# Preclinical Genome Editing Risk Assessment Services.

MaxCyte provides assays and services to reduce risk and improve the safety profile of genome-editing-based therapies. Our suite of SeQure assays and proprietary reporting has been deliberately designed to support regulatory submissions. Our orthogonal screening, nomination and confirmation assays provide sensitive, variant-aware risk assessment across diverse genome editing platforms. Results are reported in a ready-to-file package that includes method justification, bioinformatics pipeline documentation, and tabulated data and metadata, supporting alignment with the latest FDA draft guidance for "Safety Assessment of Genome Editing in Human Gene Therapy Products Using Next-Generation Sequencing"[1].

## Enabling Regulatory Confidence

The SeQure assay portfolio was designed to address regulatory expectations and remains relevant to many of the recommendations contained in the most recent FDA draft guidance[1]. Our nomination and confirmation assays incorporate phase-appropriate GLP principles and generate data with the quality, reproducibility, and sensitivity expected for regulatory review. Our partners receive comprehensive data packages that include functional annotations to clarify the biological relevance of off-target edits and enable risk stratification.

## Designing and Screening Gene Editing Components

The FDA recommends developers optimize their genome editing components to reduce the potential for off-target activity, noting that a "description of, and rationale for, the design and screening processes should be provided in the IND"[2]. SeQure screening assays offer early insights into on- and off-target sites, providing precision in variant-aware guide RNA design and selection, delivered in a filing-ready report.  Addressing regulatory expectations during guide design may reduce program risk, accelerate development, and reduce costs.

## Screening Assays

The industry's most advanced early-stage screening platform, ensuring therapy developers select the safest and most effective guide RNAs from the start—reducing risks, optimizing efficiency, and accelerating development timelines.

### Guide Profiler

In silico screening for potential off-target effects associated with your gene editing tool. Enables you to start your gene editing risk assessment by rapidly screening sgRNAs to understanding on-target editing and off-target editing risks.

### Guide Select

ONE-seq off-target screening assay designed to evaluate multiple guide RNAs in parallel. Variant-aware searching with up to 4 differences from the intended on-target sequence. Enables high-powered, low-cost risk assessment for informed guide RNA selection.

1. Safety Assessment of Genome Editing in Human Gene Therapy Products Using Next-Generation Sequencing. Draft Guidance for Industry. April 2026. https://www.fda.gov/media/191966/download

2. Human Gene Therapy Products Incorporating Human Genome Editing; Guidance for Industry, January 2024, https://www.fda.gov/media/156894/download.

## Off-Target Nomination, Confirmation and Risk Assessment

The FDA emphasizes the need for sensitive, variant-aware, orthogonal assessment strategies to evaluate both on- and off-target editing events, genomic integrity, and associated biological impacts[1]. Our suite of nomination and confirmation assays align with these expectations, and provide results in an integrated, filing-ready report that delivers actionable insights.

## Nomination Assays

The most comprehensive off-target nomination suite available, leveraging multi-layered biochemical and cell-based approaches to deliver unmatched sensitivity and confidence in genome editing safety during preclinical development.

### ONE-seq
### ONE-seq-BE

Population-scale, variant-aware, off-target nomination. Candidate off-target quantification, enrichment, and in vitro cutting combined with NGS enables high sensitivity nomination of low frequency off-target events.

### DEUX-seq

Unbiased biochemical off-target nomination assay that provides a complementary approach to supplement your off-target risk assessment efforts.

### CHANGE-seq
### CHANGE-seq-BE

An unbiased, sensitive, high-throughput biochemical method that provides comprehensive off-target nomination as part of genome editing risk assessment.

### GUIDE-seq

Unbiased in cellulo off-target nomination assay to supplement your off-target risk assessment.

## Confirmation Assays

Regulatory-ready assays that confirm on- and off-target editing and detect structural variants, addressing key regulatory recommendations as part of the clinical submission.

### Amplicon-seq

High sensitivity confirmation of off-target editing in your cell type of interest. Informed by nomination assays, NGS pinpoints off-target edits with resolution down to exact genomic coordinates.

### SAFER Detection

High sensitivity confirmation of structural rearrangements in your cell type of interest post-editing. NGS interrogation allows for resolution down to exact genomic coordinates.

## About SeQure

We began with a mission to reduce risks in genome-edited cell therapy development and manufacturing, enabling safer therapies to reach patients faster. Our leading on- and off-target assessment assays empower precision medicine advancements across diverse viral and non-viral genome editing platforms.

Our comprehensive portfolio of regulatory-aligned screening, nomination and confirmation assays supports the rigorous analytical and safety standards required for genome editing safety assessments, from early discovery to preclinical and IND-enabling studies.

Learn more at: www.maxcyte.com/sequre



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