June 16, 2022

The Honorable Frank Pallone
Chairman
Committee on Energy & Commerce
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Patty Murray
Chairwoman
Committee on Health, Education, Labor, and Pensions
428 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Cathy McMorris Rodgers
Ranking Member
Committee on Energy & Commerce
2157 Rayburn House Office Building
Washington, DC 20515

The Honorable Richard Burr
Ranking Member
Committee on Health, Education, Labor, and Pensions
428 Dirksen Senate Office Building
Washington, DC 20510

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

We, the undersigned members of the Accelerated Approval Action Alliance (A4), are pleased to share with you the following Accelerated Approval Consensus Themes. As you work with your colleagues in both chambers on the reauthorization of the Prescription Drug User Fee Act (PDUFA), we ask you to assure that language reflecting each of these themes is included. As patient organizations representing a range of conditions, from rare disease, to oncology, to neurological diseases, we are united in advocating for the inclusion in the PDUFA reauthorization of provisions reflecting each of the following:

Comprehensive Strategy Plans

- Require FDA and sponsor to agree on a comprehensive strategy plan to define endpoints, study design (including planned confirmatory study), milestones, use of real-world evidence, etc. while the product is still in development

Selection of Biomarkers and Endpoints

- Encourage FDA to use resources and work with stakeholders to advance characterization of surrogate biomarkers and intermediate clinical outcomes

Real-World Evidence

- Encourage FDA to consider the use of real-world evidence in the development of endpoints
- Encourage FDA to consider the use of real-world evidence in confirmatory trials and other post-approval studies

Confirmatory Trials

- Require FDA and sponsor to reach an agreement on trial details (e.g., protocol, milestones, timeline) by time of approval
- Permit FDA to require that confirmatory trials are underway at time of AA

Labeling

- Any reference to accelerated approval on a product label must avoid any suggestion that the product is "experimental"

Approval Withdrawals

- Give FDA authority to expedite withdrawal of products
- In the event of proposed withdrawal require:
    - Thoughtful consideration of implication for patients and patient community; and
    - Transparent, open and effective communication with stakeholders, including the patient community, together with opportunity for input, on process, timing and rationale

Reporting

- Require regular post-marketing reports by sponsors to FDA
- Require FDA to make the post-marketing reports (or portions of the reports without confidential information) available to the public on the FDA website

Intra-Agency Communications

- Encourage FDA to promote consistent processes regarding the use of accelerated approval across review divisions with focus on getting treatments for serious and life-threatening conditions to patients quickly.

FDA Engagement with CMS

- Upon the request of the sponsor, permit FDA to facilitate a meeting with CMS and the sponsor prior to the accelerated approval of a medical product to review evidence supporting FDA's finding that:
    - the primary endpoint used in the pivotal trial is reasonably likely to predict clinical benefit; and
    - the approval is based on a finding of substantial evidence of the product's effectiveness.

Thank you for your continued and widely-appreciated efforts to reach an agreement on the final provisions of the PDUFA reauthorization. We look forward to working with you and your staff to assure that this critical legislation includes language memorializing the important themes outlined above.

Sincerely,

Alliance for Aging Research
ALS Association
Cancer Support Community
EveryLife Foundation for Rare Diseases
I AM ALS
LUNGevity Foundation
Lupus Foundation of America
Melanoma Research Alliance
Parent Project Muscular Dystrophy
UsAgainstAlzheimer's