ISRCTN16441179 https://doi.org/10.1186/ISRCTN16441179

A study protocol for interdisciplinary patient-centred care for patients with rare cancers including outpatient and inpatient settings

Submission date
01/10/2022

Recruitment status
No longer recruiting

Registration date
07/10/2022

Overall study status
Completed

Last Edited
16/10/2023

Condition category
Other

[X] Prospectively registered
[X] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

Plain English summary of protocol

Background and study aims
A tumour disease is considered rare if the incidence is less than 6/100,000 inhabitants per year. The group of rare tumour diseases includes all hematological neoplasms, bone and soft tissue tumours or tumours of the central nervous system. Endocrine and neuroendocrine tumours or bile duct carcinoma also fall into this category. In principle, rare tumour diseases can occur in any organ system. Diagnosis and treatment of rare cancers require expert knowledge. This knowledge is bundled at university competence centres, where this patient group is naturally concentrated. For instance, studies have shown that in patients with sarcoma diseases, treatment results and overall survival crucially depend on the expertise of the treatment team at the time of initial diagnosis.
Treatment and advice at expert centres also offer the opportunity to take part in clinical trials or to be offered precision oncological approaches. However, these opportunities are challenging. Especially in the context of the diverse therapy options with their advantages and disadvantages, decisions have to take patients' needs and preferences into account in order to realise quality-oriented care. In routine care, there is a lack of established patient empowerment programmes and nationwide structures to support patients in a targeted manner. Due to its history and the fragmented structure of the German healthcare system, there is a lack of uniform structures that enable the use of innovative diagnostics and therapies as well as patient-centricity across different settings. Therefore, new approaches to care are required in order to optimise the current situation in the interests of the patient.
The objective of this study is to evaluate the implementation of the multi-component programme called TARGET. This new concept of care aims to increase the needs-based, quality-oriented and person-centred care of patients with rare cancers through a cross-sectoral linkage of different health service providers and patient involvement.
We assume that TARGET in comparison to standard care will optimise the care coordination, assessed from the patient's perspective. Secondly, hints of a positive effect on patient care and their improved involvement in decision-making processes should be generated. For this purpose, we will assess clinical parameters (e.g. survival, course of therapy) as well as patient-reported outcomes.

Who can participate?
Patients with rare cancers aged 18 years old and over with a life expectancy > 3 months

What does the study involve?
In a pre-phase (control), included patients will receive standard care but will have access to the patient app that records patient-reported outcomes.

Following this, the intervention TARGET will be implemented (post-phase). The new care model TARGET includes mainly digital interventions to optimise trans-sectoral cooperation (between in- and outpatient settings) and to sustainably improve the medical care of the target population. In the office-based sector, specialists with a focus on hematology/oncology can participate. A central coordination platform will be used to establish contact with the Comprehensive Cancer Center Munich (CCC Munich) and office-based doctors to prepare for the inclusion of eligible patients.
Enrolled patients are assigned an oncology coach who accompanies the patient and acts as a link between the office-based doctors and CCC Munich. Additionally, the patient will be supported by a patient app that records patient-reported outcomes and can serve as additional orientation. A decision support tool is integrated into the patient app. Besides, the patients have access to digital psycho-oncological services.
After the diagnostics have been completed, the medical procedure will be determined across disciplines and sectors in virtual tumour boards. Medical care should then be provided as close to home as possible. Within the programme framework, a project-specific case file will be established, which will enable the collection and exchange of medical information across sectors.

What are the possible benefits and risks of participating?
Patients who receive the intervention should benefit from the optimised coordination of care regarding communication between different settings. They hopefully experience a shorter time to receive the final diagnosis and an appropriate treatment proposal.
The risks arising from participation in the study are considered to be low. Risks that may arise individually from therapeutic or diagnostic measures will be informed separately and consent will be obtained.

Where is the study run from?
Comprehensive Cancer Center Munich (CCC Munich) (Germany)

When is the study starting and how long is it expected to run for?
April 2020 to December 2025

Who is funding the study?
Federal Joint Committee (Gemeinsame Bundesausschuss) (Germany)

Who is the main contact?
Anke Steckelberg (for scientific queries) (Germany)
Anke.steckelberg@medizin.uni-halle.de

Contact information

Type(s)
Principal investigator

Contact name

Dr Karin Berger-Thürmel

Contact details
LMU Clinic of the University of Munich - Campus Großhadern
Medical Clinic and Polyclinic III
HeAlth Services Research Working Group
Outcomes Research & Health Economics
München
Germany
81377
+49 (0) 89 440057435
karin.berger@med.uni-muenchen.de

Type(s)
Scientific

Contact name
Prof Anke Steckelberg

Contact details
Martin Luther University Halle-Wittenberg
Faculty of Medicine
Institute for Health and Nursing Science
Magdeburger Strasse 8
Halle
Germany
06112
+49 (0)345 557 4106
anke.steckelberg@medizin.uni-halle.de

Type(s)
Public

Contact name
Dr Julia Lühnen

ORCID ID
https://orcid.org/0000-0001-8460-4386

Contact details
Martin Luther University Halle-Wittenberg
Faculty of Medicine
Institute for Health and Nursing Science
Magdeburger Strasse 8
Halle
Germany
06112
+49 (0)345 557-4419
julia.luehnen@medizin.uni-halle.de

Additional identifiers

Clinical Trials Information System (CTIS)
Nil known

ClinicalTrials.gov (NCT)
Nil known

Protocol serial number
Sept 2022 - Version 1

Study information

Scientific Title
Trans-sectoral, interdisciplinary patient-centred care for patients with rare cancers: protocol of a pre-post study design

Acronym
TARGET

Study objectives
We hypothesise that TARGET, a patient-centred, trans-sectoral care concept for patients with rare cancers, in comparison to an optimised standard of care will improve the care coordination, assessed from the patient's perspective. In addition, we expect to show tendencies regarding the clinical and patient-reported secondary outcomes.

From an economic point of view, we hypothesise that TARGET interventions will achieve cost parity, or cost reduction, compared to the existing standard of care procedures.

Ethics approval required
Old ethics approval format

Ethics approval(s)
1. Control (pre-phase) approved with reservations 21/09/2022, final vote pending, the ethics committee of the Faculty of Medical of Martin Luther University Halle-Wittenberg (Magdeburger Straße 12, 06112 Halle (Saale) Germany; +49 345 557 4476; ethik-kommission@uk-halle.de), ref: 2022-104
2. Intervention (post phase) submission pending, the ethics committee at the Faculty of Medicine at LMU Munich (Pettenkoferstr. 8a, 80336 München, Germany; +49 89 4400 55191; ethikkommission@med.uni-muenchen.de), ref: none available

Study design
A prospective evaluation of TARGET will be performed in a pre-post design with 16 months follow-up.
In addition, a historical comparison group from the CREDOS (Cancer Retrieval Evaluation and DOcumentation System) dataset from the Comprehensive Cancer Center Munich (CCC Munich) was defined for the collection of clinical parameters and secondary analysis.
Regarding the economic perspective, a cost-cost analysis will be performed to assess if the intervention leads to monetary savings. The intervention group will be observed prospectively, and the control group for the economic evaluation will comprise retrospective data, provided

from the involved health insurance. A matched-pairs methodology will ensure the homogeneity of these groups.
In addition, a process evaluation will be performed to identify barriers and facilitators to the implementation of the intervention, analyse mechanisms of impact and understand the interactions of the different components.

Updated 09/05/2023: In 2023, the researchers changed the study design from a pre-post to a parallel group design. This was necessary because due to a later start (finalization of contracts), the time in the planned pre-phase was insufficient to recruit enough practices and patients. In order to achieve a sufficiently large control group, it is now being recruited in parallel with the intervention group in the region of Northern Bavaria. The planned sample size, eligible criteria and the control intervention remain unchanged. In the process evaluation, the researchers will assess structural differences in the regions and practices and will discuss possible limitations.

Primary study design
Interventional

Study type(s)
Other

Health condition(s) or problem(s) studied
Care coordination for patients with rare cancers

Interventions
TARGET (intervention, post phase)
The new care model "TARGET" includes mainly digital interventions to optimise trans-sectoral cooperation and sustainably improve the medical care of the target population. In the office-based sector, specialists with a focus on hematology/oncology will participate. Enrolled doctors will receive program-specific training. A central coordination platform will be used to establish contact with the Comprehensive Cancer Center Munich (CCC Munich) and office-based doctors to prepare for the inclusion of eligible patients. Enrolled patients will be assigned an oncology coach who accompanies the patient and acts as a link between the office-based doctors and CCC Munich. Additionally, the patient will be supported by a patient app that records patient-relevant outcomes and can serve as additional orientation. A decision support tool is integrated into the patient app. Besides that, the patients will have access to digital psycho-oncological services.

Furthermore, doctors will be trained in Shared Decision Making (SDM). After the necessary diagnostics have been completed, the medical procedure will be determined across disciplines and sectors in virtual tumour boards. Medical care should then be provided as close to home as possible. Within the programme framework, a project-specific case file will be established, which will enable the trans-sectoral collection and exchange of medical information.

Control (pre-phase)
In order to ensure the best possible comparability between the intervention and the control group in the absence of randomisation, the patients in the pre-phase will be recruited in the same setting as in the post-phase. The doctors are therefore initially enrolled for the pre-phase and change then to the intervention.

The patients will receive optimised standard care. In addition to the usual care, they will have access to the patient app. No intervention components will be provided but outcome data will

be collected via the patient app and information on diet for cancer will be presented to make the app more attractive.

Patient-reported outcomes
Patient reported outcomes (PROs) are reports of patients on health-related quality of life (HRQoL), functionality, condition and symptoms. After a screening upon inclusion (T0), a weekly survey is carried out via the patient app. The German version of the EORTC QLQ-C30, 30 items, each with 4 or 7-point Likert scales, is used. The entire questionnaire is used at T0 and T3, additionally the functional scales at T2 and individual items on symptom distress weekly up to T3.

Health literacy
Measurement via the German version of the Functional Communicative Critical Health Literacy (FCCHL) questionnaire (14 items, 4-point Likert scales) at T1.

Clinical parameters
Clinical parameters will be assessed via the project-specific case file at baseline and 3, 6 and 16 months after inclusion (T0, T2, T3, T4).
Progression-free survival (PFS): Length of time from the start of therapy until disease progression or death, regardless of the cause of death; measurement 3, 6 and 16 months after inclusion (T2, T3, T4).
Overall survival (OS): OS is measured as time to an event from study entry. The follow-up is 16 months after inclusion (until T4).
Course of therapy: Percentage of curative / palliative approaches, type of therapy (including surgery, chemotherapy, radiotherapy, individualised therapy), percentage of patients with advanced cancer with standard therapy, number of therapy lines. The follow-up is 12 months after inclusion (until T4).
Time to diagnosis: A faster diagnosis suggests earlier access to individualised therapy. The time from the first contact with the doctor to the final diagnosis is recorded. If a diagnosis has already been made, data will be collected at baseline (T0), otherwise 3 months (T2) after inclusion.
Molecular diagnostics: One goal of TARGET is to enable more patients to have advanced molecular diagnostics and the therapy options that may result from this. After 16 months of follow-up (T4), it will be ascertained whether an extended molecular diagnosis has taken place, whether the patient has been presented to a molecular tumour board and whether this has resulted in a therapeutic option.

Reduction of costs
Costs associated with inpatient treatments, day-clinic treatments, outpatient treatments, pharmaceuticals, therapeutic and assistive products, rehabilitation treatments, travel expenses, costs for all of the above-mentioned categories and costs related to an inability to work of the intervention group and the control group of the economic evaluation will be evaluated.

Intervention Type
Other

Primary outcome(s)
Coordination of care from the patient's perspective measured using the German version of the Care Coordination Instrument (CCI-D), a questionnaire for cancer patients (29 items, each 4-point Likert scales, from strong to no agreement) via the patient app one month (T1) and six months (T3) after inclusion

Key secondary outcome(s))

Patient-reported outcomes
1. Health-related quality of life (HRQoL), functionality, condition and symptoms measured using the German version of the EORTC QLQ-C30 patient-reported outcomes (PROs) (30 items, each with 4 or 7-point Likert scales) undertaken at weekly intervals after screening upon inclusion (T0) via the patient app. The entire questionnaire is used at T0 and T3, additionally the functional scales at T2 and individual items on symptom distress weekly up to T3.
2. Health literacy measured using the German version of the Functional Communicative Critical Health Literacy (FCCHL) questionnaire (14 items, 4-point Likert scales) at T1

Clinical parameters
3. Clinical parameters measured using the project-specific case file at baseline and 3, 6 and 16 months after inclusion (T0, T2, T3, T4)
4. Progression-free survival (PFS; length of time from the start of therapy until disease progression or death, regardless of the cause of death) measured using doctor's documentation in the project-specific case file at 3, 6 and 16 months after inclusion (T2, T3, T4)
5. Overall survival (OS; time to an event from study entry) measured using doctor's documentation in the project-specific case file. The follow-up is 16 months after inclusion (until T4).
6. Course of therapy: Percentage of curative / palliative approaches, type of therapy (including surgery, chemotherapy, radiotherapy, individualised therapy), percentage of patients with advanced cancer with standard therapy, number of therapy lines measured using doctor's documentation in the project-specific case file. The follow-up is 12 months after inclusion (until T4).
7. Time to diagnosis (if a diagnosis has already been made) measured using doctor's documentation in the project-specific case file at baseline (T0), otherwise 3 months (T2) after inclusion.
8. Molecular diagnosis has taken place, whether the patient has been presented to a molecular tumour board and whether this has resulted in a therapeutic, measured using doctor's documentation in the project-specific case file after 16 months of follow-up (T4)

Reduction of costs
9. Costs associated with inpatient treatments, day-clinic treatments, outpatient treatments, pharmaceuticals, therapeutic and assistive products, rehabilitation treatments, travel expenses, costs for all of the above-mentioned categories and costs related to the inability to work of the intervention group and the control group of the economic evaluation measured using data from the AOK (health insurance company) up to 16 months follow-up (T4).

Updated 09/05/2023: The secondary outcome measure “Molecular diagnostics” will not be assessed. The risk was seen that the assessment could influence the doctors of the control group in such a way that they would refer the control patients to a specialised centre for such a molecular diagnosis.

Completion date
31/12/2025

Eligibility

Key inclusion criteria
1. Patients with rare cancers (suspected rare cancer, an initial diagnosis of rare cancer, an advanced rare cancer)
2. Aged 18 years and over

3. Legally insured with AOK Bayern (a German health insurance company)
4. Able to give informed consent
5. Able to use a smartphone or tablet
6. Life expectancy > 3 months

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 years

Sex
All

Key exclusion criteria
1. Patients with uncontrolled co-morbidities
2. Common cancers with a typical course
3. Insufficient knowledge of German to take part in the surveys via the app
4. Not insured with AOK Bayern

Date of first enrolment
10/10/2022

Date of final enrolment
30/09/2025

Locations

Countries of recruitment
Germany

Study participating centre
Comprehensive Cancer Center Munich (CCC Munich)
Pettenkoferstraße 8a
Munich
Germany
80336

Sponsor information

Organisation
German Aerospace Center

ROR
https://ror.org/04bwf3e34

Funder(s)

Funder type
Government

Funder Name
Gemeinsame Bundesausschuss (Federal Joint Committee)

Alternative Name(s)
Federal Joint Committee, G-BA

Funding Body Type
Government organisation

Funding Body Subtype
National government

Location
Germany

Results and Publications

Individual participant data (IPD) sharing plan
The datasets generated during and/or analysed during the current study are/will be available upon request from Anke Steckelberg, anke.steckelberg@medizin.uni-halle.de. On request, individual participant data will be shared that will underlie the results of the summative evaluation reported in the related publication (text, tables, figures, and appendices). In addition, the study protocol will be publicly available.
The participant data will be available after deidentification beginning 3 months and ending 5 years following article publication. Participants will give consent to storing and publication of the anonymous data.
Researchers who provide a methodologically sound proposal will gain access to analyses to achieve the aims of the approved proposal. The approval will be based on scientific considerations and with the involvement of the responsible data protection officer. To gain access, data requestors will need to sign a data access agreement.

IPD sharing plan summary
Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Protocol article	Protocol for a Mixed Methods Process Evaluation	12/10/2023	16/10/2023	Yes	No
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes
Poster results	version v1.0	08/04/2022	03/10/2022	No	No
Study website	Study website	11/11/2025	11/11/2025	No	Yes