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Beyond the Trial Endpoint: Why Drug Development Must Listen to Patients

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Opinion

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When a drug trial is discussed, attention often goes immediately to its primary endpoint. Did the treatment achieve the expected outcome? Was the difference statistically significant? Did the study meet its predefined target? These questions are important, but they do not tell the whole story. A medicine can produce a statistically significant improvement while offering only a small difference in a patient's everyday life. Conversely, a treatment may improve symptoms, functioning, or quality of life in ways that are not fully captured by conventional trial endpoints. This raises an important question: are we measuring what matters most to the people who ultimately receive these medicines?

In my view, modern drug trials need to place greater emphasis on patient experience. Clinical research has traditionally been structured around scientific measurement, which is necessary for reliable evidence. However, scientific precision should not come at the expense of clinical relevance.

Consider a patient with a chronic disease. The success of treatment may not be judged by one laboratory value. The patient may instead ask whether they can return to work, climb stairs without difficulty, sleep better, experience fewer symptoms, or avoid hospitalization. These outcomes represent the practical value of treatment, yet they can sometimes receive less attention than highly specific biological measurements.

This does not mean that conventional endpoints should be discarded. Rather, drug trials should combine scientific outcomes with measures that reflect the patient's experience. A treatment that changes a laboratory value but does not meaningfully improve a person's life deserves a different interpretation from one that produces a measurable and noticeable improvement in daily functioning. Another concern is the difference between trial participants and patients in the real world. Clinical trials often have strict eligibility criteria. These criteria are useful for protecting participants and creating a relatively consistent study population, but they can also exclude people who are commonly encountered in clinical practice.

Older adults with several diseases, patients taking multiple medicines, people with varying degrees of kidney or liver function, and individuals from disadvantaged backgrounds may be underrepresented in some studies. Once the medicine becomes widely available, however, these patients may become regular users. This creates a difficult situation. We may have strong evidence that a medicine works in a carefully selected group, while having less information about how it performs in the much more complicated population that uses it in practice. The answer should not be to make every clinical trial unnecessarily large or complicated. Instead, drug development should use different types of evidence at different stages. Randomized trials remain crucial for determining efficacy and causal effects, while carefully designed observational studies, registries, and real-world data can provide additional information about longer-term use and broader patient populations.

Adaptive clinical trials are another development that deserves attention. Traditional trials can be rigid because important decisions are sometimes made only after the study has been completed. Adaptive approaches can allow predefined changes during the trial when accumulating evidence meets specific criteria. The attraction is obvious. If one treatment is clearly performing poorly, continuing to expose large numbers of participants to it may be difficult to justify. If an intervention appears highly promising, researchers may want to adjust the study accordingly. Properly designed adaptive trials can potentially make research more efficient. But flexibility should not be confused

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with freedom to change the rules whenever convenient. A trial must retain scientific integrity. Adaptations need to be carefully planned, statistically justified, and transparently reported. Otherwise, flexibility can introduce uncertainty rather than reduce it. Patient participation is equally important. People who volunteer for clinical research give researchers something that cannot be replaced: their time, experiences, and willingness to accept uncertainty. They should therefore have a meaningful voice in research.

Patients can help identify outcomes that researchers might otherwise overlook. They can also highlight practical barriers to participation, such as travel requirements, complicated schedules, frequent hospital visits, or burdensome procedures. Addressing these issues can make trials more accessible and may improve participation and retention. Digital technology could help in this area. Remote consultations, electronic questionnaires, wearable devices, and home-based monitoring may reduce some of the burden associated with traditional trial visits. For participants who live far from research centres, this could make clinical research considerably more accessible. At the same time, technology is not automatically a solution. Digital research can exclude individuals who lack reliable internet access, suitable devices, or confidence with technology. Researchers must therefore consider whether an apparently convenient trial design is genuinely convenient for all participants. There is also a larger issue of public trust. People are increasingly aware that clinical research influences decisions about medicines that can affect their lives. If trial results are communicated selectively or if uncertainties are hidden behind complicated statistics, confidence in medical research can suffer. Researchers and sponsors should therefore communicate not only what a drug does well but also where uncertainty remains. Patients deserve to understand the potential benefits, possible risks, and limitations of the available evidence. The same principle should apply after approval. A medicine's evaluation should not end when it receives authorization for use. Real-world experience can reveal information that was difficult to observe in earlier studies. Continued monitoring is therefore an important part of responsible drug development. Perhaps the greatest change required is a change in mindset. Drug trials should not be regarded merely as obstacles that a medicine must pass before reaching the market. They are opportunities to learn.

A good trial should tell us more than whether a drug achieved an endpoint. It should help us understand the size of the benefit, the nature of the risks, the patients most likely to benefit, the patients who may need additional caution, and whether the treatment makes a meaningful difference to everyday life. The future of drug development will undoubtedly involve advanced technologies, larger datasets, artificial intelligence, and increasingly sophisticated trial designs. Yet the most important element will remain remarkably simple: the patient. No matter how advanced the methodology becomes, a clinical trial ultimately exists because someone will eventually receive the medicine being studied. If drug development keeps that person at the centre of its questions, endpoints, and decisions, clinical research can become not only faster and more efficient, but also more meaningful. The real measure of a successful drug should therefore extend beyond whether it wins a statistical test. The more important question is whether the evidence helps us make better decisions for real people.

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