Base Editing

A potential Game-Changer In Genome editing

Base Editing expected to become affordable quickly and is likely to dominate the future for Genome Editing.

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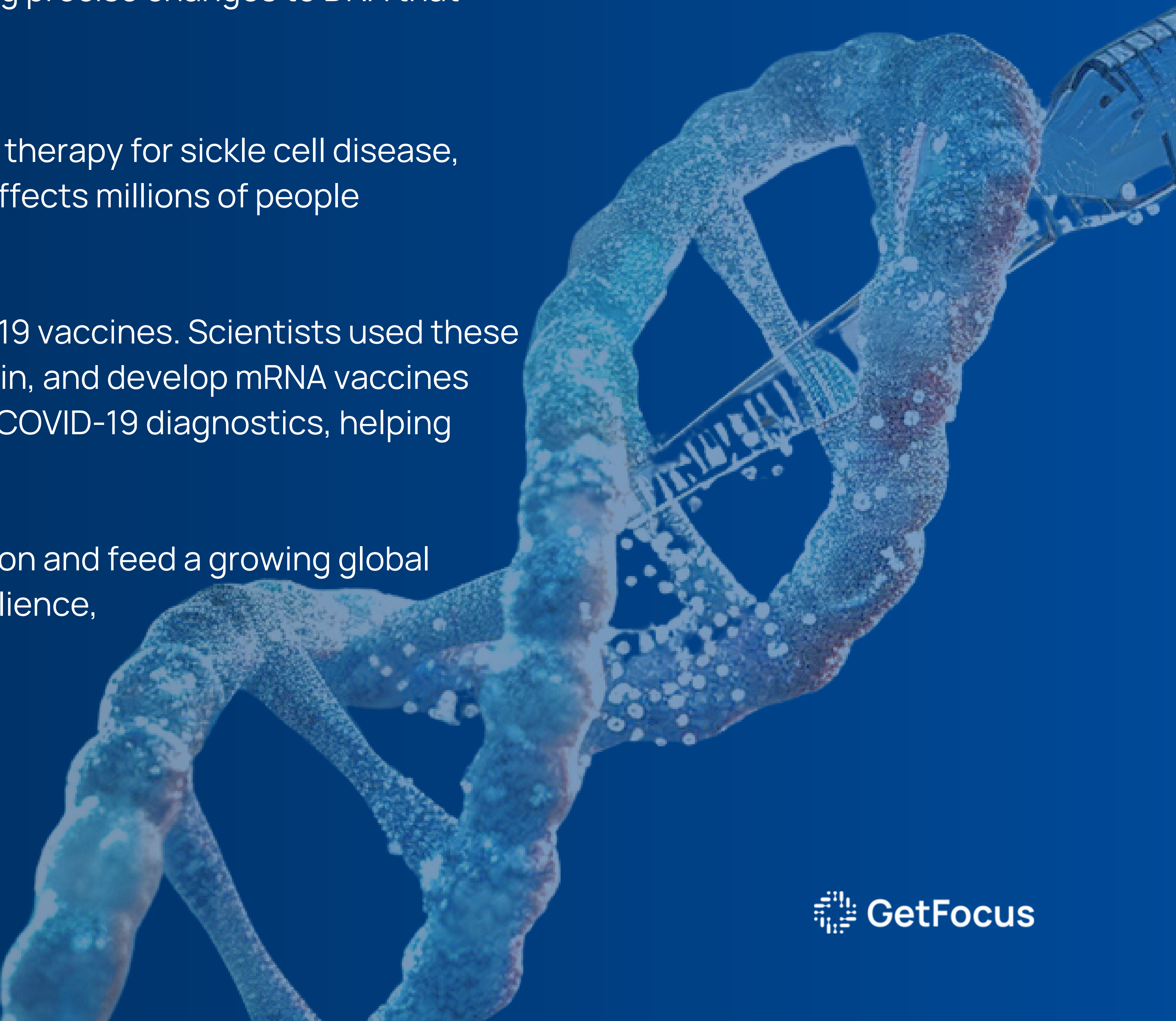
www.getfocus.eu

Why does Genome Editing matter?

Genome editing is a technique that allows scientists to change or modify the DNA in an organism's genes to alter its traits or correct genetic disorders. It has revolutionized life sciences, enabling precise changes to DNA that drive advancements in medicine, agriculture, and even pandemic response.

- In December 2023, the FDA approved the first CRISPR/Cas9-based gene therapy for sickle cell disease, marking a major milestone for treating a complex genetic disorder that affects millions of people worldwide.
- Genome editing played a critical role in the rapid development of COVID-19 vaccines. Scientists used these tools to quickly sequence the SARS-CoV-2 virus, identify the spike protein, and develop mRNA vaccines like Moderna’s. CRISPR-based technologies were also adapted for rapid COVID-19 diagnostics, helping control the virus's spread.
- In agriculture, genome editing offers solutions to increase crop production and feed a growing global population while reducing environmental impact. By enhancing crop resilience, we can produce more nutritious food with fewer resources.



However, concerns over off-target edits remain, raising safety issues.

What are Off-target edits?
Off-target edits in genetic modification are like accidentally changing "apple" to "orange" in words like "pineapple" and "applewood" when you only meant to change "apple" itself. These unintended changes can lead to potential problems and unforeseen consequences in the organism and therefore cannot be ignored.

Since 2015, ongoing efforts to refine CRISPR have led to the development of several variants, all of them promise to increase precision and minimize off-target edits.

Therefore, in this report, we evaluate CRISPR-based technologies, determine which of them is improving the fastest, and find out when can we expect it to dominate the future of Genome Editing.

The terms 'gene editing' and 'genome editing' are used interchangeably and are generally the same. Accurately speaking, 'gene editing' refers to the editing of 'genes', which are the units within heritable material that are translated into functional components such as proteins. The word 'genome' refers to the hereditary material of an organism.

When evaluating genome editing technologies, five key performance metrics are important:

Precision: This refers to how accurately the technology can target and edit specific genes. The higher the precision, the fewer unintended genetic changes occur.

Efficiency: Efficiency measures how successful the technology is at making desired genetic modifications. A higher efficiency means fewer repeated attempts are needed.

Ease of Use: This covers how simple the technology is to use, including resource availability and compatibility with existing systems.

Cost: Lower costs make the technology more accessible and easier to adopt on a wider scale.

Safety: Safety assesses the risk of unintended changes and overall biological impact. Safer technologies are more likely to gain regulatory approval and public trust.

In this table we compare the CRISPR-based Genome Editing technologies on the five key performance metrics

Technology	Brief Technical Description	Promised Advantages	Technology Readiness Level	Precision	Efficiency	Ease of Use	Cost	Safety	Avg. Score*
CRISPR/ Cas9	It utilizes the Cas9 protein and a guide RNA to locate and cut specific DNA sequences, enabling targeted gene editing.	Simple, cost-effective	9	8	8	9	7	7	**7.8**
CRISPR/ Cas12	Like Cas9, uses guide RNA to identify specific DNA sequences but has a distinct cutting mechanism, enabling it to target both single and double-stranded DNA	Ability to cut single-stranded DNA, can be more precise than Cas9.	7	8	7	8	7	8	**7.6**
CRISPR/ Cas13	Targets RNA instead of DNA, allowing for transient genetic modification and avoiding permanent changes to DNA.	Potential for real-time gene expression control. Useful in diagnostics, particularly for RNA viruses.	5	7	6	7	6	8	**6.8**
Base Editing	Allows single nucleotide changes without causing double-stranded DNA breaks. It uses modified Cas proteins fused with deaminase enzymes to directly convert one base pair to another	Very High precision. Efficiently corrects point mutations. Lower risk of inducing large-scale genomic changes.	6	9	7	8	6	9	**7.8**
Prime Editing	Combines a Cas9 nickase with a reverse transcriptase enzyme and a guide RNA to achieve precise edits	Potentially fewer off-target effects. Does not rely on double-strand breaks or donor DNA	4	9	8	7	6	9	**7.8**

Except for CRISPR/Cas13, all other technologies perform similarly across key metrics.

Therefore, to predict which technology will dominate in the future, we compare their improvement rates using the GetFocus methodology.

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GetFocus Forecasting Methodology

At GetFocus, we developed a quantitative method together with MIT to **forecast the technological future** based on metrics that can be identified in patent data.

Using the latest advancements in AI technology, we have created a system that can estimate how rapidly any area of technology is improving.

Our method revolves around **3 key steps.**

1

We identify every single patent that relates to an area of technology using AI. The resulting dataset represents the entire developmental history of an area of technology.

2

Once this dataset is created we measure 2 key metrics.

Cycle Time - How many years it takes for a technology to produce a new generation of itself.
The lower the cycle time, the better.

Knowledge Flow - How significant of a step forward a new generation represents.
The higher the knowledge flow, the better.

3

Using the previous metrics, we calculate the **'Technology Improvement Rate'**, which represents the average percentage (%) increase in performance per dollar that can be expected from an area of technology in one year.

By using the above methodology, technology improvement speeds can be accurately measured, and those speeds can be used to predict technological disruption well ahead of time.

Base Editing is set to dominate over other Genome Editing technologies.

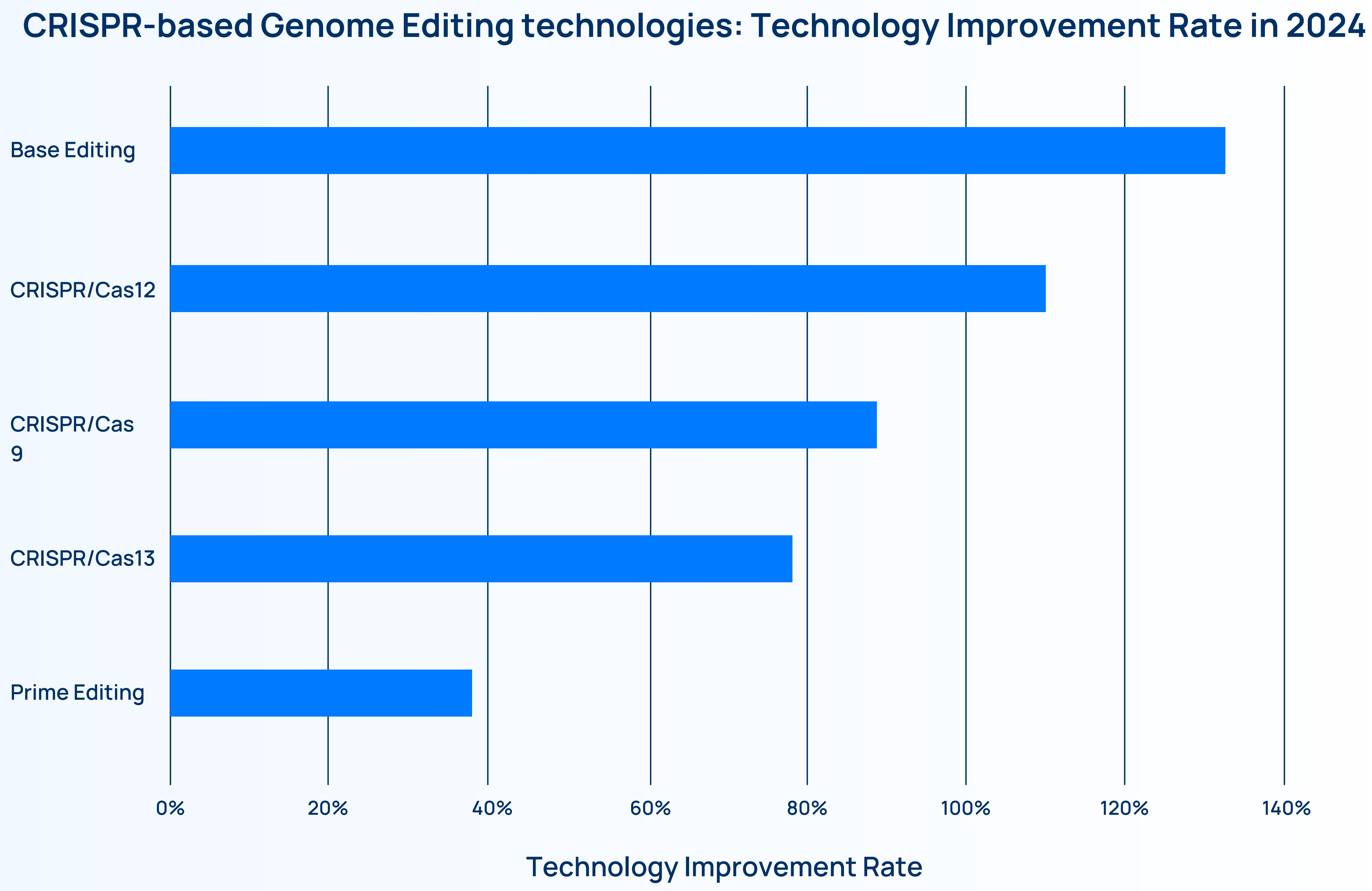
As of 2024, Base Editing is the fasting improving CRISPR-based Genome Editing technology with an **improvement rate of 136%**. This means the performance per cost of Base editing is improving the fastest compared to the competing technologies.

Base Editing compared to CRISPR Cas9:

Base Editing is a newer gene-editing technique that is still being refined. It requires fewer, more precise steps than the widely used Cas9 method, but relies on specialized materials and costly preparation, making it more expensive.

Base Editing compared to the second fastest improving CRISPR Cas 12:

Base Editing outperforms Cas12 in nearly every area except cost, despite being less mature. Cas12 involves more steps than Cas9, while Base Editing requires fewer, suggesting that once fully developed, Base Editing could be cheaper or on par with Cas12.



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Is Base Editing really too expensive?

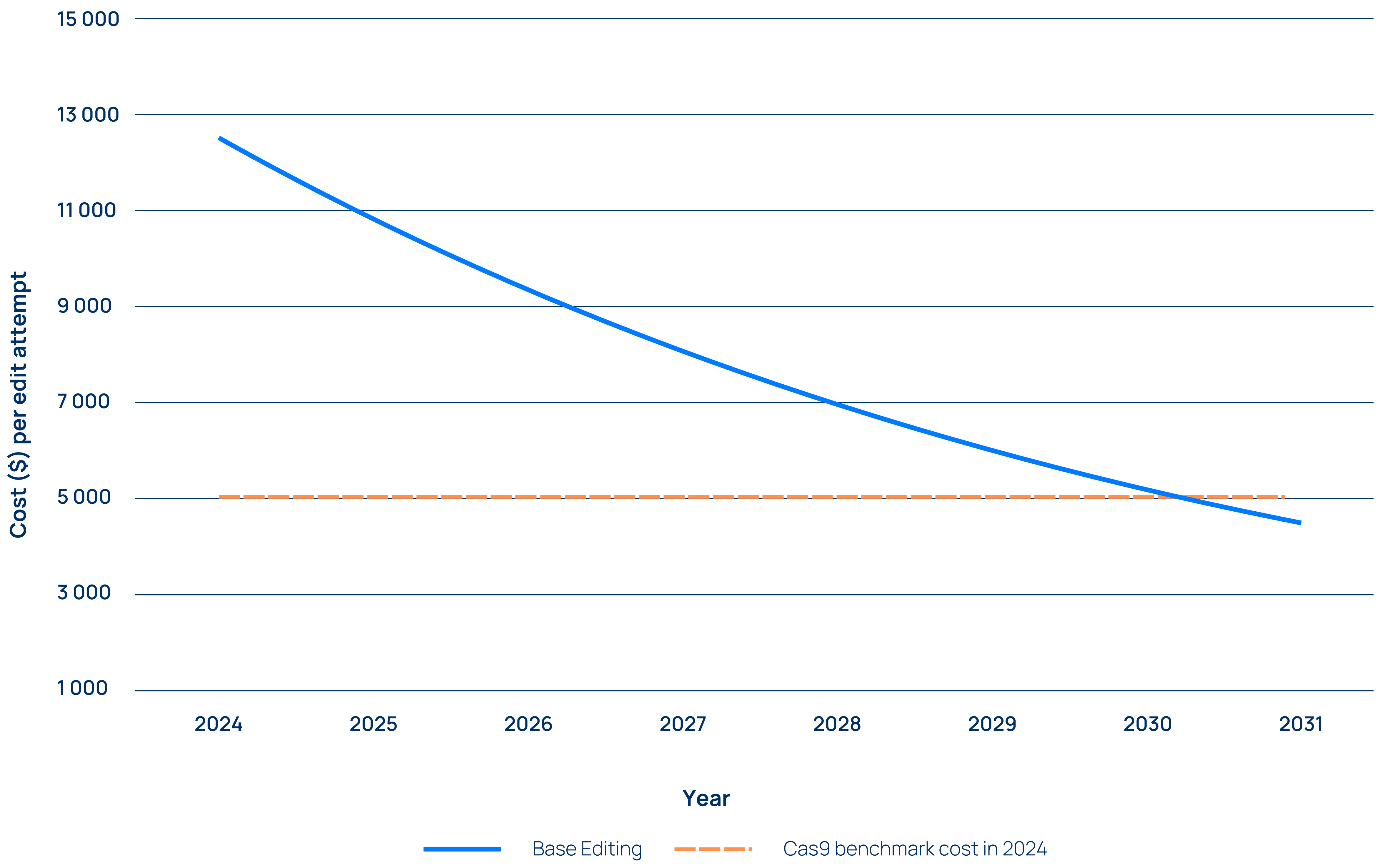
As of 2024, a CRISPR/Cas9 gene-editing attempt costs about $5,000*.

Base Editing, due to expensive materials, costs roughly **2.5 times more**, around $12,500 per attempt. However, with a **136% improvement rate**, Base Editing is expected to drop to $5,000 per attempt by 2031.

Experts believe ongoing innovations in base editor preparation and delivery will further reduce costs, following a similar path to CRISPR/Cas9's evolution, making Base Editing a competitive option for genome editing in the future.

*Source : Synthego, CRISPR Benchmark Report

Forecasting: When will Base Editing become the preferred Genome Editing Technology?



Source :GetFocus Odin platform

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The chart shows the quality of inventions compared to the average time it takes for each institution to develop a new invention. The chart focuses on institutions with the highest number of patent families related to Base Editing.

Knowledge flow indicates more valuable contributions to the technology.(Higher is better)

Cycle time indicates the average time it takes for the organization to develop a new invention. (Lower is better)

Interpretation of Chart:

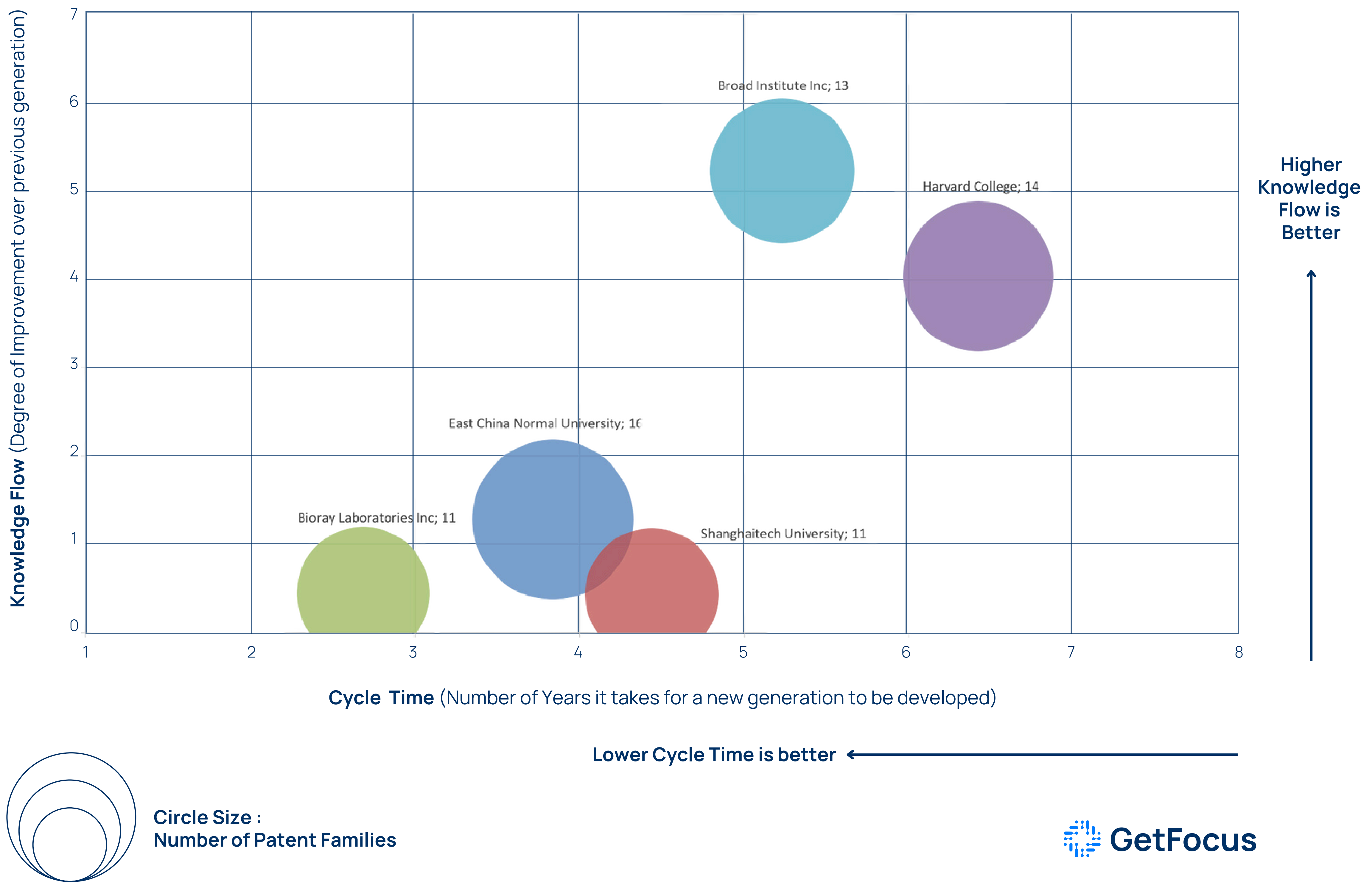
Broad Institute: With 13 patent families, it produces fewer inventions but of higher quality, indicating a greater impact on advancing Base Editing technology.

Harvard College: It holds 14 patent families, the second highest, but has slower knowledge flow and longer cycle time compared to Broad Institute.

East China Normal University: Despite having the most patent families (16) and a faster cycle time, its inventions have less impact on Base Editing technology compared to Broad Institute and Harvard College.

Which Institutions are Developing Base Editing Technology?

Genome Editing: Top 5 Institution Developing Base Editing Technologies



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Inventions of the Top 5 Institutions are focused on improving performance and efficiency, ultimately contributing to reducing costs.

Organisation	Key Focus Areas	Themes of inventions related to Base Editing
Harvard College	Inventions focused on foundational innovation in base editing technologies for treating a wide range of genetic disorders with improved precision and reduced off-target effects.	1. Improved base editors, such as adenine and cytidine base editors, designed with expanded PAM recognition capabilities and increased editing windows. 2. Optimized delivery methods for base editors, including viral vectors and non-viral delivery systems, to ensure efficient and safe delivery to target cells. 3. Complexes comprising multiple base editors or combining base editors with guide RNAs (gRNAs) to enhance editing efficiency and specificity.
Broad Institute Inc	Inventions focussed on improving base editing tools and efficiency for broader applications in genetic editing.	1. Formulation of pharmaceutical compositions and the development of kits for the delivery of base editors and guide RNAs for therapeutic use. 2. Techniques and systems for evaluating the specificity of base editors by determining off-target editing frequencies, aiming to enhance the safety of base editing technologies.
East China Normal University	Inventions centered on creating specialized and precise base editing tools, particularly for therapeutic applications.	1. Development of fusion proteins combining different functional domains to enhance gene editing efficiency and specificity. 2. Fusion of different base editors with other proteins or domains to expand the range of base modifications. 3. Optimizing the size and delivery methods of base editors to make them more efficient and safer for therapeutic use. 4. PAM-free adenine base editing products, which indicate efforts to overcome sequence limitations of traditional CRISPR systems.
Bioray Laboratories Inc	Bioray laboratories share a majority of patents with East China Normal university	1. Development and application of novel base editors, such as adenine deaminase and cytosine deaminase, as well as improved editing mechanisms for targeted gene therapy, disease model construction, and crop genetic breeding.
Shanghaitech University	Inventions focused on developing fusion proteins for precise base editing, with applications in non-disease diagnostics and therapies, and RNA editing.	1. Advancements in base editing tools and their applications. The patents primarily focus on the development of various base editors, which use fusion proteins combining different components such as Cas proteins (e.g., Cas9, Cas12a), deaminases (e.g., APOBEC, TadA), and other relevant fragments (e.g., UGI). These base editors are designed to perform precise and efficient base editing at specific genomic sites.

About GetFocus

We are on a mission to **fast-track technological progress worldwide**.
What started with foundations laid by MIT researchers, is now a full blown technology forecasting system. By equipping innovators with **data-driven technological foresight**, we help them make the right investment decisions and innovate faster.

Emerging technologies that turn into winners show clear and measurable signals early on in their development. By giving you access to this data, we help you innovate faster.

Our method has been verified to work on more than **50 technological areas.**

If GetFocus and our method had been around in the past, one could have known that:

- Lithium-ion batteries would eventually become cheaper than combustion engines for vehicles by **1995**,
- Digital photography would disrupt film by **1975.**
- SSDs would become cheaper than HDDs by the early '**80s**

If you'd like to see the full data set of this report or discuss a technology you'd like us to analyse, please contact us via :

contact@getfocus.eu

Invest in Winning Technologies

without the Guesswork

Eliminate blind spots

AI technology scouting

Instant overview of all emerging technologies in your area

See which tech works for you

AI technology evaluation

Compare and analyze technologies using AI, reducing months of work to minutes

Spot winners early

Forecasting

Predict which emerging technologies will dominate and when

Summarized in actionable insight

Reporting & Monitoring

Competitive intelligence, partnering options, AI patent analysis, landscaping & more

Trusted by Innovators Globally

Renault Group
CATERPILLAR
Department of Defense – United States of America
LVMH
Ministry of Economic Affairs, Agriculture and Innovation
Ministry of Defence
Rabobank
BASF We create chemistry
eDF
PHILIPS
Shell
TNO
GUCCI
ALFA LAVAL
ALCOMEX SPRINGS
MIG CAPITAL
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ROUSE
HESS
Qurin
suez
HIGH TECH XL
WAGENINGEN UNIVERSITY & RESEARCH
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What customers says about us

LVMH

Christophe Perthuisot
Head of R&D – Moët Hennessy

"In **one week with GetFocus**, we gained more technology insights than we previously could in 9 months"