Accelerated Approval Pathway Ensures Access for Vulnerable Patients, Addresses Unmet Need, and Leverages Scientific Innovation

BIO strongly opposes efforts to restrict access to innovative therapies approved under the Accelerated Approval Pathway (AAP). AAP is often the only mechanism for approving effective therapies to address critical unmet patient need in challenging and serious disease states. Undermining this pathway would have detrimental effects on vulnerable patient populations and hinder innovation.

Why is preserving the AAP so important?

- The AAP is an essential regulatory tool to **expedite patients' access to innovative products** that address some of the most pressing public health needs and save lives.
- Using the same well-established evidentiary standard as traditional approvals, the AAP has **facilitated approval of treatments for many severe diseases**, including various cancers and bacterial infections, HIV, Multiple Sclerosis, Sickle Cell Disease, and other serious conditions.
- The AAP **encourages scientific and medical innovation** by leveraging the use of surrogate or intermediate clinical endpoints that are reasonably likely to predict clinical benefit.

The approval of the first HIV/AIDS drug—based on the use of surrogate endpoints—prolonged and saved millions of lives

- The AAP is **only available for a drug that treats a serious condition** and generally provides a meaningful advantage over available therapies. In these circumstances, uncertainties regarding the ultimate clinical benefit of the drug and a shorter period of study are outweighed by the importance of unlocking therapies for these serious diseases with unmet need.
- By necessity, therapies that utilize the AAP rely on surrogate endpoints. Limiting access or reimbursement for **therapies would deprive patients who suffer from certain conditions the safe and effective therapies they need**.

What's at stake?

- Proposals in Medicare and Medicaid to limit coverage for therapies approved through AAP **portend a grim future for patients** without or limited treatment options today.
- Medicare's recent decision to subject Alzheimer's drugs approved under the AAP to expensive and time-consuming new trials **only serves to delay patient access**. Medicare has never restricted coverage in such a way for any FDA-approved drug, including those approved under the AAP. This is an alarming development for patients, particularly those with hard-to-treat diseases.

It is critical that any proposed policy change to AAP should be contemplated holistically, not based on individual product approvals.