



Prime Editing Platform for Precise and Efficient Gene Editing

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COMPANY NAME XXX	TECHNOLOGY Gene Editing Tools	TYPE OF OWNERSHIP Public company
YEAR FOUNDED 2019	HEADQUARTERS Cambridge, Massachusetts, United States	KEY MARKETS United States
INVESTMENT STAGE Initial Public Offering (IPO)	TOTAL FUNDING \$ 315 Million	TOTAL REVENUE XXX
EMPLOYEES 51-200		

ABOUT THE COMPANY
XXX is a biotechnology company that uses prime editing as the foundation to develop novel classes of differentiated, one-time curative genetic therapies. Its co-founders, David R. Liu and Andrew Anzalone, also invented prime editing technology. The company licensed rights from the Broad Institute of MIT and Harvard, where the technology was developed, to use prime editing in its research on new human therapeutics. XXX is a gene-editing start-up and one of the first high-profile biotechnology companies to file for an IPO in several months, raising \$175 million in its IPO in October 2022.

KEY CONTACT	 NAME XXX	 PHONE XXX	 EMAIL XXX	 URL XXX
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Technology Snapshot

Prime editing technology works like a deoxyribonucleic acid (DNA) word processor, searching and replacing disease-causing genetic sequences in their exact location in the genome while avoiding double-stranded DNA breaks that cause unwanted cellular changes. The technology consists of a prime editor (PE) that the CRISPR-associated protein 9 (Cas9) nickase fusion, a Cas9 variant that nicks the DNA rather than generates DSBs, forms with a reverse transcriptase. Prime editing guide ribonucleic acid (pegRNA) is significantly larger than standard single guide (sg) RNAs, which have common usage for CRISPR gene editing (>100 nucleotides [nt] vs. 20 nt). The complex PE:pegRNA is responsible for genome editing within the cell.

What Problem does the thecnology solve?

The lack of specificity, efficiency, and off-target activities owing to the double-strand break's (DSB) repair mechanism are some of current gene-editing technology's major issues. In addition, advanced clustered regularly interspaced short palindromic repeats (CRISPR), such as base editing, can only correct transition point mutations and not non-transition mutations of genetic disease, which account for more than 70% of the 75,000 or more known disease-associated gene variants. Prime editing technology can correct these non-transition mutations, overcoming the technical barriers of CRIPSR or other CRIPSR-based technology, including base editing.

Attributes

Minimal Off-target Activity
The prime technology's main attribute is its ability to carry out gene editing without inducing DSBs in target DNA, resulting in negligible off-target activity.

Broadly Applicable in Cell Types
Prime editing can target multiple cell types, such as rapidly dividing and non-dividing mammalian and non-mammalian cell types and organs.

Versatility
Prime editing is versatile, allowing the targeted substitution, deletion, addition, transversion, or combination of all of the above without requiring a donor DNA template. All 24 single-base conversions of DNA base pairs can be done which base editing does not.

Source: Frost & Sullivan

Technology Assessment

Prime editing is a base editing technology known as search-and-replace, which uses a fusion protein and reverse transcriptase enzyme to edit gene sequences. It can overcome some of the challenges with existing CRISPR based editing which can lead to off target mutations.

Key Competitors

- XXX
- XXX
- XXX
- XXX

Strategic Analysis

S Strengths

- Prime editing is efficient and offers high precision and a wide targeting scope.

W Weaknesses

- Prime editing only had promising results in commonly used cell lines and testing in a few genetic diseases.
- The technology is still in its infancy and requires more research to fully comprehend its potential.

IP/ Patent Activity

The company filed a patent for Methods and Compositions for Editing Nucleotide Sequences.

O Opportunities

- Prime editing can potentially treat numerous genetic diseases that have no available treatment.

T Threats

- Other innovative technologies such as twin prime editing, gene writing could compete with prime editing methods.

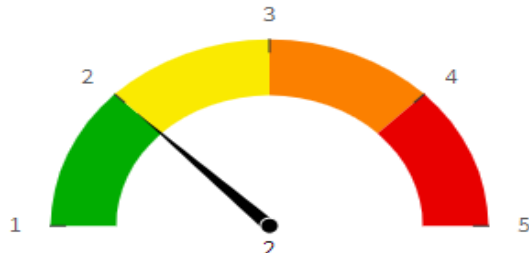
Future Focus Areas

XXX uses prime editing to develop innovative therapeutics covering a broad range of diseases that do not have disclosure yet, drug discovery programs targeting the liver, eye, and neuromuscular indications, and hematopoietic stem cells outside of the body. Prime editing can treat possible disease indications, including the rare nerve disease Tay-Sachs and sickle cell and provide resistance to neuromuscular prion-related diseases.

- The company will expand its platform's capabilities to unlock prime editing's potential and enter into more partnerships.

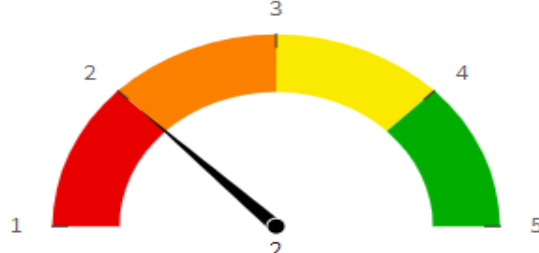
Source: Frost & Sullivan

Risk Analysis



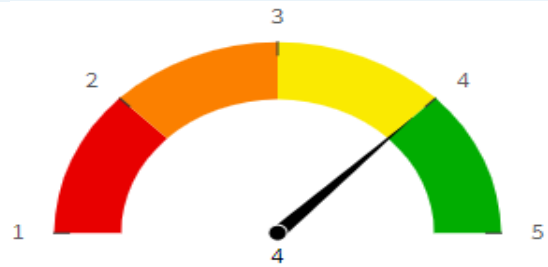
- Prime editing has high potential and showed promising results in an animal models, posing a lower risk to investors exploring this technology platform.

Revenue Magnitude Potential



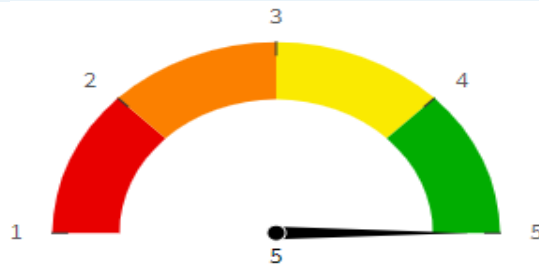
- Some large investors are backing the company. XXX currently has no clinical pipeline and has a low to medium revenue potential, but the approval of its technology-based products will lead to high revenue potential in the long term.
- The company can also generate revenue by out-licensing its technology.

Buzz Worthiness



- The company's prime technology is next-gen gene-editing technology and has the potential to treat various genetic conditions that existing gene-editing solutions cannot.

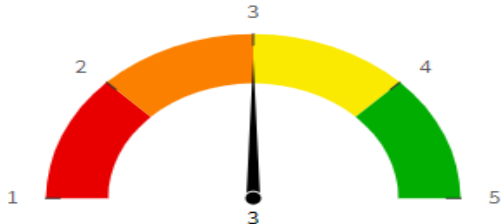
Investor Lens



- XXX raised \$200 million in a series B round in 2021 and \$175 million in an IPO in 2022.
- The company is working with Myeloid Therapeutics and Beam Therapeutics under an R&D agreement.

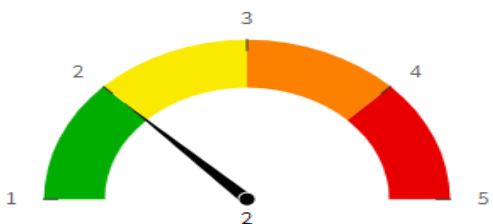
Source: Frost & Sullivan

Commercialization Readiness Level



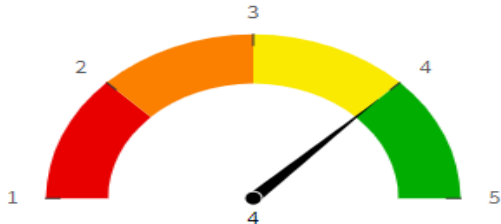
- The company is still in the early stages of drug discovery and has no product candidates in the clinical pipeline yet, hence it may take long for investors to gain ROI.

Technology Competition Level



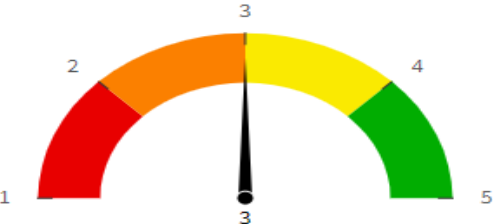
- XXX offers a innovative Platform which is not likely to face very high competition because its technology offers advantages to existing gene-editing solutions. Future technologies can compete with prime editing.

Regional Impact



- XXX ' regional impact will be the United States in the short term and potentially global in the long term.

Application Potential



- Auto-immune diseases
- Immuno-oncology
- Neurological disorders
- Genetic diseases

Analyst's Insights

XXX is working on a novel gene-editing technology that can transform the future of genome editing. Although the company only tested its solution on mice as the highest-level model organism and still has a long way to go before human trials, further research of the technology will uncover its immense potential in therapeutic applications. The company can form partnerships to strengthen its position and support its pipeline development.

Source: Frost & Sullivan