PENNSYLVANIA BIO
The Voice of Advancement for the Bioscience Industry

TITLE I—PUTTING PATIENTS FIRST BY INCORPORATING THEIR PERSPECTIVES INTO THE REGULATORY PROCESS AND ADDRESSING UNMET NEEDS

SUBTITLE C—APPROVAL OF BREAKTHROUGH THERAPIES

Section 1041, led by Rep. Michael C. Burgess, M.D. (R-TX), would clarify that FDA may approve a drug that has received a breakthrough therapy designation under Section 506(a) of the Federal Food, Drug, and Cosmetic Act (FFDCA) when early stage clinical data provides sufficient evidence under the current safety and efficacy standards, considering the risks and benefits of the drug and the risks associated with the disease or condition for which unmet medical needs exist.

SUBTITLE D— ANTIBIOTIC DRUG DEVELOPMENT

The Antibiotic Development to Advance Patient Treatment (ADAPT) Act (Sections 1061-1063), authored by Reps. John Shimkus (R-IL), Gene Green (D-TX), Diana DeGette (D-CO), Ed Whitfield (R-KY), Anna Eshoo (D-CA), Morgan Griffith (R-VA), Eliot Engel (D-NY), Full Committee Vice Chair Marsha Blackburn (R-TN), and G.K. Butterfield (D-NC), would help foster the development of new antibiotics by supporting greater collaboration between industry and FDA around adaptive clinical trials and labeling changes. In addition, the sections would create a new transferable exclusivity program in order to spur additional investment in the area. The President's Council of Advisory on Science and Technology has recommended both of these proposals to help support the type of robust drug development that will be needed to ensure patients are protected from bacterial resistance.

Section 1064, led by Reps. Peter Roskam (R-IL) and Danny Davis (D-IL), is the Developing an Innovative Strategy for Antimicrobial Resistant Microorganisms (DISARM) Act, which would incentivize new drug development by improving the process of hospital payments for purposes of encouraging new drug development of antibiotic drugs for unmet medical needs.

SUBTITLE E—PRIORITY REVIEW FOR BREAKTHROUGH DEVICES

This provision (Sections 1081-1082), led by Health Subcommittee Chairman Joe Pitts (R-PA), would establish a process at FDA for the designation and expedited review of devices that represent breakthrough technologies with the potential to address unmet medical needs. If FDA designates a medical device as such under Section 1161 and approves/clears it, Section 1162 would translate into Medicare and Medicaid transitional coverage benefits. As this policy is still under development, Section 1162 currently contains a placeholder.

SUBTITLE F—ACCELERATED APPROVAL FOR BREAKTHROUGH DEVICES

This provision (Section 1101) would establish an accelerated approval pathway for medical devices, similar to the pathway that currently exists for drugs.

SUBTITLE G— EXPANDED ACCESS

These sections (1121-1125), led by Reps. Michael McCaul (R-TX) and Michael C. Burgess, M.D. (R-TX), are based on the Expanded Access Improvement Act and would place transparency requirements on certain drug companies regarding their expanded access programs (programs for patients to access drugs before they are approved). It also would create an expanded access task force to provide recommendations to Congress for further reforms of the program.

SUBTITLE H— FACILITATING RESPONSIBLE COMMUNICATION OF SCIENTIFIC AND MEDICAL DEVELOPMENTS

FDA's current rules and policies governing what drug and device developers may say about their own products were designed decades ago. Since then, the way that medicine is practiced and delivered and the way that information is communicated have fundamentally changed. Section 1141 includes placeholder language because the committee is working on a proposal that would clarify and rationalize these rules of the road so that scientific and medical developments can be shared with physicians, insurers, and researchers, with appropriate safeguards, in order to optimize patient care.

SUBTITLE J—STREAMLINED DATA REVIEW

The provision (Section 1181) led by Rep. Michael C. Burgess, M.D. (R-TX), would streamline the review process for adding indications to a drug label by allowing FDA to accept and review data summaries rather than full data packages.

SUBTITLE L— DORMANT THERAPIES

The provision (Sections 1221-1223) was introduced by Senators Orrin Hatch (R-UT) and Michael Bennet (D-CO) in December 2014 and is based on the MODDERN Cures Act, which has been spearheaded in the House by Rep. Leonard Lance (R-NJ) and supported by 48 Republicans and 47 Democrats.

The time and expense to develop therapies for complex diseases, such as Alzheimer's, pose unique challenges that make it harder to bring treatments and cures to market. In many ways, the current framework rewards companies for researching and developing treatments where development is relatively easier and faster, and it discourages investment in therapies for scientifically complex and rare diseases. The Dormant Therapies Act would address this issue by rewarding investment in treatments and cures for patients where there are unmet medical needs. It would allow innovators to choose a new pathway and receive a fixed year protection period for these therapies upon FDA approval. This change would shift research and development towards therapies based on scientific promise and patient need, rather than patent life. It also would reward investment in treatments and cures for complex diseases where it takes longer to develop safe and effective therapeutics.

SUBTITLE M—NEW THERAPEUTIC ENTITIES

The New Therapeutic Entities Act (Section 1241), led by Rep. Gus Bilirakis (R-FL), would extend exclusivity for two years for significant improvements to existing molecules under Section 505(b)(2) of the FFDCA. These improvements could include developing new delivery systems, new drug combinations, and new formulations that lead to less adverse events and increase patient benefits and adherence.

SUBTITLE N— ORPHAN PRODUCT EXTENSIONS NOW

This Orphan Drug Extension Act (Section 1261), led by Reps. Gus Bilirakis (R-FL) and G.K. Butterfield (D-NC), would provide six months of additional market exclusivity for a drug if the company establishes that the drug treats a rare disease and receives a rare disease indication from the FDA on its label.

TITLE II—BUILDING THE FOUNDATION FOR 21ST CENTURY MEDICINE, INCLUDING HELPING YOUNG SCIENTISTS

SUBTITLE I—COMBINATION PRODUCTS

Sections 2141-2142, led by Rep. Gus Bilirakis (R-FL), would require FDA to set forth additional guidance on the review process for products that include both drugs and devices.

SUBTITLE J—MODERNIZING REGULATION OF DIAGNOSTICS

This provision (Section 2161) includes placeholder language.

SUBTITLE L— NIH – FEDERAL DATA SHARING

This provision (Section 2201), led by Health Subcommittee Chairman Joe Pitts (R-PA), would require those receiving NIH grants to share their data, subject to confidentiality and trade secret protections.

SUBTITLE N—21ST CENTURY CHRONIC DISEASE INITIATIVE ACT

This provision (Section 2241) would require the Secretary of Health and Human Services (HHS) to develop a plan to carry out a longitudinal study designed to improve the outcomes of patients with chronic disease.

SUBTITLE O— HELPING YOUNG EMERGING SCIENTISTS

These sections (2261-2262), authored by Rep. Andy Harris (R-MD), would establish a program at NIH to help young emerging scientists.

SUBTITLE P—FOSTERING HIGH-RISK, HIGH-REWARD SCIENCE

This provision (Section 2281), led by Rep. Andy Harris (R-MD), would require NIH to support projects that pursue innovative approaches to major challenges in biomedical research that are high-risk, but have the potential to lead to breakthroughs.

TITLE III—MODERNIZING CLINICAL TRIALS

SUBTITLE A—CLINICAL RESEARCH MODERNIZATION ACT

This provision (Section 3001-3002), led by Reps. Cathy McMorris Rodgers (R-WA) and Diana DeGette (D-CO), would help streamline the institutional review board (IRB) process, particularly for clinical trials conducted at multiple sites, by minimizing regulatory duplication and unnecessary delays.

SUBTITLE B— BROADER APPLICATION OF BAYESIAN STATISTICS AND ADAPTIVE TRIAL DESIGNS

This provision (Section 3021), led by Rep. Chris Collins (R-NY), would encourage the broader application of Bayesian statistics and adaptive trial designs.

SUBTITLE C—POST-APPROVAL STUDIES AND CLINICAL TRIALS

This provision (Section 3031), sponsored by Rep. Chris Collins (R-NY), would ensure that FDA and sponsors periodically evaluate whether post-approval studies remain scientifically warranted.

SUBTITLE D—PEDIATRIC RESEARCH NETWORK IMPROVEMENT

This provision (Section 3041), led by Rep. Cathy McMorris Rodgers (R-WA), would require NIH to implement the National Pediatric Research Network Act, which was established as part of the PREEMIE Reauthorization Act (P.L. 113-55).

SUBTITLE E—GLOBAL PEDIATRIC CLINICAL TRIAL

This provision (Section 3061), led by Health Subcommittee Chairman Joe Pitts (R-PA), would set forth a Sense of Congress that NIH and FDA should work with European Union, industry, and others to establish a global pediatric clinical trial network.

TITLE IV—ACCELERATING THE DISCOVERY, DEVELOPMENT, AND DELIVERY CYCLE AND CONTINUING 21ST CENTURY INNOVATION AT NIH, FDA, CDC, AND CMS

SUBTITLE A— NATIONAL INSTITUTES OF HEALTH

Section 4001 – NIH research strategic investment plan

Section 4001, based on the work of Rep. Andy Harris (R-MD), would require NIH to issue a strategic plan.

Section 4002 – Biomedical research working group to reduce administrative burden on researchers
Section 4002, led by Rep. Andy Harris (R-MD), would establish a working group composed of NIH and stakeholders to provide recommendations on how to streamline the grant process for researchers.

Section 4004 – Increasing accountability at the National Institutes of Health
Section 4004, based on the work of Chairman Emeritus Joe Barton (R-TX) and Rep. Andy Harris (R-MD), would provide the NIH Director with more authority over the institutes and centers at NIH.

Section 4007 – Additional Funding for NIH Common Fund
Section 4007 would authorize additional funding for the NIH Common Fund.

Section 4008 – Additional Funding for NIH Brain Research
Section 4008, based on the work of Rep. Tim Murphy (R-PA), would authorize funding for the NIH's BRAIN initiative.

SUBTITLE B—ADVANCING RESEARCH FOR NEUROLOGICAL DISEASES
This provision (Section 4021), led by Reps. Michael C. Burgess, M.D. (R-TX) and Chris Van Hollen (D-MD), would require the Centers for Disease Control and Prevention (CDC) to set up a surveillance system for neurological diseases.

SUBTITLE C—VACCINE ACCESS, CERTAINTY, AND INNOVATION
These provisions (Sections 4041- 4048, 4061-4063), led by Rep. Renee Ellmers (R-NC), would provide certainty and transparency with respect to the regulation of vaccines, including with respect to CDC and CMS.

SUBTITLE E— FDA HIRING, TRAVEL, AND TRAINING
This provision (Section 4101) contains placeholder language.

SUBTITLE F—FDA SUCCESSION PLANNING
These provisions (Sections 4121-4122), led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), would ensure that FDA staff has the ability to continue to improve their expertise and that FDA develops a succession plan for management positions.

SUBTITLE G— DISPOSABLE MEDICAL TECHNOLOGIES
This provision (Section 4141), led by Rep. Renee Ellmers (R-NC), would reform the coverage requirements under the Medicare program for certain disposable medical technologies.

SUBTITLE N—MEDICARE PART D PATIENT SAFETY AND DRUG ABUSE PREVENTION
This provision (Sections 4281-4284), led by Reps. Gus Bilirakis (R-FL), Ed Whitfield (R-KY), Billy Long (R-MO) and Ben Ray Lujan (D-NM), would help prevent high-risk Medicare beneficiaries from abusing controlled substances.

SUBTITLE O— ACCELERATING INNOVATION IN MEDICINE
This provision (Section 4301), led by Rep. Erik Paulsen (R-MN), would establish a program that allows for patients to access medical device treatments sooner than otherwise would be available.

SUBTITLE P—MEDICARE PHARMACEUTICAL AND TECHNOLOGY OMBUDSMAN
This provision (Section 4321), led by Rep. Susan Brooks (R-IN), would establish an ombudsman at CMS to allow medical device and pharmaceutical companies to appeal decisions and better understand the reasoning behind Medicare coverage decisions.

SUBTITLE S—CONTINUING MEDICAL EDUCATION SUNSHINE EXEMPTION

This provision (Section 4381), based on H.R. 293, which was introduced by Reps. Michael C. Burgess, M.D. (R-TX) and Peter DeFazio (D-OR) would clarify that peer-reviewed journals, journal reprints, journal supplements, and medical textbooks are excluded from the reporting requirement under the Sunshine Act.

TITLE V—MODERNIZING MEDICAL PRODUCT REGULATION

SUBTITLE B—21ST CENTURY MANUFACTURING

This provision (Section 5021), led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), would require FDA to update its guidance regarding novel manufacturing techniques.

SUBTITLE C—CONTROLLED SUBSTANCE MANUFACTURING AND EXPORTS

This provision (Section 5041), led by Health Subcommittee Chairman Joe Pitts (R-PA), would provide U.S. pharmaceutical companies with a level-playing field regarding controlled substances exports.

SUBTITLE D—MEDICAL DEVICE REFORMS

Section 5061 – Third-party quality system assessment

Section 5061, led by Rep. John Shimkus (R-IL), would allow FDA to rely on third party accredited bodies to certify minor manufacturing changes.

Section 5062 – Valid scientific evidence

Section 5062, led by Rep. John Shimkus (R-IL), would clarify that valid scientific evidence includes well-documented, real world evidence gathered from clinical registries and studies published in peer-reviewed journals.

Section 5063 –Training and oversight in least burdensome means concept

Section 5063, led by Rep. John Shimkus (R-IL), would ensure that FDA reviewers are trained on the least burdensome concept.

Section 5064 – Recognition of standards

Section 5064, authored by Rep. John Shimkus (R-IL), would improve the process of government recognition of appropriate standards set by the medical community.

Section 5065 – Notification of marketing of certain class I devices

Section 5065, led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), would streamline the process of marketing Class I medical devices.

Section 5066 – General and specific uses

Section 5066, led by Rep. John Shimkus (R-IL), would streamline the 510(k) process for medical devices.

Section 5067 – Humanitarian device exemption application to in vitro diagnostics

Section 5067, led by Rep. Leonard Lance (R-NJ), would allow FDA the authority to apply the Humanitarian Device Exemption (HDE) to areas that impact more than 4,000 patients where the public health requires a greater availability to treat or diagnose such patients and there is no alternative.

Section 5068 – Advisory committee process

Section 5068, led by Rep. John Shimkus (R-IL), would streamline the FDA committee advisory process.

SUBTITLE E—SUPPLY CHAIN SECURITY FOR DEVICES

This provision (Sections 5081-5088), led by Rep. Bob Latta (R-OH), would establish a national framework for licensure of medical device wholesalers and third-party logistics providers, similar to what Congress enacted for prescription drugs in the Drug Quality and Security Act (P.L. 113-54)