

Capturing the Patient's Voice

Citation for published version (APA):

Draak, T. H. P. (2019). *Capturing the Patient's Voice: Peripheral Neuropathy outcome measures Standardisation (PeriNomS) study part 3*. ProefschriftMaken Maastricht. <https://doi.org/10.26481/dis.20190621td>

Document status and date:

Published: 01/01/2019

DOI:

[10.26481/dis.20190621td](https://doi.org/10.26481/dis.20190621td)

Document Version:

Publisher's PDF, also known as Version of record

Please check the document version of this publication:

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- The final published version features the final layout of the paper including the volume, issue and page numbers.

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VALORISATION

Valorisation is the translation of scientific knowledge to social, economic and financial value and its relevance to society.

As part of the PeriNomS study, this thesis originated from an unmet need for proper outcome measures in inflammatory neuropathies.

Inflammatory neuropathies are relative rare but severe disorders which can lead to severe impairments in daily functioning and reduced quality of life. They require expensive treatments and long-term follow-up by medical professionals, and patients are limited in fulfilling socio-economical positions in society. The disease burden for the individual patient and their care-givers is high. For inflammatory neuropathies no cure is available, only treatments that slow progression, help maintain or partially recover lost functionality.

The development of new drugs through clinical trials in these disorders is expensive and challenging due to their rarity. This is further hampered by a wide range of outcome measures used in these trials, many of which lack proper clinimetric standards. This leads to less comparability between trials, as well as risking false study results. A treatment could be falsely deemed successful or ineffective if incorrect outcome measures are used.

By improving or creating proper outcome measures to be used in clinical trials in these disorders, we can prevent misinterpretation of these trials, protecting patients from unnecessary or ineffective treatments and prevent withholding effective treatments. This will lead to better patient care, resulting in a better socio-economical position for these patients as well as reducing their dependence on medical care. By protecting patients from ineffective treatments we reduce their health care cost, also by protecting them from potential side-effects which can burden both the patient and the health care system.

Furthermore, by establishing core sets of outcome measures to be used, we ensure comparability between studies. Due to the rarity of these disorders, the ability to compare studies results and perform meta-analyses is of major importance to help draw strong and evidence based recommendations from these trials. This will result in fewer trials needed to provide quality evidence regarding potential new treatments, which will allow for research funds to be invested into other research subjects or perform follow-up studies more easily.

Also, to be able to avoid the use of redundant or incorrect outcome measures will help decrease the burden for study participants and can lead to better patient participation in clinical trials, as the nature of these disorders requires long-term follow-up studies. This too will result in better quality evidence for new treatments, which will ultimately result in better patient care with less health care costs involved.

This thesis has resulted in further improving the much-needed outcome measures in inflammatory neuropathies as well as developing a new quality of life scale for these patients. Furthermore we have established a sound basis on how to define a responder as well as give investigators recommendations on which outcome measures they should use in inflammatory neuropathies.

These results are all part of a major international endeavour, and our results have been announced in organized international meetings and congresses as well as many publications in high-quality medical journals. These have led to changes in guidelines for international trials in inflammatory neuropathies as well as on how to evaluate our patients in daily clinical practice.