

Technological Prospecting in Gene and Cell Therapies: A Global Analysis of Patents and Innovation in Human Health

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This study carries out an analysis of technological prospecting in biotechnology applied to human health, with an emphasis on gene and cell therapies. The aim is to identify the main technological innovations, the main investors and the most developed regions in the sector. Using the ORBIT Intelligence database, the 25 most cited patents (minimum of 10 citations) were analyzed. The abstracts of the patents were analyzed using IRAMUTEQ software, allowing thematic and technological patterns to be identified. The results show a concentration of investments in advanced therapies, highlighting the growing relevance of these technologies for the pharmaceutical industry and clinical research. The conclusion is that biotechnology for human health is on a growth path, with a direct impact on therapeutic innovation and the development of new medical approaches.

Keywords: biotechnology, technology prospecting, gene therapy, cell therapy, patents

Introduction

Modern biotechnology is the subject of doubts, fears and concerns, as well as intense and forceful debate around the world about the potential risks to human health, the environment and society. Applications aimed at human health have advanced significantly, contributing to the development of innovative treatments and improving quality of life (Amaral et al., 2020), renewing hopes of a cure for diseases that were previously incurable (De Lima Júnior, 2024). In this context, understanding patent filing trends in this area is crucial to mapping the technological future, identifying innovation opportunities and understanding market dynamics. This technology foresight analysis focuses on biotechnology for mental health, an emerging field with high potential to transform global health.

In this context, the relationship between biotechnology and human health is a very topical area of study, particularly with regard to the use of gene and cell therapies, with an emphasis on gene editing (Regularly Interspaced Short Palindromic Repeats—CRISPR, for example) and stem cell treatments, which cannot be

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distanced from the deontological ethics guaranteed by bioethics (De Lima Júnior, 2024). These fields have seen exponential growth in terms of patent filings (Amaral et al., 2020), as new methods for DNA manipulation, cell regeneration and the treatment of genetic and degenerative diseases are developed.

The justification for carrying out this study is based on the need to map technological development in critical areas for health, identifying possible gaps and opportunities for new investments, especially considering the significant incidence of chronic, degenerative and genetic diseases in the population. The research problem is to understand the evolution of biomedical technologies in order to support innovation policies and identify strategic trends that could impact the future of human health.

Given the possibilities for exploiting patents in this area, including biomarkers, neuroscience and innovative biotechnological interventions in the treatment of chronic, degenerative and genetic diseases, based on the area of specialized medicine, the aim of this study is to analyze the main trends in patent filings in biotechnology applied to human health, identifying the main players, the regions with the greatest technological concentration and the most relevant innovations. In addition, the growing incidence of morbidities and the demand for innovative treatments justify this study, which makes it possible to identify technological opportunities and potential leaders in the field of biotechnology for human health.

Methodology

For data collection, the ORBIT database was used, chosen for the quality of the data, periodic updating, ease of export, comprehensiveness of patent data and statistical analysis tools, with the following search string: ((gene therapy OR “genetic therapy” OR “genetic editing” OR “CRISPR” OR “gene editing” OR “gene modification” OR “genome editing”) OR (“stem cells” OR “cell therapy” OR “cell-based therapy” OR “regenerative medicine” OR “cellular therapy”)) AND (“health” OR “human health” OR “medical treatment” OR “disease treatment”) AND (PUB_AFTER=2015) AND (IPC=A61K OR CPC=A61K38 OR CPC=A61K48 OR CPC=C12N15/10 OR CPC=C12N5/00). It also added a filter for selecting patents that have been cited at least 10 (ten) times in the past, in order to ensure the identification of technologies and innovations of greater application and quality.

The *string* was selected because of its ability to cover the main areas of innovation in biotechnology applied to human health, such as gene and cell therapies, while limiting the results to recent and relevant documents. It included relevant international patent classifications (IPC) and was built to capture data on the main *players* in the field, patents pending, granted, legal status of patents and investment trends. In detail, the choice of each segment was based on strategy:

- Main keywords: terms such as “gene therapy”, “CRISPR”, “stem cells”, and “cell therapy” have been used to ensure that the search focuses on the areas of gene and cell therapies.
- Words related to human health: terms such as “human health”, “medical treatment” and “disease treatment” helped to focus research on the use of these technologies for health treatments.
- Classification codes (CPC/IPC): The codes A61K (preparations for medical purposes), A61K38 (preparations based on proteins or peptides), A61K48 (gene therapies) and C12N15/10 (gene editing) and C12N5/00 (stem cells) have enabled a more precise search within the patent classification system. Thus, the A61K classification and its subclasses focus on therapeutic preparations biotechnologies, including proteins and gene therapies; and C12N focuses on processes for manipulating genetic material (mutation, editing) and the use of stem cells, covering both techniques and treatments derived from these technologies. Both were chosen to capture recent innovations in cell and gene therapies and biotechnological drugs in the field of human health.

- Date filter: The PUB_AFTER=2015 filter sought to ensure a focus on more recent patents (last 10 years) related to emerging innovations in the field.

The search was carried out in the database on October 28, 2024 and resulted in 34,767 patents that met the established criteria. For analysis purposes, the data from the 25 (twenty-five) most cited patents was used. Graphs generated from the data searched and tabulated by ORBIT were used, showing the evolution over time, the main applicants, legal status, investment trends, areas of technological activity and the most active inventors. Each graph was analyzed individually and correlated with data from the literature. The search was conducted using a specific search *string* to locate patents in biotechnology aimed at mental health. It included relevant IPC classifications and was constructed to capture data on the main players in the field, patents pending, granted, and the legal status of each patent. The analysis included a count of patent families and a detailed assessment of the profile of each player in the market.

For the qualitative analysis of the abstracts of the 25 most cited patents, we used the IRAMUTEQ software, a statistical tool that allows us to explore the frequency and co-occurrence of words, facilitating the identification of recurring themes and the categorization of content. This approach allowed for a more in-depth understanding of technological trends and focuses of innovation in the field of biotechnology applied to human health. The similarity analysis and word cloud generated by IRAMUTEQ made it possible to visualize connections between key terms, highlighting areas of convergence between different patented innovations.

Based on the results obtained with IRAMUTEQ, it was possible to identify thematic groupings that reflect the most promising areas and the areas of greatest investment in the sector. This analysis contributed to a detailed overview of emerging technologies, making it easier to identify gaps and opportunities for future research. The use of IRAMUTEQ therefore provided a robust methodological framework for the analysis of patents, enriching the interpretation of the data and providing a clearer view of development trends in biotechnology for human health.

Results and Discussion

The results indicate that the main players in the biotechnology sector applied to health include universities and pharmaceutical companies, including the Institut National de la Santé et de la Recherche Médicale (INSERM), the University of California, the Massachusetts Institute of Technology (MIT) and Hoffmann-La Roche. These organizations have robust patent portfolios, suggesting high investment in research and development (R&D). Figure 1 shows the main players in the field of biotechnology for human health, ranked by the number of patent families, with INSERM in the lead (650 families), followed by the University of California and MIT. The predominance of renowned academic institutions highlights the close relationship between scientific research and innovation applied to health (Tseng et al., 2020). This scenario reinforces the strategic role of academic-industrial collaborations in converting scientific knowledge into intellectual property (IP) and technological solutions.

In addition, the strong presence of universities and research centers in biotechnology, especially in emerging areas such as gene editing and stem cell therapies (Peter et al., 2023), reflects a Western dominance driven by substantial public and private investment in biotechnology. The joint action of institutions such as MIT and INSERM with pharmaceutical companies demonstrates the importance of technology transfer, whether through the creation of startups or strategic partnerships, fostering the generation and licensing of biotechnology. The institutional diversity highlighted in Figure 1 points to collaboration between the public

and private sectors as an essential factor in accelerating innovation in biotechnology. This collaborative ecosystem reduces the time between scientific discoveries and commercial applications, strengthening the global competitiveness of the companies involved. Studies indicate that such partnerships result in higher patenting rates and faster product development cycles, driving significant advances in biotechnology for human health (Bikard et al., 2019).

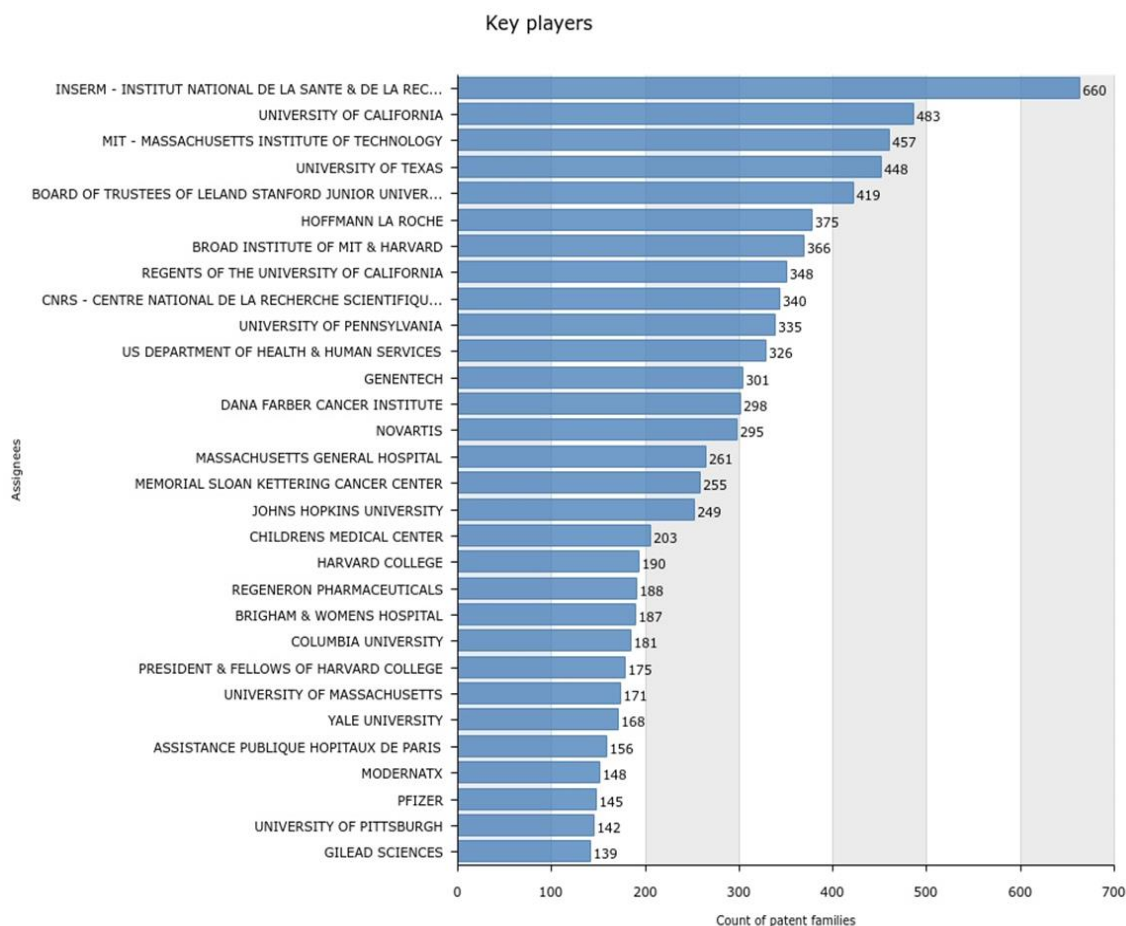


Figure 1. Main players in the biotechnology area for human health.

The data presented in Figure 2 shows the concentration of technological knowledge in the top 10 players, especially INSERM. This scenario suggests the existence of barriers to entry for new competitors, indicating a highly competitive and restricted field of innovation. The continuous expansion of these players' portfolios reflects their high propensity for innovation and intense investment in R&D, consolidating their strategic position in protecting IP in emerging technologies. Analysis of the legal status of patents (Figure 2) reveals that the main players, such as INSERM, the University of California and MIT, have a significant number of pending applications and grants, demonstrating a dynamic innovation environment. The constant volume of new registrations indicates an expanding sector in which the longevity of innovations and the viability of long-term investments are determining factors in maintaining competitiveness.

When comparing the players by legal patent status, there is a continued commitment to research and innovation, evidenced by the predominance of active patents over those that have been abandoned or expired.

The lack of significant departures reinforces the stability and growth of the sector. In addition, the significant number of patents granted and in the process of being applied for confirms the leadership of INSERM, the University of California and the Board of Trustees of Leland Stanford Junior University at the forefront of biotechnological innovations, signaling a growing trend in the expansion of their portfolios.

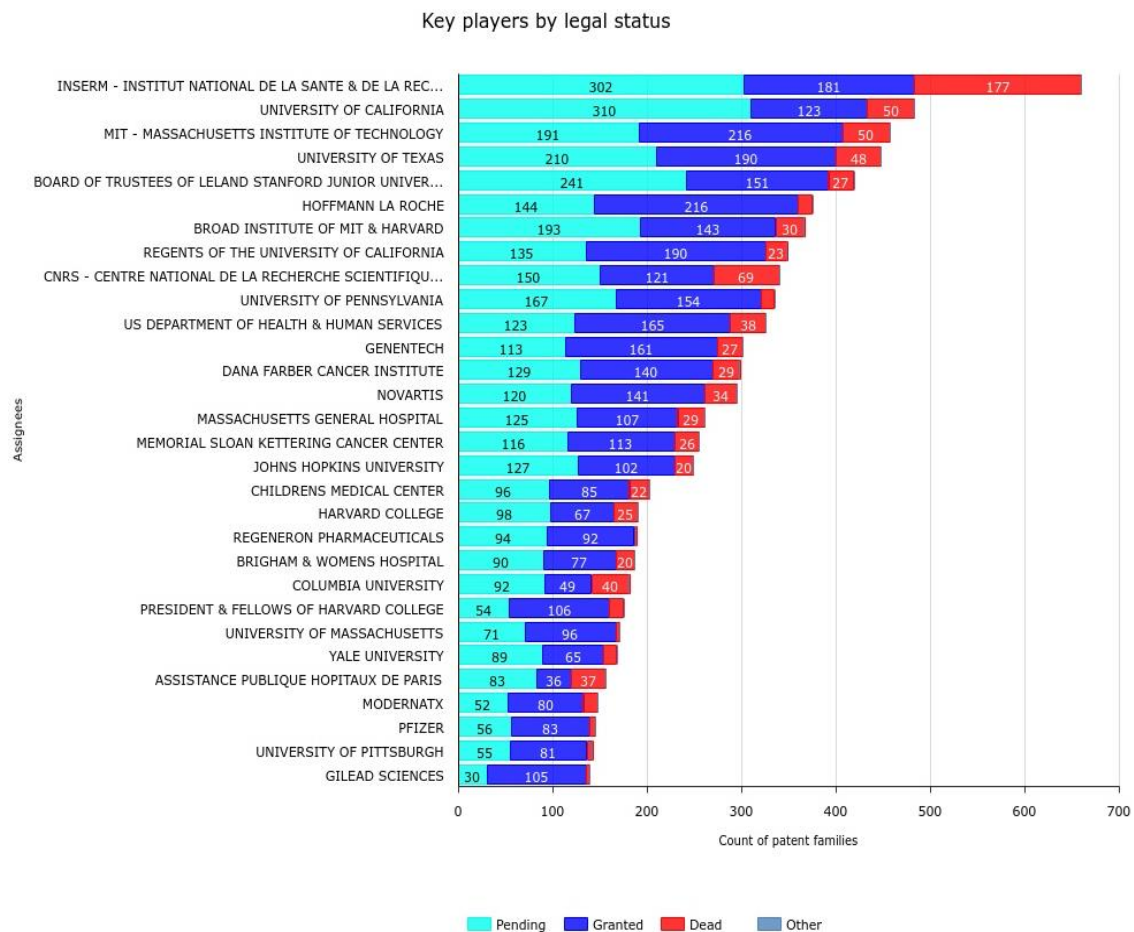


Figure 2. Main players by patent legal status.

Figure 3 shows exponential growth in the number of patents since 2013, with a peak in 2020. This increase reflects the advancement of biotechnology as a strategic area, driven by investments in innovative research such as gene editing and advanced therapies, which are essential for the treatment of complex diseases (K. M. A. Gartland & J. S. Gartland, 2018). The concentration of patents in recent years suggests a race for innovation, especially in technologies such as CRISPR, which have revolutionized human health. Studies indicate that medical biotechnology is largely funded by government and private investment (Lee & Vavitsas, 2021), aimed at developing more effective and affordable treatments (Kliegman et al., 2024).

The drop in the number of patents after 2021 may be associated with market saturation in some areas and increased regulatory complexity, which imposes stricter requirements for the approval of new technologies. In addition, the challenge of aligning legislation with biotechnological advances is becoming essential in order to guarantee fundamental rights. In addition, the global economic impact of the COVID-19 pandemic may have influenced investments in emerging sectors.

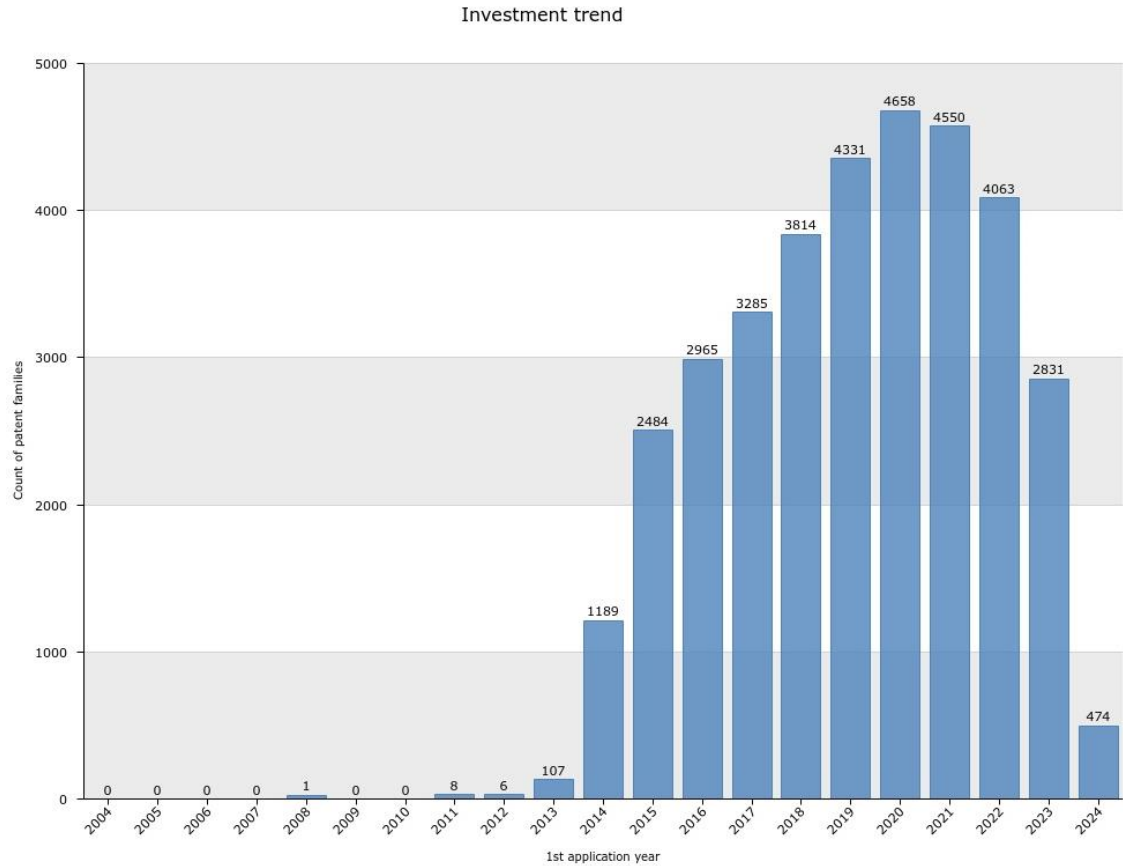


Figure 3. Investment trends.

Figure 4 shows the distribution of investments in patents among the main institutions and companies, such as INSERM, the University of California and MIT. These players lead innovation in biotechnology, indicating a concentration of capital and resources in highly skilled research centers. Such concentration is essential for the development of complex and disruptive technologies, such as biotechnology applied to health (Ari Yuka et al., 2023).

The dominant presence of universities and public research centers highlights the importance of government funding in health research. Nevertheless, Lee and Vavitsas (2021) demonstrate the relevance of public-private partnerships in the advancement of biotechnology, especially in the formation of a formidable impetus for innovation, meeting business and social objectives and contributing to the development of countries. In this vein, they state that life sciences and biomedical research, in particular, have a precedent for successful collaborations, a fact that corroborates the findings of this study when analyzing the main players listed.

Finally, there is the involvement of pharmaceutical companies such as Novartis and Roche, which license technologies developed in academic institutions, speeding up the translation of research into commercial products. The combination of academic expertise and the industrial capacity (investor consortium) of these companies, combined with a well-established regulatory path and clearly defined market demand, drives the transformation of biotechnology into effective health solutions, increasing the chances of success (Charlier et al., 2024).

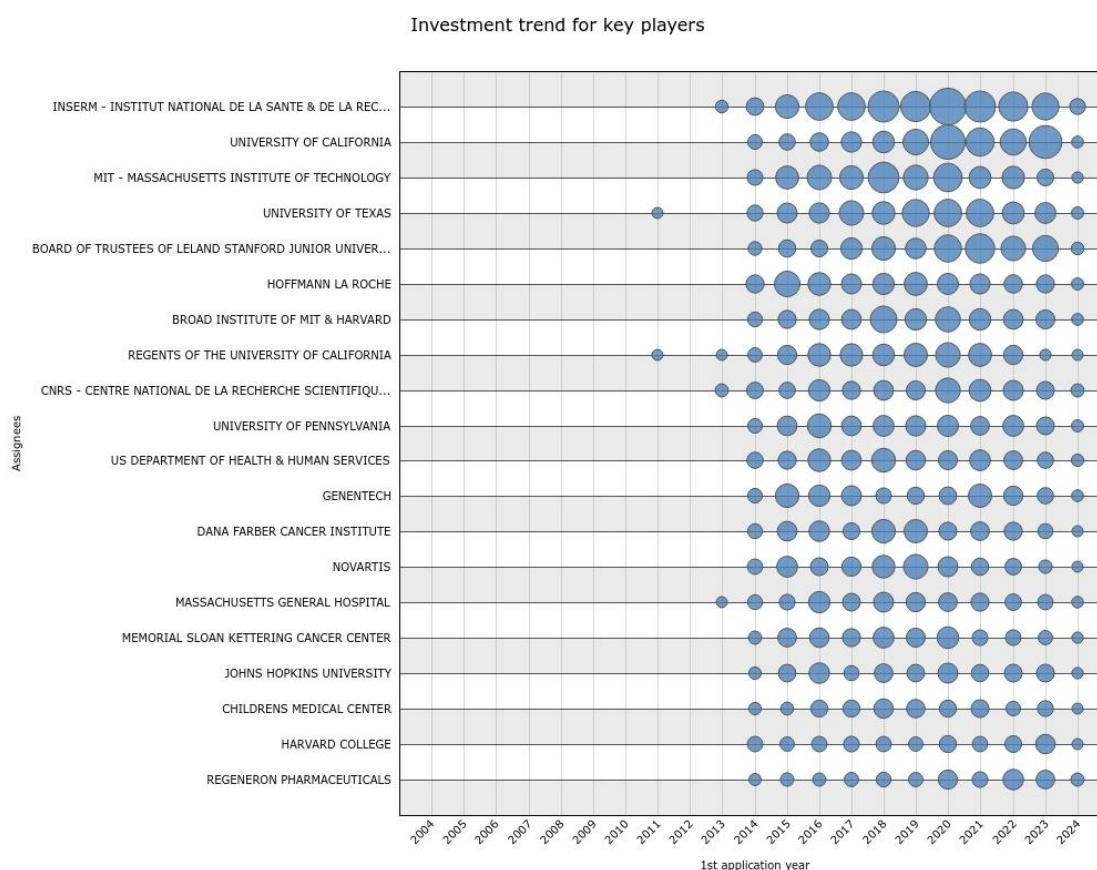


Figure 4. Investment trends by the main players.

The geographical distribution of patent registrations, shown in Figure 5, reveals the leadership of regions such as the United States, China and Europe, where the regulatory environment and government incentives drive innovation, favoring high investments in R&D, which maintains their competitiveness in the global market (Hrebenuk et al., 2024). The predominance of the United States and China in biotechnology is particularly relevant, given the growing demand for innovations to address global health challenges (Dos Santos Fonseca et al., 2024). This leadership is reflected in the competitiveness of these countries in strategic areas such as artificial intelligence and genomics, which drive the development of disruptive technologies (Da Silva, 2024). Continuous investment in R&D has generated a cycle of innovation, promoting the emergence of new patents and advanced technologies, and consolidating a sustainable competitive advantage (Taherdoost, 2024).

In Asia, China and Japan stand out, in line with national policies aimed at advancing biotechnology and developing technologies for well-being and mental health. China, for example, invests significantly in scientific research and infrastructure development for digital health, encouraging collaborations between the private and public sectors (Liu et al., 2020). This focus is driven by the growing demand for healthcare solutions in response to an ageing population and an increase in mental disorders, especially in urban areas (Wang et al., 2024).

In addition to the United States and China, countries such as Germany and Japan also stand out due to industrial and research policies that promote the development of advanced technologies and collaboration between companies and universities. These policies encourage the creation of science parks, which are key to advancing national and regional innovation strategies, acting as catalysts for technological innovation and

economic diversification (Hrebennyk et al., 2024). This environment conducive to innovation is essential for the competitiveness of these countries, especially in high-tech sectors. The geographical distribution shown highlights the importance of robust investments in R&D and public policies in the advancement of biotechnology and health. In addition, the European Union's favorable regulatory environment and its funding programs encourage both established companies and startups to invest in the creation of medical devices and digital tools for psychotherapeutic support, strengthening interconnected regional ecosystems where cooperation and competition drive advances in health.

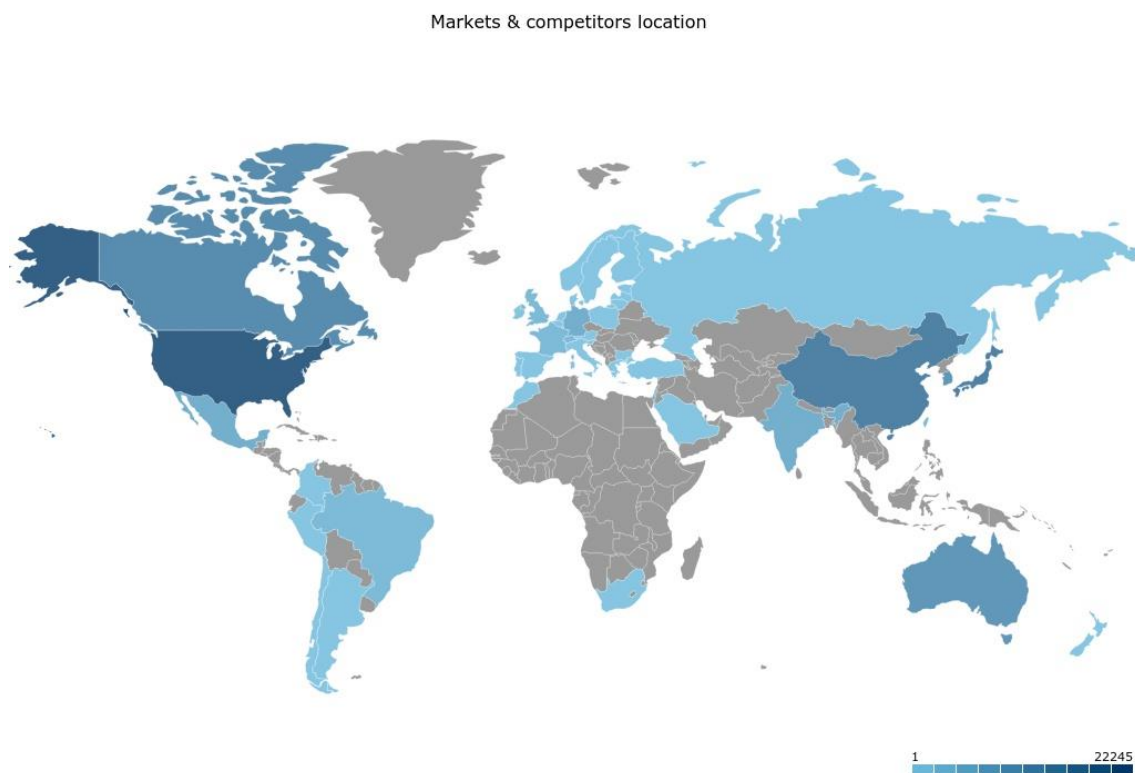


Figure 5. Location of markets and competitors.

Figure 6 offers a clear view of the expanding technological areas, highlighting sectors such as biotechnology, medical technology and pharmaceuticals, which are the main focus of innovation and patent filing (Dos Santos Fonseca et al., 2024). The intensity of the coloring indicates a high concentration of patents, reflecting R&D efforts, especially by pharmaceutical companies and research institutions, to meet global demands for new treatments and health solutions. The pharmaceutical sector is distinguished by the development of new formulations and precision therapies, with an emphasis on digital medicines, also addressing regulatory and ethical issues (Prajapati et al., 2024). This advancement is driven by a collaborative ecosystem between companies and research centers, aiming for more effective and personalized treatments, especially for mental disorders that are on the rise.

The interdisciplinary nature of biotechnological innovations is evidenced by the presence of areas such as organic chemistry and nanotechnology, indicating that health innovations depend on a diversified technological base (Figueiredo, 2023). The convergence of these areas results in more robust and adaptable solutions, promoting significant advances in the treatment of diseases and the development of personalized therapies

(Esfandyarpour et al., 2024). The growing demand for medical technologies, driven by an ageing population and an increase in chronic diseases, highlights the importance of remote monitoring and wearable devices, reflecting an expansion of the market and the consolidation of preventive health solutions (Giansanti, 2024; Azizan et al., 2024). Biotechnology, meanwhile, is central to the development of biomarkers and early diagnosis technologies, offering new, more effective and personalized treatments, transforming the approach to complex pathological conditions (Chaudhary et al., 2023; Bhatia et al., 2024).

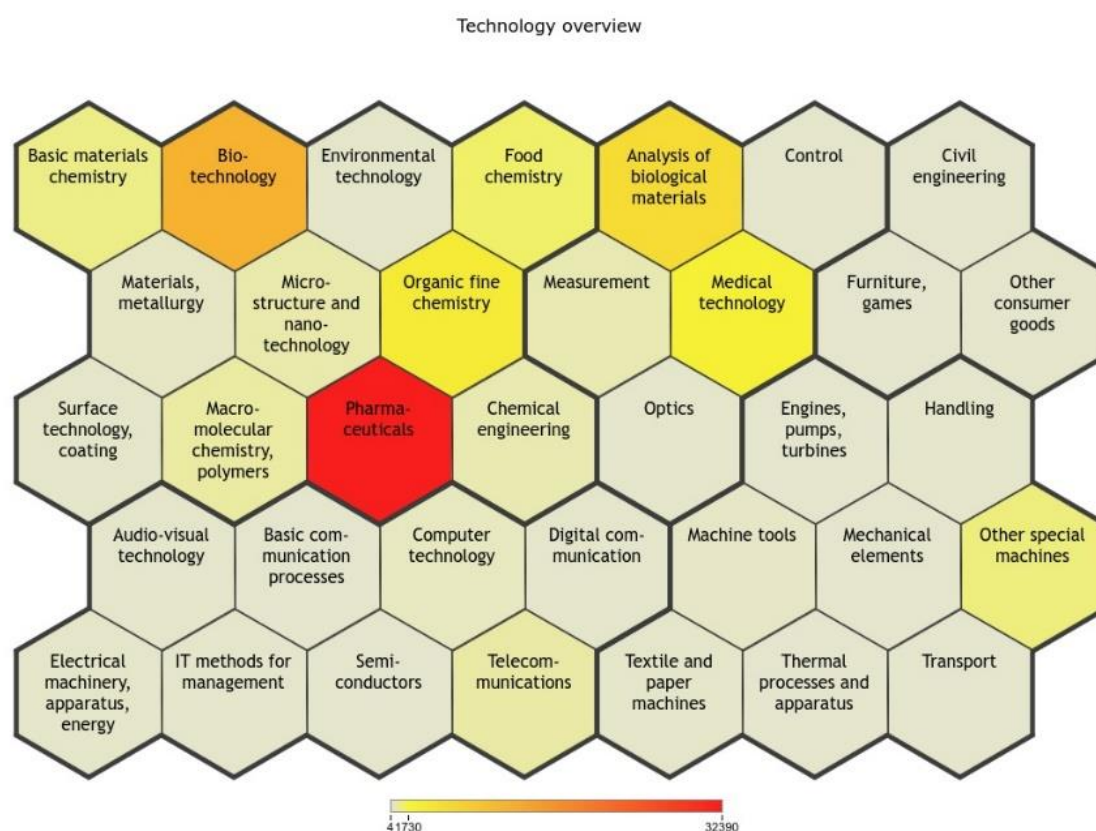


Figure 6. Technology overview.

Figure 7 provides a comprehensive overview of the main institutions and companies operating in different technical domains within biotechnology and mental health. It can be seen that academic institutions and research centers, such as MIT, University of California, and INSERM - Institut National de la Santé et de la Recherche Médicale, have a large presence in various technological areas, especially in biotechnology and the development of new compounds for mental health. These institutions are key to the advancement of scientific and technological knowledge (Bikard et al., 2019), especially in pharmacology and the analysis of biological materials, highlighting the importance of collaboration between the public and private sectors for innovation in health (Liu et al., 2020; Brandão Neto et al., 2024).

Large pharmaceutical companies such as Hoffmann-La Roche and Novartis also play a dominant role, with substantial investments in R&D to create innovative drugs and therapies. Their presence in the chart reflects the growing demand for advanced health technologies, especially in mental health, in line with increased global awareness of the importance of this field (Jeon et al., 2023). In addition, hospital centers and medical research

institutes, such as Memorial Sloan Kettering Cancer Center and Massachusetts General Hospital, stand out as key players. This indicates a growing interaction between clinical research and technological development, aimed at improving diagnoses and treatments. Such institutions are essential for integrating innovations into healthcare, promoting a feedback loop between research and clinical practice, driven by personalized medicine and biomarker-based treatments, especially in neuroscience and psychiatry (Chaudhary et al., 2023; Bhatia et al., 2024).

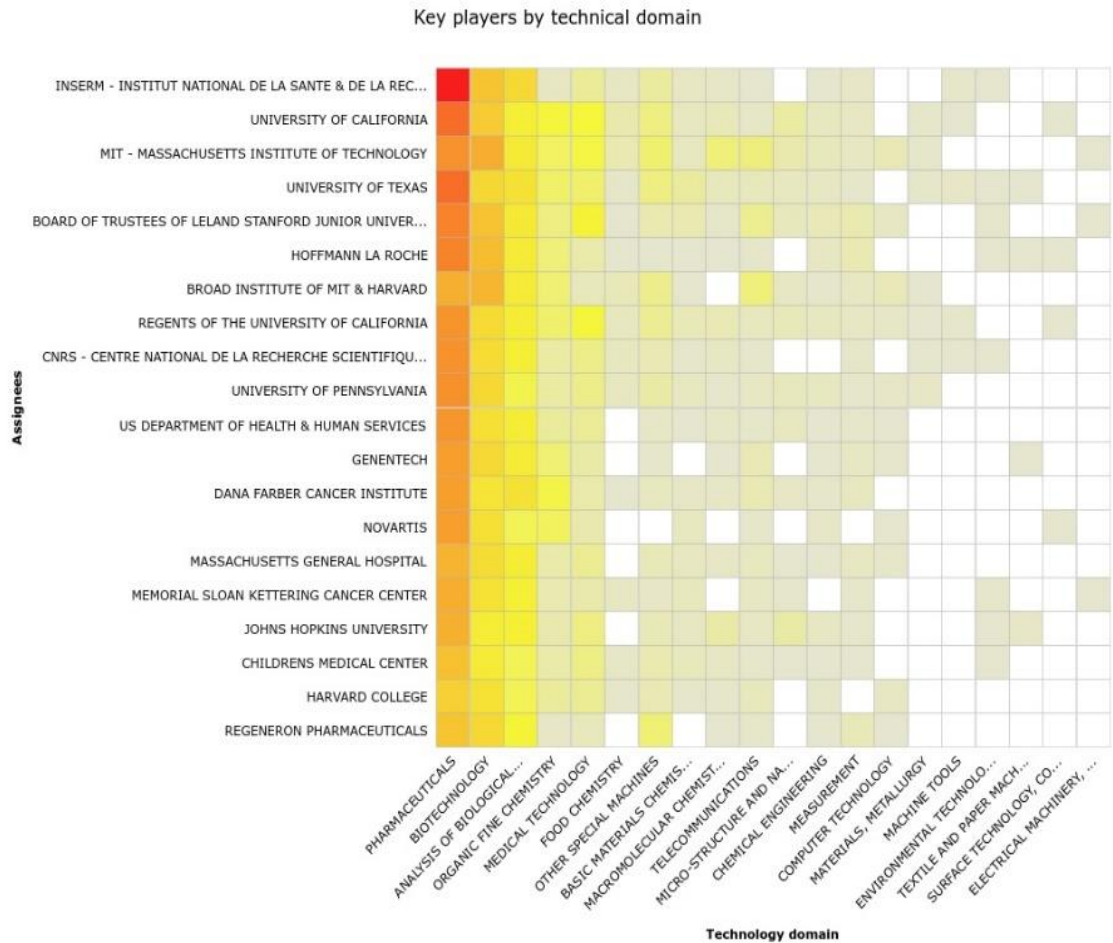


Figure 7. Technological domains of the main players.

Figure 8 shows the number of patent families per inventor, highlighting the main players in the production of patents in biotechnology applied to human health. Feng Zhang stands out with 107 patent families, consolidating his influence in the development of innovative technologies, especially in gene editing, such as CRISPR (Zhang, 2019). The pre-eminence of Zhang and other inventors suggests a strong connection with well-funded research centers of excellence, reflecting the advancement of gene therapies and precision medicine, promising in the treatment of complex diseases (Dunbar et al., 2018).

Christian Klein and Ugur Sahin are among the inventors with significant patent production. Klein is recognized for his contributions to immunotherapy, particularly in antibody-based therapies for the treatment of cancer (Klein et al., 2024), highlighting the search for more effective and personalized oncological approaches.

The concentration of patents in these researchers signals the centrality of immunotherapy and gene editing in contemporary medical biotechnology, reflecting global trends (Anyanwu et al., 2024).

The predominance of a restricted group of inventors points to a possible monopoly of knowledge, the result of high specialization, intensive funding and the infrastructure needed for advanced research. Studies indicate that biotechnology applied to health is a field with high barriers to entry (Zhou & Sun, 2022), mostly dominated by researchers linked to large academic centers and multinational companies (Liao et al., 2023). While this structure favors technological innovation, it can also limit the diversity of new approaches and ideas in the sector.

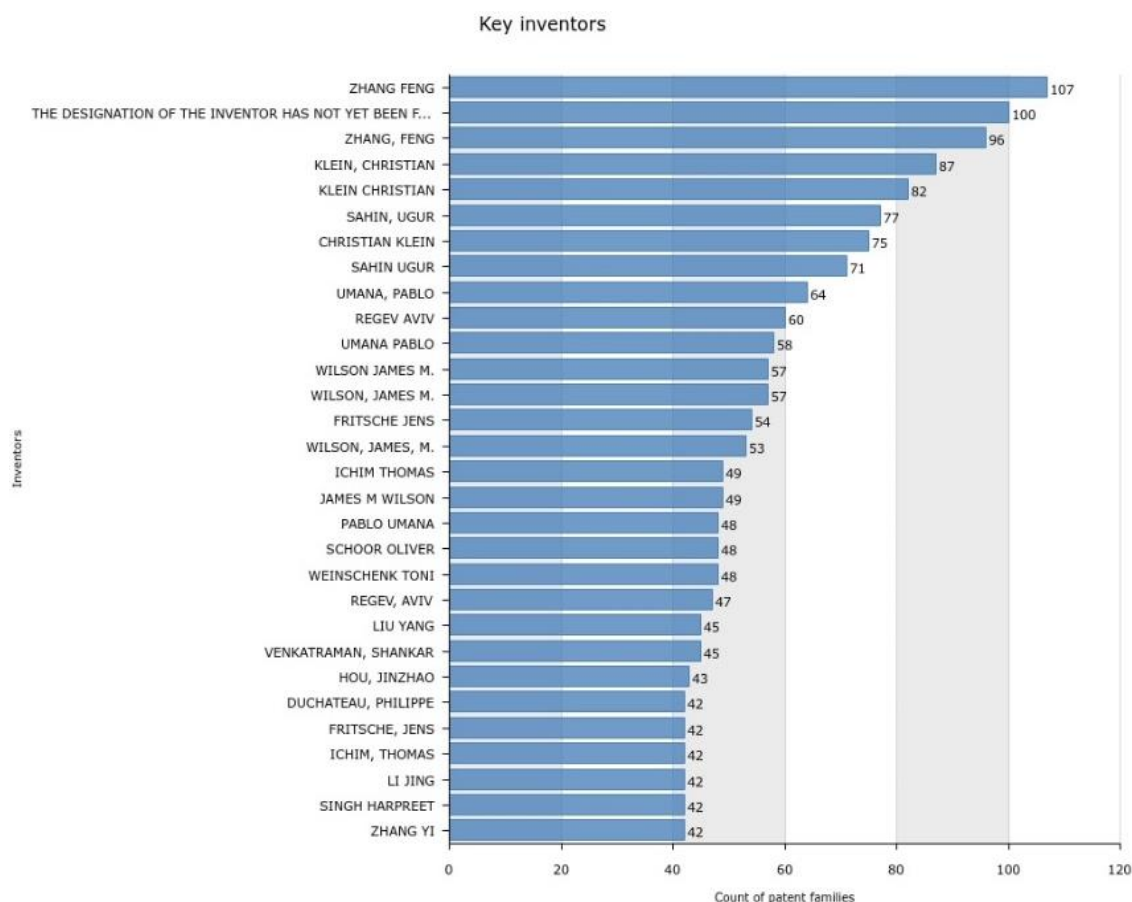


Figure 8. Main inventors.

The data shown in Figure 9 illustrates the main inventions by patent families, highlighting the leading institutions and companies in the field of biotechnology and health sciences. The Institut National de la Santé et de la Recherche Médicale (INSERM) leads the way with 650 patent families, followed by the University of California (480) and the Massachusetts Institute of Technology (446). This scenario reflects the strong emphasis of these institutions on the R&D of innovative medical technologies, in line with the growing demand for public health solutions.

In addition to academic institutions, pharmaceutical companies such as Hoffmann-La Roche and Novartis are also among the main patent holders, highlighting the strategic role of IP in protecting R&D investments. The long development cycle for new drugs, coupled with high costs and regulatory requirements, makes IP a critical factor in this sector. The presence of licensed and litigated patents, as indicated in the figure legend, reinforces

the complexity of this environment. The significant participation of institutions such as Harvard, CNRS and the Broad Institute underscores the importance of collaboration between academia and industry in accelerating innovation. The significant participation of institutions such as Harvard, CNRS and the Broad Institute underlines the importance of collaboration between academia and industry in accelerating innovation.

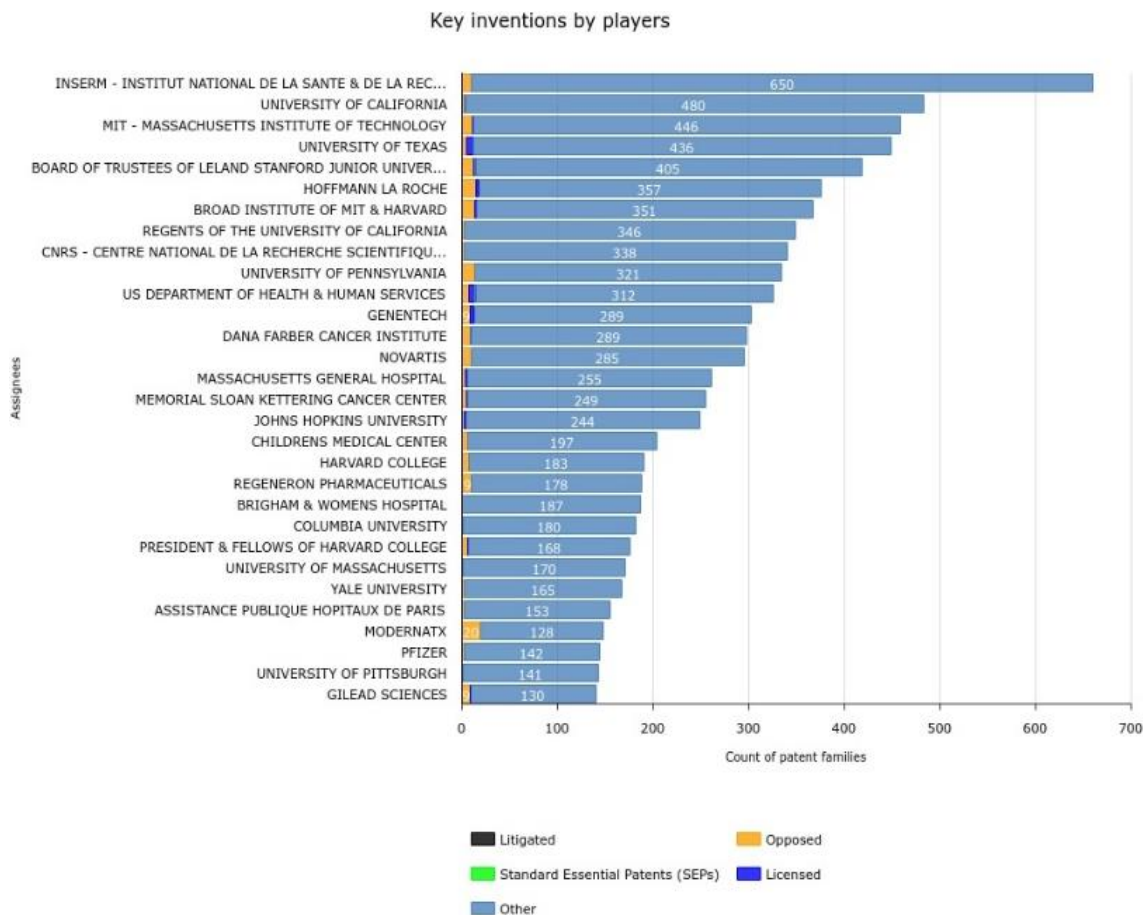


Figure 9. Main inventions by players.

The word cloud is a visual representation that highlights the frequency of terms in proportion to their size, allowing a quick understanding of the main topics covered in the patents. In this cloud, terms such as “gene”, “cell”, “edit” and “stem” are highlighted, showing that gene editing and cell biotechnology are central themes in the patents analyzed. This representation confirms the trends observed in the similarity and Zipf analyses, reinforcing the importance of these concepts in the current biotechnology scenario.

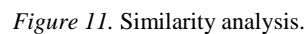
In addition, Figure 10 allows us to quickly identify other complementary themes and associated technologies, such as “CRISPR” and “Cas9”, which are specific technologies widely used for gene editing. The prominent presence of these terms corroborates recent studies showing the dominance of *CRISPR-Cas9* technology in the biotechnology patent landscape, as it offers high precision and efficiency compared to previous methods (Cox, et al., 2017). This dominance is a reflection of the increasing application of CRISPR in areas such as personalized medicine and targeted therapies.

The word cloud also shows an interdisciplinary trend in patents, as it includes terms related to different areas such as “composition”, “method”, “system” and “disease”. This suggests that biotechnology applied to human

[illegible]

The similarity analysis presented in Figure 11 shows the relationships between key terms extracted from the abstracts of the 25 (twenty-five) most cited patents. This type of analysis is valuable for identifying thematic groupings and the hierarchy between concepts, making it possible to observe the centrality and interrelationships of terms in the field of biotechnology and gene editing. The term “*CRISPR*” is strongly related to “*genome*” and “*cas9*”, indicating that these concepts are intrinsically linked, especially in modern gene editing approaches. These findings corroborate recent studies that point to the importance of the CRISPR-Cas9 system as an essential tool for gene editing, enabling significant advances in gene therapy (Arif et al., 2024).

Looking at Figure 11 helps to highlight the words with the greatest impact in the field, as well as suggesting the existence of emerging research subdomains. The strong relationship between “stem” and “cell” highlights the continued interest in stem cells and their therapeutic potential. Importantly, stem cells remain at the center of medical advances due to their potential for differentiation and self-renewal, which makes them ideal for tissue regeneration and tissue engineering research (Abusalah et al., 2024).



In the second quadrant, there is a concentration of terms related to genetic innovation and CRISPR therapies, such as “sequence”, “genetic”, and “technology”. These terms reflect the advance and popularization of *CRISPR-Cas9* as an essential tool for genetic manipulation, already consolidated in the field of medical biotechnologies. The arrangement of these terms close to the central axis indicates that they are often central concepts in mental health patents, reinforcing their growing importance in contemporary research.

In the third quadrant, terms such as “disclose” and “nucleic acid” stand out, suggesting an emphasis on new discoveries about nucleic acids and their application in genetics-based diagnostics or therapies. The position away from the center suggests that these terms, although relevant, represent a specific area of research focused on

molecular diagnostics and genetic identification methods, which complements the development of more personalized therapies.

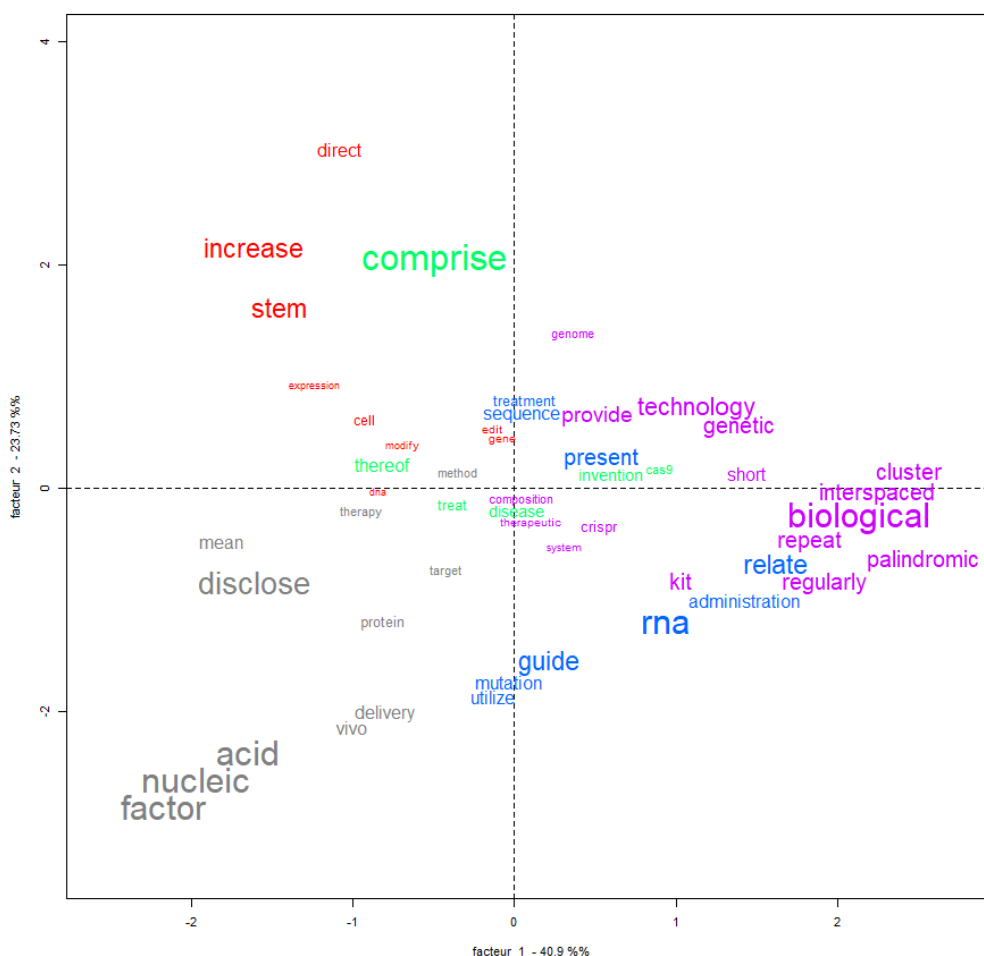


Figure 12. Factorial correspondence analysis.

The dendrogram shown in Figure 13 indicates the presence of clusters of strongly correlated terms, reinforcing the interconnection between technological advances in biotechnology. The grouping of these terms indicates a hierarchical classification where the upper classes encompass broader themes, while the branches closer to the leaves represent specificities of the patents analyzed. This hierarchical structure is useful for identifying thematic domains within the patent corpus, offering insights into priority areas for research.

In Figure 13, Class 3 groups together terms such as “comprise” and “increase”, suggesting an emphasis on processes and methods to increase the efficiency or scope of biological technologies. This focus may be related to patents that seek to optimize cellular or genetic therapies, reinforcing current trends in health biotechnology (Klein et al., 2024). Class 4, which includes terms such as “guide” and “rna”, seems to be related to genetic manipulation, specifically involving RNA, which is indicative of advances in CRISPR technologies and gene editing therapies. Analysis of the classes allows us to understand how the different terms and themes interact to form a cohesive field of research, especially with regard to innovation in human health, allowing us to map the development of new technologies and predict potential growth areas in the area studied.

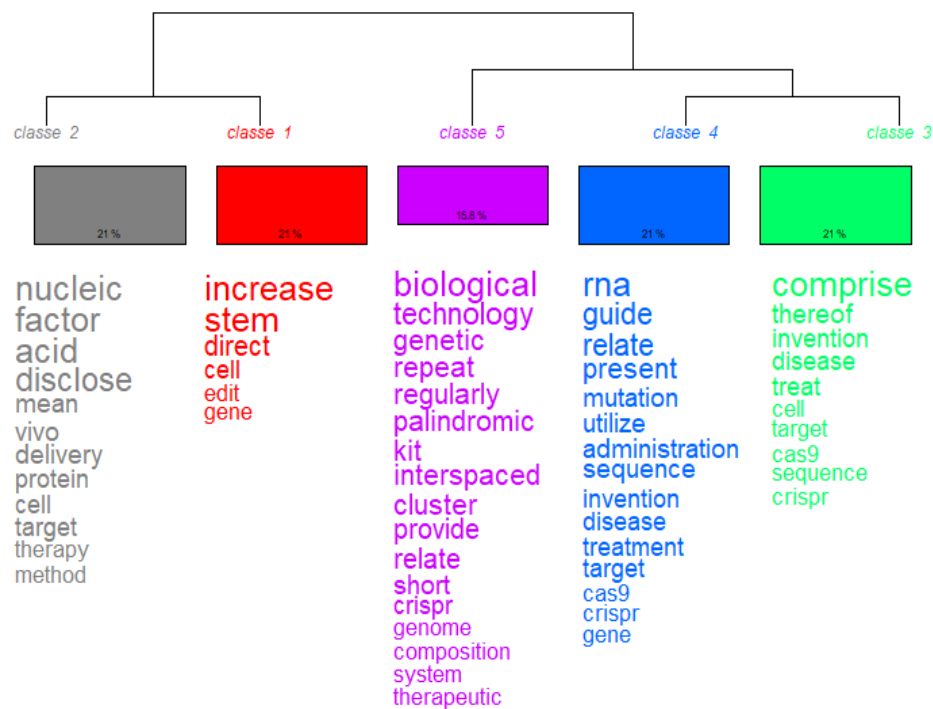


Figure 13. Dendrogram.

Final Considerations

The analysis carried out allows us to infer that biotechnology aimed at human health represents an area of high potential for future development, especially in collaborations between the academic sector and industry. The current scenario of patent filings demonstrates that there is still room for disruptive innovations that can bring significant benefits to global mental health, as well as suggesting opportunities for new investments and intellectual protection strategies, especially in countries with a high concentration of patents.

These patent analyses show how different organizations contribute to technological advancement in strategic areas and how the interactions between them promote innovation. These graphs not only reveal the leadership of certain institutions, but also illustrate the complex networks of dependence and collaboration that are fundamental to continuous technological advancement. The use of patent analysis, including citation mapping and licensing, can assist in creating effective strategies for R&D and commercialization of new technologies, as also suggested in the studies by Jeon et al. (2023) and Hrebeniuk et al. (2024).

The data analyzed shows that gene and cell therapies are expanding, consolidating themselves as key areas of modern biotechnology. The growth of patent registrations suggests a highly competitive scenario, driven by scientific advances and favorable regulations. Future studies could explore the interactions between the academic and industrial sectors, as well as the impacts of technological innovations on global regulation.

Acknowledgements

This work was completed during my doctoral studies and was supported by the Federal University of Sergipe, which provided access to and use of the ORBIT database. The opinions expressed in this article are those of the authors.

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