## **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

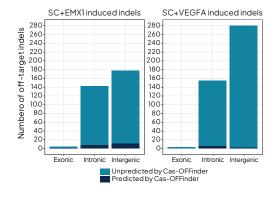


#### Induced Pluripotent Stem Cell (iPSC) Therapies

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

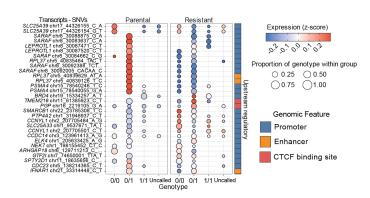
#### Solutions from BioSkryb Genomics

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.





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.



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.<sup>2</sup>

#### 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)

### **Assay Performance**

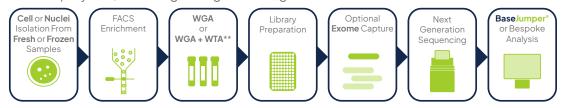
Table 1: ResolveOME WGS DNA Performance		
Characteristic	Observed Values	
Accuracy	99.99%	
Sensitivity	96.65%	
Specificity	99.99%	
Allelic Balance	91.20%	
Genomic Coverage	97.59%	

Table 2: ResolveOME WTS RNA Performance			
Characteristic	Observed Values		
Protein Coding Genes	3451±732		
Concordance	0.97		
Variance (CV)	32.9%		

Assay performance characteristics of DNA (Table 1) and RNA (Table 2) isolated using ResolveOME Whole Genome and Transcriptome Single-Cell Core Kit. Analysis of FACS-sorted NA12878 single cells prepared with ResolveOME versus gold-standard reference. WGS: whole genome sequencing. WTS: whole transcriptome sequencing.

#### ResolveServices<sup>SM</sup>

Custom-built service projects, from singulating cells to figures. Services can include:



 $FACS: Fluorescence-Activated Cell Sorting, WGA: Whole Genome Amplification, WTA: Whole Transcriptome Amplification, \\ **Optional {\bf targeted protein} detection available$ 

#### **Products**

Product	Description	Codes	Well Format
ResolveOME <sup>TM</sup>	PTA-based kit for whole genome and	100956	96
Whole Genome and Transcriptome Single-Cell Core Kit	transcriptome amplification plus NGS library preparation from single cells.	100957	384
ResolveDNA®	PTA-based kit for whole genome amplification plus NGS library preparation from single cells.	100954	96
Whole Genome Single-Cell Core Kit		100955	384
ResolveDNA® Whole Genome Amplification Kit	PTA-based kit for whole genome amplification from as little as 4 pg input DNA.	100545	Up to 384*
BaseJumper® Bioinformatics Platform	A platform for multiomic data analysis.	100605	-
ResolveXOME™ Exome Capture Module	Exome capture module for use with ResolveDNA or ResolveOME Single-Cell Core Kits.	Early- Access	-

<sup>\*</sup>Dependent on workflow option used

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



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All data on file

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