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Authors' Affiliation:
[1]Central Teaching Hospital of The Medical University of Lodz: Łódź, 92-213, ul. Pomorska 251 Poland
[2]University Clinical Hospital No. 1 of the Medical University of Lodz: Łódź, 90-153, ul. Kopcińskiego 22, Poland
[3]Medical Centre of Pabianice, Pabianice, 95-200, ul. Jana Pawła II 68, Poland
[4]5 Military Clinical Hospital in Cracow, 30-901 Cracow, ul. Wrocławska 1-3, Poland

***Corresponding author:**
Monika Krasoń, address: Giedlarowa 1079; 37-300 Leżajsk, Poland, e-mail: monikakrason0901@gmail.com telephone number: +48 884 086 332

ORICIDs:

Monika Krasoń	0009-0000-5571-9440
Julia Witkowska	0009-0008-6106-9665
Ksenia Jakubiak	0009-0003-0291-9942
Krystian Woźniak	0009-0007-1569-8025
Adam Brożyna	0009-0009-1702-9890
Agnieszka Mackiewicz	0009-0009-5275-1088
Aleksandra Pietrucień	0009-0000-5777-3301
Natalia Kursa	0009-0006-4595-3958
Katarzyna Bielak	0009-0001-4572-0509
Mikołaj Kurczyński	0009-0005-6484-6229

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The Future of DMD Therapy: A Systematic Review of CRISPR-Cas9 Innovations and Therapeutic Challenges

Monika Krasoń[1]*, Julia Witkowska[1], Ksenia Jakubiak[1], Krystian Woźniak[1], Adam Brożyna[2], Agnieszka Mackiewicz[3], Aleksandra Pietrucień[3], Natalia Kursa[1], Katarzyna Bielak[4], Mikołaj Kurczyński[1]

ABSTRACT

Duchenne muscular dystrophy (DMD) is a rare progressive neuromuscular disorder caused by an absence of dystrophin that results in generalized muscle weakness and muscle wasting. DMD is inherited in an X-linked manner and results in irreversible loss of muscle tissue and early death. Standard treatment includes corticosteroids and palliative care, which help manage some symptoms without treating the actual genetic defect. CRISPR-Cas9 genome editing is a rapidly advancing area of neuromuscular research, with the potential to permanently fix the underlying genetic cause at the DNA level. In preclinical studies it has been shown that CRISPR technology can restore dystrophin through the use of methods such as exon skipping or frame-restoration, and thus decrease tissue degeneration due to dystrophin deficit. New advances in base and prime editing provide more precise methods for genetic correction and less likelihood of double-strand breaks than some previous-generation "cut-and-paste" gene-editing strategies. Nevertheless, shifting these methods from animal models to human therapies brings significant challenges. Considerations include the immune response to adeno-associated virus (AAV) vectors, the potential for off-target effects, and challenges with delivering the treatment to all muscle groups, most notably the heart joint and diaphragm. New clinical research, including the EMBARK clinical trial results, shows how difficult it is for patients to achieve long-lasting benefits. Despite these challenges, continued improvement in the accuracy of gene editing presents some hope that DMD will have an opportunity to be transitioned from a symptom management to a cure for the disease if current safety and delivery challenges can be overcome.

Keywords: Duchenne muscular dystrophy, CRISPR Cas9 genome editing, dystrophin restoration

1. INTRODUCTION

Duchenne muscular dystrophy is a severe genetic disorder. It predominantly affects males due to its X-linked inheritance. Mutations in the DMD gene lead to a

complete lack or a shortened version of dystrophin. The main function of this protein is to provide structural and mechanical integrity to the sarcolemma during muscle contraction and relaxation (Erkut and Yokota, 2022; Haque and Yokota, 2025). The first signs generally arise in early childhood. The most common symptoms at this age are frequent falls and the use of hands to help get back up. Most patients lose the ability to walk between the ages of 11 and 14. It will lead to eventual respiratory failure and heart disease, which are the two most common causes of early deaths in patients with DMD (Chen et al., 2022; Haque and Yokota, 2025). According to epidemiological studies, 1 in every 3,500 to 5,000 individuals born worldwide is affected by Duchenne muscular dystrophy. It represents one of the highest rates of occurrence among fatal genetic neurodegenerative diseases (Ali et al., 2024; Arudkumar et al., 2025). While the disease is not very common among the general population, it creates a huge clinical burden since many of these patients will require ongoing care from numerous specialists throughout their lifetime and will eventually lose their ability to walk (Haque and Yokota 2025).

Dystrophin is an essential structural protein that connects the muscle fiber's internal cytoskeleton to the extracellular matrix. Without dystrophin, muscle fibers would weaken and get damaged every time they contract. This would lead to ongoing cycles of injury that the body cannot fully fix. Over time, fatty and fibrous tissue (Erkut and Yokota, 2022; Wang et al., 2025) replace muscle fibers as part of Duchenne's muscular dystrophy pathology. Currently, pharmacological treatments for DMD are limited to managing symptoms and preventing further deterioration. Corticosteroids like prednisone and deflazacort can slow the rate of clinical progression. However, these interventions address neither the genetic defect that ultimately leads to muscular dystrophy nor the lack of dystrophin. Patients require physiotherapy, respiratory care and cardiac management. Therefore, recent advances in gene-editing strategies have become increasingly attractive to clinicians. Of those techniques, CRISPR-Cas9 — which enables the alteration of specific areas of DNA and, at the same time, promotes the expression of dystrophin — appears to have the greatest utility (Haque and Yokota, 2025; Ali et al., 2024). The purpose of this review is to provide an update on recent advances in CRISPR-based therapies for treating patients with DMD and on their future clinical applications.

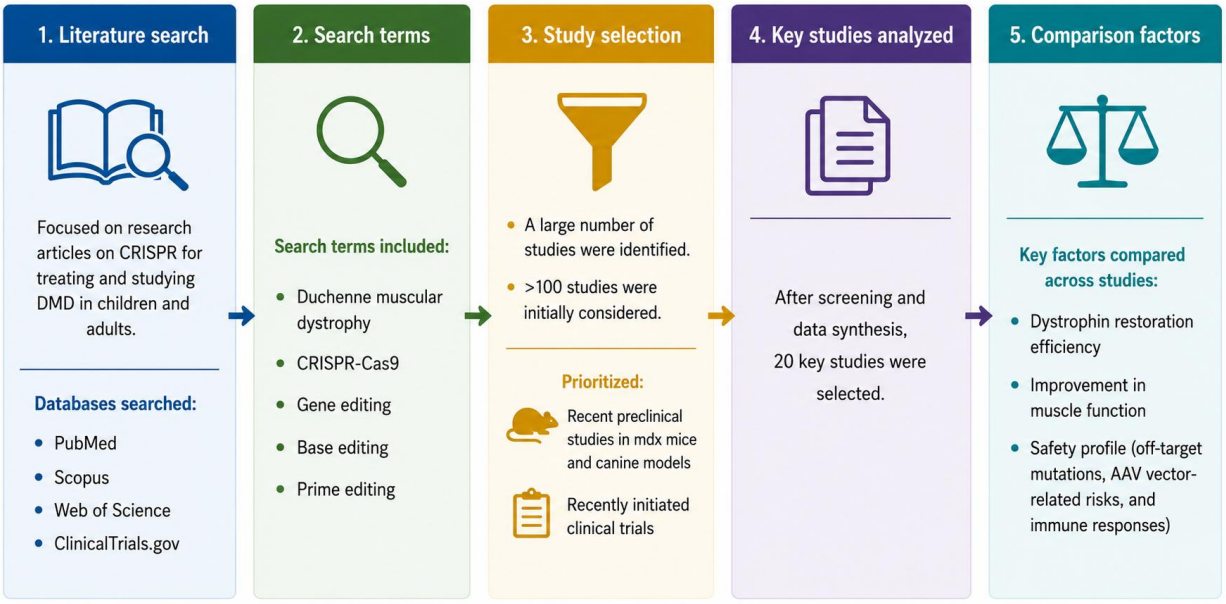

Figure 1. Flowchart of the literature selection process.

2. REVIEW METHODS

To evaluate how far CRISPR has advanced and whether it can be applied as a treatment for Duchenne muscular dystrophy, an extensive literature review from 2016 to 2025 was conducted, focusing on research articles on CRISPR for treating/researching DMD in children/adults. Research articles were found across multiple medical databases, including PubMed, Scopus, Web of Science and ClinicalTrials.gov. By using search terms including Duchenne muscular dystrophy, CRISPR-Cas9, and gene editing, as well as newer technologies such as base and prime editing, we were able to collect a very large number of studies published from 2016 onward.

Studies eligible for preclinical research in MDX mice or canines, as well as those recently initiated in clinical trials, were prioritized over older or less current studies. Over 100 studies were initially considered. After synthesizing the 20 key studies (Figure 1), we compared many different factors, including: how efficiently the CRISPR therapy restored dystrophin, how much improvement was noted in terms of muscle function thereafter and the safety profile of each study (for example, the risk for off-target mutations due to AAV vector usage and/or immune responses due to AAV vector usage). The compiled data show that the long-term safety profile and efficacy of CRISPR technology for DMD remain limited.

3. RESULTS AND DISCUSSION

Molecular Basis of DMD

DMD stems from mutations in the DMD gene, which is recognized as one of the longest genes in humans. The DMD gene encodes dystrophin, which is located on the X chromosome. Due to providing protection against damage to muscle cell membranes throughout contraction and relaxation, this protein is functioning as a molecular cushion (Chemello et al., 2023; Duan, 2018). The DMD gene contains 79 exons that are easily impacted by deletions, duplications and point mutations; however, most cases have been attributed to large deletions that interfere with the reading frame. The disruption causes a premature stop codon which inhibits the formation of dystrophin that functions (Arudkumar et al., 2025). Dystrophin is located on the inner surface of muscle membranes and connects the muscle cytoskeletal system to the host extracellular matrix. This creates the link between muscle membranes and the muscle itself so that muscle contraction will not be damaged by the contraction of muscle (Angulski et al., 2023). Without dystrophin, the muscle membrane loses structural integrity, becomes unstable and is susceptible to damage with any routine activity. This ongoing injury/insufficient repair cycle leads to chronic inflammation. Chronic degeneration of muscle fibers results in gradual replacement with fibro-fatty tissue and subsequently results in loss of significant, irreversible muscle strength (Angulski et al., 2023; Duan, 2018). This inability to sustain muscle health begins with deterioration of skeletal muscle but does not stop there since cardiac muscle and respiratory muscle are also affected. Poor health results from this decline in health for patients. Therefore, it is necessary to understand at the molecular level how this phenomenon occurs if we want to design effective new therapeutic options. A great target for finding new therapies utilizing genetics for treatment is Dystrophinopathy (DMD); as a monogenic disease, DMD allows researchers to explore genetic-based therapies such as: 1) exon skipping (i.e., removing portions of the DMD gene), 2) micro-dystrophin replacement (i.e., adding an abbreviated DMD gene), and 3) gene editing by CRISPR-Cas9 technology (Chemello et al., 2023; Duan, 2018).

CRISPR-Cas9 Technology in DMD

Through the use of CRISPR-Cas9, a highly precise gene-editing system, targeted modifications can be introduced into genomic DNA. The process of making these changes involves using a guide RNA (gRNA) that takes the enzyme (Cas9) directly to the specific location in the target double-stranded DNA, and once located, the enzyme cleaves the double-stranded DNA at the targeted locations. Cells will begin repairing the cut using their normal pathways, most often they will use either NHEJ or HDR. These two pathways can be used to correct pathogenic mutations (Chemello et al., 2023).

In the case of DMD research, CRISPR/Cas9 platforms are already being used to restore the reading frame of the dystrophin gene allowing a truncated but functional dystrophin protein to be synthesized. Also, by using the technique called exon skipping, specific exons can be bypassed to realign the reading frame of the gene. Through this technique, DMD could be converted from a severe form of the disease into a much less severe form similar to Becker muscular dystrophy (Chemello et al., 2023). Another option is to utilize methods for the restoration of a reading frame (the mutation is deleted or altered) via CRISPR as a way to create a functional dystrophin. Studies using mdx mice showed that restoring dystrophin expression was effective at improving muscle dysfunction (Arudkumar et al., 2025).

Another approach for improving accuracy uses gene knock-in techniques and relies on donor sequences from donor cells provided as a functionally equivalent external chromosomal area. However, the existence of non-dividing cells, such as muscle or fibers, creates significant barriers to the effective delivery of clinical products (Haque & Yokota, 2025). Fortunately, new technologies developed for genome editing have greatly improved these methods. Base editing now allows for a nucleotide to be converted without creating double-strand breaks, which reduces the likelihood of introducing unintentional mutations and offers the safest means of making gene changes. In addition to the improved precision of base editing, prime editing enables the precise insertion and removal of genetic sequences, as well as the correction of single-stranded DNA templates at specific genomic locations (Duan, 2018; Chemello et al., 2023).

Table 1 provides a summary of the molecular mechanisms, clinical targets, and clinical limitations associated with each of these technologies. AAV-mediated gene replacement therapy has lower efficacy than that of CRISPR-based gene editing. However, AAV delivery can result in a functional micro-dystrophin but does not correct the underlying genetic mutation. In contrast, CRISPR can provide a permanent correction of the genetic mutation at the DNA level. While CRISPR can correct genetic defects, there are still many barriers to its eventual use in the clinic such as; Method of Delivery, Off-Target Effects and Immune Response (Haque & Yokota, 2025). In conclusion, advanced genome-editing technologies offer a promising paradigm for treating DMD, though their transition into clinical practice is still in its infancy.

Table 1. Mechanisms, therapeutic strategies, and clinical barriers of CRISPR-based platforms in DMD.

Technology / Approach	Molecular Mechanism	Therapeutic Outcome in DMD	Key Limitations and Constraints
Standard CRISPR-Cas9	• gRNA-directed Cas9 cleavage • Induces double-strand breaks (DSBs) • Activates error-prone NHEJ or HDR	• Restores the translational reading frame • Enables production of truncated, functional dystrophin	• Risk of off-target mutations • Lower efficiency in post-mitotic muscle fibers
Exon Skipping	• CRISPR-mediated deletion of specific mutated exons • Bypasses frame-disrupting mutations to realign the reading frame	• Converts severe DMD phenotype to a milder Becker-like variant	• Requires permanent vector-mediated delivery • Produces a shortened, non-wild-type protein
Frame Restoration	• Targeted correction of frame-disrupting mutations	• Re-establishes endogenous production of functional dystrophin	• Efficacy varies depending on the specific patient mutation site
Gene Knock-In	• HDR-mediated insertion of a functional exogenous donor sequence at the mutated locus	• Corrects the genetic defect at the target site	• Exceptionally low efficiency in non-dividing muscle cells.
Base Editing	• Direct single-nucleotide transition without inducing DSBs	• Corrects nonsense mutations to restore dystrophin expression	• Restricted solely to transition mutations (e.g., C-to-T or A-to-G)
Prime Editing	• Guide-templated reverse transcription without causing DSBs	• Corrects diverse structural mutations (insertions, deletions, transversions)	• Large size complicates packaging into standard AAV vectors

Preclinical Studies

Preclinical studies have proven the efficacy of CRISPR-based therapies for DMD. The majority of available data comes from animal models, particularly mdx mice and canine models, which effectively replicate the main features of the human disease (Arudkumar et al., 2025; Haque and Yokota, 2025). The mouse model used is mdx for DMD. This individual has a mutation in his DMD gene resulting in no functional dystrophin being produced by his body. Scientists were able to use CRISPR/Cas9 to either repair or eliminate faulty exons within the genomes of the experimental animals and thereby correct the reading frame of the DMD gene. Preclinical evidence

indicates that both systemic and localized delivery of CRISPR/Cas9 to skeletal muscle has resulted in a partial restoration of dystrophin expression (Chemello et al., 2023), which is important because only a small amount of dystrophin will still produce a substantial increase in stability of the skeletal muscle cell membrane.

Beyond mdx mice, larger animal models with dystrophic muscle damage (e.g., golden retriever muscular dystrophy model) have been used to more closely mimic human disease progression. This is especially important regarding muscle size, immune responses, and disease severity, as these models are closer to humans. In canine models, genome editing successfully rescues partial dystrophin expression, leading to marked improvements in muscle histology (Haque and Yokota, 2025). Functional results are part of the preclinical assessment. There is an increase in muscle force, injury resistance, and general mobility in the treated animals. Histological studies have also revealed decreased muscle fiber degeneration and reduced fibrosis in some cases.

That said, functional rehabilitation will depend on several factors including: The manner in which therapeutic agents are delivered, the type of tissue to be repaired, and the efficacy of gene editing procedures (Arudkumar et al., 2025). While many advances have been made, there are still significant translational barriers. For example, it is often difficult to deliver CRISPR-based therapeutics specifically to the target muscles. For instance, CRISPR-based products may never reach the heart, diaphragm or other critical muscle tissues due to issues relating to safe delivery. Most studies utilize adeno-associated viral (AAV) vectors, which have a restricted capacity for packaged therapeutic agents and may activate complex immune reactions. For example, there may be off-target consequences associated with the use of AAVs and unintended disease-related consequences due to a response by the host immune system that are caused by genome editing (Haque and Yokota, 2025).

Moreover, discrepancies between animal models and human patients limit the direct translation of laboratory results into clinical outcomes. On the other hand, preclinical studies confirm the effectiveness of CRISPR-Cas9 by demonstrating its ability to restore dystrophin expression and muscle function. Optimization remains essential for a variety of medical purposes.

Applications and Clinical Trials

Clinical translation of CRISPR-Cas9 for DMD treatment is currently in its infancy. Most of the evidence comes from preclinical studies or a few early-phase clinical trials listed on ClinicalTrials.gov. Although this review relies on preclinical studies, clinical trials are shifting toward genome editing to correct or bypass the loss of function caused by DMD mutations. Some studies may find early evidence of the exon excision approach, but more sophisticated editing systems, including next-generation CRISPR platforms, are also under study. Curiously, the most recent gene-based treatment for DMD is not CRISPR but adeno-associated virus (AAV)-based micro-dystrophin gene replacement. This approach delivers a shortened but functional dystrophin gene to muscle cells. Several regulatory authorities have approved AAV-based gene therapy. For example, delandistrogene moxeparvovec has received formal licensure because of improved measures of dystrophin expression obtained in clinical trials.

Ongoing studies are evaluating the durability of the functional outcomes produced by these therapies (Mendell et al., 2024). CRISPR-Cas9 offers a conceptual advantage over AAV gene therapy if, in addition to the above, AAV gene therapy is not inclusive. Instead of injecting an external version of the gene into someone else's genome, CRISPR edits the patient's own DNA and might, if implemented, be a permanent fix. Yet, this also introduces new safety concerns — off-target mutations, immune reactions to the Cas9 protein, and genomic instability over time (Chemello et al., 2023; Haque and Yokota, 2025). Now, AAV-based therapeutics are already being reported clinically more advanced than CRISPR-based therapies, which are still in the experimental stage. AAV can make selective dystrophin, though it does not fully target the mutation. CRISPR offers the potential for a single-use curative treatment. However, effective and safe targeted delivery to skeletal, and cardiac muscle remains a significant challenge (Duan, 2018; Haque and Yokota, 2025). Earlier clinical data indicate that gene therapy is still an emerging treatment for DMD. While we expect to gain further clinical insights, CRISPR-based therapies require additional optimization before they can be applied in clinical practice.

Challenges and Limitations

While the preclinical data is promising, one of the major hurdles for using CRISPR-Cas9 as a therapy for DMD is delivering the entire product specifically to the tissues requiring treatment. Currently, the most common method of delivering genes using AAV (adeno-associated virus), is to deliver genes using AAV. The main challenge was the amount of space available in AAV for delivering larger or more complex CRISPR components (Duan, 2018; Haque and Yokota, 2025). Several components may be needed to deliver a CRISPR component effectively, and AAV may not be able to accommodate multiple components. The potential for the development of humoral immune responses against previously administered AAV vectors also poses problems for efficacy (Chemello et al., 2023). Off-target

effects are another serious barrier regarding the safety of this type of therapy using CRISPR-Cas9. Occasionally, CRISPR-Cas9 causes a genomic change at an unintended site during the course of treatment. Potentially harmful off-target therapies have a much higher long-term risk of causing genetic mutations than traditional treatment methods, and this is particularly worrisome for children who need regular clinical evaluations throughout their lives (Haque and Yokota, 2025). The durability of the effects of off-target therapies can also be difficult to determine. And although dystrophin expression should ultimately return, the duration of this functional period in human muscle remains unknown. Muscle turnover, immune clearance of edited cells, and reduced editing efficiency will all reduce long-term benefits (Duan, 2018). Finally, gene and genome editing therapies have an extremely high price tag. Development and clinical applications require advanced technology and complicated manufacturing processes. The above-mentioned barriers create challenges for patients accessing care and add to the burden of maintaining healthcare systems long-term, particularly with increasing global consensus around clinical consensus (Chemello et al. 2023). While there is significant promise in the advancement of CRISPR gene therapy as a treatment for DMD, before this technology can be adopted widely and adopted into mainstream practice, it must overcome challenges regarding delivery method and safety that currently remain unresolved.

Future Perspectives

It is very important to note that future therapies for DMD are currently targeting a new frontier in genome editing to enhance accuracy and safety. Current research focuses heavily on novel gene-editing platforms, such as base and prime editing. These new types of technologies will bypass double-stranded DNA breaks, thus reducing the likelihood of off-target mutations relative to present-day CRISPR-Cas9 systems. In addition, much of the research currently being conducted has started to focus on creating therapies tailored to each individual patient. Given that many mutations in the DMD gene can cause DMD, researchers will likely need to design specific therapies for each unique genotype and patient. Thus, clinicians will need to adapt interventions to each individual's specific phenotype and the genetic mutation that caused the disorder. As sequencing technology becomes more widespread, it will be easier to design targeted therapies and improve treatment efficacy. A core long-term target in this area will be to develop a genuine causal therapy. Unlike conventional treatments, which merely delay the progression of a developing disease, genome manipulation could be a direct antidote to the underlying genetic defect. Theoretically, this may result in possible chronic or even permanent restoration of dystrophin expression after a single treatment. Early intervention is also considered an important factor for future success. Early-stage drug administration, before irreversible muscle wasting takes place, could enhance treatment outcomes. To be effective in treating DMD, programs must include safe deliveries for infants, as well as genetic testing of individuals prior to birth. Currently there are programs to develop treatments to DMD using CRISPR/Cas9 or CRISPR-Cas9 based strategies which offer a potential way to cure DMD through very targeted and precise methods.

4. CONCLUSION

Duchenne Muscular Dystrophy (DMD) is an inherited muscle disease that is degenerative in nature affecting patients and their families dramatically. There are many commonly used treatments for DMD, such as steroids and various types of additional therapies, that have been shown to have a positive effect on delaying the progression of the disease. However, these therapies cannot address the underlying cause of the condition—the abnormal gene. The attention of the research on SMS has moved from supportive care to be able to provide therapies that are targetted at specific patients, such as gene therapy or gene editing, all methods to correct the primary genetic cause of SMS. Preclinical data has demonstrated the ability of CRISPR-Cas9 to restore dystrophin protein expression and improve motor function in animal models in vivo. Although these treatment strategies are promising, few clinical studies have used them so far, and most are limited to Phase I trials in humans. Lack of gene therapy delivery efficiency, potential adverse side effects, and the presence of off-target effects are obstacles to the broad application of genome-modifying therapies for the treatment of individuals with DMD. Based on data collected to date, it is unclear when CRISPR will become an established treatment. However, it is one of the most likely, effective long-term therapies in the search for a cure for DMD. If these barriers are resolved, genome editing could ultimately change the treatment for DMD from a symptomatic to a modifying, then a curative approach.

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Authors' Contributions

Conceptualization: Monika Krasoń, Julia Witkowska
Methodology: Krystian Woźniak, Katarzyna Bielak.
Formal Analysis: Mikołaj Kurczyński, Adam Brożyna.
Sources: Agnieszka Mackiewicz, Aleksandra Pietrucień.
Visualization: Natalia Kursa, Monika Krasoń.
Investigation: Aleksandra Pietrucień, Ksenia Jakubiak.
Supervision: Krystian Woźniak, Adam Brożyna.
Validation: Julia Witkowska, Ksenia Jakubiak.
Writing – Review and Editing: Natalia Kursa, Mikołaj Kurczyński
Project administration: Monika Krasoń, Katarzyna Bielak
All authors have read and agreed with the published version of the manuscript.

Informed consent

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Ethical approval

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Conflict of interest

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Data and materials availability

All data associated with this study will be available based on the reasonable request to corresponding author.

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