

**From:** [Sarris, Christina \(NIH/OD\) \[E\]](#)  
**To:** [Hammersla, Ann \(NIH/OD\) \[E\]](#)  
**Subject:** RE: Fabry's - it is time to act  
**Date:** Friday, August 05, 2011 5:19:22 PM

---

Oh my goodness. I'll be in Monday. Of course, Shire does not need a patent license for the US.

---

**From:** Hammersla, Ann (NIH/OD) [E]  
**Sent:** Friday, August 05, 2011 3:58 PM  
**To:** Sarris, Christina (NIH/OD) [E]  
**Subject:** FW: Fabry's - it is time to act

---

**From:** Rohrbaugh, Mark (NIH/OD) [E]  
**Sent:** Friday, August 05, 2011 3:53 PM  
**To:** Hammersla, Ann (NIH/OD) [E]  
**Subject:** FW: Fabry's - it is time to act

---

**From:** McGarey, Barbara (NIH/OD) [E]  
**Sent:** Friday, August 05, 2011 2:36 PM  
**To:** Berkley, Dale (NIH/OD) [E]; Rohrbaugh, Mark (NIH/OD) [E]  
**Subject:** FW: Fabry's - it is time to act

Fyi.

Barbara M. McGarey, J.D.  
Deputy Associate General Counsel for Public Health, NIH  
Office of the General Counsel, PHD, NIH Branch  
31 Center Drive, Rm 2B-50  
Bethesda, MD 20892-2111  
(301) 496-6043 (phone)  
(301) 402-1034 (fax)  
[mcgareyb@od.nih.gov](mailto:mcgareyb@od.nih.gov)

*This message is intended for the exclusive use of the recipient(s) named above. It may contain information that is PROTECTED or PRIVILEGED, and it should not be disseminated, distributed, or copied to persons not authorized to receive such information.*

---

**From:** Hudson, Kathy (NIH/OD) [E]  
**Sent:** Friday, August 05, 2011 2:31 PM  
**To:** Collins, Francis (NIH/OD) [E]  
**Cc:** Tabak, Lawrence (NIH/OD) [E]; McGarey, Barbara (NIH/OD) [E]; Devaney, Stephanie (NIH/OD) [E]; Hudson, Kathy (NIH/OD) [E]  
**Subject:** Fabry's - it is time to act

Hi,

Last week we spoke to FDA who said that "in theory" they could use enforcement discretion to permit Shire to market replagal in the US.

Yesterday Genzyme reported a new problem with fabryzyme supply that some are reporting as NO enzyme for month of august (gulp...) <http://www.pharmalot.com/2011/08/genzyme-patients-angry-over-fabrazyme-supplies/> and pasted below

I talked to Shire today about whether, in a hypothetical scenario, they would be able to supply replagal to cover drug shortage in the US. I was told that they would be able to provide HRT to hundreds of patients immediately growing to full supply to end shortage in 4 months though she was doing back of envelope and would need to get better data if in fact we had a magic wand to wave.

I would like to discuss this with you but I would propose that we:

1. Ask FDA to give replagal temporary okay to sell in US. We might ask KGS to back us....
2. Set up call with mt sinai, genzyme, and sanofi and say that we are prepared to march in unless they give Shire a license for US and Europe at reasonable costs and that the deal be done immediately.

Patients will only be helped in the short term if (1) happens.

Patients will only be helped in the long term if both 1 and 2 happen.

I think we should act right away... This is heart breaking.

---

**From:** Flynn, Kerry [mailto:kflynn@shire.com]  
**Sent:** Friday, August 05, 2011 1:06 PM  
**To:** Hudson, Kathy (NIH/OD) [E]  
**Cc:** Gregoire, Sylvie  
**Subject:**

• [Pharma Blog](#) » [2011](#) » [August](#) » [04](#)

## [Genzyme Angers Patients Over Fabrazyme Supplies](#)

[Make a comment](#)

By Ed Silverman // [August 4th, 2011](#) // 11:34 am



What consent decree? Just one week after sending [a July 26 letter](#) to patients that limited allocations of the Fabrazyme medication would remain available this month, Genzyme is now telling patients that unspecified quality control problems have disrupted supplies. Although the disruption is supposed to be brief, Genzyme was unable to say when

Fabrazyme would be shipped, according to patients.

“I got a call from my nurse case manager (at Genzyme) to say there’s no drug available this month,” Tom Olshewski of Grayling, Michigan, tells us. “I was told there’ll be no medicine until further notice.” And Michael Masula of Kilbuck, Pennsylvania says that “it was due to a quality control issue and they anticipate it being short lived...but they always say it’s going to be short lived” (read about Masula [here](#)). The latest snafu is also discussed on patient chat boards, [such as this one](#).

A Genzyme spokeswoman confirmed that shipments have been disrupted. “We have told some of the US patient community that there is a delay in our August shipments, because there is a delay in the release of some of the product. Since we are operating without inventory, this delay will disrupt our shipping schedule, which is why we began calling affected patients. We’ll know more in the upcoming days and will continue to update the community,” she wrote us. No further explanation was given.

This is the latest setback for the beleaguered biotech, which was recently purchased by Sanofi, and Fabry’s disease patients, who have suffered supply disruptions and shortages for more than two years as Genzyme struggled with manufacturing problems at its Allston Landing, Massachusetts facility. These led to a [consent decree](#), a \$175 million fine and a remediation plan that has missed some goals. The med is the only treatment approved by the FDA for the rare, but life-threatening genetic disease.

Last March, for instance, Genzyme disclosed that a lot of Fabrazyme was rejected for failing to meet quality standards, further limiting supplies to patients who are already suffering from rationing. At the time, Genzyme insisted normal supplies would resume in the second half of 2011, after a new facility in Framingham, Ma., is approved by the FDA. Until then, Genzyme warned that inventory will remain low and availability of existing supplies will vary from region to region.

Investors, however, may not be entirely surprised by the latest disruption. In a [July 28 statement](#), Sanofi boasted that Genzyme “continues to make progress at its Allston Landing manufacturing plant and the company is on track with requirements of the consent decree....Nevertheless, based upon actual production trends to date and lead times to release products for the market, Sanofi does not expect that the 2011 contingent value right production milestone will be met.” The CVR payment was contingent upon producing a certain amount of Fabrazyme.

The conflicting information - the July 28 letter suggests a production problem, but is not specific; the July 26 letter to patients indicates that some amount of Fabrazyme will be available and the recent phone calls that contradict the July 26 letter - has further angered patients, many of whom say they worry about declining health, since their doses have been rationed for a protracted period of time.

Many patients also charge that Genzyme has employed a double standard when it comes to providing its Fabrazyme med to US patients by offering full dosages to patients in Europe, which Genzyme denied ([back story](#)). And there is anger over reports that 62 percent of Fabrazyme made in the US is allocated to overseas patients, even though those patients have access to an alternative, Replagal, which is made by Shire Pharmaceuticals.

In response, a half dozen people have filed a lawsuit against Genzyme and Mt. Sinai Medical School for the way ongoing shortages of the Fabrazyme treatment is being handled. The lawsuit named Mt. Sinai because the medical center licensed Fabrazyme to Genzyme and purportedly went along with the rationing plan ([read this](#)). Some of the same patients petitioned the FDA to insist that overseas stock of the med is first made available to US citizens (back story). They had previously tried unsuccessfully to convince the NIH to override the Fabrazyme patent in a bid to find another means of production ([look here](#)). “I thought it was a wonder drug,” Olshewski tells us. “I was 100 percent for this company. I

would do speaking engagements for them. But now, I wouldn't give them two cents. I think they should be held on criminal charges for what they're doing to people...our tax dollars paid for the research and development of this drug, and they turn around and ship it to another country...it's nothing but greed..Every time you turn around, there's a different excuse from them. Two and a half years we've been going through this."

Kerry A. Flynn, CLP  
Vice President, Intellectual Property  
**Shire**  
**300 Shire Way**  
**Lexington, Massachusetts 02421**

(781) 482-0795

---

At Shire we believe caregivers are some of the bravest people we know, so we created the Shire BRAVE Awards to help honor them. If you know a caregiver, nominate them today through the Shire BRAVE Awards website!

\*\*\*\*\*

Shire plc, the ultimate parent of the Shire Group of companies, is registered in Jersey No. 99854

Registered Office: 22 Grenville Street, St Helier, Jersey JE4 8PX

\*\*\*\*\*

Please consider the environment before printing this e-mail

This email and any files transmitted with it are confidential and may be legally privileged and are intended solely for the use of the individual or entity to whom they are addressed. If you are not the intended recipient please note that any disclosure, distribution, or copying of this email is strictly prohibited and may be unlawful. If received in error, please delete this email and any attachments and confirm this to the sender.

[www.shire.com](http://www.shire.com)