FINEDA

CRISPR TECHNOLOGY

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CRISPR TECHNOLOGY

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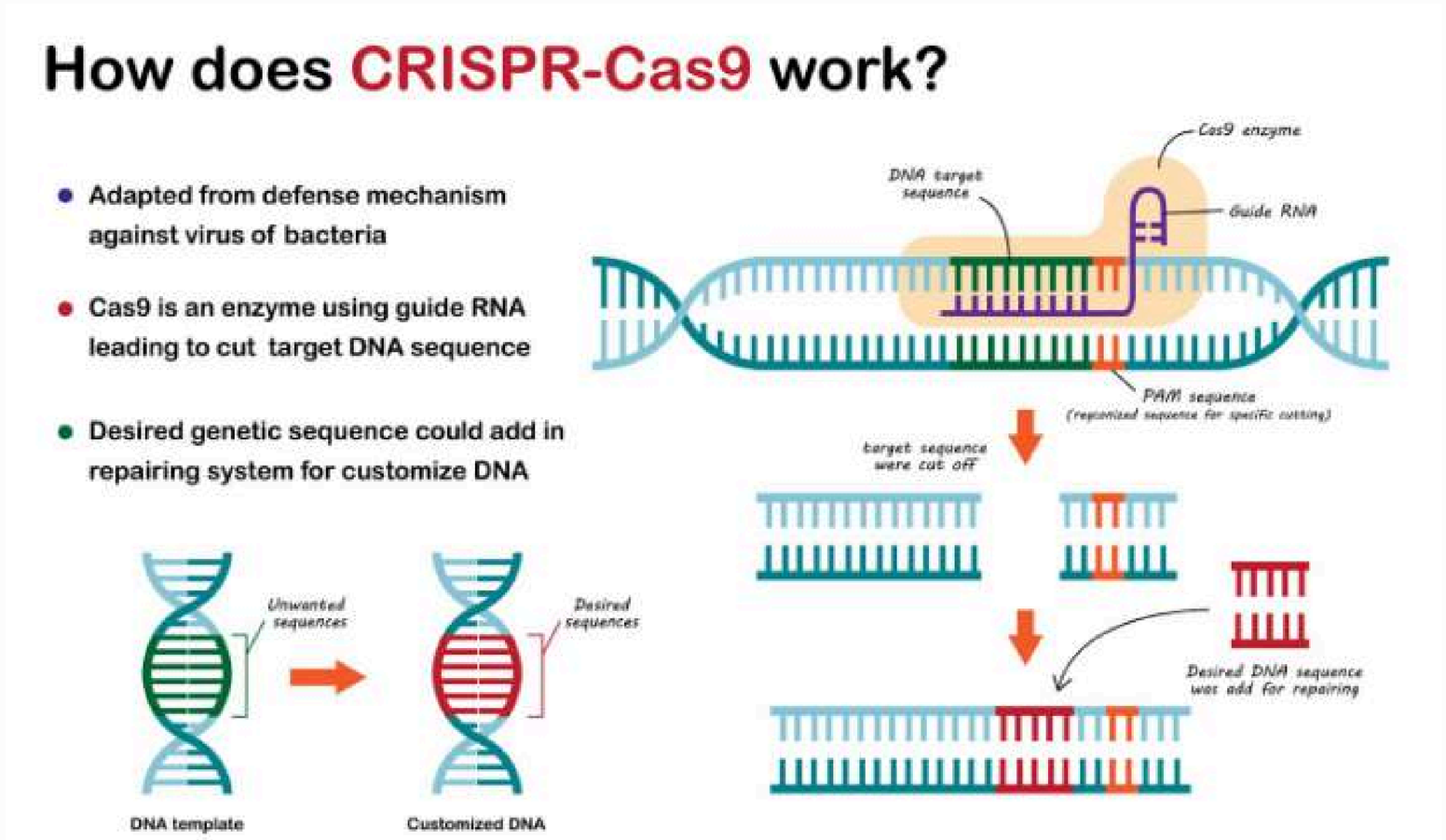

Fig 1: Working of CRISPR-Cas9

The story of CRISPR technology began with an accidental discovery in 1987 by Japanese scientist Yoshizumi Ishino at the University of Osaka in Japan. Ishino and his team, while sequencing a gene from an E.Coli microbe, identified an unusual repetition of DNA sequences. However, the significance of these clustered regularly interspaced short palindromic repeats (CRISPR) remained unclear for some time.

It wasn't until the early 2000s that the Cas (CRISPR-associated) genes were identified, and the adaptive immune nature of the CRISPR system in bacteria and archaea began to be elucidated. These systems were found to function by recognizing and destroying invading viral or plasmid DNA. This natural defense mechanism forms the basis of the revolutionary gene-editing tool.

A pivotal moment arrived in 2012 when French microbiologist Emmanuelle Charpentier, based in Europe, and American biochemist Jennifer Doudna published groundbreaking work in Science. Their research demonstrated how the CRISPR-Cas9 system could be reprogrammed to cut DNA at targeted locations in human and other eukaryotic cells. This discovery effectively transformed a bacterial immune system into a precise and efficient gene-editing tool. This foundational innovation allows geneticists and medical researchers to edit portions of the genome by removing, inserting, or changing DNA sequences.

At its core, CRISPR **(Clustered Regularly Interspaced Short Palindromic Repeats)** functions as a **natural defense mechanism found in bacteria**, which they use to protect against viruses. Scientists adapted this system for genome editing, using the Cas9 protein (CRISPR-associated protein 9) to introduce precise cuts at specific locations in the DNA.

The system involves two key components: a **guide RNA (gRNA)** and the **Cas9 enzyme**. The guide RNA is designed to match a specific DNA sequence within the genome. When introduced into a cell, the gRNA directs the Cas9 protein to the target location by base pairing with the complementary DNA sequence.

Once bound, Cas9 acts as molecular scissors, making a double-stranded break in the DNA at that exact point. After the DNA is cut, the cell's natural repair mechanisms are triggered. This system allows researchers to "edit" genes with high specificity, paving the way for applications ranging from disease correction to crop enhancement.

Following this breakthrough, the field of CRISPR technology experienced rapid development. Key milestones include the **development of CRISPR-derived diagnostic tools such as DETECTR and SHERLOCK**, which utilize the collateral cleavage activity of Cas12a and Cas13a for pathogen detection. These tools revolutionized infectious disease diagnosis, exemplified by the Sherlock CRISPR Sars-Cov-2 Kit. Furthermore, the technology evolved beyond basic genome editing to encompass more complex applications such as **multiplexed gene regulation, precision base editing, and targeted gene imaging**. The ease, speed, and low cost of accessing vectors through nonprofit plasmid repositories like Addgene since 2013 have also been a key driver in the technology's adoption. Addgene has shipped over 100,000 CRISPR plasmids to more than 75 countries worldwide, facilitating global research efforts.

The journey culminated in the **approval of the first-ever CRISPR-based therapies** for conditions like sickle cell disease and beta-thalassemia, marking a significant step in translating this technology into clinical applications. The foundational roots of CRISPR-Cas9 in basic biological research, including significant contributions from European scientists like Emmanuelle Charpentier, underscore the importance of fundamental science in driving technological progress and its applications in biomedicine, agriculture, and other fields.

2. Early Investors in Technology

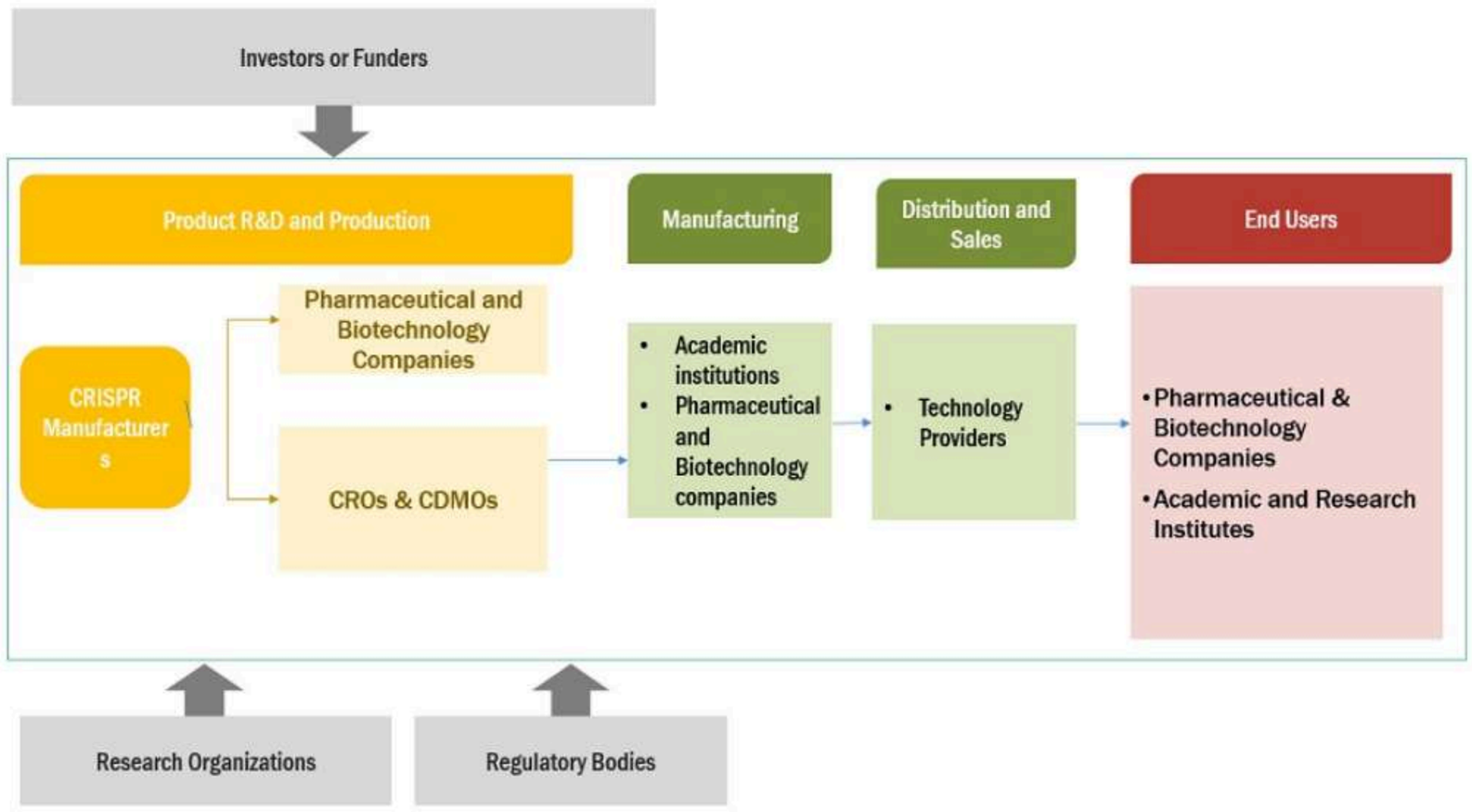

Fig 2: CRISPR Market Landscape

The development and expansion of CRISPR technology have been significantly supported by various funding sources, including government agencies, philanthropic organizations, and venture capital. Growing investment in genetic research, particularly in developed countries such as those in North America and Europe, has been a crucial early driver of the market.

In the **United States, government facilities have heavily invested in new materials, equipment, and procedures for genetic research**. The **National Institutes of Health (NIH)**, through initiatives like the **Somatic Cell Genome Editing (SCGE) Programme**, has provided substantial funding to researchers in the US and Canada for genome editing research, including CRISPR. For instance, in 2020, the SCGE Programme awarded 24 grants, with a total of USD 89 million in advance grants for genome editing to be distributed over four years.

European markets have also benefited from government funding for genomics research, including support from the National Human Genome Research Institute. The **European Research Council (ERC)** has played a significant role in funding CRISPR/Cas research, primarily in the Life Sciences domain. Between Horizon 2020 (2014–2020) and Horizon Europe (2021–2022), the ERC funded 1297 projects involving CRISPR/Cas technology, with a total investment of **2.66 billion EUR**.

Philanthropic organizations have also contributed to the funding landscape. Interestingly, **US philanthropic organizations, with the exception of HHMI, tend to specialize in funding CRISPR as a genome editing technology**, while US government agencies focus on both the biological study of CRISPR and its technological development as a biomedical tool. Models of co-funding networks at prominent institutions like the University of California system and the Broad/Harvard/MIT system illustrate the articulation between philanthropic organizations and government agencies in co-financing CRISPR discovery and development.

The establishment of **early companies focused on CRISPR technology also attracted significant investment**. **CRISPR Therapeutics**, co-founded in 2013 and based in Switzerland, went public and raised substantial funding through Series B preferred stock financing totaling approximately **$140.0 million** net proceeds by June 2016, and an additional **$54.1 million** net proceeds from an Initial Public Offering (IPO) in October 2016, along with **$35.0 million** from a concurrent private placement with Bayer. Similarly, other early-stage companies like Mammoth Biosciences raised about **USD 195 million** in investment in September 2021 based on their CRISPR technology. This influx of capital from various sources fueled the rapid advancement and commercialization of CRISPR technologies.

3. Catalysts of Growth

The CRISPR technology market has experienced exponential growth in recent years, driven by a multitude of factors spanning scientific advancements, increasing demand across various applications, and supportive financial and regulatory landscapes.

One of the primary catalysts is the **rising demand for gene therapeutics** to treat a wide range of genetic disorders. CRISPR's ability to precisely edit the genome offers unprecedented opportunities for developing curative therapies for diseases with a genetic basis. This is coupled with **technological advancements in the field of genome editing**, with continuous improvements in CRISPR systems leading to better efficiency, accuracy, and reduced off-target effects. The development of novel CRISPR variants like base editors and prime editors has further expanded the technology's therapeutic potential.

The **growing demand for the discovery of drugs** and the need for better biological models of human diseases have also fueled CRISPR adoption. Pharmaceutical companies increasingly use CRISPR technologies to rapidly identify new therapeutic targets and to develop more accurate disease models in less time. Furthermore, there is an **increasing demand for CRISPR in diagnostics**, leading to the development of rapid and sensitive tools for detecting pathogens and genetic markers.

The **rising demand for CRISPR technologies in agriculture applications** represents another significant growth driver. CRISPR enables precise genetic modifications in plants and animals, leading to improvements in crop yields, nutritional content, and disease resistance. The development of risk assessment guidelines for genetically modified organisms by authorities like the European Food Safety Authority (EFSA) can further support growth in this sector.

Supportive factors such as a **rise in funding and an increase in pharmaceutical R&D expenditure** have provided the necessary capital for research and development activities. Additionally, major trends like developing innovative next-generation technologies, improving CRISPR for better drug development results, using AI to enhance gene editing efficiency, and establishing strategic collaborations are expected to sustain market growth. The increasing prevalence of genetic disorders and a rising awareness regarding the potential of gene therapeutics also contribute significantly. The global reach and affordable distribution of CRISPR technologies facilitated by repositories like Addgene have further democratized access and accelerated adoption.

4. Key Countries in Europe for CRISPR Expansion

Europe has been a significant hub for CRISPR research and development, with several countries playing key roles in its expansion.

Switzerland stands out as the home of CRISPR Therapeutics, a prominent company in the field known for its pioneering efforts in gene editing for therapeutic purposes. Basel, Switzerland, serves as the location for CRISPR Therapeutics. Switzerland is also home to other genomics companies like BC Platforms.

Germany has the **largest market share in the European CRISPR technology market**. The Paul-Ehrlich Institute in Germany played a crucial role in vetting and approving CRISPR Therapeutics' preclinical program for beta-thalassemia. **Merck KGaA**, a major global player in life sciences, is based in Germany and is actively involved in the CRISPR market.

The **United Kingdom** represents the **fastest-growing market in the European CRISPR region**. The UK's health regulatory authority (MHRA) also approved CRISPR Therapeutics' preclinical program. **Horizon Discovery**, a key player in gene editing technologies, is based in the UK. The UK government has also shown a progressive stance on gene-editing technology regulation, particularly if it produces results achievable through traditional breeding.

France has also contributed to the CRISPR landscape, with institutions like **Institut Pasteur** involved in research. Denmark led Europe in the deposition of CRISPR plasmids to Addgene, indicating a strong research activity. Other key countries in Europe for CRISPR expansion and market growth include **Italy**, which is expected to register the highest CAGR in the European genome editing market, as well as the **Netherlands**, **Russia**, and **Spain**, all of which have been subjects of market analysis and are witnessing growth in the field.

The **European Research Council (ERC)** has supported a vast number of CRISPR/Cas-related projects across various European countries, highlighting the widespread engagement with this technology within the continent's research institutions and universities. The development of risk assessment guidelines by the European Food Safety Authority (EFSA) indicates a proactive approach towards the application of CRISPR in agriculture across Europe.

5. Market Size Growth

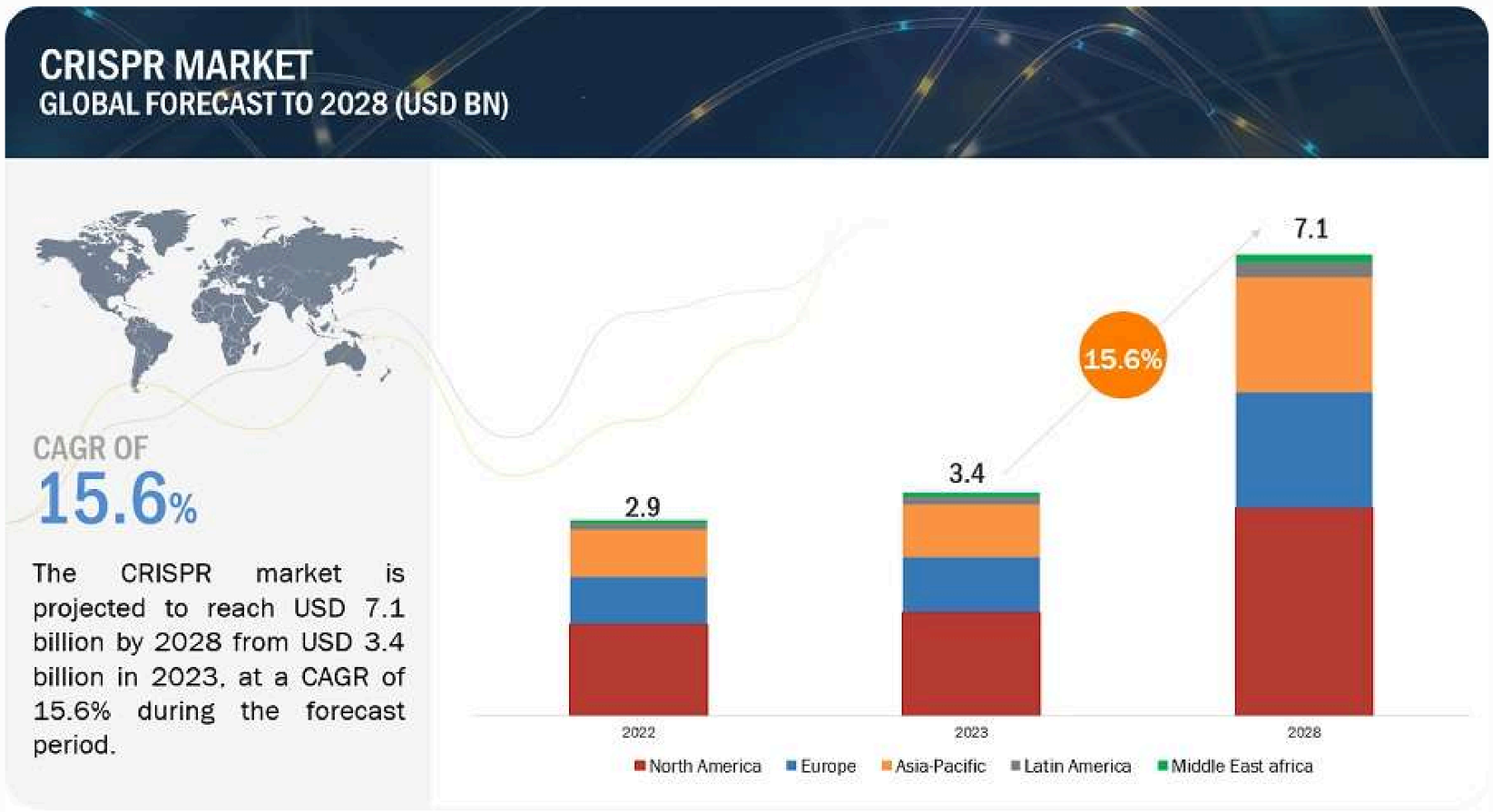

Fig 3: CRISPR Global Forecast to 2028

The CRISPR technology market has demonstrated significant growth historically and is projected for substantial expansion in the coming years.

Globally, the CRISPR technology market size **grew exponentially in recent years, increasing from $2.05 billion in 2024 to $2.53 billion in 2025,** representing a compound annual growth rate (CAGR) of 23.7%. This growth was attributed to a rise in funding and increased pharmaceutical R&D expenditure. The market is expected to continue its exponential growth, reaching $5.75 billion in 2029 with a projected CAGR of 22.7% from 2024 to 2033. Another report estimated the global market size at USD 3.5 billion in 2023, poised to grow to USD 13.84 billion by 2032 at a CAGR of 16.5% during the forecast period of 2025-2032. A further analysis estimated the global market size at $5.69 billion in 2024, projecting it to reach $26.22 billion by 2034 with a CAGR of 16.50% between 2025 and 2034.

The European CRISPR market is also showing strong growth. **It is projected to record a CAGR of 20.39% over the estimated years of 2021 to 2028**. The Europe CRISPR gene detection and diagnostic market was valued at USD 700 million in 2022 and is expected to grow to USD 2,631.20 million by 2030, with an anticipated CAGR of around 18% during the forecast period of 2023-2030. The broader Europe genome editing market generated a revenue of USD 2,260.8 million in 2023 and is expected to grow at a CAGR of 18.5% from 2024 to 2030, reaching USD 7,399.1 million by 2030. Another analysis valued the Europe genome editing market at USD 1.16 billion in 2024, expecting it to reach USD 3.81 billion by 2033 with a CAGR of 14.13% from 2025 to 2033.

These figures collectively indicate a robust and rapidly expanding market for CRISPR technology, both globally and within Europe, driven by its diverse applications and continuous advancements.

Recent Transactions

Vertex and Orna (Jan 8, 2025)

Orna Therapeutics is the creator of oRNA, leading circular RNA technology platform, and together with LNP delivery solutions, they have been able to significantly expand therapeutic possibilities, and pioneer into the next stage of in vivo therapies. Currently, they have been working in delivery systems targeting several genetic diseases located in the bone marrow, autoimmune B cell diseases, among others.

Vertex Pharmaceuticals and Orna Therapeutics have announced a three-year partnership to come together in developing next-gen gene therapies focused on sickle cell disease and transfusion-dependent beta-thalassemia.

By using Orna's lipid nanoparticle delivery technology, the partnership shall be able to improve the efficiency of gene editing and potentially address limitations in existing therapies.

Orna will receive $65m upfront and be eligible for up to $635m in milestone payments for SCD and TDT products. Moreover, they could receive up to $365m per product, alongside royalties on future sales.

Editas Medicine (Oct 24, 2024)

Editas Medicine is an American company, established internationally, with its headquarters situated in Cambridge, Massachusetts, USA. As pioneers in the gene editing industry, they have set developing transformative medicines through CRISPR/Cas9 and CRISPR/Cas12a genome editing tech as their main goals.
Vertex Pharmaceuticals is an R&D focused company, with its headquarters in Boston, Massachusetts, USA. Vertex is one of the co-developers of the very first approved gene therapy, Casgevy.

Editas trades up to 100% of future annual payments from Vertex (ranging between 5-40 million dollars per year) to a subsidiary of DRI Healthcare for $57m liquid. This follows a license agreement between both parties in December 2023. In this agreement, Vertex paid $50m upfront to Editas for non-exclusive rights to its ex vivo Cas9 gene editing tech. This technology targets the BCL11A gene in blood disorders of sickle cell disease and beta-thalassemia, used to further develop Casgevy tech.

Arbor Biotech (March 18, 2025)

Arbor biotech is a gene editing focused company, with its headquarters located in Massachusetts (USA), being in the forefront in the development of new therapies targeting specific organs (liver and CNS, for example). In doing so, they have also collaborated with other industry leaders, like Vertex, in the development of medicine to target type one diabetes, and sickle cell disease, among others.

Despite the current struggle in the industry in securing venture investment, Arbor was able to raise $74m in funding by Arch Venture Partners and TCGX, in order to take its lead project to clinical testing. Arbor is currently developing a gene editing therapy, ABO-101, that targets a rare kidney condition called primary hyperoxaluria type 1. Even though it only affects one or three per million of citizens in the USA and Europe, Devyn Smith (CEO of Arbor) assures that this new project will result in a one dosage needed treatment, when compared to existing long-lasting treatments.

Epicrispr Biotech (March 26, 2025)

Epicrispr Biotech is an up and coming start-up starting to establish themselves within the genetic editing industry. Located in California, USA, Epicrispr focuses on creating a vast therapeutic pipeline around epigenetic engineering. The epigenome is a naturally occurring system that controls gene expression, i.e. it determines which genes are expressed and at what level. Hence, being able to control the epigenome it becomes possible to control the activity of cells without making permanent edits to their DNA.

Epicrispr has recently raised $68m to test epigenetic editing on rare muscle disease. The start-up is trying to use CRISPR technology to create a first-of-its-kind genetic therapy for a neuromuscular disorder called facioscapulohumeral muscular dystrophy.

Other comments:

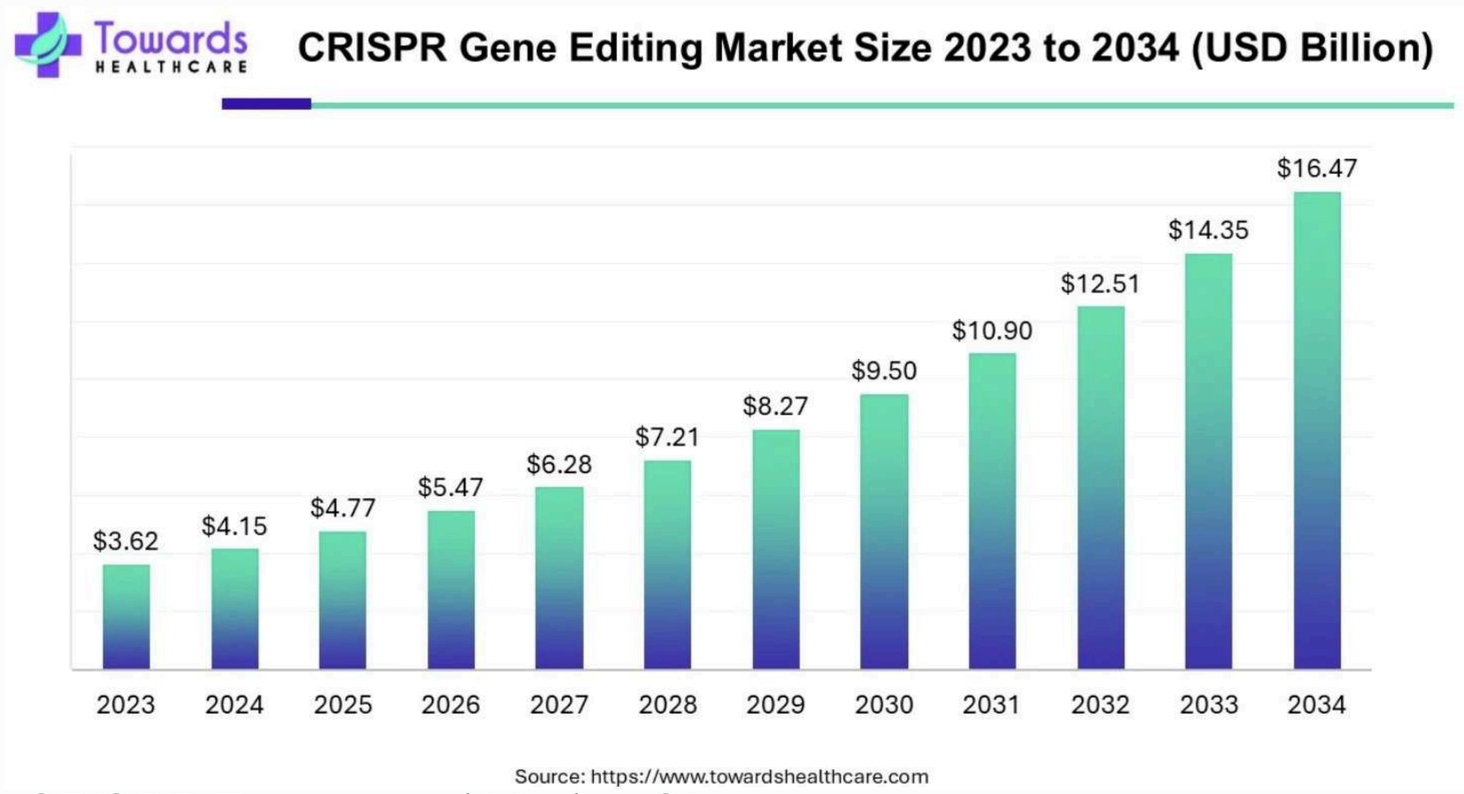

Fig 4: CRISPR Forecasted Market Size

Biotech venture capital experienced a resurgence in 2024, with U.S. and European funding increasing From $21.2b to $28.1b. Regardless of uncertainties relating to inflation and interest rates persisting, investors are focusing on larger investments in fewer companies, with mega-rounds being notably prominent.

Despite having decreased since its peak in the pandemic, private equity firms are still a main investor for this sector, investing €300m into European biotech.
The European biotech market has witnessed M&A activity aimed at expanding market presence and leveraging synergies. In 2023, Lonza acquired Synaffix B.V., summing up their capabilities in antibody-drug conjugate development. Also, Merck KGaA partnered with BenevolentAI and Exscientia to harness AI for drug discovery in oncology, neurology, and immunology.

European biotech companies are increasingly engaging with public markets to fuel growth. Despite not being present in all European stock exchanges and their presence being much lower there (60%) than in the US (85%), they are paving the way for a much larger-scale public funding. The UK is setting the grounds for this expansion in Europe, with £3.7 billion raised in 2024 and attracting 40% of total VC investment across the continent.

Future trends

Potential Applications

The European biotech industry is currently undergoing a transformative moment, as revolutionary inventions are entering the horizon, and therefore generating great investment opportunities. This section delves into what the future poses for this sector, with the main focus on CRISPR technology, AI integration into biopharmaceuticals and how all that shapes the investment landscape.

The CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) has truly shaked the ground in genetics work, allowing sharpness and precision in gene modifications and diagnosis. This technology is projected to grow at a compound annual growth rate (CAGR) of 18.0%, reaching USD 2.63 billion by 2030. Its main future applications,which are being worked on, are:

- **Medical applications:** Gene therapies are being developed to target gene disorders, infectious diseases and cancer. A highlighted example is Casgevy, the world's first medicine using Crispr technology to modify genes. It received approval in UK, US and Europe for the treatment of sickle cell disease in people 12 years and older with recurrent vaso-occlusive crises, offering a potential cure for patients with limited options.
- **Agriculture:** The gene modification can be applied as well to crops, to improve their growth, resistance and adaptability. One of the central advances has been in tomatoes, which now can be adjusted to increase their flavor without modifying their size.
- **Industry and farming:** This tech can be used for creating microorganisms for biofuel production and biodegradable materials, which could cover the need for greener energy use and ease the overcharge in fossil fuels. Moreover, farm animals can be more resistant to diseases. For example, the Roslin Institute, the ‘parents’ of sheep Dolly, has used CRISPR to develop pigs resistant to porcine reproductive and respiratory syndrome and chickens with some resistance to avian influenza.

The regulatory framework for this discovery is still evolving, especially in Europe, where ethical concerns on genetically modified organisms (GMOs) are far more rigid. Differentiating those from precise gene-edited organisms (like the ones edited by CRISPR) is an ongoing debate that could find a loophole into the regulation, leading to commercialization in the future. Of course, establishing legal and ethical frameworks remains a challenge yet to be confronted, but necessary to put to use a technology that could lead to a true medical revolution.

Integration of AI in Biotech

AI is increasingly making its way into biotech, especially in drug discovery.

Algorithms can analyse large data sets to identify potential best drugs, predict their efficiency and optimize clinical trials. This way, their cost and the time a new drug takes to go into commercialization safely can be reduced, saving countless lives. Antiverse, is a Biotech company based in Cardiff, pioneer in this field. They combine machine learning techniques and advanced cell line engineering to become an antibody discovery company, accelerating drug development.

Integrating AI can also allow the personalisation of pharmaceutics to tailor to any patients' needs. By analyzing individual genetic profiles and their subsequent disease probability, the best treatment responses can be created taking into account possible adverse effects.

Upgrade in Biopharmaceuticals

Once a futuristic challenge, innovations in cell and gene therapies are now becoming increasingly more sophisticated. CAR T-cell therapy, for example, has shown incredible success in treating certain blood cancers. Companies like Cellares are developing automated systems to manufacture these therapies more efficiently, aiming to reduce costs and increase accessibility.

RNA-based therapies are gaining momentum, for example vaccines and treatments that can treat infections or genetic disorders straight from the cell liquid.

Market and Investment Trends

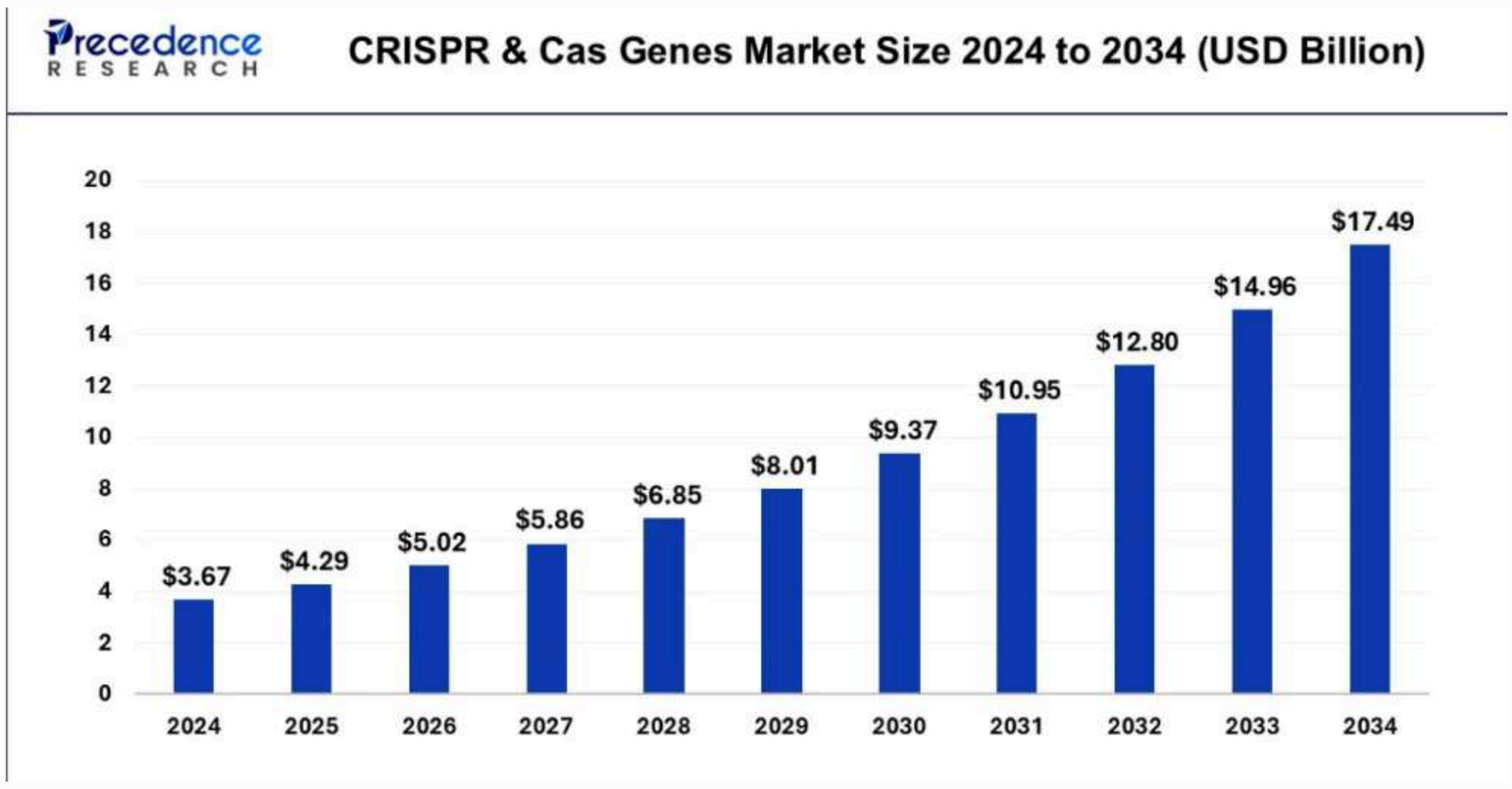

Fig 5: CRISPR and CAS Genes Market Size 2024 to 2034

Such cutting-edge technologies, even Nobel-prize worthy, are of course going to alter market and investment trends, things they are already doing.

The CRISPR technology market is booming. It is projected to grow from $5.72 billion in 2024 to $26.22 billion by 2034, with a compound annual growth rate (CAGR) of 16.54%. This growth reflects the increasing interest and investment in CRISPR applications across various sectors. Europe is witnessing a surge in biotech and CRISPR-focused startups. These emerging companies are attracting significant investments and forming collaborations with academic institutions and pharmaceutical companies to accelerate research and development of new applications. The financing is being received through diverse sources:

- **Via VC influx:** Biotech venture capital experienced a resurgence in 2024, with U.S. and European funding increasing to $28.1 billion from $21.2 billion in the previous year.
- **Via PE activity:** Despite having decreased since its peak in the pandemic, private equity firms are still a main investor for this sector, investing €300 million into European biotech.
- **Via M&A:** The European biotech market has witnessed M&A activity aimed at expanding market presence and leveraging synergies. In 2023, Lonza acquired Synaffix B.V., summing up their capabilities in antibody-drug conjugate development. Also, Merck KGaA partnered with BenevolentAI and Exscientia to harness AI for drug discovery in oncology, neurology, and immunology.
- **Via Public market:** European biotech companies are increasingly engaging with public markets to fuel growth. Despite not being present in all European stock exchanges and their presence being much lower there (60%) than in the US (85%), they are paving the way for a much larger-scale public funding. The UK is setting the grounds for this expansion in Europe, with £3.7 billion raised in 2024 and attracting 40% of total VC investment across the continent.

Despite all this investor interest, their efforts could be hindered by policy directions and regulatory frameworks, which significantly influence investment decisions. For instance, changes in regulatory data protection (RDP) could impact the number of biotech startups and the economic viability of their products.

Challenges and Opportunities

- **Accessibility and cost:** At this moment, technologies like Casgevy have already started being tested in real patients outside clinical trials. However, their price remains sky-high, more than $2 million. This remains a challenge, as a real change can’t be seen in the world's health if these advances are available to only a few privileged.

- **Regulatory Harmonization:** As mentioned before, agreeing into a regulatory framework for these technologies is one of the remaining trials the sector is facing. It is clear that, as with all great innovations, there will be advocaters and critics. A common ground should be found, prioritising both safety and embracing modernization. Harmonizing regulatory standards across European countries could lead to further approvals of processes and encourage investment.
- **Ethical Considerations:** The agile advancement of technologies like CRISPR and AI raises ethical questions, particularly concerning gene editing in humans, data privacy and explores the limits of life creation, a dilemma previously raised in science history. Establishing robust ethical guidelines is essential to guide responsible research and application.
- **Talent Acquisition and Retention:** As the biotech industry grows, there is an increasing demand for skilled professionals. Investing in education and training programs will be crucial to develop a workforce capable of driving innovation and retaining it long term.

In conclusion, the European biotech industry stands at a pivotal juncture, fueled by revolutionary technologies like **CRISPR** and **AI integration**, which are attracting substantial investment and driving innovation across medicine, agriculture, and industry. While the **CRISPR market is projected for significant growth** and advancements in gene and cell therapies are becoming increasingly sophisticated, the sector faces crucial challenges including **navigating evolving regulatory frameworks**, addressing **ethical considerations**, ensuring **accessibility and affordability** of new therapies, and securing the necessary talent. Overcoming these hurdles while capitalizing on the immense potential of these technologies will be critical in shaping the future of European biotech and realizing its promise of a medical revolution.

Our View

Having analyzed the European CRISPR technology landscape, we see compelling investment opportunities driven by rapid scientific advancement, commercial expansion, and evolving regulatory frameworks. The sector is poised for significant growth, with the European market projected to reach $2.63 billion by 2030 at an impressive 18% CAGR.

Key Investment Considerations:

1) Market Momentum

The European CRISPR market demonstrates strong fundamentals with projected growth rates exceeding 20% in some segments through 2028. Particularly noteworthy is the gene detection and diagnostic market's anticipated expansion from $700 million in 2022 to $2.6 billion by 2030. This growth trajectory presents substantial opportunities for early investors who can identify companies with scientific excellence and commercial potential.

2) Regulatory Evolution

We view the evolving European regulatory landscape as a potential catalyst rather than an obstacle. The recent progress toward deregulating genetically modified crops created using new genomic techniques signals a more permissive environment for CRISPR applications in agriculture. Similarly, the European Commission's landmark approval of CASGEVY™ as the first CRISPR/Cas9 gene-edited therapy establishes a regulatory pathway for therapeutic applications. These developments suggest a pragmatic regulatory approach that balances innovation with safety considerations.

3) Commercial Validation

The approval and commercialization of CASGEVY™ for sickle cell disease and beta-thalassemia represents a crucial inflection point for the industry. This milestone validates the technology's therapeutic potential and demonstrates viable commercialization pathways despite high upfront costs. The establishment of specialized treatment centers across Europe further indicates institutional commitment to CRISPR-based therapeutics.

4) IP Landscape

We note the ongoing patent disputes in the CRISPR space, including the recent revocation of two foundational European patents by the Charpentier-Doudna group. While creating some uncertainty, this dynamic IP environment also presents opportunities for strategic licensing agreements and partnerships, as evidenced by ERS Genomics' expanding portfolio of over 150 licensees. Investors should prioritize companies with robust IP strategies and diversified licensing arrangements.

Strategic Opportunity Areas:

1) Therapeutics

The therapeutic applications of CRISPR technology represent the most immediate and substantial market opportunity. Early success with genetic disorders like sickle cell disease and beta-thalassemia has validated the approach, with treatments commanding premium prices ($1.9 million per course for CASGEVY™). We anticipate expanding applications in oncology and rare diseases, with particular investment potential in companies developing next-generation delivery systems and enhanced editing precision.

2) Diagnostics

The European CRISPR gene detection and diagnostic market presents a compelling growth opportunity, with an expected 18% CAGR through 2030. Companies developing rapid, sensitive diagnostic tools for pathogen detection and genetic markers are particularly well-positioned. The licensing agreement between ERS Genomics and Jumpcode Genomics highlights the commercial interest in applying CRISPR to enhance next-generation sequencing technologies.

3) Agriculture

With European regulatory authorities moving toward deregulation of gene-edited crops, agricultural applications represent an emerging opportunity. Companies developing CRISPR-modified crops with improved yield, nutritional content, and disease resistance may find a more receptive market environment than previously anticipated. Early movers in this space could capture significant market share as regulatory.

Investment Strategy

1) Geographic Focus

While the CRISPR ecosystem spans Europe, we identify Switzerland, Germany, the UK, and France as priority markets. Switzerland hosts CRISPR Therapeutics and benefits from a favorable regulatory environment. Germany holds the largest European market share and hosts major players like Merck KGaA. The UK represents the fastest-growing market with progressive regulatory stance, while France contributes significant research expertise.

2) Funding Approach

We recommend a balanced approach to investment, combining strategic early-stage funding for innovative startups with selective follow-on investments in companies demonstrating clinical progress or significant technological advantages. The recent $68 million funding round for Epicrispr Biotech exemplifies the considerable capital available for companies with differentiated approaches, in this case using epigenetic editing to target rare muscle diseases.

3) Partnership Model

Strategic partnerships and licensing agreements are increasingly critical in this space, as evidenced by Vertex Pharmaceuticals' collaboration with Orna Therapeutics to develop next-generation gene therapies. We favor companies demonstrating ability to forge meaningful strategic alliances that accelerate development timelines and expand commercial reach.

Challenges and Risks

Despite the sector's promise, several challenges warrant investor attention:
Access and Affordability: The high cost of CRISPR therapies (currently $2+ million per treatment) may limit market penetration and invite pricing pressure from European health systems. **Regulatory Uncertainty**: While trending positive, regulatory frameworks remain in flux, particularly for agricultural applications and novel therapeutic modalities. **Patent Complexity**: The fragmented intellectual property landscape creates licensing complexity and potential litigation exposure. **Long Development Timelines**: Therapeutic applications face extensive clinical development and regulatory review cycles, requiring patient capital.

Conclusion

The European CRISPR technology sector presents a compelling investment thesis based on accelerating scientific progress, expanding commercial applications, and an increasingly supportive regulatory environment. While acknowledging the sector's inherent risks, we believe selective investments in companies with differentiated technology, strong intellectual property positions, and strategic partnerships can deliver exceptional returns. The approval of first-generation therapies has significantly de-risked the space, creating a favorable environment for capital deployment across the CRISPR ecosystem.

Newsrun

European Commission Approves First CRISPR/Cas9 Gene-Edited Therapy, CASGEVY™ (exagamglogene autotemcel), for the Treatment of Sickle Cell Disease (SCD) and Transfusion-Dependent Beta Thalassemia (TDT)

(CRISPR Therapeutics AG, 13 February 2024)

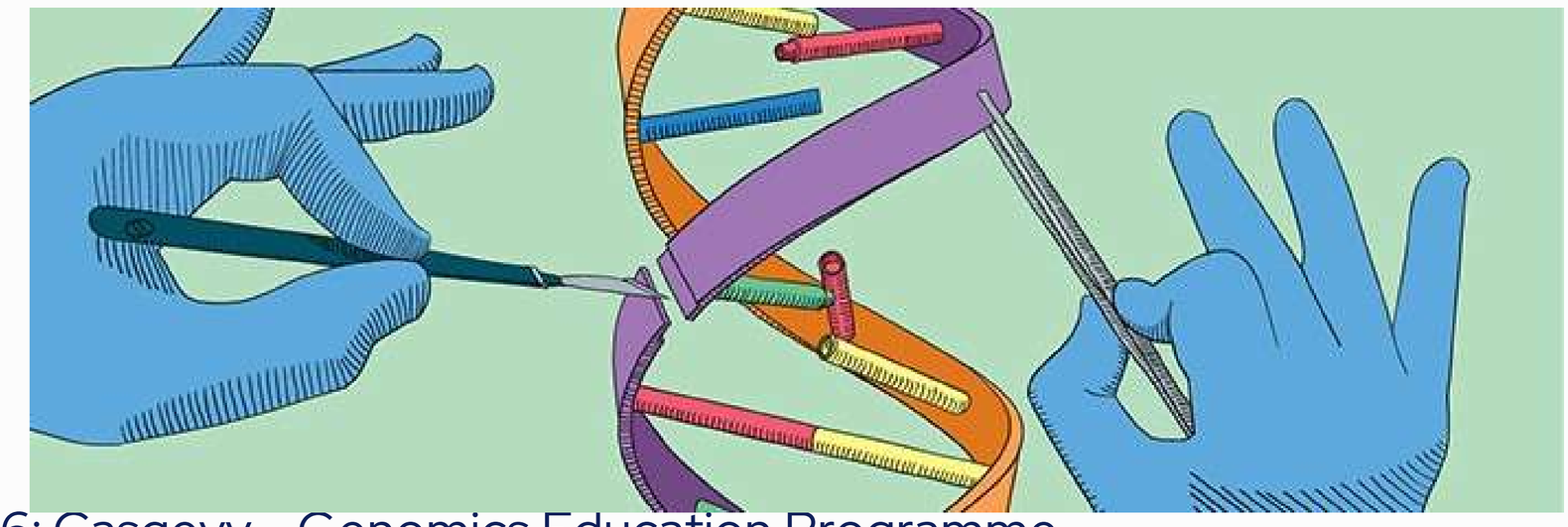

Fig 6: Casgevy - Genomics Education Programme

The European Commission **granted conditional marketing authorization** to CASGEVY™ (exagamglogene autotemcel), marking it as the **first CRISPR/Cas9 gene-edited therapy approved in Europe**. Developed through a strategic collaboration between CRISPR Therapeutics (NASDAQ: CRSP) and Vertex Pharmaceuticals, CASGEVY targets the treatment of **severe sickle cell disease (SCD)** and **transfusion-dependent beta thalassemia (TDT)** in patients 12 years of age and older. This approval opens the door to treatment for over 8,000 potentially eligible patients across the European Union.

Under the terms of their amended collaboration agreement, Vertex leads global development, manufacturing, and commercialization of CASGEVY with a **60/40 profit-sharing arrangement** with CRISPR Therapeutics. To ensure patient access, Vertex is actively engaging with national health authorities and establishing a network of **authorized treatment centers (ATCs)** experienced in stem cell transplantation. Currently, three ATCs are activated in the EU, with plans to expand to approximately 25 centers across Europe. Notably, Vertex has secured **early access for eligible TDT patients in France** ahead of the national reimbursement process.

CASGEVY's mechanism involves **editing the BCL11A gene** in a patient's hematopoietic stem and progenitor cells, leading to the production of high levels of fetal hemoglobin (HbF). This has been shown to reduce or eliminate **vaso-occlusive crises (VOCs)** in SCD and transfusion requirements in TDT. This regulatory milestone underscores the **transformative potential of CRISPR/Cas9 gene editing technologies** and further validates CRISPR Therapeutics' broader portfolio across disease areas such as oncology, regenerative medicine, cardiovascular, and rare diseases. The conditional marketing authorization reflects the **significant unmet medical need** for these serious genetic diseases, where curative options like matched donor stem cell transplant are available to only a small fraction of patients.

ERS Genomics and Jumpcode Genomics sign CRISPR/Cas9 license agreement

(ERS Genomics, 27 March 2025)

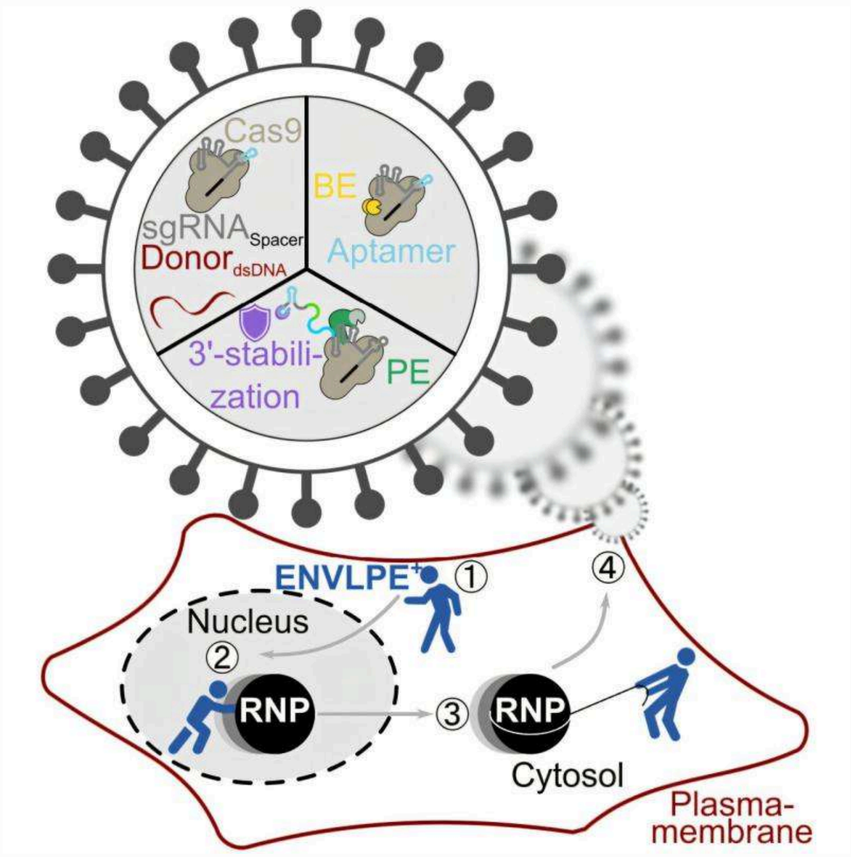

Fig 7: ERS Genomics

ERS Genomics and Jumpcode Genomics have forged a non-exclusive license agreement granting Jumpcode access to ERS' foundational and Nobel Prize-winning CRISPR/Cas9 patent portfolio.
This strategic move empowers Jumpcode Genomics to **significantly enhance the sensitivity and efficiency of next-generation sequencing (NGS)**. Jumpcode intends to leverage this access to further develop its **DepleteX™ and CRISPRcleanR technologies**, which are designed to remove unwanted sequences, thereby improving the quality and reducing the cost of NGS.

This collaboration strategically combines **ERS Genomics' extensive intellectual property** in CRISPR/Cas9 technology with Jumpcode's **innovative approach to sequencing**. The expected outcomes include the **acceleration of breakthroughs** across diverse fields such as basic research, infectious disease detection, molecular diagnostics, and oncology. As a key licensor of CRISPR/Cas9 technology, ERS Genomics adds Jumpcode to its **growing portfolio of over 150 licensees worldwide**, highlighting the broad commercial applicability and foundational nature of its IP. This agreement underscores the **continued investment and innovation in CRISPR-based tools** for advancing genomic analysis. Financial details of the agreement remain confidential.

Procedural insights from the latest twist in the CRISPR patent saga

(The Global Legal Post, October, 28 2024)

Fig 8: Nobel Prize to chemistry - CRISPR Gene Editing

The CRISPR patent dispute has taken a surprising turn as the **Charpentier-Doudna group (CVC)** revoked two foundational European patents related to their Nobel Prize-winning gene editing technology ahead of crucial appeal hearings. CVC attributed this decision to **"serious procedural concerns"** regarding the **European Patent Office's Board of Appeal** approach, fearing a violation of their right to be heard. Specifically, they cited a recent case law that might prevent them from amending claims effectively.

Opponents, however, view this as a tactical maneuver to avoid likely invalidation of the patents and to protect CVC's **broader patent portfolio**, which includes pending and issued divisional patents. This revocation introduces uncertainty for companies holding licenses to CRISPR technology and for future licensing agreements. Investors in the CRISPR space should closely monitor the **downstream effects on ownership rights** and the numerous licenses already granted worldwide, as well as the ongoing **commercialization efforts** in this rapidly evolving market. The dispute also highlights the importance of patent strategy, particularly the timing of filing claim amendments at the European Patent Office.

European governments heading towards GMO deregulation

(Euronews, 24 February 2025)

The **EU is progressing towards deregulating genetically modified crops** created using **new genomic techniques (NGT)**, with a majority of member states supporting a **compromise proposal to treat them as largely equivalent to conventional strains**.

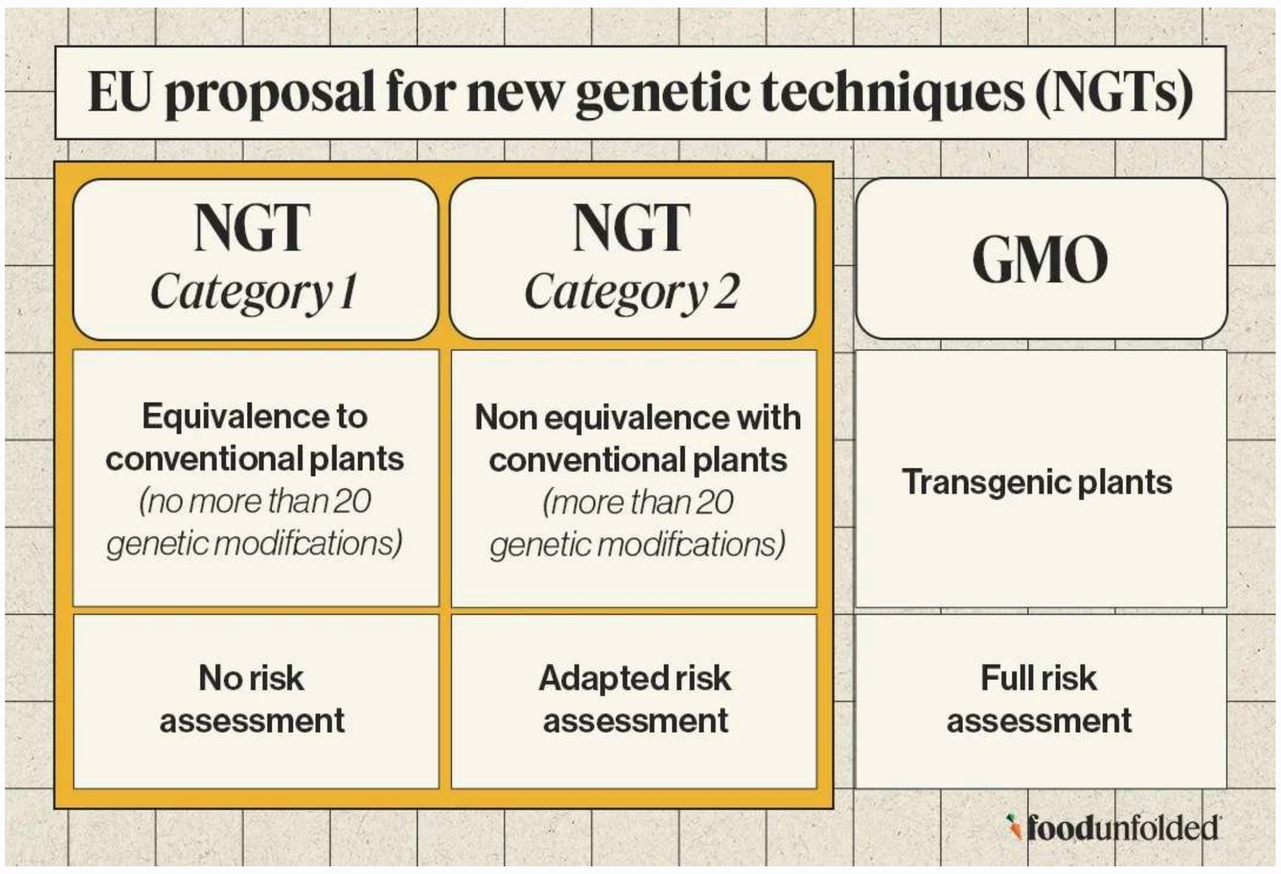

Fig 9: EU Proposal for New Genetic Techniques (NGTs)

This would establish a **new category of gene-edited crops subject to light-touch regulation**, a significant departure from the **strict safety testing and traceability requirements** for traditional GMOs. The **European Parliament has also agreed to back** the core deregulatory elements. This alignment between legislative bodies suggests that **final legislation could be approved within months**, paving the way for a **substantial market opportunity in agricultural biotechnology**.

Companies utilizing technologies like CRISPR/Cas9 will likely benefit from **reduced regulatory hurdles**. While environmental groups voice concerns over corporate interests and safety, and some member states remain opposed, the momentum appears to be in favor of **deregulation**, creating a potentially **lucrative landscape for investment and innovation** in the gene-editing sector for agriculture. The ongoing debate includes the **patentability of NGT crops**, which could further shape market dynamics.

‘Much-needed hope’ as CRISPR gene editing therapy recommended for sickle cell disease

(Euronews, 31 January 2025)

England's National Institute for Health and Care Excellence (NICE) has approved exagamglogene autotemcel (exa-cel/Casgevy) for use by the **National Health Service (NHS)** in England to treat some individuals with **severe sickle cell disease**. This decision marks a **reversal from NICE's draft rejection in March**, aligning with their earlier approval for **beta thalassemia**. The gene-editing therapy, **priced at £1.6 million (€1.9 million) per course**, modifies a patient’s blood stem cells using **CRISPR technology**, a breakthrough that earned its discoverers the **Nobel Prize in Chemistry.**

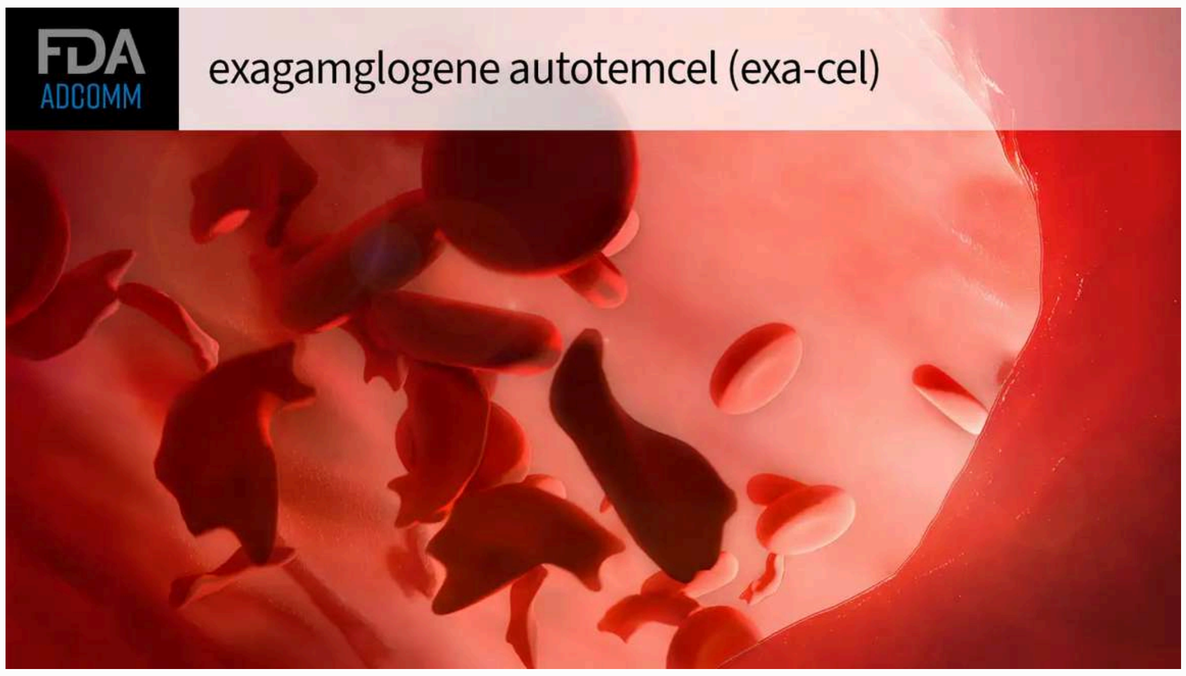

Fig 10: exagamglogene autotemcel - MedPage Today

This approval is viewed as a **"significant shift" in treating the inherited blood disorder**, which disproportionately affects individuals of **African, Caribbean, Middle Eastern, or South Asian** family backgrounds. While experts hail this as a **"potentially curative treatment"** and a moment of **"much-needed hope"**, it's important to note that exa-cel is **not a cure for all SCD patients**, and concerns regarding its **long-term effectiveness, safety, and accessibility** persist. The NHS will **collect data on patient outcomes**, leading to a future **re-evaluation by NICE**. This development signifies a **significant investment in innovative gene therapy**, despite the high upfront cost, aiming to **address a critical unmet medical need**.

APPENDIX:

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a) Brief summary of industry in Europe

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