

What the future holds for treatment - the medical and rehabilitation challenges as seen from the perspective of the clinician

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"What new promising treatments are around the corner", we are often asked and ask ourselves.

This question may be viewed from different perspectives. We will look at them from two of these: a time aspect and a pathophysiological aspect.

Looking at future treatments from a time perspective there are three major levels, diffusely denoted present time, near future, and distant future. Although dealing with the future we will have to start at the present time. Do we now treat according to the best available knowledge? How do we know if we do so? The task to establish and follow an already present golden standard of treatment is here a first important step. In the near future we have treatments in or close to later phase clinical trials that are likely to result in additions in the therapeutic arsenal. In a more distant future we foresee novel therapies that are now at a state of basic research or early clinical trials, and also not yet defined strategies. Among these are means of directly compensating for the genetic alteration that often underlie neuromuscular disorders.

If we instead look at future treatment options from a pathophysiological aspect, we can see areas of treatment and care arising that are directed at 1) counteracting the primary pathogenetic mechanism underlying the disorder (e.g. corticosteroid treatment of DMD, medical attempts to increase SMN2 levels in SMA, and gene therapies), or 2) symptomatic for an affected tissue or organ (e.g. ACE-inhibitor treatment of approaching cardiac insufficiency, scoliosis surgery, ventilatory support etc).

Yet another level we need see developed in the future is a global habilitation/rehabilitation view for measures that are supportive for well-being and life in society, including measures to increase independence, ease communication, and participation in society, e.g. in the educational system and at work.

No matter what perspective we look at future measures, a key feature is that large efforts are needed at a local plane, but more importantly, at an overall multinational level. Therefore, the best measure to allow for this improvement in future therapies at various levels is the creation of large international networks to implement the best current knowledge, to increase awareness in society, and to collaborate for finding novel therapeutic strategies, and to allow for continually ongoing, good clinical trials to improve present therapies and test new ones.