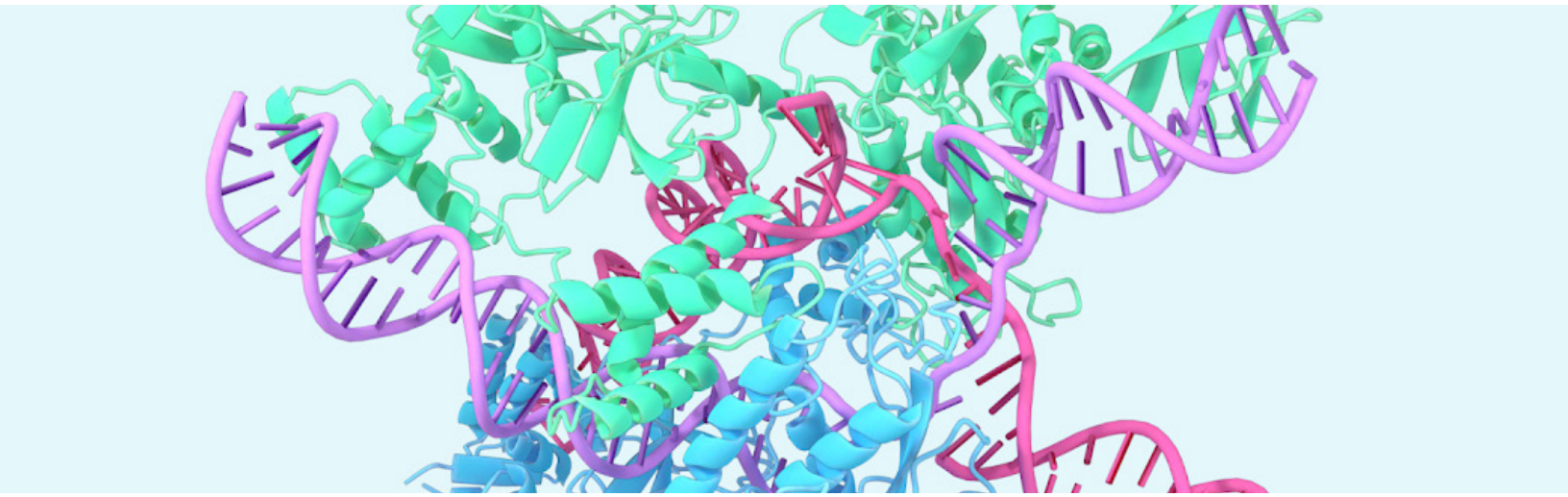
BroadBranch Advisors

Recent Successful Use Cases of Gen AI in Drug Discovery



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One of the more formidable bottlenecks in biopharma R&D is the immense amount of time and resources required to develop a therapeutic agent and bring it to market. Some estimates indicate it can take as long as 10–15 years and cost around $1 - $2 billion to commercialize a drug, with a daunting less than 10% success rate for any Phase I candidate. To better manage costs, timelines, and risk, many biopharma companies have turned their attention and hope toward employing Generative AI (Gen AI) in their early drug discovery efforts.

Gen AI, driven by Deep Learning, Machine Learning, and Computer-Aided Drug Design (CADD), was initially hailed as a 'silver bullet' for the data processing and analytical burdens faced in the pharmaceutical and medical technology industries. However, this promise has yet to consistently deliver significant, differentiating results. According to McKinsey's Summer 2024 survey, more than 100 pharma and medical technology leaders responsible for AI initiatives reported experimenting with Gen AI, with 32% taking steps to scale the technology. Yet only 5% have realized Gen AI as a true competitive differentiator capable of delivering consistent and substantial financial value.

Despite slower-than-expected value generation, industry leaders remain optimistic about GenAI, recognizing that the question is no longer whether to apply Gen AI broadly, but rather "how" and "where" to do so most effectively. Over the past year, the most promising results have emerged in select AI-assisted drug discovery and lead development use cases, which we explore below.

Recent examples of target-based discovery using computational tools

Over the past decade, CADD programs, including structure-based and ligand-based methods, have carved out a path for enhanced compound design and optimization. Recently, deep learning AI/ML models like AlphaFold2, RoseTTAFold, and DeepChem have advanced protein structure prediction, molecular property analysis, and molecular representation using

graph neural networks. Gen AI can leverage previous foundational models and offer a complementary approach: producing diverse data outputs from multimodal data and query inputs. These generative models can be trained via supervised or unsupervised methods. Supervised learning excels in classification and regression but may introduce dataset biases, while unsupervised learning can uncover unbiased patterns, aiding clustering and anomaly detection in large datasets.

In drug discovery, leveraging Gen AI to accelerate drug design and assist with lead optimization by providing suggestions for compound refinement has shown promising results.

Accelerating Drug Design

Drawing from a multitude of data sources including existing chemical structures, patient tissue data, animal models, and in-vitro screening data and more, Gen AI can approach small-molecule design as a process of generating compounds conditioned on specific substructures. Through multiparameter optimization, it has the potential to accelerate drug design by proposing multiple viable candidates at the outset of the discovery journey.

The significance of this use case is evident in partnerships like Sanofi and (now a Recursion company), Novartis and Eli Lilly's collaboration with Isomorphic Labs (Alphabet Umbrella), and AstraZeneca's oncology discovery process. In 2022, Sanofi engaged Exscientia to develop up to 15 novel small-molecule candidates across oncology and immunology. Recently, they announced that two AI-assisted small-molecule drug designs have met the product profile requirements set by both companies, enabling their transition to the lead optimization phase.

Isomorphic Labs, a sister company of DeepMind operating under the Alphabet umbrella and building on DeepMind's AlphaFold model, announced its first pharmaceutical partnership with Eli Lilly in January 2024, followed closely by a second collaboration with Novartis. Under the terms of both agreements, Isomorphic will leverage its multimodal AI platform, which extends beyond AlphaFold's original protein-only predictive capabilities to include small molecules and nucleic acids, to assist Eli Lilly and Novartis in small molecule design against select therapeutic targets.

AstraZeneca's Oncology discovery process leverages vast datasets from over 100,000 patients—including clinical, imaging, and multiomic data—to develop AI models that generate novel hypotheses for oncology drug design, enhancing and guiding discovery. In addition to proprietary data, they also incorporate real-world evidence through partnerships with Tempus Labs, broadening patient representation on a global scale.

AI-assisted Lead Optimization

Lead optimization involves refining and enhancing the properties of potential drug candidates. This includes maintaining or improving functionality, effectiveness, availability, and affinity, while also addressing undesirable effects on their pharmacokinetic properties and safety profile.

So far, Gen AI models have shown the most promise in small-molecule compounds, where existing extensive compound libraries and foundational machine learning (both deep learning and graph neural networks) support computational predictions and guide molecular engineering.

MAYO CLINIC illumina IQVIA Arc Institute

An interesting lead optimization platform example is Charles River & Valo Health's Logica platform, which leverages vast proprietary preclinical data to generate predictive models for drug candidate identification and lead optimization. Logica's appeal lies not only in its access to a broad range of biological assay data for identifying actionable chemistries but also in its seamless integration of AI-driven selection and optimization with laboratory services under one umbrella. This approach facilitates the translation of computationally optimized leads into synthesized agents within a wet lab, priming drug candidates for traditional testing outside of computational refinement.

In this shifting landscape, partnerships and collaboration are becoming ever-salient for developing custom Gen AI models and integrated solutions. By joining forces, biopharma and Contract Research Organizations (CROs) can tap into the expertise of medtech, healthcare technology, and computational platform providers, combining proprietary, company-specific data with curated real-world clinical and molecular datasets. These collaborations not only enrich data sources for AI deployment but also foster cross-pollination of knowledge between drug and software developer teams, improving data architecture, model training, and overall AI strategy. By integrating internal data structures with external capabilities, these collaborations can enable companies to bridge critical gaps, enhance model performance, and take a significant step towards unlocking the full potential of AI-assisted innovation.

This partnership strategy is further evidenced by recent collaboration announcements such as NVIDIA's partnerships with IQVIA, Illumina, Mayo Clinic and Arc Institute to accelerate discovery, enhance genomic research and pioneer advanced healthcare services with agentic and Gen AI.

The narrative around Gen AI applications in biopharma and medtech is evolving. While setbacks and slower-than-expected value realization prompt the industry to reassess what works and what doesn't, critical learnings are emerging on how to build more sustainable, integrated models, guided by the enduring principle that Gen AI is only as good as the dataset it was trained on.

BroadBranch Advisors has deep expertise helping customers lead in the biopharma, lifesciences, and bioprocessing markets providing go-to-market strategy recommendations, competitive benchmarking, and voice-of-customer analyses. If you are interested in better understanding changing market dynamics or seek strategic guidance to help you make better decisions, please reach out to Frank Criscione (frank@brbradv.com) to learn more about how we can collaborate.

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