

Neuromuscular disorders : NMD.  
DJP - General Collection  
W1 NE337GB  
v. 12, suppl. 1  
Oct. 2002



# Neuromuscular Disorders

Supplement

**ENMC CENTENNIAL WORKSHOP ON THERAPEUTIC  
POSSIBILITIES IN DUCHENNE MUSCULAR DYSTROPHY**

Naarden, The Netherlands, 30 November-2 December, 2001

Guest Editor: V Dubowitz

Editor-in-Chief

**V Dubowitz *UK***

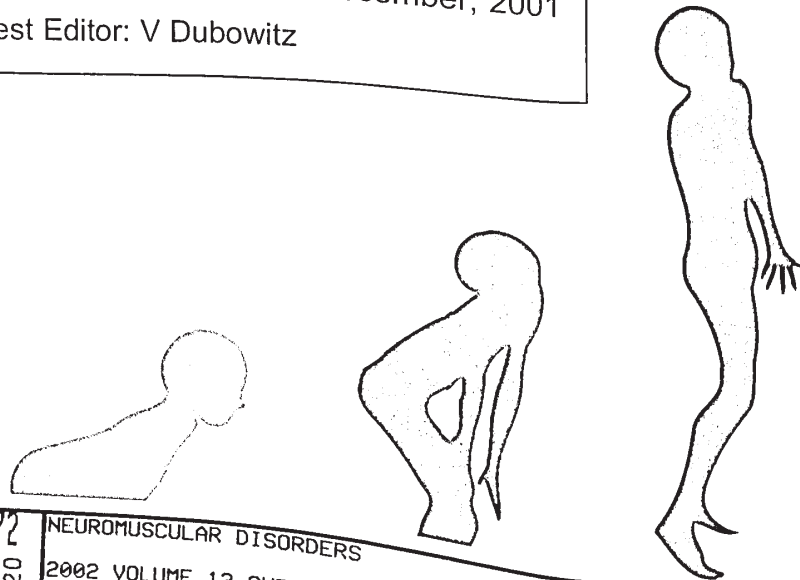
Associate Editors

**A G Engel *USA***

**E P Hoffman *USA***

**L Merlini *Italy***

**F M S Tomé *France***



040572 NEUROMUSCULAR DISORDERS  
2002 VOLUME 12 SUPPLEMENT  
SISAC  
0960-8966(2002)12+;1-N  
S219869

00206243290120  
SWETS

88776239



PERGAMON

This material is available at the NLM and may be subject to US Copyright Laws

Official Journal of the World Muscle Society

© 2002, Elsevier Science B.V. All rights reserved

This journal and the individual contributions contained in it are protected under copyright by Elsevier Science B.V., and the following terms and conditions apply to their use:

#### **Photocopying**

Single photocopies of single articles may be made for personal use as allowed by national copyright laws. Permission of the Publisher and payment of a fee is required for all other photocopying, including multiple or systematic copying, copying for advertising or promotional purposes, resale, and all forms of document delivery. Special rates are available for educational institutions that wish to make photocopies for non-profit educational classroom use.

Permissions may be sought directly from Elsevier Science via their homepage (<http://www.elsevier.com>) by selecting "Customer support" and then "Permissions". Alternatively you can send an e-mail to: [permissions@elsevier.co.uk](mailto:permissions@elsevier.co.uk), or fax to: (+44) 1865 853333.

In the USA, users may clear permissions and make payment through the Copyright Clearance Center, Inc., 222 Rosewood Drive, Danvers, MA 01923, USA; phone: (+1) (978) 7508400, fax: (+1) (978) 7504744, and in the UK through the Copyright Licensing Agency Rapid Clearance Service (CLARCS), 90 Tottenham Court Road, London W1P 0LP, UK; phone: (+44) 20 7631 5555; fax: (+44) 20 7631 5500. Other countries may have a local reprographic rights agency for payments.

#### **Derivative Works**

Subscribers may reproduce tables of contents or prepare lists of articles including abstracts for internal circulation within their institutions. Permission of the Publisher is required for resale or distribution outside the institution.

Permission of the Publisher is required for all other derivative works, including compilations and translations.

#### **Electronic Storage or Usage**

Permission of the Publisher is required to store or use electronically any material contained in this journal, including any article or part of an article.

Except as outlined above, no part of this publication may be reproduced, stored in a retrieval system or transmitted in any form or by any means, electronic, mechanical, photocopying, recording or otherwise, without prior written permission of the Publisher.

Address permissions requests to: Elsevier Science Global Rights Department, at the mail, fax and e-mail addresses noted above.

#### **Notice**

No responsibility is assumed by the Publisher for any injury and/or damage to persons or property as a matter of products liability, negligence or otherwise, or from any use or operation of any methods, products, instructions or ideas contained in the material herein. Because of rapid advances in the medical sciences, in particular, independent verification of diagnoses and drug dosages should be made.

Although all advertising material is expected to conform to ethical (medical) standards, inclusion in this publication does not constitute a guarantee or endorsement of the quality or value of such product or of the claims made of it by its manufacturer.

⊗ The paper used in this publication meets the requirements of ANSI/NISO Z39.48-1992 (Permanence of Paper).

Printed in The United Kingdom

---

#### **Author enquiries**

For enquiries relating to the submission of articles (including electronic submission), the status of accepted articles through our Online Article Status Information System (OASIS), author Frequently Asked Questions and any other enquiries relating to Elsevier Science, please consult <http://www.elsevier.com/locate/authors/>

For specific enquiries on the preparation of electronic artwork, consult <http://www.elsevier.com/locate/authorartwork/>

Contact details for questions arising after acceptance of an article, especially those relating to proofs, are provided when an article is accepted for publication.



# Neuromuscular Disorders

Editor-in-Chief

**V Dubowitz**

**Volume 12 Supplement 1 (2002)**

## **Therapeutic Possibilities in Duchenne Muscular Dystrophy**

Proceedings of the Special Centennial International ENMC Workshop

Naarden, The Netherlands, 30 November–2 December 2001

Guest Editor:

**Victor Dubowitz**



PERGAMON

# Neuromuscular Disorders

Volume 12 Suppl. 1

October 2002

Cited in: *Current Contents/Life Sciences, Elsevier BIOBASE/Current Awareness in Biological Sciences, Index Medicus, MEDLINE, Neuroscience Citation Index, Reference Update, Research Alert, Science Citation Index, SciSearch*

## CONTENTS

### Foreword

V. Dubowitz

### Myoblast transplantation

T. Partridge

### Myogenic stem cells from the bone marrow: a therapeutic alternative for muscular dystrophy?

G. Ferrari, F. Mavilio

### Gene transfer studies in animals: what do they really tell us about the prospects for gene therapy in DMD?

D.J. Wells, K.E. Wells

### Viral vectors for gene transfer of micro-, mini-, or full-length dystrophin

J.M. Scott, S. Li, S.Q. Harper, R. Welikson, D. Bourque, C. DelloRusso, S.D. Hauschka, J.S. Chamberlain

### Strategies for muscle-specific targeting of adenoviral gene transfer vectors

C. Thirion, N. Laroche, C. Volpers, P. Dunant, R. Stucka, P. Holland, J. Nalbantoglu, S. Kochanek, H. Lochmüller

### Recombinant micro-genes and dystrophin viral vectors

G. Dickson, M.L. Roberts, D.J. Wells, S.A. Fabb

### Current protocol of a research phase I clinical trial of full-length dystrophin plasmid DNA in Duchenne/Becker muscular dystrophies Part II: clinical protocol

N.B. Romero, O. Benveniste, C. Payan, S. Braun, P. Squiban, S. Herson, M. Fardeau

### Current protocol of a research phase I clinical trial of full-length dystrophin plasmid DNA in Duchenne/Becker muscular dystrophies Part I: rationale

C. Thioudellet, S. Blot, P. Squiban, M. Fardeau, S. Braun

### Current protocol of a research phase I clinical trial of full-length dystrophin plasmid DNA in Duchenne/Becker muscular dystrophies Part III. Ethical considerations

M. Fardeau

### Oligonucleotide-mediated gene therapy for muscular dystrophies

T.A. Rando

### CD45 fraction bone marrow cells as potential delivery vehicles for genetically corrected dystrophin loci

R.M.I. Kapsa, S.H.A. Wong, I. Bertonecello, A.F. Quigley, B. Williams, K. Sells, R. Marotta, M. Kita, P. Simmons, E. Byrne, A.J. Kornberg

### Screening for antisense modulation of dystrophin pre-mRNA splicing

G. Dickson, V. Hill, I.R. Graham

### Targeted exon skipping as a potential gene correction therapy for Duchenne muscular dystrophy

A. Aartsma-Rus, M. Bremmer-Bout, A.A.M. Janson, J.T. den Dunnen, G.-J.B. van Ommen, J.C.T. van Deutekom

### The role of utrophin in the potential therapy of Duchenne muscular dystrophy

K.J. Perkins, K.E. Davies

### Multivariate evaluation of the functional recovery obtained by the overexpression of utrophin in skeletal muscles of the *mdx* mouse

J.-M. Gillis

### Glucocorticoid-mediated regulation of utrophin levels in human muscle fibers

I. Courdier-Fruh, L. Barman, A. Briguet, T. Meier

### Dystrophin and functionally related proteins in the nematode *Caenorhabditis elegans*

L. Ségalat

### Dystrobrevin dynamics in muscle-cell signaling: a possible target for therapeutic intervention in Duchenne muscular dystrophy?

D.J. Blake

### Expression profiling in stably regenerating skeletal muscle of dystrophin-deficient *mdx* mice

J.M. Boer, E.J. de Meijer, E.M. Mank, G.B. van Ommen, J.T. den Dunnen

### A web-accessible complete transcriptome of normal human and DMD muscle

M. Bakay, P. Zhao, J. Chen, E.P. Hoffman

### Pre-clinical trials in Duchenne dystrophy: what animal models can tell us about potential drug effectiveness

A. De Luca, S. Pierno, A. Liantonio, D. Conte Camerino

**Collaborative translational research leading to multicenter clinical trials in Duchenne muscular dystrophy: the Cooperative International Neuromuscular Research Group (CINRG)**

D.M. Escolar, E.K. Henricson, L. Pasquali, K. Gorni, E.P. Hoffman S147

**Pharmacological control of cellular calcium handling in dystrophic skeletal muscle**

U.T. Ruegg, V. Nicolas-Métral, C. Challet, K. Bernard-Hélary, O.M. Dorchies, S. Wagner, T.M. Buetler S155

**Steroids in Duchenne muscular dystrophy: from clinical trials to genomic research**

F. Muntoni, I. Fisher, J.E. Morgan, D. Abraham S162

**Considerations to the policy of future clinical therapeutic trials in DMD**

B. Reitter S166

**An effective, low-dosage, intermittent schedule of prednisolone in the long-term treatment of early cases of Duchenne dystrophy**

M. Kinali, E. Mercuri, M. Main, F. Muntoni, V. Dubowitz S169

# Explore Litigation Insights

Docket Alarm provides insights to develop a more informed litigation strategy and the peace of mind of knowing you're on top of things.

## Real-Time Litigation Alerts



Keep your litigation team up-to-date with **real-time alerts** and advanced team management tools built for the enterprise, all while greatly reducing PACER spend.

Our comprehensive service means we can handle Federal, State, and Administrative courts across the country.

## Advanced Docket Research



With over 230 million records, Docket Alarm's cloud-native docket research platform finds what other services can't. Coverage includes Federal, State, plus PTAB, TTAB, ITC and NLRB decisions, all in one place.

Identify arguments that have been successful in the past with full text, pinpoint searching. Link to case law cited within any court document via Fastcase.

## Analytics At Your Fingertips



Learn what happened the last time a particular judge, opposing counsel or company faced cases similar to yours.

Advanced out-of-the-box PTAB and TTAB analytics are always at your fingertips.

## API

Docket Alarm offers a powerful API (application programming interface) to developers that want to integrate case filings into their apps.

## LAW FIRMS

Build custom dashboards for your attorneys and clients with live data direct from the court.

Automate many repetitive legal tasks like conflict checks, document management, and marketing.

## FINANCIAL INSTITUTIONS

Litigation and bankruptcy checks for companies and debtors.

## E-DISCOVERY AND LEGAL VENDORS

Sync your system to PACER to automate legal marketing.