

Issues Surrounding Comparative Effectiveness Research: *Supporting Patient-Centered Care to Improve Health Outcomes*

As a representative of an industry committed to discovering new cures and ensuring patient access to them, BIO strongly supports efforts to increase the availability of accurate, scientific evidence to inform clinical decision-making. BIO believes that individual patients and their doctors should be armed with the best available information to help assess the relative clinical benefits and risks of various treatment alternatives. When appropriately applied, comparative effectiveness information is a valuable tool that, together with a variety of other types of medical evidence, can contribute to improving health care delivery. However, BIO is concerned that comparative effectiveness information may be used strictly as a means to contain costs, rather than deliver health care value by improving patient health outcomes.

The following are several issues decision-makers should consider in crafting policies related to comparative effectiveness:

- **Comparative effectiveness information should inform clinical judgment and individual needs in medical decision-making.** The results of comparative effectiveness studies often illustrate the experience of the “average” patient on the “average” course of therapy. However, patients may respond differently to the same intervention in ways that cannot be anticipated—for example, the treatment may interact with medications they are taking, or known genetic characteristics may modify response to the treatment. In order to achieve the best possible outcomes, providers must have the flexibility to tailor the appropriate course of treatment for each patient based on individual patient preferences and clinical circumstances. Imposing rigid practice guidelines that fail to recognize such variations among patients can interfere with the ability of providers to deliver the most appropriate care for each patient and lead to suboptimal outcomes and increased health care costs.
- **Comparative effectiveness research should focus on the totality of the health care delivery system, and not just drugs and biologics.** Much of the interest in comparative effectiveness research to date has been narrowly focused on drugs, biologics, and medical devices. However, comparative effectiveness studies are most likely to improve health outcomes if they encompass all aspects of the health care delivery system. In addition to drugs, biologics, and medical devices, comparative effective research should equally examine preventative services, diagnostic tests, and medical procedures. Comparative effectiveness information that reflects the interactions among all of the various components of the health care system has the greatest potential to empower clinicians and patients to make more appropriate decisions when faced with “real world” clinical situations. In addition to comparing specific treatment interventions, research should also focus on how innovations in care delivery models, such as disease management programs, may produce better health outcomes.



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- **The application of comparative effectiveness research should advance the goals of personalized medicine and encourage the development of targeted therapies.** Advancements in the development of innovative therapies are grounded in the ability of researchers to focus on the mechanisms of action that allow particular therapies to work in specific patient populations. Promoting innovation in personalized medicine requires clinicians to have the ability to make patient-centered treatment choices without conforming to inflexible standards or practice guidelines. In addition, many therapies targeting rare or “orphan” diseases, as well as severe, rapidly progressive, or life-threatening diseases, are not conducive to comparative effectiveness studies due to the vulnerabilities, small size, heterogeneity, and other characteristics of these patient populations. Government policies addressing comparative effectiveness should acknowledge these limitations, and not discourage innovation into unmet medical needs.
- **If considering comparative effectiveness information in coverage and payment decision-making, payers should take into account the overall value of a treatment intervention and allow for individual patient variation, rather than just consider cost.** Too often, comparative effectiveness research is viewed by payers and policymakers primarily as an instrument to contain costs, rather than provide health care value by improving patient health outcomes. Comparative effectiveness information should be considered by payers as one of many factors encompassing the overall value of specific health care interventions. Payers should not use comparative effectiveness information to establish “one-size-fits-all” coverage and payment policies that ignore the variability among individual patients in treatment efficacy, safety, and tolerability of treatment interventions. The consequences of inappropriately applying comparative effectiveness research in this manner are exemplified in other countries, including the United Kingdom and Australia, where entire patient populations are denied access to innovative, breakthrough therapies because they do not meet economic thresholds.
- **Comparative effectiveness research should be conducted through an open and transparent process involving all stakeholders, starting from the research planning stage.** Different comparative effectiveness studies evaluating the same item or service can produce widely disparate results, depending on the methods and assumptions used in the analysis. To enhance the credibility and usefulness of any comparative effectiveness study, stakeholders should be afforded the opportunity to provide meaningful input into all steps along the study process, including the identification of priority areas to research, study design and research methods, and dissemination of results. Any approach to comparative effectiveness study design and research should encourage the conduct of independent studies that meet certain standards and that can serve as a basis for consideration by the Food and Drug Administration, in addition to the Centers for Medicare and Medicaid Services and other payers. Careful consideration should be given and rigorous standards applied to the research method selected, such as randomized controlled trials, observational studies, or data synthesis. In their final form, comparative effectiveness research studies should include a concise description of the research question, transparency

as to inclusion or exclusion of evidence or clinical information, transparent analytical methods, discussion of limitations in the quality of the evidence and overall conclusions and recommendations for areas to further research or more fully develop evidence.

- **Comparative effectiveness studies should capture all relevant aspects of diseases and their treatments using the highest possible standards of evidence.** Comparative effectiveness analyses often ignore many important aspects of treatment interventions that affect patients, or may not account for spectra of disease severities. For example, many of the unique benefits provided by drugs and biologics, such as increased safety and improved patient quality of life, are often not captured in comparative effectiveness analyses, or when evaluated do not reflect the use of well-validated methodologies. Increased worker productivity and savings to other parts of the health care system are also important benefits that may not be reflected in studies conducted with a narrow perspective. Risk-adjusted comparisons of treatment interventions are also important to attempt to understand differences among patient subgroups in treatment efficacy, safety, tolerability, quality of life, and health economic outcomes.

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BIO's members remain committed to advancing innovation and developing new therapies to improve patient health. BIO welcomes the opportunity to continue to work with policymakers and other stakeholders on ways to improve value in the health care system by ensuring that the most appropriate care is delivered to each patient.