



# The Fabrazyme Dilution Case

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# Overview

- **1,500** U.S. Fabry Disease Patients
- **Genetic illness** that results in the body being unable to break down fats. Patients are unable to make a specific enzyme termed agalsidase beta.
- Fabry disease is **lethal without treatment**. Most patients die of renal failure, heart attack and stroke by the time they reach their 50s. The symptoms of the disease are severe pain, loss of hearing, loss of eyesight, grave intestinal disorders, and degenerative nerve loss.
- From **taxpayer funding** under the Bayh-Dole act, Mt. Sinai hospital patented **Fabrazyme**<sup>®</sup>, which replaces the defective enzyme in patients. Mt. Sinai licensed Fabrazyme<sup>®</sup> to Genzyme Corporation.
- \$250,000 per year for a patient administered IV every 2 weeks for the rest of their life.

# Fabrazyme Shortage

- **Prior to June 2009 Genzyme contaminated** the drug supply with a rodent virus which decreased output.
- Genzyme also contaminated injectable vials with glass, rubber, and steel particles. The FDA fined Genzyme **\$175 million** and implemented oversight over Genzyme for 7 years under a consent decree.
- Genzyme made **\$400 million** per year before the shortage.

# Genzyme Rationing Plan

- 1) Reduced doses by 70% or more in US only
- 2) Banned new U.S. patients from access.
- 3) Shortage continued for almost 3 years.
- 4) FDA approval based on 1mg/kg every 2 weeks. No U.S. patient was allowed to receive FDA approved doses.
- 5) Lower doses never tested or FDA approved.

**Joseph Carik:** Allen, I am dying without this drug. They ran out. Is there anything you can do. You teach Intellectual Property Law at the law school

**Allen:** Maybe, it was developed at Mt. Sinai with Federal Money so you have right to access. We could ask for March-In so other companies could make it.

**Allen:** Jamie, would you help me guide me in this process?

**Jamie:** Yes. (Thank you **Jamie, Manon and KEI** for all of your help!)

# Role of FDA and NIH?

- Petitioned NIH (Nation Institutes of Health) for “March In” to allow other manufacturers to license patent.
- Bayh-Dole Act bans non-use or misuse of a patent funded the U.S. taxpayers. Compulsory license system for non-use.
- Denied because NIH argued it would take too long to get another company up and running. Everything will be fine.
- Genzyme told NIH it would provide a full supply by early 2011.

# Is a little better than nothing?

## Posology (Science of Dosage)

-----Only treating doctors may lawfully determine posology.

-----Genzyme advised European doctors to give full doses---

-----Genzyme advised American doctors to give 1/3 doses---

EMA banned low-dose of Fabrazyme as **dangerous and ineffective**. Most EU patients had a **return of symptoms**, progression of disease, accelerated disease, including heart attack, stroke, and renal failure.

- Genzyme had returned to **full dose** in Europe and 80 more **new** patients were added as of December 2010, while more Americans were wait-listed and rationed.
- I re-petitioned the FDA and NIH for full doses and March-In.
- FDA and NIH was still “considering” the petitions almost two years later. “March-in” is a dead letter– will not protect public.

# Why Did Genzyme Ration U.S. but not Europe? Dilution is illegal everywhere

- Market Share Loss in Europe to Competitor
- Shire Pharmaceutical manufactured Replagal (essentially the same enzyme) but the FDA has not approved it in U.S.
- Genzyme was losing global market share to Replagal but not in US
- No alternative in US market. No threat market share loss.
- Mt. Sinai sued Shire in Germany for patent infringement during the shortage (undisclosed settlement).
- NIH monitored settlement under March-In “considerations.” (Newly self-created regulatory power not in statute)



# Current Status

- FDA declared shortage “over” in March 2012,
- Some of my clients did not get doses until July
- Shire refuses to market in US due to FDA animosity to Replagal.
- U.S. Fabry patients must purchase Fabrazyme for the rest of their lives even after Genzyme caused them personal injury and killed family and friends. (Fabry is hereditary)
- All patients have reported increased severe symptoms on low dose– kidney disease, heart transplant, kidney transplant, multiple strokes, hearing loss, inability to work, loss of nervous function in legs, degenerative vascular disease, etc.

# Who chooses survivors—those who get access during a shortage

- M.D.s at FDA? **No**
- Public Health Service Corp. at NIH? **No**
- Trained Public Health Authorities? **No**
- Patient's Doctors? **No**
- Patients? **No**
- Pharma M.B.A.s/CEOs untrained in medicine? **YES**

# U.S. Doctors Opposed Non-FDA Approved Low Dosing

- Childrens' Center For Cancer and Blood Diseases of Las Vegas

7-14-2009

Dear Patient,

I am writing to you in response to the recent event resulting in shortage of your IV medications Fabrazyme or Cerezyme. It is my firm understanding that treatment with enzyme replacement medications are only beneficial at doses studied. Any less than recommended, **the dosing results in an inadequate therapeutic treatment.**

I will not be changing your prescription, decreasing your dosing or asking that you skip any doses of enzyme replacement treatments. If you have any questions, please call me at my office.

Thank you,

Dr Jonathan Bernstein

# Who will help Drug Shortage Patients

- Call FDA? **No access to drug.** Call NIH? **No access.**
- Call Public Health Authorities? **No access.**
- Call your doctor? **No access.**
- Call inventors or scientists? **No access.**
- Call pharma or pharma-funded patient groups?  
**Everything will be fine—the shortage is almost over.**
- **Call a lawyer/?** The worst solution for dying people—lawyers only have the power to petition but better than nothing.

# A New U.S. Healthcare Paradigm

If a patient needs long-term drug access, the patient (or child's parents) must stockpile needed medication to survive the inevitable shortages.

Stockpiling medication is a federal crime, but has become a crime of necessity for survival in the United States.

If a patient does not have a stockpile, then the only chance for survival is to go to court. March-in or breach of individual fiduciary to patient (failure to supply under contract)

# Hochendoner et al. v. Genzyme and Mt. Sinai filed March 9, 2011

Contingency fee Class Action— named 14 patients in WD Pa. Transferred and pending over one year in Boston (D. Mass). 70 more.

- At least three deaths attributed to shortage.
- One wrongful death case already brought in Utah (copied our complaint).
- Genzyme defense is that doctors/hospitals are responsible because doctors gave the doses and should pay the damages.

# Carik v. FDA, NIH, HHS filed Feb 17, 2012 (D.D.C.)

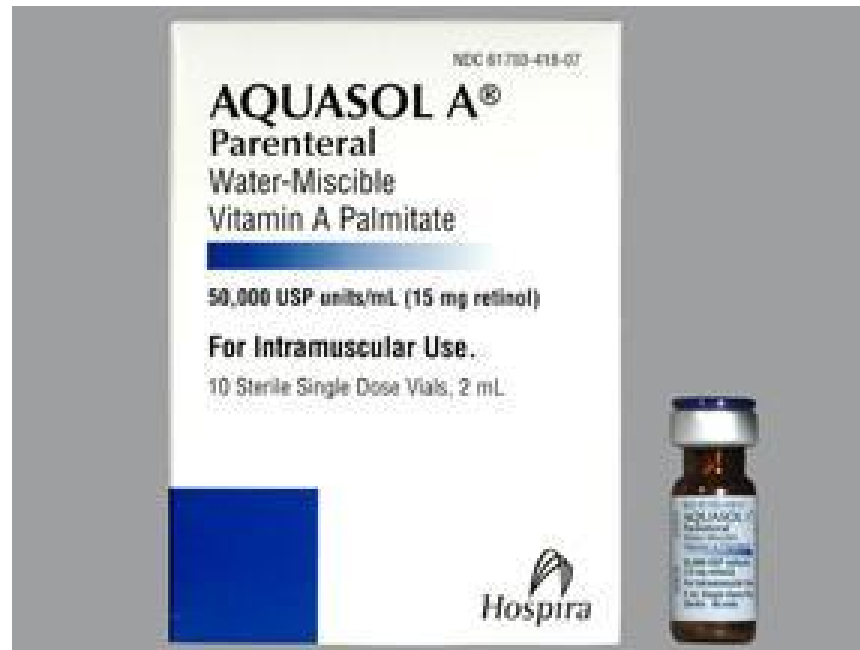
## Pro bono civil rights case– Allegations

- HHS knew of severe injuries, gross FDA violations and criminality regarding U.S.-targeted drug dilution scheme. FDA oversaw production of Fabrazyme under its consent decree and oversaw distribution of diluted and adulterated drug.
- Mt. Sinai patent is invalid and unenforceable.
- Government violation of Due Process, right to self determination in medical matters, right to safe and effective drugs under law, right to second source of manufacture (no monopolies granted for necessities of life).

# The World Ran Out of Vitamin A

- Hospira is a monopoly. Sole FDA and EMA licensed producer of injectable vitamin A for world.
- It decided to change manufacturing sites in 2010.
- Shut down production, stockpiled a couple months supply, but never opened the second plant.
- No human being has been treated--short-bowel, malnourished children, total parenteral nutrition patients (i.v., feeding) since 2010.
- Small reserve supply still may exist at Hospira, but not released.





# The Aquasol (vitamin) A shortage

Co-counsel

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# Overview

- **140–250 million children affected by vitamin A deficiency. These children suffer a dramatically increased risk of death, blindness and illness. Leading cause of preventable blindness in children.**



# Lacognata v. Hospira (MD Fla.)

## Dismissed with prejudice

- US District Court Judge James Moody Jr. shot down all of her arguments. For instance, he wrote that “there is no authority that supports (her) argument that a drug manufacturer, like Hospira, has a duty to continue supplying a patient with a drug that it knows the patient relies upon for his or her medical health.” And he disagreed that Hospira was negligent for failing to stockpile adequate reserves and because its representatives assured Lacognata the shortage would end, but in fact, later did not.
- Ed Silverman, Pharmalot, Woman Loses Suit Against Hospira Shortage, July 5, 2012

# What's a Lawyer/Scientist to do?

- Injectable vitamin A is simple chemistry.
- Vitamin A is an oil. Mixed with emulsifier to make it more soluble in blood.
- Injected Intramuscularly or Intradermally.
- Manufactured continuously since 1946 until 2010.
- Fabrazyme was a complex biologic.



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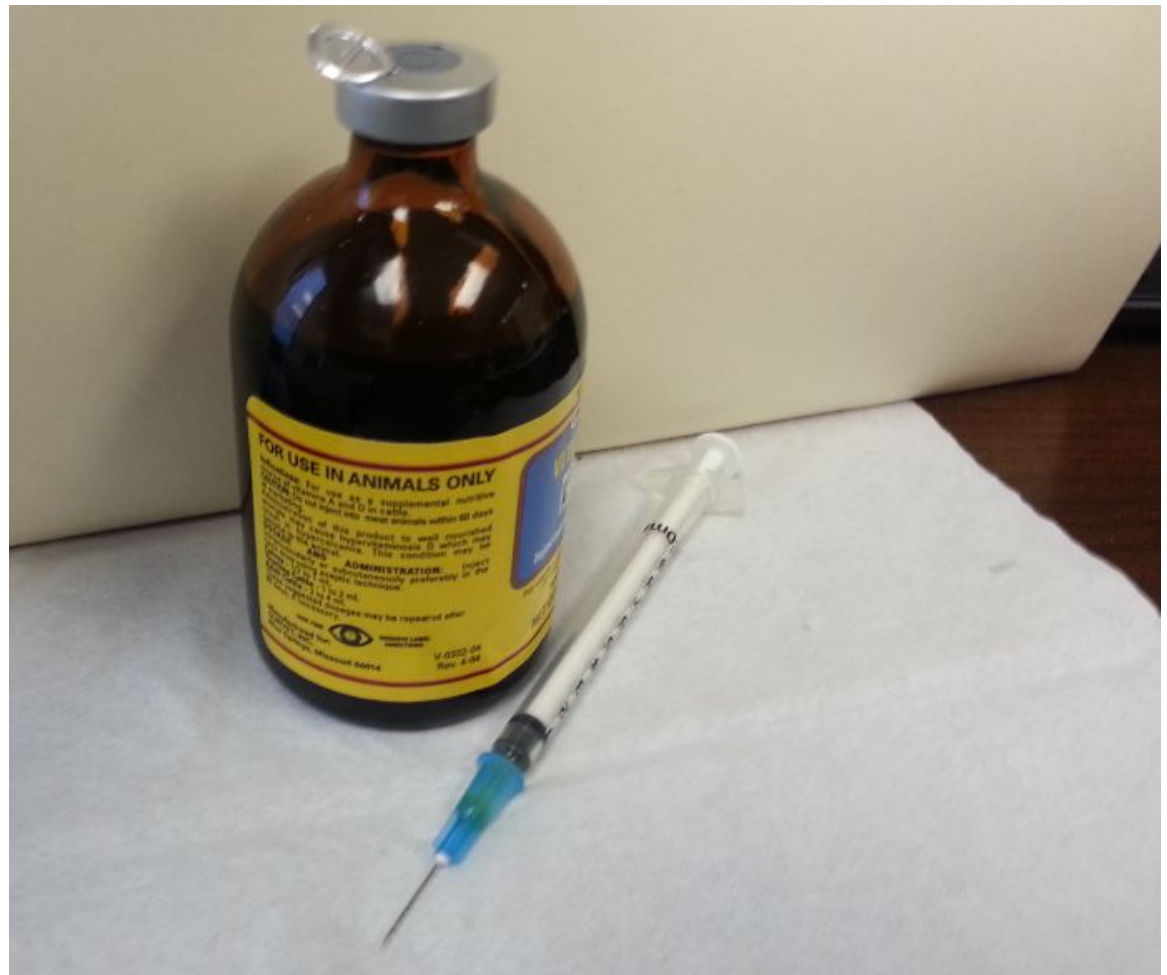
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Do not do this. It is unsafe and could cause injury or death.



Experiment performed  
Oct. 15, 2012

Intradermal self-injection  
Veterinary vitamin A/D  
100 $\mu$ l (50,000 IU A)



Do not do this. It is unsafe and could cause injury or death.



Do not do this. It is unsafe and could cause injury or death.





Do not do this. It is unsafe and could cause injury or death.



# Results Disappointing

- Cattle vitamin A does not kill lawyers.
- But, we have another source of vitamin that can be tested and, if appropriate, purified to be made safe for humans.
- Will petition FDA to assist patients (hopefully won't wait years to reply)

# We've Been Here Before

“It’s imperative that we confront a serious threat to the health of our nation. And that threat is complacency – a false sense of security, a false sense of calm that hides ... a serious epidemic.

**Dr. Kevin Fenton**

Director, CDC’s National Center for HIV/AIDS, Viral Hepatitis,  
STD, and TB Prevention

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- Postdoctoral Fellow (HIV transmission/vaccines) 1998
- Assistant Professor Obstetrics and Gynecology 1999
- Law Degree/ Registered Patent Attorney 2003
- Adjunct Professor, Biotechnology Law, 2006-present
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