



Navigating Existential Terrain: First-Time Patient Perspectives on Gene Therapy for Rare Disorders

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Article Info

Article history:

Received 26-06-2025

Revised 21-07-2025

Accepted 17-08-2025

Keyword:

Gene Therapy; Rare Disorders;
Patient Narratives; Emotional
Landscape; Identity Shift;
Ethical Reflection

ABSTRACT

Gene therapy has ushered in a transformative era in the treatment of rare genetic disorders, offering previously unattainable therapeutic possibilities. Yet, the personal dimensions of undergoing such innovative procedures remain insufficiently understood. This study adopts a descriptive phenomenological methodology to explore the lived experiences of patients receiving gene therapy for the first time. Through in-depth, semi-structured interviews with eight individuals diagnosed with rare disorders, the research delves into how hope, uncertainty, identity, and ethical burden shape the patient journey. Four interrelated themes emerged: hope as a fluctuating life-affirming force, psychological uncertainty as a persistent presence, the body as a transformed and sometimes alienated space, and the weight of being among the earliest recipients of experimental treatment. These findings uncover the emotional and existential complexity of engaging with cutting-edge biomedical interventions and challenge traditional outcome-based paradigms in clinical research. Rather than portraying patients merely as subjects of medical innovation, the study foregrounds their active meaning-making and ethical considerations. Insights gained offer valuable implications for developing patient-centered care frameworks that acknowledge and support emotional resilience and identity transformation. Ultimately, the study contributes to a richer understanding of how patients navigate unfamiliar therapeutic landscapes and underscores the importance of incorporating subjective perspectives into the ethical discourse of emerging medical technologies.



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INTRODUCTION

Gene therapy has emerged as a transformative advancement in molecular medicine, offering new possibilities for the treatment of previously incurable genetic disorders (Carey dkk., 2020; Theil dkk., 2019). As biomedical technologies continue to evolve, therapies that alter the human genome are transitioning from experimental concepts to clinical realities (Springer dkk., 2023). These developments are particularly significant for individuals diagnosed with rare genetic diseases, many of whom face limited treatment options and a progressive decline in quality of life. Within this context, gene therapy represents more than a biomedical intervention; it symbolizes the convergence of science, hope, and existential uncertainty.

Beyond its clinical promise, gene therapy introduces a complex set of psychosocial and ethical dimensions, especially for first-time recipients (Debeer dkk., 2019; Heutink dkk., 2022). Individuals undergoing such therapies often navigate uncharted emotional terrains, balancing optimism with fear, and autonomy with vulnerability. In many cases, these patients find themselves participating in early-phase trials where therapeutic outcomes remain unpredictable and long-term consequences are unknown. The uniqueness of their situation generates profound personal and

cultural meanings that are not easily captured by biomedical metrics or outcome-based evaluations alone.

Given the deeply human implications of undergoing gene therapy for the first time, there is a critical need to explore how individuals interpret and give meaning to their experiences. The subjective dimension of this phenomenon marked by hope, doubt, identity transformation, and ethical self-reflection demands scholarly attention beyond clinical efficacy or technological success. Phenomenology offers a suitable lens through which to investigate these lived experiences, enabling researchers to uncover the essence of what it means to face a radically novel form of therapy in the context of chronic uncertainty (Tian dkk., 2021). As such, this study is situated within a broader effort to integrate the lived experiences of patients into the ethical and humanistic discourse of contemporary biomedical innovation.

Research exploring how individuals experience advanced biomedical interventions such as gene therapy has become increasingly important in the fields of medical humanities, bioethics, and patient-centered care (Alaseri & Alasraj, 2020; Kunisada dkk., 2019). These studies seek to understand not merely the biological or clinical outcomes of treatment, but the personal meanings that individuals construct as they navigate unprecedented therapeutic landscapes. In the case of rare disease patients undergoing first-time gene therapy, such experiences are often imbued with complex emotional, existential, and identity-related dimensions that resist quantification.

However, methodological challenges persist in capturing these nuances. Quantitative studies, while valuable for measuring efficacy and safety, often fall short in articulating the depth and variability of lived experiences (Vandekerckhove dkk., 2019). Surveys and standardized instruments tend to reduce rich emotional and cognitive processes into categorical variables, thereby overlooking how patients interpret their condition, their bodies, and the moral implications of participating in experimental treatment. Even some qualitative studies have adopted overly structured approaches that limit participants' narrative agency, failing to elicit the fullness of their existential concerns.

These limitations have resulted in a knowledge base that, while informative from a clinical perspective, lacks the phenomenological depth required to grasp the essence of what it means to live through gene therapy under conditions of radical uncertainty (Ciolli dkk., 2021; Walker dkk., 2019). The scarcity of research that centers on patients' own words, feelings, and reflections underscores the need for approaches that prioritize subjectivity and meaning. A phenomenological lens, particularly one grounded in descriptive traditions, offers a robust framework to explore these dimensions with the philosophical and methodological rigor necessary for holistic understanding.

In current clinical practice and research surrounding gene therapy for rare diseases, emphasis is primarily placed on biomedical outcomes, therapeutic efficacy, and safety profiles (Aprato dkk., 2019). Standard approaches often rely on measurable indicators such as genetic correction rates, biomarker fluctuations, or survival metrics. While these metrics are essential for clinical validation, they offer limited insight into the subjective world of patients particularly those encountering gene therapy for the first time (Watson dkk., 2019). As a result, prevailing methodologies provide a fragmented picture that marginalizes the patient's inner experience.

Commonly used patient-reported outcome measures and standardized psychological assessments tend to simplify the complex emotional and existential challenges that accompany experimental treatments (Goryń dkk., 2019; Sandoval dkk., 2020). These instruments frequently fail to capture the nuanced interplay between hope, uncertainty, and personal identity that shapes the lived experience of undergoing gene therapy. Consequently, there remains a critical gap in understanding how individuals personally interpret, navigate, and assign meaning to this transformative medical journey.

Addressing this gap requires a methodological shift from objectivist paradigms to frameworks that honor the richness of individual experience (Jovičić dkk., 2021; Williams dkk., 2021). Phenomenology particularly in its descriptive form offers a compelling alternative by centering the

voices of participants and revealing the essence of phenomena as they are lived. Through detailed, reflective inquiry, phenomenological research enables the uncovering of meanings that are often inaccessible through traditional empirical lenses. In the context of first-time gene therapy, such an approach is uniquely suited to illuminate the emotional and existential landscape that defines the patient's journey, thus contributing to a more holistic understanding of therapeutic innovation.

Previous studies have explored the emotional and ethical dimensions of gene therapy, particularly in the context of rare diseases. These investigations have highlighted patients' uncertainty and moral concerns but often lack a deep examination of personal meaning-making. Theoretical discussions have focused on bioethics, patient autonomy, and the future of genomic medicine, with limited emphasis on subjective experience (Zaleta dkk., 2020). Methodologically, many studies have relied on structured interviews or surveys, which may constrain the expression of rich emotional narratives. As a result, the deeper experiential layers of undergoing gene therapy remain insufficiently understood.

This study adopts a descriptive phenomenological approach to address this gap. It focuses on how individuals with rare diseases make sense of their first experience with gene therapy, emphasizing hope, uncertainty, and identity. The method was selected for its ability to capture the essence of lived experiences through open-ended, reflective accounts. By using Colaizzi's analysis framework, the study systematically explores significant meanings shared by participants. This approach responds directly to the need for a more holistic understanding of the patient experience in the face of clinical innovation.

The article is structured as follows. The introduction outlines the background, knowledge gap, and rationale for the study (Chang & Wei, 2021; Reif dkk., 2021). The methods section describes the phenomenological framework, data collection through in-depth interviews, and thematic data analysis. Results are presented through major themes supported by direct participant quotes (Al-Massri dkk., 2020). The discussion connects findings with existing literature and highlights implications for ethical care and policy. Finally, the conclusion summarizes key insights and suggests directions for future phenomenological research in biomedical contexts.

RESEARCH METHODS

Study Design

A descriptive phenomenological approach, rooted in the philosophical tradition of Edmund Husserl, was employed to explore the lived experiences of individuals with rare diseases undergoing gene therapy for the first time (Iosifides, 2011; Longhofer dkk., 2012). This design was selected for its capacity to uncover the essential structure of subjective experiences as they are perceived and interpreted by individuals in their natural contexts (Anand dkk., 2019). Phenomenology focuses on understanding phenomena through the lens of those who have directly encountered them, allowing for deep insights into emotional, psychological, and existential dimensions. In this study, the descriptive approach enabled the elucidation of participants' conscious experiences of hope and uncertainty without the imposition of prior theoretical assumptions or interpretive frameworks.

Participants

Participants consisted of individuals aged between 18 and 50 who had been diagnosed with a rare genetic disorder and had undergone gene therapy for the first time within the past six months (Daly, 2007; Murphy & Dingwall, 2017). Selection was conducted using purposive sampling to ensure that participants had rich, first-hand experiences relevant to the phenomenon under investigation. Inclusion criteria required that participants be mentally and physically capable of engaging in in-depth interviews, have completed at least one round of gene therapy, and be willing to share their experiences openly. Individuals with comorbid psychiatric conditions or who were unable to communicate effectively in the language of data collection were excluded (Dzobo dkk., 2021). The sample included eight participants (5 males, 3 females), with an average age of 34.6 years, representing diverse cultural and socio-economic backgrounds to enrich contextual understanding.

Data Collection

Data were collected through in-depth, semi-structured interviews conducted face-to-face in a private clinical consultation room or the participant's home, depending on their preference. An interview guide was used to facilitate open-ended dialogue, focusing on participants' experiences of hope, uncertainty, bodily perception, and ethical reflections during gene therapy. Each interview lasted between 45 and 75 minutes and was audio-recorded with the participants' consent. Interviews were transcribed verbatim to preserve the authenticity of responses (Gholizadeh-Ghaleh Aziz dkk., 2019). A safe and supportive environment was ensured to promote honest and reflective communication. Field notes were also recorded to capture non-verbal cues and contextual observations. The interview protocol was adapted from validated instruments used in phenomenological health research, with modifications made to suit the specific context of gene therapy.

Data Analysis

Data were analyzed using Colaizzi's method for descriptive phenomenological analysis, which involves a rigorous, step-by-step process to derive essential meanings from participants' narratives (Hartheimer dkk., 2019). The process began with reading each transcript multiple times to achieve immersion, followed by the identification of significant statements related to the phenomenon. Meaning units were then extracted and organized into clusters of themes through thematic reduction. These themes were synthesized into exhaustive descriptions that reflected the essence of the lived experience. Software such as NVivo 14 was used to manage and organize qualitative data but did not dictate thematic development. The final themes were validated by returning them to participants for verification, ensuring fidelity to their intended meanings and reducing interpretive bias.

Ethical Considerations

Ethical approval was obtained from the appropriate institutional review board prior to data collection. Participants provided written informed consent after being thoroughly briefed on the aims, procedures, and potential risks of the study (Clair, 2003; Fenton & Baxter, 2016). Anonymity and confidentiality were maintained by using coded identifiers and securing all data in encrypted files accessible only to the research team. All procedures adhered to the ethical guidelines outlined in the Declaration of Helsinki and followed local ethical standards for research involving human subjects.

RESULTS

Hope as a Negotiated Lifeline

Participants described hope not as a static belief but as a dynamic construct that evolved in response to their deteriorating conditions and exposure to gene therapy. Hope was experienced as an internal strategy to sustain meaning in the face of biomedical uncertainty.

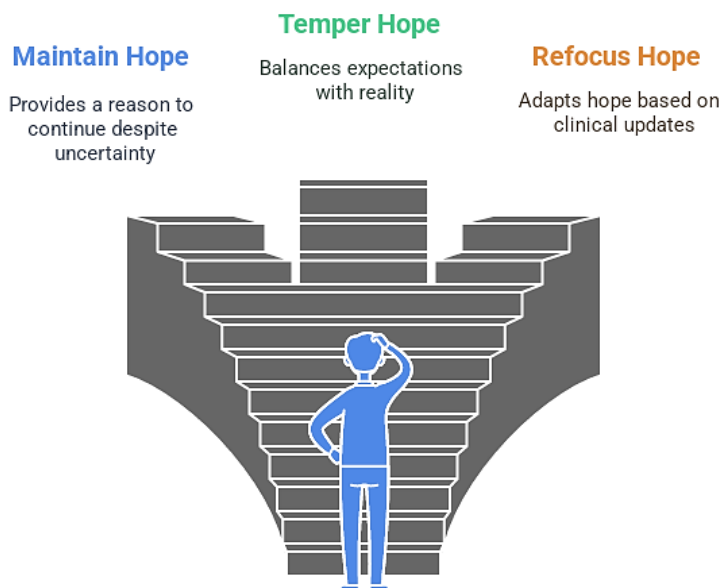
“Every morning I wake up and remind myself that this therapy might not cure me, but it gives me a reason to keep going. It's like holding onto a rope in a storm.” (P4)

This sense of hope was not naïve or overly optimistic but was consciously tempered with caution. Participants negotiated their hope in parallel with clinical updates, often reframing it to maintain emotional balance.

“I don't expect miracles. I just want one more year with my kids, maybe less pain. That's what this treatment means to me.” (P2)

Hope, therefore, served as both a coping mechanism and a framework through which participants interpreted the value of the therapy.

How to manage hope during gene therapy?



Uncertainty as a Daily Psychological Terrain

Participants unanimously conveyed that uncertainty dominated their cognitive and emotional landscape throughout the therapy process. It manifested not only in fear of adverse effects but also in existential ambiguity about identity, bodily changes, and future prospects.

“What terrifies me isn’t the treatment, it’s not knowing what’s next. Am I still me if my genes change?” (P7)

The uncertainty extended beyond scientific unknowns into deeply personal domains. The unpredictability of gene therapy outcomes caused emotional vacillation between hope and despair, stability and fragility.

“Some days I feel like a medical experiment. Other days I believe I’m a pioneer. It’s mentally exhausting.” (P1)

Uncertainty, thus, became an ever-present psychological terrain that participants had to navigate, often without clear guidance or emotional support.

The Body as a Locus of Transformation and Ambivalence

For many, the body was not merely a site of disease but became the subject of profound transformation through therapy. This transformation was accompanied by ambivalence both a sense of renewal and alienation.

“After the second infusion, I started feeling... different. My body doesn’t hurt like before, but I don’t recognize myself fully.” (P6)

Some participants expressed a spiritual reconnection with their physical selves, while others reported a distancing effect, as if their identity was shifting with the biological changes induced by the therapy.

“This new body feels like it doesn’t belong to the old me. It’s like I’m sharing my skin with someone else.” (P3)

These narratives reveal that the body, post-therapy, becomes both a source of hope and a complex terrain of estrangement.

Ethical Tensions and the Burden of Being a ‘First’

Participants conveyed a heavy ethical weight associated with being among the first to receive experimental gene therapy. While some embraced the label of “pioneer,” others perceived themselves as reluctant subjects in an unpredictable biomedical journey.

“I want to help science, but sometimes I wonder if I’m just part of a trial. A data point.”
(P5)

This sense of burden was often coupled with gratitude but complicated by feelings of being “medicalized” or “used.” Participants highlighted a lack of psychosocial infrastructure to support their ethical and emotional concerns.

“No one asks how we feel about being first. They just want to know if the therapy works.”
(P8)

Such reflections point to an unmet need for ethical dialogue and psychosocial care alongside clinical treatment.

The findings illuminate the intricate interplay between hope, uncertainty, bodily transformation, and ethical reflection in the lived experiences of individuals undergoing gene therapy for rare diseases. These themes do not exist in isolation but interact to form a complex emotional and existential reality that extends beyond clinical metrics. The essence of the phenomenon lies in the continuous negotiation of meaning amidst scientific innovation and personal vulnerability.

DISCUSSION

The findings of this study reveal that individuals undergoing gene therapy for rare diseases experience a profound interplay between hope, uncertainty, bodily transformation, and ethical responsibility (Hu & Pan, 2020). These core themes illustrate how patients construct meaning from their participation in a novel and uncertain therapeutic journey, responding to it with complex emotional and existential reflections that extend beyond clinical outcomes. This interpretation directly addresses the central research question: How do individuals with rare diseases make sense of hope and uncertainty when undergoing gene therapy for the first time?

The study contributes to answering this question by offering a nuanced understanding of the lived experience of patients at the intersection of biomedical innovation and personal vulnerability (Magnani dkk., 2020; Mason dkk., 2021). Rather than portraying patients as passive recipients of treatment, the findings highlight their active negotiation of meaning through hope, ambivalence, and moral self-awareness (Lightner dkk., 2023). This interpretation enriches our comprehension of patient-centered care by emphasizing the necessity of acknowledging emotional landscapes and existential reflections in the clinical context. In particular, the recognition of ethical tension and altered bodily perception as part of the therapeutic experience presents a novel insight into how identity is reconfigured through biomedical transformation.

The results align with and expand upon previous qualitative studies in medical ethics and narrative medicine. For instance, Mammana dkk., (2019) described patients’ narratives of uncertainty and decision-making in gene therapy but did not delve into the existential layers of identity and moral responsibility. Similarly Mishra dkk., (2020) identified existential anxiety among trial participants but lacked thematic coherence regarding bodily transformation and ambivalence. The current study complements and deepens this body of work by offering a coherent thematic structure grounded in participants’ own words, interpreted through a phenomenological lens. These findings support the argument by Nasb dkk., (2020) that illness experiences must be understood as disruptions of embodied being and not merely biomedical events. Through this lens, hope becomes not simply a psychological state but an existential orientation, and uncertainty becomes a terrain of ethical and ontological reflection.

The findings of this study carry meaningful implications for both clinical practice and the broader understanding of patient experience in emerging biomedical contexts. From a professional standpoint, healthcare providers must recognize that hope and uncertainty are not merely emotional states but existential processes that shape how patients engage with their treatment. This perspective necessitates a more empathetic, dialogic approach to patient care, where emotional and ethical concerns are integrated into clinical communication. Culturally, the results suggest that perceptions of bodily transformation and medical pioneering are deeply influenced by social narratives and moral

values, indicating the need for context-sensitive patient support systems. At a societal level, the study urges a rethinking of how experimental therapies are introduced and discussed not solely as technological breakthroughs, but as deeply personal and ethical encounters.

Like all qualitative studies, this research is subject to limitations that may influence its transferability to other settings (Adejoh dkk., 2021; Kaye dkk., 2021). The sample size was intentionally small and purposive, aimed at capturing in-depth narratives rather than statistical generalizations. The participants were drawn from a specific demographic and geographic context, which may not reflect the experiences of all individuals undergoing gene therapy globally. Additionally, as the analysis was guided by a descriptive phenomenological approach, the interpretation of meaning was bounded by methodological rigor that may exclude alternative frameworks of understanding. These limitations do not diminish the value of the findings but rather point to the importance of contextualizing their applicability.

Future research may benefit from expanding this inquiry across diverse cultural, clinical, and ethical landscapes. Comparative phenomenological studies across different patient populations could further reveal how hope and uncertainty are constructed in varied sociocultural contexts. Longitudinal designs may also help track how meanings evolve over time as gene therapy transitions from novelty to standard practice. Moreover, integrating phenomenological insights into interdisciplinary frameworks such as bioethics, narrative medicine, and science and technology studies could deepen theoretical engagement with lived experiences of experimental medical technologies. These directions may ultimately enrich patient-centered innovation and contribute to more ethically attuned biomedical practices.

CONCLUSION

This study explored how individuals with rare genetic disorders make sense of hope and uncertainty when undergoing gene therapy for the first time. Using a descriptive phenomenological approach, the findings revealed four central themes: negotiated hope, psychological uncertainty, bodily transformation, and ethical tension. These insights offer a deeper understanding of the lived experiences that conventional clinical research often overlooks. By highlighting the existential and emotional dimensions of gene therapy, the study addresses a critical gap in current literature and contributes to more patient-centered and ethically informed care practices. The findings also underscore the importance of integrating subjective experiences into the development of supportive clinical frameworks. Future research could expand this work across diverse cultural settings or explore longitudinal experiences to understand how meanings evolve as gene therapy becomes more widespread.

CONFLICT OF INTEREST

The authors declare no conflict of interest. This research was conducted independently, and the funder had no role in the design, execution, interpretation, or writing of the study. All views expressed are solely those of the authors.

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