

## Abstract

### **Societal and governmental perspective of patients with Dravet syndrome and Lennox-Gastaut syndrome**

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**Introduction and aims:** Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS) are rare diseases. The orphan drug Epidyolex is indicated to reduce the frequency of seizures in these diseases. The aim of this work is to provide a comprehensive overview of orphan drug legislation and the potential of its reimbursement from health insurance and determine the costs of DS and LGS from a societal and governmental perspective.

**Methods:** The data for the analysis is obtained from the questionnaire focused on the social benefit drawdown, increase in household costs, and loss of productivity of patients and their caregivers. Respondents are recruited in a cooperation with patient organisations and specialised centres. The costs from both perspectives are determined by adding the expenses of the health insurance company and the costs of lost productivity (societal perspective), or the costs to the social security system (governmental perspective). The human capital approach method (HCA) is used for the societal perspective.

**Results:** The costs from a societal perspective consisting of patient and caregiver costs are determined at 1,4 to 2,3 mil. CZK/year for DS and 1,6 to 4,2 mil. CZK/year for LGS depending on the severity of the disease. The share of the indirect costs from a societal perspective is 65 % (DS) or 76 % (LGS). The costs from a governmental perspective are determined at 0,3 to 1,5 mil. CZK/year for DS and 0,5 to 1,3 mil. CZK/year for LGS depending on the severity of the disease.

**Conclusions:** Loss of productivity or drawn benefits form a non-negligible part of the costs of illness, especially rare ones. This source of information should be a full-fledged part of the decision-making practice on reimbursements.

**Key words:** rare diseases, epilepsy, societal perspective, governmental perspective