

THE 6-MINUTE WALK TEST AND OTHER CLINICAL ENDPOINTS IN DUCHENNE MUSCULAR DYSTROPHY: RELIABILITY, CONCURRENT VALIDITY, AND MINIMAL CLINICALLY IMPORTANT DIFFERENCES FROM A MULTICENTER STUDY

GRAIG M. McDONALD, MD,¹ ERIK K. HENRICSON, MPH,¹ R. TED ABRESCH, MS,¹ JULAINE FLORENCE, PhD,² MICHELLE EAGLE, PhD,³ EDUARD GAPPMAIER, PhD,⁴ AND ALLAN M. GLANZMAN, DPT,⁵ for the PFC124-GD-007-DMD STUDY GROUP,* ROBERT SPIEGEL, MD,⁶ JAY BARTH, MD,⁶ GARY ELFRING, MS,⁶ ALLEN REHA, MS,⁶ and STUART W. FELTZ, PhD⁷

¹Department of Physical Medicine and Rehabilitation, Neuromuscular Medicine and Rehabilitation Research Center, University of California Davis School of Medicine, Davis, California 95617, USA

²Department of Neurology, Washington University School of Medicine, St. Louis, Missouri, USA

³Institute of Genetic Medicine, Newcastle University, Newcastle upon Tyne, UK

⁴Department of Physical Therapy, University of Utah School of Medicine, Salt Lake City, Utah, USA

⁵Department of Physical Therapy, Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, USA

⁶PFC Therapeutics, South Plainfield, New Jersey, USA

⁷Accepted 7 May 2013

ABSTRACT: Introduction: An international clinical trial enrolled 174 ambulatory males ≥ 5 years old with nonsense mutation Duchenne muscular dystrophy (nmDMD). Pretreatment data provide insight into reliability, concurrent validity, and minimal clinically important differences (MCIDs) of the 6-minute walk test (6MWT) and other endpoints. Methods: Screening and baseline

evaluations included the 6-minute walk distance (6MWD), timed function tests (TFTs), quantitative strength by myometry, the PedsQL heart rate–determined energy expenditure index, and other exploratory endpoints. Results: The 6MWT proved feasible and reliable in a multicenter context. Concurrent validity with other endpoints was excellent. The MCID for 6MWD was 28.5 and 31.7 meters based on 2 statistical distribution methods. Conclusions: The ratio of MCID to baseline mean is lower for 6MWD than for other endpoints. The 6MWD is an optimal primary endpoint for Duchenne muscular dystrophy (DMD) clinical trials that are focused therapeutically on preservation of ambulation and slowing of disease progression.

Muscle Nerve 48: 357–368, 2013

Abbreviations: 6MWD, 6-minute walk distance; 6MWT, 6-minute walk test; ACS, American Thoracic Society; CNGR, Cooperative International Neuromuscular Research Group; CK, creatine kinase; DMD, Duchenne muscular dystrophy; EEI, energy expenditure index; HRQL, health-related quality of life; MCID, minimal clinically important difference; MPS, mucopolysaccharidosis; nmDMD, nonsense mutation DMD; NSAA, North Star Ambulatory Assessment; PedsQL, Pediatric Quality of Life Inventory; TFT, timed function test

Key words: 6-minute walk test; ambulation; Duchenne muscular dystrophy; energy expenditure index; muscular dystrophy; myometry; natural history; PedsQL; timed function test

The study was sponsored by PFC Therapeutics, Inc. (South Plainfield, New Jersey, USA), which was responsible for all aspects of the study, and by a grant from the FDA Office of Orphan Products.

Disclosures: G.M.M. is a member of the Cooperative Neuromuscular Research Group (CNGR) Executive Committee and has served on advisory committees for PFC Therapeutics, Inc., Sarepta Therapeutics, Inc., GlaxoSmithKline, Progenia, Halo Therapeutics, Shire HGT, Cardex Therapeutics, and Novartis AG. E.K.H. is a member of the CNGR Executive Committee and has served as a consultant for Genzyme Corporation and PFC Therapeutics, Inc. R.T.A. has served as a consultant for PFC Therapeutics, Inc., and Sarepta Therapeutics. J.M.F. serves on a scientific advisory board for Progenia, on the editorial board of *Neuromuscular Disorders*, and serves as a member of the CNGR Executive Committee and as a consultant for Progenia, GlaxoSmithKline, Genzyme Corporation, PFC Therapeutics, Inc., and Acceleron Pharma. M.E. has served as a clinical evaluator/trainer for PFC Therapeutics, and as a consultant for Progenia and GlaxoSmithKline. E.G. has served as a clinical evaluator/trainer for PFC Therapeutics. A.M.G. has served as a clinical evaluator/trainer for PFC Therapeutics. R.S. is the Chief Medical Officer for PFC Therapeutics. J.B. is the Vice President for Clinical Development at PFC Therapeutics. G.E. is the Executive Director of Biostatistics at PFC Therapeutics. A.R. is the Director of Clinical Development at PFC Therapeutics. S.W.F. is the Chief Executive Officer and founding scientist, PFC Therapeutics, Inc.

Additional Supporting Information may be found in the online version of this article.

*Collaborating authors are listed in Appendix 1. Other members of the PFC124-GD-007-DMD Study Group are listed in Appendix 2 in the Supporting Information.

Correspondence to: G.M. McDonald; e-mail: gmcDonald@ucdavis.edu

© 2013 Wiley Periodicals, Inc.
Published online 17 July 2013 in Wiley Online Library (wileyonlinelibrary.com). DOI 10.1002/mus.23805

Reliability, Validity and MCID of 6MWT and other Endpoints in DMD

Duchenne muscular dystrophy (DMD) is a disabling and life-threatening X-linked genetic disorder caused by defects in the gene for dystrophin, a protein that stabilizes muscle cell membranes.¹ DMD is characterized by complete loss of dystrophin and is the most common neuromuscular disease of childhood. It affects 1 in 3800–6300 males, and there are an estimated 15,000 patients with the disease in the USA.^{2,3} There is no approved therapy that addresses the underlying cause of DMD.^{4,5} In $\sim 13\%$ of boys with nonsense mutation DMD (nmDMD), which represents ~ 1700 boys in the USA and ~ 2400 boys in Europe), the causative defect in the dystrophin gene is a nonsense mutation that truncates dystrophin protein production by introducing a premature stop codon into dystrophin mRNA.^{6–8}

THE NEED FOR CLINICALLY MEANINGFUL ENDPOINTS IN DMD

Given that several novel approaches to treatment of DMD have shown promise in preclinical and/or proof-of-concept clinical studies,^{9–12} the research community has faced the need to identify and develop clinically meaningful outcome measures for use in pivotal therapeutic trials. In boys with DMD, walking abnormalities are a major disease manifestation that has great importance to patients and

Northstar 2015 Hm Study Guide

**Leonhard Schilbach,Danilo
Bzdok,Victoria Leong,Elizabeth
Redcay,Frieder Michel Paulus,Kevin A.
Pelphrey**

Northstar 2015 Hm Study Guide:

If you ally infatuation such a referred **Northstar 2015 Hm Study Guide** books that will have enough money you worth, get the definitely best seller from us currently from several preferred authors. If you want to comical books, lots of novels, tale, jokes, and more fictions collections are next launched, from best seller to one of the most current released.

You may not be perplexed to enjoy every book collections Northstar 2015 Hm Study Guide that we will no question offer. It is not in the region of the costs. Its roughly what you need currently. This Northstar 2015 Hm Study Guide, as one of the most committed sellers here will totally be in the middle of the best options to review.

https://crm.avenza.com/public/scholarship/Download_PDFS/psychology%20study%20guide%20germantownschools%20org.pdf

Table of Contents Northstar 2015 Hm Study Guide

1. Understanding the eBook Northstar 2015 Hm Study Guide
 - The Rise of Digital Reading Northstar 2015 Hm Study Guide
 - Advantages of eBooks Over Traditional Books
2. Identifying Northstar 2015 Hm Study Guide
 - Exploring Different Genres
 - Considering Fiction vs. Non-Fiction
 - Determining Your Reading Goals
3. Choosing the Right eBook Platform
 - Popular eBook Platforms
 - Features to Look for in an Northstar 2015 Hm Study Guide
 - User-Friendly Interface
4. Exploring eBook Recommendations from Northstar 2015 Hm Study Guide
 - Personalized Recommendations
 - Northstar 2015 Hm Study Guide User Reviews and Ratings
 - Northstar 2015 Hm Study Guide and Bestseller Lists

5. Accessing Northstar 2015 Hm Study Guide Free and Paid eBooks
 - Northstar 2015 Hm Study Guide Public Domain eBooks
 - Northstar 2015 Hm Study Guide eBook Subscription Services
 - Northstar 2015 Hm Study Guide Budget-Friendly Options
6. Navigating Northstar 2015 Hm Study Guide eBook Formats
 - ePub, PDF, MOBI, and More
 - Northstar 2015 Hm Study Guide Compatibility with Devices
 - Northstar 2015 Hm Study Guide Enhanced eBook Features
7. Enhancing Your Reading Experience
 - Adjustable Fonts and Text Sizes of Northstar 2015 Hm Study Guide
 - Highlighting and Note-Taking Northstar 2015 Hm Study Guide
 - Interactive Elements Northstar 2015 Hm Study Guide
8. Staying Engaged with Northstar 2015 Hm Study Guide
 - Joining Online Reading Communities
 - Participating in Virtual Book Clubs
 - Following Authors and Publishers Northstar 2015 Hm Study Guide
9. Balancing eBooks and Physical Books Northstar 2015 Hm Study Guide
 - Benefits of a Digital Library
 - Creating a Diverse Reading Collection Northstar 2015 Hm Study Guide
10. Overcoming Reading Challenges
 - Dealing with Digital Eye Strain
 - Minimizing Distractions
 - Managing Screen Time
11. Cultivating a Reading Routine Northstar 2015 Hm Study Guide
 - Setting Reading Goals Northstar 2015 Hm Study Guide
 - Carving Out Dedicated Reading Time
12. Sourcing Reliable Information of Northstar 2015 Hm Study Guide
 - Fact-Checking eBook Content of Northstar 2015 Hm Study Guide
 - Distinguishing Credible Sources
13. Promoting Lifelong Learning

- Utilizing eBooks for Skill Development
- Exploring Educational eBooks

14. Embracing eBook Trends

- Integration of Multimedia Elements
- Interactive and Gamified eBooks

Northstar 2015 Hm Study Guide Introduction

In today's digital age, the availability of Northstar 2015 Hm Study Guide books and manuals for download has revolutionized the way we access information. Gone are the days of physically flipping through pages and carrying heavy textbooks or manuals. With just a few clicks, we can now access a wealth of knowledge from the comfort of our own homes or on the go. This article will explore the advantages of Northstar 2015 Hm Study Guide books and manuals for download, along with some popular platforms that offer these resources. One of the significant advantages of Northstar 2015 Hm Study Guide books and manuals for download is the cost-saving aspect. Traditional books and manuals can be costly, especially if you need to purchase several of them for educational or professional purposes. By accessing Northstar 2015 Hm Study Guide versions, you eliminate the need to spend money on physical copies. This not only saves you money but also reduces the environmental impact associated with book production and transportation. Furthermore, Northstar 2015 Hm Study Guide books and manuals for download are incredibly convenient. With just a computer or smartphone and an internet connection, you can access a vast library of resources on any subject imaginable. Whether you're a student looking for textbooks, a professional seeking industry-specific manuals, or someone interested in self-improvement, these digital resources provide an efficient and accessible means of acquiring knowledge. Moreover, PDF books and manuals offer a range of benefits compared to other digital formats. PDF files are designed to retain their formatting regardless of the device used to open them. This ensures that the content appears exactly as intended by the author, with no loss of formatting or missing graphics. Additionally, PDF files can be easily annotated, bookmarked, and searched for specific terms, making them highly practical for studying or referencing. When it comes to accessing Northstar 2015 Hm Study Guide books and manuals, several platforms offer an extensive collection of resources. One such platform is Project Gutenberg, a nonprofit organization that provides over 60,000 free eBooks. These books are primarily in the public domain, meaning they can be freely distributed and downloaded. Project Gutenberg offers a wide range of classic literature, making it an excellent resource for literature enthusiasts. Another popular platform for Northstar 2015 Hm Study Guide books and manuals is Open Library. Open Library is an initiative of the Internet Archive, a non-profit organization dedicated to digitizing cultural artifacts and making them accessible to the public. Open Library hosts millions of books, including both public domain works and contemporary titles. It

also allows users to borrow digital copies of certain books for a limited period, similar to a library lending system. Additionally, many universities and educational institutions have their own digital libraries that provide free access to PDF books and manuals. These libraries often offer academic texts, research papers, and technical manuals, making them invaluable resources for students and researchers. Some notable examples include MIT OpenCourseWare, which offers free access to course materials from the Massachusetts Institute of Technology, and the Digital Public Library of America, which provides a vast collection of digitized books and historical documents. In conclusion, Northstar 2015 Hm Study Guide books and manuals for download have transformed the way we access information. They provide a cost-effective and convenient means of acquiring knowledge, offering the ability to access a vast library of resources at our fingertips. With platforms like Project Gutenberg, Open Library, and various digital libraries offered by educational institutions, we have access to an ever-expanding collection of books and manuals. Whether for educational, professional, or personal purposes, these digital resources serve as valuable tools for continuous learning and self-improvement. So why not take advantage of the vast world of Northstar 2015 Hm Study Guide books and manuals for download and embark on your journey of knowledge?

FAQs About Northstar 2015 Hm Study Guide Books

How do I know which eBook platform is the best for me? Finding the best eBook platform depends on your reading preferences and device compatibility. Research different platforms, read user reviews, and explore their features before making a choice. Are free eBooks of good quality? Yes, many reputable platforms offer high-quality free eBooks, including classics and public domain works. However, make sure to verify the source to ensure the eBook credibility. Can I read eBooks without an eReader? Absolutely! Most eBook platforms offer webbased readers or mobile apps that allow you to read eBooks on your computer, tablet, or smartphone. How do I avoid digital eye strain while reading eBooks? To prevent digital eye strain, take regular breaks, adjust the font size and background color, and ensure proper lighting while reading eBooks. What the advantage of interactive eBooks? Interactive eBooks incorporate multimedia elements, quizzes, and activities, enhancing the reader engagement and providing a more immersive learning experience. Northstar 2015 Hm Study Guide is one of the best book in our library for free trial. We provide copy of Northstar 2015 Hm Study Guide in digital format, so the resources that you find are reliable. There are also many Ebooks of related with Northstar 2015 Hm Study Guide. Where to download Northstar 2015 Hm Study Guide online for free? Are you looking for Northstar 2015 Hm Study Guide PDF? This is definitely going to save you time and cash in something you should think about. If you trying to find then search around for online. Without a doubt there are numerous these available and many of them have the freedom. However without doubt you receive whatever you purchase. An alternate way to get ideas is always to check another Northstar 2015 Hm Study Guide.

This method for see exactly what may be included and adopt these ideas to your book. This site will almost certainly help you save time and effort, money and stress. If you are looking for free books then you really should consider finding to assist you try this. Several of Northstar 2015 Hm Study Guide are for sale to free while some are payable. If you arent sure if the books you would like to download works with for usage along with your computer, it is possible to download free trials. The free guides make it easy for someone to free access online library for download books to your device. You can get free download on free trial for lots of books categories. Our library is the biggest of these that have literally hundreds of thousands of different products categories represented. You will also see that there are specific sites catered to different product types or categories, brands or niches related with Northstar 2015 Hm Study Guide. So depending on what exactly you are searching, you will be able to choose e books to suit your own need. Need to access completely for Campbell Biology Seventh Edition book? Access Ebook without any digging. And by having access to our ebook online or by storing it on your computer, you have convenient answers with Northstar 2015 Hm Study Guide To get started finding Northstar 2015 Hm Study Guide, you are right to find our website which has a comprehensive collection of books online. Our library is the biggest of these that have literally hundreds of thousands of different products represented. You will also see that there are specific sites catered to different categories or niches related with Northstar 2015 Hm Study Guide So depending on what exactly you are searching, you will be able to choose ebook to suit your own need. Thank you for reading Northstar 2015 Hm Study Guide. Maybe you have knowledge that, people have search numerous times for their favorite readings like this Northstar 2015 Hm Study Guide, but end up in harmful downloads. Rather than reading a good book with a cup of coffee in the afternoon, instead they juggled with some harmful bugs inside their laptop. Northstar 2015 Hm Study Guide is available in our book collection an online access to it is set as public so you can download it instantly. Our digital library spans in multiple locations, allowing you to get the most less latency time to download any of our books like this one. Merely said, Northstar 2015 Hm Study Guide is universally compatible with any devices to read.

Find Northstar 2015 Hm Study Guide :

~~psychology study guide germantownschools-org~~

punishing the coed brat english edition

purrfect protector shifter protection specialists inc volume 1

psychology frontiers and applications 4th canadian edition

public procurement reforms in africa challenges in institutions and governance

psychology david myers 8th edition

punishment room bdsm female domination male submission erotica english edition

~~puch magnum owners manual~~

~~psychology exam multiple choice~~

~~pure mathematics ii~~

psychology saundra k ciccarelli study guide

public universities joint admissions 2015 2016

punch recipe 20jelsoft enterprises ltd

puyallup school district salary schedule

pugel international economics 15th edition answers

Northstar 2015 Hm Study Guide :

Services Marketing: People, Technology, Strategy Services Marketing: People, Technology, Strategy. 7th Edition. ISBN-13: 978-0136107217, ISBN-10: 0136107214. 4.1 out of 5 stars 109 Reviews. 4.1 on ... Services Marketing (7th Edition) by Lovelock, Christopher ... Written on a 5th grade level, with cases that are out of date, and dated. the author is very verbose, and repetitive, its for an introductory freshmen level ... Services Marketing: Integrating Customer Focus Across ... The seventh edition maintains a managerial focus by incorporating company examples and strategies for addressing issues in every chapter, emphasizing the ... Services Marketing: People, Technology, Strategy, 7th edition Oct 31, 2023 — An examination of the relationship between the key elements of the services marketing management model (internal and external marketing, ... Services Marketing: People, Technology, Strategy, 7th ... This globally leading textbook extensively updated to feature the latest academic research, industry trends, and technology, social media and case examples. Services Marketing 7th edition 9781260083521 Services Marketing 7th Edition is written by Valarie Zeithaml; Mary Jo Bitner; Dwayne Gremler and published by McGraw-Hill Higher Education (International). Services Marketing, Global Edition Services Marketing, Global Edition, 7th edition. Published by Pearson ... Services Marketing, Global Edition. Published 2015. Paperback. £76.99. Buy now. Free ... Services Marketing: Integrating Customer Focus Across ... The seventh edition maintains a managerial focus by incorporating company examples and strategies for addressing issues in every chapter, emphasizing the ... Services Marketing: People, Technology, ... Services Marketing: People, Technology, Strategy, by Lovelock, 7th Edition by Jochen Wirtz, Christopher H Lovelock - ISBN 10: 0136107249 - ISBN 13: ... Services Marketing 7th edition 9780078112102 0078112109 Rent Services Marketing 7th edition (978-0078112102) today, or search our site for other textbooks by Zeithaml. Every textbook comes with a 21-day "Any ... Kit Road Ranger Travel Trailer Manual | Semer From Sun to Snow, we definitely want to help! Results for kit companion travel trailer owners manual High Speed Direct Downloads. Wildwood delivers a wide. RV MANUALS - Good Old RVs Hello everyone. Just got my 1979 leocraft motorhome

and joined this fine group. I am in search of a repair manual. Any info will be very helpful. Old RV Owners Manuals: Tips and Tricks on How to Find ... Apr 28, 2020 — In this post, we'll give you the insider secrets to finding old motorhome and travel trailer manuals online in case you need to look up ... 1966 Kit Companion Trailer 1966 Kit Companion Trailer ... I am trying to identify the year, make, and model of the TT pictured below. I think the logo says "Kit Companion", but I'm not sure ... Where to Find Old RV Owners Manuals Online? Sep 30, 2020 - Find old RV owners manuals online. Access valuable resources for your vintage RV or travel trailer and know all about them. Skip the Books, Find Your Handy RV Owners Manuals Online Dec 4, 2022 — In many cases, you can find your RV owners manuals online. Read on as we take a closer look at how and where to find your RVs owners manual. How ... Vintage Trailer Manuals Mar 18, 2021 — I am having trouble locating an owners manual for a 1967 Cardinal Deluxe Travel Trailer. ... Looking for a manual for an '87 Kit Companion. Need ... Companion Series Companion 24GT (*) Specs - 1996 Kit 1996 Kit Companion Series Companion 24GT (*) Specs and Standard Equipment | J.D. Power. Can't Find Your Old RV Owner's Manual? Try These Tips May 4, 2022 — We put together a list of the best places to find old RV owner's manuals online. But some RV manuals can be tougher to track down than others! Kit Manufacturing Co. Kit opened a new RV manufacturing facility in Caldwell in 1995, about three miles from the plant producing manufactured homes and the Road Ranger and Companion ... Clustering | Introduction, Different Methods and Applications Clustering | Introduction, Different Methods and Applications Cluster analysis Cluster analysis or clustering is the task of grouping a set of objects in such a way that objects in the same group (called a cluster) are more similar (in ... What is cluster analysis? Overview and examples Cluster analysis is a statistical method for processing data. It works by organizing items into groups - or clusters - based on how closely associated they are. A Comprehensive Guide to Cluster Analysis Cluster Analysis is a useful tool for identifying patterns and relationships within complex datasets and uses algorithms to group data points into clusters. Cluster Analysis - Methods, Applications, and Algorithms What is cluster analysis? Cluster analysis is a data analysis technique that explores the naturally occurring groups within a data set known as clusters. What is Cluster Analysis in Marketing? | Adobe Basics Mar 26, 2021 — Cluster analysis in marketing refers to the practice of analyzing shared characteristics between groups and comparing them. Conduct and Interpret a Cluster Analysis The Cluster Analysis is an explorative analysis that tries to identify structures within the data. Cluster analysis is also called segmentation analysis. Cluster Analysis - What Is It and Why Does It Matter? Cluster analysis is the grouping of objects based on their characteristics such that there is high intra-cluster similarity and low inter-cluster ... What is Cluster Analysis? What is Cluster Analysis? • Cluster: a collection of data objects. - Similar to one another within the same cluster. - Dissimilar to the objects in other ... Statistics: 3.1 Cluster Analysis 1 Introduction 2 Approaches to ... Cluster analysis is a multivariate method which aims to classify a sample of subjects (or ob- jects) on the basis of a set of measured variables into a ...