now a second Shire drug target for Fabry disease is all but certain to be fast-tracked by the FDA, too.

In June, Shire received a very unusual call from the FDA. Genzyme had temporarily closed its Allston plant, following the discovery of a virus. The FDA needed Shire's help to avert a crisis for patients of two rare diseases, since Genzyme's inventory could not meet demand. The FDA offered to allow Shire's drug targets to treat Gaucher disease and Fabry disease to be prescribed before regulatory approval, and to fast-track approval for the Cerezyme drug target, velaglucerase alfa. For the specialty pharmaceutical company, whose Human Genetic Therapies division is headquartered in Cambridge and Lexington, it was a tremendous opportunity and a great challenge.

"There was definitely a lot of frenzied activity, but the workers had a strong commitment to get this done," said Bill Ciabrone, senior vice president of technical operations for Shire HGT. Ciabrone lengthened shifts and shortened the waiting time between different steps in the process. The company also pushed up construction of its new plant in Lexington.

Michelle Neumann of Long Island, N.Y., was distressed to find she would not receive her Cerezyme treatment. She said without years of treatment, she would likely have died from complications of an enlarged liver, a common Gaucher symptom. Neumann said

she's grateful to Genzyme, the first company to invest in a treatment for her disease. But after forgoing treatments for a few months due to the shortage, some symptoms returned.

"The company was very good about keeping us informed, but it was quite a few months longer than what they said at first," Neumann said. Her doctor suggested she enroll in the clinical trial for Shire's velaglucerase alfa, and so far, she said, she is feeling better. The hard part is deciding what to do once Cerezyme becomes widely available again. Genzyme said Wednesday that won't be until late April.

"That's kind of the million-dollar question. Right now I'm thinking of sticking with this (Shire's drug), unless I can't tolerate it anymore, or my insurance won't cover it," Neumann said.

Shire officials say they haven't projected how much of a market share they can hope to command, except to say that between 300 and 600 patients are now taking the drug target. Jon Stephenson, senior biopharmaceutical analyst for the Boston-based health care brokerage firm Summer Street Research Partners, estimates that Shire could gain 20 percent of the Gaucher market by 2011. Given that Genzyme's worldwide revenue for Cerezyme in 2008 — before the shortage — was \$1.2 billion, this would be a hefty sum. Stephenson noted that there is uncertainty about this because an Israeli firm, Protalix, also has a late-stage drug target for Gaucher that has also been provided to some patients before approval. Shire officials would not disclose the price of its drug, but it is not expected to be much cheaper than the \$200,000 per year price tag for Cerezyme.

Shire will also receive a bump from the expected accelerated approval of its Fabry drug target. Shire's treatment, Replagal, is already approved outside the U.S. and enjoys between 30 percent and 40 percent of the foreign market. Stephenson expects Shire will take in an additional \$100 million per year, but not for several years. Still, Stephenson, said, it's enough of a revenue infusion to boost his target share price for the company by \$1 or \$2.

"I asked Shire recently if they had sent Genzyme a thank-you note," Stephenson said.

Lori Gorski, a spokeswoman for Genzyme, said the company is not concerned about the threat posed by Shire. "We've been expecting competition, and planning for competition for a long time, well before the supply disruptions," she said.

Those supply disruptions continue. Genzyme announced Thursday that in order to build up inventory, the company would reduce shipments of Cerezyme to 50 percent for eight weeks, meaning that patients will get half their doses. Shipments of Fabrazyme now stand at just 30 percent of demand, down from 70 percent, due to lower than expected production. Gorski said the company will bring on new measures to increase fabrazyme production by June, pending approval by the FDA.