

Target. Silence. Transform: Advancing ASO Therapeutics with Sai Life Sciences

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Abstract*:
Antisense oligonucleotide (ASO) therapeutics are rapidly emerging as a precise and versatile platform for modulating gene expression. With expanding clinical success across oncology, neurology, and metabolic diseases, ASOs offer significant therapeutic potential. Yet, their development is hindered by unique challenges—including poor cellular uptake, rapid clearance, nuclease degradation, and difficulty achieving tissue-specific delivery—making PK/PD prediction and translation complex.

At Sai Life Sciences, we offer an integrated platform to accelerate ASO discovery and development. Our workflow begins with ASO design and in vitro screening in disease-relevant models. Internal case studies targeting oncogenic and metabolic genes utilize literature-reported ASOs and are evaluated through qPCR, Western blot, ELISA, and phenotypic assays. This enables multi-parametric analysis of potency, specificity, and mechanism of action, supporting high-throughput rank-ordering and efficient candidate triaging.

To bridge discovery and translation, we leverage high-resolution LC-MS for quantitative analysis of ASOs and their metabolites in plasma, tissues, and cellular lysates. These methods provide sensitive, accurate PK and ADME profiling, capturing full-length sequences and truncated forms to inform lead optimization, stability, and biodistribution.

Complementing this, our in vivo studies deliver critical data on efficacy, toxicity, and tissue-specific uptake—closing the gap between bench and bedside.

Sai Life Sciences brings together scientific rigor, operational flexibility, and a collaborative mindset to support partners across the ASO development continuum. From design to in vivo validation and PK/ADME analytics, our end-to-end platform empowers data-driven decisions, enabling faster development of next-generation oligonucleotide therapies that can transform patient outcomes.