

The use of patient-reported outcome measures (PROMs) in rare diseases and implications for health technology assessment (HTA)

The Patient

Amanda Whittal^{1*}, Michela Meregaglia¹, Elena Nicod¹

¹ Research Centre on Health and Social Care Management (CERGAS)
SDA Bocconi School of Management
Via Sarfatti 10, 20136 Milan (Italy)

*Corresponding author: Amanda Whittal - amanda.whittal@unibocconi.it

Appendix 1. Search strings

PubMed search strings:

patient reported outcome measure*[Title/Abstract]) OR prom[Title/Abstract]) OR patient reported outcome*[Title/Abstract]) AND rare disease*[Title/Abstract]);

patient reported outcome measure*[Title/Abstract]) OR prom[Title/Abstract]) OR patient reported outcome*[Title/Abstract]) AND RDT*[Title/Abstract];

patient reported outcome measure*[Title/Abstract]) OR prom[Title/Abstract]) OR patient reported outcome*[Title/Abstract]) AND orphan medicinal product [Title/Abstract];

patient reported outcome measure*[Title/Abstract]) OR prom[Title/Abstract]) OR patient reported outcome*[Title/Abstract]) AND OMP [Title/Abstract];

patient reported outcome measure*[Title/Abstract]) OR prom[Title/Abstract]) OR patient reported outcome*[Title/Abstract]) AND rare disease*[Title/Abstract]) AND challenge*[Title/Abstract]);

patient reported outcome measure*[Title/Abstract]) OR prom[Title/Abstract]) OR patient reported outcome*[Title/Abstract]) AND rare disease*[Title/Abstract]) AND recommend*[Title/Abstract]);

health technology assessment[Title/Abstract]) AND (appraisal*[Title/Abstract])) AND (rare disease*[Title/Abstract]);

health technology assessment[Title/Abstract]) AND (appraisal*[Title/Abstract])) AND (RDT*[Title/Abstract]);

health technology assessment[Title/Abstract]) AND (appraisal*[Title/Abstract])) AND (orphan medicinal product*[Title/Abstract]);

health technology assessment[Title/Abstract]) AND (appraisal*[Title/Abstract])) AND (OMP[Title/Abstract])

Grey literature searches:

using patient reported outcome measures in HTA for rare disease;

using patient reported outcome measures in health technology assessment for rare disease