

# 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 activity results in mutations that vary between cells
- Difficult to confirm zygosity

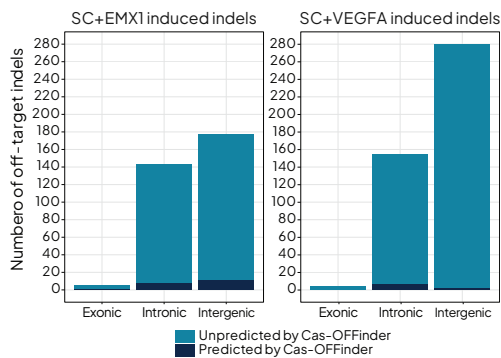


#### Induced Pluripotent Stem Cell (iPSC) Therapies

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

### 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.

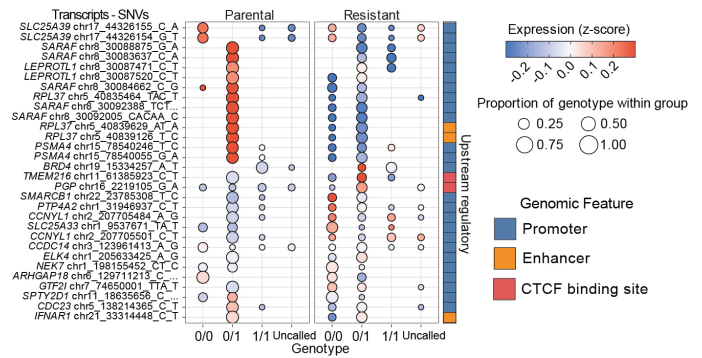


#### Detect off-target events in single cells comprehensively.

Analysis of PTA-enabled whole genome sequencing data<sup>1</sup> reveals hundreds of indels detected following CRISPR/Cas9 gene editing with EMX1 or VEGFA guide RNAs in single cells (SC). Total number of indels detected versus predicted in each group shown.

#### 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)



#### Identify underlying mechanisms of gene expression changes in individual cells.

Analysis of parental and drug-resistant 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.<sup>2</sup>

## ResolveDNA® and ResolveOME™ Assay Performance

**Table 1: ResolveOME WGS DNA Performance\***

| Characteristic   | Observed Values |
|------------------|-----------------|
| Accuracy         | 99.99%          |
| Sensitivity      | 96.65%          |
| Specificity      | 99.99%          |
| Allelic Balance  | 91.20%          |
| Genomic Coverage | 97.59%          |

**Table 1: Assay performance characteristics of DNA isolated using ResolveOME.** Analysis of NA12878 single cells prepared with ResolveOME Whole Genome Single-Cell Core Kit versus gold standard reference.WGS: whole genome sequencing.

**Table 2: ResolveOME WTS RNA Performance**

| Characteristic       | Observed Values |
|----------------------|-----------------|
| Protein Coding Genes | 3451 ± 732      |
| Concordance          | 0.97            |
| Variance (CV)        | 32.9%           |

**Table 2: Assay performance characteristics of RNA isolated using ResolveOME.** Analysis of NA12878 single cells prepared with ResolveOME Whole Genome Single-Cell Core Kit versus gold-standard reference. WTS: whole transcriptome sequencing.

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

### 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™<br>Whole Genome and<br>Transcriptome Amplification System | PTA-based kit for accurate and reproducible whole genome and transcriptome amplification from single cells.        |
| 100545 | ResolveDNA®<br>Whole Genome Amplification Kit                         | PTA-based kit for accurate and reproducible whole genome amplification from single cells and low-input DNA inputs. |
| 100605 | BaseJumper™<br>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](mailto:info@bioskryb.com)**

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GENOMICS

All data on file.

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