

# GTC gets surprise boost from EU



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**GTC Biotherapeutics's anticlotting treatment is extracted from the milk of goats whose genes were altered before they were born so they produce a human blood protein in their milk.**

**Firm's treatment moves closer to becoming the 1st drug made in a genetically altered animal to win regulatory approval**

**By Stephen Heuser**  
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**T**he tiny Massachusetts biotechnology company GTC Biotherapeutics won a surprise victory yesterday in its bid to develop the first-ever approved drug made in the body of a genetically altered animal.

The scientific committee of the European Medicines Agency reversed a February decision and said it would recommend approval of ATryn, GTC's anticlotting drug grown in the milk of goats. European regulators are expected to issue a final decision on the application within three months.

The Framingham company said a clinical trial of ATryn in the United States should enroll its last patient by the end of the year, and that it hopes to apply for approval from the US Food and Drug Administration by the first half of 2007.

Investors reacted swiftly to yesterday's news, with GTC's stock price almost immediately doubling after the morning disclosure, from 98 cents to \$2 a share. It finished the day at \$1.81, up about 86 percent.

GTC keeps 1,400 goats on a farm in the central Massachusetts town of Charlton, essentially using the herd as a living drug factory. It alters the goats' genes before they are born so they produce a human blood protein in their milk. The milk is then purified, and the human protein extracted to make an injectible anticlotting drug for people with a hereditary lack of the protein.

The deficiency is rare, affecting about one in 3,000 to 5,000 people — about 60,000 in the United States. It can normally be treated with blood-thinning pills, but can cause dangerous clotting when patients go off the pills during childbirth or surgery.

European approval would mark a vindication for the 20-year effort to create useful drugs in animal milk. Many companies have abandoned the field because of the uncertainty clouding the approval process — specifically, the question of whether regulators would ever feel comfortable giving the OK to a drug extracted from milk, which is full of potentially risky animal proteins.

Yesterday's decision gives a cash boost to GTC, which has been trying to quell investor doubts that it would succeed. The company lost half its market value on a single day in February when European officials refused to approve its drug after a two-year campaign by the firm to win approval.

The European committee's positive opinion triggered a \$1 million payment from the company's European marketing partner Leo Pharma A/S, with an additional \$2 million on the way if the broader European Commission signs off and the drug can be sold.

Geoffrey F. Cox, GTC's chief executive, said in a statement that the approval was a "very significant achievement" because the company is the first "to obtain a positive regu-

latory opinion on the commercial use of a therapeutic protein that is produced transgenically."

An analyst with Cowen and Co. LLC, an investment and research firm, said it expects GTC's drug to go on the European market in the fourth quarter.

Currently, antithrombin is available only through donated blood. It costs \$1.72 per international unit, which translates to more than \$100,000 a pound. Each GTC goat can produce more than a pound and a half of protein per year — more than \$150,000 worth. Because of the goats' value, they are among the most carefully supervised farm animals in the world, with a rigorously monitored food supply and 24-hour veterinary care.

Analyst Navdeep Jaikaria has estimated the potential market for antithrombin at only \$50 million, far from blockbuster status. But GTC hopes primarily to use ATryn as proof that the concept will work, and then manufacture drugs for other clients.

GTC has 135 employees, about 70 of whom work on the farm. Although it is a far smaller biotech player than the region's billion-dollar firms such as Biogen Idec Inc. and Genzyme Corp., it is one of the few companies trying to create a market for an entirely new approach to drug making.

Biotechnology companies see transgenic goats as a potentially inexpensive source of protein drugs that are currently too complex to make with traditional techniques. Another local company, Merrimack Pharmaceuticals, is producing its experimental biotechnology drug in goats that live on GTC's Charlton farm.

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