SUMMARY OF SUSPENSION DOCUMENT FOR H.R. 5651

The United States has led the global medical device and biopharmaceutical industries for decades. This leadership has made the U.S. the medical innovation capital of the world, bringing hundreds of thousands of high-paying jobs to our country and life-saving devices and drugs to our nation's patients. U.S. medical device-related employment totals over 2 million jobs, and these are good, rewarding jobs as employees in the device industry earn an average of $60,000 per year. The U.S. biopharmaceutical industry is responsible for over 4 million U.S. jobs.

Unfortunately, our nation's device and biopharmaceutical leadership is under threat. The threat comes from Food and Drug Administration's (FDA) unpredictable, inconsistent, non-transparent and inefficient regulation of devices and drugs. Because of the lack of predictability, consistency, transparency and efficiency, U.S. device and drugs jobs have gone overseas and foreign patients, not U.S. patients, have been the first to benefit from U.S. innovation.[1] [2] [3]

The significant policy reforms contained in H.R. 5651, coupled with the accountability and transparency measures in the user fee agreements, would address the lack of predictability, consistency, transparency and efficiency at FDA so we can ensure that the U.S. remains the world leader in medical innovation, device and drug jobs stay in the U.S., U.S patients benefit first from new device and drugs, and FDA no longer wastes U.S. taxpayer and innovators' resources because of bureaucratic red tape.

On May 10, 2012, the Energy and Commerce Committee voted 46-0 in favor of H.R. 5651. The Suspension Document contains technical changes and the addition of language in Section 863 that the Committee believes will result in the bill reducing mandatory spending by roughly $350 million over ten years. With the addition of this language, H.R. 5651 is pay-go compliant.

Title I- The Prescription Drug User Fee Act Reauthorization

Under this section, the drug industry would pay over $700 million in FY 2013 and higher amounts in the remaining four years. In exchange, FDA would commit to the following: (1)

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[1] Even the Administration's own Jobs Council believes that regulatory uncertainty at FDA has caused our medical innovation ecosystem to suffer. The Council commented: "[O]ur medical innovation ecosystem is in jeopardy. Investment in the life sciences area is declining at an alarming rate because of the escalating cost, time, and risk of developing new drugs and devices. While many factors have contributed to this decline . . . an important factor is the uncertain FDA regulatory environment." President's Council on Jobs and Competitiveness, Interim Report, *Building Confidence: Five Common-Sense Initiatives to Boost Jobs and Competitiveness*.

[2] A study by the California Healthcare Institute and The Boston Consulting Group found that in 2010, the European Union (EU) approved applications for new medical devices on average about 46.8 months faster than the U.S.

[3] Last July, the Energy and Commerce Committee heard testimony from American patients who had to travel overseas to access U.S. innovation. One of these patients, Marti Conger, had to travel to England to get access to a device made by a company located 40 miles from her home in California.

meeting performance goals regarding the timely review of drug applications; (2) increasing interaction between drug sponsors and FDA during the review process; (3) improving engagement with patients, including those with rare diseases; (4) providing more granular data from its review divisions to improve transparency, and (5) undertaking an independent assessment by a third party of FDA's performance in FDA's reviewing applications for novel drugs.

Title II- The Medical Device User Fee Act (MDUFA) Reauthorization

Industry would pay $595 million in user fees for FY 2013-2017 in return for significant changes in FDA performance and accountability. The bill would authorize the following changes: FDA would report its total time for reviewing devices; the review process would include greater interaction between sponsors and the agency; an independent entity would review the device approval and clearance process; and FDA would have to implement a corrective action plan to address deficiencies found in the independent review.

Title III: Generic Drug User Fee Act (GDUFA)

This title would authorize the new Generic Drug User Fee Act (GDUFA). The proposed generic drug user fee would provide additional resources for the review and regulation of generic drugs. Under GDUFA, the generic drug industry would pay approximately $1.5 billion over five years. The industry agreed to this fee in return for faster and more predictable review of generic drug applications and increased inspections of drug facilities.

Title IV: Biosimilars User Fee Act (BSUFA)

Title IV contains language that would authorize the new Biosimilars User Fee Act (BSUFA). This user fee would apply to products approved under the abbreviated approval pathway for biological products shown to be biosimilar to an FDA-licensed biological product. BSUFA would authorize the following four types of fees: application, product, establishment and biosimilar product development. The first three would be set equal to the PDUFA rate for each type of fee. The product development fee would be set at 10 percent of the PDUFA application fee.

Title V: Best Pharmaceuticals for Children Act (BPCA) and Pediatric Research Equity Act (PREA)

This title includes language from legislation offered by Mr. Rogers, Ms. Eshoo and Mr. Markey (H.R. 4274) that would permanently authorize the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA). BPCA and PREA foster the development of prescription drugs for children and safe use of drugs by children. BPCA was established in 1997. It provides FDA with the authority to grant a six-month marketing exclusivity period to a manufacturer of a drug in return for FDA-requested pediatric use studies and reports. BPCA has been very successful in spurring research in the pediatric population for rare conditions and encouraging companies to undertake research where there was no incentive to do so. For example, almost 50 percent of all of the oncology products that have received pediatric exclusivity since BPCA's enactment were for drugs for rare conditions. This success is

not limited to oncology. According to GAO, BPCA has led to additional research into over 16 different broad categories of disease.

The Pediatric Research Equity Act (Section 505B of the FFDCA) requires a manufacturer of a drug or biologic who submits an application to market a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration to also submit a pediatric assessment for that product.

Title VI: FDA Administrative Reforms

Section 601: The section would improve FDA's guidance process by ensuring public participation in the guidance development process and requiring FDA to finalize draft guidance documents. This section would reduce administrative costs by requiring FDA to review/revise guidance documents every five years to make them less burdensome on the agency and industry

Section 602: This section would improve FDA's conflict of interest rules so the agency's advisory committees have access to the most knowledgeable experts. Congress established these rules as part of the 2007 reauthorization, and they have led to significant vacancy rates, especially for advisory committees for rare diseases. This section would reduce administrative costs by ending the waiver limits and the burden associated with complying with them.

Section 603: This section would require electronic submission of drug, generic drug, biologic, and biosimilar applications following issuance of a final guidance by the Secretary. This section would reduce administrative costs by making reviews more efficient through the use of electronic submissions instead of paper.

Section 604: The section would prohibit the FDA from issuing guidance on the regulation of laboratory developed tests unless it notifies the Committee of its intent to take such action 60 days prior to the issuance of the guidance.

Title VII: Medical Device Regulatory Reforms

Sections 701: This section would return the Investigational Device Exemption (IDE) approval process to the standard authorized by the statute and implementing regulations. IDEs are needed for manufacturers to conduct clinical trials on devices. This section would significantly reduce administrative costs. The proposed guidance by FDA would have turned the IDE approval process into virtually another device approval process. This section would end the double reviews.

Section 702: This section would reaffirm the least burdensome provisions added during the Food and Drug Administration Modernization Act of 1997 (FDAMA). This section would reduce costs by ending unnecessary information requests by reviewers, which extends reviews and wastes money.

Section 703: This section would require FDA reviewers to provide the scientific or regulatory rationale for major decisions, and it would establish an expedited appellate process for challenging those decisions. This section would reduce costs by ending unnecessary information

requests and time-consuming, endless reviews. The section would also inform applicants of the information FDA would require for a submission. Poor applications waste FDA resources. This section would lead to better applications and less time and resource-consuming multi-cycle reviews.

Section 704: This section would require FDA to provide review decision summaries for those 510(k) clearances involving clinical data. This section would reduce costs by ensuring that FDA reviewers, management and industry understand what is required for submissions. This section also would lead to better application submissions, reducing the need for multi-cycle reviews.

Section 705: This section would withdrawal FDA's recent guidance documents on medical device modifications and prevent FDA from issuing any new guidance on the subject until it completes a report to Congress on the subject. Until FDA issues a new guidance on the subject, the 1997 guidance would be the standard. This section would substantially reduce costs by forcing the withdrawal of this guidance, which by a number of estimates, could lead to an increase of 510(k) submissions of 300 to 500%.

Sections 711-712: The section would reaffirm the mandate of the Safe Medical Devices Act of 1990 (and recommendation of the Government Accountability Office (GAO)) that FDA require premarket approval applications from pre-amendment Class III devices or move them to Class II. Second, this legislation would codify GAO recommendations as to FDA's use of its current device recall authority.

Section 721: This section would streamline the de novo classification process, which is used for novel, innovative devices, by striking the requirement that a sponsor receive a Not Substantially Equivalent finding before entering the de novo process, thus making the process more efficient. This section would reduce costs by allowing FDA to review a de novo application without first reviewing it as a 510(k), ending double reviews.

Sections 731-732: These sections would allow FDA to enter into agreements with foreign countries on harmonizing inspections and common international labels of medical devices. It also would provide transparency regarding FDA's involvement in international activities.

Sections 741-742: These sections would reauthorize the third party review and third party inspection programs. This section would reduce costs by allowing to FDA to partner with foreign countries in inspections.

Sections 751: This section would extend the exemption on profit for pediatric devices that have been granted Humanitarian Device Exemptions to include certain devices intended for use in adults. The Humanitarian Device Exemption is used to provide access to medical devices with conditions that affect 4,000 individuals or fewer.

Section 761: In 2007, FDA was required to develop a regulation that would allow for a unique identifier on each medical device. FDA has not yet promulgated the regulation. The section would require FDA to issue the regulations not later than 120 days after enactment.

Section 762: This section would extend FDA's "Sentinel" postmarket risk identification and evaluation system to include medical devices. The section would require FDA to engage stakeholders when developing the system for devices.

Section 771: This section would clarify FDA's policy on custom devices for small populations.

Section 772: This section would reauthorize, at the current authorization level, demonstration grants for non-profit consortia to promote pediatric device development.

Section 773: Under this section the Secretary of HHS, in consultation with the FDA Commissioner, the National Coordinator of Health Information Technology, and the Chairman of the Federal Communication Commission, would be charged with developing a report containing (1) recommendations on an appropriate regulatory framework for health information technology that is risk based and (2) a strategy to avoid regulatory duplication. This report would save money overall as it would force the regulators to speak to one another to avoid duplication of resources.

Title VIII: Drug Regulatory Reforms

Section 801: This section would require FDA to modernize its drug registration and listing so it has accurate and up-to-date information on drug manufacturing facilities. This section would save money as FDA would have an updated list of facilities and no longer conduct unnecessary inspections.

Section 802: This section would allow FDA to inspect those drug facilities that pose the greatest risk to safety whenever it needs to inspect them.

Section 803: This section would force FDA to build this quality risk management into its good manufacturing practices (GMP) authority.

Section 804: This section would bar the entry of imported drugs from an establishment that is deemed to have delayed, limited or denied an inspection.

Section 805: This section would allow FDA to destroy counterfeit or adulterated imported drug products of minor monetary value.

Section 806: This section would allow FDA to detain, for a reasonable period, drugs found during inspection which the inspecting officer has reason to believe are adulterated or misbranded.

Section 807: This section would provide for 20 years of imprisonment for any person who knowingly holds, sells or dispenses a counterfeit drug.

Section 808: This section would require drug facilities and importers to have a unique number. This section would save money as FDA would have an updated list of facilities and no longer conduct unnecessary inspections.

Section 809: This section would require certain information from importers so FDA can implement a risk-based approach to import screening.

Section 810: This section would require drug importers to register with the FDA.

Section 811: This section would require a manufacturer to notify the FDA if it knows the use of the drug could lead to serious injury or death, or if the drug is stolen, or if it is counterfeited.

Section 812: This section would allow the FDA to share certain information with trusted foreign countries to better leverage resources.

Section 813: This section would provide for explicit extraterritorial federal jurisdiction over a violation of the Federal Food, Drug and Cosmetic Act so U.S. authorities could hold accountable those violating the Act, like those who caused the heparin deaths.

Section 814: This section would increase penalties for intentional adulteration of drugs that could cause serious adverse health consequences or death.

Section 815: This section would allow the FDA to request inspection documents in lieu of or in advance of conducting a physical inspection.

Section 821-823: These sections would streamline and modernize the regulation of medical gas.

Section 831-835: These sections would provide new incentives for the development of antibiotics to address the public health threat of antibiotic resistance. The provision also would require the FDA to issue guidance to improve the clinical development of antibiotic drugs.

Section 841-843: These sections would improve access to the Accelerated Approval pathway, including for drugs that treat rare diseases.

Section 851: The section would reauthorize the Critical Path Public-Private Partnerships program at the current level.

Section 861: This section would reauthorize the enantiomer exclusivity authorized in 2007. An enantiomer is one of a pair of compounds that have a mirror image relationship.

Section 862: Under current law, if a generic drug manufacturer challenges a brand manufacturer's patent as being either invalid or would not be infringed, and if it is the first generic drug manufacturer to make such challenge or makes it on the same day as the first challenger, it can be awarded 180 days of exclusivity from other generic competition. Except in certain circumstances, if FDA does not grant tentative approval within 30 months of the filing of the generic drug application, the generic company forfeits the 180-day exclusivity period. The provision would temporarily increase that tentative approval time period to 45 months. (The current average time for FDA to approve a generic drug application is 31 months.) This 45-month period would be gradually phased back down to 30 months as the FDA eliminates the backlog of pending generic applications pursuant to the generic drug user fee agreement.

Section 863: Certain citizen petitions ask the agency for a stay of action related to FDA approval of a pending application submitted under section 505(b)(2) (a new drug application that relies on safety and efficacy data produced by someone else) or 505(j) (generic application) of the Act due to scientific or medical questions about the application. The FDA is required to take final agency action on the petition within 180 days. This section would require the FDA to take final agency action on the petition within 150 days. Since Committee passage, this section has been amended so the policy also applies to biosimilar applications, thus making the bill pay-go compliant.

Section 864: Current law allows FDA to deny a generic application if the drug the generic application is referencing was withdrawn for safety or effectiveness reasons. Current FDA regulations allow a company to petition the FDA to determine if the reference drug was withdrawn for safety or effectiveness reasons. However, there is no timeframe under which the FDA must respond to the petition, and there have been instances of decision times lasting over two years. This section would require the FDA to respond to these petitions within 270 days which should result in the quicker approval of generic drugs.

Section 865: This section would create a demonstration project that provides priority review vouchers to companies that develop a drug for a pediatric rare disease. The voucher would be redeemed by the company for a subsequent application or could be transferred to another company. The section would require the GAO to conduct a study for Congress on the effectiveness of the program. The study is required to be completed one year after the third priority review voucher is issued. The Secretary could no longer issue new priority review vouchers one year after the third voucher is issued so Congress may determine whether to continue the incentive.

Section 866: This section would task the Secretary of HHS with reviewing and reporting to Congress on current Federal initiatives and identifying gaps and opportunities with respect to ensuring the safe use of prescription drugs with the potential for abuse.

Section 867: This section would make the Risk Evaluation and Mitigation Strategy (REMS) system more efficient by allowing for minor modifications of a drug's REMS. Certain drugs are required to have REMS to ensure that the benefit of the drug outweigh the risk. Currently the law is unclear as to whether sponsors can make minor modifications to the REMS without the need to have a full reassessment of the REMS.

Section 868: This section would require FDA to ensure that opportunities exist for consultation with external experts on products for rare diseases.

Section 869: This section would expedite the development and review of a drug designated a "breakthrough therapy" through increased interaction with the agency during the development process. To achieve this designation, a drug must be intended to treat a serious or life-threatening disease or condition, and preliminary clinical evidence must indicate that it may demonstrate substantial improvement over existing therapies.

Section 870: This section would reauthorize FDA's orphan drug grant program at the current level for five years. This grant program helps facilitate the development of orphan disease products.

Title IX: Drug Shortages

Section 901**:** This section would modify existing reporting requirements for manufacturers of drugs that are life-supporting, life-sustaining, and intended for use in the prevention or treatment of a debilitating disease or condition. This would ensure better information flow to the FDA and help the agency to better marshal its resources to prevent anticipated drug shortages and mitigate existing shortages.

Section 902**:** The section would require FDA to maintain a drug shortage list and provide patients, providers and the public with such information in order to prevent, mitigate, and manage drug shortages on the ground. The bill includes safeguards to prevent the release of confidential business information or information that could adversely affect public health.

Section 903: The section would require the Drug Enforcement Administration (DEA) to provide timely approvals or denials of increases in quotas of controlled substances in instances where such an increase could help address a drug shortage.

Section 904: This section would require the FDA to expedite the approval of manufacturing changes that could help prevent or mitigate a drug shortage while ensuring good manufacturing practices continue to be used.

Section 905**:** The section would authorize the GAO to conduct a study to examine the causes of drug shortages and issue recommendations on how to prevent or alleviate a drug shortage. This provision would provide needed data on how the regulatory framework, manufacturing challenges, or other factors contribute to drug shortages, as well as recommendations to address such issues.

Section 906-907: These sections would require the FDA and DEA to report annually on their efforts on drugs shortages based on the metrics set forth by Congress.

Section 908: The section develops interim rules to allow repackaging of drugs into smaller units by hospitals within the same hospital system for drugs that are in shortage. The interim rules would