Examining the Validity of Efforts of the Muscular Dystrophy Medical Network in Nagano : A Questionnaire Study

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Background : We administered questionnaires to six institutions in Nagano prefecture that make up the Muscular Dystrophy Medical Network in Nagano. The organization was established in 2011 and aims to improve the effective treatment of Duchenne muscular dystrophy (DMD). Specifically, we examined the validity of the network's activities.

Materials and Methods : Questionnaires were distributed to team members, including 20 physical therapists, 6 occupational therapists, 2 clinical genetic counselors, and 17 doctors. Participants' responses were returned to an unrelated person, who was not directly associated with this research. An unrelated person then provided the responses to the first author of this study.

Results : Significant improvements were seen in all items, such as "the ease of solving patient problems" and "the ease of communication with other occupations and staff at other facilities."

Conclusion : All team members recognized the activities of the Muscular Dystrophy Medical Network in Nagano as effective. Specifically, the understanding of information about other institutions, treatment methods, and evaluation methods was deepened, and the activities of the Muscular Dystrophy Medical Network in Nagano could contribute to the reduction of disparity in medical treatment for DMD among prefectural facilities.

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I Introduction

Duchenne muscular dystrophy (DMD) is an X chromosome-linked disorder that causes progressive muscle atrophy and weakness. It is the most prevalent hereditary muscle disease, with an occurrence rate of one in every 3,600 male births[1)-3)]. The onset of DMD begins with gait disturbance at age 2-5 years. Then, whole-body muscle atrophy and weakness progress, and patients become unable to walk when they are approximately 13 years old[4)]. They typically die of respiratory or cardiac failure at around age 30[5)].

To date, a radical therapy for DMD has not yet been established, nor are there standardized assessments or therapeutic methods available in physical therapy. However, in addition to the active development of treatments, such as gene and regeneration therapies, insurance adaptation and clinical trials for corticosteroids use[6)-9)] are also being considered.

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Moreover, in Japan, the muscular dystrophy patient registration system, the Registry of Muscular Dystrophy (REMUDY) (http://www.remudy.jp/), has been established[10], and the Muscular Dystrophy Clinical Trial Network has been created to help facilitate clinical trials and clinical research. Consequently, since the medical circumstances surrounding patients with DMD is changing rapidly, consideration and ingenuity are required to ensure there are no differences in medical treatment and physical therapy among communities or medical institutions.

In order to overcome these potential complications, a multidisciplinary medical team comprised of pediatricians, neurologists, geneticists, and physical therapists from six medical institutions, known as the Muscular Dystrophy Medical Network in Nagano, was established in May 2011 in Nagano prefecture. This network conducts comprehensive medical care and support, including sharing information about patients with DMD. To date, there are no other network systems from multiple disciplines and multiple institutions for muscular dystrophy in Japan. This network is considered to be a multi-center multi-occupation collaboration within the same prefecture. This approach reduces disparity among facilities. Furthermore, it is advantageous because it could have access to many patient cases and new findings.

The Muscular Dystrophy Medical Network in Nagano holds a bimonthly conference using the remote conference system of each facility. In these meetings, team members discuss treatment policies for "patient-in-charge," share information on evaluation results, and discuss how to solve problems. Additionally, conference attendees may obtain a wide range of information other than that which directly involves the patient, which can also aid in the reduction of medical disparities between facilities. Particularly in the area of physical therapy, there is no standard evaluation or treatment for DMD; therefore, disparity among facilities tends to be substantial. Furthermore, for international clinical trials and clinical investigations on DMD, independent assessment of physical therapists in motor function tests such as the North Star Ambulatory Assessment (NSAA) and the Six-Minute Walk Test (6MWT), is essential to eliminate bias among doctors' evaluation results. Based on the current situation, it is important to understand the perception of physical therapists regarding the medical treatment of DMD, particularly the change in perception due to subscription to this network. Only the physical therapists answered other question items.

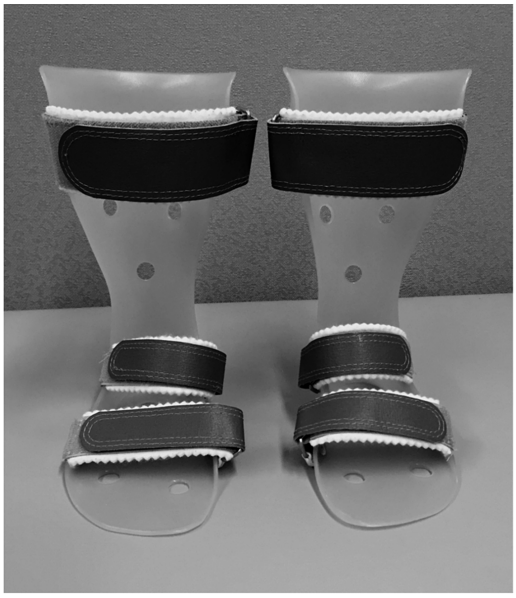

Fig. 1　Night splints
This photograph shows a model of night splints created at each institution. This type is made of plastic, and is securely fitted to the patient's lower leg and ankle joints with straps.

As such, the primary purpose of the network is to judge the timing of corticosteroids introduction and standardize motor-function evaluation methods, such as how to introduce night splints effectively **(Fig. 1)** in order to maintain a patient's gait function. Consequently, we evaluated the effectiveness of the Muscular Dystrophy Medical Network in Nagano's activities, and examined how to best utilize the obtained information to provide effective muscular dystrophy medical treatment and reduce disparity between institutions and care workers. Specifically, in the questionnaire, we addressed (1) the effectiveness of the Muscular Dystrophy Medical Network in Nagano's activity in deciding treatment and evaluation choice for each medical carer, and (2) the effectiveness of

the Muscular Dystrophy Medical Network in Nagano's information sharing.

Ⅱ Materials and Methods

A Participants

The team comprises 20 physical therapists, six occupational therapists, two clinical genetic counselors, ten pediatricians, three neurologists, two geneticists, one pulmonologist, and one orthopedic surgeon. Team members were affiliated with Shinshu University Hospital, Shinano Handicapped Children's Hospital, the Inariyama Medical Welfare Center, Nagano Children's Hospital, the National Hospital Organization Matsumoto Medical Center, and the Kakeyu-Misayama Rehabilitation Center at Misayama Hospital.

B Questionnaire method

1 Questionnaire contents

There were two questionnaires : one targeted physical therapists and the other targeted all other healthcare occupations **(Table 1)**. On the questionnaire for physical therapists, question items addressed how much they understood evaluation methods, and the importance of NSAA and 6MWT. Both NSAA and 6MWT are necessary for determining the timing and effectiveness of corticosteroids introduction, and are required for development and promotion to clinical trials. Conversely, in preparation of night splints, which is related to treatment standardization, question items were prepared that addressed whether physical therapists understood the purpose and importance of creating night splints. Night splints are the most commonly prescribed brace during the initial stages of DMD that help prevent Achilles tendon contracture and promote maintenance of gait ability[11)-14)]. The basic policy of this network is to prepare night splints, which were developed through reference of previous studies and international guidelines[15)-17)], for all DMD patients older than age four. All the questionnaire surveys conducted were in Japanese.

2 Answering method

All questions were answered using a five-point Likert scale : 1. "*I do not think so at all (absolutely not applicable)*;" 2. "*I do not think so (not very much)*;" 3. "*Neither*;" 4. "*Relatively agree (relatively true)*;" and 5. "*I think so (very true).*" Questions were answered once before network participation and once at the time of the survey.

3 Distribution and collection method and data analyses method

Questionnaires were distributed to those on the mailing list of the DMD network. Participants who provided consent to participate in the study completed the questionnaire and emailed their responses to an unrelated person, who was not directly associated with the Muscular Dystrophy Medical Network in Nagano. The unrelated person printed only the answer sheets and provided them to the first author **(Fig. 2)**. Next, the authors analyzed the collected answer sheets, which were responded to anonymously. For data analyses, we used paired *t*-tests. All analyses were conducted using the PASW Statistics software (version 24.0, SPSS, Inc., Chicago, IL, USA).

This study was approved by the institutional ethics committee of the Shinshu University School of Medicine, Japan (approval number : 3763).

Ⅲ Results

A Questionnaire results of all members (Table 2)

Of all members, 71 % responded to the questionnaire. Among all question items, mean scores at the time of the questionnaire were significantly higher than before participation. The ease of solving patient problems ($p<.001$), utilizing information about other occupations ($p<.001$), and the ease of communication with other occupations and staff at other facilities ($p<.001$) were significant **(Table 2)**.

B Questionnaire results of physical therapists (Table 3)

Among physical therapists, 75 % responded to the questionnaire. Among all question items, mean scores at the time of the questionnaire were significantly higher than before participation. Specifically, understanding the evaluation method and importance of NSAA and 6MWT ($p=.001$), understanding the purpose and importance of night splints ($p=.01$), and grasping the contents of doctors' treatments (p

Table 1 Questionnaire contents

Questions for all members	
Q-1. Is it easy for you to solve the problems of patients with DMD in your work environment?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)
Q-2. Do you use information from other occupations and facilities for medical treatment of DMD patients?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)
→ Please describe concretely when you mark ○ in (5), (4) of [At the time of survey]	
Q-3. Is it easy for you to communicate with other professions or staff at other facilities?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)
Questions for physical therapists	
QP-1. Do you understand the evaluation method and importance of NSAA or the Six-Minute Walk Test?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)
QP-2. Do you know the purpose and importance of making night splints?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)
QP-3. Are you performing physical therapy while referring to the doctor's treatment?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)
→ Please describe concretely when you mark ○ in (5), (4) of [At the time of survey]	
Questions for other occupations	
QO-1. Do you get confused concerning the treatment policy decisions for DMD patients?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)
QO-2. Do you not get confused in grasping the condition of patients other than the ones you are responsible for?	
[Before participation in this team medicine]	[At the time of survey]
(5) I think so (very true)	(5) I think so (very true)
(4) Relatively agree (relatively true)	(4) Relatively agree (relatively true)
(3) Neither	(3) Neither
(2) I do not think so (not very much)	(2) I do not think so (not very much)
(1) I do not think so at all (absolutely not applicable)	(1) I do not think so at all (absolutely not applicable)

DMD ; Duchenne muscular dystrophy, NSAA ; North Star Ambulatory Assessment.

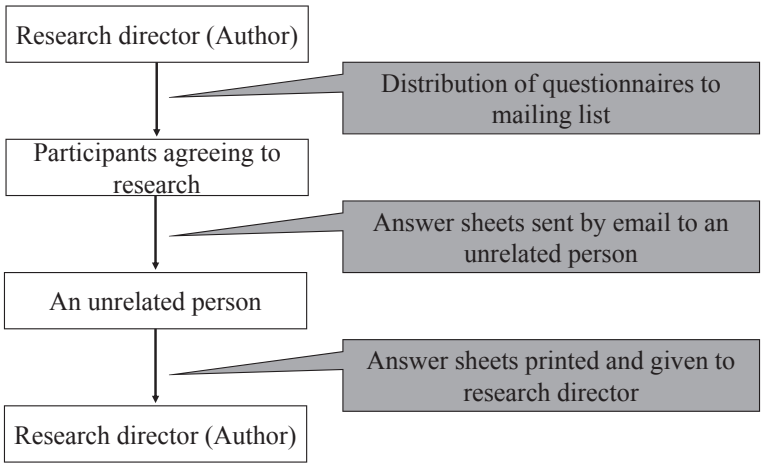

Fig 2　Questionnaire distribution and collection procedure

The questionnaires were distributed to participants using the mailing list that the Muscular Dystrophy Medical Network in Nagano routinely uses. Participants who provided consent responded to the questionnaires and emailed their results to an unrelated person not directly involved in this research. The unrelated person printed only the answer sheets, which were responded to anonymously, and turned them over to the first author.

Table 2　Questionnaire results of all members. (n = 32)

		mean ± SD (/5)	p-value[a]
Q-1	Before participation in this team medicine	3.0 ± 0.9	$<0.001^{***}$
	At the time of survey	4.0 ± 0.8	
Q-2	Before participation in this team medicine	2.6 ± 0.9	$<0.001^{***}$
	At the time of survey	3.8 ± 0.9	
Q-3	Before participation in this team medicine	2.8 ± 0.9	$<0.001^{***}$
	At the time of survey	3.8 ± 0.9	

[a] Wilcoxon signed-rank test ; *** $p<0.001$.

Table 3　Questionnaire results of physical therapists. (n = 15)

		mean ± SD (/5)	p-value[b]
QP-1	Before participation in this team medicine	2.8 ± 0.9	0.001^{**}
	At the time of survey	3.5 ± 0.9	
QP-2	Before participation in this team medicine	2.8 ± 1.2	0.01^{*}
	At the time of survey	3.8 ± 1.1	
QP-3	Before participation in this team medicine	2.9 ± 0.9	$<0.001^{***}$
	At the time of survey	3.8 ± 0.7	

[b] paired t-test ; $^{*}p<0.05$; ** $p<0.01$; *** $p<0.001$.

＜.001) were significant **(Table 3)**.

In participants' free descriptions, which were concrete examples related to problem-solving, some opinions included "grasping the condition by medication and introduction of equipment" (23 %), "ventilator setting" (23 %), and "information sharing with other facilities and departments" (15 %) **(Table 5)**.

C　Questionnaire results of occupations other than physical therapists (Table 4)

Among occupations other than physical therapists, 68 % responded to the questionnaire. Among all question items, mean scores at the time of the questionnaire were significantly higher than before participation. Specifically, the ease of deciding a treatment

Table 4 Questionnaire results of occupations other than physical therapists. (n = 17)

		mean ± SD (/5)	p-value[b]
QO-1	Before participation in this team medicine	2.6 ± 0.9	$<0.001^{***}$
	At the time of survey	3.4 ± 0.8	
QO-2	Before participation in this team medicine	2.8 ± 1.3	$<0.001^{***}$
	At the time of survey	4.0 ± 0.8	

[b] paired t-test ; $^{***}p<0.001$.

Table 5 Free descriptions of physical therapists and other occupations in the questionnaire

Job category	Contents of free description
Physical therapists	I was able to learn about the patients' status by their medication and equipment introduction. (23 %) I was able to learn about the ventilator setting. (23 %) I was able to share and understand the information with other facilities and departments. (15 %) I would like to discuss cases of adult DMD. (15 %) I want to learn more about other diseases. (10 %)
Other occupations	I became easier to consult about DMD. (47 %) I was able to learn the status of rehabilitation. (31 %) I was able to learn about daily life and appearance at school for DMD. (13 %) I was able to obtain information on both early and advanced conditions for DMD. (13 %) I would like to focus on the necessary matters of the case. (13 %) I want to learn more about other diseases. (12 %) I would like to discuss cases of adult DMD. (8 %)

DMD ; Duchenne muscular dystrophy.

for patients ($p<.001$), and the ease of grasping the status of other patients ($p<.001$) were significant **(Table 4)**.

In participants' free descriptions, which were concrete examples related to problem-solving, some opinions that were expressed included "becoming easier to consult" (47 %), "a policy of rehabilitation" (13 %), and "future treatment policies" (13%) **(Table 5)**. In addition, participants' free descriptions regarding whether other information about other occupations and facilities was useful included "status of rehabilitation" (31 %), "daily life and appearance at school" (13 %), and "information on both early and advanced conditions" (13 %) **(Table 5)**.

Ⅳ Discussion

In this study, questionnaires were used to examine the effectiveness of the Muscular Dystrophy Medical Network in Nagano, which has been working to treat DMD for six years. Based on the results of the questionnaires, it was found that participation in the Muscular Dystrophy Medical Network in Nagano made it feel easier to solve patients' problems. By participating in the activities of the network, participants could use information from other occupations to enhance their own level of care. This avenue of information-sharing was promoted because participants felt comfortable communicating with staff from other occupations and facilities through the use of the remote conferencing system. Furthermore, per the free description results of both questionnaires, problems relating to treatment policies and understanding the status of patients-in-charge are being solved. Particularly, physical therapists felt it was easier to grasp medical concepts, whereas others grasped the implementation contents of physical therapy.

Furthermore, there were suggestions for improvements to conference management, such as it being too arduous to use information because participants

do not have a patient, how to improve current conferences, and suggestions for new themes. These suggestions were related to how to continue the team's activities more effectively, thus engendering a feeling of successful collaboration among all participating occupations. Meanwhile, physical therapists are required to evaluate independently from doctors and others when conducting clinical trials ; hence, raising awareness about the evaluation and treatment for muscular disorders among physical therapists is important. Therefore, in this questionnaire survey, a specific question on the evaluation and treatment was set only for physical therapists, which helped us analyze the change in perception of the physical therapists. Moreover, the results showed that almost all physical therapists were able to understand the content and purpose of the evaluation and treatment better than before participation in this survey.

Furthermore, disparity in medical treatment for DMD in Nagano prefecture has been reduced because of effective utilization of medical treatment information between occupations and facilities. In the future, we will expand the other rare and intractable diseases that the Muscular Dystrophy Medical Network in Nagano addresses, and consider new themes to improve the quality of medical care by continuing activities and disseminating new findings.

In this study, we used a questionnaire only at the time of survey. With this method, there was a possibility that participants positively evaluated what they had learned in the 6-year activities of our network, and the bias could influence the results, at the time of survey ; this is a limitation of this study. Since we need to review the activities in this network, and to clarify the problem of developing further activities for future network efforts, we conducted the questionnaire survey in a way that members looked back to the past around the present moment. In fact, free descriptions, such as "I want to learn more about other diseases" and "I would like to discuss cases of adult DMD," were included in **Table 5**. We speculate that these opinions should be improved in the future.

Moreover, this questionnaire survey was conducted for a limited number of members, the recovery rate being 75 % and 68 % for physical therapists and other occupations, respectively ; therefore, there might be biases that opinions not reflected in the survey and negative opinions were hidden. We need to take into consideration that the opinions of all members were not reflected in the survey results, devise measures to address the issues written in the free entry section, and further improve the interests of the members.

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References

1) Bushby K, Finkel R, Birnkrant DJ, Case LE, Clemens PR, Cripe L, Kaul A, Kinnett K, McDonald C. Pandya S, Poysky J, Shapiro F, Tomezsko J, Constantin C : Diagnosis and management of Duchenne muscular dystrophy, part 1 : diagnosis, and pharmacological and psychosocial management. Lancet Neurol 9 : 77-93, 2010
2) Bushby K, Finkel R, Birnkrant DJ, Case LE, Clemens PR, Cripe L, Kaul A, Kinnett K, McDonald C, Pandya S, Poysky J, Shapiro F, Tomezsko J, Constantin C : Diagnosis and management of Duchenne muscular dystrophy, part 2 : implementation of multidisciplinary care. Lancet Neurol 9 : 177-189, 2010
3) Hoffman EP, Brown RH, Kunkel LM : Dystrophin : the protein product of the Duchenne muscular dystrophy locus. Cell 51 : 919-928, 1987
4) Fenichel GM, Florence JM, Pestronk A : Long-term benefit from prednisone therapy in Duchenne muscular dystrophy. Neurology 41 : 1874, 1991
5) Eagle M, Baudouin SV, Chandler C, Giddings DR, Bullock R, Bushby K : Survival in Duchenne muscular dystrophy : improvements in life expectancy since 1967 and the impact of home nocturnal ventilation. Neuromuscul Disord 12 :

926–929, 2002

6) Drachman DB, Toyka KV, Myer E : Prednisone in Duchenne muscular dystrophy. Lancet 304 : 1409–1412, 1974

7) Balaban B, Matthews DJ, Clayton GH, Carry T : Corticosteroid treatment and functional improvement in Duchenne muscular dystrophy : long–term effect. Am J Phys Med Rehabil 84 : 843–850, 2005

8) Angelini C, Pegoraro E, Turella E, Intino MT, Pini A, Costa C : Deflazacort in Duchenne dystrophy : study of long term effect. Muscle Nerve 17 : 386–391, 1994

9) DeSilva S, Drachman DB, Mellits D, Kuncl RW : Prednisone treatment in Duchenne muscular dystrophy : long–term benefit. Arch Neurol 44 : 818–822, 1987

10) Mercuri E, Coratti G, Messina S, Ricotti V, Baranello G, D'Amico A, Pera MC, Albamonte E, Sivo S, Mazzone ES, Armoldi MT, Fanelli L, De Sanctis R, Romeo DM, Vita GL, Battini R, Bertini E, Muntoni F, Pane M : Revised North Star Ambulatory Assessment for young boys with Duchenne muscular dystrophy. PLoS One 11 : e0160195, 2016

11) Hyde SA, Flùytrup I, Glent S, Kroksmark AK, Salling B, Steffensen BF, Werlauff U, Erlandsend M : A randomized comparative study of two methods for controlling Tendo Achilles contracture in Duchenne muscular dystrophy. Neuromuscul Disord 10 : 257–263, 2000

12) Brooke MH, Fenichel GM, Griggs RC, Mendell JR, Moxley R, Florence J, King WM, Pandya S, Robison J, Schierbecker J, Signore L, Miller JP, Gilder BF, Kaiser KK, Mandel S, Arfken C : Duchenne muscular dystrophy : patterns of clinical progression and effects of supportive therapy. Neurol 39 : 475, 1989

13) Scott OM, Hyde SA, Goddard CM, Dubowitz V : Prevention of deformity in Duchenne muscular dystrophy : a prospective study of passive stretching and splintage. Physiother 67 : 177–180, 1981

14) Vignos PJ, Wagner MB, Karlinchak B : Evaluation of a program for long–term treatment of Duchenne muscular dystrophy. J Bone Joint Surg Am 78 : 1844–1852, 1996

15) McKim DA, Road J, Avendano M, Abdool S, Côté F, Duguid N, Fraser J, Maltais F, Morrison DL, O'Connell C, Petrof BJ, Rimmer K, Skomro R, Canadian Thoracic Society : Home Mechanical Ventilation Committee. Home mechanical ventilation : a Canadian Thoracic Society clinical practice guideline. Can Respir J 18 : 197–215, 2011

16) Mullender MG, Blom NA, De Kleuver M, Fock JM, Hitters WMGC, Horemans AMC, Kalkman CJ, Pruijs JEH, Timmer RR, Titarsolej PJ, Van Haasteren NC, Van Tol–de Jager MJ, Van Vught AJ, Van Royen BJ : A Dutch guideline for the treatment of scoliosis in neuromuscular disorders. Scoliosis 3 : 1–14, 2008

17) Toussaint M, Davidson Z, Bouvoie V, Evenepoel N, Haan J, Soudon P : Dysphagia in Duchenne muscular dystrophy : practical recommendations to guide management. Disabil Rehabil 38 : 2052–2062, 2016

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