MedicalXpress

Most ongoing diabetes trials do not include outcomes important to patients

June 3 2008

An analysis of ongoing randomized clinical trials (RCTs) in diabetes finds that only about 20 percent have as primary outcomes results that patients consider important, such as illness, pain, effect on function and death, according to a study in the June 4 issue of JAMA.

Concerns about the safety and efficacy of diabetes interventions continue, in part because RCTs have not measured their effect on patient-important outcomes as quality of life and death, according to background information in the article. "Are future diabetes trials likely to be more informative to patients and clinicians?" the researchers ask.

Gunjan Y. Gandhi, M.D., M.Sc., and M. Hassan Murad, M.D., M.P.H., of Mayo Clinic, Rochester, Minn., and colleagues examined large public clinical trial registries to systematically determine the extent to which ongoing and future registered RCTs plan to measure patient-important outcomes in patients with diabetes. The researchers identified phase 2 through 4 RCTs enrolling patients with diabetes. Of 2,019 RCTs, 1,054 proved eligible, and 50 percent of these (527) were randomly sampled, and 436 trials registered since registration became mandatory in 2004 were selected. Of these, 6 percent (24) had not started enrollment, 25 percent (109) were actively enrolling, and 69 percent (303) had completed enrollment.

Reviewers collected study characteristics and determined the outcomes measured and their type (physiological outcomes [insulin, C-peptide levels], surrogate outcomes thought to reflect an increased risk for

patient-important outcomes [such as cholesterol levels, worsening kidney function], and patient-important outcomes [illness, pain, function and death]).

The researchers found that primary outcomes were patient-important outcomes in 18 percent of the RCTs, physiological and laboratory outcomes in 16 percent, and surrogate outcomes in 61 percent of the 436 RCTs. Patient-important outcomes were reported as primary or secondary outcomes in 46 percent of the RCTs.

Independent predictors of patient-important outcomes were larger trial size and longer trial duration while trials of patients with type 2 diabetes were significantly less likely to report patient-important outcomes as a primary outcome. For primary and secondary outcomes, predictors of patient-important outcomes were trial length and trial phase (phase 3 or 4 vs. phase 2), with trials of patients with type 2 diabetes significantly less likely to report patient-important outcomes.

"… the importance of our findings is that (1) without pooling, the individual diabetes trials will largely fail in providing information about the effect of interventions on patient-important outcomes; and (2) trials that are planning to measure patient-important outcomes as secondary end points need to overcome the temptation to selectively report outcomes with statistically significant results as well as to report these findings transparently and carefully. Journals also may need to publish the less-than-interesting results to enable meta-analyses of these results to produce precise enough estimates that can guide practice," the authors write.

"We believe the time has come for a broad consensus on a standard set of important outcomes for patients in diabetes trials, similar to the Outcome Measures in Rheumatology (OMERACT) initiative. The OMERACT approach allows for the uniform measurement of outcomes

in RCTs with emphasis on outcomes that experts—and ultimately patients—thought would better capture the experience of rheumatological conditions."

Source: JAMA and Archives Journals