



MEMORANDUM

March 15, 2022

To: Subcommittee on Health Members and Staff

Fr: Committee on Energy and Commerce Staff

Re: Legislative Hearing on “The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight”

On **Thursday, March 17, 2022, at 10:30 a.m. (EDT), in the John D. Dingell Room, 2123 of the Rayburn House Office Building, and via Cisco WebEx online video conferencing**, the Subcommittee on Health will hold a legislative hearing entitled, “The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight.” The hearing will focus on 22 bills to streamline development and approval processes for drugs and therapeutics, strengthen program integrity, and improve diversity and equity in biomedical research.

I. H.R. 1730, THE “SPEEDING THERAPY ACCESS TODAY ACT OF 2021”

H.R. 1730, the “Speeding Therapy Access Today Act of 2021,” introduced by Reps. Bilirakis (R-FL) and Butterfield (D-NC), requires and authorizes various actions to accelerate the development of therapies for rare diseases. The bill requires the Food and Drug Administration (FDA) to establish the Intercenter Institute on Rare Diseases and Conditions. The bill also authorizes FDA to make grants to assist in developing practices related to the development and production of individualized therapies or therapies to treat very small populations, and establishes an advisory committee to advise FDA on issues related to the development of therapies to treat rare diseases.

II. H.R. 2565, THE “FDA MODERNIZATION ACT OF 2021”

H.R. 2565, the “FDA Modernization Act of 2021,” introduced by Reps. Buchanan (R-FL), Luria (D-VA), Mace (R-SC), Sherrill (D-NJ), and Boyle (R-PA), allows an applicant for market approval for a new drug to use methods other than animal testing to establish the drug’s safety and effectiveness. Alternative methods may include cell-based assays, organ chips and microphysiological systems, sophisticated computer modeling, and other human biology-based test methods.

III. H.R. 3085, THE “EQUITY IN NEUROSCIENCE AND ALZHEIMER’S CLINICAL TRIALS ACT OF 2021” OR THE “ENACT ACT OF 2021”

H.R. 3085, the “ENACT Act of 2021,” introduced by Reps. Blunt Rochester (D-DE), Herrera Beutler (R-WA), Smith (R-NJ), Curtis (R-UT), and Waters (D-CA), aims to improve diversity in clinical trials for Alzheimer’s Disease and reduce participation burden in clinical trials by requiring the National Institutes of Health (NIH) to locate more Alzheimer’s Disease Centers in areas with higher concentrations of minority groups, authorizing centers to operate clinical trials, and increasing diversity among Alzheimer’s Disease researchers. The bill authorizes \$60 million annually for fiscal years (FY) 2022 through 2026 to carry out the activities described in the bill.

IV. H.R. 3927, THE “MANUFACTURING API, DRUGS, AND EXCIPIENTS IN AMERICA ACT” OR THE “MADE IN AMERICA ACT”

H.R. 3927, the “MADE in America Act,” introduced by Rep. Carter (R-GA) and eight other original cosponsors, requires the Secretary of Health and Human Services (HHS) to submit a report to Congress on barriers to domestic manufacturing of active pharmaceutical ingredients, finished drug products, and devices that are imported from outside of the United States no later than six months after the date of enactment. It also requires the Secretary to ensure timely and effective internal coordination and alignment among the field investigators of FDA and the staff of the Center for Drug Evaluation and Research’s (CDER) Office of Compliance and Drug Shortage Program regarding the reviews of reports on drug shortages, and any feedback or corrective or preventive actions in response to such reports. The bill also requires the Secretary to publish a report on FDA’s public website on the utilization of agreements with foreign governments to recognize foreign entities’ ability to conduct inspections in accordance with FDA’s standards. Additionally, the bill requires the Secretary to post information related to inspections of facilities on FDA’s public website on an annual basis, establishes an expedited approval program for drugs or biological products that are manufactured using one or more advanced manufacturing technologies, and provides a tax credit for pharmaceutical and medical device production activities in distressed zones.

V. H.R. 4472, THE “BETTER EMPOWERMENT NOW TO ENHANCE FRAMEWORK AND IMPROVE TREATMENTS ACT OF 2021” OR THE “BENEFIT ACT OF 2021”

H.R. 4472, the “BENEFIT Act of 2021,” introduced by Reps. Matsui (D-CA) and Wenstrup (R-OH), would require FDA to consider relevant patient-focused drug development data, such as patient experience data, as part of the risk-benefit framework of the drug. The bill would also require FDA to include a description of how the patient-focused drug development data were considered as part of the risk-benefit assessment.

VI. H.R. 4511, THE “FDA ADVANCING COLLECTION OF TRANSFORMATIVE SCIENCE ACT” OR THE “FACTS ACT”

H.R. 4511, the “FACTS Act,” introduced by Reps. Burgess (R-TX) and Craig (D-MN), would require FDA to consider emergency use authorization (EUA) data and real-world evidence (RWE) gathered during a public health emergency to support premarket applications for drugs, biological products, and devices.

VII. H.R. 5030, THE “DIVERSIFYING INVESTIGATIONS VIA EQUITABLE RESEARCH STUDIES FOR EVERYONE TRIALS ACT” OR THE “DIVERSE TRIALS ACT”

H.R. 5030, the “DIVERSE Trials Act,” introduced by Reps. Ruiz (D-CA) and Bucshon (R-IN), aims to improve diversity in clinical trials and data collection for the coronavirus disease of 2019 (COVID-19) and future public health threats to address social determinants of health. This bill requires FDA to issue guidance on decentralized clinical trials to promote meaningful demographic and geographic diversity in patient engagement, enrollment, and participation. Decentralized clinical trials include those executed through telemedicine or other digital technologies to allow for the remote collection and assessment of clinical trial data. FDA may also work with foreign regulators to harmonize international regulations governing decentralized clinical trials and the use of digital health technology. In addition, HHS may support community education, outreach, and recruitment activities for clinical trials of treatments for conditions that disproportionately impact populations underrepresented in clinical trials.

The bill also specifies that drug or device manufacturers may provide, subject to some limits, free digital health technologies and other remuneration to patients in approved clinical trials without violating laws that address fraud and abuse in federal programs. Furthermore, laboratories that test for and diagnose COVID-19 must report additional demographic data, including information about social determinants of health.

VIII. H.R. 5566, THE “FINDING ORPHAN-DISEASE REMEDIES WITH ANTIFUNGAL RESEARCH AND DEVELOPMENT ACT OF 2021” OR THE “FORWARD ACT OF 2021”

H.R. 5566, the “FORWARD Act of 2021,” introduced by Reps. McCarthy (R-CA), Schweikert (R-AZ), Bass (D-CA), and O’Halloran (D-AZ), would support fungal disease research and incentivize antifungal drug development. The bill would prioritize basic research for Valley Fever and other fungal diseases, establish a blockchain pilot program so that medical researchers can more easily access clinical data for research while preserving patient privacy, and create a federal working group to coordinate research efforts on Valley Fever and other fungal diseases. It would also expedite the FDA approval process to establish new antifungal diagnostics, treatments, and vaccines approved for use in humans, and create a program that promotes public-private development of antifungal diagnostics, treatments, and vaccines. Finally, the bill would extend expedited approval pathways for Valley Fever drugs at FDA and create an FDA priority review voucher for Valley Fever to further incentivize the development of new treatments, cures, and vaccines for diseases like Valley Fever.

IX. H.R. 5585, THE “ADVANCED RESEARCH PROJECT AGENCY–HEALTH ACT” OR THE “ARPA–H ACT”

H.R. 5585, the “ARPA–H Act,” introduced by Rep. Eshoo (D-CA), would establish an independent Advanced Research Projects Agency for Health (ARPA–H) within HHS. This bill would direct the Secretary of HHS to appoint the Director of ARPA–H. The bill authorizes the

director to hire agency personnel, including program managers, to execute research projects and fulfill the mission of ARPA–H. The bill requires the Director to submit a report to Congress on the actions undertaken, and results generated, by ARPA–H no later than one year after enactment. It also requires the Secretary of HHS to enter into an agreement with the National Academies of Sciences, Engineering, and Medicine (NASEM) to study and evaluate whether ARPA–H has met its goals no later than 8 years after enactment. The bill also establishes a collaborative body of relevant agency and department leaders to advise the Director of ARPA–H and avoid duplication of efforts across the federal government.

X. H.R. 6000, THE “CURES 2.0 ACT”

H.R. 6000, the “Cures 2.0 Act,” introduced by Reps. DeGette (D-CO) and Upton (R-MI), would establish or amend a number of programs with the goal of accelerating the discovery, development, and delivery of medical treatments and cures. The bill outlines new reporting requirements for FDA on the collaboration and alignment of the regulation of digital health technologies and the current state of cell and gene therapy regulation. It also provides grants for innovative clinical trial design and patient data experience. The legislation makes modifications to FDA operations by requiring the expansion of the use of real-world evidence (RWE) at HHS, requiring additional communication between FDA and the Centers for Medicare and Medicaid Services (CMS) for Breakthrough Therapy drugs, and directing the Secretary to establish two additional FDA Centers of Excellence. It requires FDA to publish guidance on the standards and factors it will employ regarding use of other evidence, such as clinical evidence, patient registries, or other RWE, to fulfill post approval study requirements to confirm the predicted clinical benefit of a therapy approved under accelerated approval. It also directs the Secretary to convene a multi-stakeholder meeting to explore innovative ways and incentives to foster the adoption of decentralized trials.

The bill also makes a number of investments for patients and caregivers, including by establishing educational programs for caregivers, investing in health literacy, requiring more diversity in clinical trials, procuring patient experience data in clinical trials, and allowing Medicare to cover the costs of clinical trials funded by the Patient-Centered Outcomes Research Institute (PCORI). It establishes a subscription model to pay for novel antimicrobial drugs, provides federal support for the use of genetic and genomic testing for pediatric patients with rare diseases, and provides Medicare coverage for pharmacogenetic consultations by qualified clinical pharmacists and genetic counselors. Additionally, the bill creates a generally accepted standard for electronic prescribing and provides clinician-led clinical data registries with access to Medicare claims data for research to improve quality and cost efficiency by linking the data with clinical data in registries. Finally, the bill reauthorizes the Research Policy Board authorized by the 21st Century Cures Act.

XI. H.R. 6584, THE “DIVERSE AND EQUITABLE PARTICIPATION IN CLINICAL TRIALS ACT” OR THE “DEPICT ACT”

H.R. 6584, the “DEPICT Act,” introduced by Reps. Eshoo, Fitzpatrick (R-PA), and Kelly (D-IL), directs the FDA Commissioner to amend certain regulations to increase clinical trial diversity. The bill requires Investigational New Drug (IND) and Investigational Device

Exemption (IDE) applicants to report clinical trial enrollment targets by demographic subgroup, including age, race, ethnicity, and sex, and provide a rationale for those targets, and to provide a Diversity Action Plan detailing the actions the sponsor will take, such as outreach and engagement strategies, to reach these enrollment targets. The bill also provides FDA with the authority to mandate post-market studies when sponsors fail to meet diversity enrollment targets and do not provide a sufficient justification. It also requires FDA to publish an annual report aggregating and analyzing the data provided by sponsors on their progress toward and strategies for improving diversity in clinical trials. The bill requires FDA to hold a workshop to assess how sponsors utilized the clinical trial flexibilities initiated during the COVID-19 public health emergency and their impact on improving access to clinical trials in underserved populations. It also provides funding to NIH for community engagement and outreach to increase inclusion of underrepresented minorities in clinical trials and research and provides grant funding to Community Health Centers to increase their capacity to participate in clinical trials and research.

XII. H.R. 6888, THE “HELPING EXPERTS ACCELERATE RARE TREATMENTS ACT OF 2022”

H.R. 6888, the “Helping Experts Accelerate Rare Treatments Act of 2022,” introduced by Reps. Tonko (D-NY) and McKinley (R-WV), requires the Secretary to submit an annual report summarizing FDA’s activities relating to designating, approving, and licensing drugs used to treat rare diseases no later than four years after enactment. The bill also requires the FDA Commissioner to convene one or more public meetings to solicit input from stakeholders regarding approaches to improving engagement with rare disease condition patients, patient groups, and experts. The bill also incorporates experts on the science of small population studies in FDA’s existing list of external consultants on rare disease drugs and biologics.

XIII. H.R. 6963, THE “ACCELERATED APPROVAL INTEGRITY ACT OF 2022”

H.R. 6963, the Accelerating Approval Integrity Act of 2022, introduced by Rep. Pallone (D-NJ), makes several changes to enhance program integrity within the accelerated approval pathway at FDA. The bill would codify requirements for adequate and well-controlled post-approval studies for drugs approved under the program, require studies to be designed prior to approval, and allow FDA to require that post-approval studies be underway at the time of approval. The bill also streamlines processes to withdraw a drug’s approval if post-approval studies are not conducted with due diligence or do not show a clinical benefit. The bill would also require sponsors to publish additional information on a drug’s label if it is approved under accelerated approval and would make failure to conduct studies with due diligence, or failure to report on such studies, a prohibited act subject to monetary penalties.

XIV. H.R. 6972, THE “GIVE KIDS A CHANCE ACT”

H.R. 6972, the “Give Kids a Chance Act,” introduced by Reps. Butterfield and McCaul (R-TX), would establish additional FDA authorities regarding the conduct of pediatric investigations of molecularly targeted drugs to treat cancer. In current law, FDA is authorized to require drug sponsors to conduct pediatric investigations of drugs intended to treat adult cancers if the agency determines the drug is directed at a molecular target that is substantially relevant to

the growth or progression of pediatric cancer. This legislation would extend FDA’s authority to require pediatric studies in certain combinations of drugs.

XV. H.R. 6973, THE “ENHANCED ACCESS TO AFFORDABLE MEDICINES ACT”

H.R. 6973, the “Enhanced Access to Affordable Medicines Act,” introduced by Rep. Carter, would allow new generic drugs to be approved with a different label from the listed (also known as the “brand”) drug if the generic drug application is otherwise eligible for approval, except that the listed drug has revised its label within 90 days of its patent expiring or within 90 days of when a generic drug application is otherwise eligible for approval. The sponsor of the generic drug application granted approval would be required to revise its own label to bring it in line with the updated listed drug label within 60 days of approval. If the listed drug updated its label with respect to drug warnings, the generic drug would not be eligible for approval before updating its own label.

XVI. H.R. 6988, THE “DRUG MANUFACTURING INNOVATION ACT”

H.R. 6988, the “Drug Manufacturing Innovation Act,” introduced by Reps. Levin (D-CA) and Joyce (R-PA), authorizes the Emerging Technologies Program at FDA, a collaborative program where industry representatives, academics, and others can meet with FDA officials “to discuss and identify potential technical and regulatory issues regarding the development and implementation of a novel technology prior to filing a regulatory submission.”¹ The bill authorizes FDA to make grants to carry out the program, and authorizes \$20 million each year for FY 2023 through FY 2027 to carry out the program.

XVII. H.R. 6696, THE “ACCELERATING ACCESS FOR PATIENTS ACT”

H.R. 6696, the “Accelerating Access for Patients Act”, introduced by Rep. Rodgers (R-WA), broadens the pathway for accelerated approval by allowing such approvals if the Secretary determines the drug’s safety and effectiveness based on the known benefit-risk profile in the intended population, taking into account the severity, rarity, or prevalence of the disease and the availability or lack of alternative treatments. Current law requires a determination that the drug is safe for the approved population and has an effect on a surrogate endpoint or intermediate endpoint that is reasonably likely to predict clinical benefit. The Secretary is required to establish a comprehensive clinical development plan to determine whether a drug is subject to approval under these circumstances, and issue guidance describing criteria, processes, and other considerations for demonstrating the safety and effectiveness of such a drug. The bill also allows clinical benefit to be proven by means beyond a clinical trial. It also requires an annual report to Congress on the use of RWE in post-approval studies. Finally, the bill requires the Secretary to submit draft guidance no later than 18 months after the date of enactment and final guidance no later than 18 months after the draft guidance is released.

¹ Food and Drug Administration, *Emerging Technology Program* (Feb. 22, 2022) (www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/emerging-technology-program).

XVIII. H.R. 7006, THE “IMPROVING THE NATION’S SAFE PHARMECEUTICALS AND EXCIPIENTS BY CREATING TOOLS FOR INSPECTING AND OVERSEEING NEEDED SUPPLIES ACT” OR THE “INSPECTIONS ACT”

H.R. 7006, the “INSPECTIONS Act,” introduced by Reps. Griffith (R-VA) and Welch (D-VT), would enhance FDA’s inspection tools and study when and how they are used. The bill would allow FDA to consider the compliance history of establishments in a country or region as a factor when establishing a schedule for risk-based inspections. It would also allow the Secretary to use any records or other information collected for the purposes of or in lieu of an inspection to satisfy requirements for a preapproval or risk-based surveillance inspection, including resolving the findings of such inspections, if applicable and appropriate. The bill also allows the recognition of foreign government inspections as sufficient for preapproval inspections if the Secretary has entered into an agreement with that foreign government. Additionally, the bill requires a periodic assessment of whether additional arrangements and agreements with foreign governments as allowed in this legislation are appropriate.

XIX. H.R. 7008, THE “PRE-APPROVAL INFORMATION EXCHANGE ACT”

H.R. 7008, the “Pre-Approval Information Exchange Act”, introduced by Rep. Guthrie (R-KY), would allow drug and device sponsors to share certain information, including health care economic information, scientific information, and pre-clinical trial results, with health insurers, pharmacy benefit managers, formulary committees, and other payors before a drug is approved by FDA and before an application for the drug is submitted to the agency. The information would be required to include a clear statement that the drug or device it discusses has not been approved, and that the safety and efficacy of the drug or device has not been established. Additional required disclosures include information about studies the drug or device is undergoing, how the studies relate to the overall plan for the development of the drug or device, and whether an application for the drug or device has been submitted to FDA and when such submission is planned.

XX. H.R. 7032, THE “INCREASING TRANSPARENCY IN GENERIC DRUG APPLICATIONS ACT OF 2022”

H.R. 7032, the “Increasing Transparency in Generic Drug Applications Act of 2022,” introduced by Rep. Kuster (D-NH), requires the Secretary to determine whether proposed new generic drugs are qualitatively or quantitatively the same as the listed drug. If it is determined that a proposed drug is not the same as the listed drug, the Secretary must inform the generic drug applicant how the proposed drug differs from the listed drug. The bill also requires the Secretary to publish guidance on the process of determining whether a new drug is qualitatively or quantitatively the same as a listed drug no later than one year after enactment.

XXI. H.R. 7035, THE “BIOLOGICS MARKET TRANSPARENCY ACT”

H.R. 7035, the “Biologics Market Transparency Act,” introduced by Reps. Manning (D-NC) and Hudson (R-NC), amends section 506I of the Food, Drug, and Cosmetic Act to require prompt reports of marketing status by holders of approved applications for biologics and

biosimilars products. Specifically, the legislation requires all holders of approved Biologics License Applications (BLA) to conduct a one-time report to confirm that their products listed in the Purple Book are still available for sale. Approved BLA holders must report to the Secretary when withdrawing a product from the market. Similar requirements are already in place for small molecule drugs; this bill would extend the requirements to biologics and biosimilars.

XXII. H.R. 7047, A BILL TO AMEND TITLE III OF THE PUBLIC HEALTH SERVICE ACT WITH RESPECT TO THE DETERMINATION BY THE SECRETARY REGARDING CERTAIN BIOSIMILAR APPLICATION ELEMENTS, AND FOR OTHER PURPOSES

H.R. 7047, a bill to amend title III of the Public Health Service Act with respect to the determination by the Secretary regarding certain biosimilar application elements, and for other purposes, introduced by Rep. Schrader (D-OR), would give the Secretary discretion to determine whether it is necessary for a biosimilar product to demonstrate that it has the same strength as that of its reference product. Current law requires that a biosimilar or interchangeable product demonstrate that the strength of the biological product is the same as that of the reference product to be granted a license. A reference product is a single biological product already approved by the FDA.

XXIII. WITNESSES

The following witnesses have been invited to testify:

Jeff Allen, Ph.D.
President and CEO
Friends of Cancer Research

Cartier Esham, Ph.D.
Chief Scientific Officer
Executive Vice President, Emerging Companies
Biotechnology Innovation Organization

David Gaugh
Senior Vice President, Sciences and Regulatory Affairs
Association for Accessible Medicines

Ruben Mesa, M.D.
Executive Director, Mays Cancer Center
UT Health San Antonio MD Anderson

Reshma Ramachandran, M.D.
Chair, Doctors for America FDA Task Force
Physician-Fellow, Yale National Clinician Scholars Program
Yale School of Medicine

Lucy Vereshchagina, Ph.D.
Vice President, Science and Regulatory Advocacy
Pharmaceutical Research and Manufacturers of America