

potential for the Federal Government to pay for such technologies twice—once through support of the R&D process and again as a health insurer. The Federal Government has no mechanism to ensure that the process American pay for drugs and other technologies reflect the public's contribution to their development.

Perhaps the most significant lesson to emerge from the case of alglucerase is that cost considerations cannot be ignored in the development and diffusion of any treatment. Payments for alglucerase, like expenditures for any other treatment or diagnostic procedure, divert health care resources from other uses. Less expensive treatments, however, are unlikely to attract comparable scrutiny from insurers, and few other treatments will so predictably deplete insurance coverage. The high price of alglucerase threatens to put this promising treatment out of reach of many patients, even those who are well-insured.

REFERENCES

1. Abrahamov, A., Hadas-Halpern, L., Levy-Lahad, E., et al., "Enzyme Replacement Therapy for Children With Gaucher Disease: Low Dose, High Frequency, Lower Cost, High Efficacy," *Pediatric Research* 31:137A, 1992.
2. Aerts, J.M.F.G., Donker-Koopman, W.E., Murray, G.J., et al., "A Procedure for the Rapid Purification in High Yield of Human Glucocerebrosidase Using Immunoaffinity Chromatography With Monoclonal Antibodies," *Analytic Biochemistry* 154:655-663, 1986.
3. Aleski, W., Director of Reimbursement, Genzyme Corporation, Boston, MA, personal communications, Feb. 26, 1992, Apr. 1 and 9, 1992, and Sept. 8, 1992.
4. Alster, N., "Henri Termeer's Orphan Drug Strategy," *Forbes* 147(11):202-205, 1991.
5. Aubrey, W., Senior Vice President and Medical Director, Blue Shield of California, San Francisco, CA, personal communication, Mar. 5, 1992.
6. Baker, C., and Kramer, N., "Employer-Sponsored Prescription Drug Benefits," *Monthly Labor Review* 114:31-35, 1991.
7. Barranger, J., Department of Human Genetics, University of Pittsburgh, Pittsburgh, PA, personal communication, Sept. 23, 1991.
8. Barranger, J.A., and Ginns, E.I., "Glucosylceramide Lipidosis: Gaucher Disease," *The Metabolic Basis of Inherited Disease*, 6th Ed., C.R. Scriver, A.L. Beaudet, W.S. Sly, and D. Valle (eds.) (New York, NY: McGraw-Hill, 1989).
9. Barton, N., "Ceredase (Macrophage-Targeted Glucocerebrosidase) for the Treatment of Type 1 Gaucher's Disease," *Proceedings From the Endocrinologic and Metabolic Advisory Committee* 1:135-248, 1990.
10. Barton, N., Chief, Clinical Investigation Section, Developmental and Metabolic Branch, National Institute of Neurological Disorders and Stroke, National Institutes of Health, Bethesda, MD, personal communications, Sept. 19 and 26, 1991, Dec. 11, 1991, Jan. 14 and 21, 1992, Feb. 24, 1992, Mar. 16, 1992, and July 14, 1992.
11. Barton, N.W., Brady, R.O., Dambrosia, J.M., et al., "Replacement Therapy for Inherited Enzyme Deficiency—Macrophage Targeted Glucocerebrosidase for Gaucher's Disease," *New England Journal of Medicine* 324:1464-1470, 1991.
12. Barton, N.W., Brady, R.O., Dambrosia, J.M., et al., "Dose-Dependent Responses to Macrophage-Targeted Glucocerebrosidase in a Child With Gaucher Disease," *Journal of Pediatrics* 120:277-280, 1992.
13. Barton, N.W., Brady, R.O., Murray, G.J., et al., letter, *New England Journal of Medicine* 325:1811, 1991.
14. Barton, N.W., Furbish, F. S., Murray, G.J., et al., "Therapeutic Response to Intravenous Infusions of Glucocerebrosidase in a Patient With Gaucher Disease," *Proceedings of the National Academy of Sciences USA* 87:1913-1916, 1990.
15. Barton, N.W., Murray, G.J., and Brady, R.O., "Hematological Responses Are Dependent on the Amount of Glucocerebrosidase Administered to Patients With Gaucher's Disease," *Blood* 78:431-436, 1991.
16. Beisel, R., Director of the Division of Medicaid Statistics, Health Care Financing Administration, Baltimore, MD, personal communications, Mar. 30, 1992, and Apr. 1, 1992.
17. Belchzel, P.E., Crawley, J. C.W., Braichman, I.P., et al., G., "Treatment of Gaucher's Disease With Liposome-Entrapped Glucocerebrosidase: B Glucosidase," *Lancet* 11:116-117, 1977.
18. Beutler, E., "Gaucher Disease," *Blood Reviews* 2:59-70, 1988.
19. Beutler, E., "Monocyte and Macrophage Disorders: Lipid Storage Disorders," *Hematology*, 4th Ed., W.J. Williams, E. Beutler, A.J. Erslev, and M.A. Lichtman (eds.) (New York NY: McGraw-Hill, 1990).
20. Beutler, E., "Gaucher's Disease," *New England Journal of Medicine* 325:1354-1360, 1991.
21. Beutler, E., Chairman Department of Molecular and Experimental Medicine, Scripps Research Institute, La Jolla, CA, personal communications,

- Oct. 22, 1991, Jan. 27, 1992, Feb. 26, 1992, Mar. 24, 1992, and May 29, 1992.
22. Beutler, E., "Gaucher Disease: New Molecular Approaches to Diagnosis and Treatment," *Science* 256:794-799, 1992.
 23. Beutler, E., Dale, G.L., Guinto, E., and Kuhl, W., "Enzyme Replacement Therapy in Gaucher's Disease: preliminary Clinical Trial of a New Enzyme Preparation," *Proceedings of the National Academy of Sciences USA* 74:4620-4623, 1977.
 24. Beutler, E., Dale, G.L., and Kuhl, W., "Replacement Therapy in Gaucher Disease," *Enzyme Replacement Therapy in Genetic Diseases: 2*, J. Desnick (ed.) (New York, NY: Alan R. Liss, Inc., 1980).
 25. Beutler, E., Gelbart, T., Kuhl, W., Serge, J., et al., "Identification of the Second Common Jewish Gaucher Disease Mutation Makes Possible Population-Based Screening for the Heterozygous State," *Proceeding of the National Academy of Sciences USA* 88:10544-10547, 1991.
 26. Beutler, E., Gelbart, T., Kuhl, W., et al., "Mutations in Jewish Patients With Gaucher Disease," *Blood* 79:1662-1666, 1992.
 27. Beutler, E., Kay, A., Saven, A., et al., "Enzyme Replacement Therapy for Gaucher Disease," *Blood* 78:1183-1190, 1991.
 28. Beutler, E., Kay, A., Saven, A., et al., letter, *New Journal of Medicine* 325:1809-1810, 1991.
 29. Beutler, E., and Saven, A., "Misuse of Marrow Examination in the Diagnosis of Gaucher Disease," *Blood* 76:646-648, 1990.
 30. Blitz, M., Vice President of Medical Policy and Research, Blue Cross and Blue Shield of New York, New York, NY, personal communication, Mar. 30, 1992.
 31. Booth, C., Director of Office of Payment Policy, Health Care Financing Administration, Baltimore, MD, personal communications, Oct. 3, 1991 and June 17, 1992.
 32. Brady, R., Developmental and Metabolic Branch, National Institute of Neurological Disorders and Stroke, National Institutes of Health, Bethesda, MD, personal communication, Jan. 14, 1992.
 33. Brady, R.O., and Barranger, J.A., "Glucosylceramide Lipidosis: Gaucher's Disease," *The Metabolic Basis of Inherited Disease*, 5th Ed., J.B. Stanbury, J.B. Wyngaarden, D.S. Fredrickson, et al. (eds.) (New York, NY: McGraw-Hill, 1983).
 34. Brady, R. O., Barranger, J.A., Furbish, F. S., et al., "Prospects for Enzyme Replacement Therapy in Gaucher Disease," *Gaucher Disease: A Century of Delineation and Research*, R.J. Desnick, S. Gatt, and G.A. Grabowski (eds.) (New York, NY: Alan R. Liss, Inc., 1982).
 35. Brady, R. O., Barranger, J. A., Gal, A. E., et al., "Status of Enzyme Replacement Therapy for Gaucher Disease," *Enzyme Therapy in Genetic Diseases: 2*, R.J. Desnick (ed.) (New York, NY: Alan R. Liss, Inc., 1980).
 36. Brady, R.O., Dambrosia, J.M., Barton, N. W., "Effectiveness of Low-Dose Replacement Therapy in Gaucher's Disease," *Clinical Research* 40:144A, 1992.
 37. Brady, R. O., Pentchev, P. G., Gal, A. E., et al., "Replacement Therapy for Inherited Enzyme Deficiency. Use of Purified Glucocerebrosidase in Gaucher's Disease," *New England Journal of Medicine* 291:989-993, 1974.
 38. Chirnoff, A., Medica Health Plan of United Health Care, Minneapolis, MN, personal communication, Mar. 30, 1992.
 39. Choy, F.Y.M., "Purification of Human Placental Glucocerebrosidase Using a Two-Step High-Performance Hydrophobic and Gel Permeation Column Chromatography Method," *Analytic Biochemistry* 156:515-520, 1986.
 40. Cohen, K., Executive Director, National Gaucher's Foundation, Gaithersburg, MD, personal communication, Apr. 13, 1992.
 41. Cohen, L., Medical Director, Blue Cross and Blue Shield of Virginia, Richmond, VA, personal communication, Mar. 31, 1992.
 42. Comanor, W. S., "The Political Economy of the Pharmaceutical Industry," *Journal of Economic Literature* 24:1178-1217, 1986.
 43. *Consumer Reports*, "Beyond Medicare," *Consumer Reports* 54(6):375-391, June 1989.
 44. Dale, G. L., and Beutler, E., "Enzyme Replacement Therapy in Gaucher's Disease: A Rapid High-Yield Method for Purification of Glucocerebrosidase," *Proceedings of the National Academy of Sciences USA* 73:4672-4674, 1976.
 45. Dale, G. L., Beutler, E., Fournier, P., et al., "Large Scale Purification of Glucocerebrosidase From Human Placentas," *Enzyme Therapy in Genetic Diseases: 2*, R.J. Desnick (ed.) (New York, NY: Alan R. Liss, Inc., 1980).
 46. Dennally, J., Medical Director, Blue Cross and Blue Shield of Connecticut, North Haven, CT, personal communication, Mar. 26, 1992.
 47. DiMasi, J. A., Hansen, R.W., Grabowski, H. G., et al., "The Cost of Innovation in the Pharmaceutical Industry," *Journal of Health Economics* 10:107-142, 1991.
 48. Doebber, T.W., Wu, M. S., Bugianesi, R.L., et al., "Enhanced Microphage Uptake of Synthetically Glycosylated Human Placental Beta_Glucocerebrosidase," *Journal of Biological Chemistry* 257:2193-2199, 1982.

49. Ebert, K.M., Selgrath, J.P., DiTullio, P., et al., "Transgenic Production of a Variant of Human Tissue-type Plasminogen Activator in Goat Milk: Generation of Transgenic Goats and Analysis of Expression," *Biotechnology* 9:835-838, 1991.
50. Erikson, E., Groth, G. C., Mansson, J.E., et al., "Clinical and Biochemical Outcome of Marrow Transplantation for Gaucher Disease of the Norrbottnian Type," *Acta Paediatrica Scandinavica* 79:680-685, 1990.
51. Eyal, N., Wilder, S., and Horowitz, M., "Prevalent and Rare Mutations Among Gaucher Patients," *Gene* 96:277-283, 1990.
52. Fallet, S., Grace, M.E., Sibille, A., et al., "Enzyme Augmentation in Moderate to Life-Threatening Gaucher Disease," *Pediatric Research* 31:496-502, 1992.
53. Figueroa M.L., Sate, Y., Kay, A., et al., "Efficacy of Frequently Administered Macrophage-Targeted Glucocerebrosidase in the Treatment of Gaucher Disease," *Clinical Research* 40:167A, 1992.
54. Fink, J.K., Correll, P.H., Perry, L.K., et al., "Correction of Glucocerebrosidase Deficiency After Retroviral-Mediated Gene Transfer Into Hematopoietic Progenitor Cells From Patients With Gaucher Disease," *Proceedings of the National Academy of Sciences USA* 87:2334-2338, 1990.
55. Ford, M., *Medicaid: Reimbursement for Outpatient Prescription Drugs*, Congressional Research Service Report to Congress No. 91-235 EPW (Washington, DC: U.S. Government Printing Office, 1991).
56. Furbish, F. S., Blair, H.E., Shiloach, J., et al., "Enzyme Replacement Therapy in Gaucher's Disease: Large-Scale Purification of Glucocerebrosidase Suitable for Human Administration," *Proceedings of the National Academy of Sciences USA* 74:3560-3563, 1977.
57. Furbish, F. S., Steer, C.J., Krett, N.L., et al., "Uptake and Distribution of Placental Glucocerebrosidase in Rat Hepatic Cells and Effects of Sequential Deglycosylation," *Biochimica et Biophysica Acta* 673:425-34, 1981.
58. Gauthier, A.K., Associate Director, Alpha Center, Washington, DC, personal communication, Sept. 8, 1992.
59. Genzyme Corporation, Annual Report and Form 10-K, Filings With the U.S. Security Exchange Commission, Washington, DC, 1987 to 1990.
60. Gold, M., and Hodges, D., *HMO Industry Profile, Volume 1: Benefits, Premiums, and Market Structure in 1990* (Washington, DC: Group Health Association of America, 1991).
61. Grabowski, G., Professor, Department of Genetics and Molecular Biology, Mount Sinai Medical Center, New York, NY, personal communications, Sept. 17, 1991, Jan. 15 and 28, 1992, and June 1, 1992.
62. Grabowski, G.A., and Dagan, A., "Human Lysosomal B-Glucosidase: Purification by Affinity Chromatography," *Analytic Biochemistry* 141:267-279, 1984.
63. Grabowski, G.A., Gatt, S., and Horowitz, M., "Acid B-Glucosidase: Enzymology and Molecular Biology of Gaucher Disease," *Critical Reviews in Biochemistry and Molecular Biology* 25:385-414, 1990.
64. Gregoriadis, G., Neerunjun, D., Meade, T.W., et al., "Experiences After Long-Term Treatment of a Type 1 Gaucher Disease Patient With Liposome-Entrapped Glucocerebrosidase: B-Glucosidase," *Enzyme Therapy in Genetic Diseases: 2*, R.J. Desnick (ed.) (New York, NY: Alan R. Liss, Inc., 1980).
65. Gribble, T.J., and Latimer, K., "Successful Use of Enzyme Therapy in Gaucher's Disease" [abstract no. 556], *American Journal of Human Genetics* 49:113, 1991.
66. Groth, C.G., Collste, H., Dreborg, S., et al., "Attempt at Enzyme Replacement by Organ Transplantation; Renal Transplantation in Gaucher's Disease," *Transplantation Proceedings* 11:1218-1219, 1979.
67. Groth, C.G., Dreborg, S., Oeckerman, P.A., et al., "Splenic Transplantation in a Case of Gaucher's Disease," *Lancet* I:260-1264, 1971.
68. Hansen, R., "The Pharmaceutical Development Process: Estimates of Development Costs and Times and the Effect of Proposed Regulatory Changes," *Issues in Pharmaceutical Economics*, R.A. Chien (ed.) (Lexington, MA: D.C. Heath and co., 1979).
69. Hessen, J., Administrator of Pharmacy, Policy and Education, Aetna Insurance, personal communication, Mar. 31, 1992.
70. Hickman, W., Director of the Office of Medicaid Policy, Health Care Financing Administration, personal communication, Mar. 24, 1992.
71. Hobbs, J.R., Shaw, P.J., Hugh-Jones, K., et al., "Beneficial Effect of Pre-Transplant Splenectomy on Displacement Bone Marrow Transplantation for Gaucher's Syndrome," *Lancet* I:1111-1115, 1987.
72. Karson, E.M., "Principles of Gene Transfer and the Treatment of Disease," *Biotechnology* 16:189-212, 1991.
73. Keefe, J., Comptroller, Genzyme Corporation, Boston, MA, personal communications, Feb. 26, 1992 and Sept. 4, 1992.

74. Kirkpatrick, D. V., Barrios, N. J., Shapira, E., et al., "Treatment of Enzyme Storage Disease With Matched and Partially Matched Bone Marrow Transplantation: The Tulane Marrow Transplant Group Experience" [abstract No. 2184], *Blood* 76:548A, 1990.
75. Kleinschmidt, T., Christomanou, H., and Braunitzer, G., "Complete Amino-Acid Sequence and Carbohydrate Content of the Naturally Occurring Glucosylceramide Activator Protein (AI activator) Absent From a New Human Gaucher Disease Variant," *Biological Chemistry Hoppe-Seyler* 368:1571-1578, 1987.
76. Klipstein, E., Medical Director, Metropolitan Insurance Company, personal communication, Mar. 27, 1992.
77. Kohn, D. B., Nolta, J.A., and Weinthal, J., "Toward Gene Therapy for Gaucher Disease," *Human Gene Therapy* 2:101-105, 1991.
78. Krivit, W., "Lysosomal Storage Diseases," *Hematology Basic Principles and Practice*, R. Hoffman, E.J. Benz, S.J. Shattil, et al. (eds.) (New York, NY: Churchill Livingstone, 1991).
79. Krivit, W., "Bone Marrow Transplantation as Treatment for Storage Diseases: Background Review and Specific Application to Gaucher's Disease," *Japanese Journal of Inborn Errors of Metabolism*, in press.
80. Latham, T.E., Theophilus, B.D.M., Grabowski, G.A., and Smith, F.I., "Heterogeneity of Mutations in the Acid Beta-Glucosidase Gene of Gaucher Disease Patients," *DNA and Cell Biology* 10:15-21, 1991.
81. Ludman, M.D., Lipton, J., Sadhev, I., et al., "Gaucher Disease: Bone Marrow Transplantation in Rapidly Progressive Disease" [abstract no. 237], *American Journal of Human Genetics* 43:A60, 1988.
82. Maltz, D., Vice President of Medical Policy, Blue Cross and Blue Shield of Massachusetts, Boston, MA, personal communication, Mar. 25, 1992.
83. Matoth, Y., Chazan, S., Cnaan, A., et al., "Frequency of Carriers of Chronic (Type 1) Gaucher Disease in Ashkenazi Jews," *American Journal of Medical Genetics* 27:561-565, 1987.
84. McCollam, A., Medical Director, The Prudential Insurance Company, personal communication, Mar. 24, 1991.
85. McDonough, K., Medical Director, Mutual of Omaha, Inc., personal communication, Mar. 24, 1992.
86. McLachlan, D., Senior Vice President and Chief Financial Officer, Genzyme Corporation, Boston, MA, personal communications, Feb. 26, 1992, and Sept. 4, 1992.
87. Molyneaux, G., Assistant Medical Director, Blue Cross and Blue Shield of California, San Francisco, CA, personal communication, Mar. 26, 1992.
88. Murray, G. J., "Lectin-Specific Targeting of Lysosomal Enzymes to Reticuloendothelial Cells," *Methods in Enzymology* 149:25-42, 1987.
89. Murray, G.J., Howard, K.D., Richards, S. M., et al., "Gaucher's Disease: Lack of Antibody Response in 12 Patients Following Repeated Intravenous Infusions of Mannose Terminal Glucocerebrosidase," *Journal of Immunological Methods* 137:113-120, 1991.
90. Murray, G.J., Youle, R. J., Gandy, S. E., et al., "Purification of Beta-Glucocerebrosidase by Preparative-Scale High-Performance Liquid Chromatography: The Use of Ethylene Glycol-Containing Buffers for Chromatography of Hydrophobic Glycoprotein Enzymes," *Analytic Biochemist* 147:301-130, 1985.
91. Nelson, C., Census Bureau, U.S. Department of Commerce, personal communication, Mar. 26, 1991.
92. Nitkowsky, H. M., Madan, S., and Goldman, H., "Experience With Intravenous Infusions of Glucocerebrosidase in a Patient With Gaucher Disease" [abstract no. 560], *American Journal of Human Genetics*, vol. 49, 1991.
93. Ohashi, T., Marchese, S., Pegram, D., and Barranger, J.A., "Development and Application of a Treatment for Gaucher Disease Using Glucocerebrosidase Targeted to Macrophages" [abstract no. 552], *American Journal of Human Genetics*, vol. 49, 1991.
94. Osiecki-Newman, K. M., Fabbro, D., Dinur, T., et al., "Human Acid Beta-glucosidase: Affinity Purification of the Normal Placental and Gaucher Disease Splenic Enzymes on N-alkyl-deoxynojirimycin-sepharose," *Enzyme* 35:147-153, 1986.
95. Pastores, G. M., Sibille, A. R., and Grabowski, G. A., "Enzyme Augmentation Therapy in Gaucher Disease Type I: Dosage, Efficacy and Adverse Effects," *Clinical Research* 40:357A, 1992.
96. Pentchev, P. G., Brady, R. O., Hibbert, S.R., et al., "Isolation and Characterization of Glucocerebrosidase From Human Placental Tissue," *Journal of Biological Chemistry* 248:5256-61, 1973.
97. Rappeport, J.M., Barranger, J.A., and Ginns, E. I., "Bone Marrow Transplantation in Gaucher Disease," *Birth Defects* 22:101-109, 1986.
98. Reed, L., Chief of Medicaid Non-Institutional Payment Policy Branch, Health Care Financing Administration, Baltimore, MD, personal communication, Mar. 26, 1992.
99. Ringden, O., Groth, C. G., Aschan, J., et al., "Bone Marrow Transplantation for Metabolic Disorders

- at Huddinge Hospital,' *Transplantation Proceedings* 22:198-202, 1990.
100. Russell, L.B., "Some of the Tough Decisions Required by a National Health Plan," *Science* 240:892-896, 1989.
 101. Shore, R., Vice President of Research, Enzon Inc., Plainfield, NJ, personal communication, July 10, 1992.
 102. Short, J., Executive Secretary, Endocrinologic and Metabolic Drugs Advisory Committee, Food and Drug Administration, Washington, DC, personal communication, Oct. 14, 1992.
 103. Sorge, J., Kuhl, W., West, C., et al., Complete Correction of the Enzymatic Deficit of Type 1 Gaucher Disease Fibroblasts by Retroviral-mediated Gene Transfer," *Proceedings of the National Academy of Sciences USA* 84:906-909, 1987.
 104. Stahl, P.D., Rodman, J.S., Miller, M.J., et al., "Evidence for Receptor-Mediated Binding of Glycoproteins, Glycoconjugates, and Lysosomal Glycosidases by Alveolar Macrophages," *Proceedings of the National Academy of Sciences USA* 75:1399-1403, 1978.
 105. Starer, F., Sargent, J.D., and Hobbs, J.R., "Regression of the Radiological Changes of Gaucher's Disease Following Bone Marrow Transplantation," *British Journal of Radiology* 60:1189-1195, 1987.
 106. Strasberg, P.M., Lowden, J.A., and Mahuran, D., "Purification of Glucosylceramidase by Affinity Chromatography," *Canadian Journal of Biochemistry* 60:1025-1031, 1982.
 107. Taunton-Rigby, A., Vice President of Therapeutics, Genzyme Corporation, Chief Executive Officer, Henri Termeer, Treasurer, William Aleski, Director of Reimbursement, Genzyme Corporation, Boston, MA, personal communications, Oct. 20, 1991, Nov. 11, 1991, Dec. 6, 1991, Feb. 26, 1992, and Mar. 10, 1992.
 - 107a. Taunton-Rigby, A., Vice President of Therapeutics, Genzyme Corporation, Boston, MA, personal communication, June 12, 1992.
 - 107b. Taunton-Rigby, A., Vice President of Therapeutics, Genzyme Corporation, Boston, MA, personal communication, Sept. 4, 1992.
 108. Theophilus, B., Latham, T., Grabowski, G. A., et al., "Gaucher Disease: Molecular Heterogeneity and Phenotype-genotype Correlations," *American Journal of Human Genetics* 45:212-225, 1989.
 109. Tsai, P., Lipton, J. M., Sahdev, I., et al., "Allogeneic Bone Marrow Transplantation in Severe Gaucher Disease," *Pediatric Research* 31:503-507, 1992.
 110. Tsuji, S., Choudary, P.V., Martin, B.M., et al., "A Mutation in the Human Glucocerebrosidase Gene in Neuronopathic Gaucher's Disease," *New England Journal of Medicine* 316:570-575, 1987.
 111. Tsuji, S., Martin, B.M., Barranger, J.A., et al., "Genetic Heterogeneity in Type 1 Gaucher Disease: Multiple Genotypes in Ashkenazic and Non-Ashkenazic Individuals," *Proceedings of the National Academy of Sciences USA* 85:2349-2352, 1988.
 112. U.S. Congress, Library of Congress, Congressional Research Service, *Medicaid Source Book: Background Data and Analysis*, Report for the Subcommittee on Health and the Environment, Committee on Energy and Commerce, U.S. House of Representatives, Committee Print 100-AA (Washington, DC: U.S. Government Printing Office, 1988).
 113. U.S. Congress, Office of Technology Assessment, *Federal Policies and Medical Devices Industry, OTA-H-230* (Washington, DC: U.S. Government Printing Office, 1984).
 114. U.S. Congress, Office of Technology Assessment, *Home Drug Infusion Under Medicare, OTA-H-509* (Washington, DC: U.S. Government Printing Office, 1992).
 115. U.S. Congress, Office of Technology Assessment, *Government Policies and Pharmaceutical Research and Development* (Washington, DC: U.S. Government Printing Office, forthcoming).
 116. U.S. Department of Health and Human Services, Health Care Financing Administration, *Health Care Financing Program Statistics: Medicare and Medicaid Data Book, 1990*, HCFA Publication No. 03314 (Washington, DC: U.S. Government Printing Office, 1991).
 117. U.S. Department of Health and Human Services, Social Security Administration, *Disability, SSA* publication No. 05-10029 (Washington, DC: U.S. Government Printing Office, 1991).
 118. U.S. Department of Labor, Bureau of Labor Statistics, *Employee Benefits in Medium and Large Firms, 1989*, Bulletin 2363 (Washington, DC: U.S. Government Printing Office, 1991).
 119. Whellam, J., Branch Chief of the Payment Policy Branch, Health Care Financing Administration, Baltimore, MD, personal communication, Apr. 20, 1992.
 120. Wren, R., Director of Coverage and Eligibility Policy, Health Care Financing Administration, Baltimore, MD, personal communications, Oct. 3, 1991 and Mar. 24, 1992.
 121. Zelliner, J., Medical Adviser, Health Care Financing Administration, Baltimore, MD, personal communications, Oct. 3, 1991 and Mar. 24, 1992.
 122. Zimran, A., Gelbart, T., Westwood, B., Grabowski, G. A., and Beutler, E., "High Frequency of the Common Jewish Mutation for Type 1 Gaucher

- Disease Among the Ashkenazi Jewish Population," *Blood* 76:199a, 1991.
123. Zimran, A., Gelbart, T., Westwood, B., et al., "High Frequency of the Gaucher Disease Mutation at Nucleotide 1226 Among Ashkenazi Jews," *American Journal of Human Genetics* 49:855-859, 1991.
124. Zimran, A., Gross, E., West, C., et al., "Prediction of Severity of Gaucher's Disease by Identification of Mutations at DNA Level," *Lancet* II:349-352, 1989.
125. Zimran, A., Hadas-Halpern, I., and Abrahamov, A., letter, *New England Journal of Medicine* 325:1810-1811, 1991.