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MONEY'S WHERE THE MOUTH IS

London calling: HCV data clash as EASL paints new landscape of all-oral therapy

By Randy Osborne, Staff Writer

Merck & Co. Inc. nearly stole the show at the European Association for the Study of the Liver (EASL) meeting in London with results from an ongoing phase II trial testing its all-oral combo drug for hepatitis C virus (HCV), but other firms kept the program lively, as Gilead Sciences Inc. held onto rock-star status. Mostly.

The C-worthy study by Merck features a pill that combines the NS3/4A protease inhibitor MK-5172 with MK-8742, an HCV NS5A replication complex inhibitor to treat patients with genotype 1 infection. Sustained viral responses (SVRs) with 12-week

See HCV, page 3

Adamas, Cerulean price IPOs as Wall Street anxiety remains high

By Jennifer Boggs, Managing Editor

Two more biopharma firms priced initial public offerings (IPOs), though Adamas Pharmaceuticals Inc. and Cerulean Pharmaceuticals Inc. received lukewarm welcomes on Nasdaq Thursday, as the Nasdaq Biotechnology Index – after a short-lived rally the day before – took another slide.

See Adamas, page 4

FINANCINGS

High tide for protides: Nucana Biomed pulls in $57M series B

By Cormac Sheridan, Staff Writer

Buoyed by unexpectedly early signs of robust clinical efficacy for its lead protide cancer drug Acelarin (NUC-1031), Nucana Biomed Ltd. raised $57 million in a series B round to accelerate its development

See Nucana, page 5

FINANCINGS

Spinifex attracts $45M, and new backers on neuropathic pain data

By Nuala Moran, Staff Writer

LONDON – Australian biotech Spinifex Pharmaceuticals carried the momentum generated by positive phase II data for its lead chronic pain program forward into a $45 million series C round that has

See Spinifex, page 6

BIO ASIA 2014

Incentives being used to kick-start innovation in Asia are working

By Cornelia Zou, Staff Writer

TOKYO – Facing long delays in access to game-changing medicines, Asian health care markets have become much more innovation-oriented over the past decade, but those efforts are conflicted

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REGULATORY

PTO tapping into the power of the crowd to seek out prior art

By Mari Serebrov, Washington Editor

The U.S. Patent and Trademark Office (PTO) is looking to the power of the crowd to strengthen the quality of patents by weeding out claims that aren't innovative.

See PTO, page 8

FINANCINGS

Minerva, Cymbay, Dance file to catch IPO wave

By Michael Fitzhugh, Staff Writer

Three clinical-stage companies, Minerva Neurosciences Inc., Cymabay Therapeutics Inc. and Dance Biopharm Inc. filed for initial public offerings (IPO) on Nasdaq in hopes of harnessing millions of dollars from enthusiastic

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CLINIC ROUNDUP

Celsion Corp. said final trial results from a 701-patient trial of its heat-sensitive liposome anticancer platform will be discussed at the 5th European Conference on Interventional Oncology, which is being held April 23-26, in Berlin. Data from the Lawrenceville, N.J.-based company's latest analysis suggests that its Thermodox platform may significantly improve overall survival, compared to a control, in patients whose lesions undergo the radiofrequency ablation treatment for 45 minutes or more.

Coronado Biosciences Inc., of Burlington, Mass., said an investigator-initiated study was launched to evaluate Trichuris suis ova (TSO), CNDO-201, to treat pediatric patients with autism spectrum disorder (ASD). The study is being conducted by the Hadassah-Hebrew University Medical Center in Jerusalem. The randomized, double-blind, placebo-controlled, 16-week study is expected to enroll 60 patients ages 6 to 17 who meet ASD diagnostic criteria. Patients will receive placebo, 2500 TSO or 7500 TSO every other week for 16 weeks. The pilot study is testing the safety of TSO compared to placebo in pediatric patients with ASD and evaluating efficacy signals on irritability, repetitive behaviors, global functioning and social cognition. Thursday, its shares (NASDAQ:CNDO) slid 13 cents, closing at $1.78. (See *BioWorld Today*, Dec. 16, 2013.)

Gene Signal International SA, of Lausanne, Switzerland, reported results from a phase IIa study in psoriasis, demonstrating that topical application of aganirsen (GS-101), an antisense oligonucleotide, reduced the size of psoriatic lesions and inflammation compared to placebo. Topical application of both doses of 0.86 mg/g or 1.72 mg/g aganirsen once per day for six weeks led to a significant reduction of -14.4 percent and -12.9 percent, respectively ($p < 0.05$), in the area of the treated psoriatic lesions compared to placebo. By contrast, an increase in the lesion area (+24.5 percent) was observed in placebo-treated patients. A significant treatment effect with aganirsen was also observed as early as three weeks. Results were published in *The Journal of Pharmacology and Experimental Therapeutics*.

Innate Pharma SA, of Marseille, France, said during its R&D update that it plans to start clinical trials of IPH2201, a humanized IgG4 antibody, in 2014 in three indications: head and neck cancer, chronic lymphocytic leukemia and ovarian cancer. The company had cash and equivalents totaling €37 million (US$51.4 million) as of March 30.

ISA Pharmaceuticals BV, of Leiden, the Netherlands, reported phase I data at the American Association for Cancer Research meeting in San Diego showing that cancer vaccine candidate ISA101 produced strong immune responses in almost all of the advanced cervical carcinoma patients treated. The study testing ISA101 in combination with standard chemotherapy cycles (carboplatin/paclitaxel). Comprehensive immune monitoring confirmed the beneficial effect of myeloid suppressor cell depletion associated with a robust induction of HPV16-specific T-cell responses that were sustained throughout several cycles of chemo. Based on those data, ISA initiated a phase I/II study last year testing ISA101 in combination with carboplatin, paclitaxel and pegylated interferon-alpha in women with advanced or recurrent cervical cancer.

Precision Biologics Inc., of Dallas, reported additional data from an ongoing phase I/IIa study with the monoclonal antibody NPC-1C. Investigators from the University of Texas Southwestern Medical Center, in collaboration with Johns Hopkins Hospital and Duke University Medical Center, presented data on 16 patients with refractory metastatic pancreatic and colorectal cancer treated with the NEO-102 version of NPC-1C. NEO-102 is a reformulation of NPC-1C with improved productivity, stability and safety. Despite advanced disease in the patient population, preliminary signs of activity based on stabilization of disease were observed in 5/9 (56 percent) of patients evaluable for response. Thirteen of 16 patients remain alive on study. Based on the results, the study was expanded and statistically powered to include 43 colorectal cancer and 30 pancreatic cancer chemotherapy refractory metastatic patients. A separate ongoing randomized study is using gemcitabine and Abraxane (nab-paclitaxel, Celgene Corp.) with or without NEO-102 in Folfirinox-refractory advanced pancreatic cancer patients. The data were reported at the American Association for Cancer Research annual meeting in San Diego.

Tocagen Inc., of San Diego, reported interim data at the American Association of Neurological Surgeons meeting in San Francisco, showing that, in patients treated to date, the combination of Toca 511 (vocimagene amiretrorepvec) and Toca FC (flucytosine) was shown to be safe and well tolerated with minimal treatment-related toxicity. In both studies, landmark six-month and 12-month survival rates were higher than historical published data. The presentation involved data from 68 patients with recurrent high-grade glioma. Toca 511/Toca FC is a gene therapy candidate designed to selectively infect and kill cancer cells via a dual mechanism of action.

OTHER NEWS TO NOTE

Ironwood Pharmaceuticals Inc., of Cambridge, Mass., and **Forest Laboratories Inc.**, of New York, launched a direct-to-consumer patient awareness campaign for Linzess (linaclotide), a once-daily treatment for adults with irritable bowel syndrome with constipation or chronic idiopathic constipation. The goal is to help patients identify symptoms of the disorders, relay them to their doctors and ask about Linzess.

Neurovive Pharmaceutical AB, of Stockholm, stock shares were registered with the OTC Reporting Facility for Foreign shares in New York with the ticker symbol NEVPF:US on March 20. Neurovive has traded on the NASDAQ OMX Stockholm (Sweden) Small Cap since April 2013 with ticker symbol NVP.ST. The NEVPF shares have been trading in the U.S. between $4 and $4.21.

OTHER NEWS TO NOTE

Portage Biotech Inc., of Toronto, said its wholly owned subsidiary, **Portage Pharmaceuticals Ltd.**, plans to focus preclinical development efforts on a cell-penetrating peptide that can carry therapeutic cargos as treatments for unmet medical needs. The company's lead candidate, which targets inflammation using a human-derived transporter protein platform, is active in isolated cells and in mice. The company plans additional animal model studies to confirm the activity of the lead candidate and to determine the most efficient path to clinical proof of concept in a therapeutic opportunity that addresses a large market and an unmet clinical need. (See *BioWorld Today*, Jan. 3, 2014.)

Prometic Life Sciences Inc., of Laval, Quebec, presented preclinical data at the 2014 annual meeting of the European Association for the Study of the Liver supporting the claim that PBI-4050's anti-fibrotic activity could also address various liver conditions such as non-alcoholic steatohepatitis. PBI-4050's favorable effect in reducing the progression of fibrosis in the liver was demonstrated in a gold standard animal model where liver fibrosis is induced by chronic administration of carbon tetrachloride, a chemical which at high chronic doses, causes irreversible damages to the liver and kidneys. Animals treated with PBI-4050 displayed a significant reduction of liver lesions as evidenced by histology and relevant biomarkers results. Following prolonged exposure to CCL4, a significant number of the non-treated animals also developed hepatocellular carcinoma unlike those treated with PBI-4050.

Xencor Inc., of Monrovia, Calif., received a milestone payment from **Merck & Co. Inc.**, of Whitehouse Station, N.J., through a subsidiary, triggered by the initiation of a phase I study of an undisclosed biologic drug candidate that uses Xencor's Xmab antibody engineering intellectual property (IP). In June 2013, Xencor granted Merck a non-exclusive license to certain patents for use in an undisclosed product and an option to license the IP for future products. Xencor received an up-front payment and continues to receive annual maintenance fees. In addition to milestone payments associated with development of Merck product candidates, Xencor is eligible for royalties on product sales.

Twenty-five international medical societies, under the umbrella of the Androgen Study Group, petitioned the *Journal of the American Medical Association* (JAMA) to retract an article that prompted recent concerns regarding cardiovascular risks with testosterone therapy. The organizations cited "gross data mismanagement," rendering the study "no longer credible." The findings by Rebecca Vigen and colleagues from the University of Colorado, published in the Nov. 13, 2013, issue of *JAMA*, were widely reported as evidence that testosterone therapy is associated with cardiovascular risks, resulting in the issuance of an FDA safety bulletin on Jan. 31. The group called the petition "a complete repudiation of the false information published by *JAMA*" and alleged the journal was complicit in creating a media frenzy regarding false risks and causing a wave of medical malpractice cases. The group said *JAMA* published a revision online Nov. 12, 2013, due to the presentation of results as raw data instead of statistically derived estimates, but failed to disclose the revision for two months. A second correction, published in March, revealed major errors in the article's text and figure, according to the group.

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