Daniel Vasella, Novartis and Kathy Bloomgarden, Ruder Finn

Patient Relations

Courage Under Fire

With one extraordinary product, Novartis showed how to make the most of relationships with patient advocacy groups.

Gleevec's discovery, development, and launch was a momentous experience that broke records at every stage: scientists delivering dramatic product efficacy never seen before in the history of cancer treatment; Novartis teams pulling together to greatly accelerate clinical trials, and rapid regulatory approvals. It seemed a perfect story.

Indeed, to an outside observer, it appeared that the events shaping the process that brought the extraordinary product to patients with chronic myelogenous leukemia (CML) were anxiety free. In reality, challenges at every step demanded strong leadership, expertise, intuition, and courage. Daniel Vasella's book, *The Magic Cancer Bullet*, tells the story behind the development of Gleevec (imatinib), and the difficult choices he and his colleagues made to develop a drug that appeared to help only a small population. (See "Excerpt from *The Magic Cancer Bullet*," page 16.) Approximately 6,650 people over the age of ten are diagnosed with CML each year in the United States, representing 1.3 per 100,000 worldwide.

Novartis recognized early that it would need to work closely with key stakeholders, because the pressures to turn its ST1571 compound into a new product and the scrutiny of the world community could have been a "time bomb" if not properly managed. The following were key drivers of success in the company's communications efforts:

Engagement of top leadership. Chairman and CEO Daniel Vasella became deeply involved in the tough choices the company faced. His book recounts many examples and illustrates his answer to the question: How do you cope with the pressures of managing such an innovative product with so many groups competing for time and attention and making contradictory demands? Complexities included a small patient population, the demand for the human and financial resources necessary to acceler-

ate development and production, and the costs to other parts of the business. Vasella was outspoken and visible at every step, which helped the entire management team move together quickly to meet tough goals. He instilled confidence in all internal and external stakeholders that the company was doing everything possible to quickly and responsibly bring Gleevec to patients.

Partnership with patients. Public pressure on Novartis was most evident in the exchanges with the CML patient community. Although Vasella was moving to have his team ramp up production, the company needed time to upgrade to the complex manufacturing process. The patient community exerted tremendous pressure on Novartis to do everything in its power to make more of the drug available. That was best exemplified by a patient petition with 3,000 signatures, pleading with the company to provide access to Gleevec treatment.

The interactive relationship with patients that emerged provided Novartis with insight that led to the rapid implementation and validation of a centralized patient referral system for the management of tremendous call volume, reaching as high as 2,000 per day during key medical meetings. The company's compassion for people in need created a bridge with the patient community that enabled Novartis to take the difficult steps necessary to maintain the clinical trials' scientific rigor. That meant saying no to patients who did not fit the eligibility criteria.

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Internal motivation throughout Novartis. The calls that Novartis executives, from the top down, received from patients and their families served as major motivations to meet every challenge and achieve every target necessary to bring Gleevec to patients as quickly as possible. Communications throughout the company kept focus on the patients who would benefit from the therapy.

Cooperation with regulatory agencies. The press conference announcing the medicine's FDA approval featured Health and Human Services secretary Tommy Thompson, acting FDA head Dr. Bernard Schwetz, National Cancer Institute head Dr. Richard Klausner, Vasella, and Gleevec patient Suzanne Drager. That group effort illustrated how closely all those individuals and their institutions worked to ensure an approval process that gave Gleevec the highest priority.

Patient assistance. Knowing that Gleevec's price would be high—given the overall R&D costs and the presence of a high-priced, less effective competitor—Novartis established a patient assistance program that ensured that patients with limited financial resources would have access to the drug. Vasella was clear with both internal and external audiences that the company would not launch Gleevec without the assistance program. That sensitivity and respect for patients helped the company avoid what could have been damaging public criticism of the price of such an innovative therapy.

The Gleevec story serves as a model for developing and launching novel medicines in the

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GOOD BUSINESS

Exceeding the company's expectations, Gleevec's worldwide sales topped $615 million in 2002. In February of that year, FDA approved the medicine for the treatment of gastrointestinal stromal tumors, which affect 5,000 people in the United States.

Novartis' current patient assistance programs include a toll-free reimbursement hotline, which patients can call to find answers to questions about insurance coverage, prescription costs, and billing.

face of intense public pressure. The development of AIDS therapies caused a storm of controversy over access and pricing, with accusations splashed across most major newspapers around the world. In the case of Gleevec, Novartis and Vasella helped manage public pressure with a strong communications effort, a willingness to take risks, an emphasis on speed and quality, and creative solutions. What made the management of Gleevec's develop-

Excerpt from

The Magic Cancer Bullet

...We felt what we were doing was right. But deep down we sometimes had doubts too. Whatever others might say, we were convinced of our mission. What could be more important than rescuing a patient on the brink of death? In the case of our breakthrough cancer drug, Gleevec, I remember that first moment when the evidence arrived that it worked in man.

It came in April 1999, the first results from Phase I patient trials for the drug's effect on CML patients. I had trouble believing it. The numbers told of 31 Phase I patients, all of whom had taken the drug at 300-milligram doses; every one of them had experienced a reduction in the number of their white blood cells to normal levels. Never before had any drug proven that effective on CML patients. Not radiation, not chemotherapy, not interferon, not Ara-C, not even the only known cure for the disease, a bone marrow transplant, as many patients die from this latter procedure. All of these therapies made some inroads into the disease; but none of them showed such a high percentage of success and so early in the treatment.

With such spectacular results in hand, the next steps were obvious, were they not? Directing large amounts of capital and human resources to developing this drug. Rushing the drug to market. How could we act otherwise? After all, who could forget that the lives of patients were at stake? What we really cared about were all the patients who were waiting for a better, safer and more effective drug. And now that our scientists had unleashed what appeared to be a breakthrough cancer drug, how could we not go full steam ahead in getting this drug to market?

But the next steps were not at all obvious. In fact, I had some agonizing choices to ponder. Forcing me to ponder these choices was one undeniable fact: the number of CML victims in America was relatively small compared to the victims of other forms of cancer. Some 6,650 Americans over the age of 10 are diagnosed with CML each year; worldwide, the figure is 1.3 per 100,000 a year. And yet, nearly 200,000 cases of prostate cancer and 195,000 cases of breast cancer were expected for 2001 in the US.

If it were up to us, we would not ask the questions that the leaders of any pharmaceutical business must ask. We would avoid asking how many patients suffer from CML, a large number or a small amount. We would not ask whether we could supply this drug without charging a high price. We would not ask what would be the costs to the other parts of our business, if we threw large amounts of capital and human resources into the development and manufacturing of this drug. We would skip those questions. But we're just not able to for, as I said, we are a business.

ment and launch so effective was the willingness of Novartis' leaders to reveal their own doubts and communicate openly. That led to a bond of trust with key stakeholders for the benefit of bringing a potentially life-saving medicine to those who needed it most. ▮

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As word of Gleevec's special properties began to surface, I had to make a tough decision: whether to break out of our usual drug development pattern by steering a large amount of resources, capital and human, at the drug far earlier than normal. I would be heightening the financial risk and perhaps creating huge "opportunity costs" by diverting resources that would have been earmarked for other drugs.

On that day in April, I immediately phoned our head of development at Novartis, Jörg Reinhardt. I told him that I was looking at the Phase I results for STI571 (the name for Gleevec during its development). "This can't be," I said to him, assuming that something was wrong, that somewhere there was an error. Finding errors in such results was uncommon but not impossible. Sometimes, one feels a surge of excitement, only to find that a lab error has occurred. Part of me wanted to jump for joy; but an inner voice warned, "Don't get excited. First double-check."

As if reading my mind, Jörg observed, "We did double-check. That's how it is."

We have to speed up the trials," I asserted. We as a management team have a duty to put a product on the market when there is a fair chance that it changes the practice of medicine even if it only helps a small number of patients. In that sense, we certainly do not function like all other corporations. Faced with the extraordinary data, I sensed that Novartis was bound to come under heavy pressures—from the media who would monitor our every move and who would delve into every aspect of the drug, its pros as well as its cons; from physicians who would hope to get the drug for patient trials; from individual patients who would want the drug; and from patient groups who would lobby us to get rapid access to it.

Let's face it: Patients have profoundly changed their behavior. Today's patients are a demanding lot. No longer ashamed or embarrassed by their illnesses, they assert a right to decide and a right to the best treatment. They hunger for information about their illnesses. They have taken to the Internet with a vengeance, and because of the Internet, they are smarter than ever before, discovering drugs at a very early stage of development; alerting their physicians to new drugs and therapies. I had every reason to believe that this Internet generation of patients had the potential of making life difficult for a pharmaceutical company that did not live up to their expectations.

The earlier example of AIDs patients transformed into activists against pharmaceutical enterprises who thought they had done a great thing in bringing the first AIDs drugs to market gave all of us at Novartis pause. We had to display our concern for CML patients; we had to show them that Novartis was doing all that it could to speed production of STI571.

by Dr. Daniel Vasella, Chairman and CEO, Novartis
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