

Azor Biotek

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# Bespoke RNA-targeted drug design at the RNA/protein interface

Azor Biotek is applying artificial intelligence solutions to the design and development of tailored, highly specific, novel small molecules, tapping into the vast potential of RNA drug targets.

RNAs are an incredibly valuable but severely under-exploited class of drug targets.

Prior efforts to discover and develop RNA-targeted small molecules have been largely ineffective, as established biophysical and computational screening methods are generally incompatible with RNA targets. Insufficient structural data exist to apply deep-learning methods for biological complexes with RNA, and DNA-encoded combinatorial libraries cannot be effectively used to screen RNA targets. The few novel RNA-targeted drugs that have emerged are the result of target-independent phenotypic screens or functional biochemical screens.

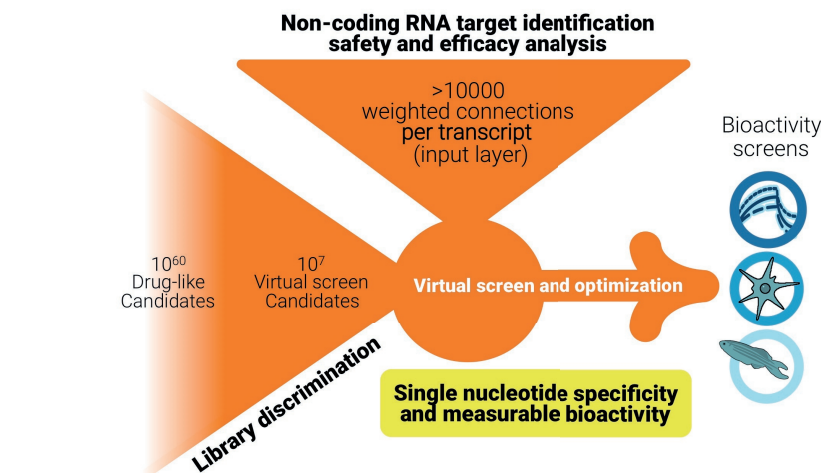
Enter Azor Biotek, a biotechnology company specializing in artificial intelligence (AI)-driven drug discovery that has been established to address the shortcomings in RNA-targeted small-molecule drug design and development. Azor has developed AI and physics-based computational chemistry techniques, as well as kinetic and in vivo screens, enabling RNA-target identification for any disease indication, and bespoke small-molecule design for any RNA target.

## Innovative RNA-guided drug design

Key to Azor's success is the AI-guided generation of novel chemical entities that target biological complexes at the RNA/protein interface. "Most companies focused on RNA use top-down approaches to discover small molecules targeting naked, labile RNA-solution structures. Unfortunately, solution structures of RNA are intrinsically disordered, fluid and ephemeral; they have yet to be clinically validated, and are only applicable to overexpressed RNA targets," explained Brock Schuman, Azor Biotek founder and CEO.

In contrast, Azor targets stabilized and ordered RNA/protein interfaces, enabling the company to design novel molecules that target any over- or under-expressed RNA. "By selectively targeting RNA molecules it becomes possible to chemically intervene at early stages in gene expression to influence protein production and, ultimately, disease progression," said Schuman. "This should enable us to unlock the tremendous potential of RNA drug targets, tailor-making and evaluating small molecules against targets previously considered undruggable, and developing therapies for many unmet medical needs."

Indeed, all RNA-acting small molecules currently in clinical use target ordered multimolecular assemblies, not 'naked' RNA-solution structures, according to Schuman. "The current market for RNA-targeted small molecules is valued at more



**Fig. 1 | RNA-targeted drug development.** To identify RNA targets safely, drug-like candidates are screened virtually and optimized using Azor's screening platform.

than \$113 billion, with only four established drug targets—each of them in biological complex with proteins," he said. "By targeting bioactive complexes at the RNA/protein interface, Azor is set to tap the clinically viable RNA drug-target market."

## Azor's novel software suite

Azor takes a comprehensive approach to RNA-targeted drug development, including target identification and analysis, library generation, virtual screening, lead optimization, and early-stage functional screening in vitro and in vivo (Fig. 1). Bioactive RNA-target engagement predicted with Azor's screening platform has been validated in vitro, evaluating specific cleavage-enhancing or -inhibiting properties of test compounds with its novel high-throughput kinetic assays. In addition to generating highly specific and bioavailable small molecules targeting RNA/protein assemblies, the company's platform can systematically identify safe and effective candidate RNA targets for a given disease indication. The company is offering these as strategic alliances and services to the drug development community.

In addition, Azor is developing its own portfolio of pharmacological assets to treat Parkinson's disease, pulmonary fibrosis and several prevalent cancers—each potentially disrupting multibillion-dollar markets. "Our pilot therapeutics are designed to target miR-29 with exquisite specificity and bioavailability, dwarfing that of comparable oligonucleotides brought to clinical trial by Viridian Therapeutics," said Schuman. The candidates,

which are available for out-licensing, are currently being evaluated in zebrafish angiogenesis models and bio-printed vascular breast cancer constructs.

## Tailored partnering

Azor is offering its innovative target selection/evaluation, novel drug design and early-stage development services to research organizations, particularly those with robust phenotypic disease models that wish to evaluate a small, bespoke, focused screen of novel molecules. Costing can be tailored to means, from commercially available compounds to ultrahigh-specificity novel entities with complicated strategies for chemical synthesis. Azor is also looking for additional chemical partners open to exchanging synthetic services for shared intellectual property (IP) or equity.

"Our innovative RNA-compatible, AI-guided novel drug development system combines structure-guided, machine-learning and other computational chemistry techniques to provide widely applicable and effective solutions for any indication," said Schuman. "We are poised to fulfil the enormous potential of RNA drug targets, developing safe and effective treatments for a wide variety of diseases."

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