

# BIOWORLD® TODAY

WEDNESDAY  
MAY 26, 2010

THE DAILY BIOTECHNOLOGY NEWSPAPER

VOLUME 21, No. 101  
PAGE 1 OF 9

## Clovis Partners with Avila in \$209M NSCLC Deal

By Donna Young  
Washington Editor

Clovis Oncology Inc. has landed a second oncology partnership in its first year of business, this time with Waltham, Mass.-based Avila Therapeutics Inc. to develop and commercialize epidermal growth factor receptor (EGFR) mutant-selective inhibitors (EMSI) to treat non-small-cell lung cancer (NSCLC) in a deal potentially worth \$209 million.

Although it is a young company, Boulder, Colo.-based Clovis' management team has broad experience as the former executive lineup for Pharmion Corp., which was acquired in 2008 by Celgene Corp. (See *BioWorld Today*, Nov. 20, 2007, and May 22, 2009.)

Clovis' team, led by CEO Patrick Mahaffy, has "very deep experience and a successful track record" in develop-  
*See Clovis, Page 6*

*CEO: 'Game Isn't Over Yet'*

## Stem Cell Therapeutics Tumbles Following Phase IIb Stroke Miss

By Jennifer Boggs  
Assistant Managing Editor

Despite promising data from an earlier midstage trial, Stem Cell Therapeutics Corp.'s stroke drug NTx-265, a combination of human chorionic gonadotropin and erythropoietin, failed to show a statistically significant improvement over placebo in a Phase IIb trial, sending shares of the Calgary, Alberta-based firm plunging 75 percent.

The stock (TSX Venture:SSS) dropped C30 cents to close Tuesday at C10 cents.

But it wasn't that the drug didn't work. Top-line data from the 96-patient Phase IIb trial, designated REGENESIS, showed substantial improvement in patients in the NTx-265 group, with a drop of 6.3+/-0.05 as measured by the  
*See Stem Cell, Page 7*

## Neurocrine Clears Endpoint Hurdle with Elagolix Results

By Catherine Hollingsworth  
Staff Writer

Neurocrine Biosciences reported another positive Phase II study of elagolix in women with endometriosis, this time clearing a major hurdle by confirming that new study endpoints, developed with input from the FDA, were sensitive enough to detect symptom improvements.

Chris O'Brien, Neurocrine's chief medical officer, said in a conference call Tuesday that the study results showed a "very nice separation" between the elagolix-treated patients and the placebo group. That was no surprise, he said, noting that "we have seen this in every Phase II study we have conducted with elagolix, with every version of dysmenorrhea scale – monthly or daily."

Investors agreed, sending Neurocrine's shares up 24.5 percent.

*See Neurocrine, Page 8*

*Coming Thursday, Special Insert*

### Biotech's Latest Frontier: Obesity

As a value-added bonus for our readers, Thursday's edition will feature an exclusive four-page insert compiled from the new *BioWorld Obesity Report: Tipping the Market Scales with Biotech Regimens 2010*.

It's a "peek" opportunity to gain insight into one of the world's fastest-growing and most underserved disease markets and the challenges and options confronting the medical prescription market.

The consequences of an overweight society are upon us, even while the biotech industry struggles to break through a so-far impenetrable drug approval wall to address this epidemic-in-the-making.

Biotech may be late to the party, but it is working to compensate by ultimately bringing the "most wanted" therapeutics on everyone's gift list.

**INSIDE:** THERATECHNOLOGIES PLUNGES 52% ON EGRIFTA CONCERNS .....4  
EARLY LUMIZYME NOD SENDS GENZYME CLIMBING .....5



## OTHER NEWS TO NOTE

• **Agilent Technologies Inc.**, of Santa Clara, Calif., and **Stemina Biomarker Discovery Inc.**, of Madison, Wis., said they will cooperate to accelerate Stemina's metabolomic research. Stemina uses metabolomic analysis of stem cells for the discovery of biomarkers for use in drug screening and drug development. Agilent is providing a 1290 Infinity UHPLC system to separate stem cellular metabolites, coupled to a 6530 Accurate Mass quadrupole time-of-flight mass spectrometer to identify those metabolites. Agilent also is providing Mass Profiler Professional software.

• **Alkermes Inc.**, of Waltham, Mass., got priority review from the FDA for its Vivitrol (naltrexone for extended-release injectable suspension) supplemental new drug application in opioid dependence. Vivitrol, an opioid antagonist, is already approved for alcohol dependence. A decision regarding approval in the opioid dependence setting is expected Oct. 12.

• **BioAlliance Pharma SA**, of Paris, has presented efficacy, safety and biodistribution results on its new biotherapy AMEP for advanced and metastatic melanoma. Efficacy studies showed that AMEP administration in human melanoma xenograft models induced a higher tumor growth inhibition than with Temozolomide, the standard chemotherapy used in metastatic melanoma treatment. Safety and biodistribution studies showed a good overall tolerance with AMEP and a progressive elimination from tissues without accumulation. Data were presented at the annual meeting of the American Society of Gene and Cell Therapy in Washington.

• **CPEX Pharmaceuticals Inc.**, of Exeter, N.H., will not be acquired by RSR Acquisition Co., a wholly owned subsidiary of Shelter Bay Holdings LLC, after the CPEX board rejected RSR's tender offer of \$16 per share. CPEX shares (NASDAQ:CPEX) have risen more than 34 percent in the past month, and closed Tuesday at \$23.29, up 80 cents.

## Coming Thursday in *BioWorld Perspectives:*

### Biotech Is at War: BioWorld is Reporting from the Trenches

In 1990, BioWorld began operations in the era of parachute pants and the commencement of a national descent into a bad recession that would daunt the plans of an aspiring biotech market. Twenty years later, BioWorld has endured to observe and report an evolution of dynamics to designer skinny genes for obesity and a mature biotech market digging its way out of another bad recession. . . . Tune into this week's *BioWorld Perspectives* in which BioWorld Executive Editor Michael Harris discusses the past 20 years of BioWorld and biotech.

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## Stock Movers

05/25/10

Company	Stock Change
NASDAQ Biotechnology	-0.23%
Genzyme Corp.	+5.5%
Neurocrine Biosciences Inc.	+24.5%
Theratechnologies Inc.	-52.2%

*(Biotechs showing significant stock changes Tuesday)*

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*Financings Roundup***Struggling La Jolla Hops Lupus Bandwagon with \$6M Financing****By Trista Morrison  
Staff Writer**

Despite years of setbacks with lupus drug Riquent (abetimus sodium), including a Phase III failure that led to the loss of partner BioMarin Pharmaceutical Inc., La Jolla Pharmaceutical Co. isn't quite ready to throw in the towel.

The San Diego biotech is raising \$6 million through a complex private placement, and it plans to use the money both to look at partnering options for beleaguered Riquent and to evaluate in-licensing opportunities.

Riquent is designed to suppress the production of certain antibodies associated with lupus. In 1999, an interim analysis of a Phase II/III study failed to meet its primary endpoint, and original partner Abbott bailed. LJP identified an active subgroup and pushed into Phase III, but that trial failed as well. Undeterred, LJP pressed forward with a new drug application, but even good data trends couldn't save Riquent from an approvable letter. BioMarin picked up the torch during the confirmatory trial, a bet that did not pan out. (See *BioWorld Today*, Sept. 16, 1999, Feb. 19, 2003, May 6, 2003, Oct. 18, 2004, and Jan. 8, 2009.)

LJP was just one of many biotechs to fail against lupus, which hasn't seen a new drug approved in half a century. But recently, other biotechs have made some progress. Last year, Human Genome Sciences Inc.'s Benlysta (belimumab) became the first drug to succeed in a Phase III lupus trial. And ImmuPharma plc's lupus drug, Lupuzor, got good interim Phase IIb data, triggering the exercise of a \$500 million option deal with Cephalon Inc. (See *BioWorld Today*, July 21, 2009, and Nov. 26, 2008.)

Those successes sparked "renewed interest" in the best ways to conduct lupus clinical trials, said LJP President and CEO Deirdre Gillespie. She added that LJP has received some partnering interest in Riquent and in "other ideas around it," despite the drug's own rocky history.

Additionally, Gillespie said LJP is interested in acquiring some clinical products. She declined to provide specific details but noted that some companies have had a difficult time surviving the recession while others simply have too many products to develop.

LJP reported \$2.6 million in cash as of March 31 after burning about \$1.8 million in the first quarter. The firm had 66 million shares outstanding.

The new private placement will generate \$870,000 through the sale of 29 million common shares for about 3 cents each, a 50 percent discount to Monday's closing price of 6 cents. The investors also will get 5,134 shares of convertible preferred stock, which converts at 1.5 cents apiece into 342.3 million common shares, generating another \$5.1 million.

Gillespie explained that the \$5.1 million is refundable to the investors if LJP doesn't close a strategic transaction

within nine months. Yet she noted that such an outcome would leave the biotech's current investors not much worse off than they were yesterday.

The private placement also provides the investors with three-year warrants for another 5,134 shares of convertible preferred stock, but those could be exercised on a cash or cashless basis. If they are done on a cashless basis, which Gillespie said is most likely, LJP would receive no proceeds but would reduce the number of warrants issued based on the current stock price.

Additionally, the deal includes three-year warrants to purchase 10,268 shares of convertible preferred stock, which would convert into 684.5 million common shares generating \$10.3 million in additional proceeds for LJP. Exercise of the warrants is mandatory if LJP secures its strategic transaction; if not, the warrants will be redeemed. If the warrants are exercised, the investors get new warrants for another 10,268 shares of convertible preferred stock on a cash or cashless basis. All convertible preferred stock pays a 15 percent dividend.

LJP will need shareholder approval for its financing, which may prove difficult. The biotech previously tried to liquidate, but 92 percent of its largely retail investor base failed to vote on the measure, leaving the company in limbo. The same fate derailed a proposed merger with Adamis Pharmaceuticals Corp.

Gillespie said LJP has new institutional shareholders that should help increase the voting response this time around, and the firm is looking at ways to get brokers to vote on behalf of some retail investors.

Shares of LJP (OTC BB:LJPC) gained one penny, or 20 percent, to close at 7 cents on Tuesday.

In other financing news:

- **T2 Biosystems**, of New York, raised \$15 million in a Series C financing to accelerate development of its decentralized diagnostic platform. It is being designed to eliminate extensive sample preparation and enable rapid molecular and immunodiagnostic testing on a single instrument. The financing was led by Physic Ventures, with new investors Arcus Ventures, RA Capital, Camros Capital and WS Investments. Existing investors, including Flagship Ventures, Polaris Venture Partners, Flybridge Capital Partners and Partners Healthcare, also participated. The company raised \$10.8 million through a Series B round of financing in 2008. ■

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## Theratechnologies Dives 52% on FDA's Egrifta Concerns

By Donna Young  
Washington Editor

WASHINGTON – Shares of Canadian biotech Theratechnologies Inc. plunged 52 percent Tuesday after the FDA raised concerns about an increase in diabetes in HIV-infected patients given the company's investigational lipodystrophy drug Egrifta (tesamorelin acetate).

While Egrifta reduced excess abdominal fat in patients with HIV-associated lipodystrophy, a metabolic complication that affects patients taking antiretroviral therapies long term, regulators said the fat reaccumulated after the injectable drug was stopped.

Shares of Montreal-based Theratechnologies (TSX:TH) closed at C\$2.09 (US\$2.74), down C\$2.28.

There currently are no drugs approved in the U.S. to treat HIV-associated lipodystrophy, which is manifested as body composition changes, including the accumulation of abdominal fat – primarily as visceral fat – loss of extremity and subcutaneous fat and interrelated metabolic abnormalities, such as blood-lipid disorders and insulin resistance.

If approved, Egrifta would be Theratechnologies' first drug on the U.S. market.

The company, which filed its application with the FDA last spring, licensed the U.S. commercialization rights for Egrifta in October 2008 to Merck KGaA affiliate EMD Serono Inc. for \$215 million, including \$30 million up front, plus undisclosed sales royalties. The deal with EMD Serono also includes a 50-50 split of costs for late-stage development of the drug in other indications. (See *BioWorld Today*, Oct. 20, 2008, and June 2, 2009.)

Egrifta's application was based on positive Phase III data, which showed that patients with HIV-associated lipodystrophy lost 18 percent of their visceral adipose tissue (VAT) after 52 weeks of treatment, while losing 11 percent after 26 weeks of treatment, compared with baseline.

The FDA's Endocrinologic and Metabolic Drugs Advisory Committee is scheduled to review Egrifta's application at a meeting in Maryland Thursday.

In briefing documents released Tuesday ahead of the meeting, FDA drug reviewers said there was a statistically significant increase in the number of patients with diabetes in the Egrifta-treated patients relative to those who got a placebo – a finding that may affect unfavorably the potential cardiovascular benefit of the drug. In addition, regulators said there was an increase in the serum IGF-1 values above the upper range of normal in a considerable number of Egrifta-treated patients.

Given that the treatment is anticipated to be given long-term and acknowledging the fact that HIV-infected patients are at risk of non-AIDS defining malignancies, "one needs to make a determination as to what is the benefit-to-risk ratio for Egrifta in the face" of those concerns,

drug reviewers said.

The FDA also said Theratechnologies' patient-reported outcome endpoints used in the Egrifta trials to support the company's proposed indication "have questionable content validity," and therefore are unclear in supporting the interpretation of the studies' results. ■

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## OTHER NEWS TO NOTE

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- **Five Prime Therapeutics Inc.**, of San Francisco, received \$1 million in funding from Fast Forward LLC, a subsidiary of the National Multiple Sclerosis Society, to develop a preclinical MS drug that targets the innate immune system.

- **Hawaii Biotech Inc.**, of Honolulu, and **Advanced BioScience Laboratories Inc.**, of Kensington, Md., are partnering for preclinical development of a dengue vaccine. Hawaii Biotech plans to begin a company-sponsored Phase I study with its tetravalent dengue vaccine candidate later this year. Under a contract from the National Institute of Allergy and Infectious Diseases, ABL will oversee the manufacture, assembly and testing of supplies.

- **iCo Therapeutics Inc.**, of Vancouver, British Columbia, has entered into a technology transfer agreement with **Isis Pharmaceuticals Inc.**, of Carlsbad, Calif., to transfer certain technology related to the manufacture of iCo-007 to iCo in support of producing clinical grade drug product for iCo's planned Phase II clinical program. iCo will issue to Isis a warrant to purchase 235,000 shares of iCo's common stock at an exercise price of 61 cents each. The warrant will have a term of two years. The agreement enables iCo to begin the process of generating a clinical supply of iCo-007 for a planned Phase II trial in patients with diffuse diabetic macular edema.

- **Immunomedics Inc.**, of Morris Plains, N.J., received a \$5 million milestone payment from **Nycomed A/S**, of Roskilde, Denmark, under the companies' 2008 collaboration to develop and commercialize subcutaneous vel-tuzumab in all noncancer indications. Under the deal, Immunomedics could receive up to \$580 million in milestones, plus escalating double-digit royalties on product sales. In separate news, the firm received \$4.3 million in proceeds from the liquidation of one of the auction rate securities. (See *BioWorld Today*, July 16, 2008.)

- **PPD Inc.**, of Wilmington, N.C., has set the shareholder of record and distribution dates in connection with the spin-off of its wholly owned subsidiary Furiex Pharmaceuticals Inc. Shareholders of record of PPD on June 1 will receive a pro-rata dividend of one share of Furiex Pharmaceuticals common stock for every 12 shares of PPD common stock. PPD common stock will continue to trade on Nasdaq under the ticker symbol "PPDI." Shares of Furiex Pharmaceuticals will be listed on Nasdaq under the ticker symbol "FURX." After the distribution is completed, the two companies will operate as independent entities.

## Sooner-than-Expected Lumizyme Nod Sends Genzyme Climbing

By Jennifer Boggs  
Assistant Managing Editor

Genzyme Corp. won approval for Lumizyme, its 4,000L version of Pompe disease drug Myozyme (alglucosidase alfa), nearly a month before the drug's June 17 PDUFDA date, marking the first bit of good news for the firm's embattled enzyme replacement therapy franchise since last summer's contamination debacle at the Allston Landing manufacturing plant.

As Robert W. Baird & Co. analyst Christopher Raymond put it, the approval provided a "much-needed credibility boost" for the Cambridge, Mass.-based company, which has faced increasing shareholder criticism and is looking at a proxy fight with billionaire, activist investor Carl Icahn, who recently doubled his stake in the big biotech.

Genzyme's troubles date back to June 2009, when the Allston facility was shut down for six weeks to resolve viral contamination in two bioreactors. That resulted in a shortage of Gaucher drug Cerezyme (imiglucerase) and Fabry drug Fabrazyme (agalsidase beta), opening the door for competing products Vpriv (velaglucerase, Shire plc) and Uplyso (taliglucerase, Protalix BioTherapeutics Inc./Pfizer Inc.). Vpriv gained approval in February, and a nod for

Uplyso is expected later this year. (See *BioWorld Today*, June 17, 2009.)

The Allston shutdown – which was followed by an FDA inspection revealing foreign particles in a small percentage of vials produced at the plant – also delayed approval of Lumizyme 2,000L. After a second complete response letter in November citing manufacturing deficiencies at Allston, Genzyme opted to bypass the 2,000L version and seek direct approval for Lumizyme 4,000L, which is manufactured at the company's plant in Geel, Belgium. (See *BioWorld Today*, Nov. 17, 2009.)

Still, some analysts had wondered whether persistent problems at Allston, which recently earned Genzyme an official consent decree calling for a \$175 million disgorgement of past profits, would continue blocking Lumizyme's approval. (See *BioWorld Today*, April 22, 2010.)

"We sense many investors feared Lumizyme's PDUFDA date," Raymond wrote in a research note, adding that the FDA's action "removes [a] huge overhang."

Shares of Genzyme (NASDAQ:GENZ) gained \$2.68, or 5.5 percent, to close Tuesday at \$51.16.

Lumizyme, a lysosomal glycogen-specific enzyme, is approved specifically for treating patients with late-onset Pompe disease, a rare, lysosomal storage disorder characterized by progressive muscle weakness. The drug also is expected to greatly expand Pompe treatment, which has previously been limited to Myozyme.

In 2006, Genzyme gained FDA approval for Myozyme manufactured at a small 160L bioreactor, but that limited capacity meant the drug has been reserved for children and infants.

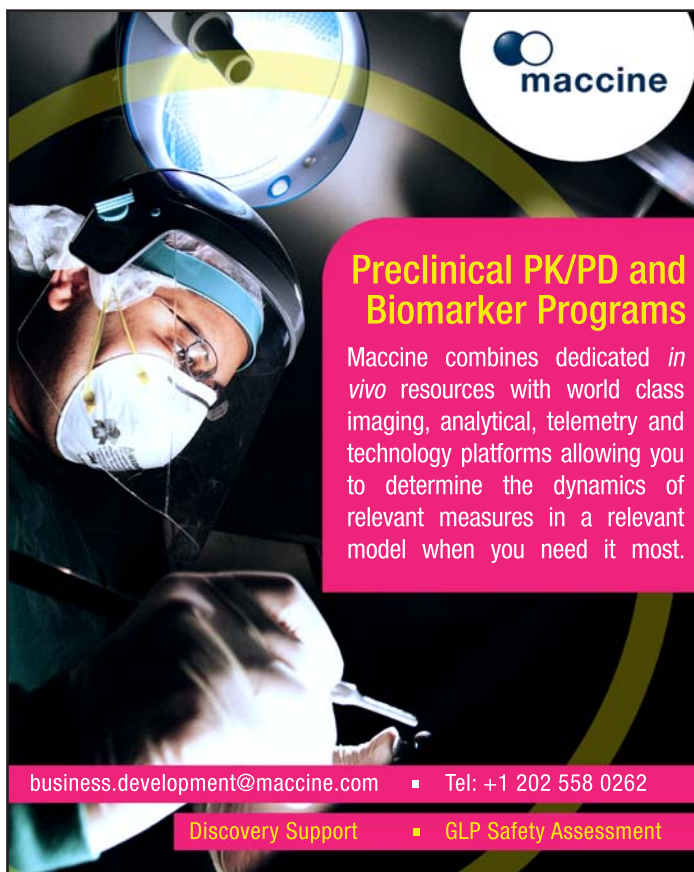
Adult Pompe patients could get access to Myozyme 2,000L, which is approved in Europe, on a compassionate-use basis.

Those make up an estimated 200 people expected to roll over to commercial therapy in the next several months, noted Brian Abrahams, analyst at Oppenheimer & Co., who has estimated 2010 sales of Myozyme/Lumizyme to reach about \$511 million.

Lumizyme is being approved with a risk evaluation and mitigation strategy and is expected to carry a boxed warning due to the risks of anaphylaxis, severe allergic reactions and immune-mediated reactions. ■

## OTHER NEWS TO NOTE

• **RegeneRx Biopharmaceuticals Inc.**, of Rockville, Md., said 19 papers on thymosin beta 4 were published in the *Annals of the New York Academy of Sciences*. The papers cover TB4's mechanism of action, preclinical data and clinical results in various indications including tissue repair and regeneration, cardiac revascularization, stroke, wound healing and multiple myeloma.



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## Clovis

*Continued from page 1*

ing and commercializing cancer drugs, said Avila CEO Katrine Bosley.

"We just have a lot of respect for that and how they operate as a team," she told *BioWorld Today*. "Clovis was very much our first choice and the most obvious partner."

Equally important, Bosley added, is Clovis' strategy and commitment to the development of companion diagnostics to ensure the right patients are identified for new cancer drugs so that the therapies can be given to the populations for whom they can be most effective.

"For this particular program, it is really important, because that's where the highest unmet need is, being able to identify these patients who have this double-mutant form of EGFR," Bosley said. "There's a very good fit and alignment of vision for what this program can do and how to develop it," she said.

Avila's EMSI program targets the T790M mutant form of the EGFR associated with clinical resistance to Genentech Inc.'s Tarceva (erlotinib) and AstraZeneca plc's Iressa (gefitinib). It also targets the initial activating EGFR mutations, including L858R and exon 19 deletions, while sparing the wild-type EGFR.

Bosley noted that EGFR inhibiting is something that Avila's founder, Juswinder Singh, "has been involved with and focused on for a long time."

Singh's work during the 1990s led to the discovery and development of some of the pan-ErbB inhibitors currently under investigation, including Pfizer Inc.'s neratinib (HKI-272), she said.

"He has been thinking about this target and this area biologically for a long time," Bosley said.

As the clinical picture matured about the role the T790M mutation plays in patients' resistance to Tarceva and Iressa, Bosley said Singh sought to solve that problem with Avila's covalent drugs, which are aimed at completely silencing disease-causing proteins by forming a durable bond intended to shut down the protein's activity.

Avila's EMSI program targeting the T790M mutation, she said, is an "excellent example of what you can do with a targeted covalent drug.

"This ability to address a resistant mutation is exactly the kind of problem that we are able to solve," Bosley added.

Because Avila's program targets both the sensitive activating mutations and T790M – the primary resistance mechanism – it has the potential to treat first- and second-line NSCLC patients with EGFR mutations, Clovis' Mahaffy said.

Under the terms of the deal, Avila and Clovis Oncology will collaborate on the preclinical development of the EMSI product candidate. Clovis will be fully responsible for all aspects of development and commercialization, including development of companion diagnostics to prospectively identify patients with clinically arising

resistance mutations of the EGFR.

In addition to research support, Avila will receive an undisclosed up-front fee and is eligible to receive development, regulatory and sales-based milestones worth potentially up to \$209 million. Avila also will receive tiered royalties on product sales and will share in selected sublicense income.

Mahaffy said the collaboration for Avila's EMSI program is "very consistent" with Clovis' \$380 million partnership with Oslo, Norway-based Clavis Pharma ASA, which the companies signed last fall. (See *BioWorld Today*, Nov. 25, 2009.)

Clovis is seeking to develop a portfolio of targeted therapies that can be developed "on a relatively accelerated basis through the use of companion diagnostics where you already know the hypothesis," Mahaffy told *BioWorld Today*.

And in both Clovis' partnerships, he said, "there's a very clear hypothesis, a very targeted therapy, and we know what the companion diagnostic will seek to identify and how we would use that during a clinical program to test that hypothesis. These are very clean programs."

Under the Clavis deal, Clovis has taken control of the clinical development for CO-1.01 (formerly CP-4126), a cytotoxic drug, which consists of gemcitabine, an anticancer nucleoside analogue, coupled to a fatty acid chain currently under investigation to treat advanced pancreatic cancer.

Last month, Clovis partnered with Ventana Medical Systems Inc. to develop a diagnostic to identify pancreatic cancer patients with the low-level tumor expression of the human equilibrative nucleoside transporter 1 (hENT1).

CO-1.01 was designed to improve on the efficacy of gemcitabine by enabling the drug to enter cancer cells without requiring uptake by hENT1.

Clovis will evaluate a "number of companies that have technologies that may be very attractive to us and commercially appropriate for us to take forward," for the Avila NSCLC program, Mahaffy said, noting that in both of his firm's deals, companion diagnostics are "critical to our development program."

Mahaffy said that had anyone told him a year ago that Clovis would have "two very important programs that address very serious unmet medical needs, with maybe the chance to really improve outcomes in the lives of a subset of patients" he would have been "more than happy then to be sitting where we are right now.

"I'm impressed with the team that we have been able to build, and we are working with two very smart, thoughtful organizations in Clavis and Avila, and I think that the partnerships themselves are going to be very productive," Mahaffy said.

He also noted that both partnerships are with biotechs, rather than big pharmas, where he said there is more of a risk of prioritization issues.

With biotechs collaborations, Mahaffy said, "our interests are more aligned," and "every drug is critical."

"We were formed to be a natural partner to biotech companies," he said. ■

## Stem Cell

*Continued from page 1*

National Institute of Health Stroke Scale (NIHSS). The problem was that the placebo worked slightly better, showing a drop of 7.3+/-0.9 NIHSS, results that surprised President and CEO Alan Moore, to say the least.

"You expect, of course, a placebo effect," he told *BioWorld Today*. "You always expect one. But we had expected a drop of 2 or 3 points at most."

Stem Cell Therapeutics will be rechecking dosages and blood levels to make sure those placebo numbers were correct. Meanwhile, it also will be analyzing results from several secondary endpoints, including the modified Rankin scale and Barthel Index. Those are just as important as the NIHSS scale and could offer clinical and regulatory paths forward if either or both yield positive data, Moore said.

"So the game isn't over yet," he added. "We're hoping one of the other endpoints will show something here."

Not that Stem Cell Therapeutics is the only firm to find stroke a particularly tough indication. Only tissue-plasminogen activator (tPA, sold as Activase by Roche AG) has gained FDA approval, but its use is limited and offers only a three-hour treatment window. Other products aimed at widening that window so far have been thwarted in the clinic, including San Carlos, Calif.-based Nuvelo Inc.'s recombinant direct-acting fibrinolytic alifimeprase and South San Francisco-based Renovis Inc.'s neuroprotectant NYX-059. (See *BioWorld Today*, Oct. 27, 2006, and Sept. 26, 2008.)

There are two Phase II-stage drugs currently looking for partners: Microplasmin, a truncated, stable form of plasmin from ThromboGenics NV, of Leuven, Belgium, and VI0152, a recombinant human thrombolytic protein from Guildford, UK-based Vernalis plc. And Rehovot, Israel-based D-Pharm Ltd. recently moved into Phase III testing with DP-b99, a drug designed to competitively bind with metal ions to inhibit metal-dependent enzymes that are overexpressed in stroke.

But Stem Cell Therapeutics' NTx-265 is different from those clotbusters and neuroprotective agents. Hailed as a neuroregenerative drug, NTx-265 aims to replace brain cells that are lost or damaged by the stroke. Given to patients one to two days post-stroke, it's designed to stimulate the growth and differentiation of existing stem cells into functional neurons and direct motor, visual and cognitive recovery.

Data from an earlier open-label Phase IIa trial were promising. NTx-265 was found to be safe and showed improved clinical outcomes compared to published data on similar untreated stroke patients, as well as a trend toward reduced infarct volumes over time in comparison to previously published data.

The Phase IIb REGENESIS trial originally had been expected to enroll 120 patients, but the study was modified

earlier this year after the firm was unable to recruit additional North American patients. The trial also had been placed on temporary clinical hold in 2008 following reports that 42 patients had died in a German study testing anemia drug Eprex (epoetin alfa, Janssen-Cilag) in stroke patients. (See *BioWorld Today*, Sept. 19, 2008.)

NTx-265 also is EPO-based, but Stem Cell Therapeutics has not seen any similar increase in mortality, Moore said. He added that further data from that German trial showed an imbalance of patients treated with tPA, so "we left tPA out" of the Phase IIb trial.

Theoretically, though, NTx-265 could be used with other agents such as clotbuster tPA, and Stem Cell Therapeutics might consider testing the two together in Phase III, he said.

An end-of-Phase II meeting is expected by the end of the year – around which time the company hopes to secure a partner for late-stage testing and commercialization – and for now, Stem Cell Therapeutics plans to keep moving the program forward.

"Everything before this said the regimen [of hCG and EPO] will work," Moore said. "So we don't think a spurious placebo effect means we should just walk away."

The company also is pursuing NTx-265 in traumatic brain injury in an ongoing Phase IIa trial. As in the stroke program, investigators again will be looking for signs of new neuron growth and direct functional recovery of motor, visual and cognitive capacity.

Stem Cell Therapeutics also has Prolactin, a regenerative treatment for multiple sclerosis. That program is set to start a proof-of-concept trial later this year.

The company, which has about C\$3.5 million (US\$3.27 million) in the bank, could pad its cash balance as warrants come due in June and October.

"That could potentially bring in a fair bit of money," Moore said, though, with only eight employees, the firm has been pretty successful in stretching its resources. Even with the full Phase IIb stroke trial ongoing, he added, the burn rate was only about \$1 million per quarter. ■

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## OTHER NEWS TO NOTE

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• **Senexis Ltd.**, of Cambridge, UK, has identified drug candidates from different chemical series that have shown potential in preclinical models as disease-modifying treatments for Alzheimer's disease. Their success secures the drawdown of a further tranche of funding from the Wellcome Trust to support preclinical development of the most promising candidates from the SENI500 series. The funds enable Senexis to initiate that planned development and file two further patents for that next generation of potentially disease-modifying Alzheimer's drugs.

## Neurocrine

*Continued from page 1*

Elagolix had shown positive results in several previous Phase II studies. But the main interest for the company in the latest study, known as Daisy PETAL, was “to show that we had a scale that could work for nonmenstrual pelvic pain,” O’Brien said. “We had to come up with a scale that performed well in this regard,” he added.

And the company did just that. The top-line data confirmed that elagolix is associated with statistically significant reductions in dysmenorrhea, nonmenstrual pelvic pain, and dyspareunia (painful intercourse) daily scores when compared to placebo.

After treatment with elagolix, patients showed a larger reduction (1.13 points) from a mean score of 2.1 at baseline, in dysmenorrhea symptoms and a reduction of 0.47 points for nonmenstrual pelvic pain (compared to a reduction of 0.37 and 0.19 points, respectively, for placebo).

At the FDA’s request, San Diego-based Neurocrine used a daily scale rather than a monthly scale to measure reductions in dysmenorrhea and nonmenstrual pelvic pain, the study’s modified co-primary endpoints. Study patients used a scale of 0-3 to rate their symptoms in a daily electronic diary.

The FDA was concerned about a statistical “floor-effect problem” with the nonmenstrual pelvic pain score, which led to the new endpoints after extensive discussions between the Neurocrine and the agency.

Given that endometriosis is challenging to treat, “we were very, very pleased that the scale shows this kind of sensitivity to change and is not plagued by a statistical floor effect,” O’Brien said. Even women with moderate-to-severe endometriosis typically have really bothersome menstrual pain only eight to nine days a months, he said.

While the elagolix study tracked dyspareunia symptoms on a monthly basis, it also had patients rate those symptoms daily, as an exploratory endpoint that also achieved statistically significant result along with the two primary pain endpoints. In addition, elagolix showed statistically significant results on secondary endpoints that scored global change in symptoms and changes in pelvic signs and symptoms over eight weeks.

The study also analyzed responder rates, showing that about two-thirds of the elagolix-treated group were responders compared to about a third for the placebo group.

Earlier this year the study completed randomization, but the six-month study is still ongoing. The company is now reporting data from the placebo-controlled, double-blind portion.

Neurocrine is now working to finalize the drafting of a special protocol assessment (SPA) request, with an anticipated filing with the FDA in late June.

Brian Abrahams, an analyst with Oppenheimer & Co., said in a research note that the company will likely need an SPA and a partner before moving forward. Long-term safety risk also remains unknown, he added. “Nonetheless, we

believe [Neurocrine] is in a far more favorable position following these data.”

Cowen & Co. analyst Phil Nadeau agreed, stating in a note, “These results should allow Neurocrine to secure a commercialization partner for elagolix, and to initiate Phase III studies by the end of 2010.”

Piper Jaffray analyst Ian Somaiya predicted elagolix could reach the market in 2013, with peak sales more than \$1 billion for the treatment of endometriosis alone.

The company also has a VMAT inhibitor for tardive dyskinesia. The next step in that program is to complete a multiple, repeated dose Phase I study in healthy male volunteers, and then initiate a proof-of-concept study in patients with tardive dyskinesia in late 2010.

It also has a CRF1 antagonist (561679) in Phase II for mood disorders that is partnered with London-based Glaxo-SmithKline plc.

Shares in Neurocrine (NASDAQ:NBIX) were up 67 cents, closing at \$3.40 Tuesday. ■

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## OTHER NEWS TO NOTE

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- **Valcor Therapeutics Inc.**, of Vancouver, British Columbia, obtained an undisclosed amount of seed financing from GrowthWorks Capital Ltd. and licensed a portfolio of dermatology products from Vancouver-based **QLT Inc.** That portfolio includes the topical photodynamic therapy Lemuteporfin for acne as well as small molecules for atopic dermatitis and vitiligo.

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## CLINIC ROUNDUP

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- **AcelRx Pharmaceuticals Inc.**, of Redwood City, Calif., reported that its ARX-02 Sufentanil NanoTab product for treating cancer breakthrough pain in opioid-tolerant patients met its primary endpoint, showing statistically significant improvement over placebo in time-weighted Sum of the Pain Intensity Difference over the first 30 minutes after dosing. In addition, secondary endpoints demonstrated that ARX-02 achieved rapid onset of analgesic efficacy. There was no statistical difference in the frequency of any class of adverse events between drug and placebo.

- **Angiotech Pharmaceuticals Inc.**, of Vancouver, British Columbia, said **Cook Medical**, of Bloomington, Ind., a license holder of Angiotech’s paclitaxel technology, presented one-year data that confirmed sustained clinical outcomes with Cook Medical’s drug-eluting peripheral stent, Zilver PTX. According to the data, 86.2 percent of all patient subgroups treated with Zilver PTX Drug-Eluting Peripheral Stent demonstrated vessel patency at 12 months without the requirement for an additional intervention.

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## CLINIC ROUNDUP

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• **Ariad Pharmaceuticals Inc.**, of Cambridge, Mass., said the independent data monitoring committee for its Phase III SUCCEED trial recommended that the study testing oral mTOR inhibitor ridaforolimus in patients with metastatic sarcomas continue to its final analysis without modification to the study protocol. The study, involving about 650 patients with metastatic soft-tissue and bone sarcomas, is expected to report final progression-free survival data in the second half of this year. While the timeline is in line with analyst expectations, some investors clearly were expecting a positive outcome following the recent interim analysis. Shares of Ariad (NASDAQ:ARIA) fell 34 cents, or 8.7 percent, to close Tuesday at \$3.58.

• **CoDa Therapeutics Inc.**, of San Diego, announced positive results from its Phase II NOVEL Study of Nexagon in patients with chronic venous leg ulcers. Nexagon achieved the endpoints of safety, reduction in wound size and complete healing after four weeks in the randomized, vehicle-controlled, double-blind, Phase II study. Based on the results, CoDa plans to initiate additional studies and has scheduled a near-term end-of-Phase II meeting with the FDA to discuss potential registration studies to support marketing approval.

• **Cytochroma Inc.**, of Markham, Ontario, reported Phase I/II data showing that a single dose of CTAPI01 capsules was safe, effective and well tolerated, and it also achieved normalization of mean blood vitamin D levels. Findings also showed that the mechanism to lower intact parathyroid hormone levels in chronic kidney disease patients with vitamin D insufficiency is more complex than simply boosting blood vitamin D levels. CTAPI01 is in development to treat secondary hyperparathyroidism associated with vitamin D insufficiency in nondialysis CKD patients. Based on the Phase I/II data, Cytochroma said it plans to conduct a larger, repeat-dose Phase II study as soon as possible.

• **Eyetech Inc.**, of Palm Beach Gardens, Fla., reported data from a 568-patient exploratory study starting with patients treated one to three times for neovascular age-related macular degeneration, primarily with a nonselective VEGF inhibitor such as Lucentis (ranibizumab, Genentech Inc./Roche AG), with improved mean visual acuity by 15.9 letters. After entering the study, patients were switched to Macugen (pegaptanib sodium), a selective VEGF inhibitor, and at the end of a 54-week maintenance phase, mean final visual acuity was 61.8 letters. From the start of the induction phase to the end of the maintenance phase, 41 percent of patients gained at least three lines of visual acuity. Those data were published in the *British Journal of Ophthalmology*.

• **Nymox Pharmaceutical Corp.**, of Hasbrouck

Heights, N.J., reported new data from its 30-month to 36-month follow-up study of patients treated with NX-1207, which showed no significant drug safety problems reported by subjects. The initial study, completed in 2007, reached statistical significance in intent-to-treat primary efficacy outcomes at 90 days and six months post-treatment, and the new data showed that more than 50 percent of patients receiving NX-1207 required no further medical or surgical treatments for their benign prostatic hyperplasia in the long-term follow-up period. Patients had a mean improvement of 11.8 points in the symptom scores.

• **Oncolytics Biotech Inc.**, of Calgary, Alberta, has opened enrollment in its Phase III trial examining Reolysin in combination with paclitaxel and carboplatin in patients with platinum-refractory head and neck cancers. The company had previously received approval from the FDA under the special protocol assessment process and the UK Medicines and Healthcare products Regulatory Agency to conduct the trial in those countries, respectively. Oncolytics intends to conduct the first stage of the trial at approximately 25 centers in the U.S., UK and Belgium but may elect to add centers in additional countries. The primary endpoint is overall survival, and secondary endpoints include progression-free survival, objective response rate and duration of response, and safety and tolerability.

• **Shire plc**, of Basingstoke, UK, presented data showing Vyvanse (lisdexamfetamine dimesylate) Capsules CII for the treatment of attention deficit hyperactivity disorder (ADHD) in adolescents ages 13 to 17 significantly improved ADHD symptoms compared to placebo. Vyvanse is currently approved for the treatment of ADHD in children ages 6 to 12 years and in adults. Shire has submitted a supplemental new drug application for use of Vyvanse in the treatment of adolescents ages 13 to 17 years with ADHD. Data were presented at the American Psychiatric Association meeting in New Orleans.

• **Trigemina Inc.**, of Sunnyvale, Calif., reported positive results from a time-based interim analysis of its Phase IIa trial in chronic daily headache. To date, 25 patients refractory to available medication have been analyzed after intranasal oxytocin or placebo in a double-blind, parallel trial. The data showed 47 percent of oxytocin-treated patients vs. 11 percent of placebo patients reporting at least a 50 percent reduction in pain scores.

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