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Hopes on Xydalba now

Brinavess SOS? Cardiome Pharma: FDA turns 'code' shoulder; stock sinks on NDA reject

By Randy Osborne, Staff Writer

Having promised to let Cardiome Pharma Corp. know about its NDA resubmission before the end of September, the FDA surprised the company by responding a month ahead, declaring the data package proposed for Brinavess (vernakalant hydrochloride) insufficient.

Shares of Vancouver, British Columbia-based Cardiome (NASDAQ:CRME) sank 32.8 percent Monday,

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In-house programs go forward

Ethris hooks big pharma partner in five-year mRNA discovery deal

By Nuala Moran, Staff Writer

LONDON – Ethris GmbH sealed a five-year agreement with Astrazeneca plc and its Medimmune biologics subsidiary for the discovery of messenger RNA (mRNA) therapeutics for treating pulmonary diseases.

Under the terms of the deal, Ethris will receive €25 million ($29.5 million) up front and be paid to carry out research on an undisclosed number of targets in asthma, chronic obstructive pulmonary disease and idiopathic pulmonary fibrosis.

In addition, the Munich, Germany-based company will be eligible for research and development milestone payments and sales royalties on products that make it to market.

That is far short of the $240 million up front that London-based Astrazeneca paid Moderna Therapeutics Inc. when the two agreed to work together in March 2013 to apply mRNA to cardiovascular and

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One piece of the FDA's funding is in place, now on to the spending bill

By Mari Serebrov, Regulatory Editor

While President Donald Trump's signature on the FDA Reauthorization Act (FDARA) Friday put to rest any lingering fears about potential layoffs at the FDA come Oct. 1, it also paved the way for the next congressional showdown – one that could still shutter parts of the FDA and other federal agencies.

When U.S. lawmakers return from their August recess in two weeks, they will be in a race against the clock to either agree to appropriations bills

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Saniona partner starts phase III trial of weight loss drug

By Michael Fitzhugh, Staff Writer

Productos Medix S.A. de C.V., a Mexican weight loss specialist leading the most advanced effort to date to get Danish drug developer Saniona AB's first product to market, started a phase III trial of the drug, tesofensine, in obesity. The Medix-funded study is due to finish within two years and could potentially support approval of the triple monoamine reuptake inhibitor as a monotherapy in Mexico, where it's estimated that more than 70 percent of people are overweight and more than 30 percent are clinically obese.

If approved in Mexico, sales of tesofensine could

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The BioWorld Biome

CAGE captures RNA function

First integrated atlas of microRNA expression compiled

By John Fox, Staff Writer

The latest study by the Functional Annotation of the Mammalian genome (FANTOM) group, an international consortium led by Japan's RIKEN Institute, has compiled the first extensive atlas of microRNA expression in human primary cells, which could help development of new cancer treatments.

MicroRNAs are small non-coding RNA molecules containing around 22 nucleotides. MicroRNA is

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Effort to create 'enabling ecosystem'

India ramps up biotech R&D with industry-academia initiative

By T.V. Padma, Staff Writer

NEW DELHI – India launched its first formal industry-academia mission to ramp up its biopharma development by promoting entrepreneurship and indigenous manufacturing to transform the country into a global hub for cutting-edge biotechnology R&D.

The mission was launched in New Delhi by India's Union Minister for Science and Technology Harsh Vardhan, who said it is "anticipated to be a game changer for the Indian biopharmaceutical industry" as it "aspires to create an enabling ecosystem to promote entrepreneurship and

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Saniona

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help Medix recover from its loss of an exclusive regional distribution deal with Medifast Inc. earlier this year while also generating key clinical data for Tesomet, a fixed-dose combination of tesofensine and the beta blocker metoprolol. Medix has exclusive rights to develop and commercialize both tesofensine and Tesomet in Mexico and Argentina.

Tesofensine's pound-shedding potential first came to light in 2005 during its development as a potential treatment for Alzheimer's and Parkinson's diseases by Neurosearch A/S, where the candidate was known as NS-2330.

A midstage trial called TIPO-1 confirmed the effect in late-2007, showing that patients lost up to an average of 11.3 kg (24.9 lb) on a 0.5 mg dose of the drug and 6.7 kg (14.8 lb) on a 0.25 mg dose vs. a 2.2 kg (4.9 lbs) loss in the placebo group.

"Neurosearch was considering going all the way with the drug," Jørgen Drejer, CEO and founder of Saniona, told *BioWorld*. "But it turned out that there was also a slight increase in heart rate with tesofensine and, for that reason, it became more complicated with clinical studies and regulatory, leading them to stop the [drug's] development."

Though there's potential for Mexican authorities to red flag the heart rate issue, they so far haven't done so, according to Drejer.

Cardiovascular concerns and the requisite big and expensive safety trials usually required to address these issues have derailed many a weight loss drug. But Drejer, once director of research at Neurosearch and its co-founder, as well, found a path forward with Tesomet. The combination with metoprolol avoids the affect on the heart rate, creating what he said is "an extremely safe drug" that could be used not only in obesity but also in more complicated metabolic disorders, like type 2 diabetes and Prader-Willi syndrome (PWS).

In January, Ballerup, Denmark-based Saniona reported top-line results from its phase IIa trial of Tesomet in patients with type 2 diabetes, with the primary endpoint showing a statistically significant reduction in heart rate for patients treated with Tesomet vs. placebo. The key secondary and exploratory endpoints regarding body weight and waist circumference also showed statistically significant reductions compared to placebo. Glycemic secondary endpoints were not statistically significantly different from placebo. The trial approximated the final formulation of Tesomet with two separate pills, so Saniona now is preparing to run a phase I trial with its single-pill formulation in an obese population that includes diabetic patients.

In parallel, the company initiated a phase IIa study in the Czech Republic and Hungary in PWS, a genetic disorder that causes obesity. If successful, that indication could pave a faster and cheaper path that the company could potentially take all the way to market on its own, Drejer said.

Meanwhile, the randomized, double-blind, placebo-controlled, parallel-arm study of tesofensine is getting underway in Mexico. Medix will recruit up to 372 ambulatory adults with obesity, randomizing them into three arms, each including 124 patients. Patients will receive either a 0.25-mg or 0.5-mg dose of tesofensine or placebo tablets once daily for 24 weeks. But first, they'll go through the study's two-week run-in period, during which they'll receive nutritional and exercise counseling.

The first group of patients has started the run-in period and will be randomized to one of the treatment arms or placebo later this month.

The primary endpoint of the trial is absolute and percent change in body weight over the treatment period. Secondary endpoints include proportions of patients achieving a weight loss of more than 5 and 10 percent, respectively, metabolic endpoints that include glycemic measures and quality of life, comprehensive tolerability and safety metrics. ♦

Other news to note

Ampliphi Biosciences Corp., of San Diego, said a case study highlighting the successful treatment of a critically ill patient with a multidrug-resistant (MDR) *Acinetobacter baumannii* infection will be featured in an oral presentation at the Centennial Celebration of Bacteriophage Research at the Institut Pasteur in Paris. The case study involves a patient first diagnosed with an abdominal *A. baumannii* infection who was treated with multiple courses of antibiotics over a four-month period, during which time the bacteria became resistant to cephalosporins, meropenem, gentamicin, amikacin, trimethoprim/sulfamethoxazole, tetracycline, ciprofloxacin and colistin. The patient continued to deteriorate and eventually fell into a coma. Ampliphi was involved in a joint effort that included several academic institutions and the U.S. Navy laboratory, producing a customized bacteriophage therapy specifically targeted to the *A. baumannii* strain infecting the patient. In March 2016, therapy was initiated under an emergency investigational new drug application approved by the FDA. Shortly after phage therapy was started, the patient emerged from the coma and continued to improve under ongoing phage therapy until the infection was cleared. To date, the infection has not returned, Ampliphi said.

Cytrx Corp., of Los Angeles, filed a preliminary proxy statement with the SEC in connection with a planned special meeting of stockholders to authorize its board to affect a reverse split of the company's common stock, if deemed necessary. The move could help the company to regain compliance with Nasdaq's $1 minimum bid price requirement and maintain its listing on the Nasdaq Capital Market. Company shares (NASDAQ:CYTR) closed at 57 cents on Monday.

Ironwood Pharmaceuticals Inc., of Cambridge, Mass., gained FDA approval for Duzallo as a once-daily oral treatment for hyperuricemia associated with gout in patients who did not achieve target serum uric acid levels with daily allopurinol alone. The drug is the first to combine allopurinol with the most recent FDA-approved treatment for the gout, lesinurad, marketed on its own as Zurampic (Astrazeneca plc). Ironwood expects Duzallo to be available commercially early in the fourth quarter.