When a long shot is worth a shot

Tom Jacobs & Jeff Fischer

Biotech speculators crave newer, productless and profitless companies with exciting late-stage, high-potential product candidates. But with information today so easy to come by, no candidate remains hidden for long. That's why it's both a mystery and a delight to find an unknown biotech speculation.

Indeed, Northfield Laboratories (Northfield, IL, USA; Nasdaq:NFLD) has received scant attention. Despite its potential billion-dollar-a-year blood substitute in pivotal phase 3 trials in the United States, the company is valued at a paltry $260 million. It's a speculation for sure—everything depends on regulatory approval—but the possible payoff from Northfield's success is so great that this is one of those rare times when a careful speculation may be worth the risk of loss.

The potential

After 16 years in business, Northfield leads the race to develop a safe and effective alternative to transfused blood. The company's flagship product, PolyHeme (human hemoglobin modified by pyridoxylation and glutaraldehyde polymerization) has several advantages over its natural counterpart: it has a 12-month shelf life (versus a few weeks for real blood); it works with any blood type (whereas real blood can be donated only to recipients with compatible ABO and rhesus antigens); and it is guaranteed disease-free (again a potential problem for real blood). The product would first be used in acute blood loss such as with trauma patients, where saline solution is the norm because ambulances don't carry blood.

Currently in phase 3 trials, PolyHeme is being administered to trauma patients in ambulances serving hospitals around the country. The plan is to continue enrolling patients until reaching 720 at up to 30 sites. The primary endpoint is survival at 30 days.

The odds of success appear favorable. During previous clinical trials of PolyHeme in trauma and emergency surgeries at multiple hospitals, the survival rate for patients infused with unprecedented amounts of the PolyHeme blood substitute (up to twice the volume of an average adult) was 75% compared with an anticipated survival rate without PolyHeme of only 20%. In current trials, Northfield believes even a modest improvement—it needn't be anywhere near as strong as 75% versus 20%—in mortality rates would support a successful application for approval.

An April development provided further grounds for optimism. In its third review of four of the phase 3 trial data with 250 patients of 720 enrolled, the Independent Data Monitoring Committee gave a green light to the trial, without requiring more time or patients. The good news boosted the stock as much as 33% in one day, though it has since given back much of those gains.

The committee's fourth and final look will come after data on 500 patients, which may not be far off. Northfield hopes to complete enrollment of 720 patients by the end of 2005, and CEO Steven Gould says that PolyHeme could be approved in late 2006 or early 2007. Meanwhile, the company can easily fund development until then. Gould foresees an initial market of $500 million, with a potential $2-3 billion worldwide for multiple uses. That's why the company's enterprise value of $260 million could appreciate many times.

The risks

Should all go well, Northfield would profit from the product's success. It retains full rights for sales in the US, but has partnered with Pfizer (New York, NY, USA; NYSE:PFE) and Israel's Hemocare for certain foreign markets. That's a great combination of US revenues and milestone payments and royalties from strong partners overseas. But what might not go well, apart from disapproval?

In a first for drug trials, PolyHeme is approved for use without patient consent. This has created some controversy that, while unlikely, may slow the trials or the eventual use of PolyHeme if approved.

Manufacturing is not a cake walk, either. The starting material for PolyHeme is in-dated and outdated human blood, so inventory depends on blood collecting services. Competition for blood donors is high and, in some areas of the United States, nursing and phlebotomist shortages already restrict the ability to collect blood. The company expects to have all the supply it needs, but there is the risk of a shortage or price jumps.

Although there is no direct near-term competitor to PolyHeme, competitors are on the horizon. Baxter International (Deerfield, IL, USA: NYSE:BAX) has a product in phase 1 trials, years away from possible approval. Biopure (Cambridge, MA, USA; Nasdaq: BPURD) filed a 2002 BLA (Biologics License Application) in the US for Hemopure (bovine hemoglobin modified by glutaraldehyde polymerization), but the US Food and Drug Administration requested more information in 2003 and the product targets medical needs different from PolyHeme. Lastly, Hemosol's (Mississauga, ON, Canada; Nasdaq:HMSL) Hemolink (human hemoglobin crosslinked with O-raffinose) has been in phase 2 and phase 3 trials in the UK and Canada, but the company's finances are again precarious.

The strategy

Northfield's positives include a huge market with no competition on the horizon and a debt-free company with low overhead, but the negatives are that this is a one-shot deal, and approval is simply more likely than not. This kind of speculation should be restricted to a small part of a portfolio. Our practice in cases like this, should the rewards come, is to sell enough to cover our initial investment after a price double, and then let the profits run.

Tom Jacobs and Jeff Fischer are the founders of Complete Growth Investor (http://www.completegrowth.com/). Northfield Laboratories is a Complete Growth Investor selection and Jeff owns shares. Tom and Jeff cannot give individual investment advice but welcome your comments at Tom@completegrowth.com or Jeff@completegrowth.com.