BIO'S EMERGING COMPANIES

APRIL 1, 2012

FOCUS ON CARDIOLOGY/ PULMONOLOGY/BLOOD

BIO SERVING AS YOUR WASHINGTON, D.C. OFFICE

A QUARTERLY REVIEW OF ISSUES, REGULATIONS, AND SCIENTIFIC DISCOVERIES IN THE FIELD OF CARDIOLOGY/PULMONOLOGY/BLOOD TREATMENTS AND THERAPIES

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SPECIAL POINTS OF INTEREST:

- **NCATS and CAN funded for FY 2012 (p. 2)**
- **TREAT Act and FAST Act introduced to reform FDA (p. 5)**
- **Congress reauthorizes SBIR; passes JOBS Act (p. 6)**

NHLBI SCIENTISTS: GENE THERAPY HELPS PATIENTS WITH HEMOPHILIA

A single dose of an experimental gene therapy boosted production of a missing blood-clotting factor in people with hemophilia, a new study shows. The therapy might give patients a long-term solution for preventing dangerous bleeding episodes.

Hemophilia is a rare, inherited disorder in which blood is unable to clot normally. As a result, people with hemophilia tend to bleed more than others after injury. They may also bleed without warning inside their bodies. This bleeding can damage organs and tissues and may be life threatening.

The main treatment, called replacement therapy, involves infusing missing clotting factor proteins into the patient's bloodstream. These proteins help to restore normal blood clotting. But replacement therapy often must be repeated regularly, and it carries other risks.

To find an alternative, researchers from the University College London and St. Jude Children's Research Hospital led a team that investigated a potential gene therapy approach. The research, funded in part by NHLBI, focused on hemophilia B. This uncommon form of the disease affects about 1 in 5 patients with hemophilia. Hemophilia B is caused by defects in the gene that codes for human clotting factor IX.

Scientists packaged a normal factor IX gene into a modified adeno-associated virus that targets liver cells. The liver is the only site that can produce a form of factor IX needed for the clotting process. The virus—acting as a delivery vehicle, or vector—was designed to transport the normal gene into liver cells and launch production of factor IX.

Six men with severe hemophilia B received one-time intravenous infusions of the gene vector at varying doses. Prior to the study, the men were producing clotting factor IX at less than 1% of normal levels. They had been receiving the standard treatment for their condition: infusions of manufactured factor IX protein several times a month.

After gene therapy, each patient generated factor IX at between 2% and 11% of normal levels. In the short-term follow-up period (6 to 16 months), 4 of the 6 men no longer needed factor IX infusions for routine bleeding. The other 2 patients needed factor IX infusions less often than before the study

"Hemophilia has long been one of the disorders thought most likely to be correctible with gene therapy, but previous approaches to deliver the gene have been disappointing," says NHLBI Acting Director Dr. Susan B. Shurin. "Results from this study represent a promising step toward making gene therapy a viable treatment option for hemophilia B. If future studies support these findings, it would bring a significant improvement in the quality of life for those living with the disease."

For more information on this research, click here.

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Bio BIOTECHNOLOGY INDUSTRY ORGANIZATION

FY 2012 FUNDING FOR NCATS AND CAN

On December 23, 2011, President Obama signed into law the Consolidated Appropriations Act, which appropriated funds for various federal agencies, including the National Institutes of Health (NIH). Included in NIH's $30.690 billion budget authority was an appropriation of $576.5 million for the newly-authorized National Center for Advancing Translational Sciences (NCATS). The goal of the Center is to work in partnership with the public and private sectors to develop innovative ways to overcome obstacles in the translational science pipeline.

NCATS unifies certain existing NIH programs to accomplish these goals, including the Clinical and Translational Science Awards, the Office of Rare Diseases and Research, the Therapeutics for Rare and Neglected Diseases (TRND) program, the Bridging Interventional Development Gaps program, and the NIH Chemical Genomics Center. NCATS will also develop the Cures Acceleration Network (CAN), which was authorized in 2010 and recently appropriated $10 million in new funds to help bridge the "valley of death" between basic and clinical research.

FY 2012 Funding Levels

NIH
$30.690 billion

NCATS
$576.5 million

CAN
$10 million

NHLBI
$3.08 billion

NEW TECHNOLOGY AVAILABLE FOR LICENSING FROM THE NIH TECHNOLOGY TRANSFER OFFICE

miR126 for the Mobilization of Hematopoietic Stem/Progenitor Cells (HSPCs) into Peripheral Blood

The NIH inventors have discovered that a micro RNA, miR126, mobilizes hematopoietic stem/progenitor cells (HSPCs) from the bone marrow into blood. These mobilized HSPCs can be easily collected from blood and used for reconstitution of ablated or functionally-impaired bone marrow. miR126 may also facilitate mobilization of bone-resident cancer cells into the circulation where they could be more easily targeted by cancer therapeutics. This discovery could replace bone marrow transplantation as we do it today. Rather than using the current non-selective agent G-CSF (which preferentially mobilizes mature myeloid cells rather than stem/progenitor cells), miR126 could be used for selective mobilization of the HSPCs needed for hematopoietic cell transplantation. Also, miR126 could be used to mobilize malignant cells from the bone marrow and render them more easy targets for therapy.

FDA Blood Products Advisory Committee

2012 Meeting Schedule

May 15-16
July 31-August 1
December 4-5

FDA PULMONARY-ALLERGY DRUGS ADVISORY COMMITTEE MEETING

On February 23, 2012, the FDA Pulmonary-Allergy Drugs Advisory Committee met to discuss NDA 202-450 from Forest Laboratories, Inc. for aclidinium bromide inhalation powder 400 mcg twice daily, proposed for the long-term, maintenance treatment of bronchospasm associated with COPD, including chronic bronchitis and emphysema. Aclidinium is a new molecular entity and is categorized as an anticholinergic agent. Due to its duration of action and its specific action on muscarinic receptors, aclidinium belongs to the subclass of long-acting antimuscarinics (LAMA). Aclidinium is supplied as a dry powder inhalation formulation administered by the Almirall inhaler device. To support the 400 mcg BID dose for the proposed indication, Forest conducted a clinical program that included two dose-ranging trials, three pivotal Phase 3 efficacy and safety trials, and three long-term safety trials. The major issue for discussion at the meeting was whether the totality of the data supports the efficacy and safety of aclidinium 400 mcg BID for the proposed indication.

For more information on this meeting, please click here.

NHLBI FUNDING ANNOUCEMENTS

PAR-12-138, NHLBI Systems Biology Collaborations (R01) – September 14, 2012

PA-12-110, Getting from Genes to Function in Lung Disease (R01) – June 5, 2012

PAR-12-043, Identifying Heart, Lung, and Blood Disease-Causing Variants (R01) – February 8, 2013

RFA-HL-016, NHLBI SBIR Phase IIB Bridge Awards to Accelerate the Commercialization of Technologies for Heart, Lung, Blood, and Sleep Disorders and Diseases (R44) – June 19, 2012

PA-11-307, Discovery of Genetic Basis of Mendelian or Monogenic Heart, Lung, and Blood Disorders (X01) – May 14, 2012

PA-11-186, Translation of Pluripotent Stem Cell Therapies for Blood Diseases (R01) – October 5, 2012

PA-11-165, Nutrition and Diet in the Causation, Prevention, and Management of Heart Failure (R01) – June 5, 2012

PA-11-121, Ribosomal Disorders & Their Role in Inherited Bone Marrow Failure Syndromes (R01) – June 5, 2012

PA-11-148, Nanoscience and Nanotechnology in Biology and Medicine (R01) – June 5, 2012

PA-10-179, Aging Studies in the Pulmonary System (R01) – June 5, 2012

PA-10-117, New Approaches to Arrhythmia Detection and Treatment (SBIR [R43/R44]) – April 5, 2012

PA-09-249, Directed Stem Cell Differentiation for Cell-Based Therapies for Heart, Lung, and Blood Diseases (SBIR [R43/R44]) – April 5, 2012

PAR-09-185, Translational Programs in Lung Diseases (P01) – May 26, 2012

PAR-10-034, Selected Topics in Transfusion Medicine (R01) – October 5, 2012

PA-09-244, Nutrition and Physical Activity Research to Promote Cardiovascular and Pulmonary Health (R21) – June 16, 2012

PAR-11-204, Early-Phase Clinical Trials for Blood Cell Therapies (R01) – October 5, 2012

For more information or to find more funding opportunities, please click here.

PATIENT ORGANIZATION EVENTS

American Thoracic Society	American Heart Association	American College of Chest Physicians
International Conference May 18-23, 2012 San Francisco, California	Scientific Sessions 2012 November 3-7, 2012 Los Angeles, California	CHEST 2012 October 20-25, 2012 Atlanta, Georgia
Click here for more details.	Click here for more details.	Click here for more details.

HOUSE OF REPRESENTATIVES HEARINGS ON BIOTECHNOLOGY

House Energy and Commerce Committee, Subcommittee on Health
"FDA User Fees 2012: Hearing on Issues Related to Accelerated Approval, Medical Gas, Antibiotic Development and Downstream Pharmaceutical Supply Chain" — March 8, 2012

At this hearing, the Health Subcommittee heard from a variety of industries that are regulated by the FDA and depending on the User Fee Acts being reauthorized. For the biopharmaceutical industry, the committee focused on the FDA Accelerated Approval process. BIO Board Member John Maraganore testified on the importance of the Accelerated Approval pathway and in support of the FAST Act, which would enhance Accelerated Approval at FDA.

House Appropriations Committee, Subcommittee on Labor, HHS, Education, & Related Agencies
"Budget Hearing – Department of Health and Human Services – NIH" — March 20, 2012

This hearing focused on the newly-established National Center for Advancing Translational Sciences (NCATS) at NIH. NIH Director Collins and NCATS Acting Director Insel testified on the process of getting NCATS up and running and the expectations they have for the Center. Industry representatives, including BIO Board Member Scott Koenig, testified on the importance of NCATS and the key role it will play in advancing translational research.

House Committee on Science, Space, and Technology, Subcommittee on Technology and Innovation
"Fostering the U.S. Competitive Edge" — March 27, 2012

The Technology and Innovation Subcommittee held this hearing to better understand how federal policies and regulations affect competition, innovation, and job growth. BIO Board Chairman Ron Cohen testified on several steps that Congress has already taken to spur innovation, including SBIR, TDP, and patent reform. He also spoke in favor of reforms to the Accelerated Approval pathway at FDA.

SENATE HEARINGS ON BIOTECHNOLOGY

Senate Committee on Banking, Housing, and Urban Affairs
"Spurring Job Growth Through Capital Formation While Protecting Investors, Part II" — March 6, 2012

The Senate Banking Committee held this hearing to discuss several proposals on how to ease capital formation for emerging companies. The bills under consideration included an on-ramp to the public market for emerging growth companies and reforms to SEC Regulation A, SEC Regulation D, and the SEC private shareholder limit. OncoMed Pharmaceuticals CFO Will Waddill testified on how these policies would affect growing biotech companies and stimulate innovation and job growth. The legislation that BIO supported at the hearing was later combined into one bill and passed as the JOBS Act.

Senate Committee on Health, Education, Labor, and Pensions
"Strengthening FDA and the Medical Products Industry for the Benefit of Patients" — March 29, 2012

The Senate HELP Committee held this hearing to discuss the upcoming reauthorization of PDUFA and how FDA and its regulated industries can speed treatments and therapies to patients. Sara Radcliffe, Executive Vice President of Health, testified on behalf of BIO; she was joined by other industry representatives as well as Janet Woodcock (CDER) and Jeffrey Shuren (CDRH) from the FDA.

FDA REFORM LEGISLATION

BIO supports two pieces of legislation that would reform and modernize the FDA to speed life-saving treatments and breakthrough medicines to patients living with debilitating diseases. BIO CEO Jim Greenwood issued statements praising both the **TREAT Act** and the **FAST Act**. BIO has also testified on the Hill in support of the proposed reforms.

S. 2113 – Transforming the Regulatory Environment to Accelerate Access to Treatments (TREAT) Act
This bill would ***reform the FDA*** by advancing regulatory science and innovation, elevating FDA and empowering operational excellence, and enabling modernized patient-centric clinical development. Specific proposed reforms include updating FDA's mission statement, enhancing the agency's access to external scientific experts, and ***strengthening the Accelerated Approval pathway***.

Sponsor: Sen. Kay Hagan (NC)
Status: Referred to the Senate HELP Committee

H.R. 4132 – Faster Access to Specialized Treatments (FAST) Act
This bill would reform the Accelerated Approval pathway at the FDA to ***expedite the approval of drugs*** for serious of life-threatening diseases or conditions while maintaining important safety standards.

Sponsors: Rep. Cliff Stearns (FL-6) and Rep. Edolphus Towns (NY-10)
Status: Referred to the House Committee on Energy and Commerce

HEART/LUNG/BLOOD-FOCUSED LEGISLATION

H.R. 1810 – Tom Lantos Pulmonary Hypertension Research and Education Act
This bill would require the Directors of NIH and NHLBI to continue aggressive work on pulmonary hypertension and also continue research to ***find a cure for pulmonary hypertension***.

Sponsor: Rep. Kevin Brady (TX-8)
Status: Referred to the House Committee on Energy and Commerce

H.R. 1394 – Lung Cancer Mortality Reduction Act
This bill would require the Secretary of HHS to implement a comprehensive program to achieve a ***50% reduction in the mortality rate of lung cancer by 2020***. The bill also establishes a Lung Cancer Early Detection Program.

Sponsor: Rep. Donna Christensen (VI)
Status: Referred to the House Committee on Energy and Commerce

H.R. 640 – Bone Marrow Failure Disease Research and Treatment Act
This bill would require the Secretary of HHS to develop a system to collect data on ***acquired bone marrow failure*** diseases and to award grants to improve diagnostic practices and quality of care for patients with such diseases.

Sponsor: Rep. Doris Matsui (CA-5)
Status: Referred to the House Committee on Energy and Commerce

S. 438 – Heart Disease Education, Analysis, Research, and Treatment for Women Act
This bill would require the Secretary of HHS to report on the quality of ***care for women with heart disease***, stroke, and other cardiovascular diseases and to include recommendations for eliminating treatment disparities.

Sponsor: Sen. Debbie Stabenow (MI)
Status: Referred to the Senate Committee on Health, Education, Labor, and Pensions

H.R. 1988 – Qualifying Therapeutic Discovery Project Tax Credit Extension Act
This bill would ***extend the Therapeutic Discovery Project*** through the year 2017 and ***fund it at $1 billion per year***. Qualifying investments in years 2011 through 2015 would qualify for the credit or grant.

Sponsors: Rep. Susan Davis (CA-53) and Rep. Allyson Schwartz (PA-13)
Status: Referred to the House Committee on Energy and Commerce

BIO'S EMERGING COMPANIES

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BIO Meetings and Conferences

BIO International Convention
June 18-21, 2012
Boston, Massachusetts

BIO India International Conference
September 12-13, 2012
Hyderabad, India

Livestock Biotech Summit
September 19-21, 2012
Kansas City, Missouri

BIO Investor Forum
October 9-10, 2012
San Francisco, California

BIO China
October 24-25, 2012
Shanghai, China

BIO Europe Fall
November 11-14, 2012
Hamburg, Germany

PRESIDENT OBAMA SIGNS SBIR REAUTHORIZATION

On December 31, 2011, President Obama signed into law the **National Defense Authorization Act**, which reauthorized the SBIR program through 2017. Included in the reauthorization was an end to the eligibility ban on venture-backed companies. Now, venture-backed companies will be able to compete for 25% of SBIR funds at NIH, NSF, and the Department of Energy and 15% of funds at all other agencies. Additionally, research agencies with SBIR programs will increase the set aside from 2.5% to 3.2% by 2017. BIO is in the process of reaching out to SBA, which will propose rules on affiliation in the upcoming months.

CONGRESS PASSES THE JOBS ACT

In late March, Congress passed the **Jumpstart Our Business Startups (JOBS) Act** to ease capital formation for growing startup companies. The legislation includes an "on-ramp" to the public market for "emerging growth companies," which will give them a five-year transition period before having to comply with certain regulatory burdens, including Sarbanes-Oxley (SOX) Section 404(b). The bill will also allow companies to conduct direct public offerings of up to $50 million under SEC Regulation A, eliminate the ban on general solicitation in Rule 506 of SEC Regulation D, and raise the shareholder limit that requires private companies to register with the SEC. The JOBS Act passed both houses of Congress with broad bipartisan support and President Obama has indicated that he will sign it into law.

BIO ADVOCATES FOR TDP EXTENSION AND EXPANSION

BIO has been advocating Congress for an extension and expansion of the Therapeutic Discovery Project (TDP) tax credit program. The TDP was enacted in 2010 and awarded $1 billion in tax credits and grants to breakthrough biotechnology companies with fewer than 250 employees. To support extension and expansion of this important program, BIO produced three video vignettes which give policymakers a view of what TDP has done for biotech companies already. The videos highlight companies that received awards through the program and emphasize the positive effect that the funding had on job creation, the development of treatments, and U.S. competitiveness. To view these videos, please click **here**, **here**, and **here**.

Bio BIOTECHNOLOGY INDUSTRY ORGANIZATION