Table R1: Comparison of baseline characteristics of patients with DM1 who accepted NIV within 1 year after prescription (eNIV group) versus the other patients (l/noNIV group)

Variables		**eNIV n=72**	**l/noNIV n=118**
Male gender, n (%)		42 (58)	73 (62)
Age at prescription, mean±SD		45.9±10.2)	44.1±12.7)
Lung function parameters			
VC <50% of predicted, n (%)		14 (19)	42 (36) †
$PaCO_2$ >45 mmHg, n (%)		52 (72)	108 (92) †
PCF[a], n (%)	*L/min*		
	<180	3 (4)	7 (7)
	180 - 270	7 (10)	12 (10)
	>270	62 (86)	98 (83)

NIV, non-invasive ventilation; VC, vital capacity as % of the predicted value according to Quanjer et al. [20]; PCF, peak cough flow; $PaCO_2$, partial pressure of carbon dioxide in arterial blood

[a]Peak cough flow cut-off values of 270 L/min and 180 L/min were used to define cough impairment [25].

Mantel-Haenszel chi-square test or Fisher's exact test or Student's *t* test or Welch-Satterthwaite's *t* test, † $p<0.05$ and †† $p<0.0001$

Annex 1: STROBE guidelines

Cohort study

This was a longitudinal cohort study of adults with DM1 whose respiratory function was evaluated as part of their usual follow-up at the home ventilation unit of the Raymond Poincaré University Hospital (Garches, France) between 1997 and 2013. DM1 was diagnosed using the conventional Southern blot-based method, according to international criteria [15]. The Myology Institute refers all patients with DM1 to our institution (Raymond Poincaré University Hospital) when respiratory dysfunction is suspected. No patient had respiratory failure before the diagnosis of DM1. Patients receiving ventilation and those deemed at risk for hypercapnia are offered a routine annual follow-up visit at our institution.

Data collection

We recorded gender, height, weight, age at the time of the evaluation, arterial blood gas values ($PaCO_2$, PaO_2, pH, CO_2, SaO_2, HCO3-), and pulmonary function test (PFT) results (vital capacity [VC] in the upright and supine positions, maximal inspiratory pressure [MIP], maximal expiratory pressure [MEP], and peak cough flow [PCF]). PFTs were performed according to ATS/ERS recommendations [16, 17] using a Vmax 229 Sensormedics System (Yorba Linda, CA, USA). MIP was measured from the functional residual capacity in the upright position [18] and MEP at total lung capacity [18]. The best value of each parameter was recorded [19, 20]. MIP and MEP were also expressed as the percentage of the estimated lower limit of normal [21]. PCF was measured using a well-fitted facemask (Leadal Medical, Limonest, France) instead of a mouthpiece, placed around the mouth to allow mouth opening and to minimise cheek compliance. Care was taken to avoid leaks around the mask. Patients were asked to cough as hard as possible, and the highest PCF obtained from three cough manoeuvres producing values within 10% of the maximal value was recorded. PCF cut-off

values of 270 L/min and 180 L/min were used to detect cough impairments, as they are considered indicative of possible respiratory failure development during respiratory tract infections and of ineffective airway clearance, respectively [22].

Accepted criteria for NIV initiation are as follows [23]: symptoms suggestive of hypercapnia (dyspnoea at rest and upon exertion, morning headaches, orthopnoea, sleep disturbances, diurnal sleepiness); combined with alveolar hypoventilation ($PaCO_2 \geq 45$ mmHg) or nocturnal arterial oxygen desaturation by pulse oximtery ($SpO_2 \leq 88\%$ for 5 consecutive minutes), or restrictive lung disease (VC <50% of predicted). However, because patients with DM1 frequently underestimate their symptoms, for this study, the presence of an objective criterion was considered sufficient to start NIV even in patients who reported no symptoms [23]. Patients who started NIV within 1 year after meeting the criteria were classified in the early NIV (eNIV) group and patients who started NIV later on or not at all were classified in the late/no NIV (l/noNIV) group.

For all patients who used NIV at home, we recorded the following: symptoms suggesting respiratory failure (dyspnoea at rest and upon exertion, morning headaches, orthopnoea, sleep disturbances, diurnal sleepiness), functional autonomy (ability to walk with or without help), NIV mode, and adherence to the prescribed NIV duration (hours/day). Mean NIV duration/day was collected from the machine's software by a technician from the healthcare company providing the NIV equipment, at the patient's home before each visit. NIV adherence, calculated as the ratio of NIV hours/day over prescribed NIV hours/day, was categorised in three groups (<75%, 75-90%, and >90%).

Methods of follow-up

Data were collected prospectively during the first respiratory function evaluation then during the routine annual follow-up evaluations, for 10 years or until death or until the patient

was lost to follow-up, whichever occurred first. Vital status was determined by contacting the vital records office of the jurisdiction of birth. Subsequently, cohort participants were informed of the progress of the study by e-mail or telephone calls."

Reference

15. Jennekens, F. G. I., Ten Kate, L. P., De Visser, M., & Wintzen, A. R. Diagnostic criteria for Duchenne and Becker muscular dystrophy and myotonic dystrophy. Neuromuscular Disorders, 1991, vol. 1, no 6, p. 389-391.

16. Miller MR, Hankinson J, Brusasco V, Burgos F, Casaburi R, Coates A, et al. Standardisation of lung function testing: the authors' replies to readers' comments. *Eur Respir J* 2010; 36:1496–8.

17. Quanjer PH, Tammeling GJ, Cotes JE, Pedersen OF, Peslin R, Yernault JC. Lung volumes and forced ventilatory flows. Report Working Party Standardization of Lung Function Tests, European Community for Steel and Coal. Official Statement of the European Respiratory Society. *Eur Respir J* 1993; 16:5–40.

18. Prigent H, Lejaille M, Falaize L, Louis A, Ruquet M, Fauroux B, et al. Assessing inspiratory muscle strength by sniff nasal inspiratory pressure. *Neurocrit Care* 2004; 1:475–8.

19. Hart N, Polkey MI, Sharshar T, Falaize L, Fauroux B, Raphaël JC, et al. Limitations of sniff nasal pressure in patients with severe neuromuscular weakness. *J Neurol Neurosurg Psychiatry* 2003; 74:1685-7.

20. Stefanutti D, Benoist MR, Scheinmann P, Chaussain M, Fitting JW. Usefulness of sniff nasal pressure in patients with neuromuscular or skeletal disorders. *Am J Respir Crit Care Med* 2000; 162:1507–11.

21. Evans JA, Whitelaw WA. The assessment of maximal respiratory mouth pressures in adults. *Respir Care* 2009; 54:1348–59.

22. Bott J, Blumenthal S, Buxton M, Ellum S, Falconer C, Garrod R, et al. Guidelines for the physiotherapy management of the adult, medical, spontaneously breathing patient. *Thorax* 2009; 64:i1–51.

23. Clinical indications for noninvasive positive pressure ventilation in chronic respiratory failure due to restrictive lung disease, COPD, and nocturnal hypoventilation--a consensus conference report. Chest 1999;116:521-34.

Table R2: Predictors of death in the non-users or late users of NIV (n=118)

Variables	Univariate model		Multivariate model	
	HR [95%CI]	***p* value**	**HR [95%CI]**	***p* value**
Male gender	0.62 [0.31 – 1.23]	0.15	0.54 [0.21 – 1.47]	NS
Age at NIV prescription				
<40	1*		1*	
40 - 50	2.44 [0.77 – 7.72]	**0.005**	1.25 [0.19 – 8.19]	**0.001**
>50	4.97 [1.83 – 13.44]		8.91 [2.16 – 38.81]	
CTG repeats	1.00 [0.95 - 1.05]	NS	-	-
BMI	1.00 [0.99 - 1.00]	NS	-	-
Respiratory function				
PCF, n (%) L/min				
>270	1*		1*	
180 - 270	2.55 [0.99 - 6.58]	**0.001**	5.51 [0.60 - 50.93]	**0.03**
<180	4.51 [1.93 - 10.58]		4.38 [1.44 - 13.29]	
Vital capacity (VC %)	0.98 [0.96 - 0.99]	**0.01**	0.99 [0.96 - 1.02]	NS
MEP	1.01 [0.99 - 1.03]	NS	-	-
MIP	0.99 [0.97 -1.00]	0.12	1.00 [0.97 - 1.03]	NS
$PaCO_2 \geq 45$ mmHg	1.33 [0.66 - 2.68]	NS	-	-

NIV, non-invasive ventilation; VC, vital capacity as % of the predicted value according to Quanjer et al. [17]; PaCO2, partial pressure of carbon dioxide in arterial blood. Peak cough flow cut-off values of 270 L/min and 180 L/min were used to define cough impairment [22]. HR, Hazard Ratio. CI, confidence interval. NS, non-significant. MEP, maximum expiratory pressure; MIP, maximum inspiratory pressure.