FACT SHEET

On- and Off-Target Gene Editing Analysis

Target Enrichment Sequencing (TES)

Why Off-target Analysis by TES?

Detecting off-target edits in gene editing-based gene therapy is essential to ensure the safety, precision, and efficacy of the treatment. Identifying unintended genetic changes enables researchers to optimize gene editing tools, enhance target specificity, and reduce risks prior to clinical application. Moreover, thorough detection is critical for meeting ethical standards, fulfilling regulatory requirements, and monitoring long-term effects in patients.

Optimized TES method achieves high sensitivity with low DNA input. It allows the detection of insertions and deletions (indels), chromosomal translocations and intended & unintended target site modifications. Our detection method does not rely on exogenous tags, nor does it require living, dividing cells or cells fixed after double-strand break induction. The method's adaptability allows it to be tailored and validated for any genome editing (GE) approach using sequence-specific probes.

This broad applicability makes it an excellent fit for the entire lifecycle of gene editing therapeutic development — from basic research through regulatory submission, manufacturing, and even patient follow-up in clinical settings.

ProtaGene delivers a full service tailored to the needs of each client, from receiving samples to report delivery, including sample QC, library preparation, next-generation sequencing, bioinformatics analysis, and reporting.

ProtaGene
Analytical Excellence

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In addition to basic analyses focusing on detection and characterization of intended and unintended gene editing events, our advanced bioinformatics packages allow for characterization of targeted and random donor template integration profile, clonality and diversity of on- and off-target modifications allowing for deeper safety assessments. Our expert team will also work with you for the development of customized techniques or analysis workflows to support your investigations or eventual regulatory requirements.

Providing Expert Support at Every Stage

The FDA and EMA emphasize a rigorous and multi-layered approach to safety assessment for gene editing-based therapies. Both agencies require detailed characterization of genome integrity, including assessments of insertions, deletions, chromosomal rearrangements and vector integration. Long-term follow-up studies are strongly recommended, particularly for therapies involving permanent or integrating modifications, to monitor delayed adverse effects such as tumorigenicity.

Working With Us

ProtaGene provides cell and gene therapy sponsors with patient testing services in compliance with FDA and EMA guidance.

We are recognized as a leading provider of gene therapy safety assessment services of different gene therapy vector platforms and cell therapies for submission to regulatory agencies.

Our test menu includes vector persistence testing, gene editing on-/off-target analysis, vector copy number, quality control of vector batches, immune repertoire analysis, and dedicated bioinformatics studies.

With a focus on gene editing safety, characterization and efficacy, our team works in compliance with GCP and GLP standards in a BSL-2 classified state-of-the-art genomics and bioinformatics laboratory.

Peter Colbourne
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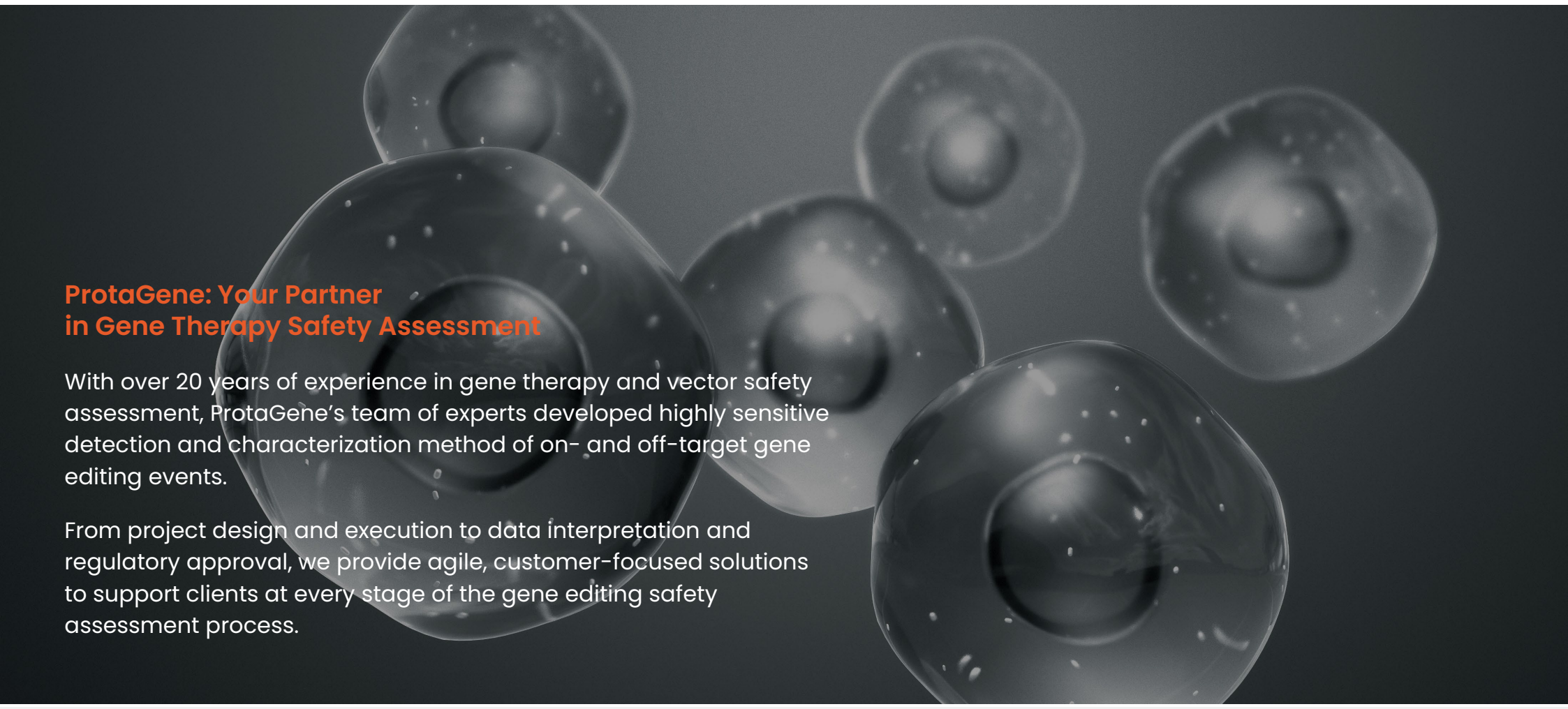

ProtaGene: Your Partner in Gene Therapy Safety Assessment

With over 20 years of experience in gene therapy and vector safety assessment, ProtaGene's team of experts developed highly sensitive detection and characterization method of on- and off-target gene editing events.

From project design and execution to data interpretation and regulatory approval, we provide agile, customer-focused solutions to support clients at every stage of the gene editing safety assessment process.

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