Whereas, In the wake of the AIDS epidemic in the 1980s, the U.S. Food and Drug Administration (FDA) created pathways by which specialty drugs could be approved based on less rigorous data, including a “fast track” pathway for drugs that treat life-threatening or severely debilitating conditions, which allows approval on the basis of uncontrolled Phase II trials, and an “accelerated approval” pathway which lowers evidentiary requirements for drugs for serious or life-threatening conditions if the drug provides a meaningful therapeutic benefit not provided by existing treatment, both of which have reduced the time to approval for designated specialty drugs; and

Whereas, In the period of 2000-2013, 82 drugs were approved under the fast-track designation, representing 22% of all drugs including biologics approved by the FDA during that time period, yet only 49 of the 82 were specialty drugs; and

Whereas, In the same period of 2000-2013, 37 new drugs were granted accelerated approval (10% of all drugs including biologics), of which 26 were specialty drugs; and

Whereas, In 2012 the United States Congress created another expedited pathway for so-called “breakthrough therapies” which could be designated by FDA based on early clinical signs of promise, expected to be used only a few times a year but which received over 100 applications for designation in 2013; and

Whereas, These expedited pathways usually allow for drug approval some time during Phase III which lasts approximately from 1-4 years, and the standard drug approval process has a median approval time of 10.1 months from receipt of application, thereby resulting in expedited pathway approval approximately 5 years before said drug would be approved via the standard pathway; and

Whereas, These expedited approval pathways pose challenges to the evidence-based prescribing of approved drugs, since designations provide strong signals to the public about the clinical importance of the drugs entering these pathways and drugs that are approved after a shortened premarket period or drugs approved based on invalidated surrogate endpoints may later be found to have greater risks, or less certain benefits, than was initially believed to be the case; and

Whereas, Approval of an expensive new specialty drug based only on preliminary data suggesting that it might improve patient outcomes and resultant use by clinicians may divert resources away from other health care interventions that have been confirmed to be effective or that present greater value; and
Whereas, These expedited pathways require post-approval testing to confirm the drugs’ predicted benefit-risk profiles, yet one 2011 review of forty-seven oncology drugs approved through the “accelerated approval” pathway in the period 1992–2010 found that trials for eighteen had not been completed at the time of the review; and

Whereas, FDA has limited power to ensure that mandatory post-approval trials for drugs approved via these pathways be conducted in a timely and rigorous manner, being able to impose civil fines of up to $10 million, which is but a fraction of the enormous profit specialty drugs can generate; and

Whereas, Removing a drug from the market often draws criticism from physicians and patient-advocacy groups, even for drugs which lack data supporting their effectiveness or safety; and

Whereas, A system by which approval for drugs brought forward under these expedited pathways would be designated as temporary and have a set expiration date, with more permanent FDA approval given under the condition of further evidence supporting safety and efficacy, would shift the burden to the manufacturer to show that its drug should remain on the market; and

Whereas, Legislative action would be required to further modify the FDA expedited pathway processes; and

Whereas, Robert M. Califf, M.D., former FDA Commissioner noted that with the passage of the 21st Century Cures Act “great progress has been made towards our shared goal of advancing regulatory science so that we can continue to speed the discovery, development, and delivery of medical products to prevent and cure disease and improve health while sustaining the evidence framework that enables assurance to the public of the safety and effectiveness of medical products;” therefore be it

RESOLVED, That our American Medical Association work with U.S. Food and Drug Administration (FDA) and other interested stakeholders to design and implement via legislative action (including ensuring appropriate FDA staffing) a process by which drugs which obtain FDA approval via the Fast Track, Accelerated Approval, or Breakthrough Therapy pathways be granted FDA approval on a temporary basis not to exceed 5 years, pending further evidence of safety and efficacy that is at the level set for the standard drug approval process (Directive to Take Action); and be it further

RESOLVED, That our AMA work with the FDA and other interested stakeholders in improving the process by which drugs are selected for the expedited pathway to improve the prevalence of these drugs that are classified as “specialty drugs.” (Directive to Take Action)

Fiscal Note: Modest - between $1,000 - $5,000.

Received: 09/06/17

RELEVANT AMA POLICY

FDA H-100.992
(1) Our AMA reaffirms its support for the principles that: (a) an FDA decision to approve a new drug, to withdraw a drug's approval, or to change the indications for use of a drug must be based on sound scientific and medical evidence derived from controlled trials and/or postmarket incident reports as provided by statute; (b) this evidence should be evaluated by the FDA, in consultation with its Advisory Committees and expert extramural advisory bodies; and (c) any risk/benefit analysis or relative safety or efficacy judgments should not be grounds for limiting access to or indications for use of a drug unless the weight of the evidence from clinical trials and postmarket reports shows that the drug is unsafe and/or ineffective for its labeled indications.
(2) The AMA believes that social and economic concerns and disputes per se should not be permitted to play a significant part in the FDA's decision-making process in the course of FDA devising either general or product specific drug regulation.
(3) It is the position of our AMA that the Food and Drug Administration should not permit political considerations or conflicts of interest to overrule scientific evidence in making policy decisions; and our AMA urges the current administration and all future administrations to consider our best and brightest scientists for positions on advisory committees and councils regardless of their political affiliation and voting history. (Res. 119, A-80; Reaffirmed: CLRPD Rep. B, I-90; Reaffirmed: Sunset Report, I-00; Reaffirmation, A-06; Appended: Sub. Res. 509, A-06; Reaffirmation, I-07; Reaffirmation, I-09; Reaffirmation, I-10)

Addressing the Exploitation of Restricted Distribution Systems by Pharmaceutical Manufacturers H-100.950
1. Our AMA will advocate with interested parties for legislative or regulatory measures that require prescription drug manufacturers to seek Food and Drug Administration and Federal Trade Commission approval before establishing a restricted distribution system.
2. Our AMA supports requiring pharmaceutical companies to allow for reasonable access to and purchase of appropriate quantities of approved out-of-patent drugs upon request to generic manufacturers seeking to perform bioequivalence assays.
3. Our AMA will advocate with interested parties for legislative or regulatory measures that expedite the FDA approval process for generic drugs, including but not limited to application review deadlines and generic priority review voucher programs. (Res. 809, I-16)

Food and Drug Administration H-100.980
(1) AMA policy states that a strong and adequately funded FDA is essential to ensuring that safe and effective medical products are made available to the American public as efficiently as possible. (2) Our AMA: (a) continue to monitor and respond appropriately to legislation that affects the FDA and to regulations proposed by the FDA; (b) continue to work with the FDA on controversial issues concerning food, drugs, biologics, radioactive tracers and pharmaceuticals, and devices to try to resolve concerns of physicians and to support FDA initiatives of potential benefit to patients and physicians; and (c) continue to affirm its support of an adequate budget for the FDA so as to favor the agency's ability to function efficiently and effectively. (3) Our AMA will continue to monitor and evaluate proposed changes in the FDA and will respond as appropriate. (Sub. Res. 548, A-92; BOT Rep. 32, A-95; BOT Rep. 32, A-95; BOT Rep. 18, A-96; Reaffirmed: BOT Rep. 7, I-01; Reaffirmation I-07; Reaffirmed: Sub. Res. 504, A-10; Reaffirmation A-15; Reaffirmed: CMS Rep. 06, I-16)