

Judicialization of delandistrogene moxeparvovec in Brazil: a cross- sectional study on clinical eligibility, litigation patterns, and public expenditure

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Research Article

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Abstract

Background

Gene therapies have expanded treatment options for rare diseases while creating governance challenges for publicly funded health systems because of high prices, limited long-term evidence, and growing judicial demand for access. In Brazil, litigation has become a common route to technologies not incorporated into the Unified Health System (SUS). Delandistrogene moxeparvovec, a gene therapy for Duchenne muscular dystrophy (DMD), obtained exceptional marketing authorisation from the Brazilian Health Regulatory Agency but subsequently received an unfavourable preliminary recommendation against incorporation into the SUS.

Methods

We conducted a cross-sectional, descriptive, census-based study of administrative data obtained under Brazil's Access to Information Law. All individual lawsuits filed against the Ministry of Health between January 2024 and February 2026 seeking delandistrogene moxeparvovec were included. Variables were age, sex, ambulatory status, legal representation, procedural status, modality of judicial compliance, public expenditure, and label-based eligibility. We used descriptive statistics and projected budget impact from unit costs observed in fully complied court orders.

Results

We identified 223 individual lawsuits, all involving male patients. Only 16.14% ($n = 36$) met both label-based criteria (age 4–7 years and preserved ambulation). Almost half (48.88%; $n = 109$) failed at least one criterion, and 34.98% ($n = 78$) were within the approved age range but had no documented ambulatory status; ambulation was unreported in 67.71% of cases. Private attorneys filed 96.86% of lawsuits. Fifteen court orders were fully complied with, at a public cost of R\$ 194.3 million; only three involved fully eligible patients, six involved patients outside approved criteria, and six lacked information to confirm eligibility. Full compliance with all lawsuits was projected to cost R\$ 2.89 billion (average-cost scenario) to R\$ 4.13 billion (maximum-cost scenario).

Conclusions

Judicialization of delandistrogene moxeparvovec in Brazil exposes major governance challenges for high-cost gene therapies in publicly funded health systems. Litigation may extend access beyond approved regulatory criteria and commit substantial public resources under clinical uncertainty, while still failing to secure timely access for patients who meet formal eligibility standards. Strengthening technical review, requiring minimum clinical documentation, and developing outcome-based access models may help balance innovation, patient safety, and fiscal sustainability.

Background

Health litigation in Brazil, the use of judicial decisions to secure access to medicines, diagnostic tests, and treatments not administratively provided by the Unified Health System (Sistema Único de Saúde, SUS), has become a major challenge for public health governance over recent decades [1]. Although litigation may serve as a legitimate mechanism to enforce the constitutional right to health, its expansion into disputes involving high-cost and highly complex technologies has raised concerns about equity, financial sustainability, and the quality of public resource-allocation decisions. Court orders addressing individual claims may redirect scarce resources away from collective health policies towards treatments still marked by uncertainty regarding efficacy, safety, or cost-effectiveness. This process may impose substantial opportunity costs on the public health system [2–4].

These concerns are particularly acute in gene therapy and other advanced therapies, which combine significant therapeutic promise with exceptionally high prices and limited clinical evidence. In many cases, evidence derives from small patient samples, surrogate or intermediate endpoints, and short follow-up periods. In Brazil, incorporation of new technologies into the SUS depends on a multistage institutional process that includes marketing authorisation by the Brazilian Health Regulatory Agency (Agência Nacional de Vigilância Sanitária, Anvisa), price regulation by the Drug Market Regulation Chamber, and health technology

assessment by the National Committee for Health Technology Incorporation into the SUS (Comissão Nacional de Incorporação de Tecnologias no SUS, Conitec), established under Law No. 12,401/2011. Yet delays between regulatory approval, Conitec assessment, and effective availability within the SUS may create coverage gaps that encourage litigation, especially in cases involving rare, severe, and progressive diseases.

Duchenne muscular dystrophy (DMD) is among the most severe paediatric neuromuscular disorders. The disease causes progressive muscle degeneration, loss of ambulation during adolescence, and premature death, usually in the third decade of life [5]. DMD is a rare X-linked recessive genetic disorder caused by mutations in the DMD gene, which lead to the absence or dysfunction of dystrophin, a protein essential for maintaining the structural integrity of muscle fibres during contraction and relaxation [6]. Historically, treatment has relied on supportive care and corticosteroids such as prednisone and deflazacort. These therapies may delay disease progression and prolong ambulation, but they do not fundamentally alter the disease course [7].

Against this backdrop of substantial unmet medical need, gene therapies based on adeno-associated viral (AAV) vectors have emerged as a promising strategy to modify disease progression. Delandistrogene moxeparvovec, uses the AAVrh74 vector to induce expression of micro-dystrophin, a shortened dystrophin protein designed to overcome the packaging limitations of viral vectors [8]. Although it represents an important biomedical innovation, the supporting clinical evidence remains controversial. The pivotal phase 3 EMBARK trial failed to meet its primary efficacy endpoint, and uncertainty persists regarding whether increased micro-dystrophin expression translates into clinically meaningful functional improvement [9–12].

International regulatory divergence has further intensified uncertainty surrounding Delandistrogene moxeparvovec. In the United States, the Food and Drug Administration (FDA) granted accelerated approval in 2023, followed by traditional approval in 2024. The decision generated substantial controversy within the scientific community because the technical reviewers responsible for the evaluation had recommended against approval [11, 13]. By contrast, the European Medicines Agency (EMA) refused marketing authorisation in July 2025, concluding that the EMBARK trial failed to demonstrate functional benefit after 12 months and that evidence linking micro-dystrophin expression to clinically relevant outcomes remained insufficiently convincing [10]. Safety concerns also became more prominent after a July 2025 FDA safety communication reported deaths associated with severe acute liver failure in patients treated with the therapy [14].

In Brazil, delandistrogene moxeparvovec received exceptional marketing authorisation from Anvisa in December 2024 under Collegiate Board Resolution No. 505/2021, based on the severity of DMD and the absence of curative therapeutic alternatives [15]. However, regulatory approval does not guarantee incorporation into the SUS. In April 2025, Conitec issued a preliminary recommendation against incorporating the therapy into the public health system, citing insufficient clinical evidence, unresolved safety and cost-effectiveness uncertainties, and the exceptionally high treatment cost, estimated at R\$ 16 million to R\$ 18 million per patient [16]. Nevertheless, lawsuits seeking public funding for Delandistrogene moxeparvovec rapidly emerged, including claims involving patients who may not satisfy the eligibility criteria established in the approved product label.

The judicialization of this therapy offers a valuable case study for examining how publicly funded health systems respond to demands for access to high-cost technologies under conditions of scientific uncertainty, regulatory disagreement, and clinical urgency. In Brazil, the debate also intersects with Theme 1234 of the Federal Supreme Court, which established criteria for court-ordered provision of medicines not incorporated into the SUS, including marketing authorisation, prior Conitec assessment, and adequate clinical justification [17]. Yet evidence remains limited regarding the clinical and legal profile of patients seeking access to Delandistrogene moxeparvovec through litigation, the extent to which these claims comply with regulatory requirements, and their potential budgetary impact on the public health system.

This study therefore examines the clinical, demographic, and legal characteristics of lawsuits filed against the Brazilian federal government seeking access to delandistrogene moxeparvovec, and estimates the public expenditure associated with compliance with court orders. Using data obtained through Brazil's Access to Information Law, we analysed variables related to patient age, ambulatory status, legal representation, procedural stage, modality of judicial compliance, and amounts disbursed by the Ministry of Health. In addition, we evaluated whether the cases aligned with the eligibility criteria established in the nationally approved product label and with the standards defined under Theme 1234 of the Federal Supreme Court.

Methods

Study design and setting

This cross-sectional, descriptive, census-based study used administrative data from individual lawsuits filed against the Brazilian federal government, specifically the Ministry of Health, seeking access to delandistrogene moxeparvovec for the treatment of DMD. A census-based approach enabled inclusion of all eligible cases identified in the governmental database during the study period and allowed comprehensive characterisation of federal-level litigation involving this therapy.

The analysis included lawsuits filed between January 2024 and February 2026. This period captures the earliest phase of litigation related to delandistrogene moxeparvovec in Brazil, following international regulatory developments and the granting of national marketing authorisation.

Data source

Data were obtained through a formal request submitted under Brazil's Access to Information Law via the Fala.BR platform on 17 March 2026 (protocol no. 25072.014824/2026-91). The request was addressed to the Department for the Management of Health Litigation Claims (Departamento de Gestão das Demandas em Judicialização na Saúde, DJUD/SE/MS), within the Executive Secretariat of the Ministry of Health, which monitors and manages federal lawsuits related to pharmaceutical assistance.

On 16 April 2026, the Ministry of Health provided a spreadsheet containing anonymised administrative records of lawsuits involving delandistrogene moxeparvovec. The dataset included demographic, clinical, procedural, legal, and financial information extracted from records available to the public authority.

Study population

The study population included all individual lawsuits recorded in the Ministry of Health database involving patients diagnosed with DMD who sought access to delandistrogene moxeparvovec through the Brazilian federal government. The unit of analysis was the individual lawsuit, corresponding to each claimant patient.

All eligible cases identified between January 2024 and February 2026 were included. No restrictions were applied regarding age, sex, clinical status, type of legal representation, or procedural stage. The analysis excluded collective actions, lawsuits unrelated to DMD, and claims involving technologies other than delandistrogene moxeparvovec.

Eligibility criteria

Patient eligibility was assessed according to the criteria established in the Brazilian approved product label for delandistrogene moxeparvovec. Patients aged 4 to 7 years were classified as eligible under the age criterion, consistent with the current national regulatory approval. Patients younger than 4 years or older than 7 years were classified as age-ineligible.

Eligibility according to ambulatory status was assessed separately. Patients recorded as ambulatory were considered eligible under this criterion, whereas non-ambulatory patients were classified as ineligible. Individuals described as "ambulatory with difficulty" remained in a separate category and were not grouped with fully ambulatory patients. This approach preserved the clinical granularity of the original records and avoided unsupported inferences from the administrative dataset.

Cases with missing information on ambulatory status were classified as "ambulatory status not reported". These cases were analysed independently, without assumptions regarding eligibility. Combined eligibility required fulfilment of both principal criteria: age between 4 and 7 years and preserved ambulation. Only cases meeting both conditions were considered fully aligned with the approved product label.

Variables

The study analysed the following variables: patient age, sex, ambulatory status, type of legal representation, procedural status, modality of judicial compliance, amounts disbursed by the Ministry of Health, and eligibility according to the product label approved in Brazil.

Legal representation was categorised as either private counsel or the Federal Public Defender's Office. Procedural status was classified according to the categories used by DJUD/SE/MS: full compliance, suspended compliance, technical-subsidy phase, expert assessment, expert assessment with technical subsidies, and denial of the request for a preliminary injunction. The modality of judicial compliance was classified as either direct purchase by the Ministry of Health or judicial deposit, based on the administrative record available in the dataset.

Cost analysis

The economic analysis used expenditure data reported by the Ministry of Health in the administrative dataset obtained through Brazil's Access to Information Law. All amounts were analysed in current Brazilian reais (R\$), as recorded in the official database, without inflation adjustment.

The analysis included disbursements associated with the enforcement of judicial decisions, including direct purchases by the Ministry of Health and judicial deposits intended to secure access to the medicine. The mean cost per patient was calculated using amounts effectively disbursed in cases with full judicial compliance.

To estimate the potential financial impact of litigation, sensitivity analyses were conducted using the unit values observed in completed purchases and judicial deposits. Three scenarios were constructed: (1) a minimum estimate based on the lowest recorded unit value; (2) an average estimate based on the mean amount effectively disbursed; and (3) a maximum estimate based on the highest recorded unit value. These scenarios were then applied to the full set of analysed cases to estimate the possible range of governmental budgetary exposure if all claims were granted and fully enforced.

Statistical analysis

We conducted descriptive statistical analysis using SAS version 9.4 (SAS Institute, Cary, NC, USA). We summarised categorical variables using absolute and relative frequencies, with 95% confidence intervals (CIs) calculated using the normal approximation to the binomial distribution. We described quantitative variables, including age and amounts disbursed, using measures of central tendency and dispersion, according to data availability and distribution.

We also performed descriptive cross-tabulations involving eligibility criteria, type of legal representation, procedural status, and amounts disbursed. These analyses aimed to identify litigation patterns, assess compliance with product-label criteria, and examine the distribution of public expenditure. All analyses relied on the administrative spreadsheet provided by the Ministry of Health.

Results

Study population and procedural status

The census-based sample, obtained through Brazil's Access to Information Law, comprised 223 individual lawsuits filed against the Brazilian federal government during the study period. All lawsuits requested delandistrogene moxeparovec for patients diagnosed with DMD. All patients identified in the dataset were male (Table 1).

Table 1
General characteristics of lawsuits requesting delandistrogene moxeparvovec, Brazil

Characteristic	n	%
Age, years		
3	2	0.90
4	11	4.93
5	17	7.62
6	17	7.62
7	81	36.32
8 to 12	79	35.43
13 to 19	16	7.17
Sex		
Male	223	100.00
Ambulatory status		
Ambulatory	50	22.42
Ambulatory with difficulty	17	7.62
Non-ambulatory	5	2.24
Not reported	151	67.71
Legal representation		
Private attorney	216	96.86
Federal Public Defender's Office	7	3.14
Procedural and compliance status		
Technical-subsidy phase	103	46.19
Suspended compliance	100	44.84
Full compliance	15	6.73
Expert assessment with technical subsidies	3	1.35
Expert assessment	1	0.45
Preliminary injunction denied	1	0.45
Modality of compliance (n = 15)		
Direct purchase	13	86.67
Judicial deposit	2	13.33
Year of compliance (n = 15)		
2024	2	13.33
2025	13	86.67
All 223 lawsuits were filed against the Brazilian Ministry of Health between January 2024 and February 2026. Percentages are calculated on the total of 223 lawsuits unless otherwise indicated and may not sum to 100.00 because of rounding.		
^a Ambulatory status not reported, but eligible according to the age criterion.		

Characteristic	n	%
Age, years		
Eligible according to the age criterion		
Yes	126	56.50
No	97	43.50
Eligible according to ambulatory status		
Yes	50	22.42
No	22	9.87
Not reported	151	67.71
Eligible according to both criteria		
Yes	36	16.14
No	109	48.88
Ambulatory status not reported ^a	78	34.98
All 223 lawsuits were filed against the Brazilian Ministry of Health between January 2024 and February 2026. Percentages are calculated on the total of 223 lawsuits unless otherwise indicated and may not sum to 100.00 because of rounding.		
^a Ambulatory status not reported, but eligible according to the age criterion.		

The age distribution showed a concentration of cases among 7-year-old patients, who accounted for 36.32% of the sample (n = 81). Overall, 56.50% of patients (n = 126) were between 4 and 7 years of age, corresponding to the age range approved in the Brazilian product label. However, 43.50% of patients (n = 97) fell outside the approved range, including children younger than 4 years and those aged 8 years or older. Of the total sample, 35.43% (n = 79) were between 8 and 12 years, whereas 7.17% (n = 16) were between 13 and 19 years old.

Most lawsuits remained at preliminary procedural stages. Nearly half of the cases (46.19%; n = 103) were still undergoing the technical-subsidy phase, while 44.84% (n = 100) had suspended compliance. Only 15 lawsuits (6.73%) reached full compliance, defined as either direct acquisition of the therapy or judicial deposit by the State. Among these fulfilled cases, the Ministry of Health completed 86.67% (n = 13) through direct purchase, whereas 13.33% (n = 2) were resolved through judicial deposit. Most completed cases occurred in 2025, which accounted for 86.67% of fulfilled decisions.

Clinical eligibility according to the Brazilian product label

The Brazilian product label indicates delandistrogene moxeparovec for ambulatory paediatric patients between 4 and 7 years of age. Analysis of the lawsuits revealed substantial divergence between the claims presented in court and the approved eligibility criteria. Although 56.50% of patients (n = 126) were within the approved age range, 43.50% (n = 97) were outside the label indication. These cases included patients younger than 4 years and patients aged 8 years or older.

Assessment of ambulatory status was substantially limited by incomplete administrative records. Information on ambulation was absent or insufficiently specified in 67.71% of lawsuits (n = 151). Among cases with available data, 22.42% of patients (n = 50) were classified as ambulatory, 7.62% (n = 17) as ambulatory with difficulty, and 2.24% (n = 5) as non-ambulatory.

When both eligibility criteria were analysed jointly, only 16.14% of lawsuits (n = 36) involved patients who clearly satisfied the two central requirements established in the product label: age between 4 and 7 years and preserved ambulation. By contrast, 48.88% of cases (n = 109) failed to meet at least one eligibility criterion. An additional 34.98% of lawsuits (n = 78) involved patients who satisfied the age criterion but lacked sufficient information on ambulatory status, which prevented confirmation of full compliance with the approved indication (Additional file 1: Table S1).

Taken together, explicitly ineligible cases and cases with missing ambulatory information represented 83.86% of all claims (n = 187; 95% CI 79.03 to 88.69). Thus, only a minority of lawsuits could be confidently classified as fully aligned with the eligibility criteria established in the Brazilian product label.

Legal representation of claimants

Analysis of legal representation revealed an overwhelming predominance of privately filed lawsuits. Of the 223 cases analysed, 96.86% (n = 216) were conducted by private attorneys, whereas only 3.14% (n = 7) were represented by the Federal Public Defender’s Office (Additional file 1: Table S2).

Among cases represented by the Federal Public Defender’s Office, all patients satisfied the approved age criterion. However, ambulatory status was missing in 71.43% of cases (n = 5), while preserved ambulation was documented in 28.57% (n = 2).

By contrast, cases filed through private attorneys showed substantially lower alignment with the eligibility criteria established in the product label. Nearly half of the patients (44.91%; n = 97) exceeded the approved age range, and ambulatory status was not reported in 67.59% of cases (n = 146). When both eligibility criteria were assessed together, only 15.74% of privately represented cases (n = 34) satisfied both the age and ambulatory requirements. Conversely, 50.46% (n = 109) failed to meet at least one criterion, while 33.80% (n = 73) met the age requirement but lacked information on ambulatory status.

Compliance with court decisions according to clinical eligibility

Among the 36 lawsuits involving patients who satisfied both the age and ambulatory status criteria, only three cases resulted in compliance with the court decision, corresponding to 8.33% of this subgroup. The remaining 33 cases showed no record of complete provision of the medicine: 25 involved suspended compliance, six remained in the technical-subsidy phase, one was undergoing expert assessment with technical subsidies, and one had a preliminary injunction denied (Table 2).

Table 2 Procedural and compliance status according to label-based eligibility criteria

Age criteria

Procedural and compliance status	Not eligible (n = 97)	Eligible (n = 126)
Suspended compliance	24 (24.74)	76 (60.32)
Technical-subsidy phase	65 (67.01)	38 (30.16)
Expert assessment with technical subsidies	1 (1.03)	2 (1.59)
Preliminary injunction denied	0 (0.00)	1 (0.79)
Expert assessment	1 (1.03)	0 (0.00)
Full compliance	6 (6.19)	9 (7.14)

Ambulatory status criteria

Procedural and compliance status	Not eligible (n = 22)	Not reported (n = 151)	Eligible (n = 50)
Suspended compliance	14 (63.64)	56 (37.09)	30 (60.00)
Technical-subsidy phase	5 (22.73)	85 (56.29)	13 (26.00)
Expert assessment with technical subsidies	0 (0.00)	2 (1.32)	1 (2.00)
Preliminary injunction denied	0 (0.00)	0 (0.00)	1 (2.00)
Expert assessment	0 (0.00)	1 (0.66)	0 (0.00)
Full compliance	3 (13.64)	7 (4.64)	5 (10.00)

Age and ambulatory status criteria c

Procedural and compliance status	Not eligible (n = 109)	Ambulatory status not reported^a (n = 78)	Eligible (n = 36)
Suspended compliance	35 (32.11)	40 (51.28)	25 (69.44)
Technical-subsidy phase	66 (60.55)	31 (39.74)	6 (16.67)
Expert assessment with technical subsidies	1 (0.92)	1 (1.28)	1 (2.78)
Preliminary injunction denied	0 (0.00)	0 (0.00)	1 (2.78)
Expert assessment	1 (0.92)	0 (0.00)	0 (0.00)
Full compliance	6 (5.50)	6 (7.69)	3 (8.33)

Data are n (%). Percentages are calculated within each eligibility column. Column totals in each panel sum to 223 lawsuits.

^aAmbulatory status not reported, but eligible according to the age criterion.

Among the 109 lawsuits involving patients who did not satisfy the criteria established in the product label, six cases achieved full compliance, representing 5.50% of this group. In addition, 35 cases involved suspended compliance, whereas 66 remained in the technical-subsidy phase. The same pattern appeared among the 78 lawsuits involving patients who met the age requirement but lacked documented ambulatory information: of these, six achieved full compliance, 40 involved suspended compliance, and 31 remained in the technical-subsidy phase.

Taken together, these findings suggest that compliance with court decisions did not depend exclusively on strict adherence to the regulatory eligibility criteria. Of the 15 cases in which the medicine was fully provided, only three involved patients who clearly satisfied both approved criteria. The remaining cases involved patients who failed to meet at least one criterion (n = 6) or patients whose ambulatory status was not documented despite meeting the age requirement (n = 6).

Yet the high proportion of cases with missing clinical information, particularly ambulatory status, limits a definitive assessment of whether judicial decisions consistently aligned with the product label requirements.

Public expenditure incurred by the Ministry of Health

The Ministry of Health disbursed a total of R\$ 194,288,487.00 across 15 fully compliant cases, corresponding to a mean expenditure of R\$ 12,952,565.80 per patient (Table 3).

Table 3
Public expenditure on court-ordered delandistrogene moxeparvovec by label-based eligibility

Eligibility category	n	Mean expenditure (R\$)	Total expenditure (R\$)
Eligible according to the age criterion			
Yes	9	13 615 487.11	122 539 384.00
No	6	11 958 183.79	71 749 102.72
Eligible according to ambulatory status			
Yes	5	11 415 381.45	57 076 907.26
No	3	10 601 177.95	31 803 533.85
Not reported	7	15 058 292.29	105 408 046.00
Eligible according to both criteria			
Yes	3	10 601 177.95	31 803 533.85
No	6	11 958 183.79	71 749 102.72
Ambulatory status not reported ^a	6	15 122 641.72	90 735 850.29
Total	15	12 952 565.80	194 288 487.00
R\$, Brazilian reais. Amounts are as recorded in the Ministry of Health administrative dataset, in current reais, without inflation adjustment. Each of the three eligibility blocks covers the same 15 fully complied court orders. Mean values are derived from the total expenditure divided by n; because of rounding in the source records, the sum of the block totals differs from the overall total by less than R\$ 0.30.			
^a Ambulatory status not reported, but eligible according to the age criterion.			

When stratified by eligibility criteria, the three patients who met both the age and ambulatory status requirements accounted for a total expenditure of R\$ 31,803,533.85, with a mean cost of R\$ 10,601,177.95 per patient.

By contrast, the six patients classified as ineligible under the product label criteria generated a total expenditure of R\$ 71,749,102.72, equivalent to a mean cost of R\$ 11,958,183.79 per patient. The subgroup with the highest aggregate expenditure comprised six patients who satisfied the age criterion but lacked documented information on ambulatory status; this subgroup accounted for R\$ 90,735,850.29 in total expenditure, with a mean cost of R\$ 15,122,641.72 per patient.

Overall, 40% of the treatments effectively funded by the State were provided to patients who did not meet the eligibility criteria established in the product label. Another 40% were provided to patients whose eligibility could not be fully verified because their ambulatory status was not reported.

Potential budget impact scenarios

Using the expenditure observed in fully compliant cases, we modelled scenarios to estimate the potential financial impact if all 223 identified lawsuits had been granted and fully executed (Table 4).

Table 4
Projected budget impact of full compliance with all 223 lawsuits, by eligibility profile

Eligibility profile	n	Minimum scenario		Average scenario		Maximum scenario	
		Mean (R\$)	Total (R\$)	Mean (R\$)	Total (R\$)	Mean (R\$)	Total (R\$)
Eligible according to both criteria	36	10 601 177.94	381 642 406	12 756 616.81	459 238 205	18 222 076.97	655 994 771
Ambulatory status not reported ^a	78	10 948 982.86	854 020 663	13 119 494.71	1 023 320 587	18 623 174.86	1 452 607 639
Not eligible according to both criteria	109	10 675 875.52	1 163 670 432	12 897 829.17	1 405 863 379	18 531 948.23	2 019 982 357
Total	223	10 759 343.05	2 399 333 501	12 952 565.79	2 888 422 171	18 513 833.04	4 128 584 767
R\$, Brazilian reais. Scenarios apply the lowest, mean, and highest per-patient unit values observed in the 15 fully complied court orders to all 223 lawsuits identified during the study period. Projections cover medicine acquisition costs and judicial deposits only; they exclude administration, monitoring, and adverse-event management costs.							
^a Ambulatory status not reported, but eligible according to the age criterion.							

Under the minimum scenario, based on the lowest recorded unit values, the projected budget impact would be approximately R\$ 2.40 billion. Under the intermediate scenario, based on the mean expenditure of R\$ 12,952,565.79 per patient, the projected budget impact would reach approximately R\$ 2.89 billion. Under the maximum scenario, using the highest recorded per-patient expenditure of approximately R\$ 18.5 million, the projected financial impact would exceed R\$ 4.13 billion.

These estimates illustrate the scale of potential fiscal exposure associated with full compliance with the lawsuits identified during the study period. The projected amounts reflect only medicine acquisition costs or judicial deposits documented in the Ministry of Health administrative records. They exclude additional expenditure related to treatment administration, clinical monitoring, and management of adverse events.

Discussion

This census-based assessment of 223 lawsuits filed against the Brazilian federal government to obtain delandistrogene moxeparovec revealed that only 16.14% of claims involved patients who met both eligibility criteria established in the Brazilian product label: age between 4 and 7 years and preserved ambulation. Approximately half of the claims involved patients who clearly fell outside at least one regulatory parameter. Moreover, 67.71% of lawsuits contained no information on ambulatory status, a key variable for determining therapeutic eligibility. Despite these evidentiary gaps, courts had already ordered full compliance in 15 cases, generating public expenditure of R\$ 194.3 million. Among treatments effectively funded by the State, only three involved patients who fully satisfied label-based criteria; six treatments were provided to patients outside the approved parameters, while another six involved incomplete eligibility assessment because ambulatory status was not documented.

These findings suggest that the judicialization of Delandistrogene moxeparovec in Brazil cannot be understood merely as an exceptional response to barriers in administrative access. Rather, the phenomenon reflects a broader conflict among clinical urgency, scientific uncertainty, regulatory standards, judicial decision-making capacity, and the financial sustainability of the public health system. Some judicial decisions may broaden access beyond the population evaluated in clinical trials and approved by regulators. However, the data also show that patients who fully met the formal eligibility criteria did not always receive timely provision. This dual pattern exposes two parallel risks: expansion of access beyond regulatory parameters, and delay in access for patients who clearly qualify for treatment.

Litigation beyond approved criteria

The high number of lawsuits involving patients who either failed to meet label-based eligibility criteria or lacked complete clinical documentation raises important concerns about patient safety. In the case of delandistrogene moxeparvovec, this concern becomes particularly significant because the therapy involves a single-dose gene transfer intervention associated with extremely high cost, potentially irreversible biological effects, and persistent clinical uncertainty. The indication approved in Brazil is limited to a narrowly defined population: ambulatory paediatric patients between 4 and 7 years of age. This restriction reflects both the available scientific evidence and the evaluative standards adopted by the Brazilian regulatory authority [15].

Litigation that expands access beyond this population may expose patients to a therapy whose benefit–risk profile remains insufficiently established for broader clinical use. The international scientific context reinforces this concern. The EMBARK trial failed to meet its primary efficacy endpoint, and the relationship between micro-dystrophin expression and clinically meaningful functional improvement remains uncertain [9, 11, 12]. Moreover, the EMA refused marketing authorisation for the therapy in the European Union, whereas United States regulators authorised its use under a different evidentiary framework. This divergence underscores the continuing international debate regarding the robustness and sufficiency of the available clinical evidence [10, 13].

Against this background, the absence of ambulatory status in more than two-thirds of lawsuits becomes particularly concerning. Ambulation is not a secondary clinical characteristic; it is a core eligibility criterion and a central parameter for evaluating the appropriateness of treatment. Without this information, the State, the Judiciary, and technical experts cannot adequately determine whether a patient satisfies the approved therapeutic criteria. Such documentary deficiencies increase the likelihood of judicial decisions based on incomplete clinical evidence.

Safety concerns also extend beyond evidentiary uncertainty. Gene therapies that use AAV vectors carry recognised risks, including hepatotoxicity. Recent international regulatory warnings regarding acute liver failure further highlight the need for strict patient selection, specialised follow-up, and rigorous adherence to technical protocols [14]. Thus, the debate surrounding access to delandistrogene moxeparvovec is not limited to financial sustainability or equitable access to innovation. It also involves the protection of vulnerable patients who may be exposed to interventions with substantial biological impact despite ongoing uncertainty regarding long-term safety and clinical benefit.

Equity, access, and opportunity cost

Health litigation is often framed as a mechanism for protecting the individual right to health. However, the overwhelming predominance of lawsuits filed through private counsel suggests that access to this pathway is unevenly distributed. In this study, 96.86% of cases were initiated by private attorneys, while only 3.14% involved representation by the Federal Public Defender's Office. This pattern aligns with literature showing that health litigation tends to favour groups with greater access to information, legal assistance, and institutional networks, thereby creating potentially regressive distributive effects within universal public health systems [2–4].

This finding does not diminish the legitimacy of individual claims, especially in the context of rare, severe, and progressive diseases. But it does suggest that judicialization may function as an unequal access mechanism. Certain groups can accelerate access to exceptionally expensive technologies, while other patients, potentially with equal or greater clinical need, remain subject to standard administrative procedures within the SUS. The inequity becomes even more pronounced when litigation involves therapies costing millions of reais per patient.

The financial impact observed in this study reinforces this concern. The State spent R\$ 194.3 million on only 15 treatments, with an approximate mean cost of R\$ 13 million per patient. Sensitivity analyses further indicate that, if all identified claims were granted and fully executed, potential budget exposure could approach R\$ 4 billion. These figures should not be interpreted as projections of actual expenditure; rather, they illustrate the scale of the resource-allocation challenge.

In publicly funded health systems with finite resources, decisions involving extremely costly individual treatments inevitably generate substantial opportunity costs. Funds allocated to technologies whose efficacy, safety, and cost-effectiveness remain uncertain cannot simultaneously support collective interventions, structured care programmes, early diagnosis initiatives, rehabilitation services, multidisciplinary follow-up, or other interventions with established population-level benefits. This tension between micro-level individual justice and macro-level distributive justice remains a defining feature of health litigation in Brazil [2, 4]. The central issue, therefore, extends beyond whether an innovative therapy should be provided to an individual patient. It also

concerns whether exceptional judicial decisions can coexist with the principles of horizontal equity, fiscal sustainability, and coherence in public health policy.

Delayed access even among eligible patients

Another important finding is that litigation not only expanded access beyond established regulatory criteria but also failed to ensure timely treatment for some patients who fully satisfied the approved eligibility requirements. Among the 36 lawsuits involving patients who met all product-label criteria, only three demonstrated full compliance with the requested treatment. The remaining cases remained at various procedural stages, including suspended compliance, technical-review proceedings, medical expert evaluation, or denial of preliminary injunction relief.

This finding highlights an often-overlooked dimension of health litigation. The problem is not limited to the judicial provision of technologies outside established technical parameters. Institutional failures may also prevent the rapid identification of cases with stronger regulatory alignment and greater clinical plausibility. In progressive conditions such as DMD, these delays may carry significant consequences because treatment eligibility depends on a narrow therapeutic window and preservation of motor function [5, 7].

The presence of eligible patients who nevertheless lacked effective access underscores the need to strengthen technical triage mechanisms within judicial proceedings. Rather than treating all claims identically, courts and health authorities should rapidly classify cases into at least three categories: (1) patients who fully satisfy regulatory criteria; (2) patients with incomplete or insufficient clinical information; and (3) patients who clearly fall outside the approved indication. Such stratification could facilitate more proportionate and technically consistent decisions while simultaneously protecting patient safety, access rights, and health-system sustainability.

Institutional fragmentation between regulation, health technology assessment, and judicial decision-making

The findings suggest substantial institutional fragmentation among the mechanisms governing access to health technologies in Brazil. Marketing authorisation granted by Anvisa permits commercialisation of a product for a specific indication, but it does not guarantee automatic incorporation into the SUS. Conitec, in turn, evaluates clinical evidence, safety, cost-effectiveness, and budget impact to guide incorporation decisions within the public system. The Judiciary operates according to a different logic: judicial decisions focus primarily on the individual case, the urgency alleged, and the medical prescription presented. This separation between regulatory approval, health technology assessment, and public financing becomes particularly problematic in the context of high-cost advanced therapies [18, 19].

Although this institutional distinction is legitimate, inadequate integration among these decision-making spheres may produce fragmented or inconsistent outcomes. Theme 1234 of the Federal Supreme Court represents an attempt to improve coordination by establishing conditions for the judicial provision of medicines not incorporated into the SUS, including marketing authorisation, prior Conitec assessment, and adequate clinical justification [17]. Yet the findings of this study indicate that significant implementation challenges remain, particularly when essential clinical information, such as ambulatory status, is absent from judicial records.

Medical prescriptions occupy a central role in this context. In cases involving rare and severe disease, physicians, patients, and families understandably seek therapeutic alternatives in the face of inevitable disease progression. However, when prescriptions depart from criteria established in the approved product label, stronger clinical justification, greater transparency regarding uncertainty, and clearer documentation of potential risks become essential. Medical autonomy remains a fundamental component of clinical practice. Yet in cases involving extremely expensive technologies supported by uncertain evidence, professional discretion must remain aligned with regulatory standards, health technology assessments, and principles of distributive justice. This tension can be interpreted through the framework of street-level bureaucracy theory, which emphasises how frontline professionals exercise discretion under conditions of institutional pressure, complex demand, and resource scarcity [20–22].

Similarly, judicial intervention should rely on technical mechanisms capable of reducing informational asymmetries. Preliminary injunctions based exclusively on individual medical prescriptions may not provide an adequate basis for evaluating highly complex technologies, particularly where international regulatory divergences, reliance on surrogate endpoints, and substantial safety

concerns persist. The challenge, therefore, is not to replace judicial discretion with technocratic governance. Rather, it is to equip courts with more robust clinical and regulatory evidence so that individual decisions remain connected to broader public health policy objectives.

Policy implications

The findings highlight an urgent need to strengthen institutional mechanisms for managing gene therapies obtained through litigation. One priority is the implementation of standardised protocols for the technical assessment of lawsuits involving high-cost technologies. These protocols should establish minimum requirements for essential clinical documentation before courts or public authorities authorise treatment. In the case of delandistrogene moxeparvovec, decision-makers should have access to key information, including patient age, ambulatory status, genetic mutation, liver function, corticosteroid history, anti-AAV antibody status, and applicable exclusion criteria.

Another important implication concerns the need for differentiated triage of patients who clearly satisfy eligibility criteria. Because the product label establishes a narrow therapeutic window, procedural delays may eliminate the possibility of treatment. Administrative and judicial systems should therefore prioritise cases supported by complete documentation and clear conformity with approved criteria. Such prioritisation could prevent patients from losing access because of institutional delay rather than clinical deterioration.

A third implication involves transparency regarding opportunity cost. Claims involving exceptionally high-cost therapies should not be evaluated solely through the lens of individual urgency. They must also account for the broader systemic effects of repeated judicial decisions on finite public health budgets. This does not require denying the individual right to health. Rather, it requires recognition that universal health systems must balance individual justice with distributive justice, especially when technologies are expensive and supported by uncertain or evolving clinical evidence [1, 2, 4].

Finally, high-cost gene therapies demand more sophisticated regulatory and financing frameworks. In contexts marked by clinical uncertainty, mechanisms such as risk-sharing agreements, long-term patient registries, real-world evidence collection, outcome-based payment models, and national eligibility protocols may reduce tension between access and sustainability. The Delandistrogene moxeparvovec case demonstrates that, in the absence of these mechanisms, complex allocation decisions shift to the Judiciary. Yet judicial timelines do not always align with the pace of scientific evaluation or public health policymaking.

Limitations

This study has several limitations that should inform interpretation of the findings. First, the analysis relied on administrative data provided by the Ministry of Health through Brazil's Access to Information Law. Although this source enabled analysis of a census-based dataset of lawsuits filed against the federal government, the reliability of the findings depends on the completeness, consistency, and standardisation of the records supplied by public authorities.

Second, the dataset did not contain complete medical records, full clinical reports, or detailed information on patients' functional trajectories. Eligibility classification therefore depended exclusively on the variables available in the dataset, particularly age and ambulatory status. The absence of ambulation data in many cases should be interpreted as a documentary limitation of the available records rather than definitive evidence that such information was absent from the underlying court files.

Third, this study did not evaluate clinical outcomes after therapy administration. The findings therefore do not support conclusions regarding treatment effectiveness, adverse events, or functional progression among treated patients. Instead, the analysis focused on compliance with regulatory eligibility criteria, the legal characteristics of claims, and associated public expenditure.

Finally, the projections of financial impact should be interpreted as scenarios of potential budgetary exposure rather than predictions of actual spending. The estimates relied on unit values recorded in completed purchases or judicial deposits and excluded additional expenditure related to hospital administration, clinical monitoring, adverse-event management, subsequent litigation, and long-term follow-up.

Conclusions

The judicialization of delandistrogene moxeparvovec in Brazil exposes the governance challenges associated with high-cost gene therapies in publicly funded health systems. This study found that only a small proportion of lawsuits involved patients who fully satisfied the eligibility criteria established in the Brazilian product label. By contrast, many claims either fell outside those criteria or lacked sufficient clinical documentation to confirm therapeutic indication. At the same time, some formally eligible patients experienced delays and procedural barriers that hindered timely access to treatment. Litigation therefore appears to generate two simultaneous effects: expansion of access beyond regulatory parameters, and delays for patients with stronger clinical eligibility.

These findings raise important concerns regarding safety, equity, and financial sustainability. The predominance of lawsuits filed by private attorneys, combined with the exceptionally high unit cost of the therapy and the substantial volume of public funds mobilised through court orders, underscores the need for closer integration among health regulation, health technology assessment, medical prescribing practices, and judicial decision-making. When therapies involve major financial consequences and uncertain clinical evidence, court-ordered access should require minimum clinical documentation, transparent eligibility standards, and specialised technical review.

The Delandistrogene moxeparvovec case suggests that the central challenge is not a conflict between innovation and scientific rigour. Rather, the challenge lies in developing institutional mechanisms capable of reconciling both. National eligibility protocols, technical screening of lawsuits, long-term monitoring registries, and outcome-based financing arrangements may help ensure that advanced therapies are incorporated into the Unified Health System in a safer, more equitable, and more sustainable manner.

Abbreviations

AAV

Adeno-associated virus

Anvisa

Agência Nacional de Vigilância Sanitária

CI

Confidence interval

Conitec

Comissão Nacional de Incorporação de Tecnologias no SUS (National Committee for Health Technology Incorporation into the SUS)

DJUD/SE/MS

Departamento de Gestão das Demandas em Judicialização na Saúde (Department for the Management of Health Litigation Claims)

DMD

Duchenne muscular dystrophy

EMA

European Medicines Agency

FDA

Food and Drug Administration

LGPD

Lei Geral de Proteção de Dados (Brazilian General Data Protection Law)

SUS

Sistema Único de Saúde (Unified Health System)

Declarations

Clinical trial number:

Not applicable

Ethics approval and consent to participate

This study was conducted in accordance with the ethical principles of the Declaration of Helsinki and applicable Brazilian regulation for research involving human data. This study used exclusively secondary, anonymised administrative data provided by a public authority in response to a request submitted under Brazil's Access to Information Law (Law No. 12,527/2011). The dataset contained no names, identification numbers, addresses, family relationships, contact details, or other information capable of directly identifying patients. Because the study analysed secondary anonymised data obtained from a public administrative source, involved no direct interaction with human participants, and provided no access to individually identifiable information, it falls within the categories exempt from review by a research ethics committee under Brazilian National Health Council Resolutions No. 466/2012 and No. 510/2016. The requirement for informed consent was therefore not applicable. All stages of the study complied with the principles of confidentiality, data minimisation, and protection of sensitive information established under Brazil's General Data Protection Law (Law No. 13,709/2018).

Consent for publication

Not applicable. The manuscript contains no data from any individual person in any form.

Availability of data and materials

The datasets analysed during the current study are available from the corresponding author on reasonable request.

Competing interests

The author declares that he has no competing interests.

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Authors contributions

ACMS conceived and designed the study, requested and curated the administrative data, performed the statistical analysis, interpreted the results, and drafted and revised the manuscript. The author read and approved the final manuscript.

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Use of generative artificial intelligence

No generative artificial intelligence or large language model tool was used in the design, conduct, analysis, or original drafting of this study.

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