Genzyme Details Market Potential of Alemtuzumab for MS

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Genzyme Corporation (NASDAQ: GENZ) today provided an extensive briefing on the market potential of alemtuzumab for multiple sclerosis at an event for investors and analysts in New York. During the two-hour presentation, the company shared internal market research and independent, third-party analysis defining the unmet needs today in MS, key features of the alemtuzumab profile that may address these needs and physician and payer perspectives on the future positioning and uptake of alemtuzumab in the MS market. This research supports forecasts for alemtuzumab adoption and anticipated peak sales of $3-3.5 billion.

The event was part of a program, initiated in October by Genzyme’s board and management, to communicate with shareholders regarding the company’s value.

Market Research

Genzyme has conducted extensive market research related to the market potential for alemtuzumab in MS, including interviews with more than 2,500 prescribers and payers in major markets. This work confirms that there is significant demand for new therapeutic options with improved efficacy, tolerability and convenience over existing therapies and that alemtuzumab has the potential to address many of these unmet needs.

“There is still a significant unmet need in multiple sclerosis and we desire new treatments,” said Edward Fox, M.D., Ph.D., Director of the Multiple Sclerosis Clinic of Central Texas, one of the largest multiple sclerosis treatment centers in the U.S. “Currently our goal is to slow disability progression and reduce relapses. However, alemtuzumab has the potential to improve function as well as to halt the accumulation of disability and relapses, setting a new bar for MS treatment efficacy.”

Genzyme’s market research found that many respondents view alemtuzumab as a potentially paradigm-shifting therapeutic option that may specifically address limitations of current care, based on the profile emerging from the phase 2 study.

Key findings from conjoint analyses, a statistical approach that assigns value to product features against those of competing products, show that:

- U.S. and European physicians strongly associate alemtuzumab with best-in-class efficacy against other disease modifying therapies, including natalizumab and emerging oral options cladribine and fingolimod;
- The most important attribute driving therapy choice in treatment-experienced patients, who comprise the majority of the MS market today, is efficacy;
- With the convenience and compliance advantage of annual dosing, a significant percentage of physicians rate alemtuzumab’s administration schedule as outstanding;
- Based upon alemtuzumab’s profile, physicians assign a higher preference share for alemtuzumab in treatment-experienced patients (43 percent) than treatment-naïve patients (11 percent), supporting the view that therapy switching by patients is anticipated to be a strong growth driver; and
- Physicians score alemtuzumab significantly higher than natalizumab on measures of safety and efficacy.

To evaluate these findings further, Genzyme’s board commissioned an independent market analysis completed in October 2010. This research was based on existing phase 2 data and assumed the approval of alemtuzumab and emerging oral therapies fingolimod and cladribine. The analysis included interviews with over 100 neurologists and payers, and surveys from more than 200 physicians across four major markets—the U.S., France, Germany and the United Kingdom. Key findings from this work include the following:

- Efficacy was the primary prescribing decision in all major markets;
- Alemtuzumab would be adopted across all lines of use;
- Second- and third-line alemtuzumab use is anticipated and held the greatest share in large markets;
- Physician adoption of alemtuzumab would approach 5 percent in the first year after launch; 10-12 percent two to three years after launch; and 18-20 percent five or more years after launch.

Revenue Projections

The consistent findings from this and other research supports market penetration launch assumptions and revenue growth models. The company expects rapid adoption and peak sales of roughly $3-3.5 billion within 5 years of launch.

The company’s projected adoption rates for alemtuzumab compare well against actual historical adoption rates for other
Early phase adoption rate projections for alemtuzumab are proportionate to the 2007-2009 adoption rates for MS therapy natalizumab (in its second launch following a post-marketing safety signal);

Peak market share projections for alemtuzumab are more conservative than the actual market share levels achieved by current MS therapies, as well as therapies with superior efficacy introduced in other disease areas, such as rheumatoid arthritis.

**Alemtuzumab MS Care Innovations and Pharmacoeconomic Impact**

Five-year follow-up data from the company’s completed phase 2 trial, a large randomized trial against an active MS comparator, found that alemtuzumab-treated patients had significant reductions in relapses and disability. For many alemtuzumab-treated patients, these benefits were sustained several years after receiving their last course of treatment. Over this period, the mean disability score for patients receiving alemtuzumab improved, but worsened for patients receiving the active comparator.

The precise mechanism of action of alemtuzumab in the treatment of MS is unknown and remains an area of active study. Emerging data suggest that the observed clinical effects of alemtuzumab in relapsing MS may be the result of immunomodulation following the repopulation of immune cells post-treatment. Specifically, research conducted by Genzyme in collaboration with investigators at the University of Cambridge suggests that:

- Depletion of CD52+ autoreactive lymphocytes may induce re-setting of the immunological clock;
- Enrichment of T cells with regulatory activity may result in a tolerogenic environment during repopulation; and
- Increased lymphocyte production of neurotrophic factors occurs post-treatment.

Adding to these innovations is a new dosing model. Unlike daily, weekly, or monthly dosing of existing therapies, alemtuzumab follows an annual course approach. The once-yearly schedule offers substantial convenience and potential tolerability advantages for patients, while avoiding some compliance issues that can occur with more frequent dosing such as daily pills or injections required for some current therapies.

Based on the phase 2 data, the company’s pharmaco-economic research shows a substantial positive impact on health system resources:

- Reduction in the costs related to chronic treatment;
- Potentially fewer hospitalizations and associated costs due to relapses; and
- A potential decrease in expenses from disability care.

The company has held more than 100 payer and regulatory interviews across major markets, and alemtuzumab’s potential innovations for MS care are recognized. Most large markets, including the U.S., are likely to support value pricing if phase 3 data confirms earlier findings.

**Phase 3 Program**

Genzyme is conducting two pivotal phase 3 trials to evaluate alemtuzumab in the treatment of MS. CARE-MS I, a randomized trial comparing alemtuzumab to Rebif, is studying early, active relapsing-remitting MS patients who have received no prior therapy. CARE-MS II, which also compares alemtuzumab to Rebif, is studying relapsing-remitting MS patients who experience disease activity while on other MS therapies. Data from the phase 3 trials are expected beginning in mid-2011. The company expects to file for U.S. and E.U. approval in early 2012, and has been granted fast track status by the FDA for this program.

Alemtuzumab is an investigational drug for the treatment of MS and must not be used in MS patients outside of a formal, regulated clinical trial setting in which appropriate patient monitoring measures are in place.

**About Genzyme**

One of the world's leading biotechnology companies, Genzyme is dedicated to making a major positive impact on the lives of people with serious diseases. Since 1981, the company has grown from a small start-up to a diversified enterprise with approximately 10,000 employees in locations spanning the globe and 2009 revenues of $4.5 billion. In 2010, Genzyme was named to the Fortune 500.

With many established products and services helping patients in 100 countries, Genzyme is a leader in the effort to develop and apply the most advanced technologies in the life sciences. The company's products and services are focused on rare inherited disorders, kidney disease, orthopaedics, cancer, transplant, and immune disease. Genzyme's commitment to innovation continues today with a substantial development program focused on these fields, as well as cardiovascular disease, neurodegenerative diseases, and other areas of unmet medical need.

**Important Information**

Genzyme has filed with the Securities and Exchange Commission a Solicitation/Recommendation Statement on Schedule 14D-9 relating to the tender offer by Sanofi-Aventis. Genzyme shareholders are advised to read the company’s Solicitation/Recommendation Statement on Schedule 14D-9 because it contains important information. Shareholders may obtain a free copy of the Solicitation/Recommendation Statement on Schedule 14D-9, as well as any other documents filed by Genzyme in connection with the tender offer, free of charge at the SEC's website at [http://www.sec.gov](http://www.sec.gov). In addition, investors can obtain free copies of these documents from Genzyme by directing a request to Genzyme at 500 Kendall Street, Cambridge, MA 02142, Attention: Shareholder Relations Department, or by calling 617-252-7500 and asking for the Shareholder Relations Department.

**Safe-Harbor**
This press release contains forward-looking statements, including the statements regarding: expectations for peak sales of alemtuzumab and related timing expectations; forecasts for adoption of alemtuzumab by physicians and the marketplace; the ability of alemtuzumab to meet patient needs in the treatment of MS; the potential of alemtuzumab to halt accumulation of disability and relapses and improve function; the potential of alemtuzumab to be a paradigm-shifting treatment option for MS; the view that patients switching to alemtuzumab will be a strong driver of growth; the possible mechanism of action of alemtuzumab; the potential tolerability advantages of alemtuzumab; the positive impact of adoption on health care system resources; the likelihood of value pricing being supported in large markets, including the U.S.; and the expected timelines for results of the phase 3 clinical trials and filing for regulatory approvals. These statements are subject to risks and uncertainties that could cause actual results to differ materially from those projected in these forward-looking statements. These risks and uncertainties include: the possibility that phase 3 trials will not support the same conclusions as the phase 2 trials; the risk that alemtuzumab will not receive regulatory approvals on the timeframes expected or at all; the risk that the results of the phase 3 clinical trials may be delayed; the possibility that peak sales of alemtuzumab will not achieve projected levels as a result of the inaccuracy of underlying assumptions or as a result of other risks and uncertainties not contemplated or adequately accounted for by Genzyme’s market research; the risks that alemtuzumab will not address the unmet needs of MS patients or that physicians and the marketplace will not adopt alemtuzumab to the extent expected; the risk that pricing will not be supported at projected levels; the risk we will not be able to implement our business plans for alemtuzumab successfully or as expected; and the risks and uncertainties described in Genzyme's SEC reports filed under the Securities Exchange Act of 1934, including the factors discussed under the caption “Risk Factors” in Genzyme's Quarterly Report on Form 10-Q for the period ended September 30, 2010. We caution investors not to place undue reliance on the forward-looking statements contained in this press release. These statements speak only as of the date of this press release, and we undertake no obligation to update or revise these statements.

Genzyme's press releases and other company information are available at www.genzyme.com and by calling Genzyme's investor information line at 1-800-905-4369 within the United States or 1-678-999-4572 outside the United States.

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