DEVELOPMENT OF A RISK-SHARING MODEL BASED ON THE CLINICAL PERFORMANCE OF ONASEMNOMEGNE ABEPARVOVEC (ZOLGENSMA)

A. Figueiredo¹, R. Gonçalves Marques¹,², F. Cosme Silva¹,², A.P. Martins¹

¹ Faculdade de Farmácia da Universidade de Lisboa, Portugal
² Centro Hospitalar Universitário de Lisboa Norte - Hospital de Santa Maria, Portugal
Corresponding Author: acmf@edu.ulisboa.pt

BACKGROUND & IMPORTANCE

Zolgensma® is an innovative gene therapy for Spinal Muscular Atrophy (SMA) infants. Nevertheless, the lifelong clinical follow-up needed for understanding the long-term effectiveness of Zolgensma® in combination with an exceptionally large single payment represents scientific and financial challenges.

AIM & OBJECTIVES

• The scope of this investigation comprises the development of a hypothetical Performance-Based Risk-Sharing Arrangements—Performance Linked Reimbursement (PBRSA-PLR) for Zolgensma® Gene Replacement Therapy (GRT).

MATERIAL & METHODS

• A review of the literature was constructed, comprising 5 phases: (a) identifying the research question; (b) searching for relevant studies; (c) selecting studies; (d) analyzing data; and (e) presenting results.
• A comprehensive English-language literature search of the electronic databases PubMed and Science Direct was undertaken for identifying published papers. Data was collected and analyzed until May 2021.

RESULTS

I. Zolgensma® Outcome-based Scheme

• Relevant Outcomes:
  • overall survival;
  • event-free survival.

II. Zolgensma® Annuity-based Payment Scheme

• Pay-over-time of 5 to 15 years:
  • reduce the annual budget impact;
  • increase patient access.

More favorable outcomes could be achieved if SMA infants started treatment earlier:

I. Maximum 50% Refund: Zolgensma® early dosing in SMA infants (< 3 months old).

II. Maximum 25% Refund: Zolgensma® late dosing in SMA patients (> 3 months < 9 months old).

If Zolgensma® fails to meet the agreed-upon outcomes in the pre-defined timing of outcome assessments.

Scheme 1: Zolgensma® GRT PBRSA-PLR (Outcome-based Scheme and Annuity-based Scheme).

CONCLUSION & RELEVANCE

• We conclude that would be possible to mitigate uncertainty around the incremental budgetary impact and cost-effectiveness of Zolgensma® GRT.


ATC code: 3. Literature reviews