elevatebio
life edit®

Life Edit Gene Editing Technologies and R&D – Making Any Edit, Anywhere, Possible

ElevateBio Life Edit is our gene editing technologies and R&D business, offering one of the world's largest and most diverse collections of RNA-guided nucleases (RGNs), base editors, and reverse transcriptase (RT) editors that provide flexible editing and unprecedented access to the genome. With multiple editing modalities and protein engineering expertise, our technology can modify gene targets both *in vivo* and *ex vivo* to potentially address the most challenging genetic diseases. In addition, Life Edit has broad therapeutic delivery capabilities, including viral and non-viral delivery platforms to provide flexibility in therapeutic design and application, unlocking new possibilities for addressing a wide range of diseases and disorders.

Life Edit's diverse DNA recognition sequences are key

Our nuclease collection features diverse DNA recognition sequences – **protospacer adjacent motifs (PAMs)**, that determine where gene editing tools can bind and operate.

Our nucleases can access virtually any region of the genome, increasing the number of specific sites where therapeutically meaningful edits can be made. Life Edit's RGNs, sourced from non-pathogenic microbes are smaller in size (~800-1,100 amino acids) when compared to conventional nucleases, while offering higher fidelity and novel functionality. This compact size enables greater versatility in packaging our systems for therapeutic delivery, potentially overcoming key limitations in getting gene editing tools to target tissues.

Advanced editing with the industry's largest gene editing toolbox:

1. **Nuclease Editing**: Nuclease-based editing involves cutting both strands of the DNA, enabling gene insertion (knock-in) or deletion (knock-out) at the cut site. Our collection of compact RGNs allows us to introduce knock-out or knock-in edits with precision across the genome, significantly expanding the range of addressable genetic targets and enabling therapeutic approaches for complex mutations.

2. **Base Editing**: Base editing converts one nucleotide (base) into another without cutting both DNA strands, achieved by coupling a modified nuclease to a deaminase that edits the target nucleotide. Our modular approach includes A and C base editors for both *ex vivo* and *in vivo* applications with demonstrated multiplex editing capabilities. This technology enables precise nucleotide-level correction of disease-causing mutations or strategic disruption of coding sequences to modulate gene expression.

3. **Reverse Transcriptase Editing**: RT editing, also known as prime editing or DNA writing – creates a targeted single-strand break, then replaces existing DNA sequence with new sequence encoded by the guide RNA. Our approach leverages an extensive panel of RGNs coupled with expertise in target screening, analysis, and optimization to achieve optimal editing outcomes at each target locus.

4. **Epigenetic Editing**: Epigenetic editing enables precise control of gene expression without altering the underlying DNA sequence. Our platform can selectively turn genes on, off, or finely tune their expression levels in a programmable manner. These changes can be durable or transient, enabling therapies designed for long-lasting benefit or reversible control. When delivered by lipid nanoparticles (LNP), epigenetic modulation supports repeat dosing, allowing gene expression to be adjusted over time based on disease biology. This flexibility makes epigenetic editing well suited for indications requiring correction of gene dosage and modulations of gene networks or pathways, where permanent DNA alterations may be unnecessary or undesirable.

5. **Targeted Gene Insertion:** Targeted gene insertion enables precise placement of large (>2 kb) therapeutic DNA sequences into the genome, supporting integrations ranging in size from single exons to full-length genes for durable expression of functional proteins. This approach can broaden access to genetic medicines by enabling a single therapeutic to address an indication with multiple causative mutations. It is particularly attractive for chimeric antigen receptor (CAR) therapeutics, combining the durability of viral delivery with the safety profile of non-viral methods. Our platform leverages proprietary engineered retrotransposons and large serine recombinases (LSRs), providing flexibility for diverse downstream applications.

Accelerating CRISPR discovery and design with artificial intelligence

We harness artificial intelligence to accelerate CRISPR discovery, combining our protein engineering expertise with a vast library of over 20 billion proteins to design optimal editing systems for thousands of previously untreatable genetic disorders.

Our sophisticated protein language models analyze vast datasets to uncover new CRISPR systems beyond what traditional methods could identify. Through our multi-year collaboration with Amazon Web Services (AWS), we train and execute these models at scale. By combining generative AI with our proprietary experimental data, we can predict, design, and optimize both naturally occurring and synthetic CRISPR proteins with unprecedented precision.

We can match the best editing modality to the indication, combining the breadth of technology and depth of expertise to develop custom CRISPR medicines, designed for greater disease access.

Breadth of Technology

- Nuclease editors
- Base editors
- Reverse transcriptase editors
- Targeted large insertion editors
- Epigenetic editors
- Viral and LNP delivery

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Depth of Expertise

- Portfolio of validated CRISPR systems
- Large protein database
- Custom Protospacer Adjacent Motifs (PAM) recongnition design
- Capability to enhance potency and specificity
- Extensive off-target characterization

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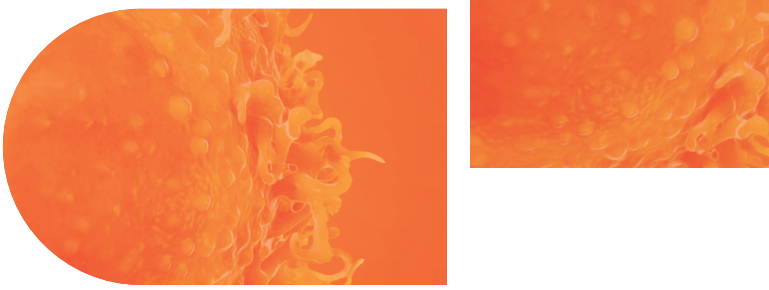
Custom CRISPR Medicines

- High potency and increased safety with greater disease access
- Multiple gene editing modalities

Combining Life Edit with BaseCamp

Powering Cell and Gene Therapy Companies with Integrated Manufacturing and Technologies

ElevateBio BaseCamp is our cGMP manufacturing business. Through BaseCamp and Life Edit, we deliver unparalleled expertise, capabilities, quality, and high-touch customer service. We have integrated a differentiated set of platform technologies, including full-spectrum gene editing, with industry-leading manufacturing expertise to develop genetic medicines faster and more efficiently, ultimately accelerating their delivery to patients.



Partner with ElevateBio

Wherever you are in your product lifecycle, we can strengthen and accelerate the development of your transformative therapies with our enabling technologies and unmatched manufacturing capabilities.

WORK WITH US →