Rethinking Clinical Research – towards more inclusive, innovative, and sustainable patient-centered clinical trials developed in partnership with patients

Clinical research remains the cornerstone of medical innovation. Achieving this requires robust evidence generated through well designed and carefully conducted clinical studies which provide the most rigorous mechanism to evaluate the safety and efficacy of interventions. Currently, the ecosystem in which clinical studies operate is rapidly evolving and reshaping how studies are designed and conducted.

The series of papers starting with a state-of-the-art overview explores several of these changes, and examines how clinical research can continue to generate reliable evidence while becoming more innovative, inclusive, sustainable, and co-developed with patients.

The first paper of the series will remind the reader that clinical studies are the foundation for evidence and remain essential to translating scientific discoveries into medical practice. Each phase (from early safety assessment to large confirmatory studies) contributes to building the knowledge, allowing interventions to be carefully evaluated especially before, but even after entering clinical use.

Beyond regulatory approval, clinical studies also provide the evidence base that informs clinical guidelines, healthcare policy, and reimbursement decisions.

However, the traditional clinical study models might be limited when new research challenges arise. Increasingly targeted therapies and rare diseases often involve small numbers of participants making large phase III randomized clinical trials more difficult to conduct. Therefore, innovative approaches to trial designs are needed to adapt to this evolving context while maintaining the scientific rigor.

The second paper will explore the recent methodological developments and how they enable evaluating new interventions more efficiently and flexibly. For example, master protocols allow researchers to test multiple hypotheses within a single trial infrastructure, modify elements of the study as evidence accumulates, and make better use of limited patient populations.

Such approaches can be valuable in rare diseases and precision medicine, where the number of eligible participants may be small or participants can be geographically dispersed.

Innovative trial designs can reduce the number of participants required, shorten development timelines, and accelerate the identification of effective therapies while maintaining robust methodology. Implementation of such approaches requires collaboration between all stakeholders, strong oversight, and transparent communication and reporting to maintain trust in clinical research results.

The third paper will focus on inclusivity as a prerequisite for meaningful evidence to better address the needs of diverse populations. Indeed, the lack of diversity among trial participants (for example, underrepresented women, older adults, ethnic minorities, or children) represents a major challenge, limits the generalizability of findings, and reduces the applicability of research results to real-world patient populations.

Inclusivity in clinical studies is therefore both an ethical responsibility and a scientific necessity, and starts with the identification of the barriers and logistical obstacles that prevent certain populations from engaging in clinical research. To broaden participation, diverse strategies such as decentralized trial models, community-based recruitment, flexible study procedures, and culturally sensitive engagement approaches can be used. However, these strategies must be implemented thoughtfully to ensure that they are truly inclusive for the specific study and population rather than inadvertently introducing new forms of inequality.

The environmental impact of clinical research is also receiving increasing attention and efforts towards more sustainable clinical research are valued. Clinical studies are complex, requiring, among other things, energy-intensive facilities (including data management structures), travels, and large volume of materials, all of which contribute to their carbon footprint.

Several initiatives can help reduce the environmental impact of trials such as decentralized or hybrid trial models, digital data capture, telemedicine consultations, and remote monitoring technologies. Streamlining trial procedures, eliminating unnecessary tests, and focusing on clinically meaningful outcomes can also reduce resource consumption without compromising scientific quality. Sustainability in clinical research should be viewed not only as an environmental priority, but also as an opportunity to improve its efficiency and patient participation.

Another significant aspect explored in this series is the role of patient involvement and the shift from participants to partners.

Traditionally viewed primarily as study participants, patients are now increasingly recognized as key contributors to the research process from the early stage. Patient and public involvement can enhance clinical research by ensuring that research and studies address questions that matter most to those affected by disease. Patients bring unique perspectives based on lived experience, helping researchers better understand priorities, outcomes that reflect meaningful improvements in quality of life, and acceptable levels of burden. Engaging patients throughout the clinical research cycle can help ensuring that research is more relevant, feasible, and impactful.

By fostering co-creation, patient involvement can strengthen the relevance of clinical research and build greater trust between researchers and the communities they aim to serve.

The topics explored in this series reflect a broader transformation in the conduct of clinical research in response to new scientific, social, and environmental challenges.

Collaboration between all stakeholders involved in clinical research is required to achieve the aforementioned changes and to ensure that clinical research can continue to drive medical innovation while remaining scientifically rigorous.

Christine Kubiak, PharmD, PhD, EMBA
Senior Advisor, ECRIN