Patient Centered Outcomes in Research

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Research for Lyme Infection-Associated Chronic Illnesses Treatment: Broadening the Lens.
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Patient-centered outcomes are outcomes important to patients

- Patient centered outcomes are outcomes that are important to patients.
- In Lyme disease, these include restoration of health, prevention of health deterioration, ability to engage in work and other activities, and improvement in quality of life
- ISPOR: A treatment benefit is a favorable effect on a meaningful aspect of how a patient feels or functions . . . **If the effect is not meaningful to the patient, it is not a benefit to the patient. . .**

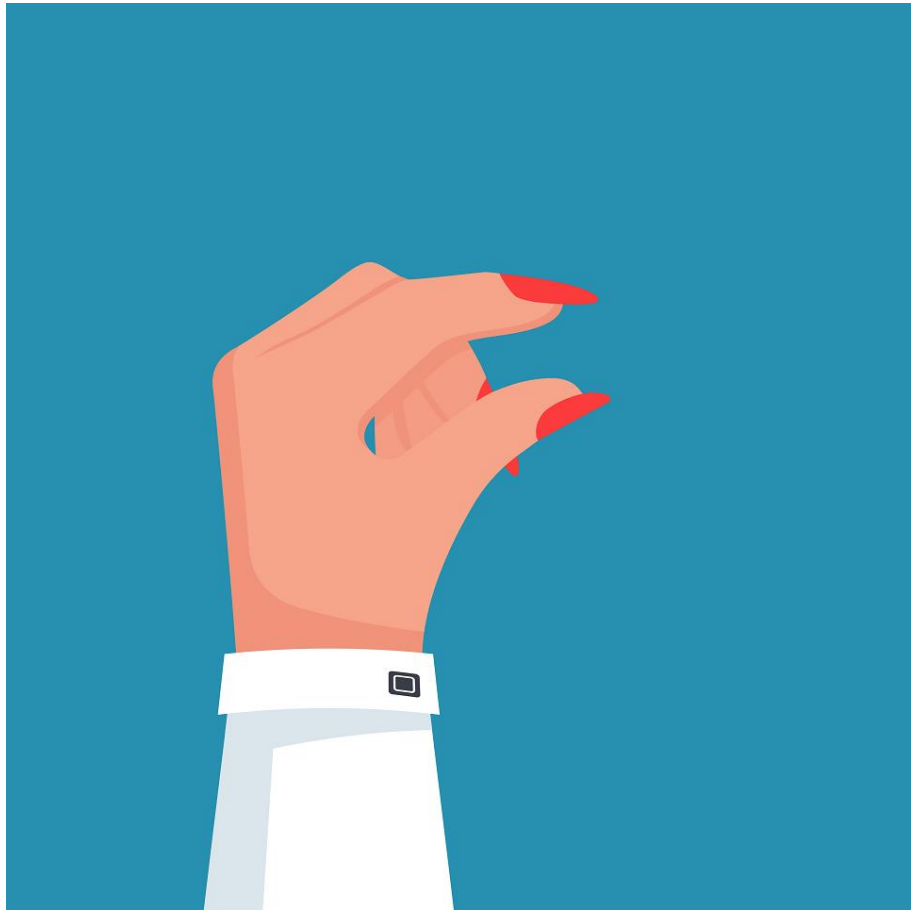
(Walton 2015, Johnson 2018, Luft 1996, Luger 1995, Nadelman 1992, Aucott 2022)

PROs are the most common way of identifying patient centered outcomes

- Patient Reported Outcomes (PROs): The FDA and National Quality Forum define a PRO as a "**report that comes directly from the patient about the status of a patient's health condition *without amendment or interpretation of the patient's response by a clinician or anyone else*."**
- Information gathered from PROs is **often information that is only known by the patient such as** symptom severity, quality of life, functional status, improvement in symptoms, treatment adherence.
- **No one else can reliably report such things for another person.** Physicians have usually been found to be poor reporters of patients' symptoms or experiences.
- To be patient-centered, PROs **should be *not only statistically significant, but also important to clinicians and patients*.**

(Snyder 2013, Deshpande 2011)

The smallest amount an outcome must change to be meaningful to a patient is called the “minimal clinically important difference” (MCID)?

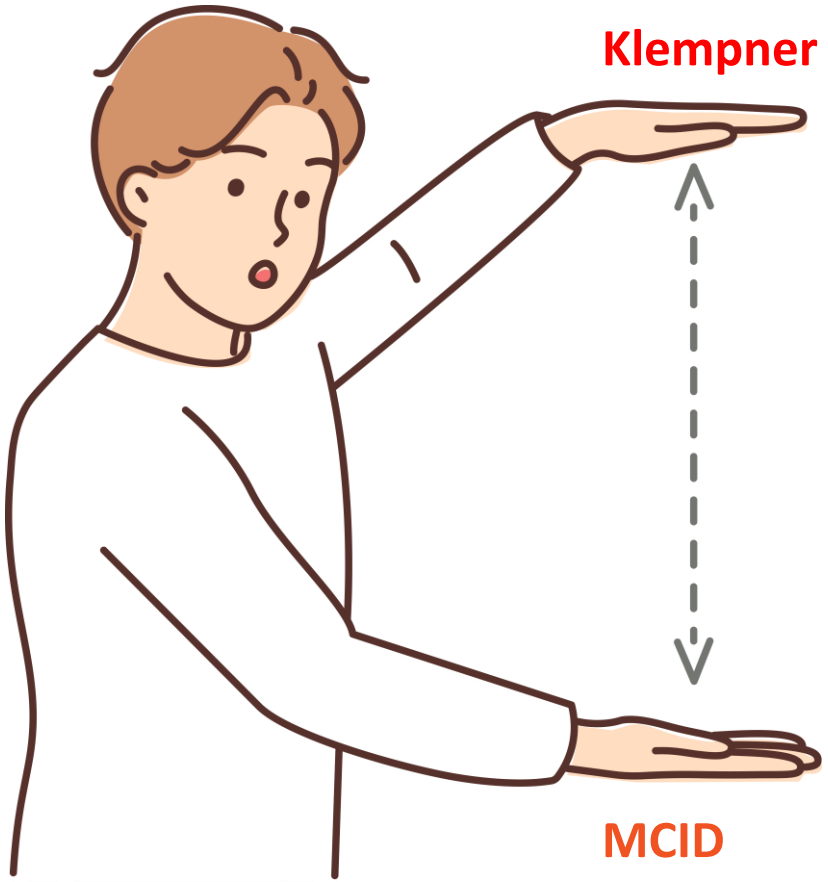


- Using patient-centered MCIDs is important for studies involving patient-reported outcomes,[2] **for which the clinical importance of a given change may not be obvious to clinicians selecting treatments.**

- The MCID. . . can be defined as "*the **smallest difference in score in the domain of interest which patients perceive as beneficial** and which would mandate, in the absence of troublesome side effects and excessive cost, a change in the patient's management*".

(McGlothlin 2014, Johnson 2014, Zhang 2023 Walters 2003)

Klempner set the bar for treatment success too high



- The researchers set the bar for improvement at least 2 times higher than the MCID for similar chronic conditions
- Because of this, improvements that patients would have regarded as meaningful were not identified as treatment successes
- To find the antibiotic treatment effective, the researchers would have needed to use the appropriate MCID and enrolled a much larger sample

(Delong 2012)

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