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A Prospective, Cross-sectional Survey Study of the Natural History of Niemann-Pick Disease Type B

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KEYWORDS

Niemann-Pick disease, acid sphingomyelinase deficiency, lysosomal storage disorder, cross-sectional study, natural history

ABBREVIATIONS

ASM—acid sphingomyelinase
 NPD—Niemann-Pick disease
 IGF₁—insulin-like growth factor 1
 ECG—electrocardiography
 FVC—forced vital capacity
 FEV₁—forced expiratory volume in 1 second
 DL_{CO}—diffusing capacity of the lung
 6MWT—6 minute walk test
 HRCT—high-resolution computed tomography
 MN—multiples of normal
 CHQ—Child Health Questionnaire
 SF₃₆—Short Form-36
 HDL—high-density lipoprotein

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FINANCIAL DISCLOSURE: Drs McGovern, Wasserstein, Giugliani, Bombi, Vanier, Mengel, Brodie, Mendelson, Skloot, and Desnick have performed clinical trials for Genzyme; Dr Desnick has received travel expenses for scientific presentations from, is a consultant for, and has licensed the use of recombinant human acid sphingomyelinase for the treatment of patients with Niemann-Pick disease and for Fabryzyme for Fabry Disease to Genzyme; and Mr Kuriyama and Dr Cox are employees of and hold stock in Genzyme.

Letters

Bulldog, 10.5/13pt Letters to the Editor continue to be a vital part of the journal's interaction with its readers. As an increasing number of our subscribers now view the Web site on a regular basis (18 million hits each year) and in order to accelerate the publication of Letters, we are implementing a new policy regarding Letters to the Editor effective January 2009. All Letters to the Editor must be submitted online as eLetters using PEDIATRICS Web site. This new policy will make eLetters (Letters to the Editor) more relevant and timely, and are reviewed by the editors for online publication. eLetters is an online forum for ongoing peer review. To submit an eLetter please go to the article you wish to respond to and click on the link that reads "eLetters: Submit a Response." Submission of eLetters are open to all health care professionals and experts in related fields.

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