RWD165

INEQUITIES RELATED TO LIFE EXPECTANCY OF DUCHENNE MUSCULAR DYSTROPHY IN BRAZIL: AN ADMINISTRATIVE DATABASE ANALYSIS

Daniela V Pachito, Harryson WG dos Santos, Paulo H. R. F. Almeida

Pfizer Brazil, São Paulo, Brazil

INTRODUCTION

Duchenne muscular dystrophy (DMD) is a X-linked recessive neuromuscular disease and the most common type of muscular dystrophy among children.[1] Symptoms usually start in the early childhood, including proximal muscle weakness, clumsiness, and difficulty in stair climbing.[2] Despite the advances in terms of the development of new modalities of care, DMD remains an incurable condition. In a recent systematic review that pooled individual participant data, life expectancy (LE) in DMD was 22 years old (95% CI 21.2 to 22.4), with increases of LE being observed for patients born after 1990 (LE = 28.1 years [95% CI 25.1 to 30.3]).[3] The vast majority of studies included in this review were conducted in developed countries. Therefore, there are still uncertainties related to the course of disease and LE in other settings, where supportive care may be less accessible.

OBJECTIVE

The objectives of the current work are to estimate LE of DMD in Brazil and to investigate the correlation between the Human Development Index (HDI) and LE in DMD and to assess regional disparities and time trends.

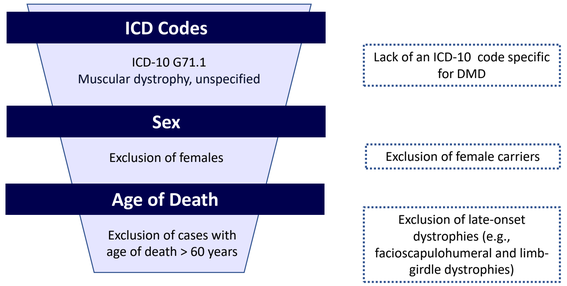
METHODS

Death records from 2016 to 2020 were extracted from the *Sistema de Informação de Mortalidade* (SIM) - DATASUS database, the information system that records deaths in Brazil. HDI of municipalities of residency of cases was extracted from www.atlasbrasil.org.[4] Period of analysis was 01 Jan 2016 to 31 Dez 2020.

The inclusion criteria were deaths coded with the generic ICD-10 code for muscular dystrophy (i.e., G71.0); male sex; and occurrence of death before 60-yr, as a conservative proxy for DMD cases, since late-life deaths are associated with other muscular dystrophies, such as facioscapulohumeral and limb-girdle dystrophies (**Figure 1**).

Descriptive analyses were performed for continuous variables. Linear regression models of the age at death by percentiles of the HDI and year of death were fitted.

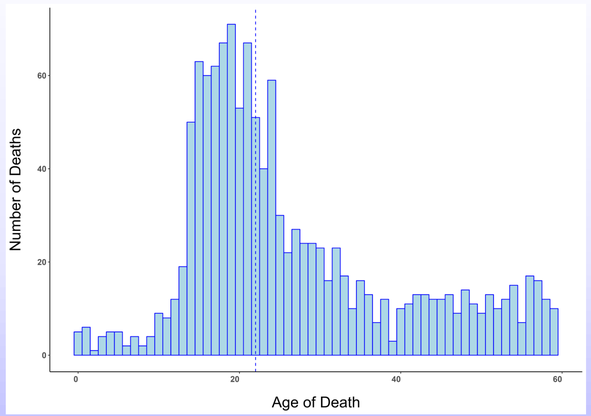
Figure 1. Proxies applied for the identification of DMD cases in DATASUS and rationale



RESULTS

A total of 1,232 deaths associated with ICD-10 code G71.0 was included. Deaths occurred in 648 out of 5570 municipalities in Brazil (11.6%). Distribution of age at the time of death are shown in **Figure 2**.

Figure 2. Distribution of age at the time of death (n= 1,232)



Characterization of municipalities according to the HDI and the number of deaths attributable to DMD is presented in **Table 1**.

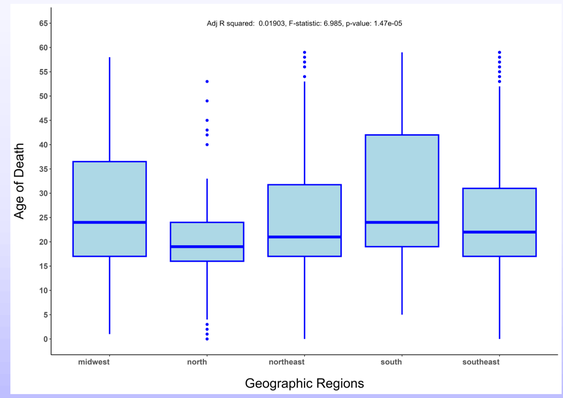
Table 1. Characterization of municipalities according to HDI, population size and number of recorded deaths in 5 years

HDI decile	HDI range	Mean HDI	Number of Municipalities	Number of Municipalities w/ deaths	Population Size	Number of deaths	Deaths/ 100.000 inhabitants
1st decile	0.0 - 0.56	0.53	544	17	9,323,072	19	0.20
2nd decile	0.56 - 0.59	0.57	560	28	8,797,526	29	0.33
3rd decile	0.59 - 0.61	0.60	565	40	10,120,194	44	0.43
4th decile	0.61 - 0.64	0.62	545	41	10,004,596	45	0.45
5th decile	0.64 - 0.67	0.65	555	54	10,515,073	61	0.58
6th decile	0.67 - 0.69	0.68	551	60	12,737,511	70	0.55
7th decile	0.69 - 0.71	0.70	555	53	11,218,179	67	0.60
8th decile	0.71 - 0.73	0.72	563	66	19,556,885	106	0.54
9th decile	0.73 - 0.75	0.74	571	95	26,462,617	172	0.65
10th decile	0.75 - 0.86	0.79	561	194	93,020,039	622	0.67
Total	-	**0.72**	**5,570**	**648**	**211,755,692**	**1,235**	**0.58**

HDI: Human Development Index

Median LE for all cases was 23 yrs (interquartile range [IQR] = 17 to 35). Median LE for the lowest HDI percentile was 17 years and for the highest percentile, 25.2 yrs. LE was strongly correlated with the HDI percentile (R^2 = 0.81, p < 0.001) but not correlated with the year of death. Lower LE was observed in the less developed North region (18 ys, IQR 16.5 to 20.25) and higher LE in the Midwest region (28 ys, IQR 18.75 to 36.75) (**Figure 3**).

Figure 3. Distribution of age of death by geographic region



CONCLUSIONS

Our results show discrepancies in the LE of patients with DMD across the country, with lower LE observed in less developed regions. This observation may be related to factors such as barriers in the access to supportive care in the public health system in Brazil. Further research to underpin equitable health programs is warranted.

Results should be interpreted carefully since the lack of a specific ICD-10 Code for DMD and the proxies applied for the identification of DMD cases may have introduced bias. The lack of an inferior cut off for excluding cases may have led to the inclusion of cases of congenital muscular dystrophy. On the other extreme, the conservative approach of excluding cases of death only after 60 years old may have led to the inclusion of late-onset dystrophies. However, overall estimates for LE are aligned with results from international studies, and assuming a homogenous distribution of non-DMD cases across the country, the correlation between LE and HDI would not be affected.

References

1. Mah JK, Korngut L, Dykeman J, Day L, Pringsheim T, Jette N. A systematic review and meta-analysis on the epidemiology of Duchenne and Becker muscular dystrophy. *Neuromuscul Disord*. 2014;24(6):482-491. doi:10.1016/j.nmd.2014.03.008
2. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neurol*. 2018;17(3):251-267. doi:10.1016/S1474-4422(18)30024-3
3. Broomfield J, Hill M, Guglieri M, Crowther M, Abrams K. Life expectancy in Duchenne muscular dystrophy. Neurology. 2021;97(23):E2304-E2314. doi:10.1212/WNL.0000000000012910
4. Atlas do desenvolvimento humano no Brasil. Retrieved April 1, 2023, from http://www.atlasbrasil.org.br/

Funding

This study was funded by Pfizer, Inc.

For more information please contact:
Pachito, Daniela.
Pfizer Brazil Inc, Alexandre Dumas st., 1860, Sao Paulo - SP, 04717-904, Brazil
email: daniela.pachito@pfizer.com
www.pfizer.com

Presented at the International Society for Pharmacoeconomics and Outcomes Research Annual Meeting; May 7-10, 2023; Boston, MA, USA