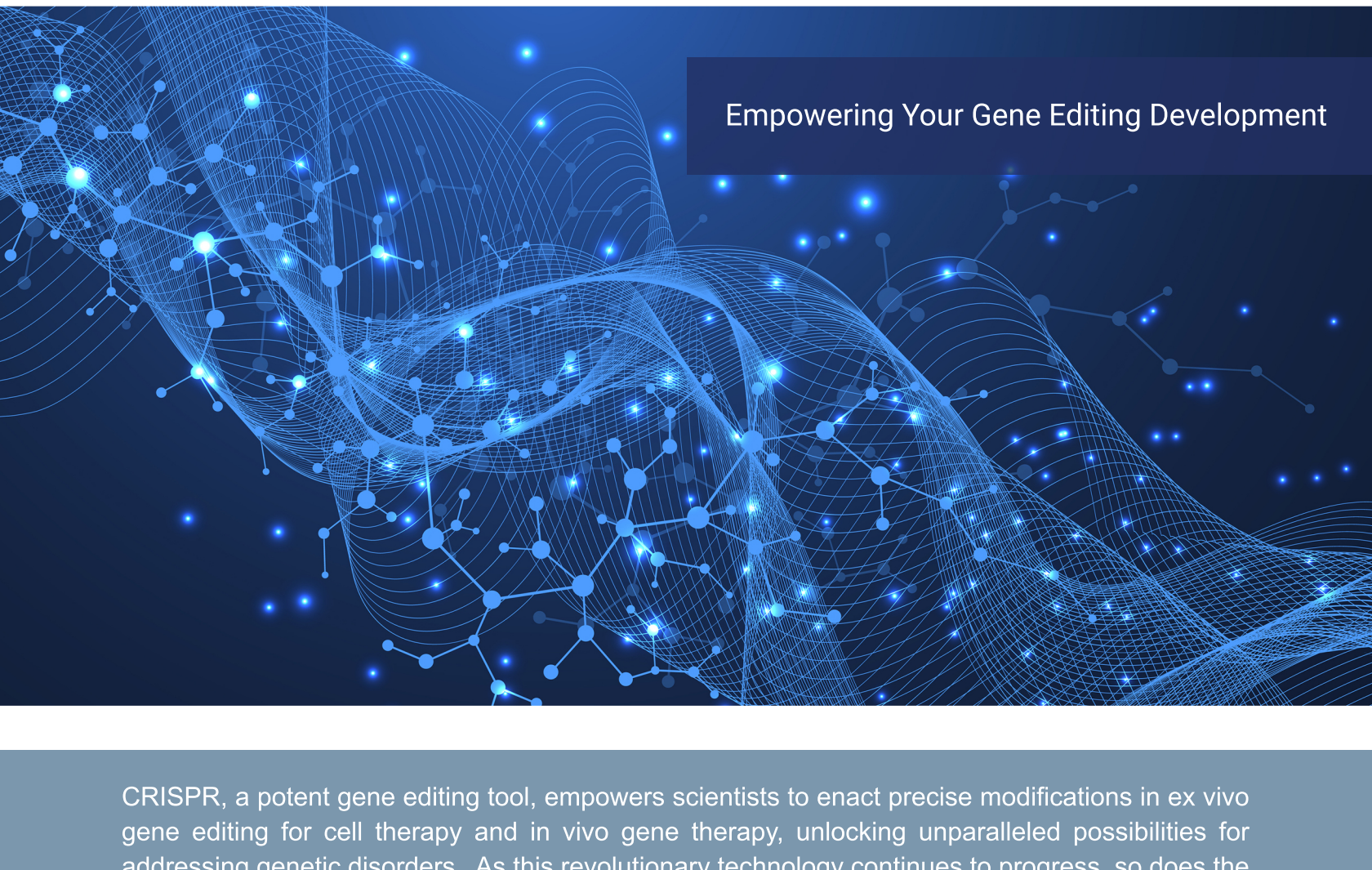
AVANCE BIOSCIENCES

CRISPR Gene Editing On/Off-Target Analysis



CRISPR, a potent gene editing tool, empowers scientists to enact precise modifications in ex vivo gene editing for cell therapy and in vivo gene therapy, unlocking unparalleled possibilities for addressing genetic disorders. As this revolutionary technology continues to progress, so does the need for rigorous characterization of the edited genes in compliance with regulatory requirements.

Avance Biosciences' team is your trusted partner in navigating the complex analytical landscape of gene editing. Boasting more than 20 years of expertise in pioneering biological assay development and validation, we deliver cutting-edge solutions for evaluating the effectiveness of on-target editing and monitoring off-target editing events in gene-edited T-cells, stem cells, iPSC, sgRNA/mRNA in LNP, and for both preclinical and clinical research studies.

Identification and Quantification of Off-Target Effects

To meet evolving regulatory standards and ensure the safety and efficacy of gene editing-based therapies, researchers must take proactive measures to detect and mitigate off-target effects. Avance Biosciences™ offers a comprehensive range of services to support your development efforts.

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A On/Off-Target Sites Screening and Verification

Our team possesses substantial experience in utilizing IDT's rhAmpSeq™ technology to screen potential off-target sites through in silico primer design and Illumina sequencing. Additionally, we have substantial experience in employing Guide-Seq, licensed from SeQure-Dx, for cross-verifying potential off-target sites. Moreover, our proficient scientific team is well-equipped to assist you in establishing alternative or complementary on/off-target analysis methodologies to meet your specific research and regulatory requirements.

B On/Off Target Sites Characterization and Quantification

Our Next-Generation Sequencing (NGS) team has collaborated closely with a diverse array of biopharmaceutical clients engaged in gene and cell therapy. Together, we've developed intricate amplicon sequencing panels designed to confirm and quantify on/off-target indel events stemming from various gene editing techniques. We have meticulously validated multiple amplicon assays in full compliance with FDA GLP and CGMP standards, all aimed at bolstering our clients' preclinical and clinical investigations, spanning in vivo and ex vivo CRISPR-based gene and cell therapies.

C Gene Editing Translocation Study

Leveraging Bio-Rad's QX200 ddPCR platform, we specialize in crafting highly sensitive assays specifically engineered to identify potential genome translocation events following gene editing. Our team boasts extensive experience in validating novel and complex assays in accordance with ICH guidelines, thus ensuring robust support for the characterization of gene editing cell therapy products.

D Other Analytical Methods to Support Gene Editing

In addition, we offer a wide array of other assays and services to facilitate the development and production of gene and cell therapeutics.

- Preclinical biodistribution and gene/protein expression studies
- Clinical PK/PD studies using QPCR, ddPCR, NGS, and ELISA
- Cell-based potency assays for characterization and lot release
- Stability study for drug substance/product characterization
- sgRNA and mRNA ID and ration testing
- On/Off-target rhAmpSeq panel development and validation
- On-target amplicon sequencing assay development and validation
- Various other assays for characterization and lot release including compendial assays

E Data Analysis Pipeline

Our adept teams in Bioinformatics and IT possess extensive experience in setting up diverse NGS analysis pipelines, whether internally or through technology transfer from our clients. We take measures to ensure a consistent computation environment for bioinformatics pipelines throughout our clients' drug development lifecycle and beyond. Moreover, we have implemented a strong data integrity mechanism in our NGS analysis, encompassing raw data verification, user controls, and an audit trail, among other measures.

Why Choose Avance Biosciences™?

- Experience: With over 20 years in the industry, our team brings unparalleled expertise to your gene editing projects.
- Regulatory Compliance: We operate in compliance with GLP and GMP guidelines, ensuring your data stands up to regulatory scrutiny.
- Cutting-Edge Technology: Our use of NGS, ddPCR, and advanced bioinformatics allows for precise and reliable analysis.
- Collaborative Partnership: We work closely with you to develop scientific and operational roadmaps aligned with your research, clinical, and commercial objectives.

Contact Us

Unlock the full potential of your gene editing programs with Avance Biosciences. Contact us today to learn more about our quantification of on/off-target gene editing services and discover how we can help you navigate the evolving CRISPR gene editing landscape.

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