

Economic Burden of Spinal Muscular Atrophy in Italy

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Abstract

OBJECTIVES : Spinal muscular atrophy(SMA) is a rare and debilitating autosomal recessive neuromuscular disorder characterized by severe muscular atrophy and weakness. The objective of the analysis is to estimate the indirect and direct non-healthcare costs associated with SMA for untreated and treated patients with nusinersen, first disease-modifying treatment reimbursed in Italy since September 2017.

METHODS : A multidisciplinary group follows the same methodological approach used in a previous project (Marcellusi et al. 2019) in order to develop the economic model. While the previous analysis referred to the economic burden of SMA in the pre-nusinersen era, this analysis refers to the pre and post nusinersen era. This analysis divides patients into two groups:a) treated with nusinersen or b)not treated or waiting for treatment. A questionnaire was computerized and sent for compilation through all the distribution channels of the association Famiglie SMA. Social channels and specific mailing-lists were considered to be closed only to SMA families. The data collection began on March, 2019 and will be closed on July, 2019. Preliminary data were analyzed within the economic model in order to estimate the average costs per patient. Results were stratified per SMA type and treated/untreated patients.

RESULTS : Preliminary data investigated 50 families that compiled the questionnaire (22.88% SMAI,48.31% SMAII,28.81% SMAIII). Preliminary results stratified per SMA type estimated an average annual cost per SMA patient comparable to last year's analysis (around €23.000). Of these costs about 53% were attributable to house modification and car adaptation, 25% in terms of indirect costs associated with caregivers, 11% for direct non-health costs directly incurred by the patient and 11% for indirect costs on patients.

CONCLUSIONS : SMA accounts for substantial costs to families of children and patients with SMA, which are driven by increased utilization of direct non-health care resources, including house modification, car adaptation and increased indirect costs associated with caregivers.