Patient-Reported Outcome Measure for Meningiomas study

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Primary objectiveThe primary objective of this study is to develop and validate a disease-specific PROM, which measures HRQoL in meningioma patients.

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Nervous system neoplasms benign
Study type	Observational non invasive

Summary

ID

NL-OMON43114

Source
ToetsingOnline

Brief title
PROMM

Condition

- Nervous system neoplasms benign

Synonym
benign primary brain tumor, Meningioma

Research involving
Human

Sponsors and support

Primary sponsor: Leids Universitair Medisch Centrum
Source(s) of monetary or material Support: geen

Intervention

Keyword: Health-related quality of life, Meningioma, PROM, Questionnaire

Outcome measures

Primary outcome

Part A: Development of a meningioma specific PROM, measuring HRQoL

Phase 1, generation of relevant HRQoL issues

- An exhaustive list of HRQoL issues.

Phase 2, construction of an item list

- An item list with questions with the same format and time frame as the EORTC questionnaires.

Phase 3, pre-testing the provisional questionnaire

- Scores on the items of the provisional questionnaire.
- Results of preliminary psychometric testing (Factor Analysis, Internal consistency and descriptive statistics).
- A preliminary questionnaire.

Part B: Validation of a meningioma specific PROM, measuring HRQoL

Phase 4, large scale field testing

- Scores of *300 patients of the preliminary questionnaire and normative data of the questionnaire based on these data.
- Validity, reliability, responsiveness and interpretability.
- Scores on the following questionnaires: EORTC QLQ-C30 and QLQ-BN20 and the

SF-36.

Secondary outcome

N/A

Study description

Background summary

Meningiomas are the most prevalent tumors of the central nervous system with an incidence of 7.86 per 100.000 persons. As these tumors originate from the arachnoid cap cells, frequent sites of development are the skull convexity/falx cerebri (56%) and skull base (40%) and a less frequent site is the spinal canal (< 1%). Based on the location of the tumor, patients may suffer from a wide variety of signs and symptoms in the physical (e.g. hemiparesis), psychological (e.g. depression and anxiety) and social domain (e.g. lack of initiative). In addition patients may suffer from the side effects or complications of anti-tumor treatment.

These signs and symptoms may result in dysfunction of patients on three distinct levels, as described by the World Health Organisation International Classification of functioning, Disability and Health (ICF, 2001) criteria: impairment (e.g. visual problems), activity limitations (e.g. not able to drive) and participation restrictions (e.g. not able to drive to work, family and friends). Health-related Quality of Life (HRQoL) is a multidimensional outcome measure, including domains on physical, psychological and social functioning as well as symptoms induced by the disease and its treatment. HRQoL is therefore an unique outcome measure, assessing functioning on all three levels.

HRQoL can be physician-, proxy- or patient-reported, but is usually assessed with patient-reported outcome measures (PROM), reflecting the patient*s perspective. Indeed, patients are thought to be the best source to rate their own health status. HRQoL can be measured using generic (e.g. SF-36, EQ-5D, FACT-G, EORTC QLQ-C30, MDASI) or disease-specific questionnaires (e.g. FACT-BR, EORTC QLQ-BN20, MDASI-BT) and can be used both in clinical trials and practice. In clinical trials it can be used as primary or secondary outcome measure, which in combination with survival rates can be used to measure the net clinical benefit of different treatment modalities. Treatment may benefit or harm both the quantity and quality of survival and when these outcomes are conflicting a trade-off discussion will arise. In clinical practice on the other hand, HRQoL can be used as a facilitating tool for doctor-patient communication, for monitoring patients* problems and functioning during the

disease trajectory, and as quality indicator of healthcare.

A recent systematic review showed that existing HRQoL questionnaires used in meningioma research are not developed for and/or validated in meningioma patients. This study also showed that in contrast to the current impression of patients and physicians, data are still insufficient and not conclusive on the effect of interventions on HRQoL in meningioma patients. In addition, results of a recently conducted pilot study suggest that existing HRQoL questionnaires used in meningioma research cover issues that are not relevant for meningioma patients and that these questionnaires miss relevant issues for this patient group. Therefore, the development and validation of a meningioma specific questionnaire is warranted.

Because of the great variety in symptoms and disease course of meningioma patients, it is difficult for physicians to systematically discuss patients* problems, and consequently their needs, during the short visits to the outpatient clinic. More than 60% of meningioma patients (n=50) reported in a survey study by *Integraal Kankercentrum Nederland* (Netherlands Comprehensive Cancer Organisation) to have experienced issues or obstacles with the patient-doctor communication (e.g. patients feel themselves unheard, no attention for psychosocial problems and poor involvement of patients in the decision making process around their treatment). It is known that routinely use of PROMs in clinical practice can facilitate doctor-patient communication and probably solve some of the obstacles in the communication.

Study objective

Primary objective
The primary objective of this study is to develop and validate a disease-specific PROM, which measures HRQoL in meningioma patients.

Study design

STUDY DESIGN
This study design consists of 2 parts.
- Part A: Development of a meningioma-specific PROM, measuring HRQoL
- Part B: Validation of a meningioma-specific PROM, measuring HRQoL

For the development of the meningioma-specific PROM, measuring HRQoL, we will follow the guidelines of the European Organisation for Research and Treatment of Cancer (EORTC) for the development of questionnaires. The EORTC guideline consists of four phases: (1) generation of relevant HRQoL issues, (2) construction of an item list, (3) pre-testing the provisional questionnaire and (4) large-scale field-testing (see figure 1). In all four phases a heterogeneous group of meningioma patients will be included, based on demographic characteristics, tumor location and moment in the disease course

(preoperative and short- and long-term postoperative). By doing so, we will ensure that the questionnaire is relevant for all meningioma patients during the entire disease course. To ensure cross-cultural validation, patients will be included from hospitals in the Netherlands, Italy and the United States.

Part A: development of a meningioma specific PROM, measuring HRQoL
Part A comprises the first three phases: (1) generation of relevant HRQoL issues, (2) construction of an item list, (3) pre-testing the provisional questionnaire.

Phase 1, generation of relevant HRQoL issues
In phase I, we will generate an exhaustive list of HRQoL issues by conducting a systematic literature search, semi-structured interviews with patients (n=40) and health care professionals (HCPs, n=18).
1. A literature search will be conducted to find relevant (HRQoL) questionnaires for this patient group. The following electronical databases will be searched: MEDLINE, Embase, Web of Science, CINAHL, PSYCHINFO, Academic Search Premier, Cochrane and ScienceDirect. Search terms will be used for meningioma, questionnaires and HRQoL.
2. The domains and issues covered by the found questionnaires will be extracted by two researchers and will be used in the semi-structured interviews with patients and HCPs.
3. The aim of these semi-structured interviews is threefold: (1) to identify relevant quality of life issues (the interview will continue until no new issues arise), (2) to determine the relevance of all issues identified in the literature search, including those in the existing HRQoL questionnaires, and (3) to determine the 5 most important issues.
4. Based on the literature search and semi-structured interviews with patients and HCPs, we will generate an issue-list. All issues will be first translated into English.

Phase 2, construction of an item list
1. In phase II, the list of HRQoL issues will be converted into questions in English with the same format and time frame as the EORTC questionnaires.
a. Questions will refer to patient*s experience of the last week or last month, based on the average frequency of the issue.
b. Responses will be recorded on a 4 point Likert scale (1 not at all - 4 very much).
c. Questions related to functioning will be positively formulated and questions related to symptoms and side effects will be negatively formulated.
2. The generated questions will be mailed to all the principal investigators to come into consensus on the formulation of the questions.
3. The questions will be translated into Dutch and Italian through a forward-backward translation, following the EORTC translation guidelines.
4. Cognitive debriefing interviews will be organised with 10 meningioma patients to assess the construct validity of the provisional questionnaire and whether the questions are clearly formulated.

Preliminary decision rules for phase 1 and 2
All issues identified in phase 1 will be included to the preliminary questionnaire, with exception of:
- Issues with a mean relevance score less than 1.5 (4 point Likert scale).
- Issues which are fairly similar to each other; the issue with the highest mean relevant score will be included to the preliminary questionnaire.
- Issues that are not applicable in all three countries.

Phase 3, pre-testing the provisional questionnaire
The aim of pre-testing the provisional questionnaire is to identify and solve potential problems in its administration (e.g. the phrasing of questions, the sequence of questions) and to identify missing or redundant questions. In phase 3, 120 meningioma patients (see, paragraph 5.4 for sample size calculation) will be asked to complete the questionnaire and to score the relevance of each issue and to assess whether the questions are clearly formulated. In addition, preliminary psychometric testing will be conducted. This will result in a preliminary questionnaire, which will be used in phase 4.
1. Patients complete the provisional questionnaire before the interview.
2. Patients will score the relevance of each question on a 4 point Likert scale ranging from *not at all* to *very much*.
3. Semi-structured interviews are organised aiming to assess completeness and acceptability of the questions. Based on these comments, the provisional questionnaire will be adapted.
4. Preliminary psychometric testing will be conducted.
Preliminary decision rules for phase 3
Questions are retained in the final questionnaire if they meet 5 of the following 7 criteria:
- Questions with a mean relevance score > 1.5 points
- Questions with a prevalence ratio > 30% (number of subjects assessing a question as relevant divided by the total number that completed the question)
- Questions with a range > 2 points
- Questions with minimal floor and/or ceiling effects
- Issues that are not applicable in all three countries.
- Issues that are upsetting and potentially distressing.
- Issues with a high compliance of at least 95%
New issues are included when at least 30% of patients reported this issue as relevant in phase 3.
Questions will be rephrased when 2 or more patients have any difficulty with the question.

4.2 Part B: validation of a meningioma specific PROM, measuring HRQoL
Phase 4, large scale field testing
In phase 4, patients from multiple centres will complete the questionnaire at multiple time points (for each item in the questionnaire, 10 patients will be included) to assess its validity, reliability, responsiveness and

interpretability (i.e. minimal important change) in accordance to the COnsensus-based Standards for the selection of health Measurement Instruments (COSMIN) criteria, see figure 2.18
1. Patients complete the questionnaire at three time points: baseline, 12 weeks and 14 weeks (patients who complete the questionnaires before surgery should have their first interview no earlier than a week before surgery).
2. In addition, patients will complete three other questionnaires the first time to assess concurrent validity: the EORTC QLQ-C30 and its brain tumor-specific module (QLQ-BN20) and the SF-36.
3. Patients complete a debriefing questionnaire. Based on these comments, the preliminary questionnaire may be adapted.
4. Psychometric analyses will be conducted in according to the COSMIN criteria.

Study burden and risks

During the semi-structured interviews, patients may be confronted with all issues and problems that meningioma patients could have, which could impose a psychological burden on them. Moreover, it will cost the patients time to attend the interviews and complete the questionnaires, although this is not substantial (max: 60 minutes). Previous experience with the development of HRQoL questionnaires is that the respondent burden is limited and that patients like to discuss these issues/problems with a health care professional.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

In order to be eligible to participate in this study, a subject should:
1. be diagnosed clinically or histopathologically with a WHO grade 1 meningioma
2. be 18 years or older
3. give written informed consent prior to inclusion

Exclusion criteria

A potential subject who meets any of the following criteria will be excluded from participation in this study:
1. Patients with a neurofibromatosis type 2 meningioma.
2. Patients not fluent in Dutch, English or Italian (depending on the country where they live).
3. Patients with physical or mental conditions interfering with the understanding and completion of questions and questionnaires.

Study design

Design

Study type: Observational non invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Other

Recruitment

NL

Recruitment status:	Recruiting

Start date (anticipated):	06-01-2017
Enrollment:	165
Type:	Actual

Ethics review

Approved WMO Date:	23-12-2016
Application type:	First submission
Review commission:	METC Leiden-Den Haag-Delft (Leiden) metc-ldd@lumc.nl

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL58154.058.16