August 27, 2010

Secretary Kathleen Sebelius
U.S. Department of Health and Human Services
200 Independence Avenue, S.W.
Washington, D.C. 20201

Re: Request for an Expedited Hearing and Expanded Scope of Issues, Regarding Petition to Use Authority Under Bayh-Dole to End Rationing of Fabrazyme®

Dear Secretary Sebelius,

Request for Expedited Hearing, and Expanded Scope of Issues

We are writing to express our support for the August 2, 2010 petition of Fabry disease patients Joseph M. Carik, Anita Hochendoner, and Anita Bova, regarding their request for a march-in of rights to patented inventions used to manufacture agalsidase beta, a product now sold exclusively by Genzyme under the trade name Fabrazyme, and to ask that this petition be considered in an **expedited hearing**, due to the gravity of the supply problem, and the harm rationing has done to patients suffering from this disease. We also request that the scope of the hearing be expanded to cover a field-of-use license for all NIH funded inventions, for the manufacturing of generic versions of Fabrazyme, either through the march-in remedy to an abuse of the patent right, or through the U.S. Government's royalty free right in the patent. We further request the DHHS to consider the high cost of Fabrazyme, and the potential benefits of competition on the price to end users and third parties who pay for Fabrazyme, as additional reasons to grant the march in request or to utilize the government's royalty free license rights.

Background

Fabrazyme is a treatment for Fabry's disease, a rare genetic condition. Left untreated, patients with Fabry's disease have a reported life expectancy that is 28 years less than persons without the disease, and suffer from a number of life threatening and debilitating aliments. Fabrazyme was approved for marketing in 2003, and is now manufactured exclusively by Genzyme. The market exclusively was protected for seven years by the Orphan Drug Act[1], as well as through patent protection, including in particular US Patent Number 5356804, which due to an extension of the patent term of 1,440 days, will expire on September 27, 2015.

In 2009, Genzyme began to experience a series of manufacturing problems for Fabrazyme and Cerezyme, treatments for Fabry's disease and Gaucher disease, respectively.[2] As a result, for some time, Fabry's disease patients have been subject to rationing, receiving one third the normal dose of the medicine.[3] In addition, newly

[1] The Orphan Drug exclusivity expired in 2010.
[2] April 21, 2010, Andrew Pollack, "Genzyme Expects a Fine of $175 Million," *New York Times.*
[3] April 15, 2010, Andrew Pollack, "Genzyme Drug Shortage Leaves Users Feeling Betrayed," *New York Times.*

diagnosed Fabry patients are presently ineligible to receive the therapy at all until the rationing is ended. Due to this rationing, patients with the disease are being forced to suffer worsened symptoms, enhanced complications, and an increased risk of premature death.

Fabrazyme is one of the most expensive medicines now on the market. A normal dose for Fabrazyme is 1 mg per kilo of body weight, administered every 14 days. Based upon on prices from a recent reimbursement (see Attachment), the **daily** cost of Fabrazyme is $9.87 **per kilo** of body weight[4]. A person of 70 kilos would pay $693 per day, or $252,878.78 per year for normal dose of Fabrazyme.

Genzyme has profited greatly from its monopoly on Fabrazyme. Before its supply problems, Genzyme was earning about $ .5 billion per year on sales of Fabrazyme. For its entire product line of similar treatments, Genzyme reports an operating profit margin of 77 percent.[5]

As discussed in the Appendix, the development of Fabrazyme (agalsidase beta) benefited greatly from NIH funded research. As a consequence, key patents on Fabrazyme are held by Mount Sinai School of Medicine of NYU, and possibly other patent owners, and are subject to either a royalty free license to be used by or for the U.S. Government[6], or a march-in request, under 35 U.S.C. 203.

The legal monopoly that currently permits Genzyme to block competitive supplies of this treatment should now come to an end. The NIH can do this by granting the requested open license in the march-in request, or by exercising its own royalty free right to have the patent used.

Greater competition in the supply of agalsidase beta will ensure that Fabry patients can benefit from the public funds invested in the treatment of their disease and will send a message that the NIH will not tolerate abuses of patent rights for government-funded inventions.

We respectfully request that the petition of Mr. Carik, Ms. Hochendoner, and Ms. Bova

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[4] $4,849.73 for a 35 mg vial. Information available here: http://keionline.org/sites/default/files/price_35mg_Fabrazym_redacted.png

[5] As reported in: The Cost of Enzyme Replacement Therapy, http://www.genzyme.com/commitment/patients/costof_treatment.asp , visited August 26, 2010, which states" "What is the profit margin on these products? Like most companies, we don't report the profitability of individual products. However, our overall corporate profit margin is public. Currently, it is approximately 77 percent of our annual revenue."

[6] 35 USC 203(c)(4) "With respect to any invention in which the contractor elects rights, the Federal agency shall have a nonexclusive, nontransferable, irrevocable, paid-up license to practice or have practiced for or on behalf of the United States any subject invention throughout the world: Provided, That the funding agreement may provide for such additional rights, including the right to assign or have assigned foreign patent rights in the subject invention, as are determined by the agency as necessary for meeting the obligations of the United States under any treaty, international agreement, arrangement of cooperation, memorandum of understanding, or similar arrangement, including military agreements relating to weapons development and production".

be given an expedited hearing, and that the issues be broadened to consider other patents necessary to manufacture treatments for Fabry's disease, the use of the government's royalty free license to NIH funded inventions, and the benefits of competition in terms of possibility lower prices for the treatment.

Sincerely,

John Brockman, National President
American Medical Student Association (AMSA)
1902 Association Drive, Reston, Virginia 20191

James Love, Director
Knowledge Ecology International (KEI)
1621 Connecticut Ave. NW, Suite 500, Washington, D.C. 20009

Rob Weissman, President
Public Citizen
1600 20th Street NW, Washington, D.C. 20009

Ethan Guillen, Executive Director
Universities Allied for Essential Medicines (UAEM)
2625 Alcatraz Avenue, #180, Berkeley, CA 94705

Larry C. McNeely II, Health Care Advocate
U.S. PIRG (Public Interest Research Group)
218 D Street SE, Washington, D.C. 20003

Appendix

The NIH RePORT database identifies 372 grants with the search term Fabry, including 25 NIH grants to Robert J. Desnick of the Mount Sinai School of Medicine of NYU. The Mount Sinai Fabry grants are associated with the following patents:

Patent Number	Title of Patent	NIH Grant
6455037	Cells expressing an .alpha.gala nucleic acid and methods of xenotransplantation	R01DK034045 R37DK034045
5491075	Cloning and expression of biologically active .alpha.-N-acetylgalactosaminidase	R37DK034045 R01DK034045
5382524	Cloning and expression of biologically active .alpha.-n-acetylgalactosaminidase	R37DK034045 R01DK034045
5401650	Cloning and expression of biologically active .alpha.-galactosidase A	R01DK034045 R37DK034045
5580757	Cloning and expression of biologically active .alpha.-galactosidase A as a fusion protein	R37DK034045 R01DK034045
5356804	Cloning and expression of biologically active human .alpha.-galactosidase A	R37DK034045 R01DK034045

See additionally: Public and Private Sector Funded Research for Fabry's Disease, KEI Research Note 2010:2. Available here: http://www.keionline.org/fabrazyme

Attachment

Claim Selected

Member Name: [redacted] **Date of Birth:** [redacted]

Status: Completed **Type:** Medical **Date(s) of Service:** 07/15/2010 - 07/15/2010

Questions about this claim? Send a Message

Total Charges Submitted: $4,849.73 **You Pay Out of Pocket:** $0.00 **Total Paid by Plan:** $4,849.73

Submitted Charge Part 1: $4,849.73 - Completed

Note: for one 35 milligram vial

Submitted Charge - Part 1

Date of Services: 07/15/2010 - 07/15/2010 **Health Care Professional:** Aetna Specialty Pharmacy, LLC

Status : Completed

Payment Made to: Provider **EFT Number:** [redacted] **Claim Paid on:** 07/27/2010

Date of Service/Service Provided	Charges Submitted	Charges at Aetna's Agreed Pricing	Paid from Your Fund	Paid By Plan	Not Paid/Excluded by Plan	Applied to Your Deductible	Applied to Your Coinsurance	Rmks
07/15/2010 Oral-Injectable Medication	$4,849.73	$4,849.73	$0.00	$4,849.73	$0.00	$0.00	$0.00	
Total	$4,849.73	$4,849.73	$0.00	$4,849.73	$0.00	$0.00	$0.00	
			Plan Pays: $4,849.73		**Your Responsibility:** $0.00			