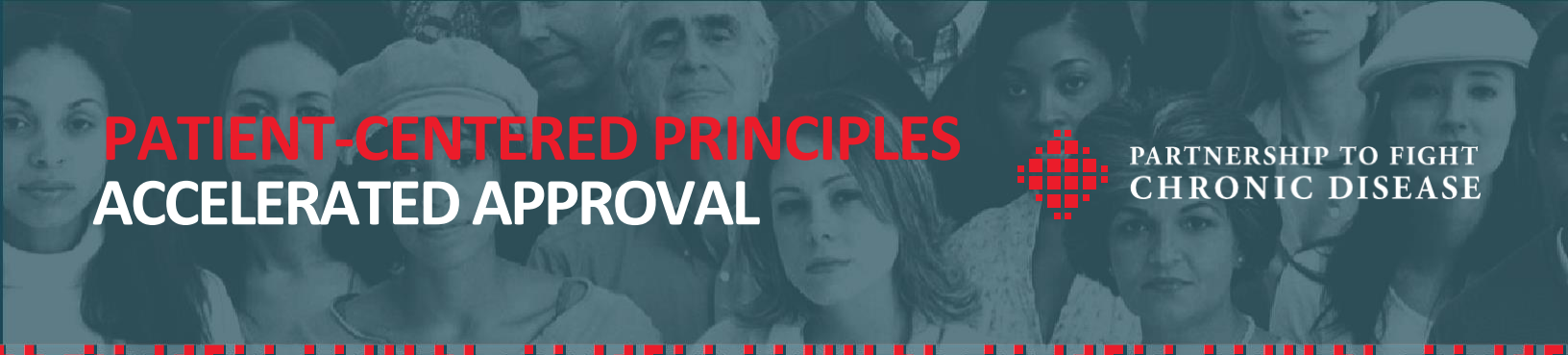


PATIENT-CENTERED PRINCIPLES
ACCELERATED APPROVAL

PARTNERSHIP TO FIGHT CHRONIC DISEASE

The Partnership to Fight Chronic Disease (PFCD) supports policies that preserve and strengthen the FDA's accelerated approval pathway for people living with rare and other chronic diseases.

Respect the capability and rights of patients, their families, and their providers to make informed treatment decisions, including weighing the risks and benefits of available treatments.

Policymakers should oppose policies that:

- Reduce the regulatory authority of the FDA to evaluate safety and efficacy.
- Compromise the personal decision-making rights of patients, families, and their providers to make informed personal decisions based on risks and benefits to them; and
- Enable payors to substitute their judgment on clinical risks and benefits.

Uphold and reinforce FDA's statutory authority to evaluate, monitor, and oversee medical therapeutics' safety and effectiveness.

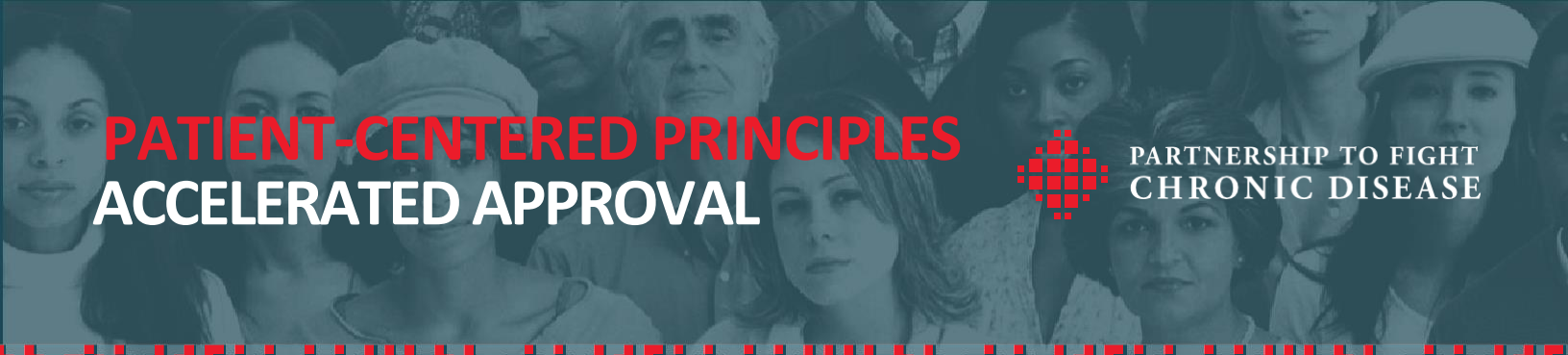
The robust framework for FDA's expert review and approval of safe and effective medicines under accelerated approval has been in practice for thirty years. The program continues to achieve the mutually essential goals of hastening patient access to safe and effective medicines for serious diseases while maintaining FDA's rigorous approval standards. Other federal and state agencies should not undermine FDA's statutory authority or create regulatory redundancy and inefficiency. Such actions counter the primary purpose behind accelerated approval: enhancing timely access to treatment for seriously ill people with limited or no other treatment options.

Promote consistent use of accelerated approval across disease areas where the science, disease understanding, and unmet needs of patients support it, including identification of novel surrogate endpoints and intermediate clinical endpoints.

Opportunities for use of accelerated approval has yet to be fully realized, particularly in rare diseases. Congress recognized the significant need and opportunities for innovation in rare diseases in passage of FDASIA, expanding the accelerated approval pathway to include intermediate clinical endpoints which can be particularly relevant to rare diseases. Innovation is leading to breakthroughs for additional uses of approved therapies and in novel technologies such as gene therapies for many rare diseases. Regulators, innovators, and expert scientists should partner to support evidence-based use of novel surrogate endpoints.

January 2023

FOR MORE ABOUT THE ACCELERATED APPROVAL PATHWAY, VISIT
WWW.FIGHTCHRONICDISEASE.ORG/RESOURCES/ACCELERATEDAPPROVAL



PATIENT-CENTERED PRINCIPLES
ACCELERATED APPROVAL

PARTNERSHIP TO FIGHT CHRONIC DISEASE

The Partnership to Fight Chronic Disease (PFCD) supports policies that preserve and strengthen the FDA's accelerated approval pathway for people living with rare and other chronic diseases.

Enable timely conduct of confirmatory studies by ensuring robust, early, and ongoing communication between drug sponsors and FDA regarding development plans and progress.

FDA and drug sponsors should discuss plans for the conduct of the required study or studies to confirm clinical benefit as early in the process as possible. Ideally, confirmatory studies should begin prior to accelerated approval. Policies should reflect, however, that unavoidable circumstances may prevent the initiation of the confirmatory studies before approval and allow for flexibility in that regard. A clearer understanding and process must be realized for completing confirmatory research that will necessarily differ depending on the disease, its progression, and ultimate clinical endpoints. FDA has the authority and should exercise it to assure that sponsors exercise diligence in completion of confirmatory studies.

Recognize unique complexities of rare and other serious chronic diseases by rejecting rigid deadlines and other policies that impose a "one-size-fits-all" approach.

There are challenges associated with studying treatments for rare diseases that are particularly acute in the post-approval setting due to small patient populations, heterogeneity, and slow disease progression as compared with other diseases. Disease specific considerations must be allowed to dictate timelines for the conduct of studies to confirm clinical benefit.

Retain and reinforce appropriate processes and assurances of scientific and regulatory rigor associated with FDA withdrawal of accelerated approval therapies from the market.

Changes to streamline or expedite FDA's processes for the withdrawal of marketing authorization must allow for due process and robust opportunities for patient engagement and public input.

Avoid coverage and reimbursement policies that would disincentivize the use of accelerated approval.

Establishing separate coverage qualifications for accelerated approval therapies undermines the intent of the pathway to the life-altering detriment of people living with untreatable, often progressive, life-threatening illnesses. Policy proposals to waive Medicaid coverage requirements, assess higher rebates or set prices until confirmatory trials finish, and limit Medicare coverage only to clinical trial participants can effectively deny seriously ill people the only treatment option available.

FOR MORE ABOUT THE ACCELERATED APPROVAL PATHWAY, VISIT
WWW.FIGHTCHRONICDISEASE.ORG/RESOURCES/ACCELERATEDAPPROVAL