Testimony before the
Committee on Energy and Commerce
Subcommittee on Health
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Chairman Pitts, Ranking Member Pallone, and members of this committee, thank you for the opportunity to testify about the importance of the user fee agreement (UFA) legislation to patients and public health. I am also pleased to discuss three key policy initiatives that should be considered along with the UFA legislation.

Based on data, science, and non-partisan research, the Pew Health Group seeks to improve the health and well-being of all Americans by reducing unnecessary risks to the safety of medical and other consumer products and supporting medical innovation. Pew applies a rigorous, analytical approach to improve public policy, inform the public, and stimulate civic life.

Since 1992, user fee agreements have given FDA significant and sustained resources that allow the agency to review new products quickly. In fact, preliminary findings of a study that Pew has funded show that FDA reviews new drugs faster than its counterparts in the European Union and Canada.

Medical device user fees are especially important for the review of devices. An analysis commissioned by the Pew Health Group compared the Center for Devices and Radiologic Health (CDRH) with the Center for Biologics Evaluation and Research, Center for Drug Evaluation and Research, and the Office of Regulatory Affairs. The report reveals that CDRH has the highest annual attrition rate of the four centers, with nearly 10 percent of the center's science, technology, and engineering staff leaving in FY 2010. Resource issues may help explain the high attrition rates; less than half of CDRH employees surveyed agreed that their workload is reasonable and even fewer reported having sufficient resources to get their job done. For it to function as efficiently and effectively as possible, CDRH must have adequate funding.

The user fee agreement would provide FDA with additional resources to review applications and add about 200 much-needed staff members to the agency. The total fees collected over the five year period from 2013 to 2017 are expected to reach $595 million, a significant increase over the previous agreement. This will help create a more efficient center that is sufficiently resourced to better protect consumer safety and facilitate the introduction of innovative devices.

The landmark new Generic Drug User Fee agreement will hasten the review of generic drugs, as well as ensure that FDA has the resources – and the mandate – to conduct more frequent inspections of overseas drug manufacturing facilities. We urge you to pass these bills swiftly to ensure that FDA can continue its important public health activities uninterrupted.

The user fee legislation is also an important opportunity to consider key updates to the Food, Drug and Cosmetic Act that will protect Americans and support innovation. I would like to discuss three particular issues that Congress is considering and urge you to include them as part of the user fee reauthorizations:

- Drug supply chain safety,
- Medical device safety and innovation, and
- Antibiotic development.

Drug supply chain safety

Many Americans would be surprised by the rapid and profound changes in how our prescription drugs are made – and the new risks this process brings. Today, 40 percent of all finished pharmaceuticals, and 80 percent of the active ingredients and bulk chemicals in U.S. drugs, are sourced from foreign countries.

Yet FDA oversight of manufacturing has not kept pace with these changes. This puts consumers at risk and American manufacturers on an uneven playing field. While the best companies are already doing thorough assessments of their supply chains, we must make sure there is no incentive for the weaker actors to gain a competitive advantage by cutting corners.

FDA needs regulatory systems that are appropriate for today's global supply chain. I've already mentioned that the GDUFA agreement will increase FDA's ability to conduct foreign inspections. However, there is more that must be done. We should ensure that every company has appropriate supply chain controls in place, and that companies that do have such systems will not face delays at the border. At the same time, FDA needs the clear authority to refuse products when the plant that made them has denied an FDA inspection.

Today, a plant outside the U.S. knows FDA may visit only once, before the product is first approved, and then never return. That reduces the incentive to make ongoing investments in quality. The FDA should inspect plants, both domestic and overseas, based on risk, which will permit the Agency to make much more efficient use of its limited resources. However, no plant should go indefinitely without an inspection. A minimum frequency of 4 years should also be established.

Risks to the drug distribution system

The United States currently has no national system to detect or prevent incidents of counterfeiting and drug diversion. Although incidents in the U.S. are far less common than in other parts of the world, a recent example illustrates the serious nature of the risks. In February, FDA warned that cancer patients in the U.S. were exposed to counterfeit Avastin® – a critical chemotherapy agent used to treat numerous cancers. Just two weeks ago, the FDA warned that it had discovered yet another batch of counterfeits of the same drug, this time being sold in the United States under the drug's Turkish brand name.

Congress is considering an industry proposal that would result in a unique serial number being affixed to each package of drugs. While this proposal contains some good elements, including national standards for wholesaler licensure, it falls short in two crucial respects. First, the industry proposal calls for keeping track of drugs only by lot number, and a lot may include thousands of vials distributed across numerous wholesalers and pharmacies. Second, the proposed system would not routinely check for, or identify, counterfeit drugs.

However, there is a way forward. We urge the Committee to ensure that any national track and trace legislation include a date certain for unit-level traceability and for routine checks – known as authentication – to detect counterfeits.

Medical Device Safety and Innovation

Medical devices are important in improving the health of many Americans. These products include, for example, artificial hips, pacemakers, and ventilators. However, as with all medical products, there are risks associated with devices, which often enter the market with little or even no clinical data.

We urge the Committee to include legislation to create a more robust system of post-marketing surveillance, which would promote safety by identifying problematic medical devices more quickly. It would also facilitate innovation by increasing confidence in the safety of medical devices on the marketplace. This system would require:

- Adding medical devices to the Sentinel, FDA's active surveillance system – currently solely for drugs;
- Requiring that FDA issue and implement rules that assign new devices a unique identifier, like a barcode;
- Clarifying FDA's authority to order safety studies, when necessary, for high-risk products that will be used in patients – and ensuring that these studies are completed in a timely fashion;
- Clarifying that FDA can order short-term studies, known as 522 studies that assess safety and efficacy at the time a device is approved or cleared. Manufacturers would have a year to commence this post-marketing study.

We also urge Congress to give FDA the authority to classify medical devices as high, medium, or low risk without going through a lengthy regulatory process. In fact, *new* device types already can be classified by FDA in this manner. This will help ensure that devices enter the marketplace with the appropriate amount of testing—neither too much nor too little.

I would also like to comment on some of the proposals the Committee is actively considering:

1. Streamlining the *de novo* pathway: We are pleased that the Committee is considering striking the requirement that certain novel devices first go through the 510(k) process. This can speed the approval of these products without jeopardizing safety. Under current law, FDA has 90 days to review a 510(k) notification for a device that is substantially equivalent to a device that FDA has reviewed before. For a streamlined *de novo* pathway for truly innovative devices to work, FDA needs at least 120 days to properly review the new devices.

2. Changing the Investigational Device Exemption: The Committee is considering a proposal which would lower the standard by which FDA approves a clinical trial. Since even seemingly low-risk clinical trials have the potential to cause harm to the patients who agree to participate in the trials, Congress must ensure that clinical trials for untested medical devices will only be conducted if they are designed to meet the ultimate endpoints for devices: safety and efficacy.

3. Changes to rules about modifying 510(k) devices: This legislation could jeopardize patient safety by undermining FDA's ability to determine which changes to a device require a full or partial 510(k) application. The agency already has clear guidance for manufacturers about the level of documentation required for different types of changes to product design. Under the current system, minor changes require only a note to the file.

4. Voidance of guidance documents: The Committee is considering legislation that would automatically void draft guidance documents if they are not finalized within 12 months. While we share the view that FDA should finalize guidances as quickly as possible, we are concerned that this policy could have unintended consequences. First, it could divert finite FDA resources away from issuing new guidance, and, secondly and more importantly, an automatic voiding of draft guidance would leave manufacturers with even less clarity about regulatory expectations. Instead of automatic expiration of draft guidance, we propose that Congress require FDA to give regular reports on the reasons

guidances are delayed and also to provide a timetable for the finalizing of guidances that have been in draft for longer than 18 months.

5. Changing FDA's mission statement: FDA's mission is to assess the safety and effectiveness of the products it regulates while, at the same time, making sure that innovative products get to market in a timely way. Changes to the FDA mission statement should not dilute these important goals. For example, we are concerned that there could be significant unintended consequences from adding job creation and economic growth to FDA's mission statement. No product developer should have to wait on an approval decision while the FDA conducts an economic analysis. Moreover, beneficial technologies are sometimes disruptive. No life-saving device should be delayed because it will take the place of some other less effective product made by a company that may employ more people.

Generating Antibiotic Incentives Now (GAIN) Act

Let me turn to a third area, one where innovation is essential – the development of new antibiotics. Antibacterial drugs are unlike other medicines. Most drugs retain their effectiveness forever. But bacteria inevitably become resistant. Alas, the number of new antibiotics has been dwindling. Today, we find ourselves on the brink of what the Centers for Disease Control and Prevention Director Dr. Thomas Frieden has warned could be a "post-antibiotic era."[i]

We wish to thank Rep. Gingrey, Rep. Green, and their fellow co-sponsors of H.R. 2182, the Generating Antibiotic Incentives Now (GAIN) Act, for their bipartisan leadership on this important legislation that will stimulate the development of new antibiotics. This bill enjoys broad support in both chambers of Congress and on both sides of the aisle.

The GAIN Act builds on precedents set by laws such as the Orphan Drug Act. It would grant an economic incentive for the development of new antibiotics by granting additional exclusivity – that is freedom from generic competition – for certain qualified products. We recognize that this is not a change that Congress undertakes lightly, and Pew is working with Members of Congress to ensure that the bill squarely targets the development of the most-needed new drugs—those to

treat serious or life-threatening diseases, such as healthcare-associated and community-acquired pneumonia, complicated skin, intra-abdominal and urinary tract infections, sepsis, tuberculosis, meningitis, and other infections of vital organs and systems.

Conclusion

The user fee agreements are essential to an effective FDA that can foster innovation while ensuring the safety and efficacy of the products we depend. We urge Congress to swiftly reauthorize this program with three important additions - drug supply chain safety, medical device safety and innovation, and antibiotic development.

Thank you, and I look forward to answering any questions.

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[i] *Antibiotic Resistance and the Threat to Public Health, before the Committee on Energy and Commerce, Subcommittee on Health, United States House of Representatives*, 111th Congress, 2nd Session (2010) (statement of Thomas Frieden, M.D., M.P.H., Director, Centers for Disease Control and Prevention, U.S. Department of Health and Human Services).