Focus on
Nephrology, Endocrinology, Metabolism,
& Gastroenterology
BIO serving as your Washington, D.C. office

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NIDDK STUDY: GLUCOSE CONTROL IMPROVES EYE
HEALTH IN PEOPLE WITH TYPE 1 DIABETES

People with type 1 diabetes who intensively control their blood glucose (blood sugar) early in their disease, versus those who do not, are 48 percent less likely to need eye surgery, and the total number of such surgeries is 37 percent less.

These reductions lead to ocular surgery costs about 32 percent lower for people who practice early tight glucose control. The findings are the latest results of the Diabetes Control and Complications Trial (DCCT) and its follow-up, the Epidemiology of Diabetes Control and Complications (EDIC) study. Results were published April 30 in the New England Journal of Medicine.

“By controlling blood glucose early in the course of type 1 diabetes, people are less likely to have severe eye problems and to suffer vision loss,” said Dr. Catherine Cowie, director of programs in diabetes epidemiology within the NIH’s National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK). “As results from the DCCT and EDIC studies show again and again, tight control of blood glucose early on for people with type 1 has far-reaching benefits for preventing or delaying many of the complications of diabetes.”

Beginning in 1983, the DCCT/EDIC study enrolled 1,441 people in the United States and Canada between ages 13 and 39 with recent-onset type 1 diabetes. In the DCCT, half were assigned at random to intensive blood glucose control designed to keep blood glucose as close to normal as safely possible, and half to the conventional treatment at the time.

The DCCT ended in 1993 when the intensive group was found to have substantially less eye, nerve and kidney disease. All participants were then taught intensive blood glucose control and followed during the ongoing EDIC study. The intensive group practiced tight blood glucose control an average 6.5 years longer than the control group. Average blood glucose has been similar in both groups since about five years after DCCT ended. In 2015, researchers reported that this early intensive blood glucose control lengthened life for people with type 1 diabetes.

Higher average blood glucose accounted for the increased long-term risk of ocular surgery in the conventional group. Most of the increased need for ocular surgery was associated with retinopathy severity. Other factors associated with ocular surgery included progression of diabetes-related kidney disease, nerve damage and high blood pressure.

For more information, click here.

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 upcoming technical negotiations for PDUFA 6. 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!
On June 10, the committee met to discuss the safety and efficacy of biologics license application 125522 for injection, submitted by Amgen Inc., as adjunct to diet to reduce low-density lipoprotein cholesterol (LDL-C), total cholesterol (TC), apolipoprotein B (ApoB), non-high-density lipoprotein cholesterol (non-HDL-C), TC/HDL-C, ApoB/ApoA1, very low-density lipoprotein cholesterol, triglyceride, and lipoprotein A, and to increase HDL-C and ApoA1, in adults with hyperlipidemia or mixed dyslipidemia, either in combination with a statin or statin with other lipid-lowering therapies (e.g., ezetimibe), or alone.

For more information about this meeting, please click here.

On April 15, the committee met to discuss the new drug application (NDA) 204958, cangrelor injection, submitted by The Medicines Company, for the proposed indication of reduction of thrombotic cardiovascular events in patients with coronary artery disease (CAD) undergoing percutaneous coronary intervention (PCI) who have not received an oral P2Y12 inhibitor prior to the PCI procedure and in whom oral therapy with P2Y12 inhibitors is not feasible or desirable (P2Y12 is a protein involved in blood clotting. Inhibiting this protein is a key mechanism of action of cangrelor).

For more information about this meeting, please click here.

On May 14, the committee met to discuss recent reports in epidemiologic investigations of transmission of infections associated with the use of duodenoscopes in endoscopic retrograde cholangiopancreatography (ERCP) procedures in hospitals.

The panel then discussed the effectiveness of cleaning, high-level disinfection, and sterilization methods for duodenoscope and endoscope reprocessing; the use of automated endoscope reprocessors; the role of pre-market human factors testing in the development of reprocessing instructions; and the performance of alternative cleaning agents and brushes.

The committee also sought feedback from the Panel about how best to communicate information to the public when we have a medical device concern, without unnecessarily alarming patients, their doctors or the healthcare facilities, or creating any unfortunate unintended consequences.

For more information about this meeting, please click here.
NIDDK FUNDING ANNOUNCEMENTS

RFA-DK-15-012 A Community Research Resource of Microbiome-Derived Factors Modulating Host Physiology in Obesity, Digestive and Liver Diseases, and Nutrition (R24) - October 20, 2016


RFA-DK-15-017 Adherence Studies in Adolescents with Chronic Kidney or Urologic Diseases (R01) - November 20, 2015

RFA-DK-15-009 Translational Research to Improve Outcomes in Kidney Diseases (R18) - November 19, 2015

PAR-15-170 Diet and Physical Activity Assessment Methodology (R01) - September 8, 2018

PA-15-169 Secondary Analyses in Obesity, Diabetes and Digestive and Kidney Diseases (R21) - May 8, 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

**Treatment of Chronic Kidney Disease with Synthetic Amphipathic Peptides**

The invention is directed to treatment of chronic kidney disease by administering a synthetic, amphipathic helical peptide known as 5A-37pA, and novel derivatives thereof. Scientists at NIDDK have demonstrated that invention peptides antagonize activity of a particular scavenger receptor known as CD36. Using an in vivo model, NIDDK scientists have shown that invention peptides slowed progression of chronic kidney disease and can potentially be utilized as a therapeutic treatment.

Additionally, certain invention peptides bind selectively to CD36 with high specificity over other homologous scavenger receptors. Thus, invention peptides can be utilized as a research tool to further evaluate the complex etiology of chronic kidney disease.

5A-37pA, and derivatives thereof, are peptide mimetic of apolipoprotein A-1. These peptides have been described in NIH owned patents and/or patent applications (see, for example, U.S. Patent Nos. 7,572,771 and 8,071,746 and 8,148,323). Use of these peptides, as well as the novel peptides of this invention, for the treatment of kidney diseases is currently available for licensing.

**Monoclonal Antibody Fragments for Targeting Therapeutics to Growth Plate Cartilage**

A child’s growth is dependent on the proper functioning of the growth plate, a specialized cartilage structure located at the ends of long bones and within the vertebrae. The primary function of the growth plate is to generate new cartilage, which is then converted into bone tissue and results in the lengthening of bones. Current treatments for severe short stature and skeletal growth disorders are limited. Recombinant human growth hormone (GH) is typically used but the results are less than optimal and have potential adverse effects. The instant invention discloses that monoclonal antibodies that bind to matrilin-3, a protein specifically expressed in cartilage tissue, could be used for treating or inhibiting growth plate disorders, such as a skeletal dysplasia or short stature.

To learn more about these technologies and to find others available for licensing, please click here.

PATIENT ORGANIZATION EVENTS

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BIO TESTIFIES ON XBRL REFORM LEGISLATION; HOUSE COMMITTEE APPROVES BILL

On April 29, BIO provided testimony before a hearing of the House Subcommittee on Capital Markets, titled Legislative Proposals to Enhance Capital Formation and Reduce Regulatory Burdens. BIO was represented at the hearing by Shane Kovacs, CFO of BIO Board Member PTC Therapeutics. The hearing was focused on a package of bipartisan legislation designed to support the growth of emerging companies on the public market.

In his testimony, Mr. Kovacs stressed the importance of a strong public market for biotech capital formation. He also discussed the detrimental impact that costly regulatory burdens can have on groundbreaking R&D by diverting capital from science to compliance. In particular, Mr. Kovacs spoke about the impact that compliance with the eXtensible Business Reporting Language (XBRL) reporting requirement can have on pre-revenue biotechs.

BIO supports H.R. 1965, the Small Company Disclosure Simplification Act, sponsored by Rep. Robert Hurt (R-VA). This bill would grant emerging growth companies and low-revenue issuers a temporary exemption from XBRL compliance, allowing them to focus investment capital on research rather than an ineffective reporting requirement that is unused by investors.

Following the hearing, at which Mr. Kovacs endorsed H.R. 1965, the House Financial Services Committee passed the Small Company Disclosure Simplification Act with a bipartisan 44-11 vote.

To read Mr. Kovacs's testimony, please click here.

REPS. MEEHAN, NEAL, KELLY, KIND, & LARSON INTRODUCE PARTNER ACT

On April 30, Reps. Patrick Meehan (R-PA), Richard Neal (D-MA), Mike Kelly (R-PA), Ron Kind (D-WI), and John Larson (D-CT) introduced H.R. 2179, the PARTNER Act. The bill would modernize the U.S. tax code to encourage the private sector to invest in breakthrough research being conducted at growing biotechs and other innovative small businesses across the country.

Specifically, the legislation would relax the passive activity loss (PAL) limitations for R&D-focused pass-through entities. Under this bill, small innovative companies would be able to enter into a joint venture with an R&D project’s investors via R&D Partnership Structures. The losses and credits generated by the project would then flow through to the company and investors, who would be able to use the tax assets to offset other income.

The innovation incentives in the current tax code do not benefit not-yet-profitable companies – yet these companies are the heart of America’s innovation ecosystem. The PARTNER Act would incentivize investment in groundbreaking R&D being conducted at companies across the country. The PARTNER Act is critical to the continued vitality of next generation innovators and is a much-needed step to ensure that America maintains its place as a leader in the 21st century global economy.

To learn more about the PARTNER Act, please click here.

SEC FINALIZES TICK SIZE PILOT PROGRAM

In May, the SEC approved a finalized version of a tick size pilot program that will begin on May 6, 2016. The pilot will test the impact of a $0.05 tick (an increase from the existing $0.01) on certain small company stocks. Eligibility for the pilot will be limited to companies with a market cap below $3 billion, an average daily trading volume of less than 1 million shares per day, and a share price above $2.

The pilot will last for two years and evaluate 1,200 companies. BIO has long been a supporter of increased trading increments for growing companies on the market, and we view this as a huge step toward that goal. BIO commented on the initial pilot proposal from the SEC and the national exchanges, and we will continue to engage with members and the SEC as the pilot start date approaches.

To learn more about the upcoming pilot program, click here.
BIO RELEASES EMERGING COMPANY FINANCING REPORT

On June 11, BIO released a new report – Emerging Therapeutic Company Investment and Deal Trends – highlighting ten years (2005-2014) of biotechnology funding and deal making across five areas: venture capital, IPOs, follow-on public offerings, licensing, and acquisitions. The report also offers a first-time look at the degree of collaboration across the industry’s clinical pipeline.

“Accessing capital and forming strategic alliances is vital to today’s emerging biotechnology companies in their search for cures and treatments for patients suffering from devastating and life-threatening diseases,” said Carrier Esham, PhD, BIO’s Executive Vice President, Emerging Companies. “This data is helpful as we seek to improve our understanding of investor and deal making trends in order to inform future policy development activities intended to bolster the industry’s ability to develop the next generation of innovative medicines.”

Key findings include:
- A decade high in 2014 for US venture capital in Novel R&D lead programs. However, Series A investments went to fewer companies and with fewer dollars vs 2013. Additionally, some disease areas affecting large populations continue to see declines.
- A decade high in 2014 for U.S. emerging company IPOs.
- A decade high in 2014 for upfront payments in R&D-stage licensing deals.
- R&D-stage acquisition volume is returning to levels not seen since 2008.
- Nearly 70% of the industry clinical pipeline is attributed to small emerging companies. A significant portion of the emerging company pipeline (43%) is partnered.

To access a copy of BIO’s emerging company financing study, please click here.

SEC FINALIZES REGULATION A+ RULE

On March 25, the SEC issued a final rule implementing the Regulation A changes directed by the JOBS Act. The rule took effect on June 20; companies are now able to conduct offerings of up to $50 million under the revised reporting and disclosure requirements of Regulation A+. Prior to the passage of the JOBS Act, Regulation A offerings were limited to $5 million. The new SEC rule creates a similar offering pathway, Regulation A+, for offerings of up to $50 million. Importantly, Regulation A+ offerings will not be subject to state-level securities law, but will instead be held to a single national standard of review.

Reforming Regulation A in order to make it a viable capital formation tool for emerging biotech companies has been a key advocacy priority for BIO. To read BIO’s comment letter to the SEC on its Regulation A+ proposal, click here. To read BIO’s press release applauding the finalized rule, please click here.

BIO PROVIDES TESTIMONY FOR SENATE BANKING HEARING

On March 24, the Senate Subcommittee on Securities, Insurance, and Investment held a hearing titled Capital Formation and Reducing Small Business Burdens. BIO provided testimony for the hearing supporting the Subcommittee’s efforts to enhance capital formation for growing companies. Specifically, BIO’s statement stressed the importance of a public market that supports capital formation while also reducing capital diversions from science to compliance. BIO also provided support for a number of specific bills, including reforms to the XBRL and SOX 404(b) compliance requirements.

For more information, please click here.

NEPH/ENDO/METABOLISM/GASTRO-FOCUSED LEGISLATION

S. 598—Chronic Kidney Disease Improvement in Research and Treatment Act
Amends the Social Security Act to revise Medicare payments for dialysis services for individuals with end stage renal disease (ESRD) and acute kidney injury. Makes individuals with ESRD eligible for Medicare Advantage.

Sponsor: Sen. Ben Cardin (D-DE)
Status: Referred to the Senate Committee on Finance

H.R. 2231—Functional Gastrointestinal and Motility Disorders Research Enhancement Act
This bill would expand the research activities of the National Institutes of Health with respect to functional gastrointestinal and motility disorders, and for other purposes.

Sponsor: Rep. James Sensenbrenner (R-WI)
Status: Referred to the House Subcommittee on Health
On May 21, BIO released the following statement regarding the approval of the 21st Century Cures Act by the House Energy and Commerce Committee:

"BIO is pleased that the legislation prioritizes placing patients at the center of the drug development process, which we believe will help spur the development of therapies for the most prevalent conditions, as well as encourage development of treatments focused on unmet medical needs. We strongly support establishing a framework for incorporating patient views into the development and regulatory review processes in a more structured and transparent way with respect to both patient input for benefit-risk assessments and use of patient experience data in regulatory decision-making.

"BIO supports modernizing clinical trials to expedite and accelerate drug development through the use of alternative clinical trial designs, biomarkers and surrogate endpoints, and modern scientific approaches and greater utilization of post-market validation and other confirmatory techniques, including the use of real-world data. We support enhancing the FDA’s scientific capacity by improving access to adequate funding. We especially note the important inclusion in this legislation of provisions to ensure privately-paid user fees to FDA are protected from the effects of any future sequestration.

"BIO looks forward to working with Chairman Upton and Rep. DeGette to ensure that the 21st Century Cures Initiative expedites the delivery of the next generation of modern medicines that will save lives and reduce and eliminate suffering.”

For more information on the 21st Century Cures Act, please click here. To read BIO’s statement, please click here.

On June 5, BIO released the following statement regarding the mark-up of the PATENT Act:

"BIO appreciates the efforts of members of the Senate Judiciary Committee to include needed reforms to the PTO’s inter partes review (IPR) and post-grant review (PGR) proceedings, aimed at addressing our concerns about the basic fairness of these proceedings to patent owners. We remain committed to working with all Senators engaged in the process to include further IPR improvements necessary to ensure that the PATENT Act reflects an appropriate balance between the interests of those who seek to enforce patent rights and those who are accused of infringement.

"Biotechnology companies rely upon the strength of their patents to raise and invest the hundreds of millions of dollars needed to develop and bring to market the next generation of innovations. Without strong patent protections, revenue streams will dry up, degrading our industry’s ability to provide solutions to the most pressing medical, agricultural, industrial and environmental challenges the world faces.”

To read BIO’s statement, please click here.