Home noninvasive ventilation in children with neuromuscular diseases

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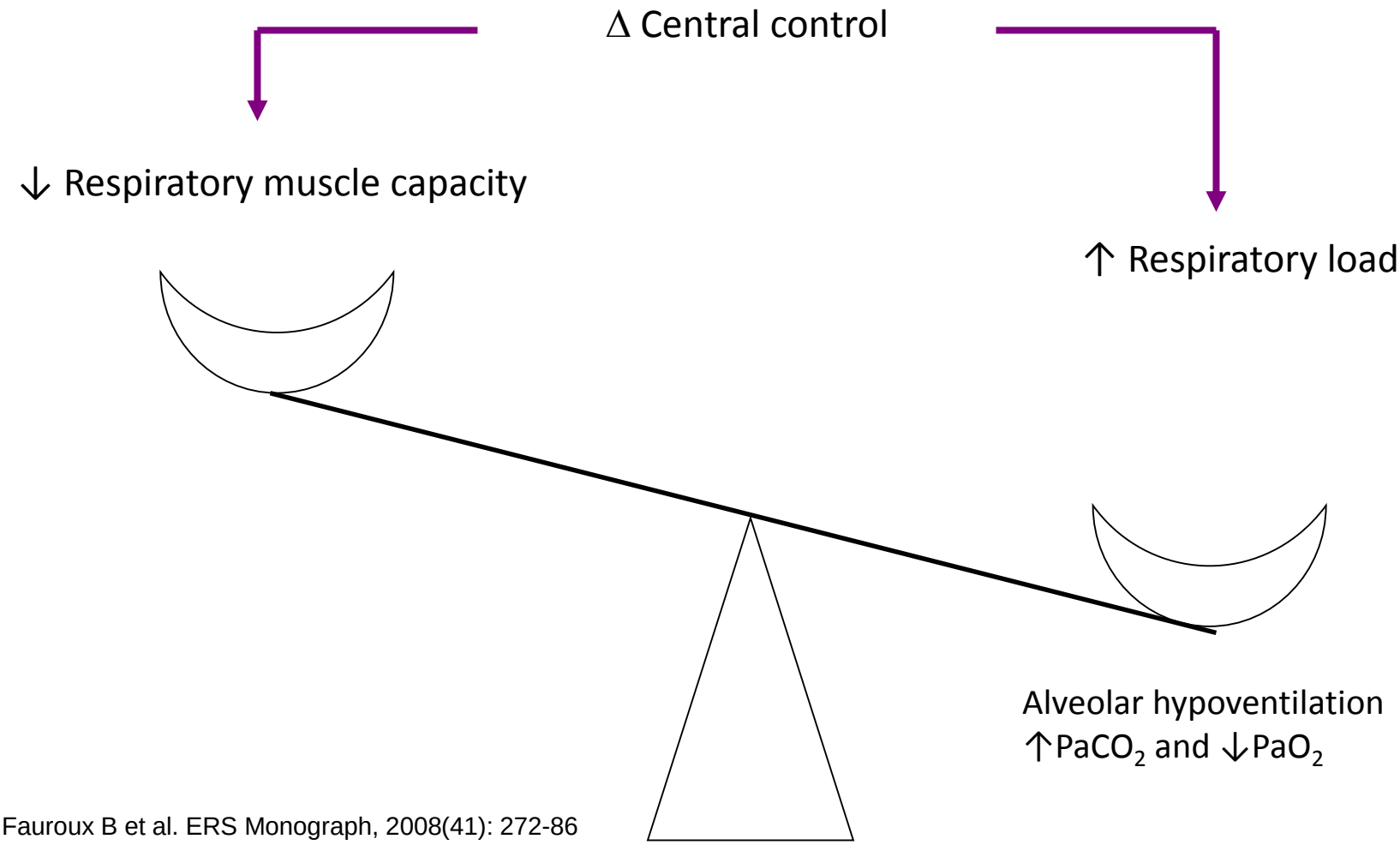
European Respiratory Society Fellowship

- Prof. Brigitte Fauroux, Paris

Noninvasive ventilation in children with neuromuscular diseases

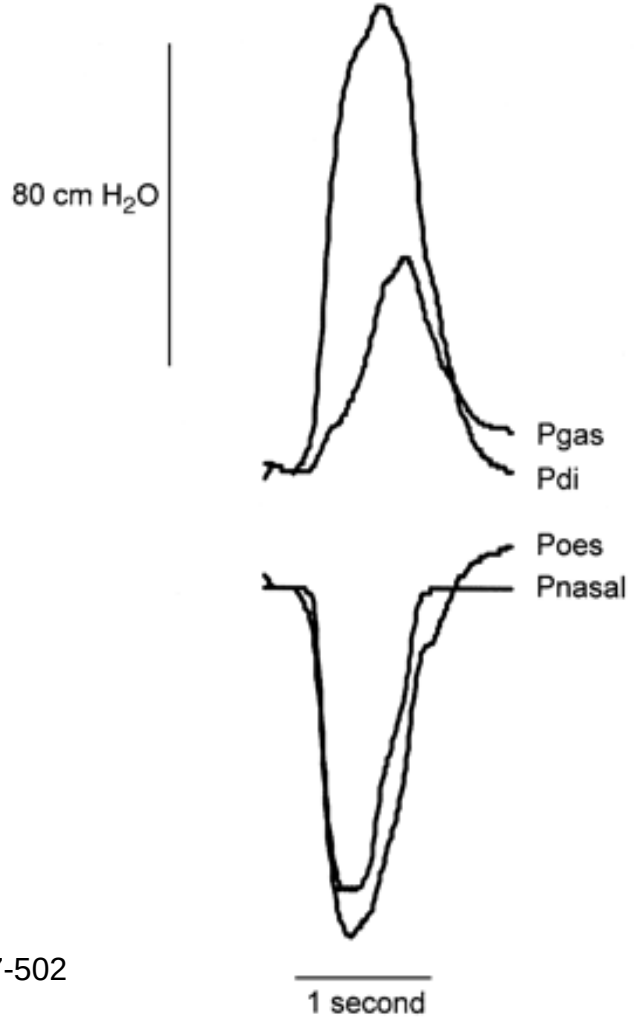
- Respiratory impairment in children with NMD
- Do children benefit from NIV?
- When should NIV be started?
- Our data

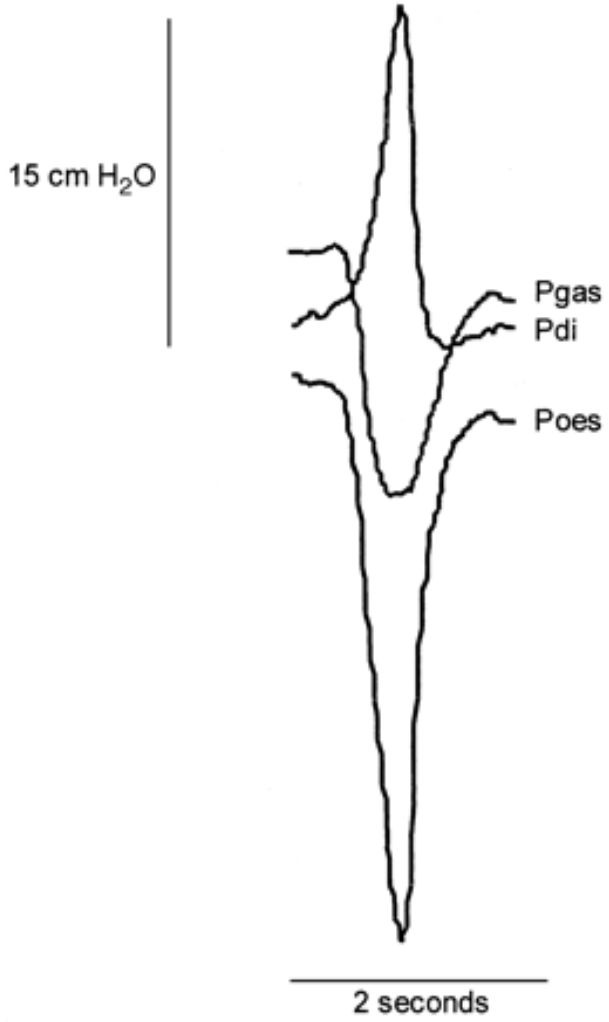
Respiratory system balance



Fauroux B et al. ERS Monograph, 2008(41): 272-86

Respiratory muscle testing



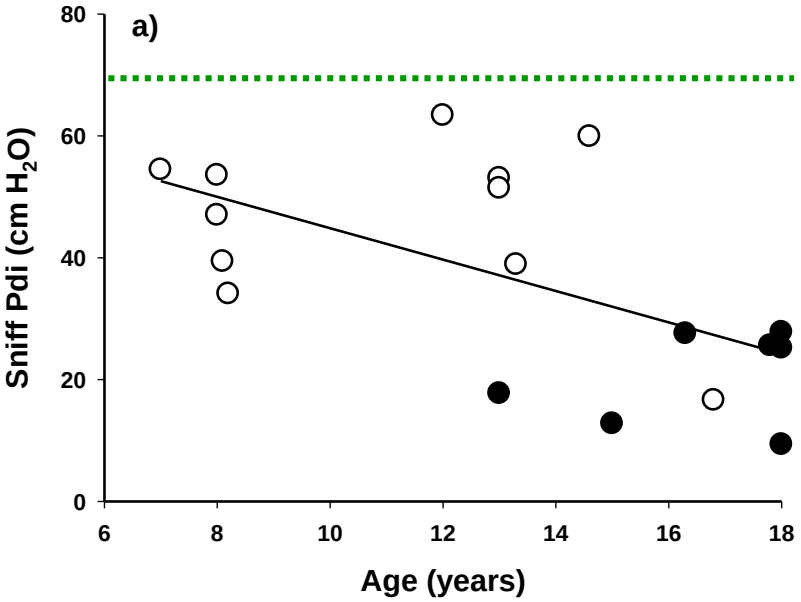


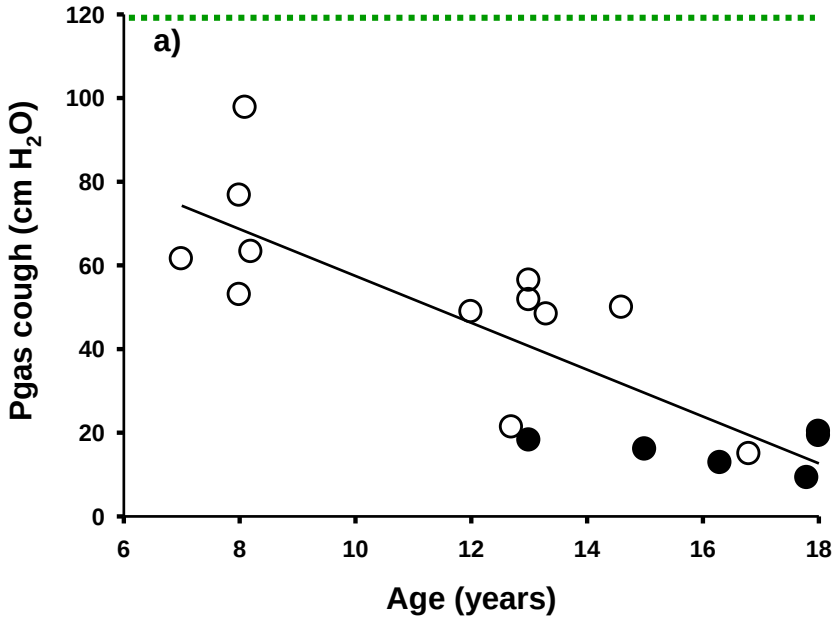
Mustfa N, Moxham J.

Q J Med 2001; 94: 497-502

Fading muscle strength

Duchenne muscular distrophy



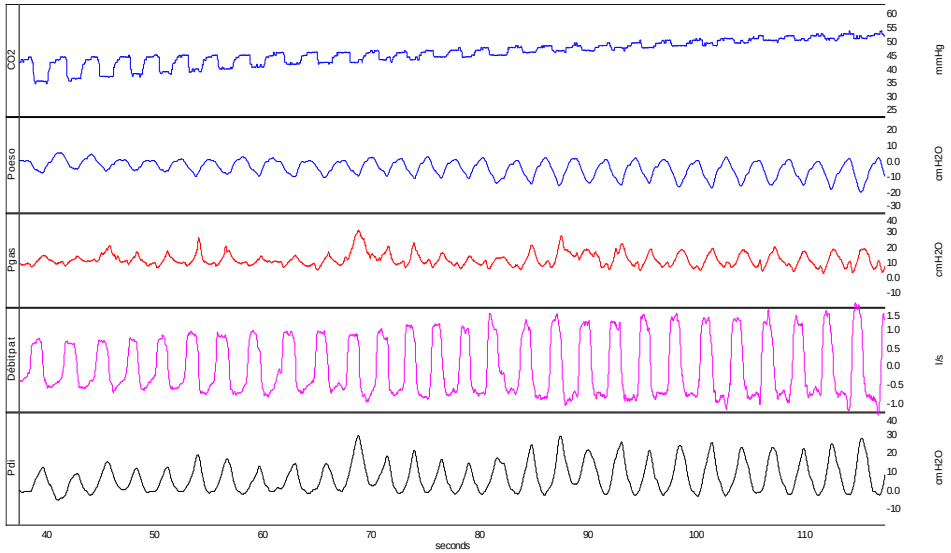


Nicot et al. AJRCCM 2006;174:67-74

Increased respiratory load

- Mechanisms
  - Micro-atelectasis
  - Thoracic cage „stiffening“
  - Chest deformations (scoliosis)
  - ± cardiomyophathy

Altered central contol



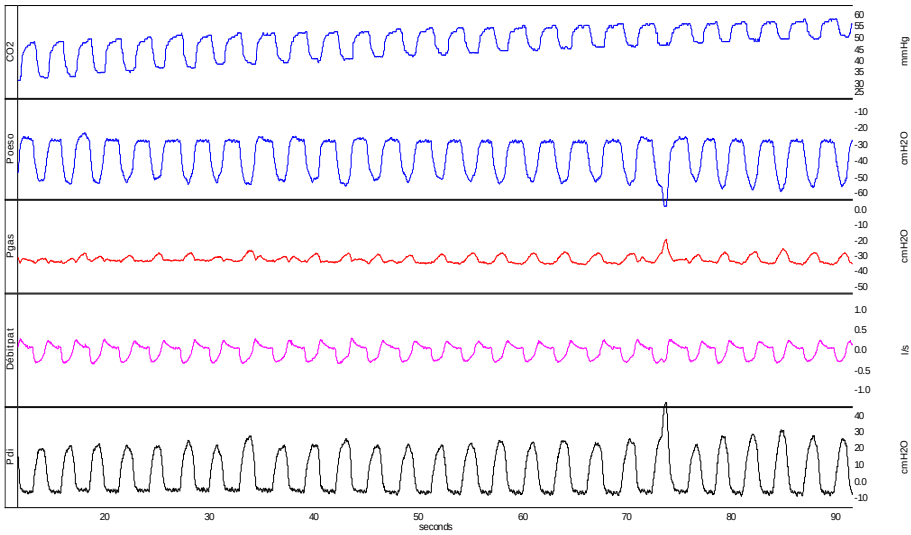
$PetCO_2$ (mm Hg)

Pes (cm H_2O)

Pgas (cm H_2O)

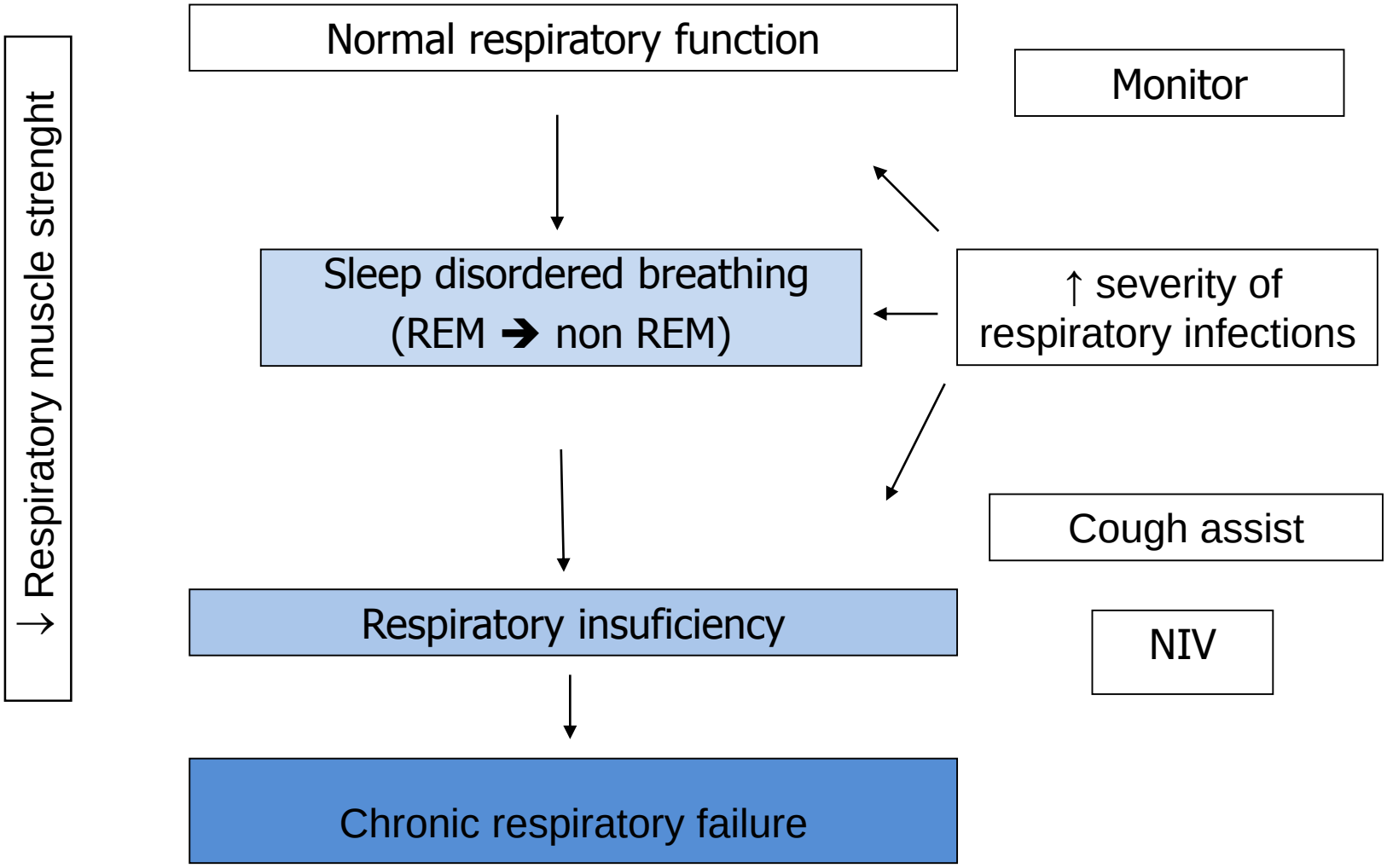
Pretok (L/s)

Pdi (cm H_2O)



Courtesy of Prof. Fauroux

Breathing disorders

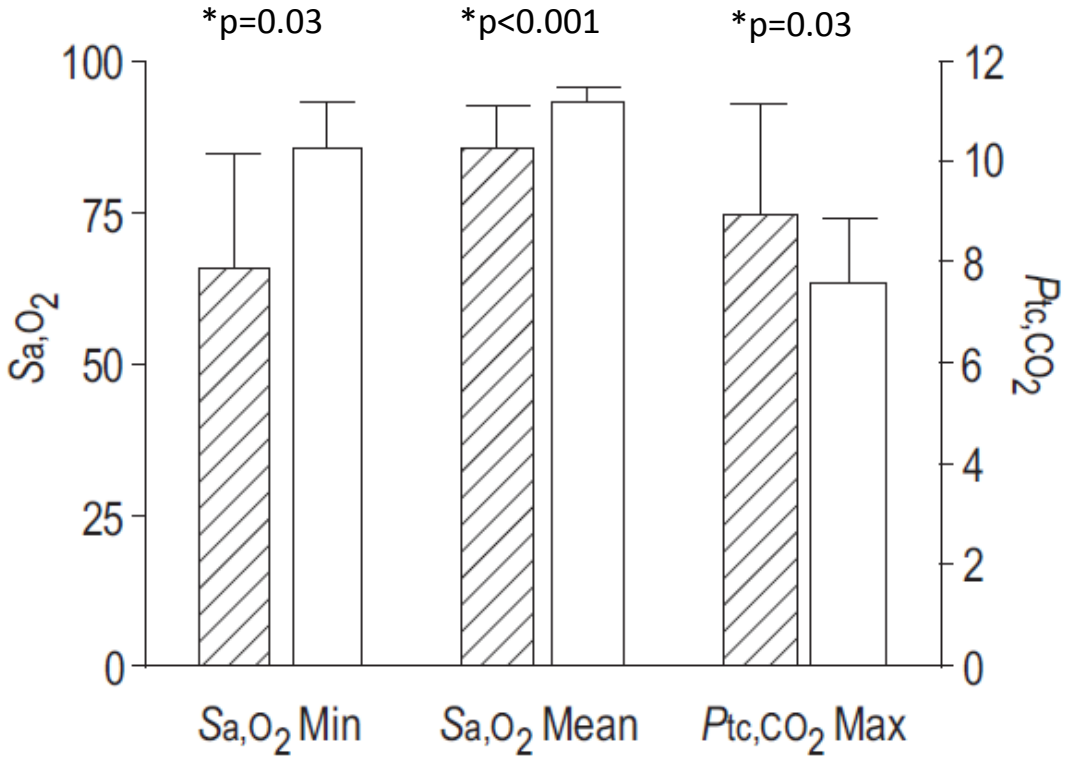


NIV in children

- *non*-invasive – on demand
- Large choice of available home care ventilators
- Nasal prongs /cushions / mask
- Face masks / helmet
- Pressure ventilatory modes preferred

NIV improves nocturnal gas exchange

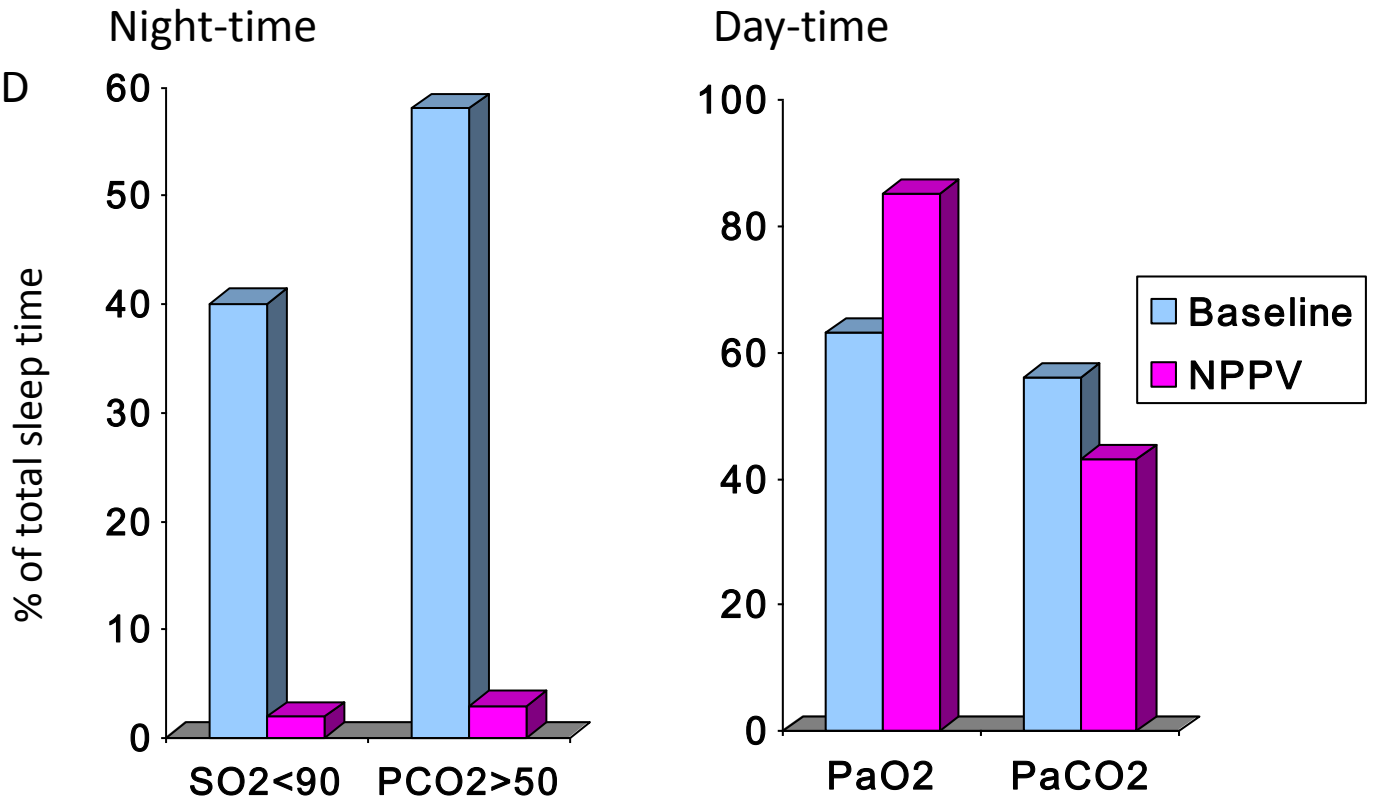
40 children with NMD
9 mo – 16 yrs
NIV during sleep



Simonds AK et al. Eur Respir J. 2000; 16(3):476-81.

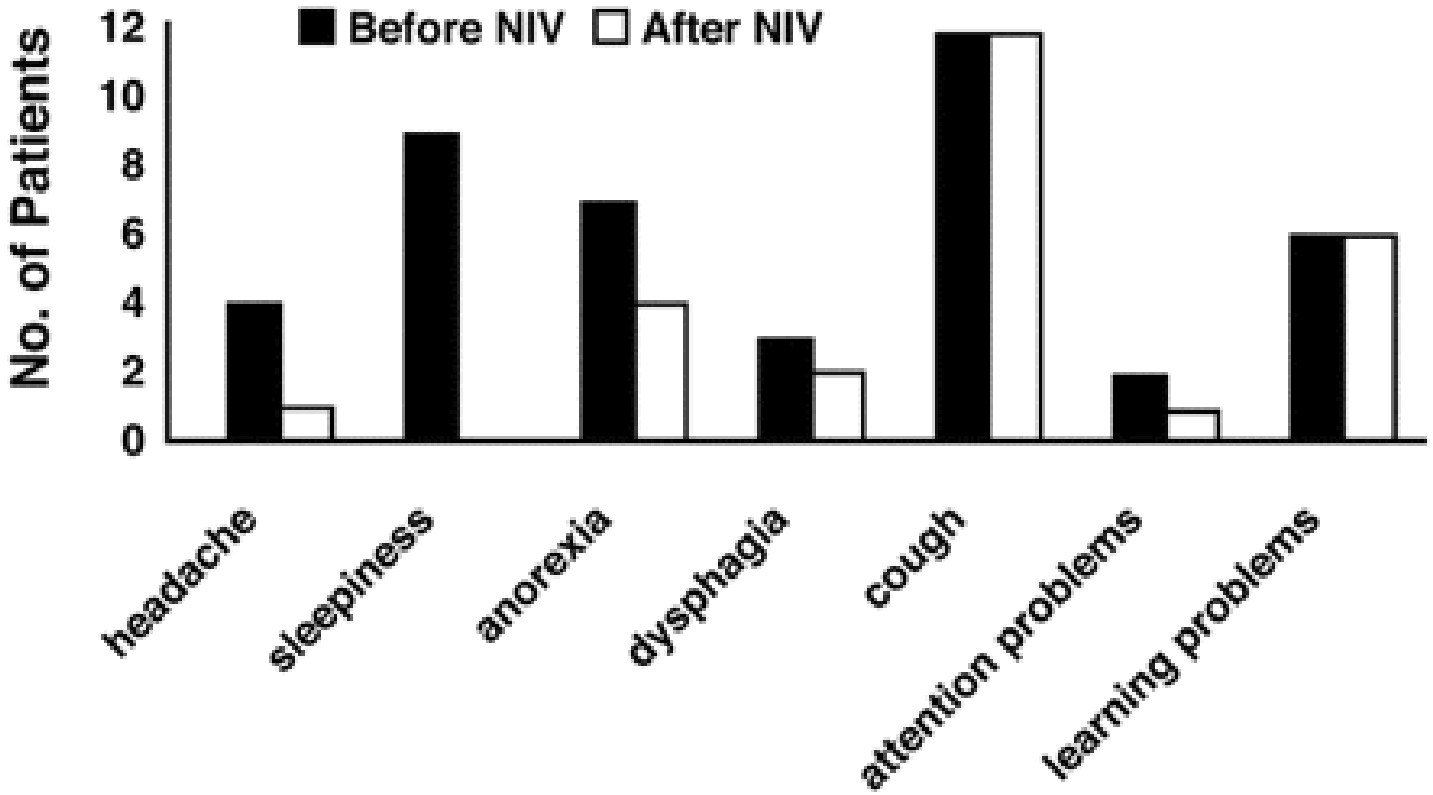
Nocturnal NIV improves day-time blood gases

30 children with NMD
Age: 12.3 ± 4.1 yrs
NIV: 25 ± 13 mo



Mellies U et al. Eur Respir J. 2003; 22(4):631-6.

Improvement in patients' symptoms



Young *et al.* Neurology 2007;68:198

Respiratory exacerbations

N = 40 (24 NIV) patients
Age 11.3 ± 4.2 yrs

	NIV and assisted coughing (n = 16)		NIV without assisted coughing (n = 8)	
	Before NIV	After NIV	Before NIV	After NIV
GP consultation (/year)	11.3 ± 25.3	3.4 ± 6.2*	5.1 ± 4.1	2.8 ± 3.1
Antibiotics (/year)	4.1 ± 3.8	1.7 ± 1.7*	4.0 ± 2.8	2.4 ± 3.1
Hospital admission (/year)	2.0 ± 1.9	0.9 ± 1.4*	0.9 ± 1.2	0.4 ± 1.1

Dohna-Schwake C et al. Pediatr Pulmonol. 2008 ; 43(1): 67-71.

Effects of NIV

Nocturnal mechanical ventilation for chronic hypoventilation in patients with neuromuscular and chest wall disorders (Review)

Annane D, Orlikowski D, Chevret S, Chevrolet JC, Raphaël JC

THE COCHRANE COLLABORATION®

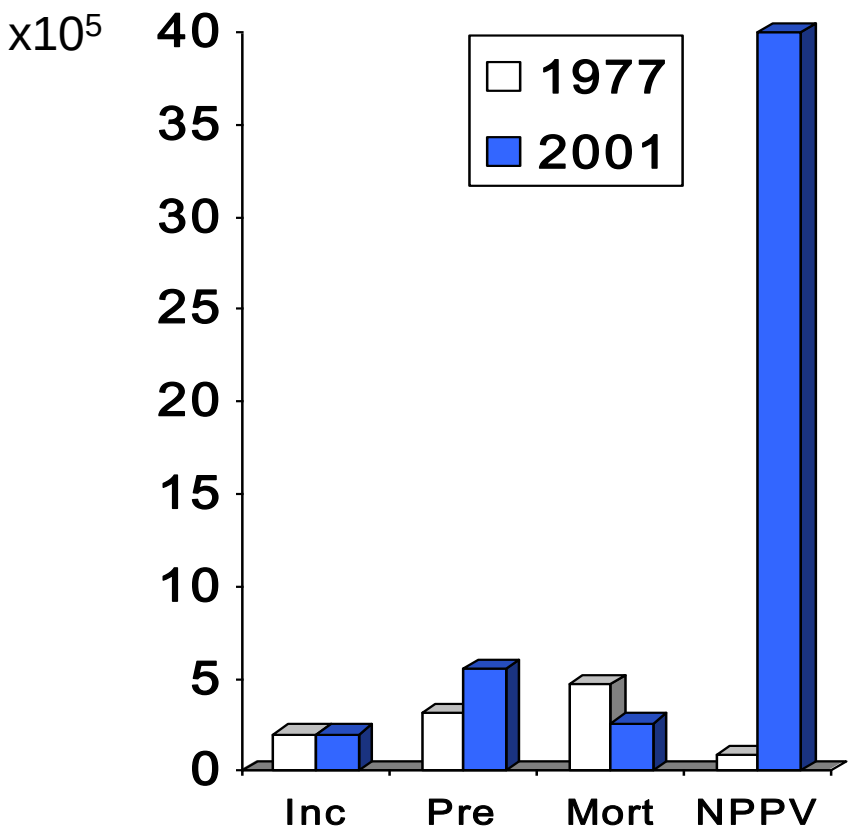
- **Benefit on**
  - nocturnal and daytime hypercapnia
  - nocturnal SaO_2
- **No benefit on**
  - clinical symptoms of hypoventilation
  - admission to hospital
  - lung function (FVC, SNIP)

NIV - increased survival in DMD

Total Duchenne muscular dystrophy population in Denmark 1977-2001

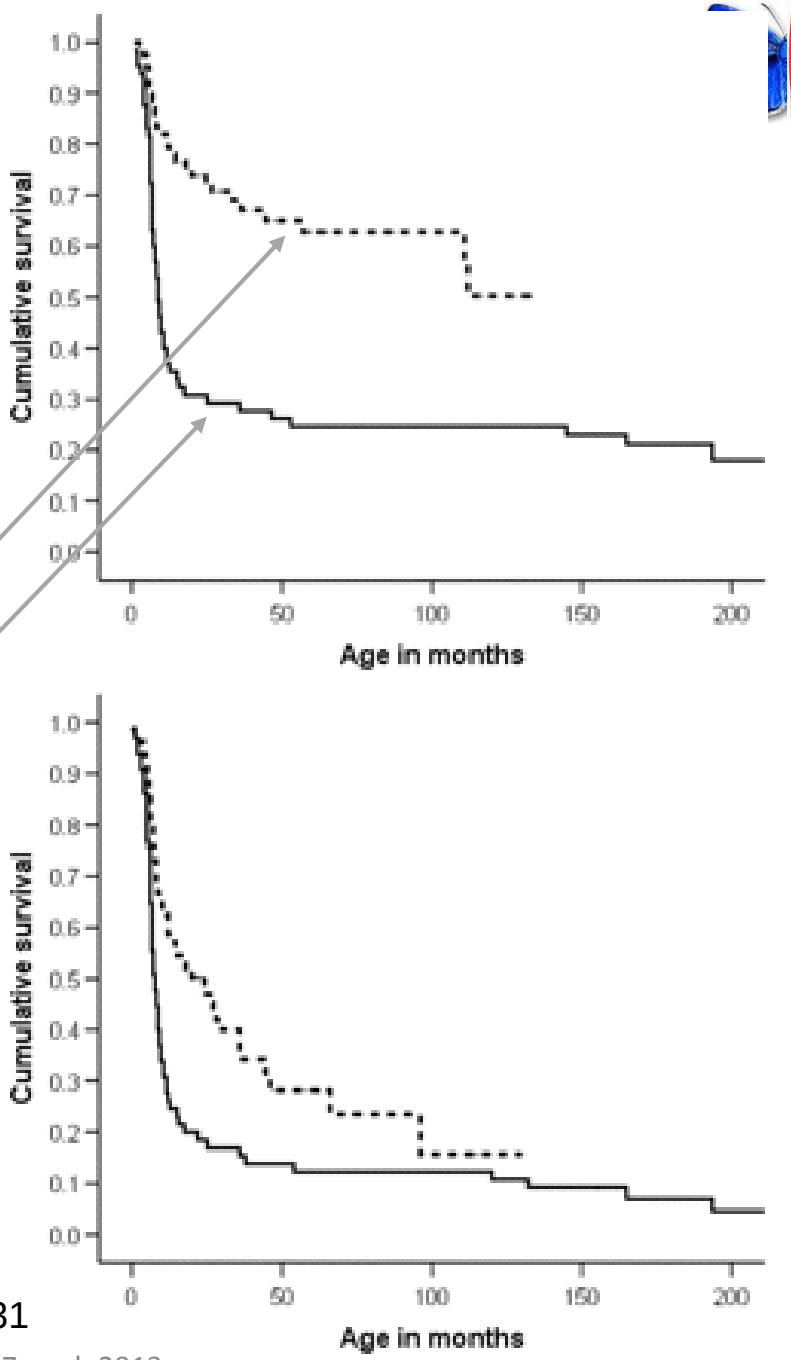
Mortality 4.7 ↓ 2.6 / 100 years

NIV users 0.9 ↑ 43.4 per 100



Jeppesen *et al.* Neuromuscular Disorders 2003;13:804

NIV – increased survival in SMA type I



Patients born in 1995-2006

Patients born in 1980-1994

Oskoui *et al.* Neurology 2007;69:1931

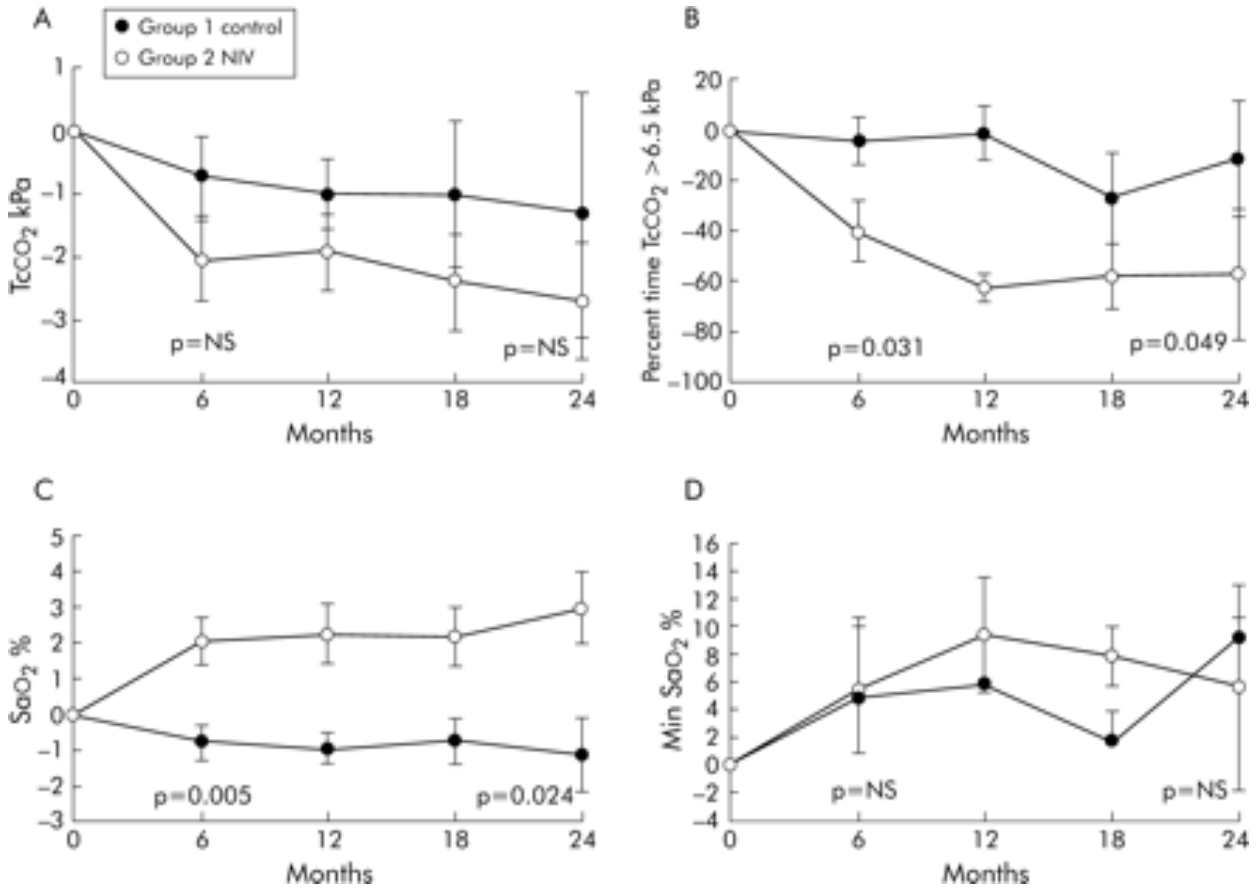
Benefits of noninvasive ventilation

Survival	DMD + SMA
Nocturnal gas exchange	yes
Daytime gas exchange	yes
Quality of sleep	?
Neuropsychological and cognitive functioning	?
Respiratory muscle function Lung function	no (Cochrane) ?
Lung growth	?
Nutritional status	?
Quality of life	no decline

When to initiate NIV in NMD ?

- **Diurnal hypercapnia**
- After an acute respiratory failure
- Nocturnal hypoventilation
  - Sleep disruption, daytime hypersomnolence, excessive fatigue, morning headaches
  - Polysomnography / poligraphy
    - no precise criteria to schedule a PSG
    - interpretation
      - mean SaO_2 ?, % of time spent with a SaO_2 < 90%
      - level of transcutaneous or end tidal CO_2 ?
      - sleep fragmentation, apnea - hypopnea index

NIV for nocturnal hypoventilation with daytime normocapnia



Ward *et al.* Thorax 2005;60:1019

N= 48 patients with Dg: NMD or chest wall disease

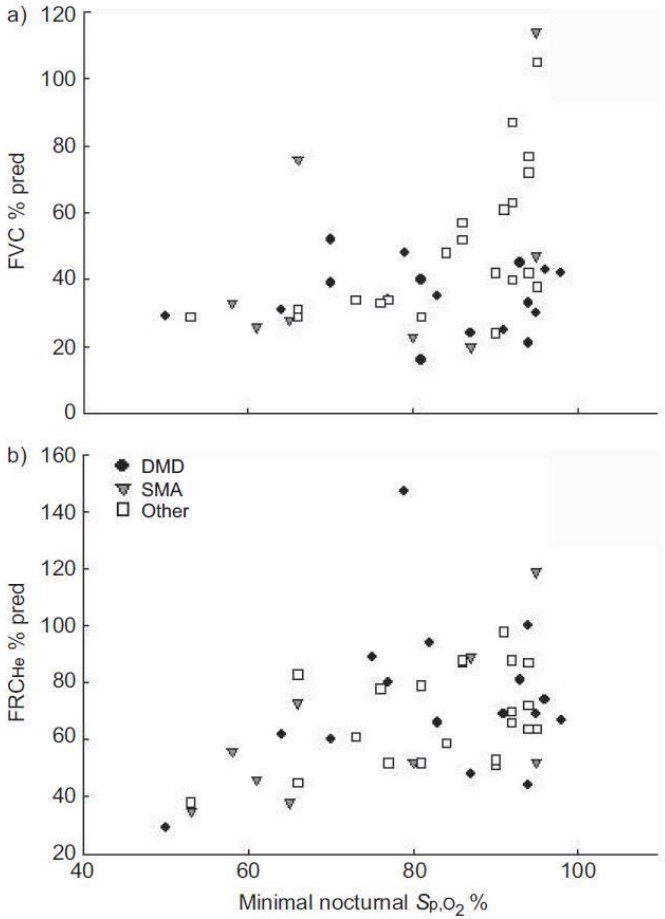
Age 7– 51 years
all VC < 50%

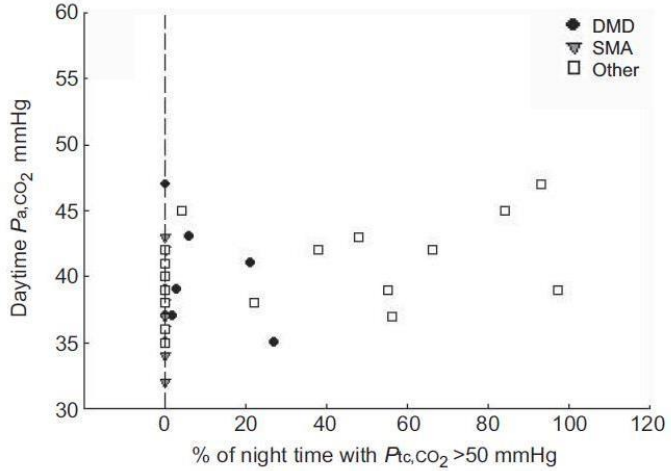
Daytime predictors of sleep disordered breathing

49 children age 5 – 18 yrs	Cut-off value	Sensitivity / specificity	AUC (%)
Sleep disordered breathing			
IVC (% pred)	< 60	97 / 87	99
PIP (kPa)	< 4	87 / 43	82
Nightime hypercapnic hypoventilation			
IVC (% pred)	< 40	96 / 88	96
PIP (kPa)	< 2.5	72 / 83	79
$PaCO_2$ (kPa)	> 5.3	92 / 72	89

Mellies *et al.* Neuromuscular Dis 2003;13:123

Daytime predictors of sleep disordered breathing





Bersanini C *et al.* ERJ 2012;39:1206

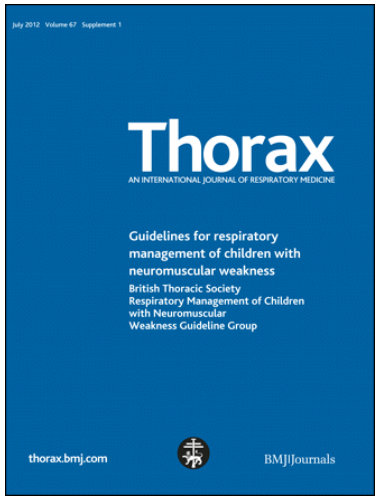
Sleep & breathing evaluation

- Polisomnography
- Limited night studies

  SpO_2, pulse rate

  airflow, chest impedance, ECG, body position, $PtcCO_2$
- Blood gases – arterial, capillary (early morning)

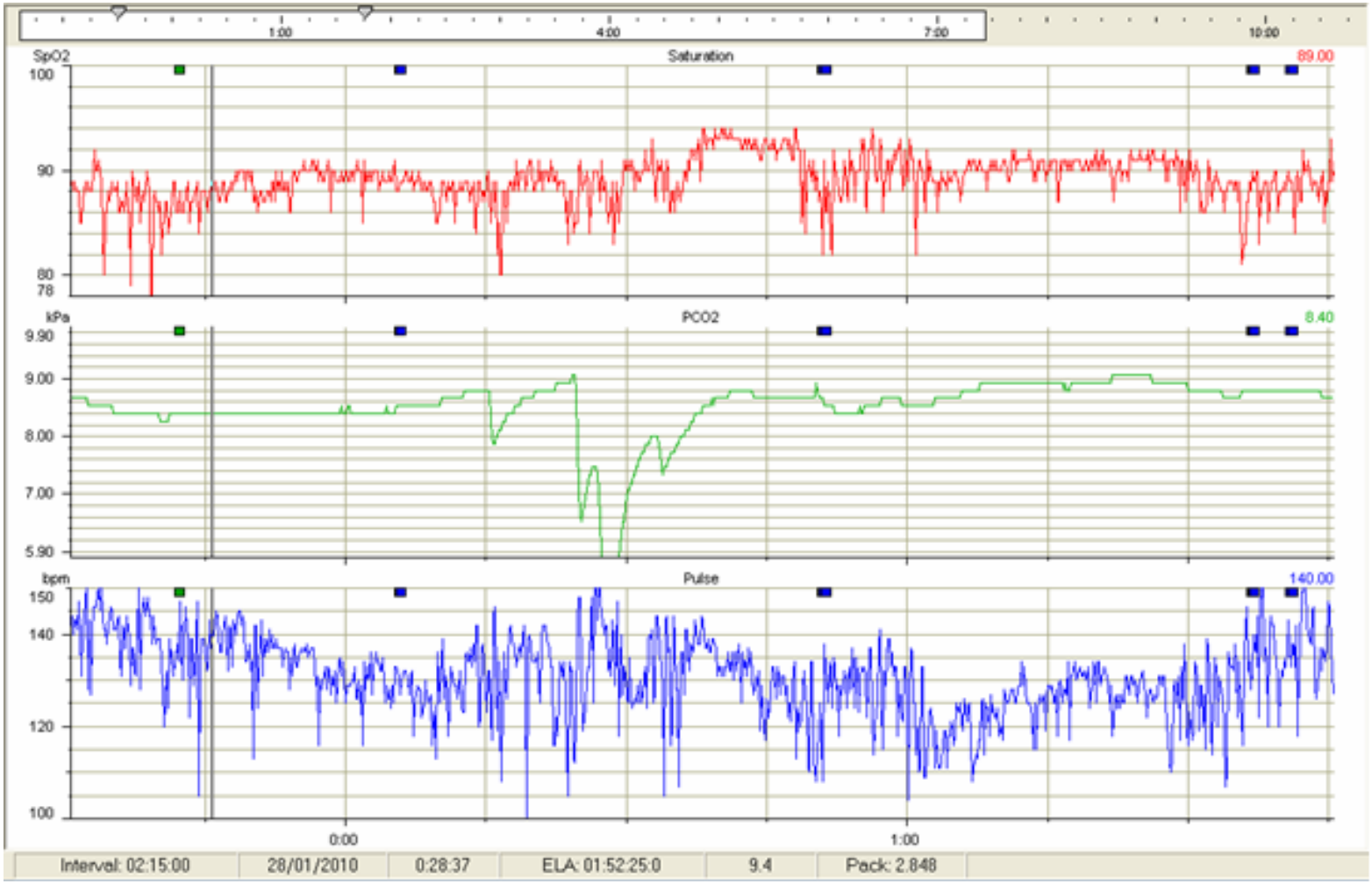
Indications for sleep studies



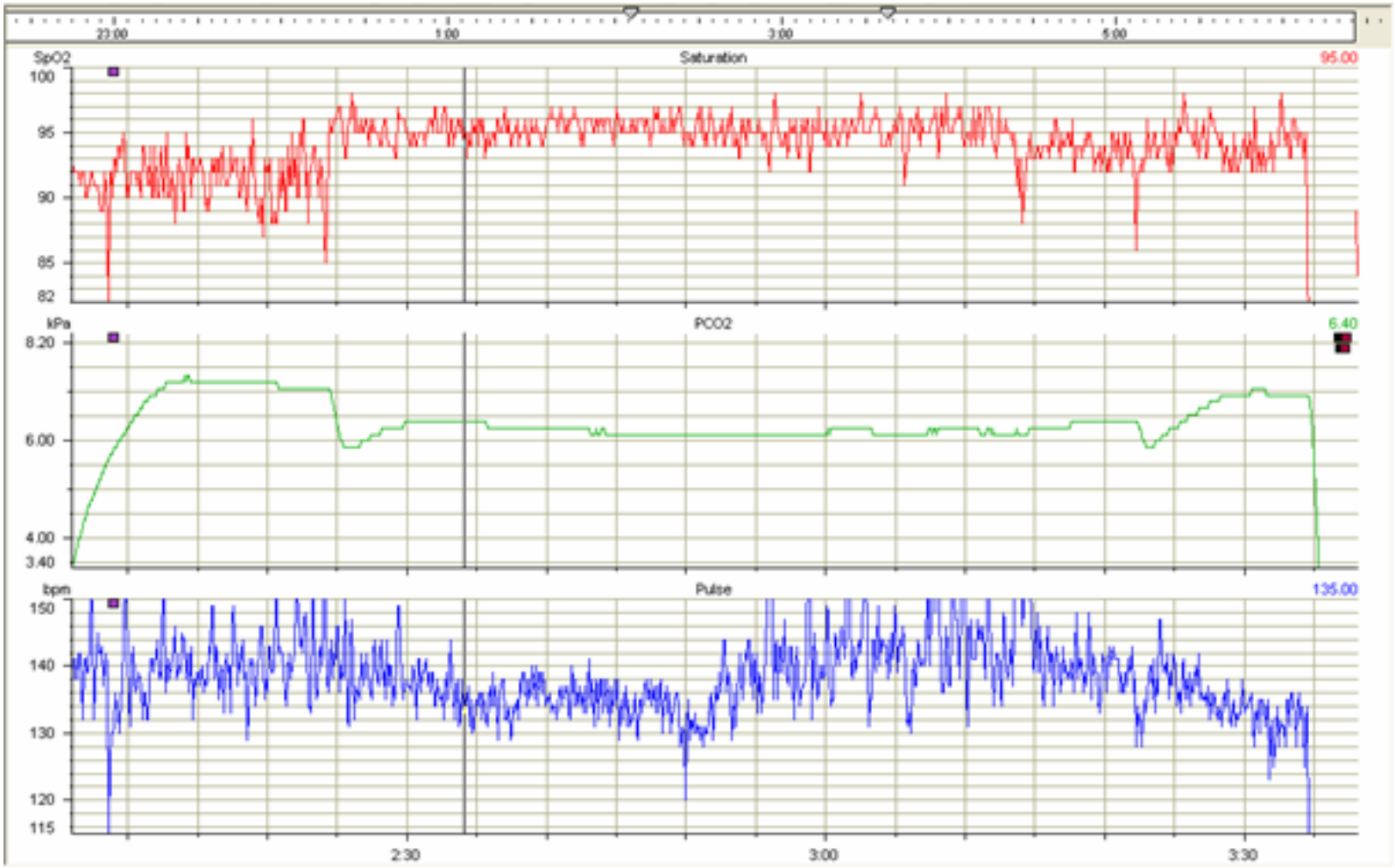
Indication	Notes
Vital capacity <60% predicted	Children generally need to be over 6 years of age to produce reliable spirometry. In boys with DMD, a vital capacity of over 1.8 litres indicates that nocturnal hypoventilation is very unlikely to be present
Loss of ambulation because of progressive weakness, or children who never attain the ability to walk	Inability to walk is a measure of moderate to severe muscle weakness
Infants with weakness	Infantile onset is often associated with more severe weakness
Children with symptoms of obstructive sleep apnoea or hypoventilation	See section on clinical assessment
Children with diaphragmatic weakness	Sleep-associated hypoventilation can occur even if general muscle strength is preserved
Children with rigid spine syndrome	These children are at particular risk of nocturnal hypoventilation despite relatively preservation of general muscle strength, ambulation and near normal vital capacity

Hull J et al. Thorax. 2012; 67 Suppl 1: i1-40.

Night-time polygraphy



Night-time polygraphy (NIV)



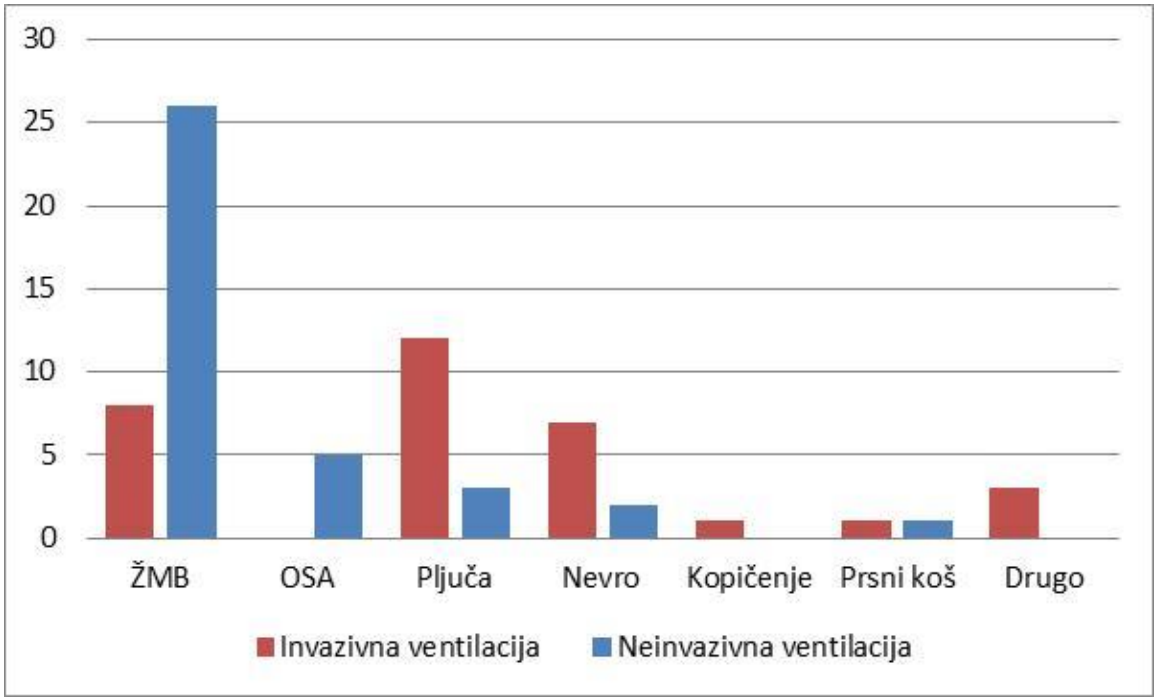
Home ventilation

Children and adolescents with home ventilation in Slovenia 2012

N: 69

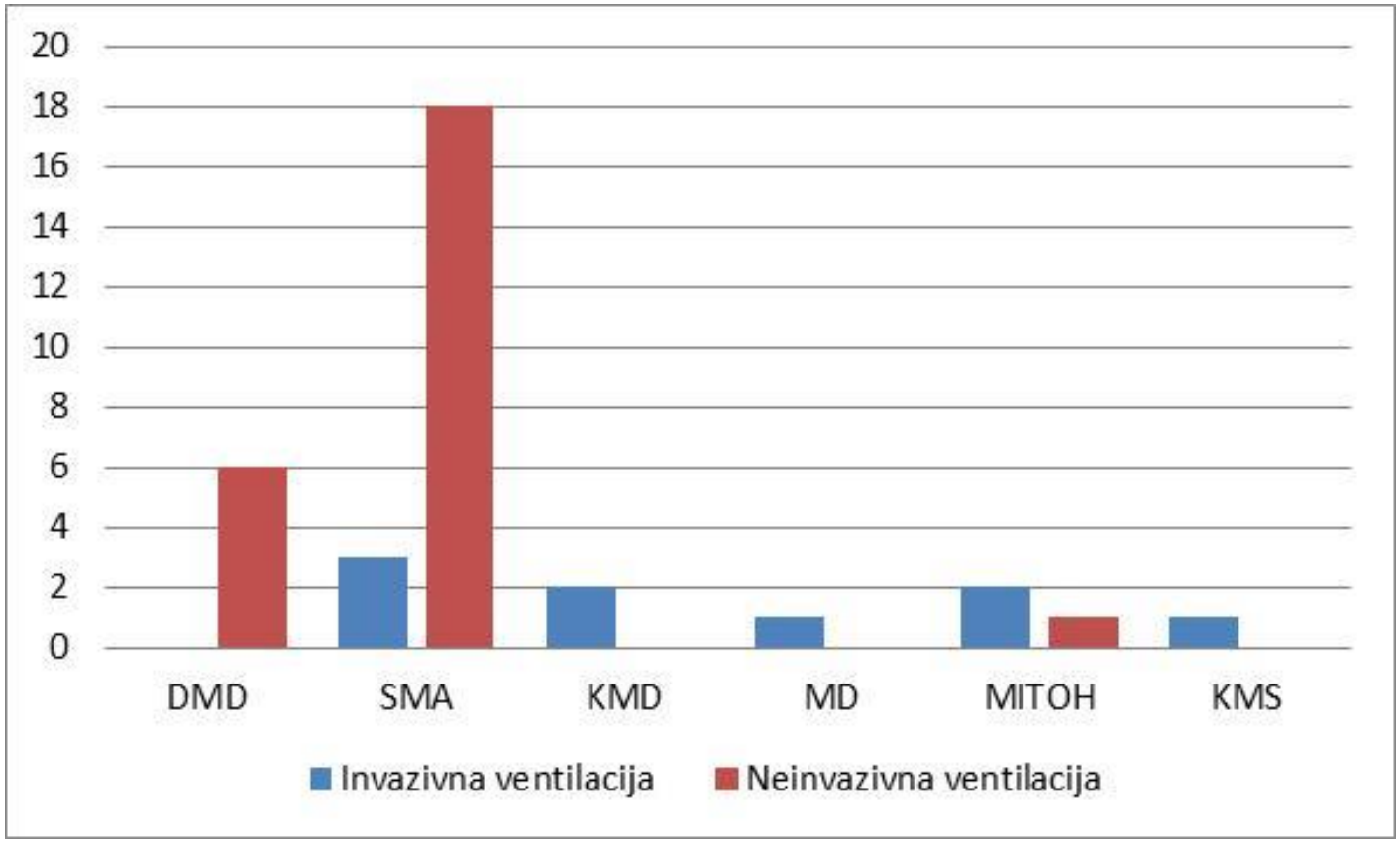
Median age: 8 (0.1–22) yrs

NIV: 37 (54%)



Home ventilation in NMD

Children and adolescents and home ventilation in Slovenia 2012



Home ventilation

Cause of ventilation	Major subgroups	Interface for support		
		Tracheostomy	Mask	Other
Central nervous system (n=168)	Congenital central hypoventilation syndrome	30	25	1*
	Spinal injury	21	1	2†
	Birth injury/cerebral palsy	2	6	
	Acquired central hypoventilation syndrome	8	12	
	Other central causes	25	35	
Musculoskeletal (n=402)	Duchenne muscular dystrophy	1	88	1‡
	Congenital myopathy	23	34	
	Other myopathy	3	5	
	Other dystrophy	7	51	
	Kyphoscoliosis	2	9	
	Spinal muscular atrophy type 1	5	7	
	Spinal muscular atrophy II/III	5	62	
	Mucopolysaccharidosis	1	2	
	Other musculoskeletal	17	79	
Respiratory (n=343)	Chronic lung disease	9	9	
	Chronic lung disease (prematurity)	9	5	
	Airway malacia	26	13	
	Prader Willi/obesity syndromes	–	58	
	Upper airway obstruction	6	153	
	Cystic fibrosis/primary ciliary dyskinesia	–	5	
	Other respiratory	12	37	1§
Unclassified (n=20)				

Wallis C et al. Arch Dis Child. 2011; 96(11):998-1002

UK (2008)

933 / 12 mio

7.8 / 100.000

SI (2012)

69 / 400.000

17.0 / 100.000

Conclusions

- In children with NMD, respiratory failure is caused by a ventilatory imbalance with
  - ↓ in respiratory muscle capacity
  - increase in respiratory load
  - and altered ventilatory drive
- This imbalance causes
  - episodes of acute respiratory failure (infections)
  - sleep-disordered breathing
  - daytime respiratory failure
  - altered chest wall / lung growth

Conclusions

- NIV is associated with an mprovement in
  - nocturnal and daytime blood gases
  - survival (SMA + DMD)
- No proven benefit on
  - quality of sleep, quality of life
  - the decline of respiratory muscle and lung function
  - lung and chest wall growth
- Criteria to start NIV (?)
  - poor sleep quality ±
  - abnormal nocturnal gas exchange

Thank you for your atention!

Questions?