VSN

Vereniging Spierziekten Nederland

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European Commission
Health and Consumer Protection Directorate-General
Rare Diseases consultation
HTC 01/198
11, Rue Eugène Ruppert
L-2557 Luxembourg

Dear Madam/Sir,

The Dutch Association for Neuromuscular Diseases (Vereniging Spierziekten Nederland, VSN) would like to contribute to the public consultation on rare diseases. Our association has over 10,000 members with a multitude of different, rare, neuromuscular disorders. Speeding up the development of therapies has been one of the missions of our organisation for many years.

Although Europe has introduced some specific measures for the benefit of patients with rare diseases, we are particularly concerned about two points which have remained unaddressed so far, in contrast to the United States. First, there should be specific funds earmarked for supporting research on early development (before market approval) of orphan medicinal products or devices. Second, there should be funds explicitly available for studying safety and efficacy of existing medicines and devices, for their expansion to novel orphan indications. The following text from the FDA Orphan Grant Program illustrates both points (**http://www.fda.gov/orphan/grants/faq.htm**):

"What Studies qualify? Only clinical studies qualify for consideration. Each application should propose one discrete clinical study designated to facilitate FDA approval of the product for use in a rare disease or condition. *The study may address an unapproved new product or an unapproved new use for a a product already on the market.*" [Our italics.]

We believe such a measure could make a huge difference to patients with rare diseases. In the field of neuromuscular diseases we have been confronted with many examples of how hard it is to go the whole route necessary for evidenced-based extention of "on-label", prevalent indications to promising orphan indications. To give some examples, the following medicines have been shown to be (potentially) beneficial orphan diseases, but high-quality clinical trials are still lacking: sodium butyrate (spinal muscular atrophy), (biological) various immunosuppressive drugs (myositis and polyneuropathy related to monoclonal gammopathy of unknown significance), modafinil (against symptoms of fatigue in various neuromuscular diseases), and iron chelators (Friedreich's ataxia).

../2

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The points we raise relate to several specific areas in the consultation document:

- strengthening the cooperation between European Union programs, especially those on Orphan Drugs (p.8). Indeed, our proposal implies coordinating research- and economic policy on a European level

- Question 8: expanding the orphan drug model
  Our proposal should indeed include medical devices

We believe that this consultation offers an important opportunity for positive change in the long term. Thus we hope that you will give our comments your consideration.

Yours sincerely,

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