(Cost-)effectiveness of optical coherence tomography versus regular punch biopsy in the diagnosis and subtyping of basal cell carcinoma: a multi-center randomized non-inferiority trial

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Primary Objective: To investigate if use of OCT is (cost-)effective in the diagnostic work-up of patients with clinically and dermoscopically suspected basal cell carcinoma (BCC).Secondary Objective(s): To explore if OCT is a more patient friendly…

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Skin neoplasms malignant and unspecified
Study type	Interventional

Summary

ID

NL-OMON48208

Source
ToetsingOnline

Brief title
(Cost-)effectiveness of optical coherence tomography (OCT)

Condition

- Skin neoplasms malignant and unspecified

Synonym
Basal cell carcinoma, skin cancer

Research involving
Human

Sponsors and support

Primary sponsor: Medisch Universitair Ziekenhuis Maastricht
Source(s) of monetary or material Support: Ministerie van OC&W,ZonMw

Intervention

Keyword: (Cost-)effectiveness, Basal cell carcinoma, Diagnosis, Optical coherence tomography

Outcome measures

Primary outcome

The main endpoint for the non-inferiority trial is the proportion of patients with treatment failure after 12 months follow-up, where treatment failure is defined as inadequate treatment or recurrence of malignant or premalignant lesions. The main endpoint for the cost-effectiveness analysis is the ICER defined as extra cost per gained QALY.

Secondary outcome

Secondary outcomes are: the proportion of patients with avoided biopsies. The design of the study also enables evaluation of the ability of OCT to discriminate between BCC and non-BCC and between BCC subtypes (superficial, nodular and infiltrative BCC) using punch biopsy as reference standard. Diagnostic performance will be expressed as sensitivity, specificity, positive and negative predictive value. A receiver operating characteristic (ROC) curve with area under the curve (AUC) will also be calculated. Patient preferences will be assessed by conducting a discrete choice experiment. Quality of life at baseline and at 12 months follow-up will be evaluated using the EQ-5D-5L questionnaire.

Study description

Background summary

Currently, the gold standard for diagnosing and subtyping basal cell carcinoma (BCC) is a punch biopsy. Since this technique is invasive, new non-invasive diagnostic methods have been developed, including optical coherence tomography (OCT). In patients with clinical and dermoscopic suspicion of BCC, OCT makes it possible to confirm and subtype BCC with high confidence, thereby obviating the need for a punch biopsy in a substantial part of patients. Hence, BCC diagnosis and treatment can be accomplished in one day. As a result, patients experience less distress and costs can be saved. We hypothesize that the use of OCT is non-inferior in terms of (cost-)effectiveness compared to regular care. We expect that punch biopsy can be avoided in a substantial part of patients. By discussing diagnosis and treatment with the patient directly, care can be provided more efficiently, preventing treatment delay and saving extra hospital visits.

Study objective

Primary Objective: To investigate if use of OCT is (cost-)effective in the diagnostic work-up of patients with clinically and dermoscopically suspected basal cell carcinoma (BCC).

Secondary Objective(s):
To explore if OCT is a more patient friendly approach compared to regular care.
To evaluate diagnostic performance of OCT with respect to the ability to discriminate between BCC and non-BCC and ability to discriminate between BCC subtypes.

Study design

Multi-centre randomized controlled multi-centre non-inferiority trial.

Intervention

OCT guided diagnosis.

Study burden and risks

This study is developed to evaluate the (cost-)effectiveness of OCT in diagnosing and subtyping BCC. Patients will be randomized into two study arms: the intervention arm, where patients receive an OCT scan and the regular care arm, where patients are diagnosed and treated according to regular care. In the intervention arm, treatment will be guided by OCT diagnosis only if the OCT

scan leads to high certainty about the presence of BCC and BCC subtype. In patients where the OCT diagnosis reveals no BCC, or if there is any doubt about the presence of a BCC, or in case the subtype is not certain, the protocol states that a biopsy will still be taken. The treatment decision will be then be based on the result of the punch biopsy. We expect that in only 40% of patients randomized to the intervention arm a biopsy can be omitted. In all patients where a biopsy can be omitted, treatment is discussed and started or planned. After discussion of the treatment a *safety* punch biopsy will be taken in all patients of the intervention arm to ensure that the treatment plan can be revised if patient prognosis would be seriously compromised. The results of the safety biopsy will be evaluated by a dermatologist specialized in skin cancer diagnosis and treatmend (Nicole Kelleners Smeets; NKS), who is not involved in the evaluation of the primary and secondary outcomes. On the basis of the result of the punch biopsy and the intended treatment strategy, this dermatologist will decide whether the chosen treatment would harm the patient or not. The following possibilities exist:

There is a risk that in patients with an OCT scan highly suspected for BCC (including subtype), the lesion is no BCC. NKS will determine whether the intended treatment is appropriate. For example in case surgery is chosen, and the appropriate treatment for this lesion is also surgery, no action will be undertaken. However, if surgery is chosen for a lesion which is no skin cancer, this would harm the patient and NKS will change the planned treatment according to regular care for that diagnosis.
There is also a risk that the treatment decision is based on a high confidence in OCT diagnosis but the BCC subtype is misclassified. For all subtypes of BCC surgery is the gold standard.[2] Therefore, if surgery is chosen as treatment, NKS will not interfere. Around 40% of the BCC*s is of a superficial subtype and these are usually localised on the trunk or extremities. Although surgery is also the gold standard treatment for superficial BCC*s, treatment with non-invasive therapy is regular care because of the nicer cosmetic appearance and relatively high long term cure rates of 80%. Patients diagnosed with a superficial BCC on the OCT scan, who upon biopsy appear to have a non-superficial BCC (solid or infiltrative BCC), requiring excision, run the risk of being treated non-invasively. However, the risk for this group to be undertreated is relatively low, since recent literature shows that imiquimod, which is the first choice treatment of superficial BCC, has cure rates of 81.8% at 3-year follow-up in nodular BCCs.[13] According to regular care all BCC*s treated non-invasively will have a 3 month follow-up visit, to ensure that treatment was effective.
In summary, we conclude that the risks for included patients who consent to participate in this study are minimalized because:
1. Only a small percentage of the patients is treated with a method that does not allow for histological control, namely the patients with high suspicion for superficial BCC;
2. All patients will receive a *safety* biopsy that will prevent us from starting a totally inappropriate treatment that would harm the patient;

3. In case of treatment of a nodular or infiltrative BCC with a non-invasive treatment, this treatment might still be effective and if not, the 3 month follow-of visit will enable us to retreat the patient with surgery.

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 must meet the following criteria:
- Adult patient (>18 years)
- Clinical and dermoscopic suspicion of basal cell carcinoma (BCC)
- BCC is in the differential diagnosis and a biopsy would normally be obtained to confirm the diagnosis and subtype or exclude other skin lesions.

Exclusion criteria

A potential subject who meets any of the following criteria will be excluded from participation in this study:
- Patients with BCC in the high-risk zone of the face (ear, nose, eye region)
- Patients with a large BCC referred to our (tertiary care) head and neck tumour working group.

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Single blinded (masking used)

Primary purpose: Health services research

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	25-02-2019
Enrollment:	598
Type:	Actual

Medical products/devices used

Generic name:	Optical Coherence Tomography
Registration:	Yes - CE intended use

Ethics review

Approved WMO

Date:	19-12-2018
Application type:	First submission
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit

	Maastricht, METC azM/UM (Maastricht)
Approved WMO Date:	13-03-2019
Application type:	Amendment
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)

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	NL67571.068.18