NCI Details Specifics of Vice President’s Cancer Moonshot

In late January 2016, President Barack Obama announced the establishment of a new Cancer Moonshot Task Force – to be led by Vice President Joe Biden – to focus on making the most of Federal investments, targeted incentives, private sector efforts from industry and philanthropy, patient engagement initiatives, and other mechanisms to support cancer research and enable progress in treatment and care.

The Administration is launching the National Cancer Moonshot with a $1 billion initiative to provide the funding necessary for researchers to accelerate the development of new ways to detect and treat cancer, including:

- The Moonshot initiative will begin immediately with $195 million in new cancer activities at the National Institutes of Health (NIH) in Fiscal Year 2016.

The Fiscal Year 2017 Budget will propose to continue this initiative with $755 million in mandatory funds for new cancer-related research activities at both NIH and the Food and Drug Administration.

The Departments of Defense and Veterans Affairs are increasing their investments in cancer research, including through funding Centers of Excellence focused on specific cancers, and conducting large longitudinal studies to help determine risk factors and enhance treatment.

Within the Department of Health and Human Services (HHS), these investments will support cutting edge research opportunities, such as prevention and cancer vaccine development, early cancer detection, cancer immunotherapy and combination therapy, genomic analysis of tumor and surrounding cells, enhanced data sharing, Oncology Center of Excellence, new technologies to combat pediatric cancer, and the Vice President’s Exceptional Opportunities in Cancer Research Fund.

The National Cancer Moonshot requires a whole-of-government approach, marshalling resources from across the Federal government to address this singular goal. Over time, other agencies will make new investments in this effort, beginning with the Departments of Defense (DOD) and Veterans Affairs (VA).

To read more, please click here.

“Today, cancer research is on the cusp of major breakthroughs. It is of critical national importance that we accelerate progress towards prevention, treatment, and a cure -- to double the rate of progress in the fight against cancer.”

Special Update: Bio Survey on FDA/Sponsor Interactions

Bio is conducting a first-of-its-kind survey designed to evaluate FDA/Sponsor Interactions During Drug Development. All BIO members and non-members are encouraged to sign up and log in at fdasurvey.bio.org to provide feedback.

The FDA has expressed particular interest in information about FDA-company interactions which inform comparisons between review divisions. The online survey tool allows participants to provide this feedback for each phase of development and each clinical program. The data collected in this important initiative will enhance the biotech industry’s relationship with FDA and inform the ongoing PDUFA 6 negotiations. All responses to the survey will be kept anonymous, and all results will be aggregated.

To sign up to participate, please visit fdasurvey.bio.org today!
**UPDATES FROM NCATS**

On Feb. 29, NIH hosted a Rare Disease Day event to raise awareness about rare diseases, the people they affect, and current research collaborations. An estimated 25 million people in the United States have rare diseases. The event featured presentations, posters, exhibits, an art show and tours of the NIH Clinical Center – a hospital at which researchers are studying nearly 600 rare diseases in partnership with over 15,000 patients. The event was hosted by the National Center for Advancing Translational Sciences and the NIH Clinical Center. Partner organizations include the U.S. Food and Drug Administration, National Organization for Rare Disorders, Genetic Alliance, Global Genes, Everylife Foundation for Rare Diseases, and Uplifting Athletes.

To read more about Rare Disease Day, please click [here](#).

NCATS is seeking applications for rigorous, pre-clinical research projects that are based on repurposing existing drugs or biologics. Through this new funding opportunity, NCATS anticipates committing $4.3 million in fiscal year 2016 to issue 10 to 15 awards in support of studies that establish the rationale for a clinical trial. Pre-clinical studies funded through this initiative will serve as “use cases” to demonstrate the utility of an independent crowdsourcing effort or of a computational algorithm to predict new therapeutic uses of an existing drug or biologic. The goal of an individual project must be to explore the potential new use of an existing investigational therapeutic or one already approved by the Food and Drug Administration to treat another disease.

To read more about this opportunity, please click [here](#).

---

**Upcoming Meetings**

**Oncologic Drugs Advisory Committee**

On November 18, 2015, the Cellular, Tissue and Gene Therapies Advisory Committee held a joint meeting to discuss the safety and efficacy of Biologics License Application (BLA) 125593, Mycobacterium phlei Cell wall-Nucleic Acid complex (MCNA), submitted by Telesta Therapeutics Inc. The proposed indication (use) for this product is treatment of non-muscle invasive bladder cancer (NMIBC) at high risk of recurrence or progression in adult patients who failed prior Bacillus Calmette-Guérin (BCG) immunotherapy, e.g., in patients who are BCG-refractory or BCG-relapsing.

To read more about the meeting, please click [here](#).

---

**Oncologic Drugs Advisory Committee**

On November 19, 2015, the Pediatric Oncology Subcommittee of the Oncologic Drugs Advisory Committee met to discuss investigator interest in exploring potential pediatric development plans for two products in various stages of development for adult cancer indications. The subcommittee considered and discussed issues concerning diseases to be studied, patient populations to be included, and possible study designs in the development of these products for pediatric use. The discussion also provided information to the Agency pertinent to the formulation of written requests for pediatric studies. The products under consideration were: (1) ABT-414, sponsored by AbbVie, Inc., and (2) lenvatinib, sponsored by Eisai, Inc.

To read more about the meeting, please click [here](#).

On April 12, the Oncologic Drugs Advisory Committee will meet to discuss new drug application (NDA) 208542 rociletinib tablets, application submitted by Clovis Oncology, Inc. The proposed indication (use) for this product is for the treatment of patients with mutant epidermal growth factor receptor (EGFR) non-small cell lung cancer (NSCLC) who have been previously treated with an EGFR-targeted therapy and have the EGFR T790M mutation as detected by an FDA approved test.

To read more about the upcoming meeting, please click [here](#).
NCI FUNDING ANNOUNCEMENTS

**RFA-CA-16-003**—**Innovative Technologies for Cancer-Related Biospecimen Science**—September 27, 2016

**PAR-14-285**—**Innovative Research in Cancer Nanotechnology**—April 15, 2017

**PAR-16-111**—**Cooperative Agreement to Develop Targeted Agents for Use with Systemic Agents Plus Radiotherapy**—December 15, 2017

**PAR-16-84**—**Feasibility Studies to Build Collaborative Partnerships in Cancer Research**—January 30, 2018

**PAR-16-105**—**Cancer Tissue Engineering Collaborative: Enabling Biomimetic Tissue-Engineered Technologies for Cancer Research**—December 1, 2018

**PAR-16-089**—**Imaging and Biomarkers for Early Cancer Detection**—December 12, 2018

For more information or to find more funding opportunities, please click [here](#).

NEW TECHNOLOGIES AVAILABLE FOR LICENSING FROM THE NIH OFFICE OF TECHNOLOGY TRANSFER

**Use of Small Molecules to Treat PARP1-deficient Cancers**

Scientists at the National Human Genome Research Institute and the National Center for Advancing Translational Sciences have identified a class of small molecules synergistically working with known Poly (ADP-ribose) polymerase 1 (PARP-1)-inhibitors. These new small molecules can each effectively kill specific PARP-1 defective tumors cells and show synergy with known PARP1 inhibitors (PARP-1i) in killing tumor cells. PARP1, a highly conserved DNA binding protein, is essential for repairing DNA damage and plays important roles in multiple DNA damage response pathways. Many cancer therapies utilize DNA-damaging agents to kill tumor cells, which often triggers DNA repair (e.g., by activating PARP1 pathways). Additionally, a variety of cancer types may also carry PARP1 mutation(s), such as glioma, breast cancer, and prostate cancer. Such mutations render the cancer cells resistant to these therapies.

**A Novel Fully-Human Anti-CD30 Chimeric Antigen Receptor for Treatment of CD30+ Lymphoma**

Chimeric antigen receptors (CARs) are hybrid proteins that consist of two major components: A targeting domain and a signaling domain. The targeting domain allows T cells which express the CAR to selectively recognize and bind to diseased cells that express a particular protein. Once the diseased cell is bound by the targeting domain of the CAR, the signaling domain of the CAR activates the T cell, thereby allowing it to kill the diseased cell. This is a promising new therapeutic approach known as adoptive cell therapy (ACT). Researchers at the National Cancer Institute’s Experimental Transplantation and Immunology Branch developed a CAR that recognizes human tumor necrosis factor receptor superfamily member 8 (TNFRSF8, also known as CD30). The expression of CD30 is deregulated in a variety of human cancers, including many lymphomas. By creating a CAR that recognizes CD30, it may be possible to treat these cancers using adoptive cell therapy.

To learn more about these technologies and to find others available for licensing, please click [here](#).

PATIENT ORGANIZATION EVENTS

<table>
<thead>
<tr>
<th>European Society for Medical Oncology</th>
<th>American Association for Cancer Research</th>
<th>American Society of Clinical Oncology</th>
</tr>
</thead>
<tbody>
<tr>
<td>European Lung Cancer Conference</td>
<td>AACR Annual</td>
<td>ASCO Annual</td>
</tr>
<tr>
<td>April 13-16, 2016</td>
<td>Meeting 2016</td>
<td>Meeting 2016</td>
</tr>
<tr>
<td>Geneva, Switzerland</td>
<td>April 16-20, 2016</td>
<td>June 3-7, 2016</td>
</tr>
<tr>
<td></td>
<td>New Orleans, LA</td>
<td>Chicago, IL</td>
</tr>
</tbody>
</table>

Click [here](#) for more details.
BIO APPLAUDS PRECISION MEDICINE INITIATIVE

In February, President Obama outlined the next steps in the effort to study a million people’s genomes and develop more targeted treatments for diseases. The plan, the Precision Medicine Initiative, was originally proposed last year.

BIO Executive Vice President for Health Policy Dan Durham released the following statement regarding the initiative:

“Precision medicine holds great promise for many health care interventions to occur much earlier and with increased accuracy. We applaud this move to begin building a national, large-scale research participant group to accelerate precision medicine technologies. The data and discoveries resulting from this Initiative may play a critical role in enabling biopharmaceutical companies to develop treatments for patients before or early on in their disease progression, and with therapeutics that are far more effective and focused on the specific characteristics of their particular disease.”

To read more, please click here.

R&D CREDIT MADE PERMANENT

In December, Congress made the R&D Tax Credit permanent, along with several other tax extenders in the Protecting Americans from Tax Hikes (PATH) Act. In addition, the R&D Credit was reformed to allow an immediate capital infusion for pre-revenue startups through a payroll tax offset. Qualified Pre-revenue companies can now claim up to $250,000 in R&D Credits against their payroll tax liability, which is applied toward payroll tax obligations in the following year. In order to qualify, a company must have $5 million in gross receipts and have zero historical gross receipts prior to the most recent five taxable years. Companies are eligible for the payroll tax offset for five years.

BIO President and CEO Jim Greenwood released the following statement regarding the Credit:

“The R&D Tax Credit is pro-innovation, pro-growth, and pro-America, and we strongly support making it permanently available to biotechnology companies as they search for new cures and treatments...We strongly support the provision to allow eligible small businesses to claim the credit against payroll tax liability, which will improve small, pre-revenue companies’ access to the tax credit.”

To read the full press release, please click here.

HOUSE PASSES SMALL COMPANY DISCLOSURE SIMPLIFICATION ACT

In early February, The House of Representatives passed H.R. 1675, the Capital Markets Improvement Act, which combined several bills that passed the House Financial Services Committee on a broad, bipartisan basis. Included in this bill was the Small Company Disclosure Simplification Act, originally introduced by Representative Robert Hurt (R-VA) in April 2015.

The Small Company Disclosure Simplification Act provides a voluntary exemption for all Emerging Growth Companies and other issuers with annual gross revenues under $250 million from the SEC’s XBRL reporting requirements.

Notwithstanding XBRL’s significant costs, it has yielded little benefit. Research from Columbia University indicates that fewer than 10% of investors have used XBRL data for analysis, with some investors complaining that the data isn’t reliable or timely. With many biotech companies at a point where they don’t currently have revenue, the costly XBRL compliance especially hurts these innovators, who have been forced to divert funds from scientific research.

BIO supports H.R. 1675 and applauds Representative Hurt and the House Financial Services Committee for taking a step in limiting costly compliances burdens, and believes the legislation will free growing companies from a costly regulatory burden that does more harm than good.

To read more, please click here.
2015 was a blockbuster year for FDA approvals of novel new medicines, many of them for serious and life-threatening conditions. As Dr. John Jenkins of the FDA’s Center for Drug Evaluation and Research (CDER) notes in a recent blog post, “During this past year, we approved many new drugs to treat various forms of cancer, including four to treat multiple myeloma, and others to treat lung, skin, breast, brain, colorectal, and other cancers. We also approved new drugs to treat heart failure, high cholesterol, cystic fibrosis, and irritable bowel syndrome, as well as the first approved reversal agent for a commonly-used blood thinner. And, for the second consecutive year, we approved more drugs to treat rare diseases than any previous year in our history.”

BIO’s Industry Analysis team has tallied the number of novel medicines approved in 2015 by both FDA’s Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) divisions. Combined, CDER and CBER approved 48 novel medicines.

To read more, please click here.

Brian Hahn, Chief Financial Officer of GlycoMimetics, Inc., provided testimony in December on behalf of BIO before the House Subcommittee on Capital Markets and Government Sponsored Enterprises. Hahn serves as the Co-Chair of BIO’s Finance & Tax Committee.

Mr. Hahn testified in support of the Fostering Innovation Act, which would extend the JOBS Act’s five-year Sarbanes-Oxley (SOX) Section 404(b) exemption for an additional five years for certain small businesses. Mr. Hahn also provided support for legislation, the SEC Small Business Advocate Act and the Small Business Capital Formation Enhancement Act, that would enhance the role of emerging companies in the SEC’s policymaking process.

To read Brian Hahn’s full testimony, please click here.

Will Waddill, SVP and CFO of Calithera Biosciences, presented on behalf of BIO during the Thirty-Fourth SEC Government-Business Forum on Small Business Capital Formation in November. The Forum has been held annually since 1982 and has served as an effective opportunity for the SEC and its staff to learn more about the important capital formation issues that the small business sector is facing, and how to best combat those issues.

Mr. Waddill’s presentation discussed registered offerings post JOBS Act, specifically on the impact of regulatory burdens on emerging company capital formation.

Mr. Waddill’s proposal to update the SRC and Non-Accelerated Filer definitions was well received and eventually included in the prioritized list of breakout group recommendations which followed the presentations. He suggested changing the SRC-Non-Accelerated Filer definition to include companies with less than $250 million in public float or $100 million in annual revenues. Making this change, he argued, would provide regulatory relief to growing companies and support early-stage capital formation.

To view Mr. Waddill’s presentation as well as other materials from the event, please click here.

S. 2262—CT Colonography Screening for Colorectal Cancer Act of 2015
To amend title XVIII of the Social Security Act to cover screening computed tomography colonography as a colorectal cancer screening test under the Medicare program.

Sponsor: Senator Jim Inhofe (R-OK)
Status: Referred to the Senate Committee on Finance

S. 2373—Lymphedema Treatment Act
To amend title XVIII of the Social Security Act to provide for Medicare coverage of certain lymphedema compression treatment items as items of durable medical equipment.

Sponsor: Senator Maria Cantwell (D-WA)
Status: Referred to the Senate Committee on Finance
**BIO VALUE OF INNOVATION CAMPAIGN**

BIO launched a new ad in early February focused on the value of biomedical innovation, highlighting the most compelling benefits of biomedical research – giving patients time to live fuller lives. The ad serves as a cornerstone for BIO’s new Value Campaign.

“With this effort, BIO is advancing the national public policy debate over the cost and value of medicine, shining a spotlight on the fact that innovative, life-saving medicines provide benefits far beyond their costs,” said BIO President & CEO Jim Greenwood. “It’s time for the public to hear the voices of those who are benefitting from medical breakthroughs and those who are bringing these breakthroughs to market.”

Through the campaign, the public will see that today’s innovative life-enhancing medicines save and extend lives, improve quality of life and drive value to patients and the healthcare system as a whole. The ad represents just the beginning of BIO’s campaign, which includes a new “Time Is Precious” website.

To view the ad, please click [here](#). To view the “Time is Precious” website, please click [here](#).

**FOSTERING INNOVATION ACT**

Reps. Kyrsten Sinema (D-AZ) and Michael Fitzpatrick (R-PA) recently introduced the Fostering Innovation Act (H.R. 4139), which would extend the JOBS Act’s SOX 404(b) exemption beyond the existing 5-year limit.

Under current law, emerging growth companies (EGCs) are given a temporary SOX 404(b) exemption by the JOBS Act. A company remains an EGC for 5 years after its IPO, unless it exceeds either $700 million in public float, $1 billion in annual revenues, or $1 billion in non-controvertible debt. Assuming it does not trip one of these tests, it retains its SOX 404(b) exemption for its entire 5-year life as an EGC.

Most biotechs remain pre-revenue long after their EGC status expires – so they will see a damaging diversion of capital from science to compliance in the form of expiring JOBS Act exemptions at the dawn of year 6 on the market.

The Fostering Innovation Act would extend the JOBS Act SOX 404(b) exemption to a certain subset of low-revenue/low-public float former EGCs for an additional 5 years.

Eligibility for the year 6-10 exemption would be limited to former EGCs that lost their EGC status because of the 5 year time limit – thus excluding those that tripped the $700 million public float, $1 billion revenue, or $1 billion non-controvertible debt tests. Companies would have to meet both a public float test (less than $700 million in public float) and a revenue test (less than $50 million in average gross revenues) throughout years 6-10 to maintain the exemption.

BIO believes that extending the JOBS Act EGC exemption from SOX 404(b) would more accurately reflect the business model and long development timelines of emerging, pre-revenue businesses.