BIOWORLD™ TODAY

THE DAILY BIOPHARMACEUTICAL NEWS SOURCE

AUGUST 4, 2015 | BIOTECH'S MOST RESPECTED NEWS SOURCE FOR MORE THAN 20 YEARS | VOLUME 26, NO. 146

SOTAGLIFLOZIN DISCLOSIN' BY END OF 2016

Value defined: Lexicon's carcinoid syndrome therapy wins in phase III, diabetes big driver

By Randy Osborne, Staff Writer

With positive top-line phase III data for its carcinoid-syndrome therapy boosting shares by 61 percent, Lexicon Pharmaceuticals Inc. was still undervalued in the view of some analysts, as Wall Street awaits late-stage data on the firm's larger opportunity: a therapy for type I diabetes, and possibly type 2.

The Woodlands, Texas-based Lexicon's stock (NASDAQ:LXRX) closed Monday at $13.60, up $5.16, as company backers cheered news that the pivotal phase III trial known as Telestar met its primary endpoint. "Now that we have these data, we will go

See Lexicon, page 3

Shire sees promise in pink eye drug; nabs Foresight for $300M

By Jennifer Boggs, Managing Editor

As it awaits an October FDA decision on lifitegrast, a dry eye drug acquired via a 2013 buyout of Sarcode Bioscience Inc., Shire plc is bolstering its ophthalmology offerings with another acquisition, snapping up privately held Foresight Biotherapeutics Inc. for $300 million in cash.

See Foresight, page 4

THE BIOWORLD BIOME

LINKING CHECKPOINTS, CLEARANCE

P53 target is a novel checkpoint inhibitor

By Anette Breindl, Senior Science Editor

Scientists have discovered a new checkpoint inhibitor that is downstream of p53, a transcription factor that is mutated in a large fraction of all cancers but has been notoriously hard to target therapeutically.

See Checkpoints, page 5

REGULATORY

FDA drug, device user fees take a hike again

By Mari Serebrov, Regulatory Editor

The cost of developing and marketing drugs and devices in the U.S. is continuing to go up as the FDA raises its annual user fees for prescription drugs, biosimilars, generics and medical devices for fiscal 2016.

The increases, which go into effect Oct.

See Regulatory, page 6

IN THE CLINIC

Awaiting FDA nod, Vonvendi proves mettle in bleeding disorder

By Marie Powers, News Editor

With a biologics license application (BLA) for BAX 111 (vonicog alfa) already pending at the FDA, investigators for the phase III study of the Baxalta Inc. therapy, branded Vonvendi, reported

See Baxalta, page 7

JAPAN

Pitch to change drug, device pricing in Japan backfires

By Pearl Liu, Contributing Writer

HONG KONG – A proposed annual adjustment on the pricing of pharmaceuticals and medical devices in Japan backfired after manufacturers publicly opposed it. A multi-country

See Pricing, page 8

IN THE CLINIC

Contravir tests ability of FV-100 to turn down shingles pain

By Michael Fitzhugh, Staff Writer

Contravir Pharmaceuticals Inc. has enrolled the first patient in what could be the sole pivotal trial of FV-100, an oral herpes zoster therapy, that it hopes to market as a treatment for both shingles

See Contravir, page 9

JAPAN

Astrazeneca makes biosimilar debut via joint venture with FKB

By Catherine Makino, Staff Writer

TOKYO – Hoping to combine the affordability of biosimilars with the promise of combination cancer therapies, London-based Astrazeneca plc is making its first foray into biosimilar development

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Contravir

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and the reduction of post-herpetic neuralgia (PHN), a painful shingles-associated condition that becomes more common as people age.

Should it succeed, Contravir CEO and President James Sapirstein told *BioWorld* Today that he believes it could achieve between $750 million and more than $1 billion in sales of the drug, depending on the dosing regimen and how significantly it reduces PNH, which is treated today with various products, including lidocaine and capsaicin patches as well as opioids in some cases.

To measure FV-100's potential, Contravir is comparing the drug to valacyclovir, an FDA-approved shingles drug in a phase III study that will measure reduction in the incidence of PHN as its primary endpoint. The double-blind, parallel-group, comparative trial, called study 007, will randomize patients to one of three arms: FV-100 400 mg once a day, FV-100 400 mg twice a day or valacyclovir 1,000 mg three times a day. Patients will then be analyzed for a seven-day treatment period, tracking their PNH pain daily, with follow-up through day 120.

Depending on how difficult it is to enroll the 825 patients needed for the trial, FDA reviewers have left open the possibility that they may require just one pivotal trial before allowing Contravir to submit its new drug application for FV-100. Determining how likely that possibility is will take time and a few meetings with agency staff, Sapirstein said.

The biggest study of FV-100 to date, a phase II trial started in May 2009, was encouraging, but failed to show a statistically significant advantage for the drug over valacyclovir in reducing the incidence of PNH. The double-blind, randomized trial, called study 005, was run by Inhibitex and included 350 patients who received either 200 mg or 400 mg of FV-100 once daily or 1,000 mg valacyclovir three times daily. Only 12 percent of patients treated with the 400 mg dose of FV-100 developed PHN vs. 20 percent of valacyclovir-treated patients, according to Thomson Reuters Cortellis Clinical Trials Intelligence. Despite pointing to FV-100's potential, the study was hobbled by sporadic collection of pain data, "so while the results were very positive, they just missed the endpoint," said Sapirstein. Given improvements in the standard measurements of pain since, he said Contravir will be able to deliver a better result, potentially approaching a 50 percent reduction in the incidence of PNH or better (See *BioWorld Today*, Oct. 26, 2009.)

Edison, N.J.-based Contravir is a spinout of Synergy Pharmaceuticals Inc., which acquired FV-100, from Bristol-Myers Squibb Co. for $1 million in August 2012. BMS had acquired the drug via the $2.5 billion Inhibitex Inc. buyout earlier that year. Prior to that, the drug was first developed by Fermavir Pharmaceuticals Inc., which identified the nucleoside analog as a prodrug of CF-1743, a compound that the Welsh School of Pharmacy had identified as a potential treatment for varicella zoster virus and HIV. (See *BioWorld Today*, April 11, 2007.)

Chris McGuigan, a professor of medicinal chemistry at Cardiff University in Wales and the discoverer of FV-100, joined Synergy's board in 2008 and continues there today. When Synergy, which chiefly focuses on gastrointestinal diseases, decided to spin out Contravir in February 2014, he joined Contravir's board as well. (See *BioWorld Today*, Nov. 30, 2011.)

To carry the drug forward, Contravir raised $9 million through the private placement of series A convertible preferred stock to a non-U.S. investor, a deal it completed in October 2014. That financing also included a side note for an additional $3.5 million that the investor exercised through the first and second quarters of this year. Despite the $12.5 million raised, Contravir will need to raise additional funds to complete the current phase III study, which could take about two years to complete, said Sapirstein. (See *BioWorld Today*, Jan. 10, 2012.)

Contravir also is developing CMX157, an analog of Gilead Sciences Inc.'s tenofovir DF (Viread), for the hepatitis B virus, a program it hopes to advance into phase II by the end of the year. Licensed from Chimerix Inc. in exchange for an up-front payment of 120,000 preferred shares of Contravir valued at $1.2 million, CMX157 is active against HBV and appears from early studies to be more than four times more potent in vitro versus tenofovir, according to the company. Chimerix is eligible to receive up to about $20 million in clinical, regulatory and initial commercial milestones in the U.S. and Europe, as well as royalties and additional milestones based on commercial sales in those territories under deal terms.

Contravir shares (NASDAQ:CTRV) rose 6.5 percent, or 31 cents, to $5.11 on Monday. //

OTHER NEWS TO NOTE

Bristol-Myers Squibb Co., of New York, established a multi-institutional initiative with U.S. academic-based cancer centers to investigate immuno-oncology therapies as potential treatment options for patients with high-risk, poor-prognostic cancers, or patients who have aggressive disease with an increased potential for early metastasis to multiple sites and/or are initially refractory or subject to early recurrences with conventional cancer therapies. As part of the new Immuno-Oncology Rare Population Malignancy program, the Robert H. Lurie Comprehensive Cancer Center of Northwestern University and the Northwestern Medicine Developmental Therapeutics Institute (NMDTI) have entered into a collaboration agreement with BMS under which they will conduct a range of early phase clinical studies. BMS will fund positions within the NMDTI Developmental Therapeutics Fellowship program.