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.
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 ANNOUNCEMENTS

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

<table>
<thead>
<tr>
<th>American Thoracic Society</th>
<th>American Heart Association</th>
<th>American College of Chest Physicians</th>
</tr>
</thead>
<tbody>
<tr>
<td>International Conference</td>
<td>Scientific Sessions 2012</td>
<td>CHEST 2012</td>
</tr>
<tr>
<td>San Francisco, California</td>
<td>Los Angeles, California</td>
<td>Atlanta, Georgia</td>
</tr>
</tbody>
</table>

Click here for more details.
HOUSE OF REPRESENTATIVES HEARINGS ON BIOTECHNOLOGY

House Energy and Commerce Committee, Subcommittee on Health  

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  

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 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
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.

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.