PUBLIC CITIZEN 50
People • Power • Progress YEARS

March 16, 2022

The Honorable Anna G. Eshoo
Chair, Subcommittee on Health
Committee on Energy & Commerce
United States House of Representatives
Washington, D.C. 20515

The Honorable Brett Guthrie
Ranking Member, Subcommittee on Health
Committee on Energy & Commerce
United States House of Representatives
Washington, D.C. 20515

**RE: Comments Regarding H.R. 6996, the Accelerating Access for Patients Act of 2022, and H.R. 6963, the Accelerated Approval Integrity Act of 2022**

Dear Chairperson Eshoo and Ranking Member Guthrie:

Public Citizen, a consumer advocacy organization with more than 500,000 members and supporters nationwide, respectfully submits the following comments regarding the above referenced bills that are to be considered tomorrow at your subcommittee's hearing entitled "The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight."

**Oppose H.R. 6996, the "Accelerating Access for Patients Act"**

Public Citizen urges you to oppose H.R. 6996, the Accelerating Access for Patients Act of 2022, because it would unacceptably weaken the criteria for Food and Drug Administration (FDA) approval of new drugs under the agency's already too lax accelerated-approval pathway. Under Section 506(c) of the Food, Drug, and Cosmetic Act, the FDA currently may approve an application for approval of a new drug for a serious or life-threatening disease or condition under the accelerated-approval pathway based on a determination that "the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments."

H.R. 6996 would add a second basis for FDA approval of a new drug under the accelerated-approval pathway. Specifically, the legislation would dangerously allow the FDA to approve an application for approval of a new drug for a serious or life-threatening disease or condition under the accelerated-approval pathway based only on a determination "of the safety and effectiveness of the product based on the known benefit-risk profile of such product in the intended population, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments." This proposed alternative basis for accelerated approval is so ambiguous that it would essentially allow the FDA to approve new drugs under the accelerated-approval pathway without actual evidence establishing that they are safe and effective.

H.R. 6996 also would substantially weaken the FDA's post-approval study requirements for new drugs approved under the accelerated-approval pathway by allowing the agency to rely on

analyses of data from patient registries or other observational clinical studies — rather than well-designed, randomized, controlled clinical trials — to confirm that drugs approved under this regulatory pathway have an effect on irreversible morbidity or mortality or other clinical benefit. Post-approval studies to establish the clinical benefit of a new drug approved under the accelerated-approval pathway should be limited to well-designed, randomized, controlled clinical trials.

**Support and Strengthen H.R. 6963, the Accelerated Approval Integrity Act of 2022**

In contrast to our opposition to H.R. 6996, Public Citizen urges you to support H.R. 6963, the Accelerated Approval Integrity Act of 2022, and to consider expanding it to further strengthen the criteria for FDA approval of a new drug under the accelerated-approval pathway. H.R. 6963 would substantially strengthen the postmarket requirements for new drugs approved under the accelerated-approval pathway by, among other things, (a) requiring sponsors to conduct well-controlled post-approval studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical benefit; (b) providing the FDA with explicit authority to require such post-approval studies begin prior to accelerated approval; (c) providing the FDA with explicit authority to withdraw approval on an expedited basis if the sponsor fails to achieve agreed-upon enrollment targets, milestones, or timely study completion; and (d) mandating automatic expiration of approval one year after any target date of post-approval study completion and not later than five years after approval unless certain conditions are met. We strongly support all of these provisions, with one recommended change: The provision in H.R. 6963 that would forestall automatic expiration of an approval granted under the accelerated-approval program if "the Secretary has determined that adequate progress has been made on completion of [the required] postapproval studies" (see page 6, lines 1-4) should be deleted because it creates a substantial loophole that is likely to be misused frequently by the FDA.

Public Citizen also recommends that H.R. 6963 be amended to include provisions that would prevent the FDA's inappropriate use of the accelerated-approval pathway, as exemplified by the agency's reckless decision to approval aducanumab (Aduhelm) for treatment of Alzheimer's disease under this pathway on June 7, 2021, based on an unvalidated surrogate endpoint — reduction of amyloid-beta plaques in the brain. Notably, the available evidence at the time of the FDA's approval of aducanumab — including evidence from the clinical trials of aducanumab itself — failed to show a compelling, meaningful correlation between changes in this surrogate endpoint and changes in clinical measures of cognitive function. We therefore urge you to include a provision in H.R. 6963 that would only allow accelerated approval of a drug based on a surrogate endpoint when there is widespread, evidence-based agreement in the research community that an effect on a particular surrogate endpoint is reasonably likely to predict clinical benefit.

Thank you for considering our views on this legislation.

Sincerely,

Michael Carome, M.D.
Director
Public Citizen's Health Research Group

cc: Members, Subcommittee on Health, Committee on Energy & Commerce, U.S. House of Representatives