WWW.IJRPR.COM

International Journal of Research Publication and Reviews

Journal homepage: www.ijrpr.com ISSN 2582-7421

Diabetes Mellitus Type 1 in Pediatric Patients and Role of Gene Therapy in Treatment.

[1]Dr. Saurav Dey, [2]Dr. Zulhema Ashraf Hakak, [3]Dr. Faheem Yaqub Shah

[1] Md Physician, Ternopil State medical university, DCH(UK). Bedford hospital,UK.E-mail:saurav.dey@bedfordhospital.nhs.uk
[2]Md Physician, Tehran University of medical sciences, Directorate of Health Services, Kashmir, India.E-mail: winwon82@yahoo.com
[3]Md Physician, Ternopil State Medical University, Avicen Alshifa Modern Polyclinic, General Practice, Al-Batinah Region, Oman.
E-mail: faheem_yaqub@yahoo.com

1: Introduction

1.1 Introduction

Type 1 Diabetes Mellitus (DM1) is a universal pediatric health issue because it is marked by the autoimmune degeneration of pancreatic beta cells. The available treatments involve a strong focus on insulin therapy that accompanies daily hassles and long-term drawbacks. Gene therapy comes to the foreground as a potential therapeutic strategy in the DM1 management since the available data indicate that the disease development is, to a considerable extent, associated with genetic factors. In this work, the state of DM1 in children will be described, with an emphasis on the difficulties presented and the lack of effective treatment methods. Through examining the incorporation of gene therapy in relation to DM1, this study aims to advance the current knowledge in treatment methods while maintaining the effectiveness of the management in the young patients.

1.2 Background

The type of diabetes called Diabetes Mellitus Type 1 (T1DM) is one the most prevalent health complications among children and young adults, whereby the body's immune system attacks and destroys the insulin producing cells in the pancreas. Modern therapy is focused on insulin replacement by injections, which creates a lifelong therapy challenge (Moazam *et al.* 2024). The discoveries in the field of gene therapy in the past years present new directions for potential cure or disease prevention or alteration of disease risk due to genetic factors.

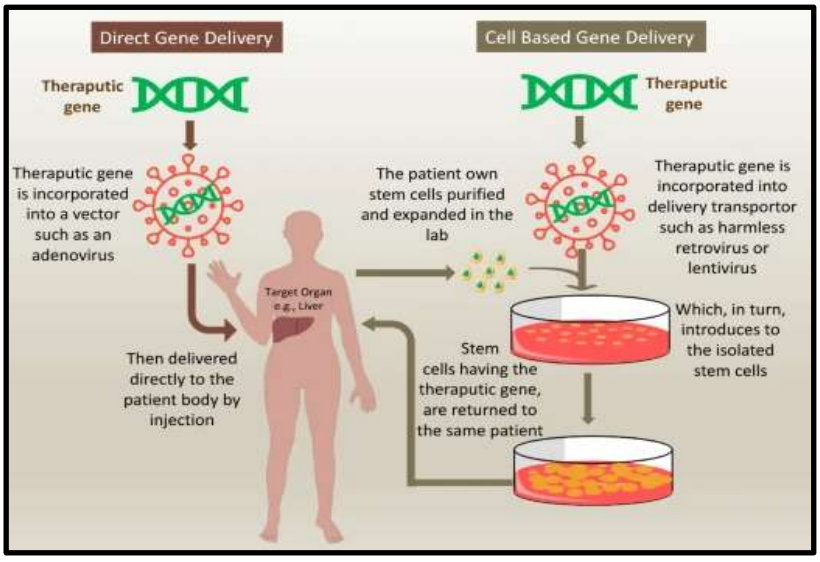

Figure 1.2: How genes are delivered to the human body during gene therapy approaches

(Source: Goyal and Malviya, 2024)

Gene therapy has applied two main strategies for the transfer of therapeutic transgenes into recipient's body The first technique involves direct infusion of the therapeutic gene into the body of the recipient through a vehicle Altered viruses are frequently used to transport the gene into certain human cells types non-viral vectors for direct delivery of genes into cells are still under consideration and include use of plain DNA and DNA confined within liposome coatings.

1.3 Rationale

The research proposal focuses on a three-partite study on diabetes mellitus type 1 (DMT1) in pediatric patients, gene therapy, and its effectiveness in the treatment of the disease that has considerable implications for the present-day understanding and management of autoimmune conditions in children The study stems from the realization that the current management of DMT1 is inadequate in providing comfort to the patients and halting the progression of the illness.

1.4 Problem Statement

T1DM is an extensive problem for children and adolescents as it need to monitor the level of blood glucose throughout It lives to avoid severe alterations. Today's management options such as insulin administration are important and necessary, but are not very effective in attaining optimal and sustainable glycemic control with minimal side effects. Thus, the requirement for significant and novel strategies is emerging, as gene therapy is to investigate the options with long-term insulin supply or improve the functionality of the pancreatic beta-cell (Mohammadi *et al.* 2024).

1.5 Research Aim

It is in this context that this research seeks to assess the effects of gene therapy in pediatric subjects diagnosed with T1DM to determine if it is a viable option in the management of the disease.

1.6 Research Objectives

- To describe the clinical phenotype and management issues regarding Type 1 Diabetes Mellitus (T1DM) in children.
- To pinpoint the current breakthroughs and constraints to use gene therapy to treat T1DM.
- To differentiate the efficiency and security of gene therapy intercessions used in pediatric population suffering from T1DM.
- To evaluate the ethical consideration and the legal working of gene therapy when used to address pediatric T1DM disease.

1.7 Research Questions

- Based on previously mentioned aspects of genetics, how do genetic factors play a role in the development regarding T1DM in Pediatric patients?
- What is the present knowledge on advancements of gene therapy methods that are applicable to T1DM?
- What are the pros and cons of gene therapy to be used in T1DM pediatric patients?
- Is gene therapy a better treatment option compared to conventional approaches for T1DM patients, especially children?

1.8 Research significances

The literature review on diabetes mellitus type 1, especially in children, and gene therapy in the management of this condition shows a wealth of information in the growth of pediatric care. Thus, investigating new strategies of gene therapy, the study can offer younger patients with this chronic disease new and more efficient and long-term solutions (Kudratova and Shamsiddinova, 2024).

1.9 Research structure

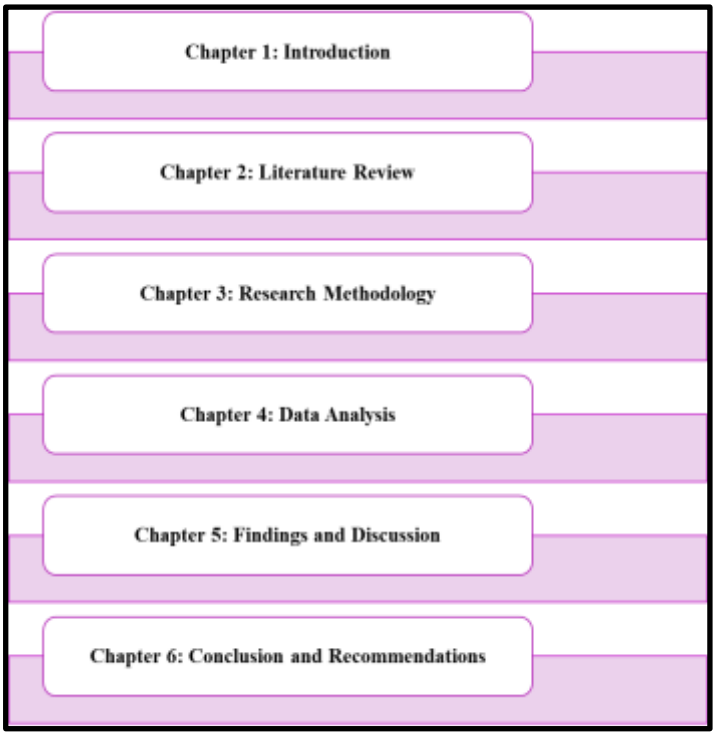

Figure 1.9: Research structure

(Source: Self-created)

1.10 Summary

The paper is specifically devoted to Diabetes Mellitus Type 1 and its development in children, with special attention to the genetic predisposing factors. It examines gene therapy as the new treatment modality it intends to explore concerning the genetic causes of insulin deficiency in diabetic patients.

2: Literature Review

2.1 Introduction

The literature review analyses the complex context of Diabetes Mellitus Type 1 in pediatric population with emphasis on genetics and new treatments. Based on a synthesis of published papers, it probes the genetic factor to see how It relate to the disease development and manifestation in children. Moreover, the review presents outcomes of contemporary investigations in the field of gene therapy, and what the writer seems to try to investigate is whether the gene therapy can play any role in altering the course of the disease by tackling genetic pathways that are related to insulin deficit. This section lays the groundwork for identifying the research deficits currently and the application of gene therapy in pediatric diabetes.

2.2 Genetic predisposition to Type 1 diabetes in pediatric populations and implications

The predisposition factor of genetic influence in type 1 diabetes in children focuses on how genetics contribute to the development of the disease in kids (Wal *et al.* 2024). Based on prior findings, it is well appreciated that several factors which encompass genetic predispositions and environmental factors account for Type 1 diabetes with genetics majoring on the ability of an individual to autoimmune, the Islet of Langerhans in the pancreas.

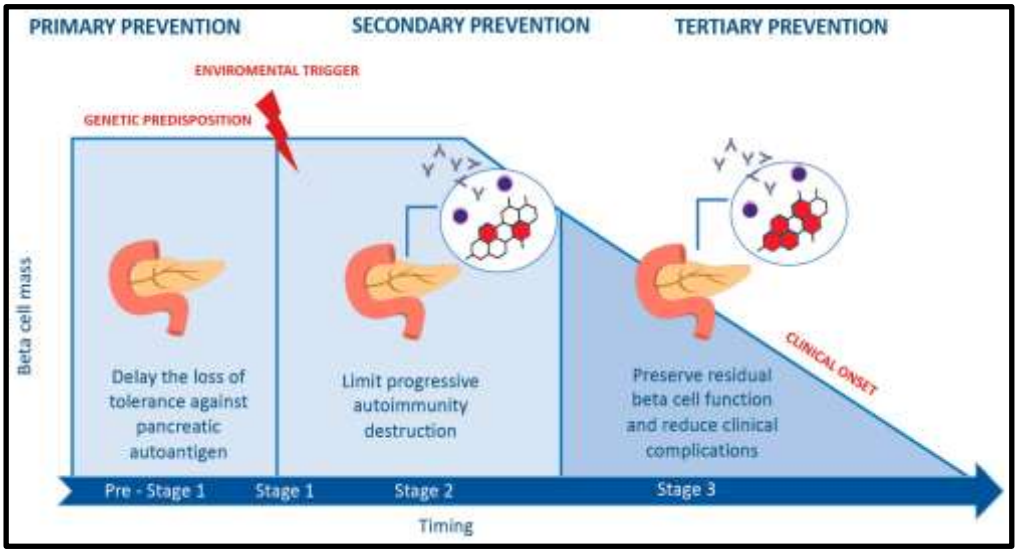

Figure 2.2: Types of prevention according to T1D stages

(Source: Al-Rrassam, 2024)

Consequences of such genetic knowledge apply to applications of genetics in custom-made medication strategies which could help to recognize children who need it due to It increased risk at early stages. The above is a clear indication that early disease identification could enable one to take preventive measures or early treatment that would halt the progression of the disease. Furthermore, future developments in genetic modification treatments could repair the root of the genetic problem or adjust the immune system reactions related with Type 1 diabetic patients, which might provide further controlled medical treatment approaches (Al-Rrassam, 2024).

2.3 Current treatment challenges and limitations in managing pediatric Type 1 diabetes

Current limitations and problems which exist in the management of pediatric, Type 1 diabetes include the following. First of all, insulin therapy, which serves as the primary treatment, comes with hurdles that include correct dosing and related dangers such as hypoglycemia (Bintang *et al.* 2024). This is a serious concern given the behavior of children with diabetes who are always challenged to remain compliant with-It insulin schedules, because of developmental issues, and changes in Its daily activities.

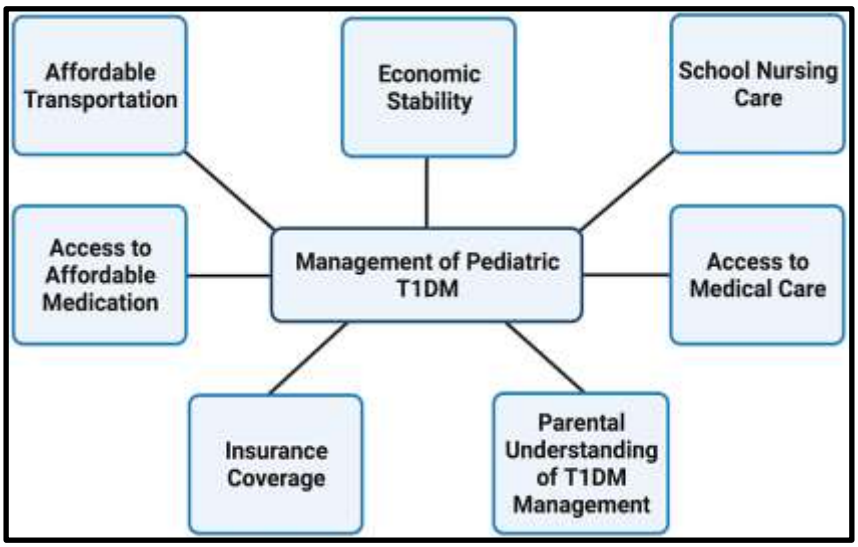

Figure 2.3: SDOH affecting management of T1DM in children

(Source: Yan et al., 2024)

Such complications as DKA do not seem to be eradicated completely, thereby stressing more on early and proper management as well as patient counselling. Furthermore, reflecting on the emotional loss and financial cost of the illness, it reaches young patients and Its families, which reproduces emotional stress and complicates the diseases' management (Yan *et al.* 2024).

2.4 Socio-economic impacts and healthcare disparities in pediatric Type 1 diabetes management

The terms "socio-economic effects and health inequalities in the management of pediatric Type 1 diabetes" describes the differences involving unequal utilization of health care facilities and resulting effects due to the economic and social status of an individual child with Type 1 diabetes. In this line of research, authors look into the role that socio-economic position plays on the use of specific services as well as the basic medications and diabetes self-monitoring technologies for children (Neiva *et al.* 2024).

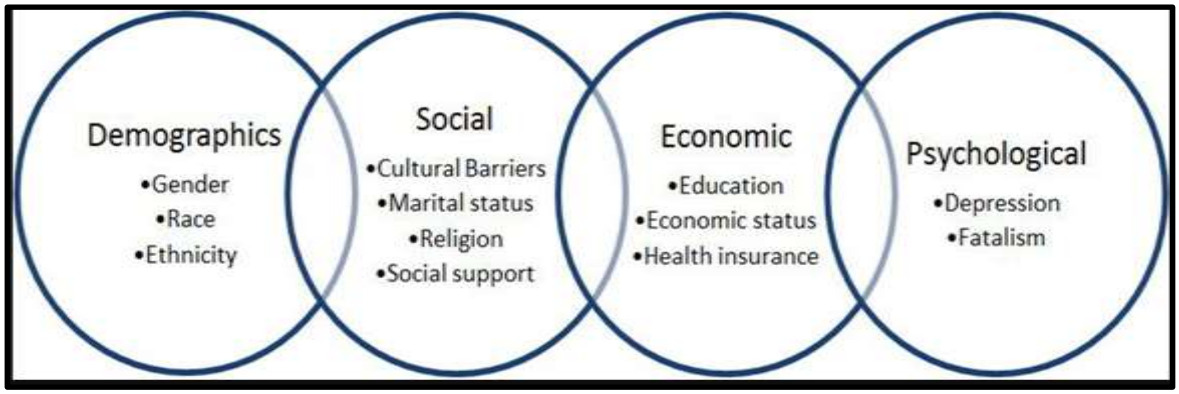

Figure 2.4: Social factors influencing glycemic control in type 2 diabetes mellitus patients

(Source: Addissouky et al. 2024)

The theme evaluates the soci economical costs on families like; the financial cost incurred in the process of managing the disease and its effects on children who have to endure the illness. Familiarizing themselves with these inequalities is essential for policy and intervention design in the treatment of Type 1 diabetes in children, especially with reference to financial limitations that affect pediatric patients (Addissouky *et al.* 2024). Through understanding and eradicating these socio-economic factors, the health care givers and policy makers ought to work harder towards achieving quality health for diabetic affected kids thus the quality of life of Its families is enhanced.

2.5 Theoretical framework

The theoretical framework for research on Diabetes Mellitus Type 1 in pediatric patients, focusing on the role of gene therapy, incorporates two relevant theories:) the Genetic Susceptibility Theory and Biomedical Model.

● Genetic Susceptibility Theory: This theory holds that several genes play a tremendous role in determining an individual's vulnerability to getting type 1 diabetes (Daniali *et al.* 2024). Special attention should be paid to certain indications associated with genetics of pediatric patients and the effects of environment on them. The research concludes that polymorphisms present in genes such as the HLA complex and insulin (INS) gene complex are determinant in autoimmune processes causing pancreatic beta-cell death. Thus, the study of these genetic vulnerabilities makes it possible to find indexes that would allow the identification of the disease in its early stage and undertake appropriate therapeutic measures.

● Biomedical Model: The model that will be elaborated and analyzed in details is the Biomedical Model which concentrates on the biological factors of disease. In the context of type 1 diabetes, it presents the centrality of the immune system failure to attack cells that produce insulin. Gene therapy interventions target these biological processes with an attempt to modify or correct It at the molecular level. This model helps in researching on gene therapies that intends to alter genes that cause autoimmunity complications that are genetically rooted (Grasso and Chiarelli, 2024).

2.6 Literature gap

There is a lack of studies describing a wide range of genetic options that regulate the development and progress of type 1 diabetes disease, focused on pediatrics as the population (Tarasiewicz *et al.* 2024). This is because many prior studies tend to concentrate on the efficiency in adult patients without putative different genetic susceptibilities and drug effects in children.

3: Research Methodology

3.1 Introduction

Diabetes Mellitus Type 1 in the context of pediatric population is researched within the framework of the given paradigm by means of secondary qualitative thematic data analysis within the methodology chapter. This approach involves a review of the secondary data sources that include the peer-reviewed journals, databases and reports to capture and review common patterns related to the genetic factors, treatment and socio-economic consequences. Thus, the presented methodological framework provides a wide coverage of the existing knowledge, and the obtained results are invaluable for further development of the topic and the definition of new research priorities in the field of pediatric Type 1 diabetes management.

3.2 Research method

This research uses secondary qualitative thematic data analysis to assess Diabetes Mellitus Type 1 in pediatric patients with an emphasis on gene therapy. Both the primary and secondary research will also entail a review of major academic periodicals, clinical studies, and medical databases (Tarasiewicz *et al.* 2024). Data analysis will involve qualitative thematic analysis to code data to obtain categories, subcategories, themes, and themes to reveal what is being said regarding the genetic determination of the disorder, the aspects of treatment and the effects of such diseases on societies.

3.3 Research design

Consequently, the study uses cross-sectional descriptive research design to assess Diabetes Mellitus Type 1 in pediatric patients and the possible application of gene therapy. It entails a process of engaging secondary sources of data and these are reviewed and subjected to a qualitative thematic analysis. In this design, a focus on genetic factors and treatment results is facilitated to systematically review the current literature and to determine further research directions and therapeutic development.

3.4 Data collection method and Data sources

Data Collection Method and Sources: As for data collection, this research will mainly use secondary research where the researcher systematically gathers information from existing sources. Such sources as Websites of the ministries and departments, online magazines, books and articles written by noted authors and researchers, peer-reviewed articles, records of hospitals and research institutions (Al Madhoun *et al.* 2024). For statistical data, reports, and policy perspectives, government documents and databases will be reviewed, whereas for research, evidence, perspectives, and outcomes of genetic study, treatments and children with Type 1 diabetes, academic literature will be consulted. This comprehensive approach helps to establish solid data acquisition for the completion of the qualitative thematic analysis.

3.5 Data Analysis

In the method used for thematic analysis, different sets of secondary qualitative data sources that include journals associated with pediatric Type 1 diabetes and gene therapy will be systematically coded for themes and the investigator will interpret these themes. Information collected from published works, research papers, clinical trials and other sources such as medical databases shall be identified, qualitatively coded across the following domains such as genetic susceptibility, treatment effectiveness, and social implication (Maloney *et al.* 2024). By conducting a coding and interpretation process of the data collected the study will seek to find out patterns and meanings that will enhance the determination of the possibilities and the consequences of gene therapy as a therapeutic approach to treatment of Type 1 diabetes in children.

3.6 Ethical considerations

Ethical Considerations: As the research focuses on secondary data, ethical issues include the protection of the identity of pediatric patients' data collected. Protocol of informed accession consent as well as the ethical practices that concern the use of humans in research should be followed (Al Madhoun *et al.* 2024). Moreover, the study will also focus on the issues of the ethics of gene therapy in children, the possible advantages and disadvantages. Professional and ethical conduct in the use of data and results hence in the analysis and presentation of the results shall dictate the conduct of this research.

3.7 Research limitations

One of the possible drawbacks in this type of research is the use of secondary sources that might result in the lack or inadequate quality of the data obtained exclusively for children with Type 1 diabetes. Some differences may have arisen due to the approach used in the data collection part in different studies, different methods used might have created differences in results and interpretation.

4: Data Analysis

4.1 Genetic markers influencing Type 1 diabetes susceptibility in pediatric patients

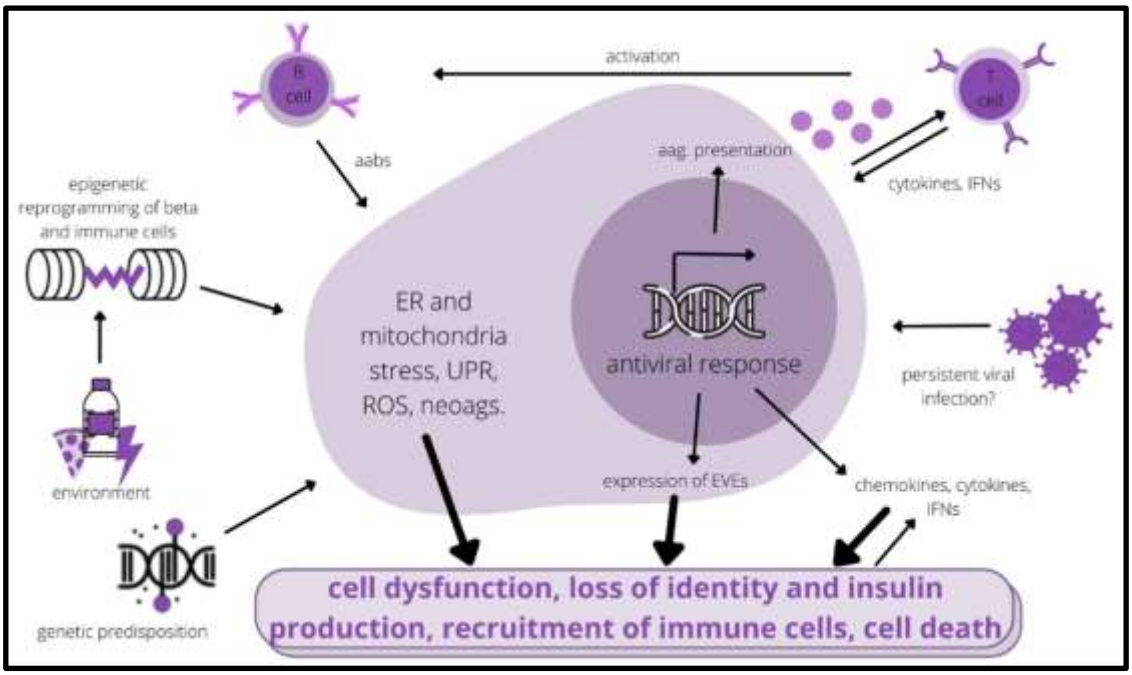

Figure 4.1: Simplified scheme of the pathogenic influences on Beta-cells in T1D

(Source: Moazam et al., 2024)

knowledge of genetic factors to type It diabetes in pediatric patients can guide the management of patients accordingly. Based on the past studies, the body genetic markers including HLA and INS are strong markers that associate individuals with Type 1 diabetes. These markers affect the autoimmune reactions that are aimed at insulin-producing beta cells of the pancreas (Moazam *et al.* 2024).

4.2 Comparative efficacy of gene therapy versus conventional treatments in children

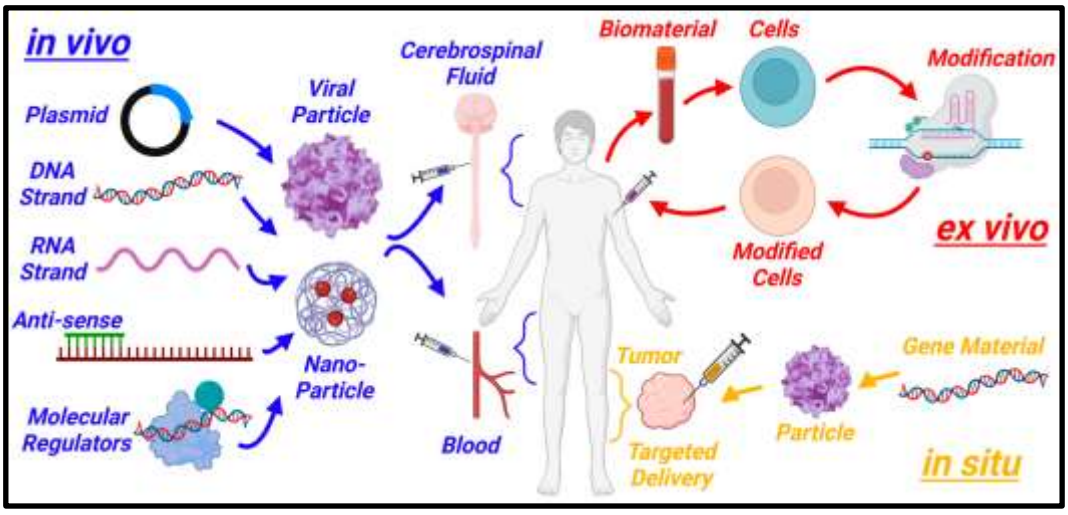

Figure 4.2: Schematic of three gene therapy approaches: in vivo, ex vivo, and in situ. Generated with BioRender

(Source: Andreadi et al., 2024)

Capability assessment of gene therapy in relation to other successful traditional therapies for diabetes including insulin therapy and immunomodulator drugs is very essential in the treatment of pediatric diabetes. Gene therapy intends to correct or replace the genes that have promoted autoimmune degradation of beta cells and can offer other advantages which can include long-term benefits such as good glycemic control and minimal insulin

supplementation. Studies of this type evaluate efficacies, risks and benefits, as well as impacts of the treatments on progression of disease (Andreadi *et al.* 2024).

4.3 Socio-economic factors impacting access to and adoption of gene therapies

Socio-economic characteristics play a very important role on the usage and acquisition of advanced technologies such as gene therapy in pediatric Type 1 diabetes. Some of the factors may include; cost of healthcare, availability of insurance, region, and culture.

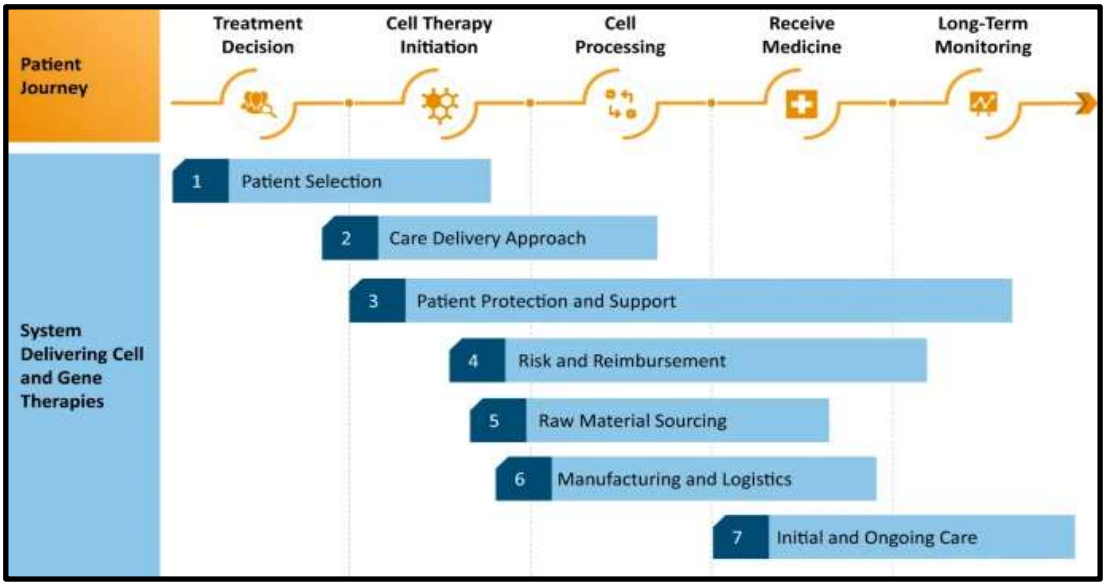

Figure 4.3: System for delivering individualized, autologous, ex vivo, cellular therapies

(Source: Mohammadi *et al.* 2024)

The issue may arise due to the high cost of such drugs or lack of access to quality healthcare of certain patients receiving what may be seen and considered progressive medication. It is only possible when the health care system joins hands with policymakers and the community to help the underprivileged sectors in terms of socio-economic status to avail and incorporate gene therapies successfully (Mohammadi *et al.* 2024).

5: Findings and Discussion

The collected studies provide valuable data concerning Diabetes Mellitus Type 1 in children and the prospects of applying gene therapy in this context. HLA region and INS have been pointed to as important to the onset of autoimmune diabetes that comprises multiple genetic components. These observations, compared with the other traditional treatments such as insulin therapy indicate that gene therapy has the potential in altering the disease process and in decreasing insulin dependence (Wal *et al.* 2024).

6: Conclusion and Recommendations

6.1 Conclusion

In conclusion, the current study's findings reveal that patients appreciate understanding the genetics of Its disease, and genetic counseling provides It with helpful information.

6.2 Recommendations

- Adopt protocols that favor the early identification among pediatric populations that are most likely to be affected.
- Enhance working relationship between research-oriented individuals, clinicians and policy makers to enhance on gene therapy discoveries.
- Formulate heath care policies that will address the issue of the provision of innovations such as gene therapy.

6.3 Future scope

Looking forward, future research should focus on: Looking forward, future research should focus on:

- Safety and effectiveness of the gene therapy in pediatric diabetes: long term studies.
- How socio-economic factors affect the gene therapies access and use and possible measures.
- Investigating the use of resources to perfect the technology needed in applying for a series of therapies for Type 1 diabetes in children.

References

Addissouky, T.A., Ali, M.M., El Sayed, I.E.T. and Wang, Y., 2024. Type 1 diabetes mellitus: retrospect and prospect. Bulletin of the National Research Centre, 48(1), p.42.

Al Madhoun, A., Koti, L., Carrió, N., Atari, M. and Al-Mulla, F., 2024. Clinical Application of Umbilical Cord Mesenchymal Stem Cells Preserves β-cells in Type 1 Diabetes. Stem Cells Translational Medicine, 13(2), pp.101-106.

Al-Rrassam, Z.T., 2024. Correlation analysis of gene expression between children with type 1 diabetes and Coxsackie viruses B. Vacunas, 25(2), pp.193-202.

Andreadi, A., Lodeserto, P., Todaro, F., Meloni, M., Romano, M., Minasi, A., Bellia, A. and Lauro, D., 2024. Nanomedicine in the Treatment of Diabetes. International Journal of Molecular Sciences, 25(13), p.7028.

Bintang, M.I., Asnawi, A. and Agita, W., 2024. RNA therapy for type 1 & 2 diabetes. Science Midwifery, 12(2), pp.944-957.

Daniali, M., Nikfar, S. and Abdollahi, M., 2024. Advancements in pharmacotherapy options for treating diabetes in children and adolescents. Expert Review of Endocrinology & Metabolism, 19(1), pp.37-47.

Goyal, P. and Malviya, R., 2024. Stem Cell Therapy for the Management of Type 1 Diabetes: Advances and Perspectives. Endocrine, Metabolic & Immune Disorders-Drug Targets (Formerly Current Drug Targets-Immune, Endocrine & Metabolic Disorders), 24(5), pp.549-561.

Grasso, E.A. and Chiarelli, F., 2024. Type 1 Diabetes and Other Autoimmune Disorders in Children. Pediatric Diabetes, 2024(1), p.5082064.

Kudratova, Z.E. and Shamsiddinova, D.K., 2024. New insights into the etiopathogenesis of type 1 diabetes mellitus. Western European Journal of Medicine and Medical Science, 2(4), pp.81-84.

Maloney, K.A., Mizerik, E., King, R.H., McGinnis, E.M., Perkowitz, S., Diamonstein, C.J., Schmanski, A.A., Saliganan, S., Shipper, A.G., Udler, M.S. and Guan, Y., 2024. Genetic counseling in diabetes mellitus: A practice resource of the National Society of Genetic Counselors. Journal of Genetic Counseling, 33(3), pp.493-505.

Moazam, M., Khan, S., Quiroga, I., Parra, K., Flores, P., Fatima, E., Amato, A., Tahera, S.I. and Mirza, M.S.S., 2024. Type 1 Diabetes Mellitus: Autoimmune Mechanisms and Treatment. Journal of Advances in Medicine and Medical Research, 36(7), pp.142-151.

Mohammadi, V., Maleki, A.J., Nazari, M., Siahmansouri, A., Moradi, A., Elahi, R. and Esmaeilzadeh, A., 2024. Chimeric antigen receptor (CAR)-based cell therapy for type 1 diabetes mellitus (T1DM); current Progress and future approaches. Stem Cell Reviews and Reports, 20(3), pp.585-600.

Neiva, L.P., Lopez, L.C., Pasiani, R.O., Serra, M.J.R. and Rullo, V.E.V., 2024. Use of probiotics and similar in pediatric patients with Type 1 Diabetes Mellitus: a systematic review. Revista Paulista de Pediatria, 42, p.e2023097.

Tarasiewicz, M., Pietrzykowska, A., Włodarczyk, J., Seget, S., Gadzalska, K., Jakiel, P., Skoczylas, S., Jarosz-Chobot, P. and Borowiec, M., 2024, June. Transient Neonatal Diabetes Mellitus with an Unknown Cause in a 1-Month-Old Infant: A Case Report. In Healthcare (Vol. 12, No. 13, p. 1257). MDPI.

Wal, P., Aziz, N., Prajapati, H., Soni, S. and Wal, A., 2024. Current Landscape of Various Techniques and Methods of Gene Therapy through CRISPR Cas9 along with its Pharmacological and Interventional Therapies in the Treatment of Type 2 Diabetes Mellitus. Current Diabetes Reviews, 20(6), pp.110-127.

Yan, Q., Li, D., Jia, S., Yang, J. and Ma, J., 2024. Novel gene-based therapeutic approaches for the management of hepatic complications in diabetes: Reviewing recent advances. Journal of Diabetes and its Complications, p.108688