Application:

Cell and Gene Therapy (CGT)



Empower Your CGT Development Programs with Sensitive and Precise Single-Cell Multiomics

Challenges in CGT Development



Viral Gene Therapies

- Insertional mutagenesis can give rise to neoplastic changes
- Uncertainty in transduction efficiency, gene expression, and tissue targeting



CRISPR/Cas9 Gene Editing

- Off-target Cas9 activitiy results in mutations that vary between cells
- · Difficult to confirm zygosity

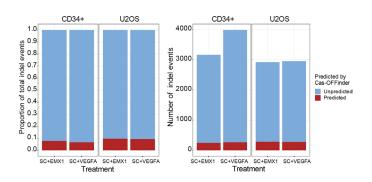


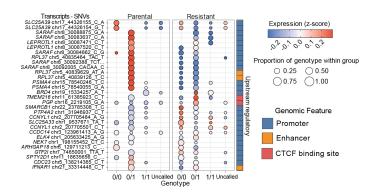
Induced Pluripotent Stem Cell (iPSC) Therapies

- Oncogenic potential from genomic and transcriptomic variation in iPSCs
- Heterogeneity in cell idenity

Solutions from BioSkryb Genomics

Primary template-directed amplification (PTA) technology, available in ResolveDNA® and ResolveOME™ kits, enables multiomic analysis at the single-cell level, empowering CGT researchers to sensitively and precisely detect both desirable and deleterious changes in individual candidate cells.





Detect off-target events in single cells comprehensively.

Analysis of PTA-enabled whole genome sequencing data¹ reveals thousands of indels detected following CRISPR/Cas9 gene editing with EMX1 or VEGFA guide RNAs in single cells (SC). Proportion of or total number of indels detected versus predicted in each group shown.

Identify underlying mechanisms of gene expression changes in individual cells. Analysis of parental and drugresistant cell lines following ResolveOME whole genome and transcriptome amplification reveals single nucleotide variants (SNVs) in non-coding upstream regulatory regions that are associated with changes in gene expression.²

References

- 1. Gonzalez-Pena et al., "Accurate genomic variant detection in single cells with primary template-directed amplification ". PNAS. (2021).
- 2. Marks et al., "Unifying comprehensive genomics and transcriptomics in individual cells to illuminate oncogenic and drug resistance mechanisms". bioRxiv. (2023)

ResolveDNA® and **ResolveOME™** Assay Performance

| Table 1: ResolveOME WGS DNA Performance* | | |
|--|-----------------|--|
| Characteristic | Observed Values | |
| Accuracy | 99.5% | |
| Sensitivity | 97.1% | |
| Specificity | 99.2% | |
| Allelic Balance | 98.4% | |
| Genomic Coverage | 97.1% | |

| Table 2: ResolveOME WTS RNA Performance | | |
|---|-----------------|--|
| Characteristic | Observed Values | |
| Genes Detected | 4,546 | |
| Reportable Range | 6,057 | |
| Average Concordance | 0.91 | |
| Reproducibility (CV) | 43.3% | |

Table 1: Assay performance characteristics of DNA isolated using ResolveOME. Analysis of FACS-sorted
NA12878 single cells prepared with ResolveOME versus
gold-standard reference. WGS: whole genome sequencing.

Table 2: Assay performance characteristics of RNA isolated using ResolveOME. Analysis of FACS-sorted
NA12878 single cells prepared with ResolveOME versus goldstandard reference. WTS: whole transcriptome sequencing.

Custom Services

We offer custom service packages from our end-to-end single-cell multiomic pipeline, from singulating cells to ready-to-publish figures. All services include quality control verification. Services include:

- Cell sorting from fresh or frozen cells and tissues
- Whole genome amplification or unified whole genome and transcriptome amplification
- · Library preparation for downstream applications, such as whole genome or targeted sequencing
- Sequencing of 550M quality reads capturing >97% of the human genome from each cell
- Analysis using our bioinformatics platform, BaseJumper™

Products

| Code | Product | Description |
|--------|---|--|
| 100500 | ResolveOME™ Whole Genome and Transcriptome Amplification System | PTA-based kit for accurate and reproducible whole genome and transcriptome amplification from single cells. |
| 100545 | ResolveDNA® Whole Genome Amplification Kit | PTA-based kit for accurate and reproducible whole genome amplification from single cells and low-input DNA inputs. |
| 100605 | BaseJumper™ Bioinformatics Platform | A complete bioinformatics solution for multiomic data analysis and visualization. |

For a complete list of services, products, and pricing, email a member of our team at info@bioskryb.com



All data on file.

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^{*}DNA amplified using ResolveDNA and ResolveOME have comparable DNA performance characteristics. All data on file.