THREE DIFFERENT METHODS TO ESTIMATE A RARE DISEASE (RD) PREVALENCE IN A COUNTRY WITH A LIMITED DATA SET: WHICH ONE FITS?

MSR42

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OUR LEARNINGS AND CONCLUSIONS

Machine Learning (ML) is an accurate tool for estimating the current prevalence of hemophilia even in data-limited settings. However, the differences between the prevalences of ML, Ordinary Least Square (OLS) and Multivariate Least Squares (MLS) were marginal.

For countries with limited data and resources, simpler and cheaper tools such as OLS or MLS may be feasible options for estimating the prevalence of rare. Especially if the time horizon is shorter. However, a number of additional patients should be considered to correct for those not estimated or even perform two of these three methods.

THE CONTEXT

Incidence and prevalence data for Rare Diseases (RDs) are difficult to obtain and imprecise, especially in Low- and Middle-Income Countries (L&MICs) (1).

Epidemiological data are essential to adequately plan the current and future service demand and resource allocations (1).

Hemophilia A (HA) and B (HB), are important RDs. Since the pandemic, clotting factors have been in intermittent supply (2-4). Accurate estimation of patients' requirements for clotting factors is imperative for effective demand prediction and patient protection.

PURPOSE

To estimate current and future prevalence of HA and HB with three different methods, identifying their advantages, disadvantages, and constraints in the context of a low or middle-income country

METHODS

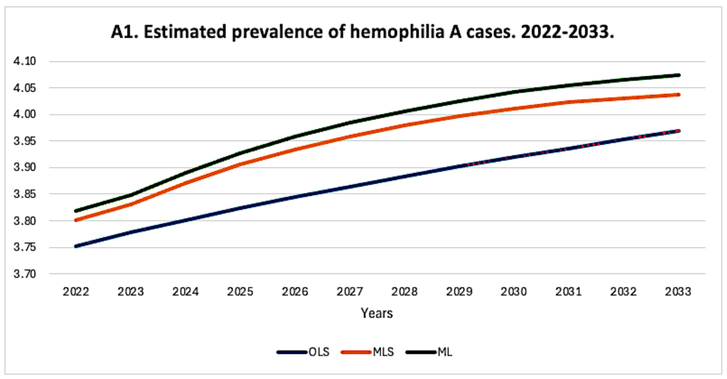
The Ordinary Least Squares (OLS), Multivariate Least Squares (MLS), and Machine Learning (ML) models were performed to reckoning the prevalence of HA and HB in Mexico from 2022 to 2033. Baseline estimators of prevalence and their potential explanatory variables were obtained from national official sources and the World Federation of Hemophilia dataset from 2006 to 2021. Linear, exponential, logarithmic, and polynomial adjustments were performed for OLS, and logarithmic regression was the best fit. For the MLS and ML modelling, "*total population*", "*number of births*", "*health coverage",* and "*mortality*" variables were considered. Due to the limited available datasets, the ML model used a non-deep learning method.

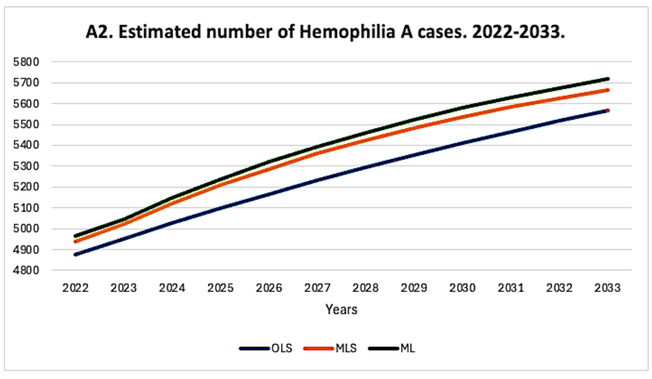
The main characteristics that determined the behaviour of Mexican hemophilia were extracted to train the system and predict the next twelve years' prevalence. Several equations of the explanatory variables were explored to get the best current and future prevalences.

RESULTS

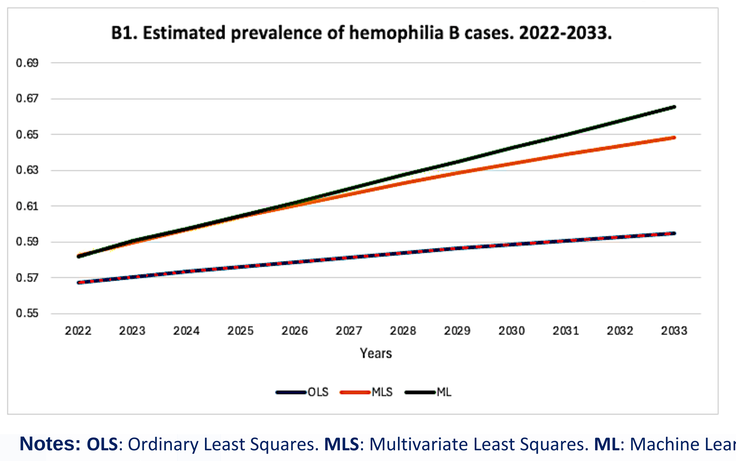
Figures A1, A2, B1 and B2. Estimation of prevalence and new hemophilia A and B cases in Mexico for 2022-2033 years using OLS (Blue line), MLS (Orange line), and ML (Green line) methods. A1. Estimated hemophilia A (HA) prevalence. A2. Estimated number of new cases of HA. B1. Estimated hemophilia B (HB) prevalence. B2. Estimated number of new cases of HB.

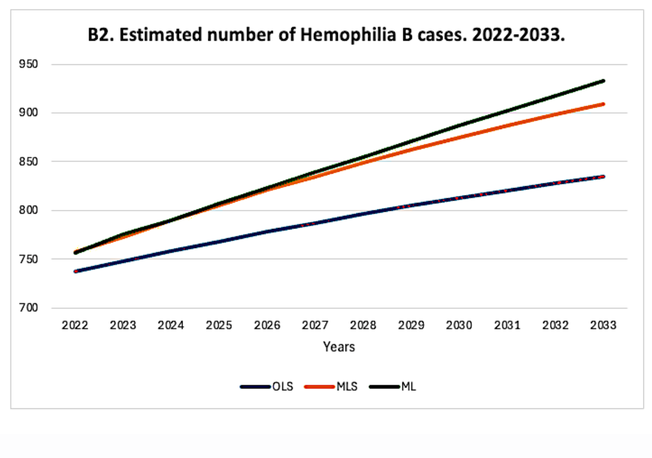
Hemophilia A





Hemophilia B





Notes: **OLS**: Ordinary Least Squares. **MLS**: Multivariate Least Squares. **ML**: Machine Learning

The Mexican prevalence for HA was 2.7 in 2006 and 3.7 in 2021, and for HB was 0.5 in 2006 and 0.56 in 2021. In a decade, the prevalence of HA and HB will increase by 15%. Each model estimated the following cases for HA: OLS 4,877/ 5,567; MLS 4,940/5,664, and ML 4,963/5,717. For HB, the cases were: OLS 737/835, MLS 757/909, and ML 756/933. A ∼2% of differences (HA n=86; HB n=20) in 2022 and, ∼3% of differences for HA n=150, and ∼10% for HB (n=98) in 2033 among these different methods were found.

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