

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

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BACKGROUND & IMPORTANCE

- Zolgensma[®] is an innovative gene therapy for Spinal Muscular Atrophy (SMA) infants. Nevertheless, the life-long 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.

[1] JØRGENSEN, Jesper; KEFALAS, Panos - The use of innovative payment mechanisms for gene therapies in Europe and the USA. Regenerative Medicine. ISSN 1746-0751. 2021). doi: 10.2217/rme-2020-0169.

[2] KIM, Andy Eunwoo et al. - Performance-Based Risk-Sharing Arrangements (PBRSA): Is it a Solution to Increase Bang for the Buck for Pharmaceutical Reimbursement Strategy for Our Nation and Around the World? Clinical Drug Investigation. ISSN 11791918. 40:12 (2020) 1107–1113. doi: 10.1007/s40261-020-00972-w.