

BioCentury

Emerging Company Profile

Acceleron: Value in the wallflower

By Michael Flanagan
Staff Writer

Acceleron Pharma Inc. is developing protein therapeutics that target the transforming growth factor beta superfamily to treat diseases ranging from musculoskeletal and metabolic disorders to cancer. Last month, Acceleron began Phase I testing of its lead candidate, ACE-011, a first-in-class activin receptor IIa antagonist to treat bone loss disorders.

President and CEO Glenn Batchelder said TGF beta bone growth factors represent "an under-appreciated space in biology that had not been exploited for its therapeutic potential."

Indeed, the first thing the company did after setting up shop was to accumulate as much of the available IP covering TGF beta as possible through licensing agreements with an assortment of academic institutions including the Salk Institute, Ludwig Institute, Karolinska Institute and the University of South Florida.

According to Batchelder, companies developing musculoskeletal products based on TGF beta in the past focused solely on the positive regulators of bone deposition. Medtronic Inc. (MDT, Minneapolis, Minn.) and Stryker Corp. (SYK, Kalamazoo, Mich.) market implantable forms of BMP-2 and BMP-7, respectively, which are approved under humani-

Acceleron Pharma Inc.

Cambridge, Mass.

Technology: Endogenous tissue-specific growth factor antagonists

Disease focus: Musculoskeletal, cancer and metabolic

Clinical status: Phase I

Founded: 2003 by John Knopf, Jasbir Sehra, Tom Maniatis, Mark Ptashne, and Wylie Vale

Number of employees: 38

Funds raised: \$26 million

Investors: Advanced Technology Ventures; Flagship Ventures; Polaris Ventures; Sutter Hill Ventures; and Venrock Associates

CEO: Glenn Batchelder

Patents: 12 issued for endogenous tissue-specific growth factor antagonists

tarian device exemptions for bone regeneration. BMP-2 and BMP-7 are both members of the TGF beta superfamily that promote osteoblast differentiation.

Acceleron's ACE-011, on the other hand, is a protein therapeutic that is administered systemically and acts by blocking activin receptor (ActR) IIa, which would otherwise bind to activin and promote the bone-resorptive activ-

ity of osteoclasts.

"In animal models of osteoporosis, ACE-011 has not just shown the ability to build bone back to normal levels," said CSO Jasbir Sehra. "The preclinical data shows we can regenerate bone in a model of severe osteoporosis to a level that is greater than you see in normal healthy young animals, and not only more bone, but high quality bone, with an increase in mass as well as strength."

CMO and SVP Matthew Sherman said ACE-011 has shown an ability to go beyond stemming the loss of bone associated with aging, offering the potential to build back bone that has been lost.

"There are a number of anti-resorptive agents out there that stop bone resorption, but they don't generate new bone," he said.

One of the only products that has shown the ability to rebuild bone, according to Batchelder, is Forteo teriparatide, a recombinant parathyroid hormone from Eli Lilly and Co. (LLY, Indianapolis, Ind.).

Indeed, much of the recent focus on potential osteoporosis therapeutics has been on recombinant PTH and PTH analogs, with a wave of newer products in late-stage development behind Forteo.

Sehra said that while PTH analogs

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PO Box 1246
San Carlos CA 94070-1246
Voice: 650-595-5333
Fax: 650-595-5589
www.biocentury.com

DAVID FLORES
President, CEO & Publisher

KAREN BERNSTEIN, Ph.D.
Chairman & Editor-in-Chief

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like Forteo have to be taken every day, ACE-011 would likely be dosed far less frequently. "We don't know the exact schedule yet, but we expect something along the lines of roughly once per month for a limited course," he said.

The Phase I trial of ACE-011 is in healthy post-menopausal women. Sherman said results are expected in next half, which would allow the company to start a Phase II trial in osteoporosis patients in 2007.

Next year, Acceleron will also look to start a Phase II to reduce bone loss in cancer patients.

Behind ACE-011, Acceleron (Cambridge, Mass.) is working on growth and differentiation factors that address disorders like muscle wasting, diabetes and neuromuscular diseases. The company expects to begin clinical development of a second product in mid-2007.