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Avalanche Drops as Gene Therapy Trial Miss High Expectations (1)
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(Updates with analyst's comment in fourth paragraph.)

By Danielle Burger

(Bloomberg) -- Avalanche Biotechnologies Inc. shares tumbled after some patients in a safety study of its gene therapy for an age-related chronic eye disease still required injections from another drug.

After the company released trial data Monday, shares fell as much as 32 percent to $26.40 in late trading. The study assessed AVA-101's safety for patients with the disease, called wet age-related macular degeneration or wet AMD.

Although the study wasn't designed to show statistically significant differences, the group treated with AVA-101 got an average of two extra injections of Roche Holding AG's Lucentis, compared with four for the control group.

"When a company is valued at $1 billion at a phase 2 trial, the expectations are quite high," said Gbola Amusa, an analyst at Chardan Capital Markets in New York who has a neutral rating on the stock.

Of the 21 patients who received the drug, 42.9 percent had better or stable vision. That compared with 9.1 percent of patients in the control group of 11 patients.

If approved, AVA-101 would be the first gene therapy for the condition and could replace current options made by Regeneron Pharmaceuticals Inc. and Roche that require monthly injections in the eye. Avalanche's treatment theoretically fixes disease-causing genetic code with only one injection. The treatments generate more than $1.7 billion in annual sales for each company, or about two-thirds of Regeneron's sales.

Current options "create a burden and an inability to comply with treatments, which leads to vision loss," Thomas Chalberg, chief executive officer of Avalanche, said in an interview. "Our goal is to bring this to the majority of wet AMD patients who have a need for frequent injections."

Avalanche plans a study on the effectiveness of AVA-101 in the second half of this year, Chalberg said.

Wet AMD affects 150,000 people in the U.S., according to Avalanche. It typically occurs with aging patients when fluid from blood vessels leaks into the retina, causing loss of vision and blindness.

No serious adverse events related to AVA-101 were recorded in the trial, and the study met its main 12-month goal for safety. Patients in the AVA-101 group also showed an average improvement on a vision test, compared with an average decline for the control group.

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