Statement for the Record
House Committee on Energy and Commerce Hearing:
"The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight"
March 17, 2022

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

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

Dear Chairman Pallone, Ranking Member McMorris Rodgers, and Members of the Committee:

Arnold Ventures appreciates the opportunity to submit this statement for the record for the committee's hearing, "The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight."

Arnold Ventures is a philanthropy dedicated to investing in evidence-based policy solutions that maximize opportunity and minimize injustice. Our work within the health care sector is driven by a recognition that the system costs too much and fails to adequately care for the people it seeks to serve. We prioritize five objectives to make health care more affordable and accessible: lower prescription drug prices; lower excessive commercial sector prices charged by providers; improve provider payment to incentivize the delivery of high-quality and efficient care; ensure Medicare's financial sustainability; and improve care for people with complex care needs.

Our work on prescription drugs focuses on the drivers of high drug and biologic prices and spending: patent abuses and anti-competitive behaviors; market distortions; and high launch prices and unjustified price increases. Our work also focuses on ensuring that the U.S. Food and Drug Administration (FDA) drug and biologic approval process incentivizes innovation by using clinically meaningful evidence that is based on patient outcomes, and requiring that trial data be publicly available and comparable across products.

To that end, we appreciate the committee's efforts to support and strengthen the critical work of the FDA, and we support continued efforts to implement policies that strike a balance between promoting innovation, creating access, and ensuring the safety and efficacy of drugs on the market.

A key piece of the FDA's efforts to bring more therapies to patients faster has been the Accelerated Approval Program. The FDA established the program 30 years ago to speed the approval of drugs and biologics that treat serious conditions and fill an unmet need by permitting clinical trials to use surrogate endpoints and requiring confirmatory clinical evidence to be collected at a later date. Some drugs and biologics approved through the accelerated approval pathway will have strong, direct patient benefits on survival, symptoms and/or patient function, as

is true with drugs used to treat HIV/AIDS.[i] Others, however, have no direct clinical benefit and pose considerable risk to patients after years of market access.

The FDA approval of the Alzheimer's disease drug aducanumab is an excellent example of why reforms to the Accelerated Approval Program are warranted. The FDA approved aducanumab despite evidence of severe clinical harms such as brain swelling and bleeding, and without clear evidence of clinical improvements in patient cognition.[ii] Further, an FDA advisory committee overwhelmingly rejected the premise that the data collected in clinical trials using a surrogate endpoint of amyloid reduction on brain scans could be extrapolated to drug effectiveness in slowing cognitive decline.[iii]

As the Accelerated Approval Program exists today, we note three areas of concern:

1. The growing use of surrogate endpoints that are divorced from direct patient outcomes (e.g., survival, gain of function, symptom abatement).
2. The lack of timely completion of confirmatory trials.
3. Inadequate efforts to withdraw from the market products that fail confirmatory trials.

Additionally, manufacturers are charging high prices for new drugs approved through the accelerated approval pathway before direct patient benefits are confirmed. Biogen, for example, is charging about $28,000 per year for aducanumab.[iv] Paying for medications with questionable efficacy and, in the case of aducanumab documented harms, uses taxpayer dollars that could otherwise be directed toward higher value services.

As is true for the majority of expedited or amended approval pathway programs used by the FDA, the percentage of drugs approved under the Accelerated Approval Program has increased over time.[v] From 1992 - 2020, FDA issued more than 250 accelerated approvals, most for oncology drugs.[vi] Some drugs or biologics are exclusively marketed for indications approved under the accelerated approval pathway, while others have several approved indications for which the accelerated approval pathway is used for a subset.

As required by law and regulation, sponsors of drugs that use the accelerated approval pathway must verify and describe the clinical benefit of the surrogate endpoint through adequate and well-controlled confirmatory trials.[vii] However, post-approval confirmatory trials are often delayed and many have been found to use the same incompletely validated surrogate endpoints from the preapproval trials.[viii,ix] As shown over time, some surrogate endpoints can be poor predictors of the effects of drugs on patient health outcomes.[x] As of July 2021, of the 18 accelerated approval indications with negative post-approval trials (those that did not confirm a clinical benefit), six remained on formal FDA approved drug labelling and continued to be recommended in clinical guidelines.[xi]

In 2019, Medicare spent $9.1 billion (Part D: $3.2 billion; Part B: $5.9 billion) on drugs with at least 1 indication that was approved using the accelerated approval pathway.[xii] From 2015 – 2020, Medicaid spent $6.7 billion on drugs with accelerated approval, of which 33 percent were on drugs exclusively marketed for indications approved under the accelerated approval pathway, and 31 percent on drugs approved under the accelerated approval pathway that, after at least five years, have yet to complete the confirmatory trial.[xiii] During this time period, total Medicaid net spending on drugs with at least 1 accelerated approval indication doubled.

It is clear that through the incentives of the Accelerated Approval Program, needed therapies have been brought to patients faster. We believe that Congress should improve guardrails to ensure that drugs coming to market under this pathway meet the same stringent safety and efficacy standards as any other product approved by the FDA. Congress can also consider adjustments to Medicare and Medicaid payment policies to incentivize manufacturers to complete confirmatory trials in a timely manner to ensure that payers, providers, and patients can trust the value of these therapies over existing standards of care.

We appreciate the committee's commitment to improving the Accelerated Approval Program. Please reach out to Mark Miller (mmiller@arnoldventures.org) or Andrea Noda (anoda@arnoldventures.org) with any questions.

Sincerely,

Andrea Noda
Vice President, Health Care
Arnold Ventures

[i] https://www.fda.gov/media/86284/download
[ii] https://www.nytimes.com/2021/11/22/health/aduhelm-death-safety.html
[iii] https://www.fda.gov/media/145690/download
[iv] https://www.fiercepharma.com/pharma/biogen-halves-aduhelm-price-layoffs-ahead-medicare-decision-controversial-alzheimer-s-drug
[v] https://jamanetwork.com/journals/jama/article-abstract/2758605
[vi] https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approvals
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