

Calculating the Prevalence of Urea Cycle Disorders in the EU5 Using an Incidence-Survival Model

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Background

- Therapies for rare diseases have ignited industry-wide debates concerning patient access, pricing and efficacy. Urea cycle disorders (UCD), a genetic rare disease, is a target for enzyme replacement therapies, yet UCD epidemiology data are scarce. For payers and pharmaceutical companies to accurately forecast patient cost of care, accurate patient population estimates are a necessity.

Objectives

- The objective of this study is to estimate the prevalence of UCD in the 0–17 year-old population in five major European markets (EU5) (France, Germany, Italy, Spain, UK).

Methods

- We included males and females ages 0-17 years. We excluded patients older than 17 years to remove the mild patients, who may not require therapy, and have a population that better represents severe patients requiring chronic, expensive treatment.
- We designed an incidence-survival model utilizing birth incidence, annual life-expectancy and country-specific newborn data. Birth incidence estimates for UCD were obtained from the Urea Cycle Disorders Consortium (UCDC).

- We conducted a literature review to determine the annual life expectancy of UCD.
- Finally, we reviewed available EU5 newborn census data to obtain the number of newborns in each country from 2001 to 2012. We extrapolated the trend from 2001-2012 to forecast the number of newborns through 2024. These data were entered into the incidence-survival model to calculate the prevalence of UCD from 2015-2024.

Results

- We estimated a prevalence of 1.71 per 100,000 population in the 0–17 year age groups resulting in 1,027 and 1,033 UCD cases in the EU5 in 2015 and 2024, respectively.
- The prevalence was 1.89 per 100,000 in the 0-4 year-old age group, 1.63 per 100,000 in the 10-14 year-old age group, and 1.57 per 100,000 in 15-17 year-old age group.
- The survival of UCD decreased considerably over the first 10 years of life, from a 73% survival at age 1 year to a 58% survival at age 10 years.
- The five most frequent subtypes of UCD (OTCD, ASLD, ASSD, ARGD) constitute collectively 93.1% of the UCD population. The proportions are 59.9%, 15.5%, 14.2%, 3.5%, respectively.

Conclusions

- To our knowledge, this study provides the first prevalence estimate of UCD in the EU5 markets.
- Incidence–survival models provide an alternative method to estimating prevalent cases when prevalence data are scarce or unreliable.
- With 1,027 estimated cases in 2015, it is expected that UCD treatments will be expensive given the small size of the patient population.
- With low expected survival, treatment options should target the 0-10 years pediatric age group.
- In conclusion, these estimates can be used to approximate the total cost burden of UCD and the resulting cost-effectiveness of UCD treatments.

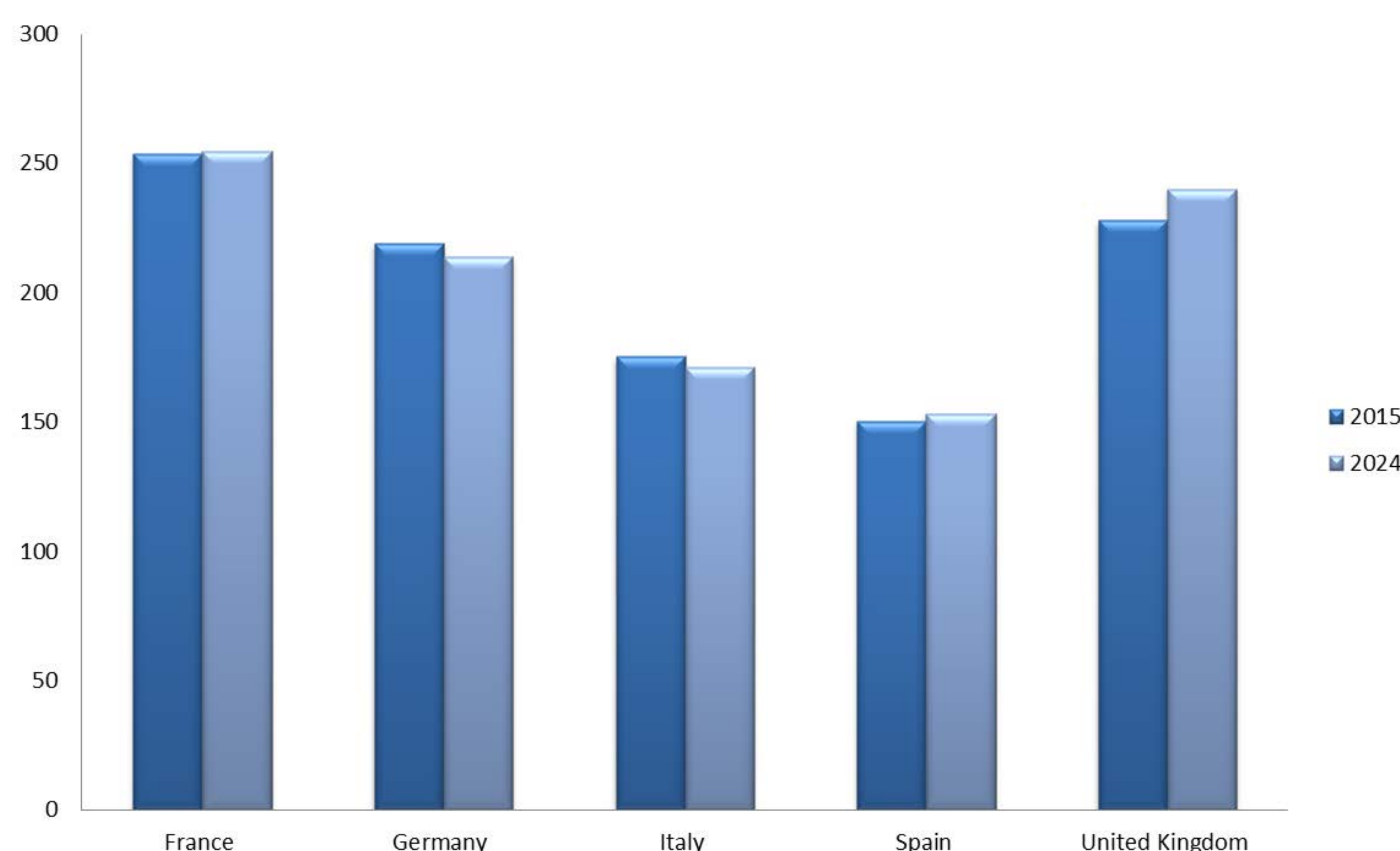


Figure 1: Number of Prevalent Cases in the EU5, 2015, 2024.

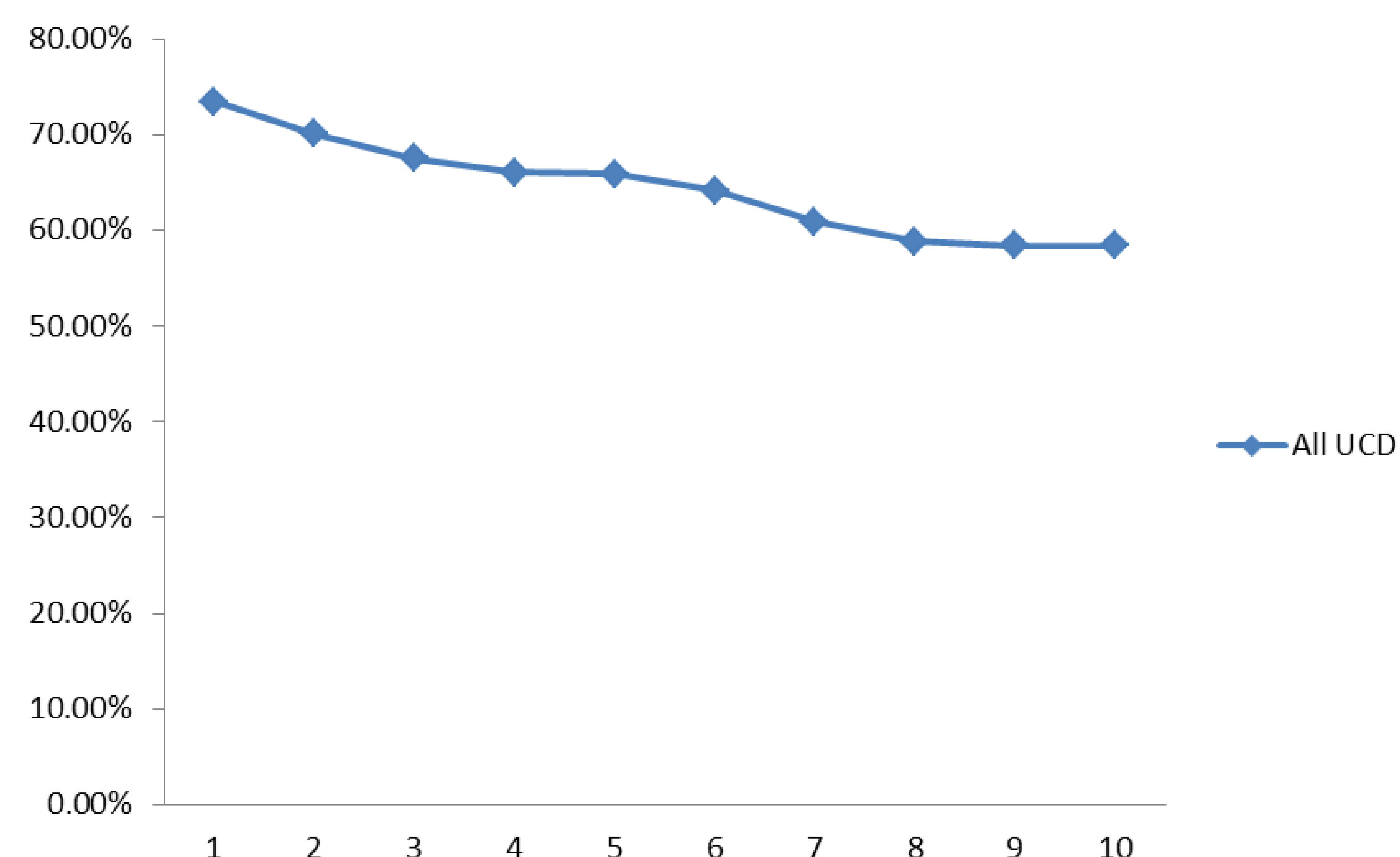


Figure 2: Survival Estimates for UCD Patients Aged 0-10 Years.

Age Groups	Proportion of Patients	Cumulative Patient Proportion
0-3 years	25.01%	25.01%
4-6 years	17.27%	42.27%
7-10 years	21.85%	64.12%
11-13 years	15.52%	79.64%
14-17 years	20.36%	100.00%

Table 1: Age Distribution of UCD Patients, Males & Females, 2015

	France	Germany	Italy	Spain	United Kingdom	EU5
OTCD	152	131	105	90	137	615
ASLD	39	34	27	23	35	159
ASSD	36	31	25	21	32	146
ARGD	9	8	6	5	8	36

Table 2: Number of Prevalent Cases by Subtype in the EU5 in 2015

References:

- Batshaw et al. Mol Gen and Metab. 2014; 113: 127-130
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