DEPARTMENT OF HEALTH & HUMAN SERVICES

Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993-0002

MAR 0 7 2014

Via UPS Delivery

Gary J. Shemaka
Associate Director, Regulatory Affairs
Genzyme Corporation
200 Crossing Blvd, 3rd Floor
Framingham, MA 01702

Re: Submission of Clinical Trial Information Pursuant to 42 U.S.C. § 282(j)
Reference Number: FDA-2014-104 (NCT00601107)

Dear Mr. Shemaka:

In reviewing certain records contained in the ClinicalTrials.gov data bank, operated by the National Institutes of Health/National Library of Medicine (NIH/NLM), and records of the Food and Drug Administration (FDA), FDA has identified potential non-compliance with certain clinical trial information related to NCT00601107. Clinical trial registration and results submission requirements are described in section 801 of the Food and Drug Administration Amendments Act of 2007. Based on an initial review, your company appears to be the responsible party for NCT00601107 and it appears that the records for the referenced clinical trial may be missing clinical trial results information required to be submitted under section 402(j)(3) of the Public Health Service Act (PHS Act) (42 U.S.C. § 282(j)(3)). Your company should review this record and determine whether your company submitted all required information. If you determine that corrections, updates, or additional information should be submitted, please submit them promptly.

Our preliminary review of records suggests that there may be (i) clinical trial results which should have been submitted in February 2010 to ClinicalTrials.gov; and (ii) a Form FDA 3674 missing from FDA records for NDA 20862 referencing NCT00601107.

We note that your company submitted an untimely certification on April 28, 2010 (14 months after the primary completion date of February 2009), stating it was seeking approval for a new use of the drug studied in NCT00601107. Under section 402(j)(3)(E)(v)(I)-(III) of the PHS Act (42 U.S.C. § 282(j)(3)(E)(v)(I)-(III)), even if a timely certification had been submitted, based upon our preliminary review of available information, results would have been due not later than February 2012.

FDA intends to conduct a further review and assessment of the above-referenced clinical trial(s) beginning 30 days after you receive this letter. If a further review and assessment of the record

related to NCT00601107 results in a determination, at that time, that your company is not in compliance with all applicable requirements of section 402(j) of the PHS Act (42 U.S.C. § 282(j)), your company may receive from FDA a notice under section 402(j)(5)(C)(ii) of the PHS Act (42 U.S.C. § 282(j)(5)(C)(ii)). After FDA sends such a notice to a responsible party, FDA could initiate an administrative action seeking a civil monetary penalty against the responsible party. Failure to submit clinical trial information required under section 402(j) of the PHS Act (42 U.S.C. § 282(j)) is a prohibited act under section 301(jj)(2) of the Federal Food, Drug, and Cosmetic Act (FD&C Act) (21 U.S.C. § 331(jj)(2)). Pursuant to section 303(f)(3)(A) of the FD&C Act (21 U.S.C. § 333(f)(3)(A)), "[a]ny person who violates section 301(jj) shall be subject to a civil monetary penalty of not more than $10,000 for all violations adjudicated in a single proceeding."

Moreover, section 303(f)(3)(B) of the FD&C Act (21 U.S.C. § 333(f)(3)(B)) provides that "[i]f a violation of section 301(jj) is not corrected within the 30-day period following receipt of a [notice issued] under section 402(j)(5)(C)(ii) [of the PHS Act (42 U.S.C. § 282(j)(5)(C)(ii))], the person shall, in addition to any penalty under subparagraph (A), be subject to a civil monetary penalty of not more than $10,000 for each day of the violation after such period until the violation is corrected."

In addition to civil monetary penalties, violations of section 301(jj) of the FD&C Act (21 U.S.C. § 331(jj)) also could result in other regulatory action without further notice, such as injunction and/or criminal prosecution.

As requested, please review your company's NCT00601107 clinical trial record at http://www.clinicaltrials.gov and make any necessary submissions or corrections of clinical trial information as required under section 402(j)(3) of the PHS Act (42 U.S.C. § 282(j)(3)). If you have any questions about this letter, please call Patrick J. McNeilly, Ph.D., at (301) 796-2941 or email patrick.mcneilly@fda.hhs.gov. Please have the reference number provided above available when you call and include it with any email communications.

Sincerely,

Douglas Stearn
Director
Office of Enforcement and Import Operations
Office of Regulatory Affairs