Genzyme steps in as Shire pulls back on Fabry drug

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Cambridge-based Genzyme Corp. [1] will step in to supply medicine to 140 U.S. Fabry disease patients who are about to lose access to rival Shire plc's (Nasdaq: SHPGY) drug, after Shire withdrew its drug's FDA application. But the sudden windfall of patients may slow Genzyme's ramp-up of Fabrazyme supply to European patients at a time when the company is just regaining its manufacturing sea legs, following an almost three-year shortage of the drug.

Patients with the rare Fabry disease were expecting this week to be sitting in a packed FDA advisory committee panel meeting, testifying in favor of U.S. approval for Shire plc's) Fabry drug, Replagal. Instead, they are scratching their heads after Shire withdrew its application from the regulatory agency following written comments from the FDA indicating the agency would require costly additional trials that would take years to complete.

"We are profoundly disappointed about this outcome," Phil Vickers [2], executive vice president of Shire Human Genetic Therapies, said. "We did not take this lightly. We sent the written comments to independent consultants and it was clear to everyone that the chances for approval were very slim without additional trials."

The FDA made it clear that there were no safety concerns but said the data was insufficient, according to Shire. Replagal is approved in 46 countries. And patients here in the U.S. have been taking the drug as part of a special treatment protocol, ahead of approval.

"We are the human data," Jerry Walter [3], president of the National Fabry Disease Association, said. "We had patients lined up ready to say this drug works."

It's a setback for a group of patients that has been to hell and back over the past three years. In June 2009, Genzyme Corp. discovered a virus in its Allston plant, causing a six-week shutdown that sent the only FDA-approved therapy, Fabrazyme, into a severe shortage. That shortage is just now lifting, with all U.S. patients receiving their full doses for March.

Some patients had to skip doses, or receive partial doses, while the shortage wore on. For some, this led to an increase in serious symptoms including kidney failure or cardiac problems. In the midst of the 2009 Genzyme shutdown, the FDA asked Shire if it would provide Replagal, which was approved overseas, to U.S. patients, ahead of U.S. approval. Shire did so, free of charge, costing the company millions. Now that group of 140 U.S. patients taking Replagal will lose access, as Shire plans to work with doctors to shift those patients to Genzyme's drug.

“We recognize that there is no alternative in the U.S. and we have a responsibility to those patients,” Rogerio Vivaldi [4], head of rare diseases for Genzyme, now owned by French drugmaker Sanofi (NYSE: SNY), said. “We are prioritizing those 140 patients and it may slow down our ramp up to other markets.” Vivaldi said the Framingham plant, which was approved in January by both the FDA, and European regulators, will be able to produce enough Fabrazyme for the 140 patients on Replagal, and an additional 200 to 300 newly diagnosed U.S. patients. Vivaldi said 50 new patients have begun treatment in the past four weeks.

If Genzyme/Sanofi were to add, on the low end, 340 Fabrazyme patients to its business, it could be worth more than $100 million per year. But Genzyme executives caution that some patients might pay discounted rates or even receive the medicine for free if it is not covered by insurance.

Vivaldi said over the next few years, the Framingham plant will be able to supply three times the current number of patients. But relying on a single manufacturer, Walter said, means patients still feel vulnerable.

“We look at it like this — either we have the drug today, or we don’t,” Walter said. “We don’t look too far ahead.”

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