Online Table S5.4: Benefits of NIV (except decrease in AHI)

Author	Country	Journal	Study design	Number of patients	Ages	Benefits
Bach et al. [1]	USA	Pediatr Pulmonol	Retrospective study	56 SMA I	RF before 2 yrs	33 treated with NIV vs 16 treated with tracheotomy 31/33 survived to 42 ± 26 m, fewer hospitalisations > 5 yrs with NIV vs tracheotomy
Bach et al. [2]	USA	Am J Phys Med Rehab	2 cases	2 SMA I	7 m, 3 yrs	No pectus excavatum, survival until 7 and 3 yrs with NIV 24h/24
Mellies et al. [3]	Germany	Neuromuscul Disord	Prospective study	7 infants SMA I (6) and SMA II (1) (+ 6 SMA controls without NIV)	6-11 yrs	After 6-12 m of NIV: improvement in SDB symptoms, sleep quality and architecture vs no improvement in controls
Bach et al. [4]	USA	Am J Phys Med Rehab	Retrospective study	106 SMA I	?	Untreated died at 9.6 ± 4 m, 22 with tracheotomy survived at 70.5 ± 43.3 m, 47 treated with NIV, 29/47 reached 65.2 ± 45.8 m, 8 died Same survival with NIV and tracheotomy but fewer hospitalisations with NIV
Vasconcelos et al. [5]	Portugal	Revista Port Pneumol	Retrospective study	7 SMA I, 11 SMA II, 4 SMA III	6 m – 26 yrs	17/22 treated with NIV NIV associated with a decrease in chest deformity and ARF episodes

Chatwin et al. [6]	UK	Arch Dis Child	Retrospective study	13 SMA I	4 – 24 m	All treated with NIV + MI-E, 5 died, duration of NIV not specified NIV + MI-E associated with a decrease in chest deformity
Ottonello et al. [7]	Italy	Am J Phys Med Rehab	Retrospective study	16 infants with SMA I	< 3 yrs	All treated with NIV NIV associated with a reduction in ARF episodes
Lemoine et al. [8]	USA	Pediatr Crit Care Med	Retrospective study	49 infants with SMA I	1 – 7 m	All treated with NIV Longer survival in the pro-active (n=26, BPAP + MI-E) vs supportive group (n=23, suctioning ± O_2)
Gregoretti et al. [9]	Italy	Pediatrics	Retrospective study 1999-2010	194 infants with SMA I		31 (16%) treated with NIV Nearly all non treated patients died < 2 yrs Survival at 24 m: 95% for IV vs 68% with NIV Survival et 48 m: 89% for IV and 45% with NIV Longer survival with NIV as compared to no respiratory support
Verrillo et al. [10]	Italy	Sleep Med	Prospective study	9 children with SMA II + 15 healthy controls	2.2 - 8.1 yrs	PSG during before and with NIV (after a mean of 2 yrs of NIV) NIV associated with a decrease in awakenings + increase in >% of N2 sleep satge NIV associated with a decrease in cyclic-alternating pattern A1 duration and an increase in A3 index
Ishikawa et al. [11]	Japan	Neuromuscul Disord	Retrospective study	3 cohorts of Duchenne: untreated, tracheotomy, NIV		88 treated with NIV Longer survival with NIV (mean 39.6 yrs)

Eagle et al. [12]	UK	Neuromuscul Disord	Retrospective study 1 center 1967-2002	197 patients with Duchenne		Improvement in survival 1960s: mean age of death = 14.4 yrs In 1990 with NIV: 25.3 yrs
Lee et al. [13]	Korea	Korean J Pediatr	Retrospective study at one center 2010-2016	54 patients with Duchenne, 24 treated with NIV	NIV treated: mean 16.3 ± 1.9 yrs	Improved cardiac function in the NIV patients As compared to the no-NIV group, the NIV group had (better): Lower early ventricular filling velocity (VFV)/late VFV Higher tissue Doppler systolic S' (i.e. better LV systolic function)
LoMauro et al. [14]	Italy	Eur Respir J	7 yr retrospective study	115 patients with Duchenne	6-24 yrs	28/115 treated with NIV NIV associated with a transient (2 yrs) increase in % vital capacity and contribution of the abdomen to the vital volume (VAB%VT) before coming comparable to the no-NIV pts
Mellies et al. [15]	Germany	Neurology	Retrospective study	7 patients with juvenile Pompe disease	3 – 27 yrs	2/7 treated with NIV NIV improves nocturnal and daytime gas exchange
Nabatame et al. [16]	Japan	Brain Dev	Retrospective study	4 juvenile Pompe disease	9 – 15 yrs	3/4 treated with NIV With NIV: no ARF and resumption of SDB symptoms
Khan et al. [17]	UK	Arch Dis Child	Retrospective study	4 congenital myopathy, 2 congenital	6 – 13 yrs	All treated with NIV NIV improves SDB symptoms, decreases WASO, increases SpO_2

				muscular dystrophy, 2 rigid spine		
Simonds et al. [18]	UK	Eur Respir J	Retrospective study	40 children with NMD or skeletal disease	9 mo 16 yrs	38/40 tolerated NIV NIV associated with an improvement in nocturnal $PtcCO_2$ and SpO_2 and daytime blood gases
Mellies et al. [19]	Germany	Eur Respir J	Prospective study	30 children with progressive NMD	12.3 ± 4.1 yrs	NIV normalized daytime and nocturnal gas exchange, improved RDI + arousal index, decreased nocturnal heart rate, decreased light sleep, and increased slow wave sleep. 10 patients were also studied with and after 3 nights without NIV: NIV withdrawal was associated with a prompt deterioration of SDB and gas exchange back to baseline but resolved immediately after resumption of NIV
Dohna-Schwake et al. [20]	Germany	Pediatr Pulmonol	Retrospective study	12 children with NMD treated with NIV > 5 yrs		As compared with the yr before NIV, after NIV: decrease in the number of GP consultations for RTI; number of antibiotic treatments and number of hospital admissions due to RTI
Katz et al. [21]	Canada	Arch Dis Child	Prospective cohort study	46 children with progressive NMD	6 - 17 yrs	7/46 had nocturnal hypoventilation (NH: increase in $PetCO_2$ > 10 mmHg ± decrease in SpO_2 > 5% for > 10 min): 6 treated with NIV After one year of NIV (5 patients): greater decrease in the general perception of health status of the Child Health Questionnaire (CHQ-PF50) as compared to the

						children without NH
Zaman-Haque et al. [22]	Canada	Child Neurol Open	Retrospective study	7 children with NMD and 7 with CNS disease		CNS: Trisomy 18, Prader Willi syndrome, Leigh sd, cerebral palsy No change in the number of pneumonias 2 yrs before and after the start of NIV
Padman et al. [23]	USA	Pediatr Pulmonol	Retrospective study	15 children with ARF treated with BPAP: 4 CF and 11 NMD	4 - 21 yrs	1 BPAP failure (1 patient with NMD) In other patients: duration of BPAP 1 day to 21 m Decrease in the number of hospitalisation days in the yr after BPAP initiation vs the yr before: 6 vs 36 days/yr
Fauroux et al. [24]	France	Pediatr Crit Care	Retrospective study	15 children who were decannulated and had a relapse OSA	2 – 12 yrs	Pathologies: upper airway obstruction (n=13), congenital diaphragmatic hypoplasia (n=1) or lung disease (n=1) In 9 patients, NIV was started after recurrence of obstructive symptoms after a delay of 1 to 48 m following a successful immediate decannulation. NIV was anticipated in 6 patients who failed repeated decannulation trials because of poor clinical tolerance of tracheal tube removal or tube closure during sleep NIV avoids re-cannulation in case of relapse of OSA

Abbreviations: m: months, yrs: years, SMA: spinal muscular atrophy, RF: respiratory failure, ARF: acute respiratory failure, NIV: noninvasive ventilation, MI-E: mechanical insufflation-exsufflation, IV: invasive ventilation, NMD: neuromuscular disease, CNS: central nervous system, CF: cystic fibrosis, BPAP: bilevel positive airway pressure,O_2: oxygen, OSA: obstructive sleep apnea, GP: general practitioner, PSG: polysomnography, WASO: wake after sleep onset, SpO_2 :pulse oximetry, RDI: respiratory disturbance index, RTI respiratory tract infection.

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