# Public Meeting on Prescription Drug User Fee Act (PDUFA) Reauthorization

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PhRMA
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# PhRMA Supports a Strong, Fully Resourced FDA

- Discovery, development, and delivery of safe and effective innovative medicines to patients is the core mission of PhRMA and its member companies.
- Robust innovation depends on a fully funded Agency providing timely, science-based regulatory decisions and leveraging modern approaches.
- FDA is resourced through a combination of appropriated funds and user fees from the regulated industry, which allows the Agency to keep pace with the rapid increase in the number and complexity of innovative drugs and biologics entering the review pipeline.

# PDUFA Has Been Successful for FDA, Industry, and The Patients We Serve

- PDUFA has played a critical role in strengthening the FDA's ability to regulate safe and effective medicines for patients.

- PDUFA has helped to improve the efficiency and effectiveness of the first cycle review process and decrease the number of review cycles necessary for approval, ensuring that patients have timely access to safe, effective, and high-quality new drugs and biologics.

- PDUFA has lowered the average review and approval times for new drug applications (NDAs) and biologics license application (BLA).

# COVID – 19 Response and Lessons Learned

- The biopharmaceutical industry has been working collaboratively to help expedite treatments for COVID-19.
- FDA has been instrumental in providing support for biopharmaceutical companies in their efforts to accelerate the development of safe and effective vaccines and therapeutics in the fight against COVID-19.
- Industry and FDA can benefit from assessing the impact of the regulatory actions taken for the COVID response and identifying “lessons learned” for the future.
- PhRMA is committed to working with FDA and other stakeholders to advance COVID-19 lessons learned, including more predictable and timely sponsor-FDA engagement and better communication, innovative drug development tools and trial designs and manufacturing approaches, including risk-based inspections, and use of real-world evidence.

# PDUFA VII Can Enhance Patient-Centric Drug Review

- Innovative drug development tools have the potential to expedite patient access to new therapies.
- FDA can provide greater clarity to sponsors to increase the use of these tools and patient perspectives for regulatory decision-making (e.g., CID, advanced statistical methods, digital health technologies, PROs).
- PDUFA VII offers the opportunity to build upon the efforts started in PDUFA VI to advance the use of innovative drug development approaches.

# PDUFA VII Can Advance Digital Health Technologies

- Digital health technologies, including artificial intelligence/machine learning, is transforming nearly every aspect of our health care system, improving efficiency, expanding access to treatments and technologies and increased development of personalized medicine.

- PDUFA VII can advance a global framework for digital technology development and build upon the “lessons learned” during the COVID-19 pandemic, e.g.:
  - Decentralized clinical trials
  - Virtual study site visits
  - Remote data collection

# PDUFA VII Can Modernize Regulatory Evidence Generation

- Real-world data and evidence can be used to demonstrate effectiveness of a drug candidate.
- Real world evidence can enable more efficient drug development programs and lead to more timely access to innovative medicines.
- PDUFA VII can help to further modernize regulatory evidence generation and increase transparency and promote stakeholder learning around acceptable uses of innovative approaches for regulatory decision-making.

# PDUFA VII Can Optimize FDA Infrastructure, Staffing, and Resources

- PDUFA ensures the foundational aspects of the human drug review program are secure and appropriately funded.
- The funding and hiring reforms established under PDUFA VI can lead to even greater efficiency and sustainability for the program.
- PDUFA VII should continue to make improvements to enhance FDA's capabilities to hire, recruit, and retain staff:
  - Ensure continuity of operations through more predictable user fee funding levels
  - Leverage statutory authorities to bolster scientific expertise at the Agency
  - Improve transparency of the program's funding and performance needs at the Agency

# PDUFA VII Can Improve the Information Technology Infrastructure

- Information technology is a critical component to the success of the human drug review program.
- FDA will need to modernize the ongoing technological efforts to accommodate large data sets and new digital solutions, such as cloud-based submissions.
- PDUFA VII should build on ongoing efforts to establish a formal data and technology modernization framework.

# Conclusion - Thank You

- PDUFA VII will help ensure that FDA keeps pace with scientific discovery and helps bring the next generation of safe and effective new medicines and potential cures to patients.

- PhRMA looks forward to working collaboratively with FDA, patient groups and other stakeholders to enhance the existing program and make improvements where appropriate in PDUFA VII.

- The timely reauthorization of the PDUFA program is important to maintain the high level of the human drug review program performance.