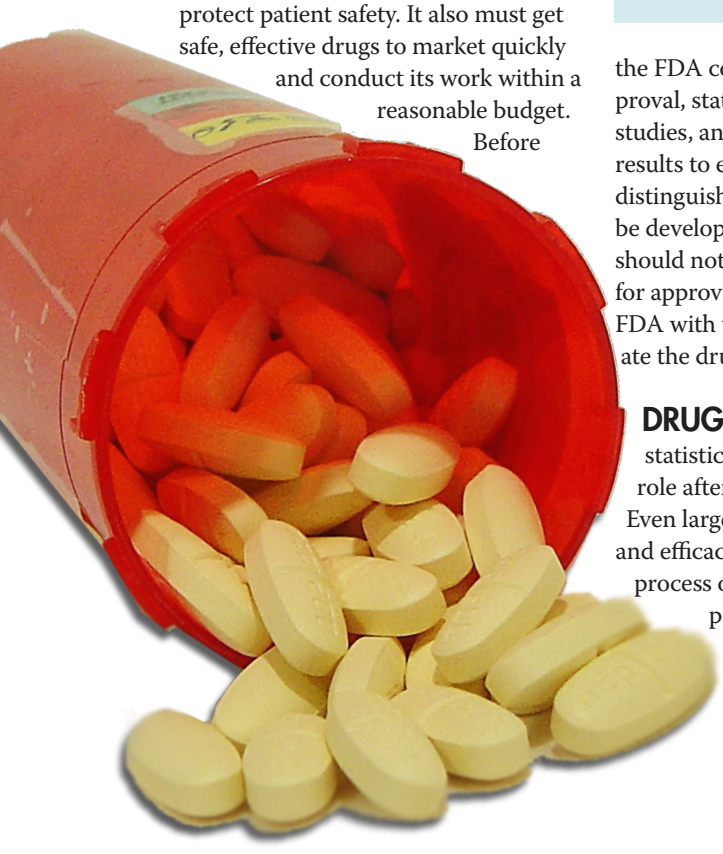
STATISTICAL SIGNIFICANCE

Any drug, device, or vaccine legally sold in the United States must go through a rigorous process of approval and oversight. Statisticians are vital at all stages to get safe, effective drugs and devices to market quickly and to monitor them thereafter. Below are a few examples illustrating just a few of the uses of statistics for the approval of drugs, biologics, and devices.

Safe Medications & Accurate Devices

DEVICE DEVELOPMENT: Laboratory tests, an invaluable part of most medical diagnoses, depend on sophisticated devices that must turn a measurement into a useful lab result. In developing such a device, one must take into account the inherent measurement uncertainty, calibration to standards, and sampling theory. Statisticians are key to each of these steps to ensure medical professionals get an accurate lab result.

DRUG APPROVAL: In considering approval for a new drug, the FDA must protect patient safety. It also must get safe, effective drugs to market quickly and conduct its work within a reasonable budget. Before the FDA considers a drug for approval, statisticians design clinical studies, analyze data, and interpret results to efficiently and effectively distinguish between drugs that should be developed further and those that should not. When a drug is submitted for approval, statisticians provide the FDA with the data it needs to evaluate the drug's safety and efficacy.



CLINICAL TRIALS: Statisticians play a key role in conducting clinical trials—randomized, controlled experiments that are now fundamental to all kinds of research involving humans and that help minimize patient and research bias. Because clinical trials must be carefully designed for each situation, statisticians play a major role in trial design and its monitoring, analysis, and reporting. Statisticians also have helped develop "adaptive clinical trial designs," in which the trial design is adjusted as results start to accumulate, thereby making trials more efficient, saving costs, and reducing patient exposure to ineffective treatments. For example, adaptive trials may enable stopping a treatment early if there is great benefit or harm, or allocating more patients to treatment groups that need more information.

DRUG SAFETY: The FDA and statisticians have a continuing role after a drug is on the market. Even large clinical trials of safety and efficacy during the drug approval process often do not have sufficient power to detect rare events that may become evident after approval. Statistical analysis helps identify these weak signals and advise the regulatory authorities of potential risks to the public. For example, the Women's Health Initiative provided crucial information in 2002 that convinced millions of post-menopausal women to stop taking hormone replacement therapy. This change is believed to have resulted in a reduction of about 20,000 cases of breast cancer in women each year in the United States alone.

"Statistics Aids in Drug & Device Development" is part of Statistical Significance, a series from the American Statistical Association documenting the contributions of statistics to our country and society. For more in this series, visit *www.amstat.org/outreach/statsig.cfm*. The American Statistical Association is the foremost professional society of statisticians, representing 18,000 scientists in industry, government, and academia: *www.amstat.org*. This Statistical Significance was produced under the supervision of the ASA Section on Health Policy Statistics and the ASA Biopharmaceutical Section.