

# Progress on the 2012 Drug Innovation Report by PCAST (President's Council of Advisors on Science and Technology)

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More than 18 months ago, a group of Presidential advisors from industry, academia and the Federal government **concluded** that while the basic biomedical sciences have seen stunning progress in past decades, challenges remain in translating those scientific advances into practical solutions. To accelerate the development of new therapies, the President's Council of Advisors on Science and Technology (PCAST) made a number of recommendations that called for action by all of the players in the innovation ecosystem including industry, academia, health care professionals and such federal agencies as the National Institutes of Health, the Centers for Medicare and Medicaid Services and the FDA.



PCAST's detailed list of recommendations for FDA generally fell into four categories: advancing regulatory science through public private partnerships; encouraging the expedited approval of drugs; improving FDA's tools for monitoring and communicating clinical benefits and risks and reforming the agency's management practices. A review of all four categories suggests that, together, FDA, Congress, industry and patient groups have made significant progress towards addressing

these recommendations since the PCAST report was released in September 2012, although some critical challenges remain.

**Public-private partnerships:** Just like PCAST, FDA believes that bridging the gap between drug discovery and development can only be achieved through creative collaborations. Public-private partnerships enable stakeholders to leverage expertise and resources for the conduct of mutually beneficial research activities in the precompetitive domain. And indeed, our Center for Drugs is involved in 22 science-driven, public-private partnerships that promote development of research tools, platforms, clinical databases and predictive models to advance knowledge of disease and safety profiles of drugs – some of which were funded under legislation authorized in the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA). The recent approval of Zykadia, for patients with a certain type of late-stage (metastatic) non-small cell lung cancer, benefited from FDA's collaborative efforts with industry, health advocacy organizations and others to identify the molecular underpinnings of cancer that would make it possible to classify and treat cancer by specific subtype.

**Expedited review:** Even before the PCAST report was issued, FDA had a number of expedited development and review programs in place. Accelerated approval allows for approval of drugs for serious conditions that fill an unmet need based on the drug's effect on a surrogate endpoint that is thought to predict clinical benefit. This can speed access to a potentially important new drug, where it might take years of study to demonstrate a survival benefit or other longer-term outcome. Fast track allows sponsors with drugs that qualify to have more frequent meetings and communications with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval. Priority review shortens the review time for certain promising drugs from 10 months to six months.

Nearly half of the 27 novel drugs approved by FDA last year took advantage of these expedited pathways, which were expanded and enhanced with the help of Congress under FDASIA. Also of note, these novel drugs were approved in as little time as 4.5 months, without compromising our high standards for safety and efficacy.

In a demonstration of the significant progress that can be made when all stakeholders come together, Congress, FDA, industry and patient groups joined together to create the Breakthrough Therapy Designation in FDASIA. This new pathway is designed for those drugs intended to treat serious or life-threatening diseases or conditions where there is preliminary clinical evidence that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint or set of endpoints. A drug that receives Breakthrough Therapy designation is eligible for all Fast Track designation features; intensive guidance on an efficient drug development program, beginning as early as Phase 1; and the commitment from FDA's review staff, including senior managers, to work closely together throughout the drug development and review process. To explain the concepts underlying these expedited programs and help companies decide whether these expedited review

programs will fit their drug, we issued a draft guidance document last June and will be issuing a final guidance soon. So far we have received 178 breakthrough designation submissions, granted 44 designations, and already approved six of the designated drugs, four of which were new molecular entities and two were for new indications for already approved drugs.

PCAST also recommended that FDA implement a drug approval pathway under which sponsors could propose, early in the development process, to study a new drug for initial approval that would be reserved for use in a specific subgroup of patients, this would thus allow a narrower development program than required for traditional approvals. While FDA has existing authority to approve products for subpopulations, in practice, drug development protocols generally evaluate risks in a broader population, resulting in larger, lengthier trials. FDA agrees that a more clearly defined Special Medical Use or Limited Population pathway could encourage novel limited population development protocols and complement FDA's existing efforts to get drugs to small populations in need faster. Legislation focused on a pathway for drugs for serious or life-threatening bacterial infections in patients with unmet medical need – a particular area of unmet medical need highlighted in the PCAST report – has been introduced to address this issue and we welcome the opportunity for continued discussions with stakeholders.

**Communicating risks and benefits:** To help guide our review process for both standard and priority review drugs, we are implementing a structured Benefit-Risk Assessment framework, as agreed to as part of our successful negotiation with industry on user fees to fund drug review activities. Information on the current statement of knowledge regarding the condition and the available therapies, the drug's individual benefits and risks and their frequency, and any efforts that could mitigate the safety concerns are put together in a table. We are currently seeking to integrate this framework into our existing review templates and memos. The purpose is to ensure better communication of the review teams thinking during review and, most importantly, FDA's decision-making when the agency approves a product.

**Management reforms:** PCAST urged a variety of management reforms, some involving staff and some involving infrastructure. We are actively modernizing our information technology platforms to advance innovation and prepare for the enormous data sets that drug sponsors are submitting from clinical studies using genome sequencing and as part of the user fee agreements we are piloting a process change that improves communication during the review process.

Much progress has been made on the PCAST recommendations through FDASIA, user fee agreements, collaborative efforts with stakeholders and the agency's own efforts to continue to improve. And yet, we recognize that challenges remain to advance policies that enhance biomedical innovation and encourage the translation of exciting discoveries into effective therapies. FDA intends to continue working on the PCAST recommendations along with our other partners in the innovation ecosystem.

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