

Duchenne Muscular Dystrophy Prevalence in the US: A Novel Incidence-Based Modeling Approach Using System Dynamics

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Introduction

- Duchenne Muscular Dystrophy (DMD) is a progressive, neuromuscular disorder caused by mutations on the X chromosome that result in an absence or deficiency in the protein dystrophin^{1,2}
- DMD primarily affects males with symptom onset typically manifesting in early to mid childhood²
- Symptoms include progressive muscle weakness resulting in loss of ambulation and, in some cases, respiratory impairment or cardiomyopathy²

Objective

- The objective of this study was to estimate the diagnosed incidence and prevalence of Duchenne muscular dystrophy (DMD) in France, Germany, Italy, Spain, and the United Kingdom (UK), accounting for improvements in treatment and care that have altered patient survival

Materials and Methods

- Diagnosed incidence of DMD was estimated for each country via a system dynamics model based on a triangular distribution of incidence rates identified from a systematic review of the available literature^{3,4}
- Diagnosed prevalent cases of DMD were calculated using an incidence to prevalence model and age-specific Kaplan-Meier survival data stratified by birth cohort to account for improvements in treatment practices⁵

Materials and Methods (cont.)

- To calculate the number of diagnosed incident and prevalent cases of DMD in 2019, the incidence rate estimate was multiplied by the sum of country-specific male birth estimates starting in 1971
- The number of patients still alive in 2019 constituted the diagnosed prevalent population, which was subsequently divided by the sum of the projected 2019 male population in each country to yield the diagnosed prevalence rate

Results

- The diagnosed incidence of DMD was estimated to be 19.8 (95% CI: 15.2, 24.7) per 100,000 live male births, corresponding to approximately 330 incident cases in 2019
- The diagnosed prevalence of DMD was found to be 6.52 (95% CI: 5.04, 8.19) per 100,000 male population, corresponding to approximately 10,406 prevalent cases in 2019
- The majority (58.2%) of DMD patients are under the age of 20 and approximately 3% live to be between 40-45 years of age
- The prevalence of DMD among male population 45 years of age or younger is approximately 12.0 (95% CI: 9.2, 15.0) per 100,000

Conclusions

- While the incidence of DMD in these five European countries has remained stable, the prevalence of the disease has increased in the past decades due to improvements in life expectancy
- Additional advancements in treatments may similarly lead to greater longevity of life, thereby further increasing prevalence

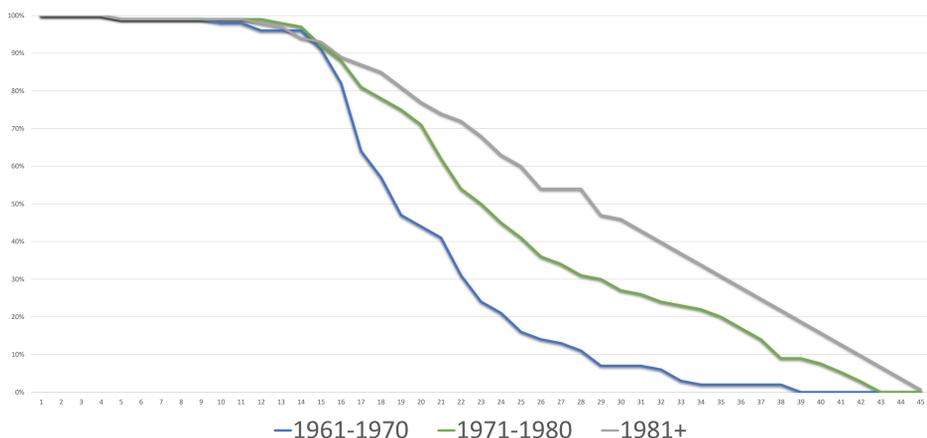


Figure 1. Birth-cohort specific survival of DMD patients.⁵

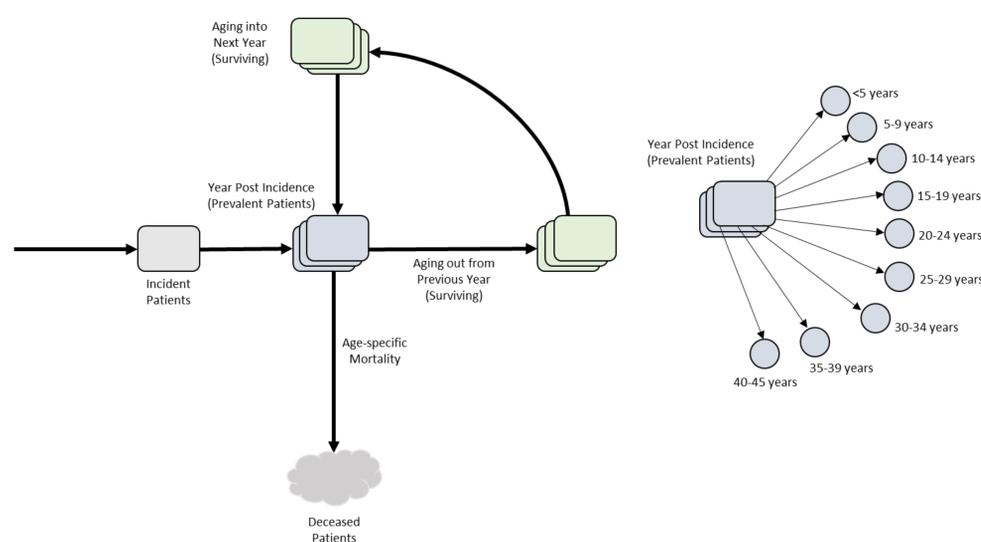


Figure 2. Structure of the system dynamics model of DMD prevalence from year post incidence.

	Rate per 100,000	95% CI
Incidence ^a	19.81	(15.21 – 24.70)
Prevalence ^b	11.96	(9.18 – 14.91)
Age		
0-4	15.75	(12.10 – 19.65)
5-9	19.30	(14.83 – 24.07)
10-14	19.17	(14.72 – 23.90)
15-19	16.77	(12.88 – 20.91)
20-24	14.98	(11.51 – 18.69)
25-29	11.59	(8.90 – 14.45)
30-34	8.93	(6.86 – 11.14)
35-39	5.72	(4.39 – 7.13)
40-45	2.42	(1.85 – 3.01)

^a Incidence rate given as function of incident cases among 2019 EU5 male births

^b Age-specific prevalence rates given as function of age-specific 2019 EU5 population

Table 1. Incidence and prevalence of DMD among 2019 male births and population, respectively.

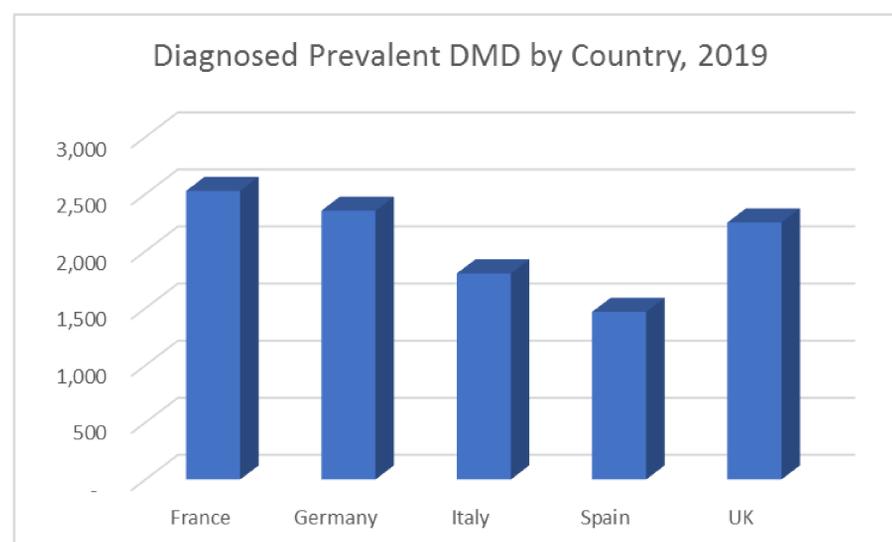


Figure 3. Proportion of prevalent DMD patients by country, 2019

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