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From Molecule to Meaning: Why Drug Discovery Needs a New Human-Centered Framework

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Editorial

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Abstract

Drug discovery is increasingly moving beyond a molecule-centered model toward approaches that place human biology, patient needs, and meaningful clinical outcomes at the center of therapeutic innovation. Conventional discovery strategies remain essential for identifying molecular targets and candidate compounds, yet substantial attrition during clinical development demonstrates the difficulty of translating promising preclinical findings into effective therapies for diverse patient populations. Human-derived experimental systems, precision medicine, patient engagement, biomarkers, real-world evidence, and advanced computational methods provide opportunities to strengthen this translational bridge. A human-centered framework can connect molecular mechanisms with patient characteristics, treatment experience, safety, accessibility, and outcomes throughout the drug development continuum. Such integration may improve the relevance of research questions, support more informative therapeutic stratification, and encourage development strategies that better reflect human disease. This Editorial discusses the rationale for moving from molecule-centered discovery toward a framework in which scientific innovation is continuously connected to the needs and experiences of patients.

Keywords: Drug Discovery; Human-Cantered; Drug Development; Precision Therapeutics; Patient-Cantered Research; Biomarkers; Real-World Evidence; Translational Medicine

Introduction

Drug discovery has historically been driven by target identification, molecular design, pharmacological activity, and preclinical testing. These approaches have generated major therapeutic advances, but the pathway from molecular promise to clinical benefit remains uncertain. Candidate compounds can demonstrate favorable biological activity in experimental systems yet fail when evaluated in humans because disease mechanisms, pharmacological responses, toxicity, comorbidities, and treatment environments are more complex than the original models suggest. Recent work on human-centric drug development highlights the persistent gap between preclinical models and human biology and argues for greater integration of human-derived experimental systems and computational approaches across the discovery continuum.

A human-centered framework does not reject molecular science; rather, it places molecular discovery within a broader evidence architecture. Human-derived cellular systems, stem-cell models, organoids, microphysiological systems, and computational approaches can provide complementary information about human disease and drug response. These methods can be combined with genomic and biomarker information to identify biological subgroups and generate

more informative hypotheses about therapeutic response. At the same time, patient engagement is increasingly recognized as an important component of clinical research because patients can contribute information about priorities, treatment burden, outcomes, and experiences that may not be captured by conventional endpoints.

The meaning of a successful medicine should therefore extend beyond molecular potency or statistical efficacy. A therapy must also address an important clinical need, provide an acceptable balance between benefit and risk, and produce outcomes that matter to the people receiving it. Patient-centered real-world evidence can strengthen this perspective by incorporating patient experience into the design and interpretation of real-world research, including the definition of research questions, populations, comparators, and outcomes.Real-world data can also complement clinical trials by reflecting heterogeneous populations and routine treatment environments, although data quality, interoperability, bias, and privacy remain important limitations.

Artificial Intelligence has become the driving force behind these intelligent ecosystems by converting complex healthcare data into meaningful clinical knowledge. Modern AI algorithms can analyze millions of medical records, radiological images, laboratory investigations, genomic sequences, and physiological measurements within seconds, identifying subtle disease patterns that may remain undetected through conventional clinical evaluation. Such capabilities allow physicians to diagnose diseases earlier, predict disease progression more accurately, and recommend individualized therapeutic strategies that improve patient outcomes while reducing unnecessary interventions.

Conclusion

The transition from molecule to meaning requires drug discovery to become more explicitly connected to human biology and patient experience. Molecular targets and candidate compounds remain fundamental, but their value is ultimately determined by how reliably they translate into safe, effective, accessible, and meaningful treatments. Human-derived models, precision medicine, patient engagement, biomarkers, and real-world evidence can strengthen this connection by providing complementary perspectives throughout development. A genuinely human-centered framework should therefore treat patients not simply as the final recipients of innovation, but as a central reference point for defining therapeutic priorities and evaluating value. Such an approach may help make future drug discovery more predictive, clinically relevant, and responsive to the realities of human disease.

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