

# Budget impact of inotersen for the treatment of stage I or II polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR) in Mexico

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## Introduction

Hereditary transthyretin-mediated amyloidosis (hATTR) is an extremely rare, multisystemic, progressive, debilitating and deadly disease caused by a hereditary mutation of the transthyretin gene (TTR), of which the main characteristic is polyneuropathy. The disease usually starts during the second and ninth decade of life, but symptoms generally manifest after the third decade of life.<sup>1,2</sup>

Current management of patients with hATTR requires multidisciplinary management due to the fact that the clinical presentation of the disease is systemic and heterogenous. Currently Inotersen is a second generation 2'-O-methoxyethyl-modified antisense oligonucleotide designed to inhibit hepatic production of mutated and wild-type TTR.<sup>3</sup>

The efficacy and safety of Inotersen has been evaluated in a randomized clinical trial in adult patients with hATTR. The modified score for neuropathy impairment +7 (mNIS+7) was evaluated, which showed a statistically significant difference between patients treated with Inotersen vs placebo with a difference in the change for Least Squares (LS) of -19.7 points (confidence interval [CI] of 95%: -26.4 to -13.0; p<0.001) and -11.7 points (95% CI, -18.3 to -5.1; P<0.001) for the Norfolk QOL-DN score.<sup>4</sup>

In a clinical trial with a 5-year follow-up, the obtained results showed the long-term benefit by the improvement of the neuropathy and quality of life.<sup>5</sup> For this, Inotersen can be considered an efficacious and safe option for the treatment of adult patients with hATTR-PN in stages I and II.

## Objectives

To perform a budget impact from the perspective of the Mexican public health sector of the use of inotersen for the treatment of polyneuropathic hATTR in Coutinho stages I and II.

## Methodology

Comparators were selected based on recommendations from international clinical practice guidelines as well as options available for the same indication in México. Estimation of annual costs was performed through a Markov model, where the evidence of efficacy was obtained through a systematic review of the literature. Direct medical costs related to the interventions evaluated were considered and a time horizon of 5 years was used. Results are presented in dollars.

Total expenditure was calculated for two scenarios; expenditure in the current scenario is that incurred by institutions using only standard care (defined by a Delphi panel and includes symptom monitoring).

The new scenario is the one that contemplates the use of inotersen based on its market access rate, which was 3% in the first year, with an annual increase of 3%, reaching 15%, in the fifth year of the analysis (these data were a conservative assumption). Budget impact was expressed as the difference between the two scenarios, expressed as a percentage of the institutions' budgets. Budget data was obtained from official online sources.<sup>6</sup>

## Results

A cohort of 111 prevalent cases was estimated for year one with four incident cases for subsequent years. The introduction of Inotersen resulted in an average annual impact of 2.3 million, which corresponds to 0.036% of the health budget.

**52,791,539** Patients 35-75 years old

**470** hATTR prevalent cases

Prevalence: 0.89 per 100 thousand (González-Duarte, *et al*, 2018)

**188** hATTR cases with neuropathic symptoms

Cases with neuropathic symptoms: 40% (González-Duarte, *et al*, 2018)

**159** Neuropathic hATTR cases in 1 and 2 stages

Stages 1 and 2 of Coutinho: 84.9% (Delphi panel)

**111** Insured Neuropathic hATTR cases in 1 and 2 stages

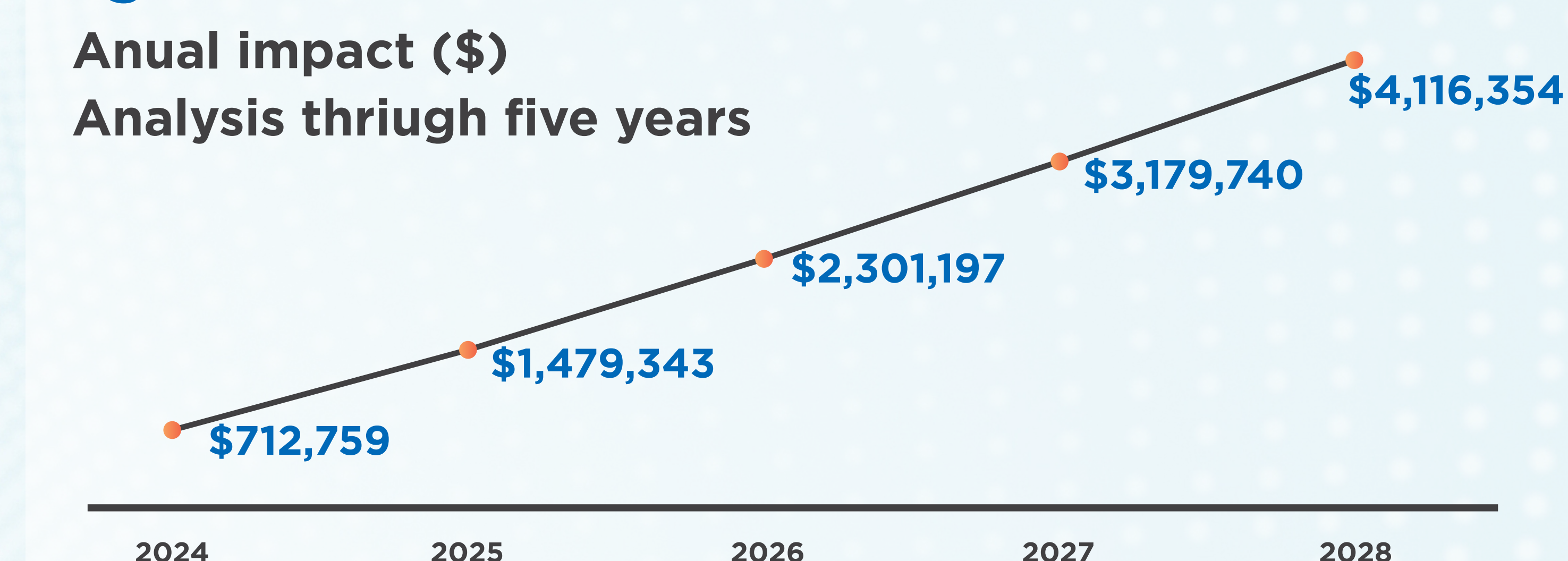
Insured: 70% (INEGI 2020)

**Figure 1.** Population estimate for year one

## References

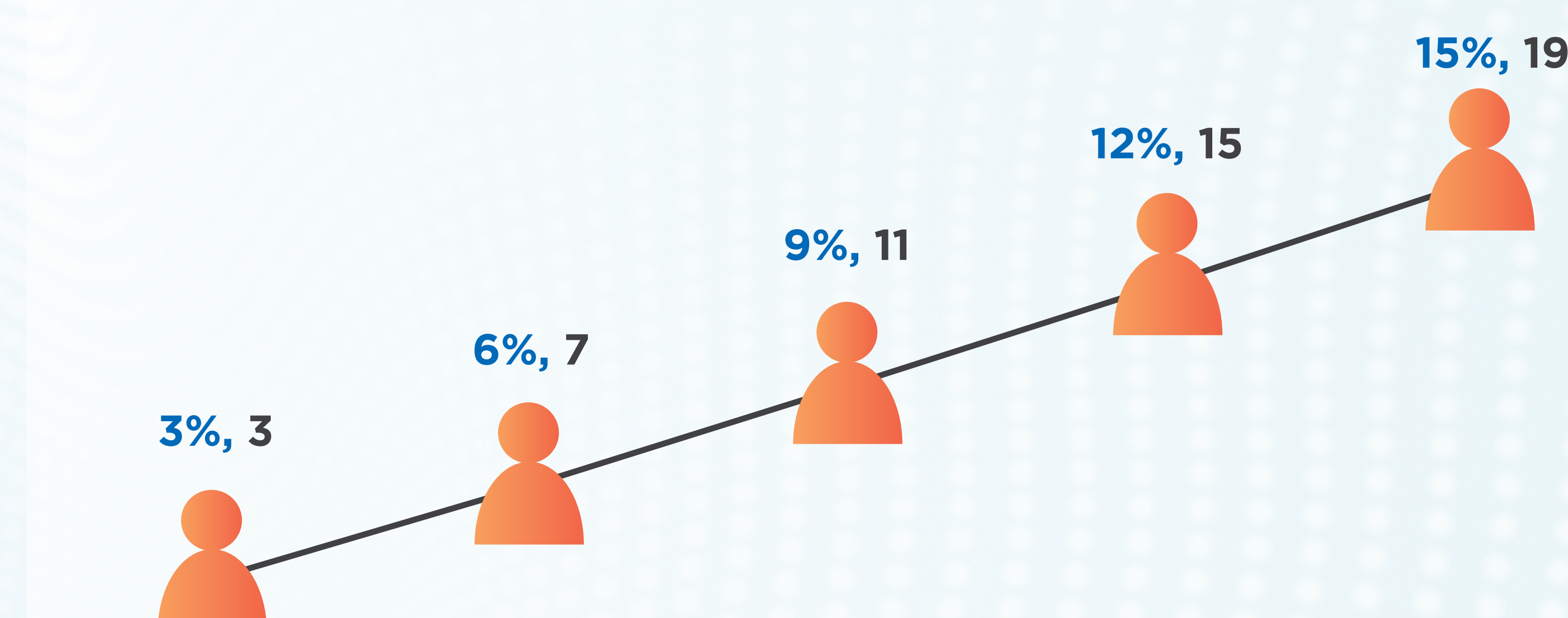
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**Figure 1.** Population estimate for year one



**Figure 2.** Economic impact and market access of inotersen, analysis through 5 years.

**Market access (%); cases treated with inotersen**



## Conclusions

Inotersen is a valuable option for the treatment of polyneuropathic hATTR in Coutinho stages I and II, since it would be the only and first pharmacological option available in Mexico for this condition. Based on this analysis the introduction of inotersen will not impact the Mexican health public sector as it is expected to have a small budget impact.

## DISCLOSURES:

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