DRUG PRICING

GENZYME STRIKES GOLD WITH CEREDASE

NEW YORK—Genzyme's (Cambridge, MA) Ceredase—perhaps the most expensive drug ever—turned 11-year-old Brian Berman's life around. Before receiving Ceredase, which treats Gaucher disease, Brian was so anemic he couldn't walk up stairs. Anemia, in fact, threatened his life. Yet Ceredase has raised Brian's hemoglobin count to normal. "I'm a purple belt in karate. I play basketball and football. And I never get tired," says Brian.

Ceredase—the company's first approved pharmaceutical—should turn Genzyme around, too. Biotechnology analysts expect the 10-year-old company to turn its first profit this year, fueled by Ceredase sales as high as $45 million. This compares to 1990, when Genzyme lost $4.5 million on continuing-operations revenues of $55 million, from sales of diagnostics and intermediates, as well as research contracts. "With Ceredase, Genzyme will start reporting some real earnings," says Jeffrey Casdin, a biotech analyst at Oppenheimer (New York).

Ceredase's cost is astronomical. Genzyme estimates that Ceredase will initially cost Gaucher patients over $100,000 a year to bring their disease under control. Life-long maintenance will then cost patients up to $60,000 annually. But Joseph Edelman, a biotech analyst at Prudential Securities (New York), projects far higher costs. For initial Ceredase treatment, Gaucher patients at a New York clinic are currently paying an annual average of $270,000, says Edelman. "Even if annual treatment costs average out to $100,000 over a life time, a 10-year-old child who lives to 80 would need over $7 million worth of Ceredase," Edelman says.

Such prohibitive costs make third-party coverage of Ceredase essential, since few patients could afford the drug on their own. Genzyme expects all third parties to cover Ceredase, says Alison Taunton-Rigby, the company's senior vice president of biotherapeutics. She adds that several insurers, including some state Medicaid agencies, covered the product before April's approval by the U.S. Food and Drug Administration (FDA, Bethesda, MD), as FDA made it available under a treatment protocol. "Even more insurers have provided coverage since FDA approval," says Taunton-Rigby.

Ceredase's dramatic benefits practically guarantee third-party coverage. The drug actually reverses Gaucher disease, a hereditary disorder afflicting up to 3,000 Americans, predominately Jews of Eastern European origin. The disease occurs when the enzyme ß-glucocerebrosidase fails to break down the blood-cell lipid glucocerebroside, which collects in macrophages. These cells accumulate in the liver, bone marrow, and spleen, enlarging the liver and spleen and causing anemia and bone erosion. In clinical trials with 12 patients, Ceredase increased hemoglobin levels and decreased spleen enlargement in all patients. It boosted platelet counts in seven patients, cut liver size in five patients, and improved bones in three patients. "It's clear that long-term Ceredase use cuts—or eliminates—most of Gaucher patients' medical needs," says Teena Lerner, a biotech analyst at Shearson Lehman Brothers (New York). These needs, moreover, are "extensive, expensive, and life long," says Lerner, since Gaucher patients "always deteriorate as the disease gets progressively worse."

Ceredase is so expensive because production is complex. Genzyme begins production by isolating ß-glucocerebrosidase from human placental tissue supplied by Institut Mérieux (Paris). Tons of tissue are processed to extract grams of enzyme, with multiple steps to remove viruses. Genzyme then modifies the enzyme by removing sugar molecules from its oligosaccharide chains so they predominantly end with mannose residues. Carbohydrate receptors on macrophage cells specifically recognize such residues, allowing Ceredase uptake.

To lower production costs, Genzyme is developing a recombinant form of Ceredase. It expects to market the product—which it will make in mammalian cells—by 1993. The recombinant product will expand supplies, now limited by production to 2,500 patients a year, or about $100 million in sales. The recombinant version—even if priced lower than the current product—will also increase Genzyme's gross margins. Because present production is so elaborate, Genzyme expects Ceredase gross margins of about 55 percent, compared to 80 percent for most pharmaceuticals, biopharmaceuticals included.

High doses also contribute to Ceredase's expense. Genzyme recommends initial doses of 60 units—at $3.50 a unit—for each kilogram of body weight, with infusions every two weeks. Dosage is progressively lowered for maintenance therapy, and "significantly lower doses may be effective," says Genzyme's Taunton-Rigby. Yet Prudential Securities' Edelman says that a clinic in New York City hasn't lowered any of its Gaucher patients' initial Ceredase doses "even after some two years of treatment. This makes me dubious about the notion of lower maintenance doses."

Whatever Ceredase's cost and dosing, Genzyme has struck gold. Even Edelman projects a windfall, with Ceredase sales reaching $33 million this year, $62 million next year, and $78 million in 1993. At least one Gaucher patient taking Ceredase also believes in the drug. "I feel like I have a new life, like I have a new body," says the patient. "I have energy, and I can even stay up late at night. My whole family feels it. Everyone sees the difference."

—B.J. Spalding