Submitted electronically

December 1, 2022

The Honorable Patty Murray
Chair
Committee on Health, Education, Labor, and Pensions
United States Senate
Washington, DC 20510

The Honorable Richard Burr
Ranking Member
Committee on Health, Education, Labor, and Pensions
United States Senate
Washington, DC 20510

The Honorable Frank Pallone
Chair
Committee on Energy and Commerce
United States House of Representatives
Washington, DC 20515

The Honorable Cathy McMorris Rodgers
Ranking Member
Committee on Energy and Commerce
United States House of Representatives
Washington, DC 20515

Re: End-of-Year Legislative Package

Dear Chairwoman Murray, Chairman Pallone, Ranking Member Burr, and Ranking Member McMorris Rodgers,

I commend your leadership in passing legislation that advanced biomedical research this year, including reauthorizing the Food and Drug Administration's (FDA) user fee programs. The reauthorization, however, marked the first time that the user fees legislation has been passed without accompanying policy riders to address much-needed reforms at the FDA. As you finalize the end-of-year omnibus package, I encourage you to consider a bipartisan way forward to address important issues that will ensure a stronger, more efficient FDA and contribute to strengthening the biomedical research ecosystem. Specifically, we would like to underscore action needed in the following four areas that are most closely aligned with our work at FasterCures and where we see opportunities to drive progress through our activities:

1. Clinical Trial Diversity
2. Accelerated Approval
3. Antimicrobial Resistance (AMR)
4. National Center for Advancing Translation Sciences (NCATS)

FasterCures, a center of the Milken Institute, is driven by a singular goal: to save lives by speeding scientific advancements to all patients. With an independent voice, FasterCures works to build a system that is effective, efficient, and driven by a clear vision: collaborating with our partners to build a patient-centric system where science is accelerated, unnecessary barriers are overcome, and lifesaving and life-enhancing treatments get to those who need them as rapidly and as safely as possible.

1. **Clinical Trial Diversity**

Communities of color and underrepresented populations continue to be underrepresented in FDA trials. Appropriate representativeness of diverse patient groups in clinical trials not only assures the safety, acceptability, and efficacy of the product for all members of the population but also promotes equitable access to care by ensuring that patient groups that bear the greatest burden of the disease have access to cutting-edge products through clinical trials. FasterCures recognizes the imperative for increasing enrollment of underrepresented populations, including racial and ethnic minorities, in clinical trials and has a dedicated workstream focused on advancing this effort by:

- identifying and promoting actionable strategies for increasing diversity in the planning, design, recruitment, enrollment, and execution of clinical trials; and
- addressing diversity and inclusion in the health research workforce.

Recent legislation proposed by Congress encouraged concerted action to promote clinical trial diversity by requiring product manufacturers and clinical trial sponsors to submit diversity action plans to the FDA that include enrollment and diversity goals for their clinical trials, requiring the FDA to submit an annual report to Congress that summarizes information on the diversity action plans they have received, and requiring the FDA to release guidance on decentralizing the conduct of clinical trials by moving them from solely academic medical centers to community health centers or participants' homes. The guidance would also consider the use of resources such as digital health tools, including telemedicine, to help increase diversity in clinical trials. We welcome the inclusion of such provisions in the end-of-year omnibus package, along with other provisions to address the recruitment and training of a culturally competent workforce to support the conduct and management of clinical trials.

2. **Accelerated Approval**

FasterCures acknowledges the accelerated approval pathway's success in delivering lifesaving and life-enhancing therapies to patients while recognizing that improvements are needed. Recent proposed legislation in the House and Senate included reforms to the pathway that did not make it into the continuing resolution that reauthorized the FDA user fee programs. We encourage bipartisan action in this area to address the challenges inherent in administering this program while preserving its original goal—that is, to allow patients with little or no treatment options earlier access to promising therapies.

Since its introduction three decades ago, the accelerated approval program has been instrumental in enabling therapeutic products to reach patients more quickly because approvals are often supported by surrogate endpoints. Oncology has especially benefitted from this expedited pathway (85 percent of approved products in the last 10 years were for cancer) by enabling anti-cancer treatments to reach patients approximately 3.4 years earlier than they would have with traditional approval. The program has also been applied to rare diseases, and we are encouraged by the promise that it holds for application to several other diseases with no current treatment options for the impacted population.

Current challenges with the accelerated approval process that can be addressed through legislation include "dangling" and "delinquent" approvals (i.e., accelerated approvals for which confirmatory trials showed insufficient clinical benefit and approvals that miss their original milestones of confirmatory trials but are still marketed, respectively). Other barriers include the lack of appropriate endpoints outside of cancer—especially in rare and orphan diseases—for regulatory decision-making. Recently-proposed congressional reforms include ways to expedite the FDA's ability to withdraw products from the market that have not started the appropriate post-approval studies within the agreed-upon timeline at the time of approval to prevent "dangling" and "delinquent" approvals. Additional reforms being considered include the establishment of an intra-agency coordinating council within the FDA with the express purpose of regular evaluation to promote continuous improvement. We support such legislative reforms that will enhance the FDA's ability to expedite the delivery of medical products to patients who need them the most while also protecting the nation from potentially harmful or non-beneficial medical products.

3. **Antimicrobial Resistance (AMR)**

We urge you to consider adding the Pioneering Antimicrobial Subscriptions to End Upsurging Resistance (PASTEUR) Act (H.R.3932/S.2076) to the end-of-year omnibus package. The PASTEUR ACT has bipartisan support and will be an important step toward tackling the growing global health emergency of AMR by strengthening the antibiotic development pipeline. AMR—when pathogens like bacteria naturally adapt to the medicines we have available to treat them—is one of the greatest public health threats of our time and has been described as a "silent pandemic." Over 3 million people suffer from antibiotic-resistant infections every year in the United States, resulting in at least 50,000 deaths. Moreover, these resistant infections cost the US at least $4.6 billion in health-care expenses annually. Globally, the disease burden is staggering, with AMR being connected to nearly 5 million deaths in 2019—more than HIV or malaria. Without any concerted action, the global death toll from AMR could reach 10 million by 2050. And the COVID-19 pandemic has only helped to boost AMR growth, with rates of drug-resistant hospital-onset infections and deaths jumping at least 15 percent in the first year of the pandemic alone.

Worsening the AMR threat is the lack of new antibiotics becoming available due to a series of financial market failures surrounding their research and development. Antibiotics have a long and expensive development timeline, involve a lot of risk in their development, and are too cheap and have a low sale volume due to the need to be used only when necessary. These factors make it difficult to recoup any return on investment and have resulted in many large pharmaceutical companies leaving the field, with smaller biotechs filling this void that are very vulnerable to these financial pressures.

A potential solution is the PASTEUR Act (H.R.3932/S.2076), which seeks to implement a financial model to boost antibiotic research and development by offering guaranteed payments from the federal government to developers for successfully developing a drug and bringing it to market. The bill proposes to guarantee access to an antibiotic and delink payments away from volume sales that have hindered any return on investments. In our recent Financial Innovation Lab® report on developing models for the financing of antibiotic development, expert participants broadly agreed that implementing the PASTEUR Act would be a vital requirement for attracting and securing any future private investment that is desperately needed. In addition to potentially financing up to 14 new antibiotics, the bill also has provisions to promote the appropriate use of the drugs we currently have and ensure a domestic supply for patients. Including this bill in the end-of-year package would help protect Americans by strengthening the pipeline for new antibiotics so that we are able to treat increasingly resistant infections and tackle the challenge of AMR.

4. **National Center for Advancing Translation Sciences (NCATS)**

NCATS is a critical but often underappreciated center at the National Institutes of Health that addresses "ongoing challenges in research so that new treatments can reach people faster," focusing on "what is common across diseases and develop[ing] solutions that reduce, remove or bypass bottlenecks in the translational process." It encompasses not only the Clinical and Translational Science Awards (CTSA) network but a breadth of other unique programs, including serving as the hub for rare disease research at NIH, drug development partnerships, and the Cures Acceleration Network's transformative initiatives. During the COVID-19 pandemic, it was a driver of critical innovations such as the National COVID Cohort Collaborative (N3C), a large-scale data-sharing platform that FasterCures has strongly advocated be continued to provide infrastructure and insight for a wider array of research efforts.

The average increase across NIH's Institutes and Centers – as well as the CTSA program within NCATS – is approximately 3% in the Senate bill. FasterCures urges Congress to provide NCATS overall with a 3% increase to create better parity within NCATS and across NIH.

Thank you for the opportunity to provide comments on the end-of-year legislative priorities. It is our hope that Congress will be able to prioritize the most pressing reforms needed for biomedical research and innovation. We remain committed to providing ongoing support as you continue in this process.

Sincerely,

Esther Krofah
Executive Vice President of Health
Executive Director, FasterCures and Center for Public Health
Milken Institute