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RESOLUTION

of Teleconference «Amyotrophic Lateral Sclerosis. Every move matters»

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Almost 3 000 health care specialists have registered to participate in the Teleconference «Amyotrophic Lateral Sclerosis. Every move matters».

The Teleconference agenda consisted of two topical units of reports: «Sharing clinical experience. Focus on a patient with ALS», «Right to life of orphan patients».

Six main reports were offered to the participants for review and discussion and they were dealing with the following issues:

- Amyotrophic Lateral Sclerosis. Tender points: testing, pathogenic treatment, patients' rights.
- False negative testing of Amyotrophic Lateral Sclerosis.
- Opportunities of genetic testing of motor neuron disease.
- Family forms of Amyotrophic Lateral Sclerosis.
- Legal aspects of obtaining medical care by the citizens suffering with rare (orphan) diseases.

Conclusions and decisions based on discussion of reports:

1. Amyotrophic Lateral Sclerosis (ALS) is a progressive neurodegenerative disease primarily affecting motor neurons of a brain and spinal cord. It is clinically manifested with progressive atonia of limbs, bulbar and breathing muscles. Every year 2-6 new cases of this disease per 100 thousand inhabitants are diagnosed in Europe. At the same time in Ukraine this rate is equal to 0,5-1 case per 100 thousand inhabitants.

2. Reasons for underestimate of ALS incidence are: incorrect diagnostics, incomplete family history, heterogeneity of disease, incomplete penetrance, and multigenic inheritance.

3. Well-timed diagnostics, involvement of a patient and his/her family and positive treatment plan are necessary conditions for a proper clinical management of patients with motor neuron disorders. Though ALS is an incurable malady, use of drugs with proven efficiency may slow down disease progression and disablement rate. And multiple symptoms respond to an adjunct therapy that may improve the course of a disease.

4. There is no final testing for ALS. Diagnose is usually confirmed with combination of suggestive clinical features with negative laboratory tests and imaging of other pathologies. Disease progression is a necessary condition.

5. Detection of potential biomarkers may be a disease diagnostic pattern. Neurofilament is one of prospective biomarkers of Amyotrophic Lateral Sclerosis. Available data prove that the level of neurofilament system indicators of asymptomatic carriers of SOD-1 gene mutation increases in a year before the first symptoms appear. Their levels are truly higher in patients with ALS and they correlate with the number of body areas that were affected with ALS and ALS progression speed, reflecting the level and speed of motor neurons degeneration.

6. Edaravone, as an acceptor of free radicals, demonstrated an ability to slow down physical functions deprivation by 33% within 6 months in comparison to the placebo according to the Revised ALS Functional Rating Scale (ALSFRS-R). U.S. Food and Drug Administration (FDA) has approved use of edaravone for treatment of patients with ALS. The drug is registered for treatment of ALS in Japan, USA, Canada, Italy and South Korea. Efficiency of edaravone for treatment of ALS is demonstrated in multiple researches, in particular in randomized controlled clinical researches.

7. Potential ability of edaravone to decrease levels of neurofilament light chains gives reasons to recommend edaravone for chronic usage by patients with ALS according to the product directions. And it is recommended to use measuring of neurofilament level as a marker of neurodegenerative process activity and amount of control over it.

8. The received results of retrospective analyses demonstrate possible long-term result of treatment of Amyotrophic Lateral Sclerosis with edaravone and maintaining the efficiency of such treatment within a year.

9. Amyotrophic Lateral Sclerosis is approved in the List of Rare (Orphan) Diseases in Ukraine that result in reduction of life duration or disablement of patients and that have recognized treatment methods (code Orpha.net 803, ICD-10 G12.2, Order of the MoH of Ukraine dated 27.10.2014 № 778 – section I V, clause 6).

10. According to the Law of Ukraine «Fundamentals of the Ukrainian Legislation on Health Care» dated 19.11.1992 № 2801-XII, as amended with the Law of Ukraine »On Amendment to the Fundamentals of the Ukrainian Legislation on Health Care with Regard to Prevention and Treatment of Rare (Orphan) Diseases» dated 15.04.2014 № 1213-VII and Resolution of the Cabinet of Ministers of Ukraine «On Approving the Procedure for Providing Citizens Suffering from Rare (Orphenic) Disease, Medicinal Products and Related Food Products for Special Dietary Consumption» dated 31.03.2015 № 160 the patients with Amyotrophic Lateral Sclerosis have to be provided with necessary medicines.

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