

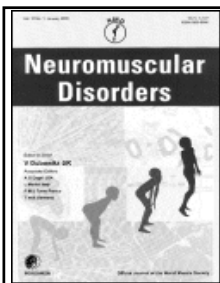
Bosisio Parini, 9 ottobre 2008

RECENTI PROGRESSI NEL TRATTAMENTO DELL'INSUFFICIENZA RESPIRATORIA DI ORIGINE NEURO- MUSCOLARE

Andrea Vianello
U.O. Fisiopatologia Respiratoria
Ospedale-Università di Padova

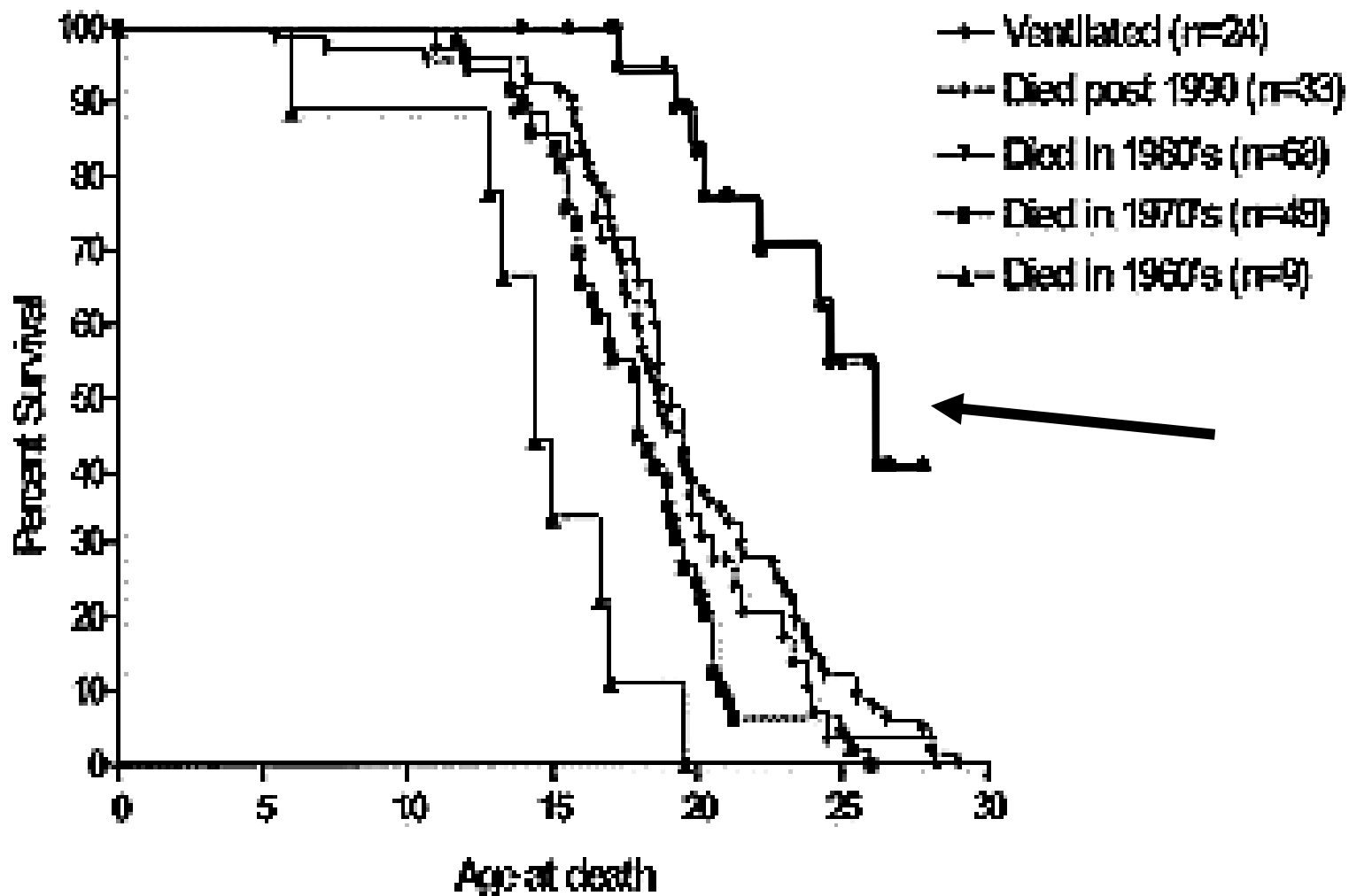
Probability of Respiratory Failure

- Inevitable: **Duchenne muscular dystrophy**
Type I Spinal muscular atrophy (SMA)
Motor Neuron Disease (MND-ALS)
- Frequent: Limb girdle MD 2C,2D,2F,2I
Nemaline myopathy
Int SMA
Acid maltase deficiency
X linked myotubular myopathy
Multicore myopathy
Congenital myasthenia
Congenital myotonic dystrophy
- Occasional: Emery Dreifuss MD, Becker MD, Bethlem myopathy, Minicore, central core myopathy
- Uncommon: Facioscapulohumeral MD, Mitochondrial myopathy, Limb girdle MD 1, 2A,B,G,H



Survival in Duchenne muscular dystrophy: improvements in life expectancy since 1967 and the impact of home nocturnal ventilation

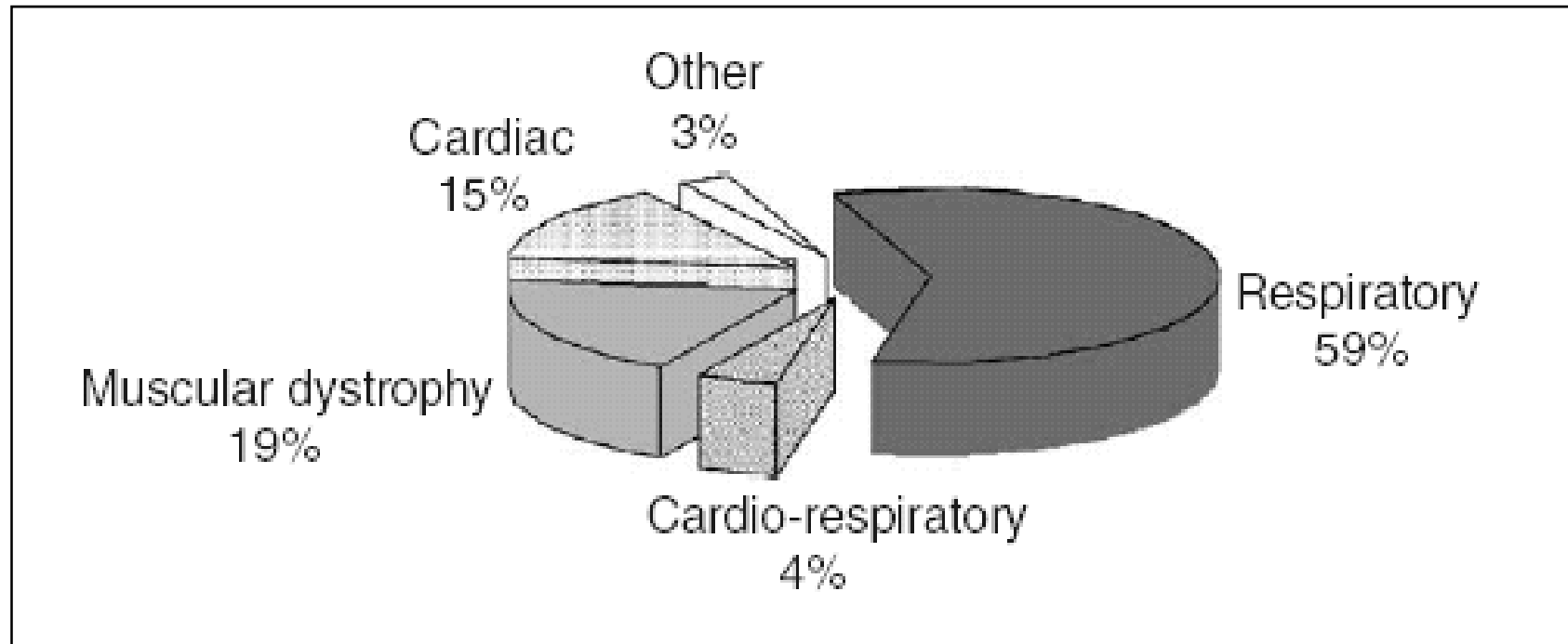
EAGLE, S.V., BAUDOIN, C. CHANDLER, D.R. GIDDINGS, R. BULLOCK, K. BUSHBY





Trends in survival from muscular dystrophy in England and Wales and impact on respiratory services.

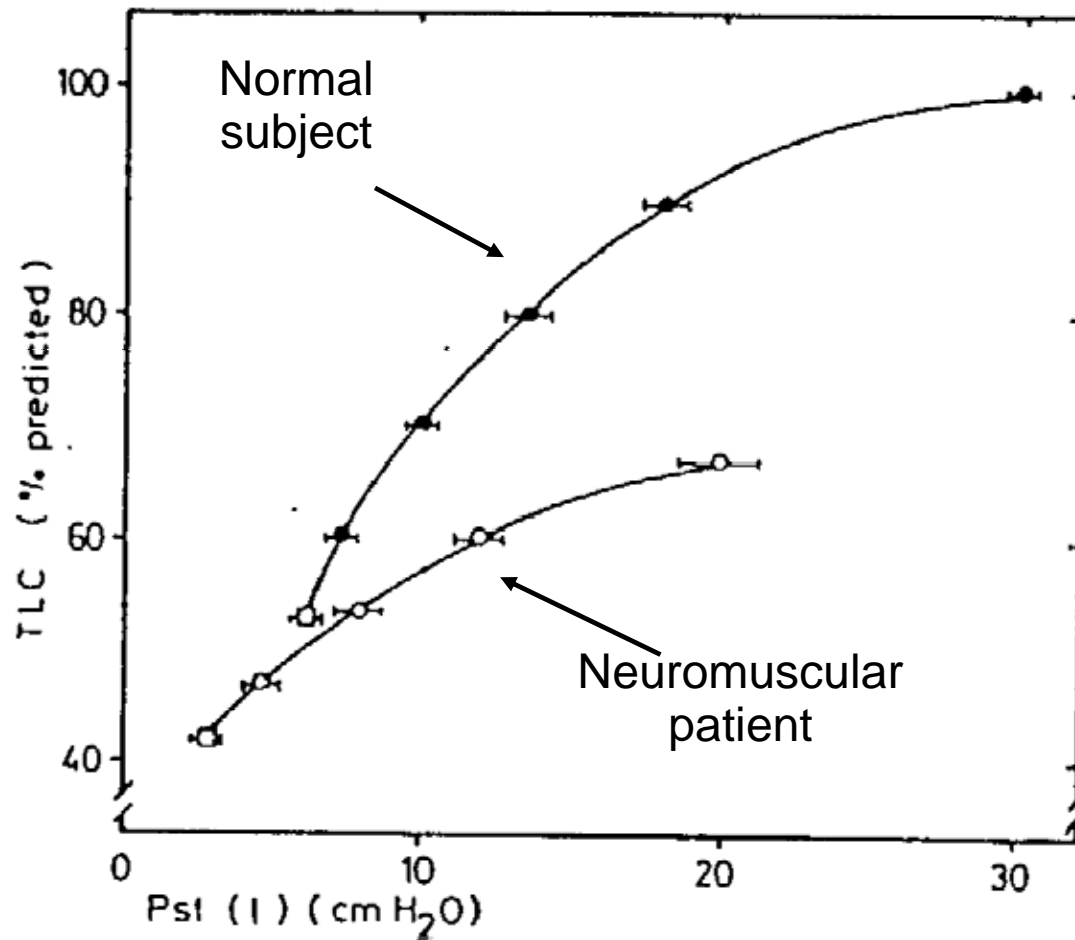
L.D. CALVERT, T.M. MC KEEVER, W.J.M. KINNEAR, J.R. BRITTON



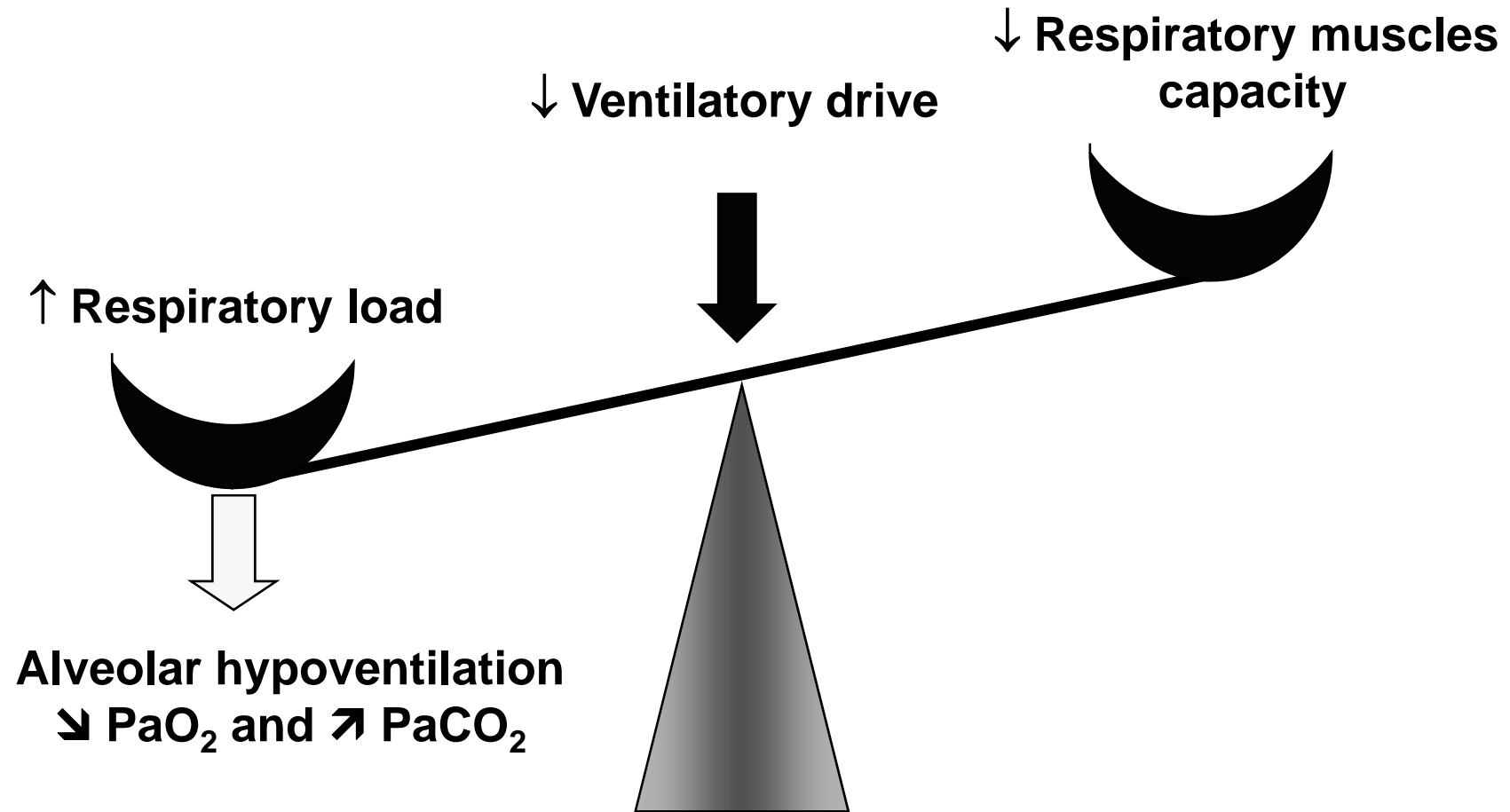
Cause of death in muscular dystrophy in England and Wales 1993–1999

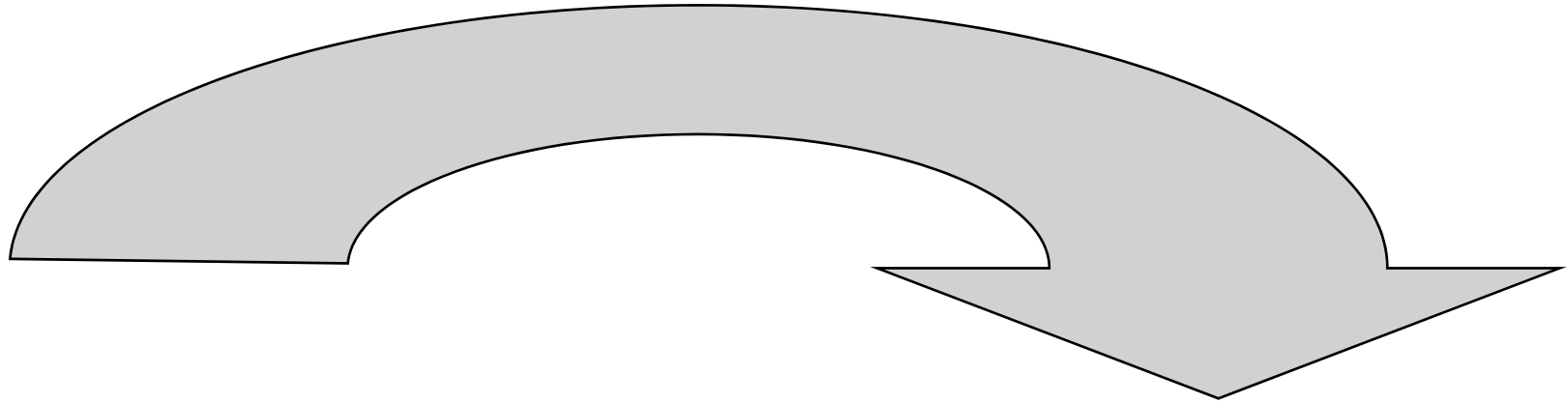
Analysis of lung volume restriction in patients with respiratory muscle weakness

A. DE TROYER, S. BORENSTEIN, AND R. CORDIER



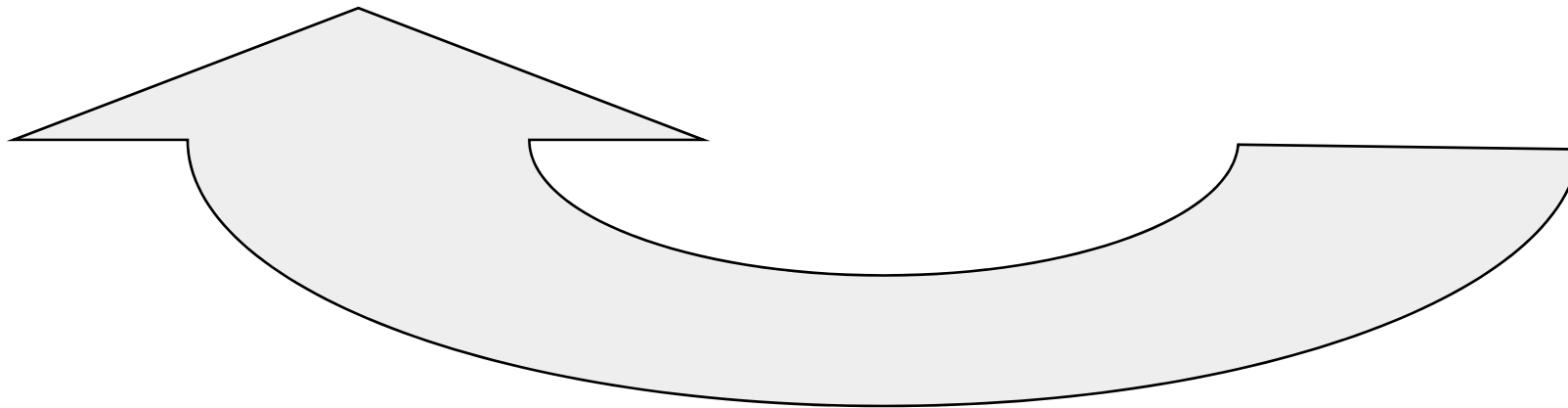
PHYSIOPATHOLOGY OF HYPOVENTILATION

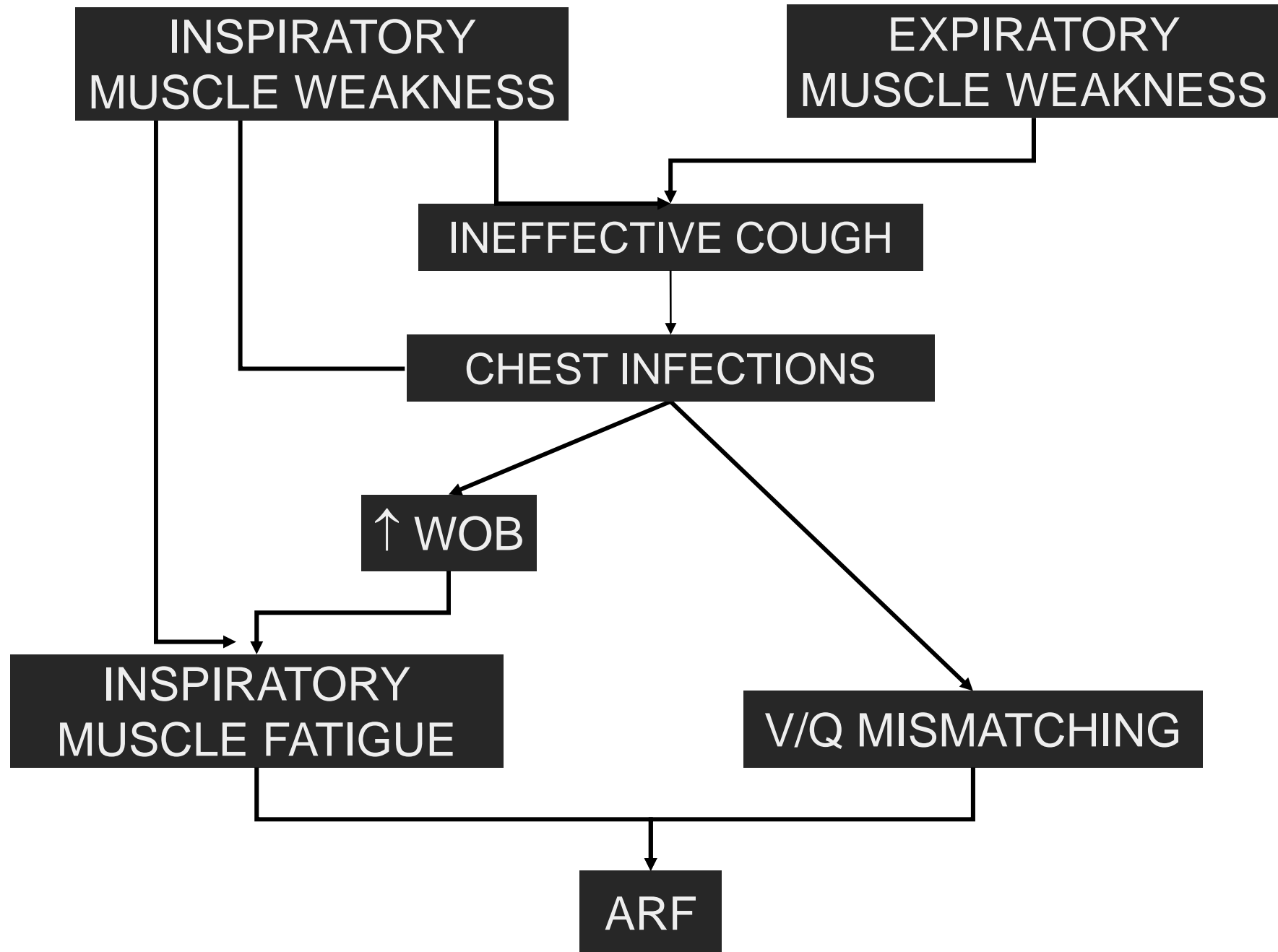




**SLEEP
DISRUPTION**

**RESPIRATORY
FAILURE**







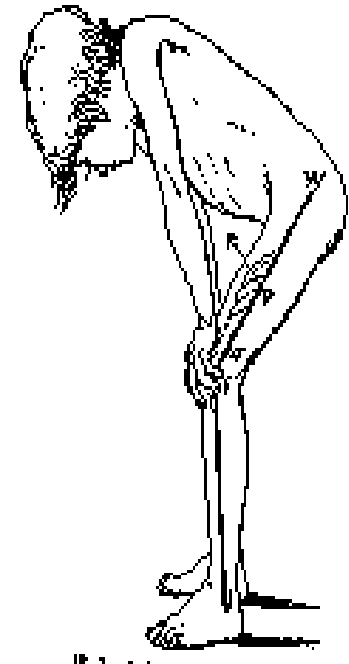
Corticosteroid Treatment and Functional Improvement in Duchenne Muscular Dystrophy

Balaban B, Matthews DJ, Clayton GH, Carry T:

Design: Retrospective medical record review.

Patients: A total of 49 boys with DMD, between the age of 12 and 15, observed over a 7-yr period. Eighteen had been treated with prednisone, 12 with deflazacort, and 19 had no drug treatment.

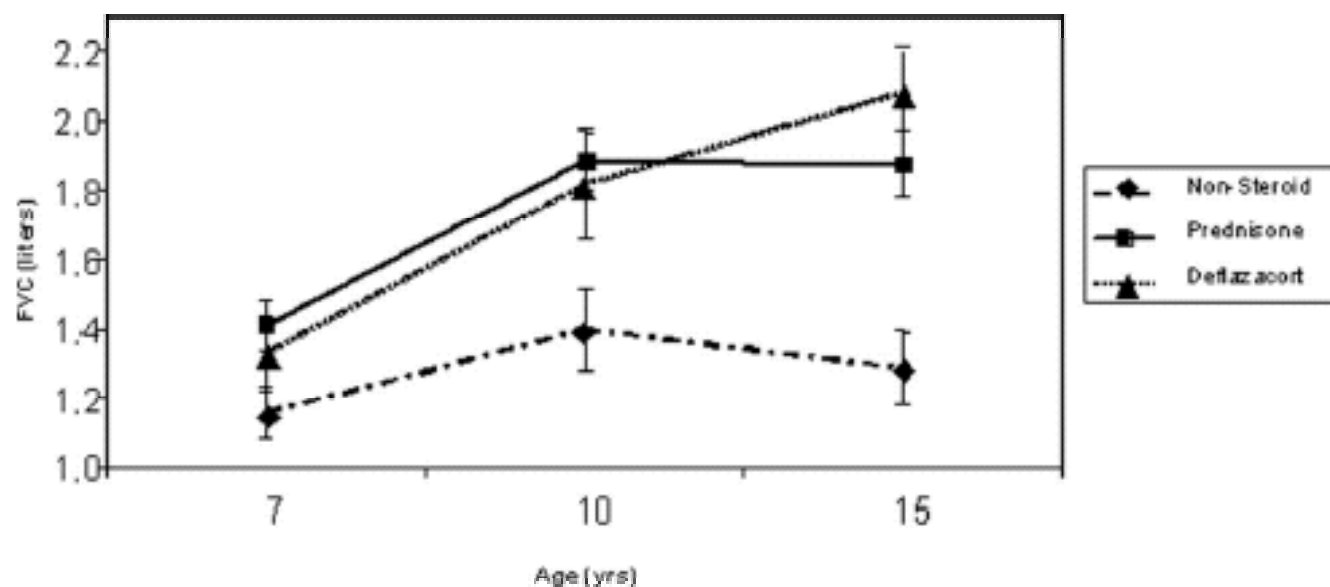
Measurements: Lower and upper limb motor functions, pulmonary function, prevalence of surgery for scoliosis.





Corticosteroid Treatment and Functional Improvement in Duchenne Muscular Dystrophy

Balaban B, Matthews DJ, Clayton GH, Carry T:



The control group capacity was decreasing slowly and significantly less both in prednisone and deflazacort treated



Effect of Long-term Steroids on Cough Efficiency and Respiratory Muscle Strength in Patients With Duchenne Muscular Dystrophy

Ameet S. Daftary, MBBS^a, Mark Crisanti, PhD^b, Maninder Kalra, MD^a, Brenda Wong, MD^c, Raouf Amin, MD^a

TABLE 1 Descriptive Statistics for the Steroid-Treated and Untreated Groups

Measure	Steroid-Treated Group (n = 10)	Untreated Group (n = 25)
Age, y	10.0 (8.0–15.0)	13.0 (10.0–17.0)
FVC, L	2.02 (1.66–2.13)	1.73 (1.06–2.09)
MEP, cm H ₂ O ^a	62.5 (54.5–75.5)	44.5 (37.0–59.0)
MIP, cm H ₂ O	63.0 (58.0–85.0)	57.0 (31.0–77.0)
MMV, L/min	56.5 (47.5–75.5)	49.0 (44.0–64.0)
PCF, L/min ^a	215.0 (160.0–250.0)	177.5 (147.5–215.0)

Data are presented as median (interquartile range).

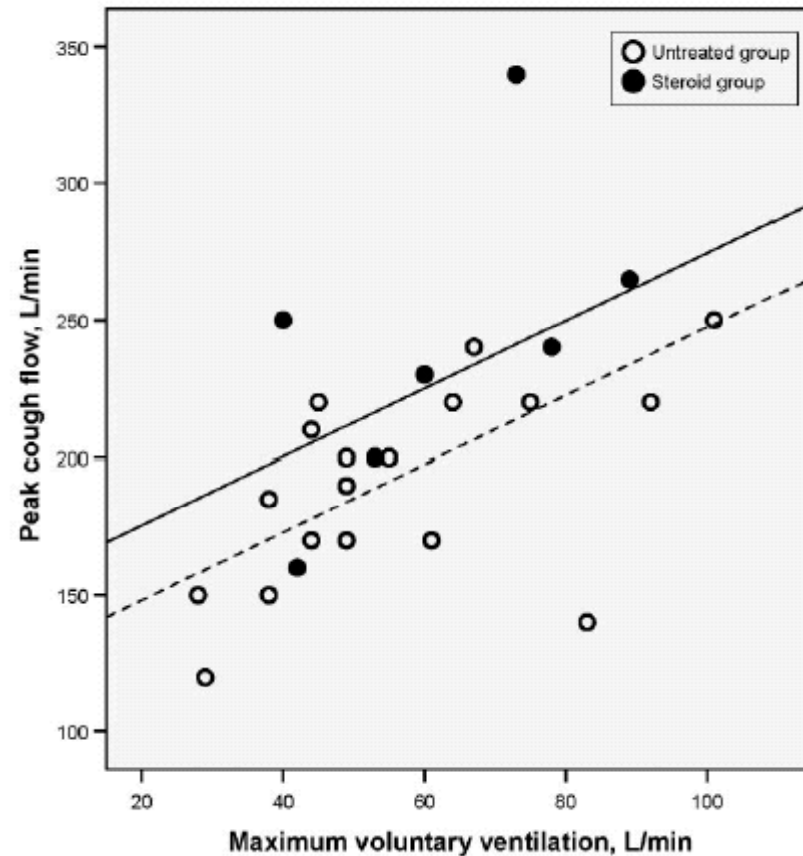
^a Difference in these variables was statistically significant at $P < .05$.

Prednisone was started at a dosage of 0.75 mg/kg per day and deflazacort at 0.9 mg/kg per day. As patients grew, the dosage was not increased for weightgain but clinically adjusted for optimal motor function.



Effect of Long-term Steroids on Cough Efficiency and Respiratory Muscle Strength in Patients With Duchenne Muscular Dystrophy

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Peak cough flow and maximum expiratory pressure were significantly higher in the steroid treated patients.

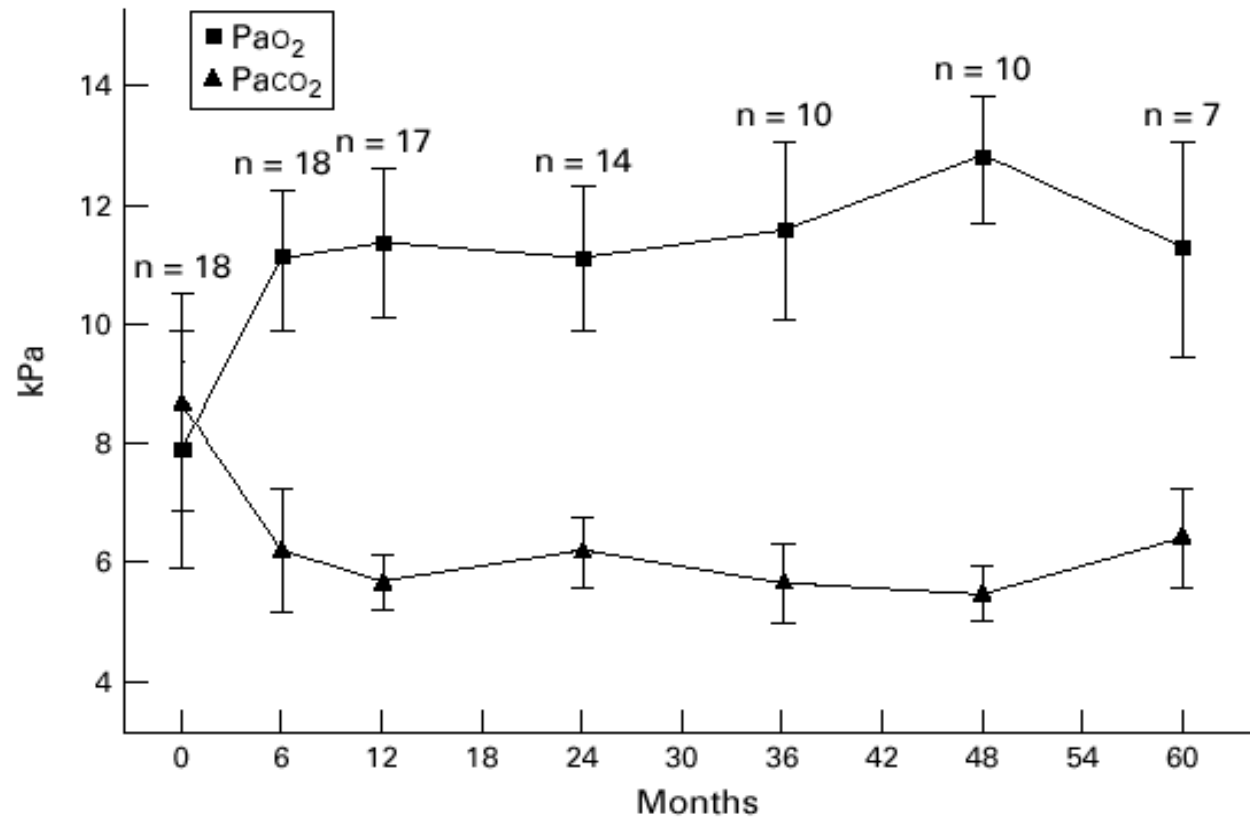
Topics

- Outcome of long-term VDP
- Timing of introduction of MV
- Quality of life

OUTCOME OF LONG-TERM VDP

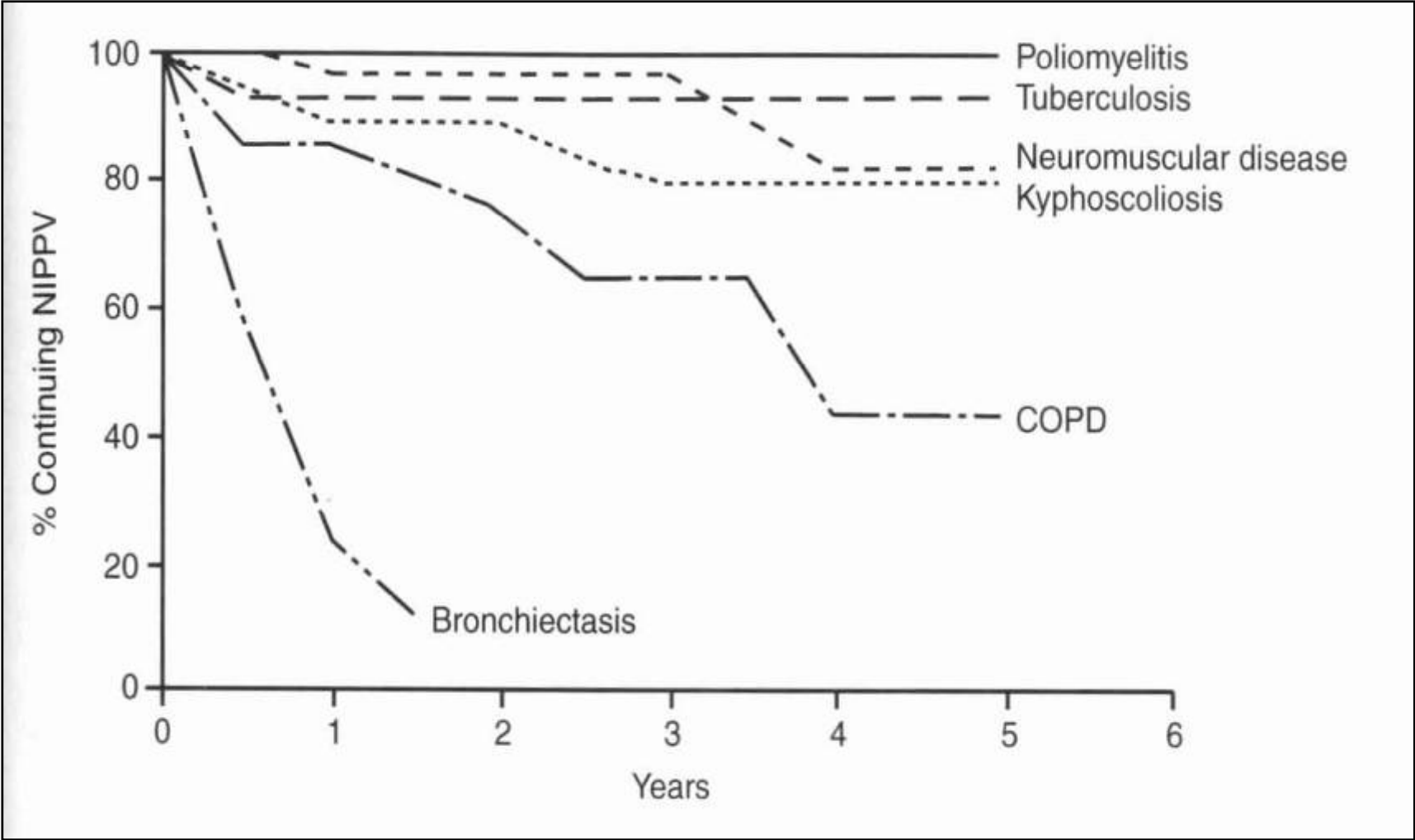
Impact of nasal ventilation on survival in hypercapnic Duchenne muscular dystrophy

A K Simonds, F Muntoni, S Heather, S Fielding



Thorax 1998;**53**:949–952 949

Survival: Probability of continuing domiciliary NIV





Long-term nasal intermittent positive pressure ventilation in advanced Duchenne's muscular dystrophy

A. VIANELLO, M. BEVILACQUA, V. SALVADOR, C. CARDAIOLI, E. VINCENTI

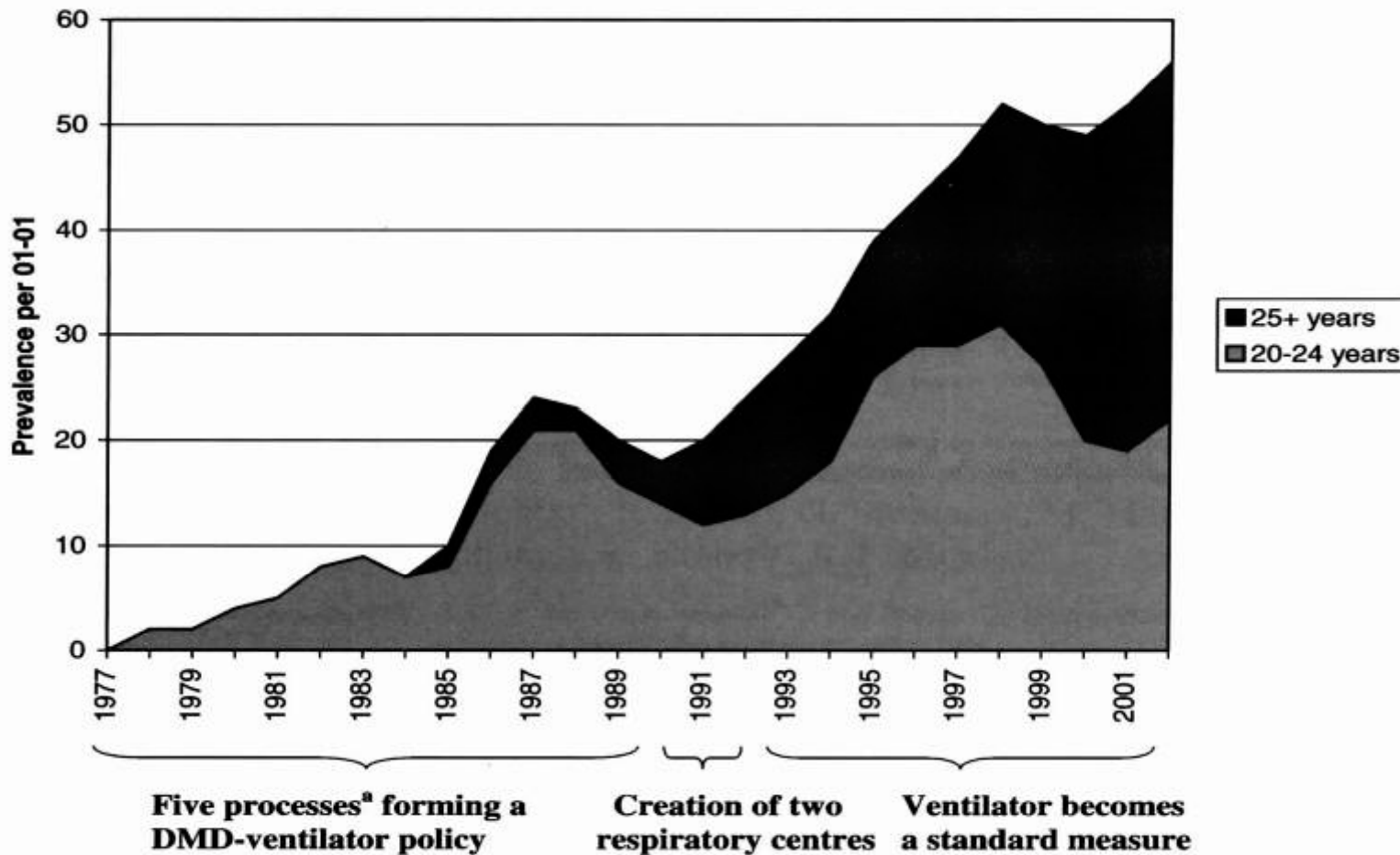
HMV IN ADVANCED DUCHENNE'S MUSCULAR DYSTROPHY

- 5 pts treated with NPPV
- 5 unventilated control pts

24 month follow-up

All pts treated with NPPV were still alive; four of five pts who underwent simple conservative treatment had died (mean survival: 9.7 ± 5.8 months)

Management trends in DMD



Jeppesen J Neuromusc Dis 2003;13:804-12

Annane D, Chevrolet JC, Chevret S, Raphael JC

Nocturnal mechanical ventilation for chronic hypoventilation in patients with neuromuscular and chest wall disorders.

Cochrane Database of Systematic Reviews. Issue 1, 2001

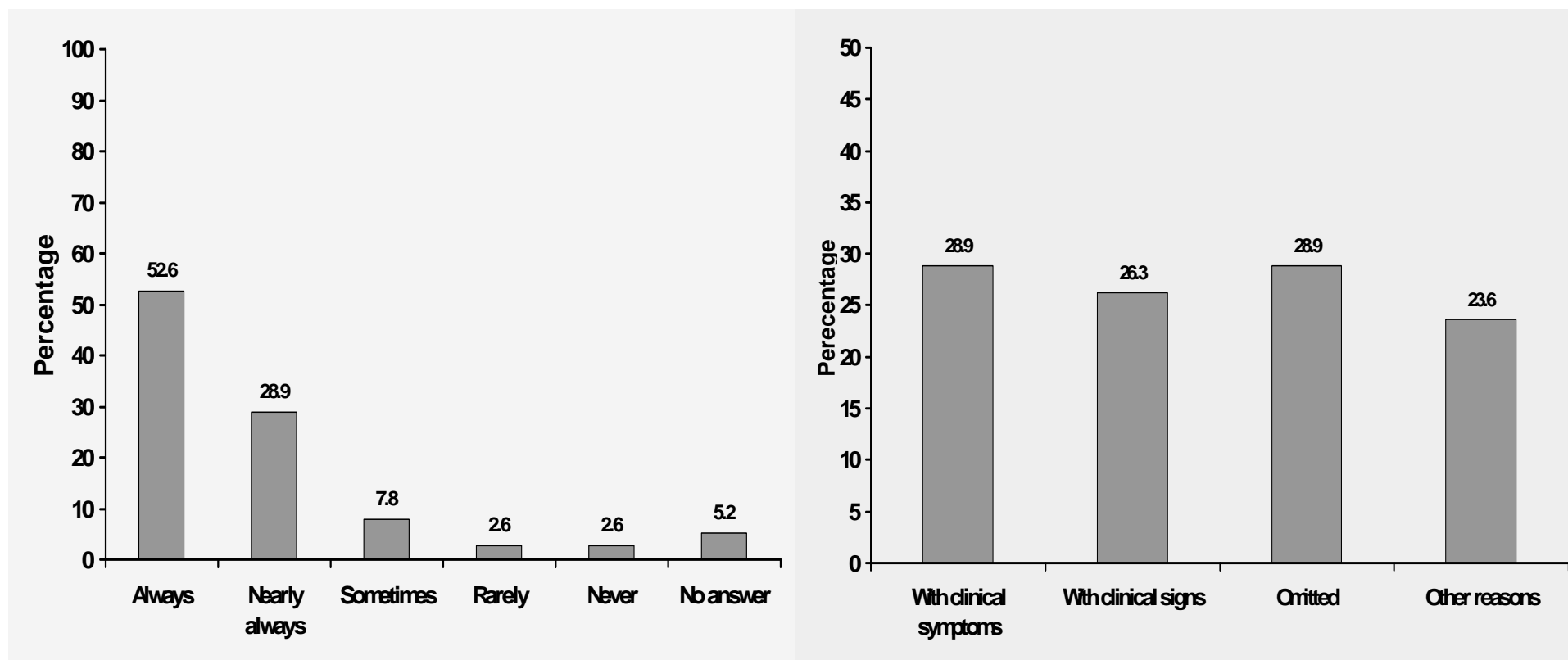
Current evidence about the therapeutic benefit of mechanical ventilation is weak, but consistent, suggesting alleviation of the symptoms of chronic hypoventilation in the short term, and in two small studies survival was prolonged. Mechanical ventilation should be offered as a therapeutic option to patients with chronic hypoventilation due to neuro-muscular diseases.

Decision-making in Duchenne MD

Gibson B, Chest 2001

- Mail questionnaire: Canadian physicians
- Response rate 45/60
- 25% do not discuss mech vent with all patients
- Most frequently cited reason for advising against vent = poor quality of life (52.6%)

Timing of decision-making : UK survey in DMD



Frequency of informing families about NIV In depth discussion about ventilation

Kinali, M.; Manzur, A. Y.; Mercuri, E.; Gibson, B. E.; Hartley, L.; Simonds, A. K.; Muntoni, F. Ped Rehab 2006:9; 351-364

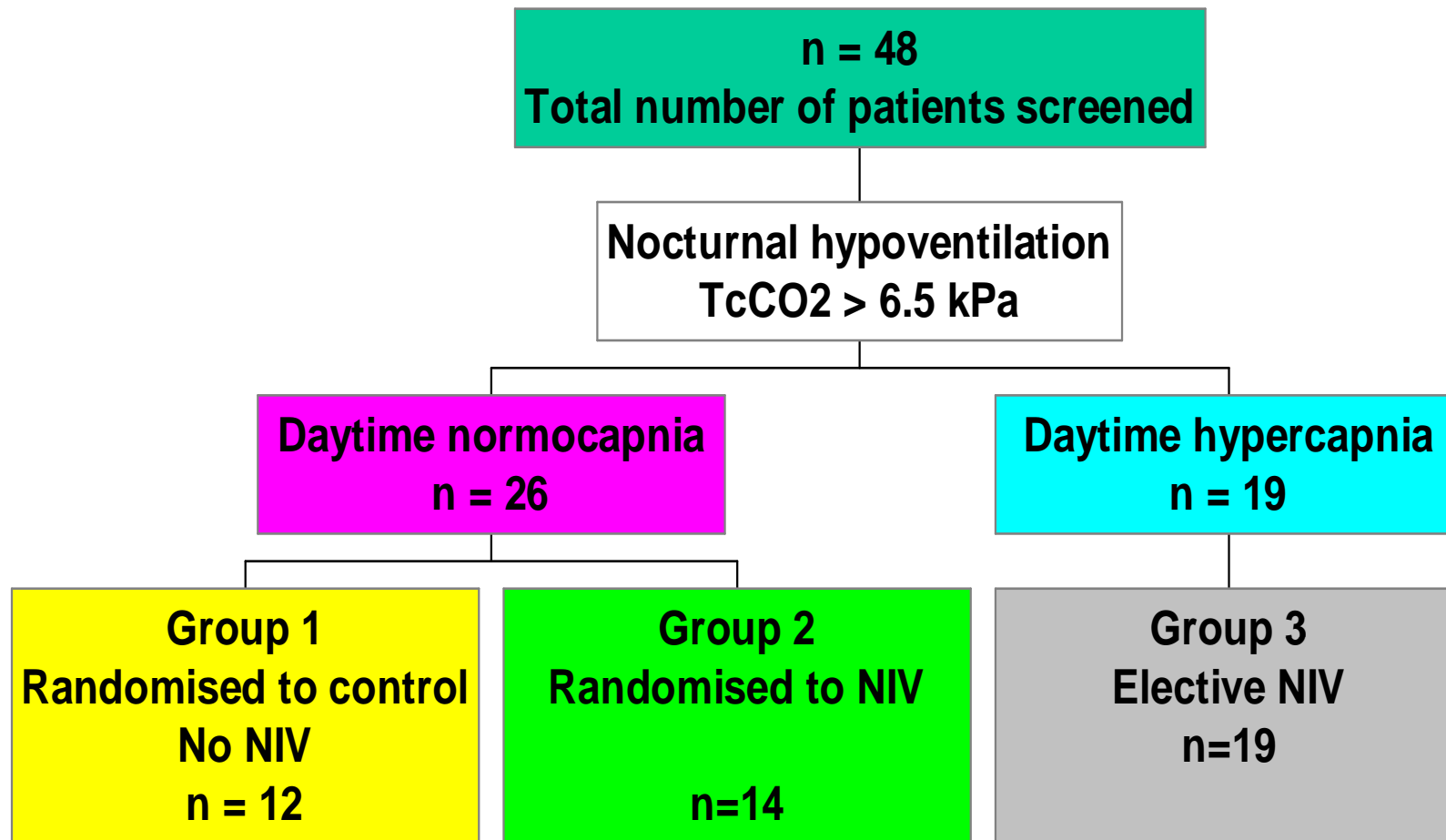
TIMING OF INTRODUCTION OF MV

Consensus Conference: Clinical Indications for NIV in CRF

Restrictive disorders

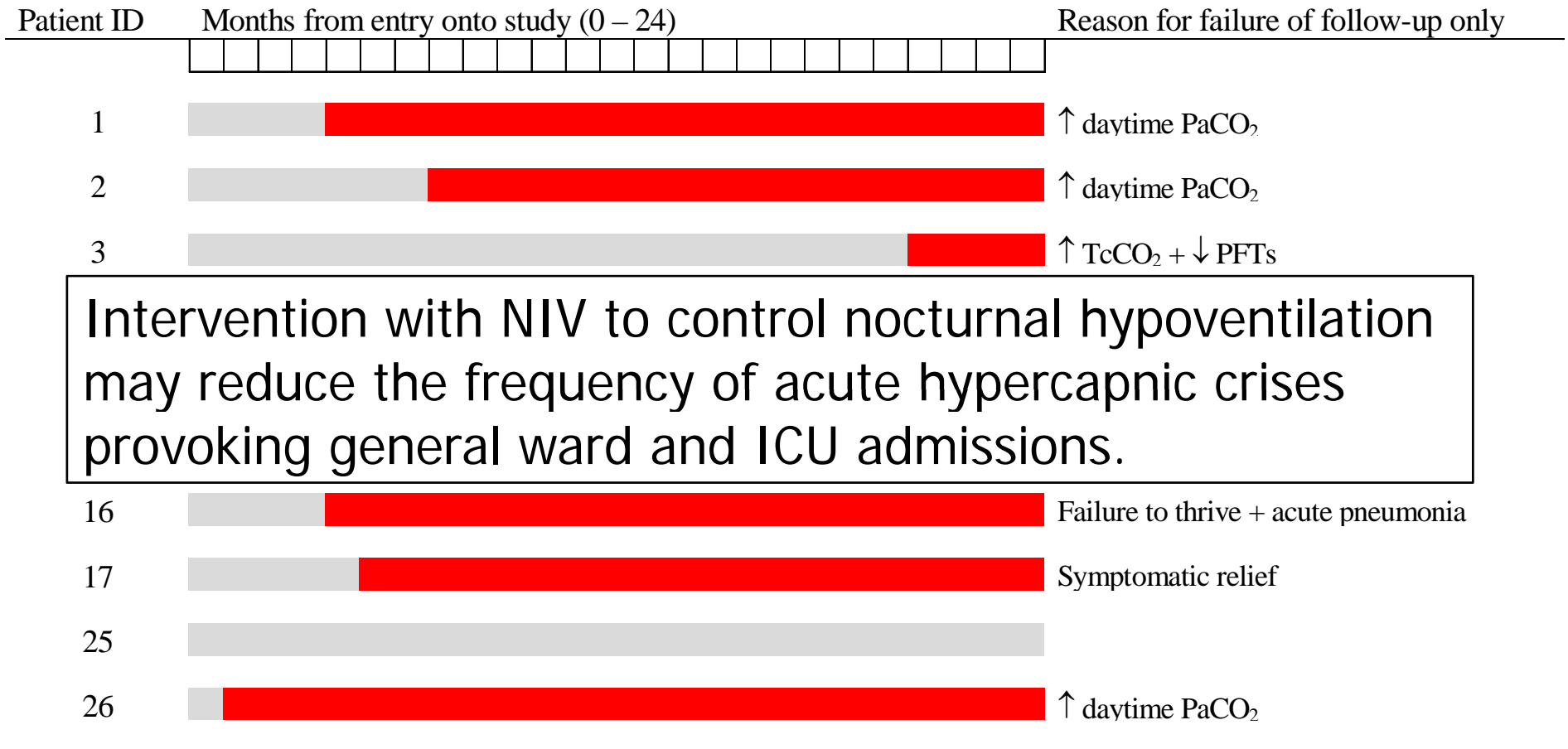
- Diagnostic certainty
- Symptoms eg. fatigue, dyspnoea, headaches
- 1 of the following:
 - PaCO₂ > 6.0 kPa
 - Nocturnal SaO₂ <88% for > 5 minutes
 - MIP < 60cmH₂O or FVC < 50% pred in progressive disorders

Randomised controlled trial of NIV in nocturnal hypoventilation in congenital neuromusculo-skeletal disease: trial design



Results

Summary of group 1 patients failing follow-up only



i.e. 9/10 patients met criteria to receive NIV by end of study (70% within 1 year)

QUALITY OF LIFE

Psychosocial, vocational, quality of life, and ethical issues

J.R. BACH

Ventilator Users	Post-Polio	DMD	SCI	SCI Controls	Controls
Housing	386 5.7±1.7	78 5.6±1.4	42 5.6±2.1	47 5.0±1.5	263 5.2±1.5
Transportation	351 5.3±2.1	77 4.7±2.0	41 4.5±2.3	47 4.6±2.0	268 5.7±1.6
Education	388 5.2±1.9	82 5.2±1.5	42 4.6±2.1	46 4.8±1.8	266 5.5±0.1
Job	216 5.2±1.9	29 4.6±1.7	15 5.2±2.2	33 3.5±2.0	269 5.2±1.4
Health	384 3.9±1.9	82 3.8±2.0	42 4.1±2.1	47 3.9±1.7	269 5.7±1.2
Family life	364 5.6±1.8	77 5.6±1.7	41 5.7±1.7	47 4.9±1.8	268 5.6±1.4
Social life	360 4.8±1.8	75 4.4±1.9	41 4.5±2.0	47 4.4±1.9	268 5.4±1.4
Sexual life	324 4.1±2.2	51 3.6±2.3	36 3.1±2.2	46 3.2±2.0	227 5.5±1.5
Life in general	380 5.1±1.7	80 4.9±1.3	42 4.4±1.8	47 4.1±1.7	259 5.4±1.2 268 2.5±1.7 [†]

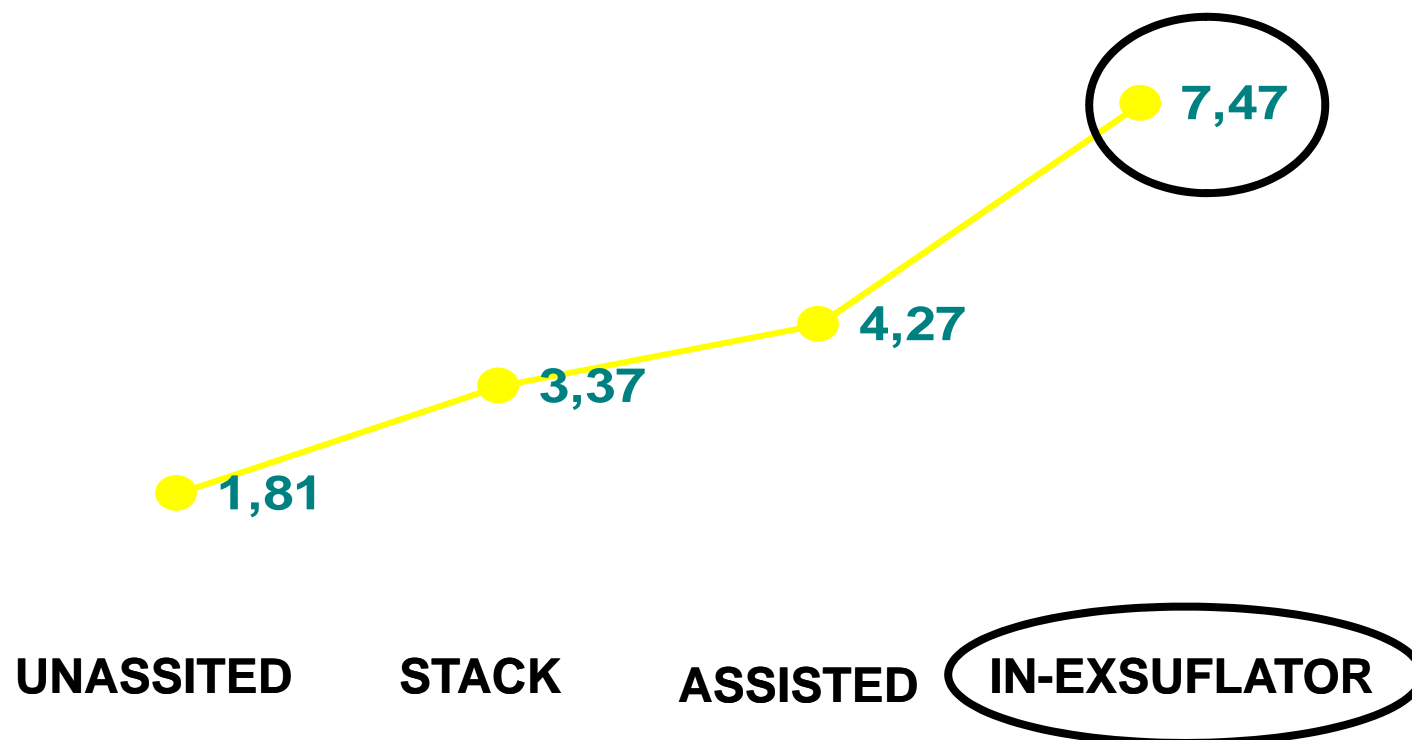
Life domain satisfaction measures in patients submitted to home mechanical ventilation.



Mechanical insufflation-exsufflation. Comparison of peak expiratory flows with manually assisted and unassisted coughing techniques

J.R. BACH

PEF
L/sec

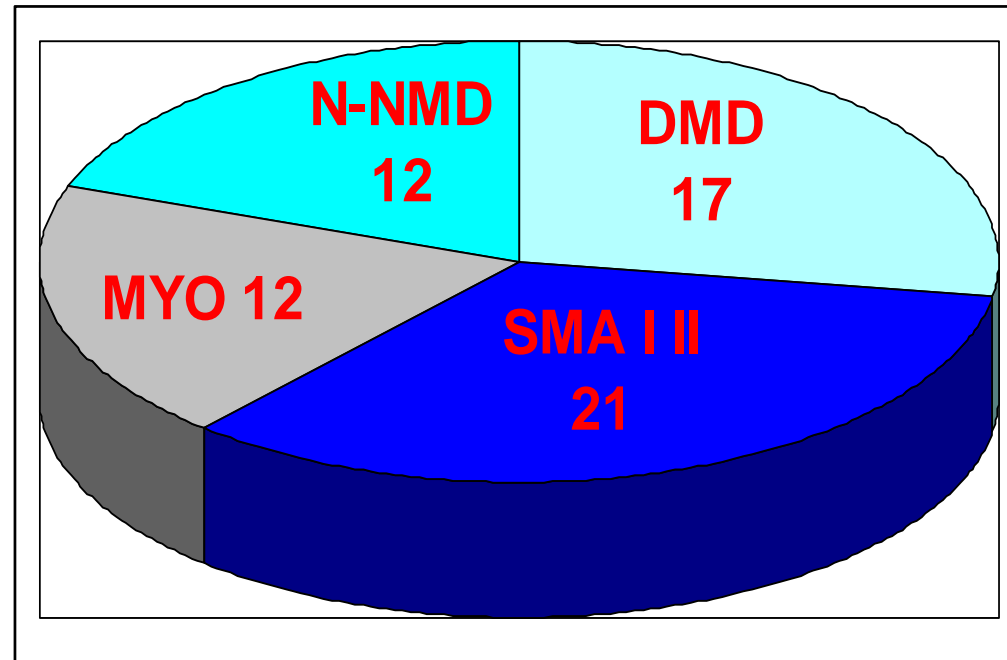


Chest 1993;104:1553-1562



Use of mechanical In-Exsufflator in Pediatric Patients with Neuromuscular Disease and Impaired Cough.

LJ MISKE, EM HICKEY, S KOLB DJ WEINER, HB PANITCH



8 pts - No Ventilation

25 pts - Non-Invasive Ventilation

29 pts - Mechanical Ventilation via tracheostomy



Use of mechanical In-Exsufflator in Pediatric Patients with Neuromuscular Disease and Impaired Cough.

LJ MISKE, EM HICKEY, S KOLB DJ WEINER, HB PANITCH

Study Endpoints

EFFICACY

- Patient report of secretion clearance or resolution of illness
- Radiographic resolution of pneumonia or atelectasis

SAFETY

- Occurrence of pulmonary, cardiac or GI complications



Use of mechanical In-Exsufflator in Pediatric Patients with Neuromuscular Disease and Impaired Cough.

LJ MISKE, EM HICKEY, S KOLB DJ WEINER, HB PANITCH

CONCLUSIONS

In 90% of the study population, the use of MI-E was safe, well-tolerated and effective in preventing pulmonary complications



Mechanical Insufflation-Exsufflation Improves Outcomes in Neuromuscular Disease Patients with Respiratory Tract Infections

A. VIANELLO, A. CORRADO, G. ARCARO, F. GALLAN, C. ORI, M. MINUZZO, M. BEVILACQUA

- Period of study: from January 2001 to March 2003
- Type of study: controlled
- Patient populations: 11 consecutive neuromuscular patients with URTI and mucous encumbrance
- All patients were treated with MI-E in addition to conventional CPT
- Cricothyroid "mini-tracheostomy" or endotracheal intubation was considered when MI-E plus CPT could not expulse airway secretions



Mechanical Insufflation-Exsufflation Improves Outcomes from Muscular Disease Patients with Respiratory Tract Infections

A. VIANELLO, A. CORRADO, G. ARCARO, F. GALLAN, C. ORI, M. MINUZZO, M. BEVILACQUA

	Group A	Group B	p Value
Time spent on MV (days)	9.4 ± 6.9	13.5 ± 11.9	n.s.
Hospital stay (days)	20.5 ± 20	19.8 ± 17	n.s.
Treatment failure, N.	2	10	< 0.05
Pts who required BAA, N.	5	6	n.s.

Group A: Mechanical In-Exsufflator + Chest Physical Treatment

Group B: Chest Physical Treatment

Am J Phys Med Rehabil, 2005; **84**:83-88



Mechanical Insufflation-Exsufflation Improves
Outcomes for Neuromuscular Disease Patients
with Respiratory Tract Infections: A step in the
right direction.

with
right

M. GONCALVES, J. BACH

COMMENTS

Insufflation pressures were suboptimal

Care providers should provide MI-E along with abdominal thrusts up to every 15 mins to maintain normal saturation

If both the inspiratory and expiratory muscle aids are used effectively, only advanced bulbar ALS and some SMA type 1 patients require tracheostomy.