Maturing biotechs turn to pharma's markets

Pleased with Sepracor's 2004 results and its anticipated launch in the first quarter of 2005 of one of biotech's rare mass-market drugs, investors have bid up the firm's stock to near three-year highs. Sepracor's focus on a primary care market is the latest example of a shift in strategy at certain maturing biotech companies to target broader, highly competitive markets traditionally served by big pharma.

Three years ago, after receipt of a 'not approvable' letter from the US Food and Drug Administration on its allergy medication Soltara, the active metabolite of the compound astemizole, Marlborough, Massachusetts-based Sepracor was in the doldrums. But now, with a $6-billion market cap at the time of writing, it is solidly among biotech's top ten, outperforming all the top-tier companies since 2002 except for Genzyme, its Boston-area neighbor, and Genentech in S. San Francisco, California.

The key to Sepracor's recovery has been Lunesta (eszopiclone), a single-isomer form of the Rhone-Poulenc Rorer compound zopiclone, approved in Europe more than a decade ago to treat insomnia. Lunesta is the product of Sepracor's original R&D approach. The company filed a slew of use patents on single-isomer forms of known dual-isomer or racemic drugs, as well as on active metabolites of existing drugs. The strategy led to development of two highly successful antihistamines, which it out-licensed: Schering-Plough's Clarinex (desloratadine, the active metabolite of loratadine) and Aventis's Allegra (fexofenadine, the active metabolite of Seldane, which was taken off the market in 1997 because of its association with cardiovascular side effects), as well several niche products including Xopenex, a single-isomer version of the asthma drug Albuterol.

But the ability to leverage a platform technology is only part of the reason for Sepracor's current attractiveness. The biotech firm is also showing it can put the resources it obtained as a result of that leverage to work—including the capital it was able to raise (over $1 billion in the past two years) after validating its platform through partnering and successful product commercialization.

Sepracor is turning to indications with a primary care market that its insomnia drug Lunesta examplifies; this could be a sign of maturation among biotech companies in the US.

For Sepracor, that meant deciding to build a commercial franchise in primary care, starting with Lunesta. And although a foray into primary care is highly unusual for a small company, the move reflects a maturation among biotechs, based on their ability to amass and efficiently use financial and commercial resources. Indeed, many biotech companies are transforming themselves in this manner.

"Companies that demonstrate that they can efficiently deploy capital are likely to get a disproportionate share of investment dollars," explains James Tananbaum, managing director at venture capital firm Prospect Venture Partners. "That's where the problems get interesting." For example, mass-market neurological therapies are one area of interest to investors: in the past three years, 3 of the 11 biopharmaceutical financings over $400 million went to companies developing treatments for sleep disorders—two by Sepracor and one by Cephalon—according to Windhover's Strategic Intelligence Systems database.

That's a relatively new marching order for biotech. The nature of early biotech specifically the development of recombinant proteins to replace or augment the body's natural protein production and disease-fighting capability, lent itself to niche applications in severe or life-threatening conditions: genetic diseases, cancer and immune disorders, in particular. Moreover, regulators have been more forgiving of safety issues when it comes to severe diseases with no alternative therapies.

Investors "blindly and indiscriminately invested in companies with that patina," Tananbaum points out. Now, however, the drug development world is bifurcated between mass-market opportunities and niche products. And other technology trends, especially those related to small-molecule drug development, now enable pursuit of broader indications, notes Alan Crane, CEO of Momenta Pharmaceuticals, a company located in Cambridge, Massachusetts, with a platform based on characterizing and protein glycosylation patterns. Plus, he notes, with a greater availability of capital, companies need to focus on bigger product opportunities. "VCs [venture capitalists] are looking for bigger returns, leading to a focus on bigger product opportunities," he points out.

"Today you see a range of capital deployment throughout biotech," adds Prospect Ventures' Tananbaum. "The incentive is to scale capital to the product opportunity. Products that serve a medical need can be either large or small. They can be produced through a major scientific advance and significant investment or through a modest scientific advance that addresses a market and/or medical need and a more modest investment."

For mass-market opportunities, which require substantial spending on marketing infrastructure, including a commitment to costly direct-to-consumer advertising, this means having a balanced approach between R&D and sales, explains Sepracor CFO David Southwell. Especially for small companies, the inherent risks of developing new drugs for primary care markets makes it important to have these downstream capabilities, to capture more value—and not lose it to partners—in the event of clinical success.

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