Edaravone: one year experience of the Italian MND Study Group

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Background

An urgent unmet need persists for effective therapies significantly improving disease progression, or survival and/or quality of life in Amyotrophic lateral sclerosis (ALS). Riluzole, the first drug approved for the disease, prolongs survival by a mean of 3 months. Edaravone (EVN) is an antioxidant drug targeting peroxyl radicals originally developed for treatment of stroke, that in a double-blind trial (MCI-186-J19) performed in a subgroup of ALS patients (pts) with specific clinical findings showed a statistically significant slowing of disease progression over the 24-week treatment period. Based on these results, the drug was approved for the treatment of pts with ALS in Japan and subsequently in United States. In July 2017, the Italian Medicines Agency (AIFA) enclosed the drug in the list of the Law 648/96, allowing the treatment of the Italian ALS pts with the specific clinical findings of the MCI-186-J19 trial.

Objectives

To collect data in a "real world" context about the safety, the adherence to the therapy and its effect on the disease progression and respiratory function in patients treated with EVN in Italy; to compare the disease progression of the Italian patients treated with EVN and matched patients obtained from the PROACT Database.

Patients and methods

We performed a retrospective observational study based on the collection of the data obtained from patients treated with EVN since July 2017 to September 2018 in 29 Italian ALS Centers according the Law 648/96. Taking into account patients with at least 6 months of treatment we compare the change of ALSFRS-Rand FVC% scores with a group of patients matched for the clinical findings obtained from the PROACT database

Results

The EVN cohort included 243 patients (147 men and 96 women) with a median age of 60 years. The median treatment period was 6.4 months (range 0.1 – 11.8) and a median number of cycles of 3 (range 1 – 11). In only 18% (N=44) of patients the treatment was interrupted after a median period of 3.2 months (range 0.4 – 7.8) for different reasons, including side effects and the feeling of not efficacy in the face of the difficulties in carrying out the therapy. The PROACT cohort included 1742 patients (1095 men and 637 women) with a median age of 55 years. No significant differences of the change of ALSFRS-R and FVC% scores were found in the three times of observation (T0, T3 and T6).

Discussion

These preliminary data obtained in a "real world" context confirm a good profile of safety and high adherence to the therapy although the burden related to the intravenous administration in hospital. No apparently effects on the disease progression and respiratory function in the first six months of observation in patients treated with EVN. The study is ongoing to continue the follow-up of the patients and obtain data useful to increase the knowledge about the safety of the EVN in long-term use and the effect of the drug on the disease progression and survival and its implication for quality of life in ALS patients.