

BIOWORLD® TODAY

TUESDAY
MARCH 29, 2011

THE DAILY BIOTECHNOLOGY NEWSPAPER

VOLUME 22, No. 60
PAGE 1 OF 8

Financings Roundup

Anergis Picks up \$19.6M in First VC Round Since Founding

By Nuala Moran

BioWorld Today Correspondent

LONDON – Anergis SA raised CHF18 million (US\$19.6 million) in a Series A round to fund the development of allergy vaccines based on synthetic versions of natural allergens, which it said will be able to induce tolerance more quickly than is the case with de-sensitizing through the long-term administration of natural allergens.

CEO Vincent Charlon said the financing will last two years. "We will now conduct a large Phase II multicenter trial with AllerT, our lead product for patients allergic to birch pollen, and we will speed up development of our preclinical product portfolio," Charlon said.

The financing was co-led by three European funds. Lausanne, Switzerland-based Anergis was founded in 2001 and has previously raised CHF3.3 million from private

See Financings Roundup, Page 3

From NIH to ATM

Alternative Going Mainstream? Biotechs Find Funding Options

By Jennifer Boggs

Assistant Managing Editor

Editor's note: This is a special reprint from the March 7, 2011, issue of BioWorld Insight, the weekly analytical companion to BioWorld Today.

If the latest economic downturn has taught anything at all, it's that there will always be more than one way to fund a biotech company.

Whether a firm is just starting out, armed with a newly in-licensed compound or promising science, or has been around for several years and edging closer to market, finding adequate funding sources takes a bit more digging than it used to. Even 10 years ago, a biotech could travel the well-worn business pathway – secure seed funding, attract a few rounds of venture investment, complete an initial public offering and then return to the

See Funding, Page 4

What's in a Name?

Cancer Stem Cell Model Holds in Ovarian Cancer, with Twists

By Anette Breindl

Science Editor

Considerable hope rides on the idea that targeting cancer stem cells may be the clinical key to lasting success against relapse.

Cancer stem cells, so the cancer stem cell theory goes, are the malignant version of regular stem cells, which retain the ability to divide and fuel tumor growth indefinitely.

In that view, the reason that some cancer drugs are initially successful at killing off tumors, but ultimately unsuccessful at preventing relapse, is that cancer stem cells are not targeted by chemotherapy, and so live to fight another day after treatment. Target the stem cell, the thinking goes, and tumors can actually be truly eradicated.

A new study, however, suggested that at least as far as ovarian cancer is concerned, those hopes should be

See Cancer Stem Cells, Page 6

Biotech Foundations

MMRF Partners with Biotech to Speed Cancer Therapies R&D

By Catherine Shaffer

BioWorld Today Contributing Writer

Not satisfied with merely providing early stage research support to fight cancer, the Multiple Myeloma Research Foundation has an "end-to-end" strategy for fostering drug development at every stage. The MMRF, headquartered in Norwalk, Conn., has served as midwife to four breakthrough therapies for multiple myeloma: Velcade, Thalomid, Revlimid and Doxil.

The foundation is shepherding another 15 therapies through clinical trials, either through its Biotech Investment Awards program or by collaborative support for clinical trials through its research consortium. Altogether the MMRF invested about \$15 million in research in 2010,

See Biotech Foundations, Page 7

INSIDE:

OTHER NEWS TO NOTE: ACTIVE SITE, ADEONA, ALNYLAM, ARIAD2
APPOINTMENTS AND ADVANCEMENTS: CUBIST, ENZON, GENESIS, HOSPIRA8



Other News To Note

• **ActiveSite Pharmaceuticals Inc.**, of Berkeley, Calif., reported the results of a study that showed the effectiveness of its plasma kallikrein inhibitor ASP-440 in reducing blood-retinal barrier breakdown in a rodent model of diabetes. The gradual buildup of fluid in the retina from leakage, caused by the breakdown of the blood-retinal barrier, can result in diabetic macular edema (DME), the primary cause of vision loss in individuals with diabetes. No FDA-approved drugs are available to treat DME, which affects more than 1 million people in the U.S. The study was published in *Diabetes*.

• **Adeona Pharmaceuticals Inc.**, of Ann Arbor, Mich., announced that the ongoing clinical trial of its Trimesta (oral estriol) drug candidate has received an additional \$409,426 in grant funding from the National Multiple Sclerosis Society. The 150-patient, randomized, double-blind, placebo-controlled clinical trial of Trimesta evaluating the reduction in the rate of relapses in female multiple sclerosis patients is currently under way at 15 centers in the U.S. and as of March 1 is 85 percent enrolled.

• **Alnylam Pharmaceuticals Inc.**, of Cambridge, Mass., reported that it has earned a \$10 million technology transfer payment from **Takeda Pharmaceutical Co. Ltd.**, of Osaka, Japan, as part of companies' May 2008 strategic alliance related to the transfer of Alnylam's platform technology to Takeda for the development of RNAi therapeutics. Alnylam had previously received \$140 million in up-front and technology transfer payments from Takeda. Alnylam also is eligible to receive significant milestones and royalties related to Takeda's successful advancement of RNAi therapeutic products, and has retained certain product opt-in rights in the U.S. market. The Alnylam-Takeda alliance has a potential value of more than \$1 billion.

• **Ariad Pharmaceuticals Inc.**, of Cambridge, Mass., will collaborate with **MolecularMD Corp.**, of Portland, Ore., to develop and commercialize a companion diagnostic test for the T315I mutation of the BCR-ABL gene in patients with chronic myeloid leukemia and Philadelphia positive acute

Stock Movers

03/28/11

Company	Stock Change	
Nasdaq Biotechnology	-\$2.80	-0.3%
Alexza Pharmaceuticals Inc.	+\$0.14	+9.2%
Aastrom Biosciences Inc.	+\$0.41	+18.6%
Response Genetics Inc.	+\$0.15	+7.2%
Rexahn Pharmaceuticals Inc.	-\$0.34	-24.1%
Vical Inc.	+\$0.22	+8.8%

(Biotechs showing significant stock changes Monday)

lymphoblastic leukemia. Ariad is developing a pan-BCR-ABL inhibitor, ponatinib, for those diseases. MolecularMD will optimize its sequencing test for the T315I mutation, and file a premarket approval application in conjunction with Ariad's new drug application filing, anticipated some time in 2012.

• **Cellceutix Corp.**, of Beverly, Mass., signed a confidential disclosure agreement (CDA) with an undisclosed pharmaceutical company regarding its autism candidate KM-39I. Cellceutix said that while the CDA does not guarantee an impending licensing deal, it is a rare vote of confidence in favor of an early stage therapeutic. Cellceutix also reported that its formulation manager, **Formatech Inc.**, of Andover, Mass., completed production on Kevetrin, on schedule for filing its investigational new drug application with the FDA in May.

• **Curasan AG**, of Kleinostheim, Germany, received European approval for Osseolive, a synthetic bone regeneration material designed for the treatment of large defects, in combination with bone growth-supporting substances, such as bone marrow aspirate, stem cells and bone morphogenetic proteins. The bioactive, polycrystalline calcium-alkali-phosphate ceramic has a stimulating effect on bone formation and mineralization.

BioWorld® Today (ISSN# 1541-0595) is published every business day by AHC Media, 3525 Piedmont Road, Building Six, Suite 400, Atlanta, GA 30305 U.S.A. Opinions expressed are not necessarily those of this publication. Mention of products or services does not constitute endorsement. BioWorld® and BioWorld® Today are trademarks of AHC Media, a Thompson Publishing Group company. Copyright © 2011 AHC Media. All Rights Reserved. No part of this publication may be reproduced without the written consent of AHC Media. (GST Registration Number R128870672).

ATLANTA NEWSROOM: Managing Editor: **Lynn Yoffee**. Assistant Managing Editor: **Jennifer Boggs**. Senior Staff Writer: **Karen Pihl-Carey**. Senior Production Editor: **Ann Duncan**. Staff Writer: **Tom Wall**.

WASHINGTON BUREAU: Washington Editor: **Mari Serebrov**.

WEST COAST BUREAU: Staff Writer: **Trista Morrison**.

EAST COAST BUREAU: Science Editor: **Anette Breindl**.

BUSINESS OFFICE: Senior Vice President/Group Publisher: **Donald R. Johnston**. Director of Product Management: **Jane Cazzorla**. Marketing Manager: **Sarah Cross**. Account Representatives: **Matt Hartzog, Chris Wiley, Scott Robinson**.

DISPLAY ADVERTISING: For ad rates and information, please call **Stephen Vance** at (404) 262-5511 or email him at stephen.vance@ahcmedia.com.

REPRINTS: For photocopy rights or reprints, call our reprints department at (404) 262-5479.

PRESS MATERIALS: Send all press releases and related information to newsdesk@bioworld.com.

SUBSCRIBER INFORMATION

Please call **(800) 688-2421** to subscribe or if you have fax transmission problems. Outside U.S. and Canada, call **(404) 262-5476**. Our customer service hours are 8:30 a.m. to 6:00 p.m. EST.

Lynn Yoffee, **(404) 262-5408**
Jennifer Boggs, **(404) 262-5427**
Anette Breindl, **(518) 595-4041**
Trista Morrison, **(858) 901-4785**
Mari Serebrov, **(703) 678-7376**
Tom Wall, **(404) 262-5417**

Senior Vice President/Group Publisher:
Donald R. Johnston, **(404) 262-5439**
Internet: <http://www.bioworld.com>



Financings Roundup

Continued from page 1

investors and a grant from the Swiss Innovation Fund. This first formal round of venture capital funding will bring in representatives of each of the VCs.

The board of the company is chaired by André Mueller, who has previous biotech experience with Biogen Idec Inc., Actelion Ltd., Addex Pharmaceuticals Ltd. and Cerenis Therapeutics SA.

The technology underpinning the company, contiguous overlapping peptides (COP), was initially developed at Lausanne University, the Federal Institute of Technology in Lausanne and Centre Hospitalier Universitaire Vaudois. COP makes it possible to reproduce fragments of the amino acid sequence of specific allergens in single peptide-like molecules. Anergis said they present all the T-cell epitopes of the original allergen, but do not cross-react with allergen-specific IgE antibodies, which are responsible for eliciting hypersensitivity to allergens.

That makes it possible to administer COPs at far higher doses than is the case in existing desensitization treatment, which may require administration of an allergen multiple times, over several years. Animal and human studies of two COPs, AllerT against birch pollen and AllerB against bee stings, have shown a good safety profile with no immediate allergic reaction and the production of antibodies and cytokines specific to the natural allergen

The company said that within a few days it is possible to administer about 10 times the amount usually reached after months of progressive increase in the dose in classical desensitization. In six to 10 weeks the dose of AllerT or AllerB reaches levels that typically take three to five years for traditional products.

In the Phase I/IIa study of AllerT, which reported in July 2009, patients were treated before the allergy season with five injections of AllerT or placebo over two months.

Birch pollen counts measured by the Lausanne Station in 2009 were very high during a relatively short period of eight to 10 days. The peak of allergic symptoms in the placebo patients occurred at week three of data collection, and there was a pronounced trend in favor of AllerT-treated subjects.

The company said the trial demonstrated that it was safe to administer high doses of AllerT and that doing that just before the birch pollen season resulted in an improvement of symptoms and quality of life.

Anergis is stepping onto the stage as the European market for allergy vaccines is in flux. To date, most desensitizing products have been available on a named patient basis, but the rules have been changed recently to require allergy vaccines to be formally registered. Many products are marketed by small specialists companies that serve national markets, and it is not economic to comply with that requirement.

Following behind AllerG and AllerB, Anergis is working on vaccines for house dust mite allergy and peanut allergy.

In other financing news:

- **Helix BioPharma Corp.**, of Aurora, Ontario, netted about \$3.9 million in a private placement, of 1.6 million units of common stock and warrants at \$2.39 per unit. Each unit consists of one common share and one purchase warrant to purchase one common share at \$3.35 within five years. Proceeds will support the company's development programs.

- **Rexahn Pharmaceuticals Inc.**, of Rockville, Md., received commitments from institutional investors to purchase an aggregate of \$10 million of Rexahn's securities in a registered direct offering. The firm agreed to sell about 8.33 million shares of stock and warrants exercisable for up to about 3.33 million additional shares in units priced at \$1.20 each. Rexahn anticipates about \$9.45 million in net proceeds, which will be used to support further work on lead programs, including Phase II studies for Serdaxin, Zoraxel and Archexin, and other general corporate purposes. Rodman & Renshaw LLC acted as exclusive placement agent. ■

NEED FASTER DATA?



In just 3 business days, we'll take you from database lock to topline analysis.

SDC delivers leading-edge biostatistics and data management services, providing our clients with total quality assurance, innovation, and rapid study execution from proof-of-concept through post-approval.



www.sdclinical.com
data@sdclinical.com
 480-632-5468 ext. 204

BioWorld is now on Twitter!
Stay Connected, Follow Us on Twitter!
www.twitter.com/bioworld

Funding

Continued from page 1

capital markets to raise money on the back of each clinical and regulatory accomplishment.

But now, venture capital is getting scarcer. *BioWorld Today* columnist Cynthia Robbins-Roth calculated a 27 percent drop in venture funding between 2009 and 2010 (\$2.75 billion vs. \$2 billion). Last year's numbers even fell below the \$2.3 billion raised in tumultuous 2008. And the biggest drop was seen in initial venture rounds, with Series A deals bringing in \$479 million for biotech last year compared to \$849 million in 2009. (See *BioWorld Today*, Jan. 26, 2011.)

The capital markets haven't exactly been kind, either. Despite 25 percent fewer deals, follow-on public offering in 2010 raised less money than the year before, \$5.6 billion vs. \$6.5 billion in 2009, and most firms have been forced to offer discounts or take on further dilution.

"The new reality is that life sciences may need to rethink their capital-raising strategy," said Todd Wyche, founder of investment banking boutique Brinson Patrick Securities.

He was referring to public fundraising, but it's clear that biotechs, both private and public, are reaching more and more into the alternative financing bag of tricks.

Preferring Not to 'Venture' Out

Ask many industry experts and they'll agree that the VC market is expected to contract, especially as many funds reach their decade mark without having generated sufficient returns. Investors also are having to put more money into existing portfolio firms just to get those companies to an exit, either through an initial public offering or M&A.

That's bad news for emerging companies. And so far this year, attracting Series A financings is not looking much better. According to *BioWorld Snapshots*, there were four designated Series A rounds to close in January and February, bringing in a total of \$54.3 million. (Dartis Pharmaceuticals Inc.'s Series A was excluded because the amount was not disclosed.) Compare that to the first two months of last year, when a total of seven firms closed Series A rounds in January and February and raised a total of \$119 million.

Plus, most of this year's Series A money stemmed from just two offerings: PanOptica Inc., which brought in \$30 million to support work on a non-injectable option for the hot age-related macular degeneration space, and Alkermes Inc. spinout Civitas Therapeutics Inc., which raised \$20 million. (See *BioWorld Today*, Jan. 4, 2011, and Jan. 10, 2011.)

But many firms have found success from other quarters. Organizations like the Michael J. Fox Foundation for Parkinson's Research, have helped bridge the funding gap. And there's the National Institutes of Health (NIH), which could increase its funding for basic and applied

biomedical research by \$740 million in fiscal year 2012 if the Obama administration has its way.

Many early stage firms already rely heavily on NIH grants. In fact, one 2008 start-up has funded its work almost entirely with NIH money. San Diego-based Renova Therapeutics Inc. has brought in about \$23 million to support work on its cardiovascular gene therapy program. It's pulled in a bit more through angel investments and the qualifying therapeutic discovery tax grant.

Renova has no VC money in the firm, and CEO Jack Reich told *BioWorld Today* last year that he has no plans to seek venture capital, preferring to "see the people who work at the company reap the benefits of a stock sale." (See *BioWorld Today*, Dec. 22, 2010.)

He's not alone. Recent start-up Dance Pharmaceuticals Inc. also has avoided venture capital by choice. The San Francisco-based firm, which is working on an inhaled insulin program, aims to fund some of its R&D activities by carving out rights to its product in multiple regional partnerships. (See *BioWorld Insight*, Jan. 24, 2011.)

There's also a dual drug development/service model. Executives at Birmingham, Ala.-based DiscoveryBioMed Inc., facing a recession shortly after being founded in 2007, employed that two-pronged strategy, running a contract research organization on one side of the business to

See Funding, Page 5

Innovative Drug Pipeline Solutions

Do You Want Compounds Or Do You Want Drugs?

SCYNEXIS delivers innovative and integrated drug discovery and development solutions to our pharmaceutical and global health partners. Our success is demonstrated by the 11 pre-clinical and clinical drug candidates that we have delivered over the last 5 years including SCYNEXIS' anti-HCV clinical compound, SCY-635, from our proprietary cyclophilin inhibitor technology platform. We offer our expertise on a fee-for-service basis or in a shared-risk arrangement.



From Concept To Clinic With Speed And Innovation™

www.scynexis.com

Funding

Continued from page 4

generate revenue for its pipeline programs in respiratory, metabolic, inflammatory and hyperproliferative disease. (See *BioWorld Today*, Nov. 5, 2010.)

While it doesn't happen often, a few firms have skirted the entire VC game by going public right away. Genesis Biopharma Inc., which is developing a CD55-targeting monoclonal antibody from failed biotech Viragen Inc., kicked off its operations last year by completing a reverse merger with public shell Freight Management Co. and gaining a listing on the Over-the-Counter Bulletin Board.

Adding to the Financing 'Arsenal'

Yet, while having access to the public markets certainly makes accessing cash easier, the toll of discounts and dilution from traditional public offerings and PIPEs can be hard on a company's stock. Shares of Worcester, Mass.-based RXi Pharmaceuticals Inc., for example, sank recently when the firm priced an offering of stock and warrants at \$1.35 per unit, 27 percent below the previous day's closing price.

"That erosion of value can affect the whole company, and it affects the cost of raising capital," said Brinson Patrick's Wyche, whose firm works specifically with companies to raise money through at-the-market (ATM) offerings, designed to avoid the stock decline associated with traditional public offerings of late.

Since Brinson conducted the first life sciences ATM offering in 2006 for Avanir Pharmaceuticals Inc., ATMs have become more frequent. In the 2008-2009 period, there were only nine ATM deals in the life sciences industry, but in 2010, there were 26, Wyche told *BioWorld Insight*. Between the years 2001 and the second quarter of 2010, his firm showed that life sciences companies raised \$247 million in total in ATM offerings.

True, an ATM might not work for a company that needs a large amount of capital within a short time frame, "but if companies are strategic in terms of planning, [ATMs] should definitely be in their arsenal," Wyche said. "You can raise 10 to 15 percent of your trading volume without adversely affecting stock price."

Less often seen in biotech – at least in the U.S. – are rights offerings. Though it takes longer to pull together compared to a traditional public offering or PIPEs, rights offerings also can help avoid substantial dilution. But there are risks, namely worries that a lack of sufficient interest by existing investors might leave too many shares on the table.

Only a few U.S. firms have pulled off a rights offering in recent years. Sunnyvale, Calif.-based Pharmacyclics Inc. brought in proceeds of about \$28.8 million in a rights offering in 2009, along with BioMimetic Therapeutics Inc., of Franklin, Tenn., which raised about \$17 million. (See *BioWorld Today*, July 17, 2009.)

Recently, Adherex Technologies Inc., of Chapel Hill, N.C., said it is trying to raise \$12.9 million through a rights offering of its own.

Beyond the capital markets, companies also increasingly are looking at selling royalty streams for cash to advance programs.

Royalty monetization, once a tool for cash-strapped biotechs, has become a way for even established firms to pad their balance sheets. And private equity-backed royalty deals continue to pick up steam. (See *BioWorld Insight*, Nov. 1, 2010.)

In today's economic environment, there's no single source for funding, and the smart firm is one that makes sure it has multiple fund-raising options, especially since a clinical or regulatory setback could put it in an unenviable position if it has to raise money through traditional means.

"We're starting to see a shift in financing strategy," Wyche said. "It's not just about getting to the next milestone, but getting well past that and [finding] multiple ways of accessing capital." ■

Other News To Note

• **CytRx Corp.**, of Los Angeles, will present results from preclinical studies of its oncology drug candidate bafetinib in inhibiting bone destruction at the annual meeting of the American Academy for Cancer Research in Orlando, Fla., April 2. CytRx also is evaluating bafetinib in three ongoing clinical trials: a pharmacokinetic trial for recurrent brain tumors, the ENABLE Phase II trial for high-risk B-cell chronic lymphocytic leukemia and the PROACT Phase II advanced prostate cancer trial.

• **Nycomed GmbH**, of Zurich, Switzerland, applied for European marketing authorization for teduglutide as a once-daily subcutaneous treatment for short bowel syndrome. The submission triggers a mid-seven-digit milestone payment to **NPS Pharmaceuticals Inc.**, of Bedminster, N.J. NPS is developing teduglutide in the U.S. under the brand name Gattex and plans to submit a marketing application to the FDA later this year. Nycomed licensed the rights to develop and commercialize the drug outside of North America. (See *BioWorld Today*, Feb. 1, 2011.)

• **Spark Acquisition Corp.**, a wholly owned subsidiary of Quest Diagnostics Inc., of Madison, N.J., commenced a tender offer for all outstanding common stock in **Celera Corp.**, of Alameda, Calif. The offer is pursuant to an offer to purchase, made on March 28, and connected to a previously announced agreement and plan of merger on March 17.

• **Yuma Therapeutics Corp.**, of New York, received a \$249,810 grant from the Alzheimer's Drug Discovery Foundation to develop small molecules against Alzheimer's. The award will fund work to develop Yuma's disease-modifying compounds designed to target neurofibrillary tangles resulting from abnormal forms of tau protein.

Cancer Stem Cells

Continued from page 1

tempered with a good dash of realism about just how complex cancer stem cells are.

In their work, which appeared in the March 28, 2011, online edition of the *Proceedings of the National Academy of Sciences*, the authors looked at tumor-initiating cells from both primary ovarian tumors and metastases, to see whether they possessed the features of true cancer stem cells.

The answer: sort of.

The terms tumor-initiating cells and cancer stem cells are sometimes used interchangeably, co-corresponding author Benjamin Neel told *BioWorld Today*. But “it’s our contention that that’s erroneous.”

What clearly exists are tumor-initiating cells. The definition of a tumor-initiating cell, Neel said, is “operational” – that is, cells that can keep dividing and start up a new tumor.

But that alone, Neel said, is not enough to make a tumor-initiating cell a stem cell: “The crux of the matter of whether tumor-initiating cells are stem cells is in the nature of the tumor-initiating cell.”

The question, he said, is whether there is something about certain cells that makes them identifiable as cells that have the capacity to keep going indefinitely – the view championed by the cancer stem cell model. Neel likened cancer stem cells to queen bees, which are the only ones who can repopulate a colony regardless of how many worker bees there are.

In the alternative view, the so-called stochastic model, any cell can, in principle, become a tumor-initiating cell, if it acquires the right genetic mutations.

Neel, who is the director of the Ontario Cancer Institute, and his colleagues looked for (and at) tumor-initiating cells in samples from ovarian tumors and their metastases. They took nearly 140 samples from ovarian cancer patients, removed leukocytes from them and transplanted them into mice to see whether they contained tumor-initiating cells that could seed new tumors.

They found that cells from primary cells contained tumor-initiating cells that keep their abilities through several passages, or rounds of splitting up the samples. However, a true cancer stem cell has to be identifiable before the fact, so the team next looked at supposed cancer stem cell surface markers to see whether their tumor-initiating cells displayed them.

The bottom line, the authors wrote, was that the stem cell model worked. By using the cell surface marker CD133, they were able to “enrich” tumor-initiating cells from the primary tumors.

But a cell decorated with CD133 was not a sure thing as a cancer stem cell.

Co-corresponding author Laurie Ailles, a scientist at the Ontario Cancer Institute, told *BioWorld Today* that CD133

was expressed on tumor initiating cells “a large percentage of the time . . . but it wasn’t an absolute result.”

Ailles, Neel and their team also found some tumor-initiating cells that did not express CD133. To further complicate matters, when CD133-expressing cells were transplanted into animals, in some cases their descendants retained their tumor-initiating abilities – but no longer expressed CD133.

There could be several reasons for why CD133 is not always expressed on tumor-initiating cells. The simplest one is that there are several types of cells that can become tumor-initiating cells.

But Neel does not think that Occam’s Razor (the idea that the simplest explanation for a phenomenon is usually correct) holds in this case. It is “more likely,” he said, that the initial tumor-initiating cells express CD133, but lose that expression later on. CD133, he elaborated, is purely a marker cell and “not important functionally” for the cells, so a cell that stops expressing CD133 does not lose any stem-cell-like properties it had.

Whatever the reason for their surface markers or lack thereof, the findings are mixed news for the possibilities of getting any practical use out of cancer stem cells.

On the one hand, there does indeed appear to be a specific population of cancer stem cells, and so those cells can be studied in more detail and perhaps, in time, be targeted therapeutically.

But the paper’s title – “Phenotypic heterogeneity and instability of human ovarian tumor-initiating cells” – sums up the findings of the report rather well. And that heterogeneity and instability, Neel said, may make true cancer stem cells “very difficult . . . to identify.” ■

Clinic Roundup

• **Addex Pharmaceuticals Ltd.**, of Geneva, and Ortho-McNeil-Janssen Pharmaceuticals Inc., a unit of New Brunswick, N.J.-based **Johnson & Johnson**, started a Phase IIa trial of ADX71149 in schizophrenia. The 105-patient study will involve two parts: Part A will treat 15 subjects with ADX71149 as a monotherapy, while Part B will test the drug as an add-on therapy in 90 subjects with residual positive symptoms or predominant negative symptoms or in subjects with insufficient response to clozapine. Endpoints will examine tolerability, safety and efficacy. Under the companies’ 2005 licensing deal, Addex will receive a \$2.8 million milestone payment.

• **Bionovo Inc.**, of Emeryville, Calif., started enrollment in a Phase I trial of Menerba in postmenopausal women for the treatment of hot flashes. The open-label study will enroll about 40 women who will be randomized to one of two doses of Menerba and treated for 28 days. The primary goal is to assess the safety of the drug after four weeks of treatment.

Biotech Foundations

Continued from page 1

and expects that number to increase to \$19 million to \$20 million in 2011.

"We at the foundation have played a key role with the rest of the community. There truly is a collaborative spirit, with us in the center of it as a foundation as well as a patient community," MMRF Vice President of Communications Anne Quinn Young told *BioWorld Today*.

MMRF's research support spans the entire life cycle of an investigational therapy, with heavy emphasis on Phase I and II trials through the Multiple Myeloma Research Consortium. Founded by Kathy Guisti, a myeloma patient, the MMRC's mission is to speed up the development of new therapies by facilitating research, especially clinical trials.

The consortium currently counts as members 16 academic institutions that are leaders in the area of myeloma research, including City of Hope, Mayo Clinic, the University of Michigan and many others.

The MMRC supports clinical trials through its 16 academic research centers. To date, it has advanced 30 clinical trials with industry partners such as Novartis AG, Merck and Co. Inc., Celgene Corp. and Onyx Pharmaceuticals Inc.

The MMRC reported that clinical trials carried out within its network opened 60 percent faster than the industry benchmark in oncology. The trials completed enrollment 14 percent earlier than usual and enrolled 25 percent more patients. That time savings is a significant benefit to MMRC's biotech partners. "Closing a trial two months early was certainly meaningful," Young said.

The MMRF and MMRC facilitate clinical trials by providing funds and research collaboration as well as access to its network of academic research centers and its experience in navigating myeloma therapeutics through the regulatory process.

"We're quite proud not only of speed metric and opening trials, but the quality as well. Right now in myeloma, we have five, soon to be seven, drugs in Phase III," Young added.

One of the foundation's earliest and biggest successes was the approval of Thalomid (thalidomide). Thalidomide was marketed briefly in the 1950's as a sedative, but was withdrawn when it was found to cause severe birth defects when given to pregnant women.

In 2006, the FDA granted accelerated approval for Thalomid to Celgene, based on trials that showed greater than 50 percent reduction in myeloma protein levels for patients receiving Thalomid and dexamethasone plus chemotherapy, compared to patients receiving chemotherapy alone.

The introduction of thalidomide improved the standard-of-care treatment for multiple myeloma for the first time in decades. The related family of immunomodulatory (IMiD) drugs, including Revlimid and Velcade, became a new generation of myeloma therapies.

The MMRF is now focusing on the pipeline of newer

drugs that build on the success of the ImiDs.

In January, the MMRF partnered with Synta Pharmaceuticals Corp. to develop its Hsp90 inhibitor ganetespib (STA-9090). The foundation will provide up to \$1 million in funding for Phase I trials of ganetespib as a single agent and in combination with bortezomib for relapsed multiple myeloma.

The MMRF has a partnership with Intellikine Inc. that focuses on the PI3K/mTOR pathway. Intellikine recently initiated Phase I trials of its TORC1 and TORC2 inhibitor, INK128, with the support of a \$1 million Biotech Investment Award.

Boston area companies Constellation Pharmaceuticals Inc., Epizyme Inc. and Karyopharm Therapeutics Inc. received similar \$1 million awards in January. Constellation and Epizyme are developing epigenetics-based therapies, and Karyopharm is working on nuclear import and export of tumor suppressor and growth regulatory proteins.

The MMRF is seeing progress in its late-stage sponsored projects as well. Onyx Pharmaceuticals Inc. recently received fast-track designation for carfilzomib, a proteasome inhibitor, for relapsed and refractory multiple myeloma, following positive results from a Phase IIb trial. Onyx initiated a rolling submission of a new drug application, with the goal of completing the submission by the middle of 2011. (See *BioWorld Today*, Dec. 8, 2010.)

The impact of the MMRF can be measured in patient survival as well as biotech dollars funded. "A few years ago, when a patient was diagnosed, that patient might have been given two or three years to live," Quinn said. "What we've done is double survival. Patients . . . are living about seven years."

Editor's note: Biotech Foundations is a BioWorld Today column that recognizes biotech-related nonprofit organizations, as well as those founded by biotech industry luminaries. Tell us about your nonprofit at newsdesk@bioworld.com. ■

ADVERTISE HERE

...and reach high-level
biotechnology professionals every day!

For advertising opportunities in
BioWorld Today, please contact
Stephen Vance at (404) 262-5511
or stephen.vance@ahcmedia.com

Clinic Roundup

• The FDA has cleared an investigational new drug request from **Evolva Holding SA**, of Reinach, Switzerland, paving the way for a human trial of EV-077 to treat influenza. In preclinical testing, the candidate showed potential as prophylaxis and treatment of various viral infections, according to the company. Evolva is in discussions with potential partners to further develop and market the dual thromboxane receptor antagonist and thromboxane synthase inhibitor. EV-077 also is being developed to treat diabetes complications and as a biodefense measure.

• **Spectrum Pharmaceuticals Inc.**, of Irvine, Calif., said the independent data monitoring committee recommended that the pivotal trial of belinostat in relapsed or refractory peripheral T-cell lymphoma (PTCL) continue according to protocol until 100 evaluable patients are enrolled. The study, designated BELIEF, is designed to test the HDAC inhibitor as a monotherapy in PTCL patients. Pending positive data, Spectrum expects to file a rolling new drug application in 2011 or 2012.

• **Versartis Inc.**, of Mountain View, Calif., started a Phase I trial of lead candidate VRS-317, a once-monthly form of recombinant human growth hormone, in growth hormone deficiency. The primary objective is to test the drug's safety and tolerability, when given as a single subcutaneous dose. The study will enroll up to 50 adult patients.

Appointments and Advancements

• **Cubist Pharmaceuticals Inc.**, of Lexington, Mass., appointed Kenneth M. (Ken) Bate nonexecutive chairman.

• **Enzon Pharmaceuticals Inc.**, of Piscataway, N.J., appointed Richard C. Mulligan vice chairman of the board.

• **Genesis Biopharma Inc.**, of Los Angeles, appointed William H. Andrews to its board.

• **Hospira Inc.**, of Lake Forest, Ill., elected F. Michael (Mike) Ball CEO and board member. Founding CEO Christopher B. Begley will assume the role of executive chairman.

• **ITherX Pharmaceuticals Inc.**, of San Diego, named Stefan Zeuzem chairman of its scientific and clinical advisory board.

BioWorld® BioTech SuperBook:

Vital Signs & The 2010 State of the Industry

We've beefed up the Annual Report with sector analyses, revenue projections, a timeline and more to augment the value you traditionally received! We've taken nothing out of the information that has made this a market summary best-seller for years, but we have added data and analysis that will surely increase its value.

The **475-page report** is published with color highlighting and is laid out in column format for the first time, which makes for more convenient reading.

You get introspective overview on the happenings from the bench to Wall Street to Washington to Europe.

How To Order:

Call: 1-800-688-2421 or 1-404-262-5476

E-mail: orders@bioworld.com

Online: Go to www.bioworld.com/superbook

Mention priority code S10406-EM/11651



Join the Largest Gathering of Biotech Leaders in Washington DC

Only one event draws the entire biotech world together: **2011 BIO International Convention**. Join more than 15,000 industry professionals from 65 countries, June 27 – 30, in Washington, DC for 4 dynamic days of insights, innovations and partnering opportunities to drive your business forward.

- Learn new perspectives from high-profile **Keynotes** and **Super Session** speakers.
- Blaze through the learning curve in 125+ expert-led sessions.
- Build partnerships in the **BIO Business Forum**.
- Discover cutting-edge solutions from over 1,700 leading companies in the **BIO Exhibition**.
- Engage with the BIO community with our enhanced personal event planner, **myBIO**.

Register Today and Save 40%

Early-bird deadline: May 19, 2011

convention.bio.org

Leadership. Partnerships. Breakthroughs.



June 27-30, 2011
Monday-Thursday

Walter E. Washington
Convention Center
Washington DC USA

A SERVICE OF:
Bio
BIOTECHNOLOGY
INDUSTRY ORGANIZATION

Follow us:

