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NEW DRUG DEVELOPMENT

Science, Business, Regulatory, and Intellectual Property Issues Cited as Hampering Drug Development Efforts

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Highlights

Highlights of GAO-07-49, a report to congressional requesters

Why GAO Did This Study

Drug development is complex and costly, requiring the testing of numerous chemical compounds for their potential to treat disease. Before a new drug can be marketed in the United States, a new drug application (NDA), which includes scientific and clinical data, must be approved by the Food and Drug Administration (FDA). Recent scientific advances have raised expectations that an increasing number of new and innovative drugs would soon be developed to more effectively prevent, treat, and cure serious illnesses. However, industry analysts and the FDA have reported that new drug development, and in particular, development of new molecular entities (NMEs)—potentially innovative drugs containing ingredients that have never been marketed in the United States—has become stagnant.

GAO was asked to provide information on (1) trends in the pharmaceutical industry's reported research and development expenses as well as trends in the number of NDAs submitted to, and approved by, FDA; and (2) experts' views on factors accounting for these trends and their suggestions for expediting and enhancing drug development. GAO analyzed data from FDA on all 1,264 NDAs submitted to the agency from 1993 through 2004. GAO also convened a panel of experts and interviewed other drug development experts and analysts to identify factors affecting, and suggestions for enhancing, drug development.

www.gao.gov/cgi-bin/getrpt?GAO-07-49.
To view the full product, including the scope and methodology, click on the link above.
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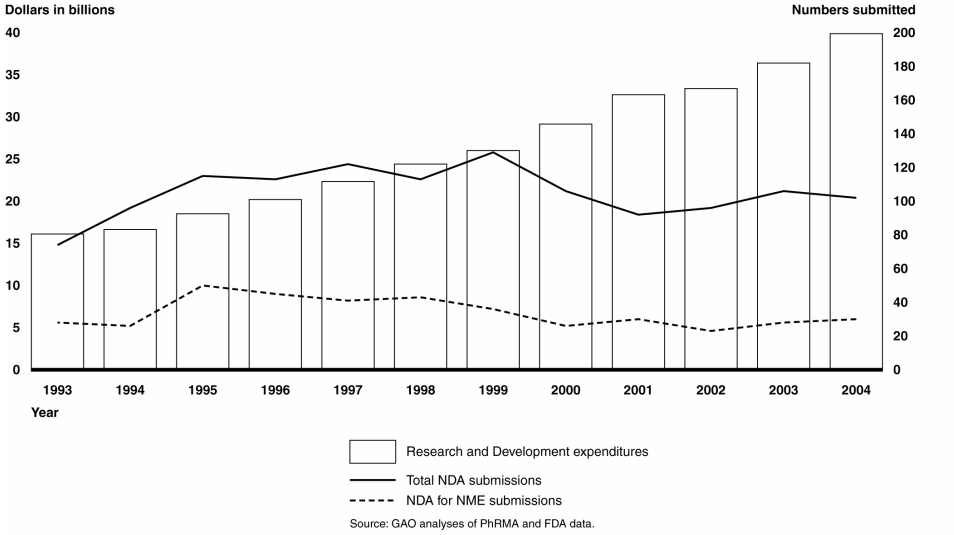
What GAO Found

Although the pharmaceutical industry reported substantial increases in annual research and development costs, the number of NDAs submitted to, and approved by, FDA has not been commensurate with these investments. From 1993 through 2004, industry reported annual inflation-adjusted research and development expenses steadily increased from nearly $16 billion to nearly $40 billion—a 147 percent increase. In contrast, the number of NDAs submitted annually to FDA increased at a slower rate—38 percent over this period. Similarly, the number of NDAs submitted to FDA for NMEs increased by only 7 percent over this period. FDA approved most NDA applications—76 percent overall, but the numbers of NDAs and NDAs for NMEs it approved annually have generally been declining since 1996.

Research and Development Expenses, Total NDA, and NDA for NME Submissions, 1993-2004



Source: GAO analyses of PhRMA and FDA data.

According to experts, several factors have hampered drug development. These include limitations on the scientific understanding of how to translate research discoveries into safe and effective drugs, business decisions by the pharmaceutical industry, uncertainty regarding regulatory standards for determining whether a drug should be approved, and certain intellectual property protections. These factors have been cited as affecting the number of drugs developed, the cost and length of the drug development process, as well as the types of drugs being produced. To address these issues, experts offered suggestions including increasing the number of scientists who can translate drug discoveries into effective new medicines and allowing conditional approval of certain drugs based on shorter clinical trials using fewer numbers of patients. In its comments on a draft of this report, the Department of Health and Human Services provided clarifications, which GAO incorporated as appropriate.