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Senator Lamar Alexander
Senator Richard Burr
Committee on Health, Education, Labor and Pensions
United States Senate
Washington, D.C. 20003

Chairman Alexander, Senator Burr, and Members of the U.S. Senate Health, Education, Labor and Pensions (HELP) Committee:

Public Citizen, a nationwide consumer protection organization with over 350,000 members and supporters, writes to express concern over the report, *Innovation for Healthier Americans.*[1]

We welcome the opportunity to discuss the critical issues raised in this report, as we have a shared interest in advancing medicine and research in the 21st century. However, we are concerned that some would seek to achieve this at the expense of science and public safety. This report has implications that would lead to deregulation of a vast scope of American medicine and healthcare. For example, it implicitly calls for lowering the standards for Food and Drug Administration (FDA) drug approval. Currently, the FDA usually requires that new drugs be approved based on evidence from one or more large, randomized, well-controlled clinical trials, or "Phase III" trials, to demonstrate safety and effectiveness. Without these trials, nearly one in three drugs approved could be unsafe, ineffective, or both (see below). This report can be interpreted as advancing approval based on much weaker evidence, including smaller clinical data sets and alternative endpoints. We oppose related efforts to deregulate medical devices without adequate safety precautions.

We understand that the report is a way to tackle similar priorities being undertaken by the U.S. House of Representatives through their 21st Century Cures initiative.[2] The HELP Committee has historically been bipartisan and this report can begin a productive conversation amongst stakeholders to delve into important health and safety matters over the next several months. Attached you will find comments concerning this report and ideas that offer a way forward.

Public Citizen has been working for more than forty years to ensure that Americans have access to safe medicines and medical devices. We hope that you will work with patient, public health, and consumer stakeholders to ensure that any reform efforts ensure that approved drugs and devices marketed in the U.S. are safe and effective for patient use.

Sincerely,

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[1] http://www.help.senate.gov/imo/media/Innovation_for_Healthier_Americans.pdf.
[2] Upton F. "Upton Applauds New Report from Senators Alexander and Burr as #Cures2015 Continues." January 29, 2015. http://1.usa.gov/1z9uORE.

Concerns

Ideas offered in the *Innovation for Healthier Americans* can potentially emerge as policies that compromise safety and science. We saw many such bad policies described in the discussion draft released as part of the House of Representatives' 21st Century Cures initiative. We cannot undermine the FDA, drive up health care costs, and harm patients and public health. We must not:

- **Dismantle clinical trials, the gold standard for ensuring drug safety and effectiveness.** Clinical trials are the gold standard for assessing drug safety and effectiveness, developed by scientific consensus over decades. Large, well-designed, randomized controlled Phase III clinical trials are critical for weeding out bad drugs: over a third of the drugs that enter Phase III testing fail to gain FDA approval, with nearly 90 percent of failures due to safety and efficacy problems.[3,4] We should not abandon the Phase III testing requirements and undermine the drug approval process. Without these trials, nearly one in three drugs approved could be unsafe, ineffective, or both.

- **Lengthen drug company monopolies to gouge consumers and drive up costs**. We should not lengthen the monopoly period available for most drugs, as suggested in the report. Such anti-competitive monopolies drive up health care costs and hurt patients. Furthermore, they are unnecessary, as drug companies continue to make record-breaking profits.

- **Remove dangerous high-risk medical devices from FDA oversight.** We cannot gut already weak FDA regulation of medical devices. The FDA must retain and expand oversight over all moderate and high-risk devices (including the highest-risk devices like brain stents or artificial heart valves), and companies should not be authorized to regulate themselves when it comes to changes to such devices. Even simple changes can be dangerous, as illustrated by recent cases of catastrophic bone and tissue damage caused by changes to the material used in hip implants.[5] Self-regulation for these types of changes would be disastrous, resulting in numerous tragic outcomes for patients.

A Way Forward

Instead of undermining drug and device safety, we should:

- **Improve access to clinical trials information.** We support improving the usability of data submitted to the clinical trials registry and results data bank. In particular, we support a system that would make de-identified clinical trial data from clinical trials available for the purposes of conducting further research. However, some proposals do not take this idea far enough to make it effective: Publication of clinical trial data for qualified clinical trials should be *mandatory* in all cases (with steps taken to protect patient privacy). Such data could provide a huge boost to public health by increasing the amount of information that is available regarding the risks and benefits of FDA-approved drugs and medical devices. It could also accelerate the pace of innovation by helping researchers learn from each other's mistakes -- details that often remain published under the current system. Sharing of clinical data was recently recommended by the Institute of Medicine,[6] and the European Medicines Agency, FDA's European counterpart, has recently moved forward with a mandatory publication policy for data submitted to support regulatory approvals.[7]

- **True Medical Device Reform for the 21st Century.** Currently, too many moderate and high-risk medical devices are cleared for marketing based on the conclusion that products are "substantially equivalent" to products already on the market, usually without any clinical testing to show the devices are safe and effective. This process creates unacceptable risks for patients, and is so deficient that a report by the Institute of Medicine recommended that the entire system be scuttled and replaced with a new

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[3] DiMasi JA, Feldman L, Seckler A, Wilson A, Trends in risks associated with new drug development: Success rates for investigational drugs. *Clin Pharmacol Ther* 2010;87(3):272-7.
[4] Arrowsmith J. Trial watch: phase III and submission failures: 2007-2010. *Nat Rev Drug Discov* 2011;10(2):87.
[5] Meier B, Concerns over "metal on metal" hip implants. *New York Times*. March 3, 2010. http://nyti.ms/1A4k85S.
[6] Silverman, E. Institute of medicine urges broader sharing of clinical trial data. *The Wall Street Journal*. January 14, 2015. http://on.wsj.com/14Cbtfg.
[7] European Medicines Agency. European Medicines Agency agrees policy on publication of clinical trial data with more user-friendly amendments. December 6, 2014. http://bit.ly/1lkvX1A.

framework that could assure safety and effectiveness throughout a device's life cycle.[8] Such changes could positively transform device regulation in the next century, and are long overdue.

- **Share data generated through NIH-funded research.** We support the sharing of data generated through publicly-funded research. However, any proposal to improve such data sharing must actually advance the status quo. The NIH already asks researchers to include a data-sharing plan in any NIH-funded grants exceeding $500,000 (or state why data sharing is not possible), and the agency has the authority to enforce compliance.[9] However, the current policy does not ensure public access, as we found when our recent request for data from the publicly funded Surfactant, Positive Pressure, and Oxygenation Randomized Trial (SUPPORT) study was rejected by the research institution that carried out the study.[10] Without further prompting from Congress, the NIH will likely continue to accept whatever data-sharing arrangement is proposed by the researchers when they apply for grant funding, which may not include appropriate transparency. To prevent this from happening and ensure broader dissemination of publicly-funded research, all data from NIH-funded grants should be made publicly available within a specified time period, through a centralized database.

- **Fund research for drugs not covered by patents.** We support incentivizing research into compounds for which patent protection and other exclusivity has expired. However, we are concerned that any proposal to encourage this type of research might be subverted as a pipeline to subsidize ever-greening (making minor product adjustments in order to extend monopoly protection and high profits). To prevent this, funding should be provided in a targeted way towards carefully defined high-needs cures. In addition, periodic reports should be required to document the indications funded and their relation to the original FDA approval, and any such program should apply to all products, not just FDA-approved drugs, as there may be valuable potential compounds that lack patent protection and have also never been approved by the FDA for any use.

- **Publish briefing materials for all Advisory Committee Meetings.** The FDA is required by law to make materials available in advance of a meeting to allow for meaningful public input. However, there is no statute requiring distribution before a certain time period in advance of the meeting, and FDA practice has often been to publish such materials not later than two days before the date of the meeting. This affords little time for individuals wishing to speak during the open public hearings at advisory committee meeting to prepare or analyze the extensive information included in the briefing packet. Moreover, the requirement is unnecessary, as the materials are often prepared and distributed to advisors weeks before the meeting. We recommend that a new provision be added to the law requiring all briefing materials to be published 14 days in advance of any advisory committee meeting.

- **Provide drug recall authority.** The FDA currently lacks authority to require a pharmaceutical company to recall a drug from the market. Instead, the FDA may request that a drug manufacturer initiate a voluntary recall.[11] Mandatory drug recall authority is long overdue, particularly in light of the recent example of NuVision Pharmacy's repeated refusal of recall requests made clear that companies that manufacture drugs are not always responsive, forcing the FDA's hand.[12]

- **Institute civil penalties for drugs and devices.** The FDA lacks authority to impose civil penalties on drug and device companies for violations of the Food, Drug, and Cosmetic Act. While selling an adulterated or misbranded drug can trigger jail time and criminal fines, the FDA rarely pushes for appropriately strong criminal penalties. There are few alternative punishments available: the FDA can issue a warning letter and press release, or pursue an injunction or seizure, both of which are time-consuming and costly. Congress should therefore grant the FDA the authority to issue civil penalties, similar to the civil fines already available for tobacco violations.

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[8] Institute of Medicine, *Medical Devices and the Public's Health: The FDA 510(K) Clearance Process at 35 Years.* National Academy of Sciences, 2011. (Brian Wolfman, former director of Public Citizen's Litigation Group, served on the committee that oversaw production of the IOM report.) A brief summary of the highlights of this report is available here: http://bit.ly/1Lv3Tlq.
[9] National Institutes of Health. NIH Data sharing policy and implementation guidance. March 5, 2003. http://1.usa.gov/1807goc.
[10] Data on file.
[11] 21 C.F.R. § 7.40.
[12] Eisler P, Schnaars C, Safety, sanitary problems prompt scores of drug recalls. October 7, 2014. http://usat.ly/1sa2V8x.