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Research Paper

Family Involvement and at-Home Physical Therapy on Duchenne Muscular Dystrophy: A Randomized Controlled Trial

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ABSTRACT

Background: Duchenne muscular dystrophy (DMD) is a genetic condition that causes muscle weakness and begins in early childhood. To treat its complications, the rehabilitation program includes physical therapy, mainly on the musculoskeletal and the respiratory complications that appear on the evolution of the disease. This study aims to explore the effects of physical therapy with or without an at-home program on motor function among children with DMD.
Methods: A randomized controlled trial was carried out for one year (one group with at-home and conventional physical therapy and another with conventional physical therapy). Motor function was measured using the Motor Function Measure (MFM) scale, the Vignos and Brooke scales, the Timed-up-and-Go test, and the six-minute walk distance test.
Results: Twenty-seven participants with DMD participated in this study. In the at-home and conventional physical therapy group, better motor function at the distal and global level was maintained, per the results of the MFM scale ($P < 0.05$). The rest of the variables did not achieve statistically significant changes.
Conclusions: Our results suggest that complementing conventional treatment with at-home treatment in which the family is involved maintains better motor function, in participants with DMD.

Introduction

Duchenne muscular dystrophy (DMD) is a genetic condition that severely affects the muscles, causing muscle weakness, which begins in early childhood.[1-3] Although rarely diagnosed in infancy (the diagnosis is usually made between age three and five), children with DMD have marked neck flexor weakness and poor head control from birth.[3]

Although in the 1960s, few survived DMD beyond adolescence, today, thanks to the advances in technology and research, the life expectancy of those with DMD has been greatly extended.[3,4] Today, there are many individuals who manage to live between 20 and 40 years,[2,3] so it is appropriate to anticipate that children with DMD will not only live to reach adulthood but also will likely live into their fifth decade of life.[5,6]

In recent years, there has been one factor that leads to an increase in survival in DMD, which is multidisciplinary care.[5,7] More specifically, physical therapy forms an integral part of this multidisciplinary approach, and its main objective is to prevent muscle deterioration by decreasing certain factors directly related to the

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complications of the disease.[1,6,7] Rehabilitation, including physical therapy interventions, is an integral part of the management of the musculoskeletal and the respiratory complications that may appear on the evolution of the disease.[5,8] Among its benefits, physical therapy has been proved to maintain upper and lower extremity function[9,10] and balance.[11] A systematic review has also shown evidence of some positive effects in respiratory muscle training in patients with DMD.[12]

In Spain, the annual social cost of this disease is estimated at €94,200, a figure that considers both health and nonhealth expenses. Among families of children with DMD, hiring and paying for an informal caregiver is reported as a major expense.[13] The main function of caregivers is to maintain the health and well-being of the patient[5]; having adequately trained caregivers can even reduce the social costs of the disease.[14] Usually these caregivers work as home-visiting professionals, to improve families' well-being and health, so that child well-being will also be improved.[15] Among these families, it is interesting to develop methodologies such as family-centered practices, the main aim of which is to recognize the family's role in helping decide on those practices, and thus making the family an active party of the patient's health.[16] This kind of intervention may improve the parent-infant interaction, which also increases the probabilities of success of an intervention.[15]

Despite this fact, there are currently no studies that demonstrate a reduction in deterioration in children with DMD who receive treatment at a clinical or at-home level, from their main caregiver or caregivers, or of the effects of a family-centered practice.

Therefore, the main objective of this study is to test whether at-home physical therapy treatment, in a family-centered practice, added to a conventional treatment program maintains better motor function in children with DMD. Secondarily, we will explore whether the specific motor function of the upper and lower limbs is maintained, as well as whether the risk of falls decreases in those individuals who receive at-home treatment associated with conventional treatment compared with those who only receive conventional treatment. Our hypothesis is that supplementing conventional treatment with at-home treatment would improve motor function compared with conventional treatment alone.

Materials and Methods

A single-blind randomized controlled trial was carried out, following the recommendations established by the CONSORT guidelines (registration number: NCT05313295).

Participants

The participants were children with a genetic diagnosis of DMD included in the Spanish Duchenne and Becker Patient Registry (https://www.duchenne-spain.org/registro-pacientes/). The inclusion criteria were as follows: (1) the parents or legal guardians of the participant agreed to their inclusion in the study, (2) the participants were included in the Spanish Duchenne and Becker Patient Registry, and (3) the participants were aged between three and 18 years. Individuals were excluded from participation if (1) they had another type of genetic or severe disease, (2) their parent/legal guardian refused to allow their participation in the study, (3) if patients were involved in another clinical trial including physical therapy, and (4) if children were participating in trials with experimental drugs.

Demographic (age, sex, performing physical activity) and clinical (age of first steps, age at diagnosis, use of glucocorticoids, use of dynamic ankle-foot orthosis, scoliosis, frequent falls, and gene mutations) data were collected at the first interview with the participants and their families. Evaluations were performed at two points throughout the study, at the beginning of the trial (T0) and one year after the initial evaluation (T1). The researchers who performed both evaluations, who were experienced in carrying out this type of study, were neither aware of the objective of this study nor did they know which group each participant was assigned to. All procedures were carried out following the principles of the Helsinki Declaration of the World Medical Association.[17] This study was also approved by the Bioethics Committee for Human Research at the University of Almeria (Ref: UALBIO2022/009). Before participating in the study, parents or legal guardians were asked to sign a form giving their corresponding informed consent.

Interventions

The participants were randomly divided into two intervention groups (1:1 ratio) with the help of computer software (EPIDAT v.4.2, Xunta de Galicia, Spain).

Both groups received a physical therapy protocol performed by an experienced physiotherapist twice a week for 60 minutes, consisting of trunk control, coordination and balance activities, passive or active-assisted stretching, and massage sessions focused on the most affected body regions (see Supplementary Material): (1) lower limbs (ankles, knees, and hips), (2) upper limbs (wrists and hands), and (3) spine (cervical and lumbosacral regions). In addition, they received a respiratory physical therapy intervention: (1) noninvasive ventilation with the indicated interfaces, (2) lung volume recruitment techniques, and (3) assisted manual coughing.

Participants in the at-home program had three hours of therapy in addition to the physical therapy they received at their respective institution, spread over three days a week. In these hours, physiotherapists agreed with parents or main caregivers on the treatment, consisting of stretching focused on the most affected regions and lower limb massage, giving the necessary guidelines to parents or main caregivers about how to perform them (see Supplementary Material). To ensure that the desired number of hours of the at-home program were administered, parents or caregivers had a meeting with the physical therapist before each of the physical therapy sessions, to solve questions that they may have regarding the at-home program. On these meetings, they were also asked to perform the stretching and massages, so the physical therapist could ensure that the intervention was being performed adequately. Moreover, parents were asked to register on a diary the techniques performed each day. For ethical reasons, once this study was completed, the families who had participated in the control group were offered to continue home sessions.

Study variables

The following questionnaires and specific tests were used to evaluate the participants' motor function.

The Motor Function Measurement (MFM) scale for the measurement of motor functional abilities in a person with neuromuscular disease

The MFM scale was created in France with the aim of better evaluating overall motor function in individuals with DMD, for both inpatient and outpatient use and for both ambulatory and non-ambulatory patients. There are two versions of the scale, the MFM 20 for children younger than six years and the MFM 32 for children older than six years. The scale takes three domains into account: (D1) standing and transfers, (D2) axial and proximal motor function, and (D3) distal motor function. The total of the three domains gives an overall percentage that shows an up-to-date overview of the functional diagnosis of the children.[18] In addition, a percentage is obtained with respect to the maximum score for each of the dimensions, therefore a higher percentage indicates better motor

function; in contrast, a percentage below 40% for domain 1 and below 70% for the overall score is related to a loss of ambulation capacity.[18,19] To calculate the score, general criteria are considered, which are specified for each item, scoring from 0 to 3. Both versions have been shown to have a test-retest reliability of 0.97 and 0.94, respectively.[18]

Brooke scale

This is a scale that uses levels 1 to 6 for the classification of motor function in the upper limbs.[20] Scoring is done according to the motor capabilities of the child, evaluating the following categories: (1) can lift the arms in a full circle until they touch above the head; (2) can raise the arms above the head only by flexing the elbow or by using accessory muscles; (3) cannot raise hands above the head, but can raise an 8-oz glass of water to the mouth (using both hands if necessary); (4) cannot raise hands above head, but can raise empty hands to the mouth; (5) cannot raise hands to the mouth, but can use the hands to hold a pen or to pick up pennies; and (6) has no useful function of the hands. The lower the score is, the better is the motor function in the upper limbs.[21] This scale is frequently used among the DMD population, and its intraclass correlation is 0.99.[22]

Vignos scale

This is a functional classification that scores from 1 to 10, where the highest number represents the most intense progressive DMD condition reflected in the children's ambulation ability. Possible categories are: (1) walks and climbs stairs without assistance, (2) walks and climbs stairs with the aid of railing, (3) walks and climbs stairs slowly with the aid of railing, (4) walks unassisted and rises from chair but cannot climb stairs, (5) walks without assistance but cannot rise from a chair or climb stairs, (6) walks only with help of long leg braces, (7) walks with long leg braces but requires assistance for balance, (8) stands in long leg braces but unable to walk even with assistance, (9) is in a wheelchair, and (10) is confined to a bed.[23,24]

Timed-Up and Go test (TUGT)

This test determines an individual's fall risk. The test, using a chronometer, is performed by asking the participant to stand up from a chair (with or without support), stop, walk 3 meters or 10 feet, turn around, and walk back to sit back down in the same chair. If participants take more than 20 seconds to perform this task, they are at a high risk of falling; between 10 and 20 seconds indicates a moderate risk.[25]

Six minutes walking test (6MWT)

This test consists of quantifying in meters the distance that an individual can travel in six minutes. The more meters covered, the lesser the deterioration.[26] Periodic individualized evaluation of the 6MWT is the most widely accepted primary clinical assessment in DMD clinical trials,[27] and it provides a better prognosis than that based on age alone. After analyzing its test-retest reliability in DMD, its intraclass correlation is 0.92.[26]

Sample size

To detect a minimal clinically important difference of 28.5 m in the 6MWT between both groups,[28] with an alpha value of 0.05 and assuming a 95% statistical power, 24 participants in total were needed (i.e., 12 participants per group), assuming a 10% dropout rate. Sample size calculation was made with G*Power v3.1.9.7 (Düsseldorf, Germany).

Statistical analysis

The variables used were described in terms of the mean and S.D. (continuous variables) and frequencies and percentages (categorical variables). The normality of the variables was analyzed using the Shapiro-Wilk test. To verify that there were no differences between the two study groups at baseline, the *t* test for independent samples or the Mann-Whitney U test was performed, respectively, depending on the normality of the variables. With the objective of analyzing the differences after the two interventions, a multivariate analysis of repeated measures was performed (analysis of variance [ANOVA]), with the type of treatment as an intergroup variable and the evaluation time (T0 and T1) as an intragroup variable. For categorical variables (Brooke and Vignos scales) an analysis with the chi-squared test or Fisher exact test was used to observe the change in score values between pre- and post-treatment within each intervention group. In addition, Cohen d value was calculated to find out the effect size of the treatment of conventional and at-home physical therapy. A statistical significance level was established at a value of $P < 0.05$. All analyses were performed with the SPSS statistical package, version 25.0 (IBM Statistics, Armonk, NY, USA).

Results

The total sample consisted of 30 participants, of which 15 were assigned to the experimental group and 15 to the control group. However, data were only gathered from 12 participants from the control group by the end of the trial (Fig 1), therefore the final sample size was 27 participants. The study period lasted from November 2018 to November 2019, and no adverse events occurred during the course of the study. The mean general age of the participants was 8.26 ± 3.59; in the experimental group, the age ranged from four to 14 years, whereas in the control group, the age ranged from four to 18 years. The vast majority of the participants were male, with a total of 21 (84%), as opposed to only four (16%) female participants. Moreover, none of the participants had a mild mutation (Supplementary Table). However, there were no significant differences in the variables analyzed in both groups at baseline. The data regarding the demographic and clinical characteristics of the participants can be seen in Table 1.

The MFM scale applied to neuromuscular diseases

The ANOVA analysis showed a statistically significant improvement in the experimental group versus the control group in domain 3 of the MFM scale ($F = 8.535$; $P = 0.007$), as well as in the overall score of the scale ($F = 4.385$; $P = 0.047$). However, there were no statistically significant differences in domains 1 and 2 on the MFM scale ($P > 0.05$). The data gathered at each collection point and about the experimental and control groups are shown in Table 2. The effect size of the combination of conventional and at-home treatment was large (d = 1.10; 95% confidence interval 0.28 to 1.91) for domain 3, as well as for the overall score of the MFM scale (d = 0.78, 95% confidence interval -0.01 to 1.57).

Brooke and Vignos Scales

The chi-square test neither showed statistically significant changes in the experimental group for the Brooke ($\chi^2 = 0.833$; $P = 0.659$) and Vignos ($\chi^2 = 3.424$; $P = 0.754$) scales nor did it show such changes in the control group on the Brooke ($\chi^2 = 1.053$; $P = 0.789$) and Vignos ($\chi^2 = 1.091$; $P = 0.896$) scales. The changes in values between the different treatment times on the Brooke and Vignos scales are detailed in Fig 2.

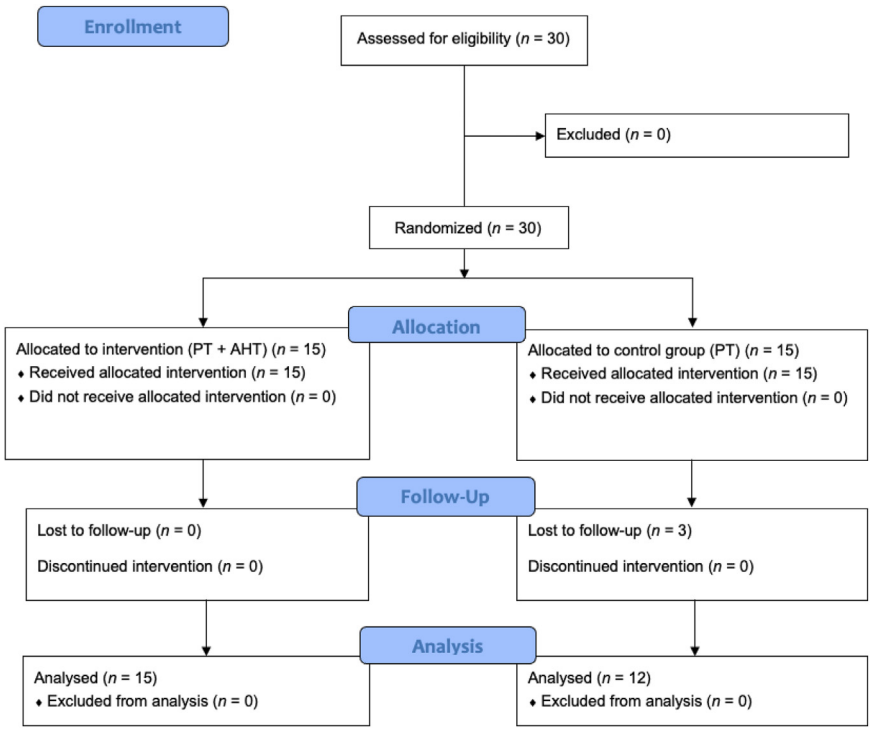

FIGURE 1. CONSORT flow diagram of participants included in the trial. AHT, at-home physical therapy; PT, physical therapy. The color version of this figure is available in the online edition.

TABLE 1.
Participants' Demographic and Clinical Characteristics (n = 27)

Outcomes	PT + AHT (n = 15)	PT (n = 12)	*P* Value
Age	7.80 (4.12)	8.83 (2.85)	0.468
Sex			0.183
Male	14 (93.3)	9 (75.1)	
Female	1 (6.7)	3 (24.9)	
Age of first steps			0.137
12-18 months	7 (46.9)	9 (75.1)	
>18 months	8 (53.1)	3 (24.9)	
Age at diagnosis			0.863
<3 years old	8 (53.1)	6 (50)	
>3 years old	7 (46.9)	6 (50)	
Use of glucocorticoids			0.326
Yes	10 (67)	10 (83.4)	
No	5 (33)	2 (16.6)	
Use of DAFO braces			0.343
Yes	6 (40.2)	7 (58.3)	
No	9 (59.8)	5 (41.7)	
Patient does physical activity			1
Yes	10 (66.5)	8 (66.4)	
No	5 (33.5)	4 (33.6)	
Scoliosis			0.468
No	3 (21)	5 (41.7)	
Inflammatory	10 (66)	6 (50)	
Yes	2 (14)	1 (8.3)	
Frequent falls			0.706
Yes	4 (26.3)	4 (33.6)	
No	11 (73.7)	8 (66.4)	
Stage of the disease			0.412
Presymptomatic	0 (0)	0 (0)	
Early ambulatory	13 (86.6)	11 (91.6)	
Late ambulatory	1 (6.7)	0 (0)	
Early nonambulatory	0 (0)	1 (8.4)	
Late nonambulatory	1 (6.7)	0 (0	

Abbreviations:
AHT = At-home physical therapy
DAFO = Dynamic ankle-foot orthosis
PT = Physical therapy

TUGT and 6MWT

The ANOVA analysis also did not show any statistically significant differences between the experimental and control groups for the TUGT (F = 0.065; *P* = 0.802) and 6MWT (F = 1.072; *P* = 0.313). The pre- and post-treatment data as well as the difference in means between evaluation times are shown in Table 3.

Discussion

The main objective of this study was to explore whether home treatment added to conventional treatment maintains better motor function among children and adolescents with DMD, in addition to showing whether specific upper and lower limb motor function improves, as well as to determine whether the fall risk decreases in individuals who receive at-home treatment in conjunction with conventional treatment, compared with those who only receive conventional treatment.

Improved maintenance of motor function, defined as the capacity of maintaining the abilities evaluated before the participation on this randomized controlled trial, was only evident in the group that received at-home and conventional treatment, when compared with the control group, in dimension 3 of the MFM scale and on the overall score of this scale. However, no statistically significant changes between groups were observed in the rest of the parameters analyzed (using the Vignos and Brooke scales and the TUGT and 6MWT).

The MFM scale was chosen because it is the only scale that assesses motor function in both ambulatory and nonambulatory patients, considering several different assessment domains with the advantage of its well-known sensitivity to change.[29-31] Regarding the MFM scale, a prospective descriptive study indicated a loss of the abilities measured in domain 1 of the scale over six months,

TABLE 2.
Pretreatment, Post-treatment, and Difference in Values on the MFM

Outcomes	PT + AHT (n = 15)	PT (n = 12)	*P** Value
Domain 1			
Pretreatment	66.211 ± 35.371	72.996 ± 18.515	
Post-treatment	65,416 ± 35.001	75.004 ± 18.751	
Pre-post difference	−0.795 ± 11.29	2.007 ± 5.303	$P = 0.401$; F = 0.729
Domain 2			
Pretreatment	90.288 ± 15.904	94.444 ± 8.704	
Post-treatment	90.275 ± 14.387	90.054 ± 10.776	
Pre-post difference	−0.013 ± 4.512	−4.390 ± 7.469	$P = 0.087$; F = 3.177
Domain 3			
Pretreatment	85.118 ± 21.825	89.127 ± 13.483	
Post-treatment	84.559 ± 11.774	70.508 ± 17.373	
Pre-post difference	−0.559 ± 16.053	−18.618 ± 15.887	**$P = 0.007$; F = 8.535**
Overall score			
Pretreatment	80.539 ± 22.904	85.527 ± 11.802	
Post-treatment	80.084 ± 18.192	78.552 ± 13.615	
Pre-post difference	−0.455 ± 8.300	−7.001 ± 7.885	**$P = 0.047$; F = 4.385**

Abbreviations:
AHT = At-home physical therapy
ANOVA = Analysis of variance
MFM = Motor Function Measurement scale
PT = Physical therapy
The values are expressed as mean ± S.D. Statistically significant values are emphasized in bold.
* Group × time interaction (through ANOVA analysis of repeated measures).

mainly due to the fact that its sample type was mostly non-ambulatory.[30] In our study, we did not analyze the changes in the MFM scale as a function of the ambulatory capacity of the participants, but with a mainly ambulatory sample, we do not believe it would have modified the results obtained. In addition, both domain 3 and the overall score were the ones that showed statistically significant changes in the group with additional at-home treatment versus the group with conventional treatment. According to our results, both distal motor and overall motor function would be influenced by at-home treatment combined with conventional treatment, maintaining better function of both.

However, no significant changes were obtained between both groups in the values indicating DMD progression, neither in improvement of the values on the Vignos and Brooke scales nor on the TUGT and 6MWT. After observing the values obtained in both evaluations, motor function worsened in both groups, so these results show that the progression of the disease continues even despite a year of treatment. There are no data on the minimum clinical difference in DMD for the TUGT, but in children with cerebral palsy, it varies from 1.40 to 8.74 seconds.[32] In our study population, both groups exceeded the minimum value, thus increasing their dynamic instability and with it, the risk of falls, over the course of a year, despite receiving physical therapy treatment.[33] Nonetheless, McDonald et al. showed that a loss of 30 m from averaged performance on 6MWT is predictive of a significant decline in ambulation over the subsequent year and that decline greater than or equal to 10% on the 10-m run/walk over the course of a year is predictive of loss of ambulation over the subsequent four years; in our case, neither of the two groups reached such a vast difference between both evaluations. Thus, the beneficial effect and the need for physical therapy treatment to maintain motor function, as previously described, is further confirmed.[6]

Regarding at-home physical therapy, there are a variety of recommendations in the existing literature. Some authors focus solely on interventions at physical therapy centers, without performing at-home treatment that complements and encompasses the daily activities of the child.[1,34,35] However, several authors also support at-home physical therapy programs for neuromuscular diseases

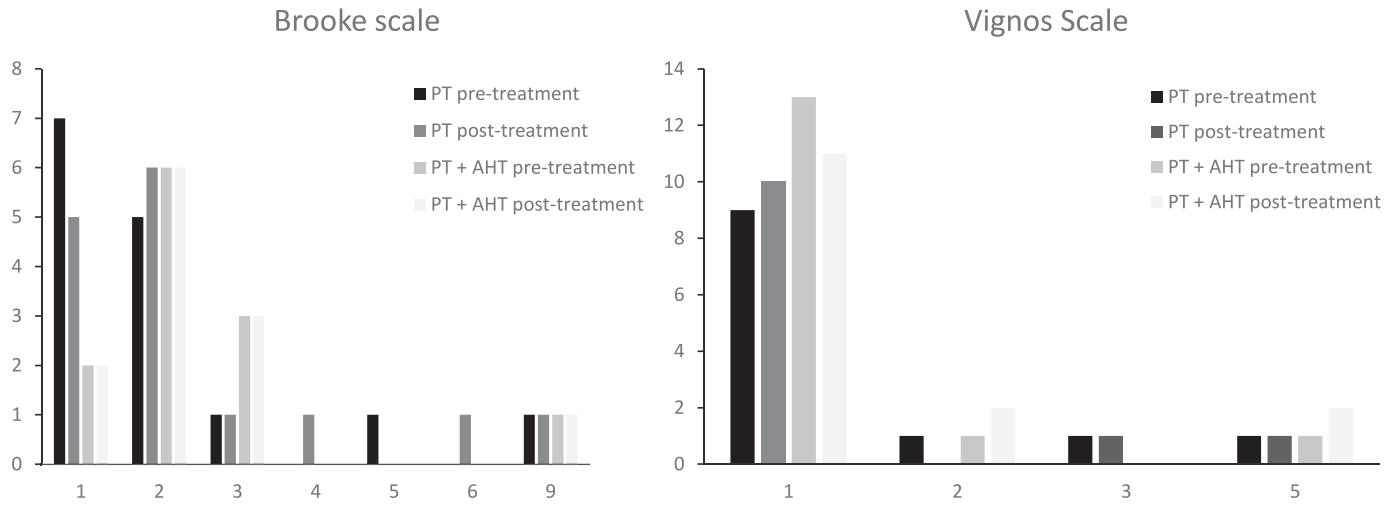

FIGURE 2. Brooke and Vignos scales.

TABLE 3.
Pretreatment, Post-treatment, and Difference in Values on the TUGT and 6MWT

Outcomes	PT + AHT (n = 15)	PT (n = 12)	*P** Value
TUGT (s)			
Pretreatment	6.024 ± 4.293	7.555 ± 8.857	
Post-treatment	8.479 ± 8.069	9.495 ± 10.063	
Pre-post difference	2.455 ± 4.527	1.94 ± 4.686	0.802
6MWT (m)			
Pretreatment	373.307 ± 75.241	422.628 ± 74.74	
Post-treatment	364.821 ± 70.595	392.772 ± 108.721	
Pre-post difference	−8.486 ± 50.314	−29.856 ± 43.217	0.313

Abbreviations:
6MWT = Six-minute walk test
AHT = At-home physical therapy
ANOVA = Analysis of variance
PT = Physical therapy
TUGT = Timed-Up-and-Go test
The values are expressed as mean ± S.D.
* Group × time interaction (through ANOVA analysis of repeated measures).6MWT: Six minutes walking test; AHT: At home physical therapy; PT: Physical Therapy; TUGT: Timed-up and go test

due to their feasibility, flexibility, and low cost.[10,34,36,37] Recommendations for the treatment of motor impairments in individuals with DMD indicate that treatment should be received daily or no less than four to six times per week, which would be difficult to carry out without an at-home program.[1] In addition, these programs should be individualized based on the initial evaluation of each child, including techniques such as stretching, manual therapy, and respiratory physical therapy. In relation to the inclusion of physical activity in treatment programs, better maintenance of motor function has been demonstrated in individuals who perform low-intensity physical activity[34]; however, endurance exercise is not recommended, since it has been associated in the literature with a decline in muscle strength and motor function in patients.[37] For these reasons, we support the use of at-home physical therapy in individuals with DMD, as it allows for them to receive the recommended number of weekly, individualized treatment sessions, and not only the inclusion of physical therapy techniques but also the use of low-intensity physical activity and the implication of the families in the treatment of these children. At-home physical therapy could be enhanced by promoting the participation of the children's families in activities of the centers where conventional therapy is performed.

However, this study does have some limitations. First, the sample size, despite being similar to that of other studies that analyze the effects of physical therapy on DMD, may be insufficient to obtain significant values in the variables analyzed. In addition, we did not evaluate some objective variables that could have been of interest, such as joint range of motion using a goniometer or muscle strength using validated scales. It also remains unclear if the beneficial effects are a consequence of the additional time given in the at-home setting or the type of physical therapy performed in it. Also, due to the pandemic situation caused by the coronavirus disease 2019, we could not perform a long-term follow-up, but this could provide additional information about the evolution of the studied population. Finally, it is possible that the wide age range of the trial participants may also have resulted in the lack of changes observed in the variables analyzed. Therefore, it is recommended for future studies to increase the sample size, in addition to segregating the sample into different age groups to observe whether changes in motor function occur depending on the age of the participants and the progression of the disease. It could also be interesting for future studies with a similar duration to perform a middle time point assessment, so that the evolution of the participants during the study could be registered and evaluated. Moreover, future studies could also investigate the effects of other treatment strategies (i.e., hydrotherapy, hippotherapy, occupational therapy, among others) in combination with the at-home therapy, to maintain function and quality of life in individuals DMD. These improvements could provide clearer data about which recommendations are more indicated on individuals with DMD.

Conclusions

Complementing conventional treatment with at-home treatment for children with DMD maintains improved motor function of the domains evaluated on the MFM questionnaire over the course of a year. However, the inclusion of this treatment neither improves the specific motor function of upper or lower limbs nor does it reduce the risk of falls in the study population. Despite these results, both at-home and conventional physical therapy treatments are recommended for children with DMD, since the results obtained suggest that such treatment decreases the progression of motor function deterioration in this population.

CRediT authorship contribution statement

Andrea Hernández-Sánchez: Data curation, Investigation, Methodology, Writing – original draft. **Lidia Parra-Sánchez:** Conceptualization, Investigation, Methodology, Resources. **Marisol Montolio:** Data curation, Formal analysis, Funding acquisition, Resources, Supervision, Validation, Writing – original draft. **Lola Rueda-Ruzafa:** Conceptualization, Investigation, Visualization, Writing – original draft, Writing – review & editing, Software. **Lucía Ortiz-Comino:** Conceptualization, Data curation, Formal analysis, Investigation, Methodology, Project administration, Supervision, Writing – original draft, Writing – review & editing. **María Del MarSánchez-Joya:** Conceptualization, Data curation, Funding acquisition, Methodology, Project administration, Resources, Supervision, Visualization, Writing – original draft, Writing – review & editing, Validation.

Declaration of competing interest

None.

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Supplementary Data

Supplementary data related to this article can be found at https://doi.org/10.1016/j.pediatrneurol.2023.12.015.

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