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'Draconian' ESA Plan Likely To Change

Kidney Limits Favor Amgen, Awaiting CMS Cancer Word

By Randall Osborne
West Coast Editor

Amgen Inc.'s stock gained ground as investors listened to pundits give forth their opinions on federal reimbursement changes disclosed Friday for the high-selling Aranesp (darbepoetin alfa) and Epogen (epoetin alfa), when used in kidney disease.

The minimal changes bode well not only for Amgen's marketing of ESAs in kidney indications, but also for the meeting by an FDA advisory panel, which next month will debate the safety of the drug class.

Due to take effect at the start of next year, the main policy revision by the Centers for Medicare & Medicaid Services for ESAs in kidney disease drops payments by half when hemoglobin levels reach 13 g/dL for three months in a row.

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Horizon's 'GI-Friendly' NSAID Work Backed By $30M Series C

By Jennifer Boggs
Staff Writer

Privately held Horizon Therapeutics Inc. raised $30 million in venture capital, securing adequate funding to complete two ongoing Phase III studies of lead product HZT-501, an NSAID combined with a gastroprotective agent, in patients suffering mild to moderate pain.

Essex Woodlands Health Ventures, of Palo Alto, led the Series C round, with participation from existing investors Scale Venture Partners, of Foster City, Calif., Sutter Hill Ventures, also of Palo Alto, and Pequot Ventures, of New York. Horizon, which previously brought in $15 million in Series B round in the fall, has raised a total of $51 million to date.

The latest financing should "bring us through Phase III trials" with the HZT-501 and, pending positive data, a new drug application either at the end of 2008 or the start of

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Advanced Magnetics Plans NDA On Phase III Ferumoxytol Data

By Jim Shrine
Staff Writer

Advanced Magnetics Inc. reported positive data from the fourth and final Phase III trial of its intravenous iron replacement therapy, and plans to file a new drug application in the fourth quarter.

The study of ferumoxytol in 230 chronic kidney disease patients on hemodialysis showed statistically significant improvement vs. oral iron on the primary endpoint of increased hemoglobin levels.

Both secondary endpoints also were met with statistical significance.

Brian Pereira, president and CEO of the Cambridge, Mass.-based company, said Advanced Magnetics intends to seek approval for use of ferumoxytol in treating CKD patients, including those on dialysis and those who are not. It also would

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NEWCO NEWS

Tacere Tries A New Strategy By Fighting HCV With RNAi

By Trista Morrison
Staff Writer

Tacere Therapeutics Inc. stands at the crossroads of two of the more lucrative partnering opportunities in the biotech space: RNAi and HCV.

Founded in July 2006, the San Jose, Calif.-based company is preparing to start clinical trials late next year with lead product TT-033, a combination of three short-hairpin RNAs (shRNAs) delivered within an adeno-associated virus (AAV) protein coat to three separate regions of the hepatitis C virus.

Tacere's work with shRNA, also known as "expressed

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Tacere

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RNA," differentiates the company from others in the field, said co-founder and Chief Financial Officer Mike Catelani. Most RNAi companies are pursuing "delivered" RNA, which uses a small-molecule type of approach to deliver short-interfering RNA (siRNA), he explained.

TT-033's AAV delivery has "a very good safety profile" and has been shown in preclinical studies to provide "very good delivery to hepatocytes," Catelani said. In a mouse study, a single administration of TT-033 provided more than 60 percent inhibition of the three targeted regions of the HCV genome for two months.

The opportunity is significant for HCV therapies that can improve on the standard of care, pegylated alpha interferon and ribavirin, which can cause serious side effects and only works in about 55 percent of patients. Leading the pack are small-molecule protease and polymerase inhibitors such as Vertex Pharmaceuticals Inc.'s telaprevir, Schering-Plough Corp.'s SCH 503034 and ViroPharma Inc.'s HCV-796.

Yet Tacere predicts that many of the new HCV drugs still will have side effects due to co-administration with interferon, not to mention resistance issues over time. TT-033 is designed to be administered in a single dose given via peripheral IV, and its ability to target three separate regions of the HCV genome is anticipated to prevent resistance. Additionally, its three shRNAs were selected to be effective against all HCV genotypes.

Although Tacere is new, TT-033 is not. The product emerged from Avocel Inc., an RNAi start-up acquired by Benitec Ltd. in 2004. After two years of early stage development, Benitec decided to divest TT-033 and return its focus to intellectual property acquisitions. Sara Hall, current CEO of Tacere, previous CEO of Benitec and founder of Avocel, teamed up with Catelani to license TT-033 along with Benitec's broad patent on expressed RNA.

Hall and Catelani named Tacere after the Italian word for silence. As of now, they are the only full-time employees, but they plan to begin building up the team in the fall as they work on expanding their RNAi pipeline and preparing for clinical trials.

Hall said a recent pre-IND meeting with CBER went "very well" and the company should have "no trouble with IND approval" unless the IND-enabling studies turn up something "completely unexpected."

Thus far, Tacere's funding has come from a seed round provided by Hokkaido Venture Capital Inc., of Tokyo. Hall said Hokkaido had been interested in investing in the Series A round for Avocel, but the financing was never completed due to the Benitec acquisition. She added that Asian investors are a good fit for RNAi because they have smaller funds and can invest in earlier-stage, riskier prospects.

Additional financing came by way of an equity investment from Oncolys BioPharma Inc., also of Tokyo. In exchange, Oncolys got an option to acquire Asian rights to TT-033.

While Tacere plans to eventually raise a Series A, Hall said she'd like to postpone the round until the company is near or in the clinic, so the "Series A has the value proposition of a Series B." In the mean time, Tacere is "in discussions" with a potential partner regarding a nonequity-based deal.

If precedent serves, Tacere should have no problem generating partnering interest. Earlier this month, Alnylam Pharmaceuticals Inc. signed a potential $1 billion nonexclusive RNAi deal with Roche Holding AG, and Silence Therapeutics plc inked a potential $403 million RNAi deal with AstraZeneca plc. Last year, Merck & Co. Inc. purchased then RNAi-leader Sirna Therapeutics Inc. for $1.1 billion. (See *BioWorld Today*, Nov. 1, 2006, July 9, 2007, and July 10, 2007.)

HCV drugs have commanded significant big pharma interest as well, including a potential $545 million ex-U.S. rights deal between Vertex and Janssen Pharmaceutica NV, a potential $530 million preclinical co-development deal between InterMune Inc. and F. Hoffmann-La Roche Ltd. and a potential $307 million preclinical deal between Enanta Pharmaceuticals Inc. and Abbott Laboratories Inc. Novartis AG has been particularly active in HCV, signing a deal worth up to $552 million with Human Genome Sciences Inc., a deal worth up to $570 million with Anadys Pharmaceuticals Inc., and deals worth well over a billion dollars with Idenix Pharmaceuticals Inc. (See *BioWorld Today*, March 27, 2003; June 3, 2005; March 30, 2006; June 7, 2006; July 5, 2006; Oct. 18, 2006; and Dec. 13, 2006.) ■

OTHER NEWS TO NOTE

• **IDM Pharma Inc.**, of Irvine, Calif., said it recently informed the FDA that it intends to take immediate action to supplement the data in its current new drug application for mifamurtide (L-MTP-PE), formerly known as Junovan, which is being reviewed for the treatment of children and adolescents with non-metastatic osteosarcoma. The company plans to submit additional data on whether subjects in the Phase III trial remain alive or have died, which was not available at the time of filing of the NDA in October 2006. After the data are analyzed, the company expects to submit an amendment to the NDA by the first quarter of 2008. In May the FDA's Oncologic Drugs Advisory Committee voted 12 to 2 that the data in the NDA do not provide substantial evidence of effectiveness of L-MTP-PE in the treatment of patients with non-metastatic, resectable osteosarcoma receiving combination chemotherapy. The company anticipates an FDA decision in late August.