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ON THE WAY TO M&A?

Spinning out hemophilia franchise, Biogen narrows focus to neurology

By Michael Fitzhugh, Staff Writer

Biogen Inc. unveiled plans Tuesday to sharpen its focus on neurology by breaking its hemophilia business out into a new publicly traded company. The tax-free transaction is expected to be a boon for Biogen shareholders, but analysts were split on its broader implications.

The new company is set to include both of Biogen's commercial hemophilia assets, Alprolix (coagulation factor IX [recombinant], Fc fusion protein) and Eloctate (antihemophilic factor [recombinant], Fc fusion protein). Together, they accounted for

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THE WORLD ACCORDING TO PARP

Capital Ideaya: $46M take propels synthetic lethality; gene pairs kicked upstairs

By Randy Osborne, Staff Writer

Ideaya Biosciences Inc. CEO Yujiro Hata told *BioWorld Today* that "you'd be hard pressed to find a more personalized-medicine approach to cancer" than synthetic lethality, and his firm hopes

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DEALS AND M&A

Kynamro dealt to newco Kastle Therapeutics for potential $95M

By Marie Powers, News Editor

Eight years after Ionis Pharmaceuticals Inc. (then Isis Pharmaceuticals Inc.) selected then-independent Genzyme Corp. as the partner for its phase III lipid-lowering drug, mipomersen,

See Kastle, page 5

THE BIOWORLD BIOME

ANGIOPOIETIN 2: THE EYES HAVE IT

Study identifies new therapeutic target for AMD

By John Fox, Staff Writer

HONG KONG – A recent study by scientists at the Department of Ophthalmology and Visual Sciences of the Faculty of Medicine at the Chinese

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REGULATORY

Counterfeits, trade secret theft plague drug, device industry

By Mari Serebrov, Regulatory Editor

In an economy driven by global competition, counterfeits and theft of trade secrets remain major hazards for U.S. drug and device companies, handing them an uneven playing field in some parts of the world.

When it comes to counterfeits, most of the problems can be traced to China, which the U.S. Trade Representative's (USTR) 2016 Special 301 Report refers to as "the manufacturing hub of counterfeit products." Together with Hong Kong, India and Singapore, China accounted for 97 percent of all counterfeit drugs seized at the U.S. border last year.

Their failure to curb counterfeits, along with other barriers to trade, once again earned China and India a spot on the USTR's Priority Watch List, along with Algeria, Argentina, Chile, Indonesia,

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REGULATORY

Tackling counterfeit drugs, India bolsters track-and-trace efforts

By T.V. Padma, Staff writer

HYDERABAD, India – India is strengthening its "track and trace" system for drugs by making it mandatory to put online information about barcodes on the packing of drugs being exported.

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Ideaya

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to build on proof of concept brought about by the approval of Astrazeneca plc's poly ADP-ribose polymerase (PARP) inhibitor Lynparza (olaparib) in BRCA-mutated advanced ovarian cancer. "We're not looking to develop a PARP inhibitor," he said, discussing the firm's $46 million series A round. "We're focused on what we calling PARP 2.0," aiming to "extend the genetic signature beyond BRCA."

Genes are called synthetic-lethal if the mutation of either of them by itself is deemed viable but a change in two or more together causes cell death. The approach will be investigated in genetically defined patient populations with an eye to exploiting inherent tumor susceptibilities. "We're starting with the specific genetic mutations of interest, so we would have a biomarker genetic signature from the get-go," Hata noted. Ideaya, of South San Francisco, plans to ransack the genome for synthetic-lethal duos with help from the CRISPR/Cas9 genome-editing system, which the firm already has begun using. Targeted PARP drug Lynparza was approved by the FDA and European regulators in 2014. (See *BioWorld Today*, Dec. 22, 2014.)

Though not developing a PARP inhibitor specifically, Ideaya (pronounced like "idea," on which the name is word play) can learn from mistakes made in that field. Paris-based Sanofi SA's PARP candidate, BSI-201 (iniparib), failed in a phase III triple-negative breast cancer study in early 2011. Sanofi, which picked that drug up with its $500 million acquisition of Bipar Sciences Inc., of Brisbane, Calif., "didn't recruit for BRCA, and there were some questions later whether [the drug] truly was a PARP inhibitor," Hata said. "Companies that came later appreciated the importance of patient screening, and, sure enough, results were quite impressive. In ovarian cancer in particular, you essentially saw a doubling of response." (See *BioWorld Today*, April 16, 2009, and Jan. 31, 2011.)

Ideaya researchers have narrowed down the cancer types to five of particular concern: non-small-cell lung, prostate, breast, colorectal and ovarian. "Within those, there are several genetic signatures or mutations that we've identified and are interested in," Hata said, adding that the series A money will take the company into 2019, by which time two candidates will have entered the clinic.

As the synthetic-lethality medicines work on DNA damage and repair, Ideaya's immuno-oncology candidates target the tumor microenvironment, with four small-molecule programs. Hata pointed to the recently discovered "intersection" of DNA damage with immunogenicity. "We'll be at the forefront of this space," he said, noting his firm's in-house efforts in both areas and citing similar work undertaken in partnerships by the likes of Kenilworth, N.J.-based Merck and Co. Inc. and Tesaro Inc., of Waltham, Mass. At last year's meeting of the American Society of Clinical Oncology in Chicago, the pair disclosed a collaboration to test the former's anti-PD-1 drug Keytruda (pembrolizumab) paired with the latter's PARP inhibitor niraparib in a phase I/II trial in triple-negative breast or ovarian cancer. Tesaro already had started two phase III studies of its own, testing single oral doses of niraparib as a maintenance therapy for patients with ovarian cancer and as a treatment for patients with BRCA-positive breast cancer. A phase II study designed to evaluate niraparib as a treatment for patients with ovarian cancer who have received prior therapies was also ongoing. Hata pointed to Third Rock Ventures-backed Neon Therapeutics Inc., of Cambridge, Mass., as a company exploring the intersection as well. (See *BioWorld Today*, July 24, 2013, June 2, 2015, and Oct. 1, 2015.)

Regarding partnerships of its own, Ideaya has "been approached by several groups already," Hata said. "We'll be thinking about that more toward the end of the year, early next year" and the firm likely will meet with "a lot of opportunities" for tie-ups, he said. "When we'll do it and how we'll do it is still to be determined."

Ideaya is led by officials formerly with San Carlos, Calif.-based Flexus Biosciences Inc., where Hata was chief operating officer. Flexus was bought by New York-based Bristol-Myers Squibb Co. in February 2015 for up to $1.25 billion. He also served in that capacity at Flexus' spin-off, Flx Bio Inc., and has served in corporate development at Onyx Pharmaceuticals Inc., of South San Francisco, where he helped advance the multiple myeloma therapy Kyprolis (carfilzomib), before Onyx was bought by Thousand Oaks, Calif.-based Amgen Inc. for $10.4 billion. Terry Rosen, former Flexus CEO – now in that capacity at Arcus Biosciences Inc., of South San Francisco – has become a member of Ideaya's board as well. (See *BioWorld Today*, Aug. 27, 2013, Feb. 24, 2015, and Oct. 1, 2015.)

Backers of Ideaya include 5AM Ventures, Canaan Partners, Celgene Corp., Wuxi Healthcare Ventures, the Novartis Institute of Biomedical Research and Alexandria Real Estate. As part of the financing, Tim Shannon, general partner at Canaan, also joined Ideaya's board. Robert Hershberg, chief scientific officer at Summit, N.J.-based Celgene, and Edward Hu, founding partner at Wuxi, joined as board observers, which also include Rosen; John Diekman, chairman and founding partner at 5AM; Jeff Stein, CEO at San Diego-based Cidara Therapeutics Inc.; and Hannah Chang, associate at 5AM. Celgene, Hata said, invested in Flexus and Flx, and was "one of the first groups we went to as we launched Ideaya." //

OTHER NEWS TO NOTE

Axsome Therapeutics Inc., of New York, gained FDA fast track status for AXS-02 (disodium zoledronate tetrahydrate) for the treatment of the pain of knee osteoarthritis associated with bone marrow lesions (BMLs), a condition for which no product is specifically approved. The osteoclast inhibitor is being developed as an oral, targeted, non-opioid, potentially first-in-class therapeutic for chronic pain. It is being evaluated in a phase III trial for the treatment of knee osteoarthritis associated with BMLs that is governed by an FDA special protocol assessment. (See *BioWorld Today*, March 18, 2016.)