

BIO WORLD[®] TODAY

THURSDAY
JANUARY 10, 2008

THE DAILY BIOTECHNOLOGY NEWSPAPER

VOLUME 19, No. 7
SPECIAL REPRINT

Inspiration Teams with Celtic in \$35M Hemophilia Deal

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While ramping up to begin clinical trials with its recombinant Factor IX product for hemophilia B, Inspiration Biopharmaceuticals Inc. entered a \$35 million collaboration with Celtic Pharmaceutical Holdings LP.

Global private equity and drug development firm Celtic Pharma, of New York, is known for investing in late-stage programs, ushering them through approval, and auctioning them off to pharmaceutical companies. Stephen Evans-Freke, Celtic's co-managing general partner, told *BioWorld Today* the structure of the Inspiration deal mirrors previous deals his firm has done, such as with Neurobiological Technologies Inc.

Under the terms of the collaboration, Celtic will make a "modest" equity investment in Inspiration, Evans-Freke said. The majority of the \$35 million, however, will support development of IB1001, Inspiration's recombinant Factor IX product. Celtic will lead strategic decision making and manage outsourcing related to the product until both parties decide to sell it to a pharmaceutical company. As the collaboration progresses, Celtic will gain a controlling interest in IB1001.

The \$650 million hemophilia B market is dominated by Wyeth's recombinant Factor IX product, BeneFIX. Yet Evans-Freke noted that BeneFIX faces patent expiration in 2011. He added that the drug can cost \$200,000 a year and is "significantly less potent than the natural protein."

Enter Laguna Niguel, Calif.-based Inspiration, which was founded in 2004 by Chairman John Taylor and Vice Chairman Scott Martin, both of whom have children with hemophilia B. Taylor and Martin funded the company themselves, along with select angel investors, and brought in Michael Griffith, formerly of Baxter International Inc., to serve as president and CEO.

Inspiration's lead product, IB1001, is a recombinant Factor IX protein manufactured in a way that increases

cell-line productivity and decreases cost of production. Inspiration filed an investigational new drug application for the product last fall and expects to begin a Phase I/II trial this year. Griffith described IB1001 as a "sort of biosimilar," since manufacturing changes to reduce cost would be the only differentiation from BeneFIX, but he added that the FDA tends to view any manufacturing changes for biologics as "significant."

Behind IB1001 is a second-generation program focused on addressing the more complex issues associated with Factor IX. Ideally, Inspiration hopes to find a noninvasive delivery route and potentially increase potency and provide a longer duration of action. The second product is tentatively slated to enter the clinic in 2009. Inspiration also is interested in applying its technology to recombinant Factor VIII, which is used to treat hemophilia A, and recombinant Factor VIIa, which is used as a fall-back treatment for hemophilia A and B as well as in other indications such as trauma.

The opportunity to improve on existing recombinant blood proteins for hemophilia has drawn significant interest. For hemophilia B, which affects an estimated 3,600 Americans, improved Factor IX products are in early development at Amsterdam Molecular Therapeutics BV, Nastech Pharmaceutical Co. (in collaboration with an unnamed partner), Neose Technologies Inc. (in collaboration with Novo Nordisk A/S) and Biogen Idec Inc. (through its \$120 million acquisition of Syntonix Pharmaceuticals Inc.).

Not wanting to be left behind, Wyeth has signed deals with Nautilus Biotech and MediVas LLC to develop longer-acting Factor IX proteins, some of which may be delivered subcutaneously or orally. (See *BioWorld Today*, Feb. 6, 2007.)

For hemophilia A, which affects about 14,500 Americans, multiple recombinant Factor VIII products already are available. Yet long-acting versions are under development. Biogen Idec Inc. gained one through its

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Syntonix deal, and Novo's deal with Neose covers Factor VIII as well. Just last month, Nektar Therapeutics and Baxter International Inc. expanded their deal for long-acting hemophilia treatments.

The market leader for recombinant Factor VIIIa is NovoSeven, from Novo Nordisk A/S, but some reports have linked it to blood clots. Catalyst Biosciences Inc. is in preclinical studies with a version designed to improve on the potency of NovoSeven, and long-acting versions are under development by Novo and Neose, CSL Behring and Maxygen Inc.

Griffith said Inspiration is hopeful that its seed funding and the money from Celtic will carry it through possible corporate deals for some of its programs, rather than pursuing traditional venture capital.

Celtic's investment in Inspiration is the last of nine to be financed through the firm's original \$400 million fund. Future investments will come from two new \$750 million funds, the first of which Evans-Freke said will close next month. (See *BioWorld Today*, Dec. 13, 2007.) ■