

Can AI Predict Biomarkers for Drug Response?

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Abstract

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The era of one-size-fits-all medicine is gradually giving way to a more personalized approach, where treatments are tailored to the individual characteristics of each patient. A cornerstone of this revolution is the identification of biomarkers—measurable indicators of a biological state or condition—that can predict how a patient will respond to a particular drug. However, the journey from identifying a potential biomarker to its clinical application is often long, complex, and fraught with challenges. The sheer volume and complexity of biological data have made it increasingly difficult for traditional methods to uncover reliable predictive biomarkers. This is where Artificial Intelligence (AI) is emerging as a transformative force, promising to revolutionize biomarker discovery and personalized medicine.

The Role of AI in Biomarker Discovery

AI, particularly machine learning, excels at identifying patterns in vast and complex datasets that are often invisible to the human eye. In the context of drug response, AI algorithms can analyze a multitude of data types, including genomics, proteomics, transcriptomics, and even medical imaging, to uncover novel biomarkers. By integrating these multi-omics datasets, AI can create a more holistic view of a patient's biology, leading to more accurate predictions of drug efficacy and toxicity. An article from Drug Target Review highlights that AI-driven pathology tools and biomarker analysis can provide deeper biological insights, which are crucial for clinical decision-making, especially in complex diseases like cancer [1]. This ability to process and interpret complex data in a reproducible and clinically meaningful way is what sets AI apart from traditional statistical methods.

AI-Powered Predictive Models

The power of AI in predicting drug response is best illustrated by the development of sophisticated predictive models. One such model is

PharmaFormer, a clinical drug response prediction model based on a custom Transformer architecture and a transfer learning strategy. As detailed in a 2025 article in *npj Precision Oncology*, PharmaFormer was pre-trained on a large dataset of 2D cell lines and then fine-tuned using data from 3D patient-derived organoids. This innovative approach, which combines the breadth of cell line data with the clinical relevance of organoid models, has been shown to dramatically improve the accuracy of clinical drug response prediction [2]. The success of models like PharmaFormer demonstrates the potential of transfer learning to leverage existing knowledge to make accurate predictions even with limited patient data.

Clinical Applications and Future Directions

The application of AI in predicting drug response is not just a theoretical concept; it is already making its way into the clinic. Researchers at the National Institutes of Health (NIH) have developed an AI tool called LORIS (Logistic Regression-Based Immunotherapy-Response Score) that uses routine clinical data, such as a simple blood test, to predict a patient's response to immune checkpoint inhibitors. This tool, which was described in a 2024 press release from the National Cancer Institute, has the potential to help oncologists make more informed treatment decisions for their patients [3]. LORIS is a prime example of how AI can be used to create practical, cost-effective tools that can be easily integrated into existing clinical workflows.

Despite the immense promise of AI in predicting biomarkers for drug response, there are still challenges to overcome. These include the need for high-quality, standardized data, the development of robust and transparent AI models, and the establishment of clear regulatory pathways for the approval of AI-based diagnostic tools. Furthermore, building trust among clinicians and patients is paramount for the widespread adoption of these technologies. Addressing these challenges will require a collaborative effort from researchers, clinicians, regulators, and industry partners.

Conclusion

In conclusion, AI is poised to revolutionize the way we predict drug response by enabling the discovery of novel biomarkers from complex, multi-dimensional data. AI-powered predictive models, such as PharmaFormer, and clinical decision support tools, like LORIS, are already demonstrating the potential of this technology to usher in a new era of personalized medicine. While there are still hurdles to overcome, the continued advancement of AI, coupled with a collaborative approach to its implementation, holds the key to unlocking a future where every patient receives the right drug at the right time.