studies; (d) analysing data and (e) presenting the results. A comprehensive English-language literature search of the electronic databases PubMed and Science Direct was undertaken to identify published papers. Data were collected and analysed until May 2021.

Results In this review, we incorporated one retrospective and one prospective cohort study. In the ongoing prospective Long-Term Follow-Up (LTFU) study (13 patients), 100% of SMA I infants in the therapeutic-dose cohort were alive and free of permanent ventilation. It was reported that 20% of SMA I infants achieved the additional milestone of standing with assistance. The LTFU study has demonstrated that SMA I infants improved their Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) scores (≥4 points). In the retrospective cohort study of SMA I (3 patients) and SMA II infants (4 patients), it was perceived that 43% of SMA patients had meaningful increases in the CHOP-INTEND score and 57% had increases in the Hammersmith Functional Motor Scale-Expanded (HFMSE) score.

Conclusion and relevance Despite the limited observation period, we conclude that Zolgensma is effective since no clinical regression or waning of effect had been reported. Nonetheless, several factors might still influence the duration of Zolgensma's effectiveness. As such, further research is needed to evaluate the persistence of the Zolgensma real-world effect in SMA infants.

REFERENCES AND/OR ACKNOWLEDGEMENTS

Conflict of interest No conflict of interest

6ER-008 DEVELOPMENT OF A RISK-SHARING MODEL BASED ON THE CLINICAL PERFORMANCE OF ONASEMNOGENE ABEPARVOVEC (ZOLGENSMA)

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Background and 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 for the pharmaceutical industry, regulators and payers. The so-called Performance-Based Risk-Sharing Arrangements-Performance Linked Reimbursement (PBRSAs-PLR) are financial models that have been developed for reducing uncertainty through greater investment in evidence collection, while a technology is used within a healthcare system.

Aim and objectives The scope of this investigation comprised the development of a hypothetical PBRSA-PLR for Zolgensma Gene Replacement Therapy (GRT).

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

Results We propose an outcome-based scheme based on Zolgensma performance in terms of sustainability of the clinical effect. The relevant outcomes should be the subsequent for a given SMA infant: (a) overall survival and (b) event-free survival. We further suggest an annuity-based payment scheme to reduce the consequences of the annual budget impact with a pay-over-time of 5 to 15 years to increase patient access. More favourable outcomes could be achieved if SMA infants started treatment earlier. Thus, we propose a maximum 50% refund for Zolgensma early dosing in SMA infants (until 3 months old), and a maximum 25% refund for Zolgensma late dosing in SMA patients (after 3 months until 9 months old), if Zolgensma fails to meet the agreed-upon outcomes and predefined timing of outcome assessments.

Conclusion and relevance We conclude that it would be possible to mitigate uncertainty around the incremental budgetary impact and cost-effectiveness of Zolgensma GRT. Nonetheless, it should be outlined that innovative payment schemes should only be applied in circumstances where there is scope for such mechanisms to effectively reduce decision uncertainty so that the probability of long-term cost-effectiveness can be improved.

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6ER-011 COTRIMOXAZOLE: HOW FOLATE SUPPLEMENTATION COULD AFFECT TREATMENT EFFICACY

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Background and importance Cotrimoxazole (CTX) is an association of sulfamethoxazole and trimethoprim which acts synergistically to inhibit folic acid synthesis and block bacterial growth. It is used in the treatment of bacterial infections and in the prophylaxis of opportunistic diseases like toxoplasmosis and infection with *Pneumocystis jirovecii* in immunosuppressed patients. CTX causes myelotoxicity since it affects the same process in human cells. To prevent toxicity, folic or folinic acid can be administered. However, there is controversy as to whether this folate supplementation could affect the efficacy of cotrimoxazole.

Aim and objectives To determine if the co-administration of CTX and folates compromises efficacy of the treatment.

Material and methods A review of the published evidence on CTX and folate supplementation was conducted. An initial search was performed in PubMed and Google Scholar using the terms 'cotrimoxazole' and 'folates' and 'efficacy' supported by federal data sheets.

Results Regarding the use of folates as a supplement in bacterial infections, there is no evidence at all. Theoretically, as these bacteria intrinsically lack mechanisms to capture exogenous folates, it seems to be more appropriate to use folic acid before folinic acid due to its lipophilicity avoiding a possible passage of this molecule through the bacterial wall, which is lipophilic in nature. *P. jirovecii* is permeable to lipophilic folates and lacks an active transport mechanism to incorporate classical folates. Therefore, the administration of folinic acid, which is more lipophilic, could reduce the antifolate activity of CTX meanwhile folic acid supplements do