



CRISPR/CAS System for Treatment of Blood Disorder

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Introduction

This report provides an analysis of the global patent landscape for CRISPR/Cas system-based treatment of blood disorders. More specifically, the report focuses on patents related to the use of CRISPR technology to develop therapies for various blood related diseases addressing factors such as Cas variants and associated delivery methods. The study identifies various companies along with their patent filing trends and provides useful insights from the observed trends.



The advent of CRISPR/Cas technology has provided a revolutionary gene-editing approach with the remarkable potential of treating diverse blood diseases and disorders. By enabling the precise correction of underlying genetic flaws, CRISPR-based therapies present a pathway towards potentially permanent cures. The CRISPR/Cas system acts as a "molecular scissors" that can be programmed to modify specific DNA sequences using various approaches such as

- **Gene Correction:** The approach involves directly targeting and correcting the disease-causing mutation at its location within the gene.
- **Gene Knockout:** Certain diseases stem from either an overactive gene or a regulatory element that suppresses a beneficial gene. CRISPR offers a solution by enabling the "knockout" or disabling these detrimental genes or regulatory sequences.
- **Gene Insertion (Knock-in):** This approach focuses on replacing a faulty or missing gene by inserting a healthy copy into the patient's cells. This is especially relevant for disorders where a functional gene is missing or severely impaired.
- **Epigenetic Modification:** In addition to DNA editing, CRISPR can be adapted to control gene expression without changing the underlying DNA sequence. This is done by targeting epigenetic regulators to specific sites, allowing for the activation or repression of gene transcription.

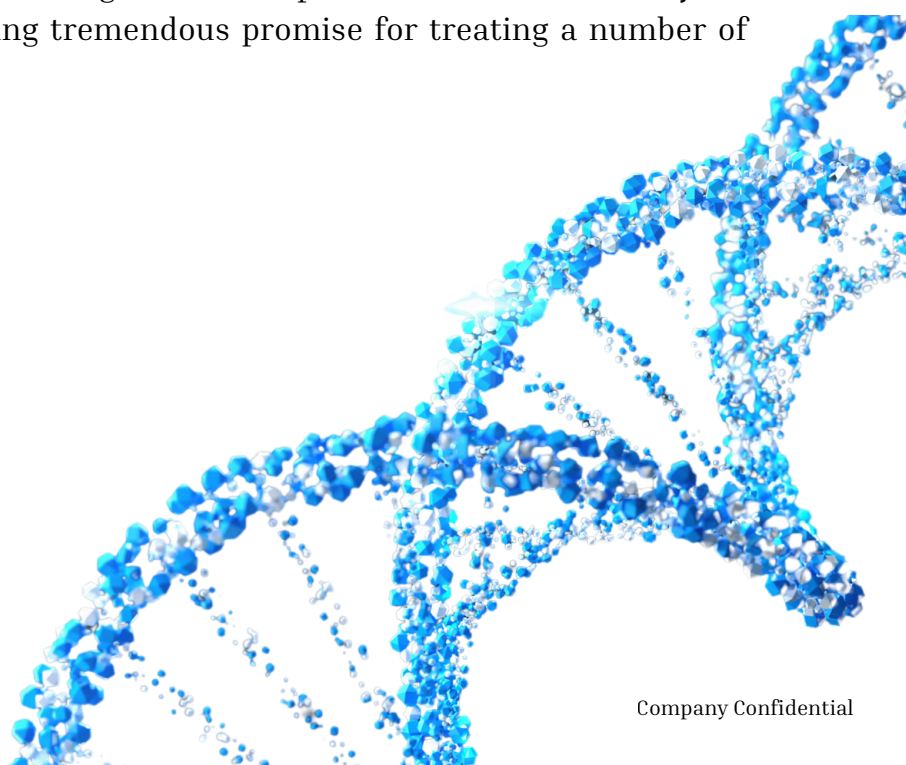
Therapeutic Applications

CRISPR/Cas technology is derived from a natural defense mechanism found in bacteria and archaea. These microorganisms use CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) arrays and associated Cas (CRISPR-associated) proteins to protect themselves from invading viruses.

CRISPR technology is being actively investigated for the treatment of a range of blood disorders, including Sickle Cell Disease (SCD), Beta-Thalassemia, Fanconi Anemia, Hemophilia and Hematological Malignancies like leukemia and lymphoma. CRISPR-based therapies, such as Casgevy (exagamglogene autotemcel), have shown remarkable success in clinical trials by either correcting the HBB gene mutation or by knocking out the BCL11A gene to increase fetal hemoglobin production. Casgevy has received regulatory approvals in several regions, including the UK, the US, and Europe, marking a significant milestone as the first CRISPR-based medicine for the treatment of sickle cell disease and beta-thalassemia. It further validates the potential of this technology to translate into effective treatments. Numerous other clinical trials are underway, investigating different CRISPR-based strategies for various blood disorders.

The success of CRISPR-based therapies pivots on the efficient and safe delivery of its components into the target cells. Current commonly used approaches include Electroporation, Microinjection, Liposomes, Lipid nanoparticles, Inorganic nanoparticles and various viral vectors.

CRISPR/Cas technology represents a significant leap forward in our ability to manipulate the genetic code, holding tremendous promise for treating a number of diseases.



Executive Summary



Portfolio Size

- Total number of Global Active IP assets (Grants, Applications) covering CRISPR/Cas system for treatment of blood disorders are ~1682 INPADOc families.
- 193 Granted Patents have been identified in the technology domain. Along with this, there are 1489 Applications.



Patenting Trend and Geographical Distribution

- There has been growth in patent filings in the domain since its inception, though a significant rise was observed from 2013 with a slight dip in the year 2021. Maximum number of patents were filed in 2022.
- 36.8% of the patent filings were done via PCT route (WIPO). CN, EP and US dominate the patent landscape with 37.1% IP assets (624 patents). Whitespaces in Germany, Denmark, France, Italy, India, New Zealand, United Kingdom and Saudi Arabia.



Top Assignees

- Broad Institute Inc (69), Massachusetts Institute of Technology (55) and Harvard Univ (55) are the top assignees. A significant number of patents are filed in collaboration with other universities.
- Among the companies, Editas Medicine Inc, Crispr Therapeutics AG and Novartis AG lead with 35, 34 and 27 IP assets, respectively.



Market Trends and Litigation

- CASGEVY™ is the first and the only approved medicine utilizing CRISPR/Cas9 gene editing technology for treatment of blood disorders. Several clinical trials are underway in various phases.
- The field of CRISPR gene editing technology for treatment of blood disorders has been subject to significant litigation.

Taxonomy

CRISPR Systems

- General
- Cas 9
- Cas 12
- Cas 13
- Others

Pharmaceutical Dosage Form

- General
- Drug
- Cream
- Spray
- Injection

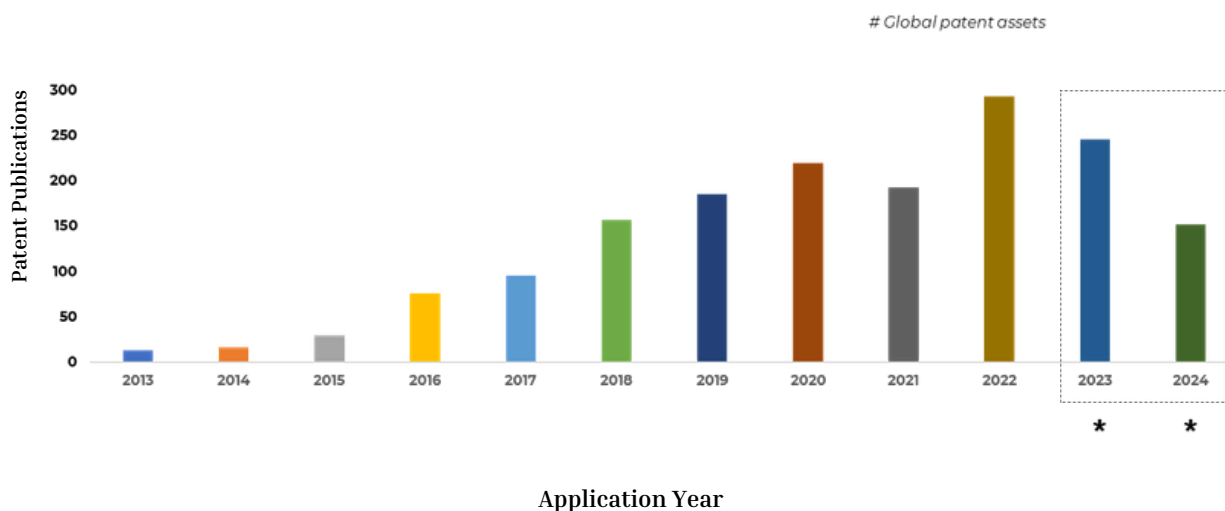
Blood Disorders

- General
- Inherited
 - Sickle Cell Disease
 - Thalassemia
 - Hemophilia
 - Thrombophilia
 - Hemoglobinopathy
 - Clotting Factor Deficiency
 - Fanconi Anemia
 - Von-Willebrand Disease
 - Others
- Non-Inherited
 - Hematological Malignancy
 - Leukemia
 - Lymphoma
 - Myeloma
 - Anemia
 - Thrombocytopenia
 - Others

Methods of Delivery

- General
- Physical
 - General
 - Electroporation
 - Microinjection
 - Gene Gun
 - Others
- Chemical
 - Liposomes
 - Lipid Nanoparticles
 - Inorganic Nanoparticles
 - Polymer Nanoparticles
 - Others
- Viral
 - General
 - Adenoviral
 - Adeno-associated Viral
 - Retro Viral
 - Lenti Viral
 - Others
- Plasmids
- Expression Vector

Overall Filing Trends

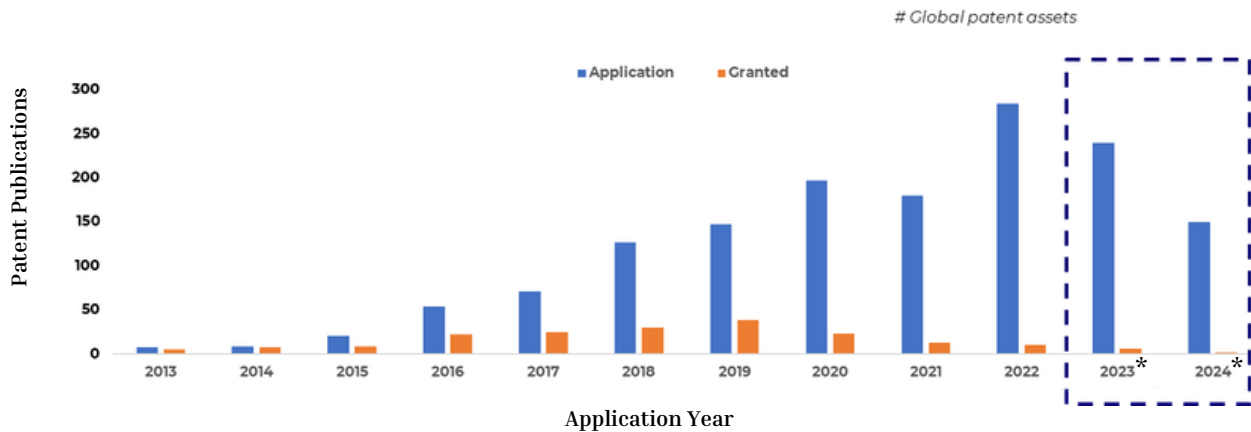


- The patent filing trends shown in the above figure depict a significant increment in the domain of “CRISPR system based treatment of blood disorders” from 2013 with a slight dip in the year 2021.
- More than 90% patent applications were filed after 2016.
- Maximum number of patent applications (294) were filed in 2022. Sana Biotechnology Inc (15), Univ of California (9) and Stanford Univ (8) were the top assignees in the year.
- The first patent filing by EDITAS MEDICINE INC was in March, 2015. The invention (EP3981876A1) related to CRISPR/CAS-related methods and components for editing of a target nucleic acid sequence, or modulating expression of a target nucleic acid sequence. It further provided use of the composition to treat sickle cell disease or beta-thalassemia.
- The first patent owned by Sana Biotechnology Inc in the domain was filed in 2020. The invention discloses a novel method involving the modification of isolated cells to reduce the expression of MHC class I and II human leukocyte antigens while enhancing the expression of DUX4 and other immune-evasive markers. It includes genetic modifications using a CRISPR/Cas specific genes for modulation of the expression of MHC I and MHC II human leukocyte antigens and can be implemented in cell therapies for treating various diseases including leukemia.

Notes: 1. The above chart is prepared using unique INPADOC family.

2. The number of patent applications filed in 2023/2024 may increase as some of the applications filed during this period may not have been published till date.

Filing Trend vs Grant Trend

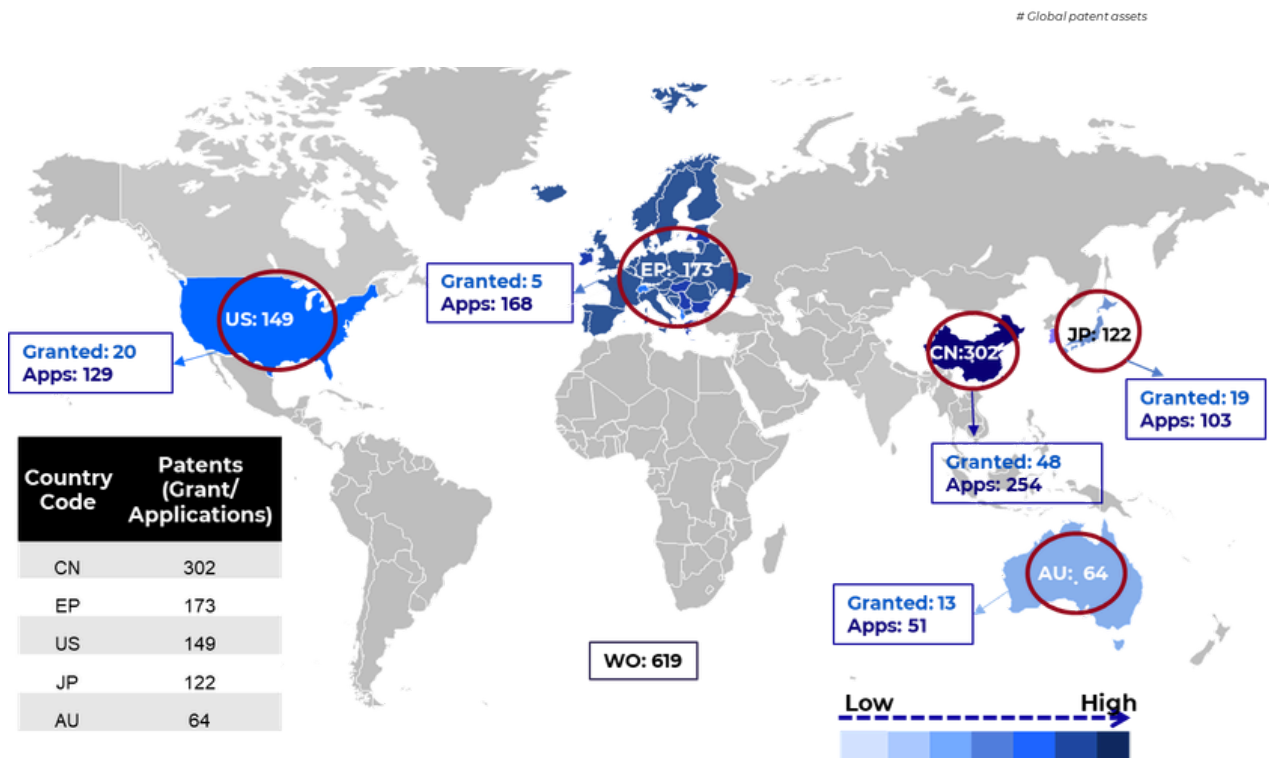


- Average number of filings per year for last 5 years is ~ 210, whereas average number of grants per year for last 5 years is ~ 11 (~ 19% of average filings per year).
- Harvard Univ has 9, while Broad Institute Inc and Massachusetts Institute of Technology own 7 granted patents each. They are closely followed by Editas Medicine Inc, Beam Therapeutics Inc and CRISPR Therapeutics AG with 6 granted patents each.
- While there was a surge of applications (284) in 2022, maximum number of patents were granted (39) in the year 2019.
- Jurisdictions with maximum number of granted patents are CN (48), SG (43), US (20), JP (19) and AU (13).
- PFIZER INC has a sole patent filing (KR20220131293A) in the domain. The invention related to the prevention and treatment of sickle cell disease using antibodies and antigen-binding fragments, additionally employing gene editing therapy by CRISPR.
- Assignees with highest number of filings are Broad Institute Inc (62) > Massachusetts Institute of Technology (48) > Harvard College (46) and Univ of California (45). Among the companies, Editas Medicine Inc, CRISPR Therapeutics AG and Fate Therapeutics Inc lead with 29, 28 and 22 patent filings, respectively.
- Out of 100 patent filings for treatment of Fanconi anemia, only 7 have been granted so far. Similarly, only 12% of the patent filings for treatment of Sickle cell disease have been granted.

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2. The number of patent applications filed in 2023/2024 may increase as some of the applications filed during this period may not have been published till date.

Top Jurisdictions



- There seems to be maximum patent filings done (36.8%) via PCT route (WIPO).
- CN is witnessed as the most preferred jurisdiction in the domain of “CRISPR system based treatment of blood disorders” (encompassing 17.9% of total filings), followed by EP and US (with 10.3% and 8.8% of filings, respectively).
- Shanghai University and BioRay Lab Inc are the leading assignees in CN jurisdiction with 16 and 9 patent filings, respectively.
- Japan marks its presence in the domain with 7.2% of total filings, followed by Australia with a mere 3.8 % of the filings).
- Except China and Japan, the presence of other Asian countries seems to be markedly inconspicuous in this domain.
- CN118726309A by Shanghai University provides a base editor system based on the micro CRISPR Cas protein Cas12f1 and its application to perform gene therapy for single-base mutation genetic diseases including those related to blood.

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Market Insights: Overview

The CRISPR gene editing market for the treatment of diseases is experiencing significant growth, driven by technological advancements, increasing prevalence of genetic disorders, and the rising demand for personalized medicine. The approval of the first CRISPR-based therapy, CASGEVYTM, has been a major catalyst, validating the potential of this technology.

- The market is populated by a mix of established pharmaceutical, biotechnology and gene-editing companies. Some of the key players include CRISPR Therapeutics, Vertex Pharmaceuticals, Editas Medicine, Beam Therapeutics, Caribou Biosciences, Regeneron Pharmaceuticals, Scribe Therapeutics and Sanofi.
- Continuous improvements in the precision, efficiency, and delivery methods of CRISPR-Cas systems are expanding its therapeutic potential.
- Being a rapidly growing industry with immense potential benefits, CRISPR-based therapeutics is witnessing significant investments from both private and public sectors in biotechnology and pharmaceutical companies.
- However, technological challenges (such as off-target unintended effects and efficient delivery of gene-editing tools) as well as substantial costs associated with research, development, and manufacturing of CRISPR-based therapies can hinder technology growth.
- The CRISPR gene editing market for the treatment of diseases holds immense promise for the future of medicine. With ongoing research, technological advancements, and increasing regulatory support, more CRISPR-based therapies are expected to reach the market.
- Addressing the current challenges related to cost, safety, and delivery will be crucial for realizing the full potential of CRISPR technology in transforming healthcare.

Key Market Players And Their Products



Casgevy

- Exa-cel (Casgevy) approved on Nov. 16, 2023, by UK MHRA.
 - Treats sickle cell disease (SCD) and transfusion-dependent beta-thalassemia (TDT).
 - Later approved by U.S. FDA for both conditions.
-



CD117 antibody-drug conjugate (ADC)/ in vivo HSC editing

- In vivo CRISPR/Cas9 delivery to HSCs.
 - Avoids ex vivo editing and HSCT.
 - Enables simpler, less invasive treatment.
-



CTX131: Anti CD-70 allogenic CAR T (Clinical Trial)

- Allogeneic CRISPR/Cas9 gene-edited CAR T therapy in development.
 - Targets solid tumors and hematological malignancies.
 - Includes novel edits to enhance CAR T potency.
 - Designed to reduce CAR T exhaustion.
-



CTX112: Anti CD-19 allogenic CAR T (Clinical Trial)

- Allogeneic CRISPR/Cas9 gene-edited CAR T therapy in development.
 - Targets CD19+ malignancies and autoimmune diseases.
 - Novel edits enhance CAR T potency.
 - Designed to reduce CAR T exhaustion.
-



CTX120 (Clinical Trial)

- Phase 1 dose escalation and cohort expansion study.
 - Evaluates safety and efficacy of CTX120.
 - Allogeneic CRISPR-Cas9-engineered T cells targeting BCMA.
 - Designed for relapsed or refractory multiple myeloma.
-



CRISPR medicines (Clinical Trial)

- Editas is developing CRISPR medicines for blood diseases.
 - Focus on in vivo gene editing medicines.
 - Targets Sickle Cell Disease (SCD) and Beta-Thalassemia.
-



CRISPR medicines (Clinical Trial)

- Editas is developing CRISPR medicines for blood diseases.
 - Focus on ex vivo gene editing medicines.
 - Targets Blood Cancer.
-

TECHNOLOGY GAP

CRISPR/Cas system-based treatment of blood disorders–The CRISPR/Cas system has emerged as a revolutionary tool for treatment of various diseases through specific gene editing and long-lasting therapeutic effects. This gene-editing technology allows to precisely target and modify DNA, offering potential cures for inherited and non-inherited blood disorders.

PATENT ACTIVITY

LOW

- Filings in the domain of CRISPR/Cas system based treatment of blood disorders employing other CRISPR associated proteins besides Cas9 and Cas12 and delivering the CRISPR/Cas system to target cells via viral methods that use poxvirus vector, herpes simplex virus vector, baculovirus vector and Sendai virus vector have not yet been fully explored thus creating a space to explore more in this domain.
- The domain has not yet been fully investigated for treatment of blood diseases such as von Willebrand disease, hemangiomas, blood clotting disorders, myeloproliferative disorders, eosinophilic inflammatory diseases, Myelodysplastic syndrome and Graft-versus-host disease.
- There are fewer filings on gaseous pharmaceutical preparations comprising compositions related to CRISPR/Cas system for treatment of blood disorders.

PRODUCTS & SERVICES

MEDIUM

- A substantial number of research papers are published in this area, and have implementation in application of CRISPR/Cas system for treatment of blood disorders e.g. Advances in CRISPR/Cas-based Gene Therapy in Human Genetic Diseases

MARKET SCOPE

HIGH

- The global CRISPR-based gene editing market is projected to grow from \$4.46 billion in 2025 to approximately \$13.39 billion by 2034, reflecting a Compound Annual Growth Rate (CAGR) of 13%. [[Source](#)]

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