Online Table S2.2: Patients (pathologies) who may benefit from noninvasive ventilation (NIV).

Author	Country	Journal	Type of study	Disorders	Ages	NIV	Comments
Birnkrant et al. [1]	USA	Pediatr Neurology	Retrospective study	4 SMA I	2, 4, 7, and 7 m	NIV for 1, 3, 5, and 5 m	All died before 1 yr
Bach et al. [2]	USA	Chest	Retrospective study	11 SMA I	2 - 11 m	11 treated with NIV after ARF, 8 continued NIV during 15 to 59 m	
Bach et al. [3]	USA	Pediatr Pulmonol	Retrospective study	56 SMA I	Respiratory failure before 2 yrs	33 treated with NIV compared to 16 treated with tracheotomy	31/33 survived to 42 ± 26 m, fewer hospitalisations > 5 yrs as compared to tracheotomy
Bach et al. [4]	USA	Am J Phys Med Rehab	2 cases	2 SMA I	7 m, 3 yrs	2 “high span” NIV (NIV settings not specified)	No pectus excavatum, survival until 7 and 3 yrs with NIV 24h/24
Bach et al. [5]	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 were 65.2 ± 45.8 m, 8 died	Same survival with NIV as compared to tracheotomy but fewer hospitalisations with NIV
Lemoine et al [6]	USA	Pediatr Crit Care Med	Retrospective study	49 infants with SMA I	1 - 7 m	All treated with NIV	Longer survival in the proactive as

							compared to the supportive group
Chatwin et al. [7]	UK	Arch Dis Childh	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. [8]	Italy	Am J Phys Med Rehab	Retrospective study	16 infants with SMA I	< 3 yrs	All treated with NIV	NIV reduces ARF
Gregoretti et al. [9]	Italy	Pediatrics	Retrospective study 1999-2010	194 children with SMA I		121 (62%) no respiratory support, 42 (22%) IV, 31 (16%) NIV The choice of NIV increased from 8% in 1999-2004 to 23% in 2005-2010	Survival with NIV was 68% at 2 yrs and 45% at 4 yrs (95% and 89% with IV) Nearly all non treated patients died < 2 yrs of age
Pane et al. [10]	Italy	Neurology	Retrospective study	122 children with SMA I	3 - 266 m	Survival only possible with NIV > 16h/24 or tracheotomy + nutritional support after the age of 2 yrs	
Ioos et al. [11]	France	Chest	Retrospective cohort	33 SMA I, 35 SMA I-II 100 SMA II 12 SMA II	? (no access)	NIV for 43% SMA I-II 38% SMA II	
Mellies et al. [12]	Germany	Neuromuscul Disord	Prospective study	6 infants SMA I + 1 SMA II (+ 6 SMA controls without NIV)	6 -11 yrs	7 treated with NIV	6 – 12 m: improvement in sleep disordered breathing symptoms, sleep

							quality + architecture
Vasconcelos et al. [13]	Portugal	Revista Port Pneumol	Retrospective study	7 SMA I, 11 type II, 4 type III	6 m -26 yrs	17/22 treated with NIV	NIV associated with a decrease in thoracic deformity and ARF
Han et al. [14]	Korea	PlosOne	Retrospective study in one center (2000-2013)	Home mechanical ventilation in 57 children with child-onset NMD: 58% SMA, 51% SMA I		NIV in only 9/57 children	Decrease of hospitalisations after start of home mechanical ventilation but most children (48/57) were on IV
Kapur et al. [15]	Australia	Pediatr Pulmonol	Cross-sectional study	3 SMA type I, 15 SMA type II and 7 SMA type III	0 - 18 yrs	10 (40%) required NIV: 5 for sleep disordered breathing, 5 for lower respiratory tract infection in the pediatric intensive care unit	
Markstrom et al. [16]	Sweden	Acta Pediatr	Retrospective study	18 infants treated with NIV: 7 SMA intermediate, 3 CCHS, 2 Down syndrome, 2 NMD, 1 diaphragmatic paralysis, 1	1 - 12 m	All treated with NIV	NIV for hypoventilation in 12 and cough/recurrent infections in 6 SMA Duration of NIV 1 – 84 m NIV was discontinued in 6

				myelomening cele, 1 Leigh's sd			infants
Ishikawa et al. [17]	Japan	Neuromuscul Disord	Retrospective study	3 cohorts of Duchenne patients: untreated, tracheotomy, NIV		88 treated with NIV	Longer survival with NIV (mean 39.6 yrs)
Mellies et al. [18]	Germany	Eur Respir J	Retrospective study	5 Duchenne 9 muscular dystrophy 12 neuropathy 4 other NMD	6 - 19 yrs	All treated with NIV	NIV improves daytime and nocturnal gas exchange and sleep quality Re-appearance of sleep disordered breathing with NIV withdrawal
Mellies et al. [19]	Germany	Neurology	Retrospective study	7 juvenile Pompe disease	3 - 27 yrs	2/7 treated with NIV	NIV improves nocturnal and daytime gas exchange
Nabatame et al. [20]	Japan	Brain & Develop	Retrospective study	4 children juvenile Pompe disease	9 - 15 yrs	3 / 4 treated with NIV	No deaths and resumption sleep disordered breathing symptoms
Suresh et al. [21]	Australia	J Pediatr Child Health	Retrospective study	34 Duchenne	1 - 15 yrs	11 treated with NIV because of hypoventilation	

Khan et al. [22]	UK	Arch Dis Childh	Retrospective study	8 children: 4 congenital myopathy 2 congenital muscular dystrophy 2 rigid spine	6 - 13 years	All treated with NIV	Decrease in sleep disordered breathing symptoms, decrease wake time, better SpO_2
Katz et al. [23]	Canada	Arch Dis Childh	Prospective study	49 children with progressive NMD	6 - 17 yrs	7 had nocturnal hypoventilation 6 were treated with NIV	After one yr of NIV: greater decrease in general percept-ion of health status on the Child Health Questionnaire (CHQ-PF50)
Kherani et al. [24]	Canada	Pediatr Pulmonol	Retrospective study	51 infants with NMD	< 1 yr	25/51 treated with NIV 56% NMD 7 /25 weaned from NIV 4 children NIV for palliative care	
Simonds et al. [25]	UK	Eur Respir J	Retrospective study	40 children with NMD or skeletal disease	9 m-16 yrs	38/40 tolerated NIV	Improvement in nighttime and daytime blood gases
Wallis et al. [26]	UK	Arch Dis Childh	Cross sectional survey	933 children on home ventilation	< 17 yrs	704 treated with NIV, 25 CCHS, 88 Duchenne, 10 SMA, 90 other NMD, 9	

			(questionnaire)			cyphoscoliosis, 58 Prader Willi syndrome/obesity	
Sato et al. [27]	Japan	Brain Development	Retrospective survey	48 patients with Fukuyama congenital muscular dystrophy	3.6-31.9 yrs	14 treated with NIV (mean age at NIV start 12 yrs)	
Nadeau et al. [28]	UK	Neurology	Retrospective study	13 patients with Ullrich congenital muscular dystrophy	> 15 yrs at last evaluation	9/13 started NIV at a mean age of 14.3 yrs	
Yonekawa et al. [29]	Japan	J Neurol, Neurosurg & Psychiatry	Cross sectional survey (questionnaire)	33 children + adults with Ullrich congenital muscular dystrophy		NIV started in 13 children, mean age 11.2 yrs	
Quijano-Roy et al. [30]	France	Neuromuscul Disord	Retrospective study	7 children with COL6 myopathy	6.7 ± 8.7 yrs	NIV in 2/7 patients	
Muntoni et al. [31]	UK	Eur J Pediatr Neurol	Retrospective study	5 patients with new form of muscular dystrophy with	< 11 yrs	3 on NIV (2 died)	

				secondary merosine deficiency			
Scoto et al. [32]	UK	Neurology	Retrospective survey	41 children and adults with SEPN1-related myopathy	1-60 yrs	Mean age of NIV start 13.9 yrs 1 child full-time NIV at 1 yr	
Schara et al. [33]	Germany	Eur J Pediatr Neurology	Retrospective survey	11 children with SEPN1-related myopathy	5-21 yrs	NIV in 4 children at a mean age of 11 yrs	
Caggiano et al. [34]	France	Neuromuscul Disord	Retrospective study	6 children with SEPN1-related myopathy (+1 adult)	1-18 yrs	5 treated with NIV (diaphragmatic dysfunction)	
Caggiano at al. [35]	France	Eur J Pediatr Neurology	Retrospective study	5 infants with congenital myasthenic syndrome	3, 6 and 24 mo	3/5 infants treated with NIV	
Payo et al [36]	Spain	Eur Spine J	Retrospective study	24 children severe scoliosis (17 NMD, 7 other)	9-19 yrs	8 children long term NIV (pre-operative)	
Kirk et al.	Canada	Pediatr	Retrospective	73 children with	0-18 yrs	25 central sleep apnea: 7 required NIV	

[37]		Pulmonol	study	myelomening ocele		30 with OSA: CPAP successful in 18/21	
Nashed et al. [38]	Canada	J Inherited Metabolic Dis	Retrospective study	11 children with mucopolysacc haridosis	08-17.8 yrs	4 treated with NIV	
Tibbals et al. [39]	Australia	Pediatr Pulmonol	Retrospective study	4 children with CCHS	6-16 yrs	4 treated with NIV	
Vanderlaan et al. [40]	USA	Pediatr Pulmonol	Cross-sectional survey (questionnaire)	196 patients with CCHS	0.4-38 yrs	55 treated with NIV, 5 with negative pressure ventilation, 17 with phrenic nerve pacing	
Hasegawa et al. [41]	Japan	Pediatr International	Cross sectional survey (questionnaire)	37 CCHS	4 m-34 yrs	14 treated with NIV + 1 with phrenic nerve pacing	
Diep et al. [42]	USA	Respiration	Retrospective study	18 CCHS	19.5 ± 10 yrs	3 prior NIV, 13 transitioned to phrenic nerve pacing with success, 1 failure due to upper airway obstruction	
Facchina et al. [43]	France	Am J Clin Gen Part A	Retrospective study	16 children with mucopolysacc haridosis type IVA	10.5 ± 4.2 yrs	2/16 treated with NIV 4./16 treated with CPAP (all > 11 yrs)	

				(Morquio)			
Tabone et al. [44]	France	Am J Med Gen Part A	Retrospective study	7 patients with mucolipidosis (5 type II, 1 II-III, 1 III)	0.3-17.4 yrs	5 treated with CPAP, 1 with NIV due to hypoventilation	
Dudoignon et al. [45]	France	Am J Med Gen Part A	Retrospective study	57 children with Down syndrome	6.2 ± 5.9 yrs	4/57 treated with NIV 15/57 treated with CPAP	
Clift et al. [46]	UK	J Sleep Research	Retrospective study	17 children with Prader Willi syndrome	?	7/17 treated with CPAP or BPAP (most obese, 2 did not tolerate)	
Pavone et al. [47]	Italy and France	Pediatr Pulmonol	Retrospective study	70 children and 18 adults with Prader Willi syndrome		16/88 treated with CPAP or BPAP (> older patients)	
Repucci et al. [48]	Canada	Orphanet J Rare Dis	Retrospective study	6 children with ROHHAD syndrome	4.7 - 10 yrs	1 died 5/6 treated with BPAP	
Padman et al. [49]	USA	Clin Pediatr	Retrospective study on effect of BPAP on sleep parameters	10 children with OSA	3 - 18 yrs	All treated with BPAP: 3 craniofacial malformation, 1 NMD, 6 obesity 8 continued BPAP	

Pellen et al. [50]	Australia	Int J Pediatr Otorhinolaryngol	Retrospective study	16 infants with congenital tracheal stenosis	0 – 9 m	All treated with NIV pre- post operative, age at start 1 – 6 m, duration 1 – 24 m, 2 (20%) discharged home on NIV	
Archangelidi et al. [51]	UK	J Cystic Fibrosis	Data from UK cystic fibrosis registry 2007-2015	1107/11079 (10%) patients with cystic fibrosis had at least on record with NIV	Children + adults	For children (only): Median age at NIV initiation 13.5 yrs	NIV associated with increased risk of death/transplant 16% of children with NIV died during follow up

Abbreviations: m: months, yrs: years, SMA: spinal muscular atrophy, ARF: acute respiratory failure, NIV: noninvasive ventilation, MI-E: mechanical insufflation-exsufflation, IV: invasive ventilation, NMD: neuromuscular disease, CCHS: congenital central hypoventilation syndrome, BPAP: bilevel positive airway pressure, ROHHAD syndrome: rapid-onset obesity with hypoventilation, hypothalamic dysfunction, and autonomic dysregulation (ROHHAD) syndrome, OSA: obstructive sleep apnea.

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