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Peer-delivered psychosocial support for patients with rare autoimmune liver diseases

Submission date
14/10/2022

Recruitment status
No longer recruiting

Registration date
13/01/2023

Overall study status
Completed

Last Edited
16/01/2025

Condition category
Digestive System

[X] Prospectively registered
[X] Protocol
[X] Statistical analysis plan
[_] Results
[_] Individual participant data
[_] Record updated in last year

Plain English summary of protocol

Background and study aims
Rare diseases can have a substantial impact on quality of life, and psychosocial support needs of patients often go unmet. In a recent efficacy study (Depping et al., 2021, JAMA Psychiatry), we showed that a newly developed psychosocial support program for patients with rare diseases led to improved quality of life, better acceptance of the disease and several further improved outcomes. The program is delivered by peers, i.e. other affected individuals. Over six weeks, participants completed a self-help booklet from home, based on Acceptance and Commitment Therapy, and also received weekly telephone-based counseling by a peer-counselor. Peer-counselors received training, counseling guidelines and supervision. As the program is independent of patients' location and disease, it has the potential to reach many individuals. However, it is not yet available to patients and its effectiveness needs to be confirmed under routine care conditions. Therefore, with the Q.RARE.LI study, we will evaluate the effectiveness of the program in routine care of five different countries (Germany, Canada, Belgium, Poland and Hungary) and initiate the implementation.

Who can participate?
Patients with rare autoimmune liver diseases i.e. autoimmune hepatitis, primary sclerosing cholangitis and primary biliary cholangitis.

What does the study involve?
Patients will be randomly assigned to an intervention group or a control group. Patients in the intervention group will participate in the program (in addition to their routine care), while patients in the control group will receive care-as-usual alone. Patients in both groups will complete a standardized questionnaire set at three time points (before the program, after the program, after 3 months). Our primary effectiveness outcome is mental health-related quality of life.

To investigate whether the program can be implemented into routine care, we will ask patients, peer-counselors, and healthcare providers to evaluate the program in a survey. In addition, we will conduct focus group discussions with patients/patient representatives, peer-counselors, healthcare providers and health insurers to discuss if and how the program can be implemented into routine care. Based on this, we will derive country-specific implementation strategies. Q.

RARE.LI will pave the way for widely available psychosocial support for the rare disease community, which can be crucial for patients' quality of life.

What are the possible benefits and risks of participating?
This research involves adult patients with rare autoimmune liver diseases and adults involved in implementation of the intervention. In general, psychosocial support interventions bear no major risk for severe adverse events. Rather, participating in the intervention as well as being involved as a peer-counselor is expected to be beneficial. Participants will receive a financial compensation for completing the study assessments.

Where is the study run from?
The Department of Psychosomatic Medicine and Psychotherapy at the University Medical Center Hamburg-Eppendorf in Germany

When is the study starting and how long is it expected to run for?
January 2021 to May 2025

Who is funding the study?
1. European Joint Program on Rare Diseases – Joint Transnational Call 2021
2. Nation funding agency in Germany: German Federal Ministry for Education and Research (BMBF)
3. Nation funding agency in Canada: Canadian Institutes of Health Research, Institute of Genetics (CIHR-IG)
4. Nation funding agency in Belgium: The Research Foundation - Flanders (FWO)
5. Nation funding agency in Poland: National Centre for Research and Development (NCBR)
6. Nation funding agency in Hungary: National Research, Development And Innovation Office, Hungary

Who is the main contact?
Dr Natalie Uhlenbusch, n.uhlenbusch@uke.de

Contact information

Type(s)
Principal investigator

Contact name
Dr Natalie Uhlenbusch

ORCID ID
https://orcid.org/0000-0002-4031-9839

Contact details
Department of Psychosomatic Medicine and Psychotherapy
University Medical Center Hamburg-Eppendorf
Martinistr. 52
Hamburg
Germany
20146
+49 40 7410 52822
n.uhlenbusch@uke.de

Additional identifiers

Study information

Scientific Title
Improving health-related quality of life in patients with rare autoimmune liver diseases by structured peer-delivered support: a transnational effectiveness-implementation hybrid trial

Acronym
Q.RARE.LI

Study objectives
The primary research objective of Q.RARE.LI is to investigate the effectiveness of a peer-delivered psychosocial support intervention (Depping et al., 2021, JAMA Psychiatry) under routine care conditions in five different healthcare settings and to prepare its implementation.

We therefore have two research foci:
A. Effectiveness
1. Primary hypothesis: Under routine care conditions, structured peer-delivered psychosocial support in addition to CAU leads to better mental health-related quality of life compared to CAU alone in patients with rare liver diseases at post-assessment.
2. Secondary hypotheses: Under routine care conditions, structured peer-delivered psychosocial support in addition to CAU leads to better mental health-related quality of life at 3-month follow-up and to improved outcomes regarding a) physical health-related quality of life, b) depression severity, c) anxiety severity, d) illness acceptance, e) perceived helplessness f) social support and g) self-management abilities compared to CAU alone in patients with rare liver diseases both at post-assessment and 3-month follow-up.

B. Implementation
1. Primary hypothesis: The intervention shows high acceptability and feasibility in routine care as indicated by: 1) >75% of the patients completing the intervention, 2) >75% of these patients rating the intervention as a) helpful and b) appropriate, 3) 75% of the stakeholders delivering the intervention rating it as a) feasible, b) appropriate and c) helpful for patients.

Ethics approval required
Old ethics approval format

Ethics approval(s)
1. Approved 31/01/2022, Ethics Committee of the Hamburg Medical Association (Weidestr. 122 b, 22083 Hamburg, Germany; +49 40 202299-240; ethik@aekhh.de), ref: 2021-100757-BO-ff
2. Approved 14/02/2023, University Health Network Research Ethics Board (700 University Ave, 4th Floor, Toronto, Ontario, M5G 1Z5, Canada; +1 (416) 581-7849; reb@uhnresearch.can), ref: 22-5056
3. Approved 22/02/2023, Committee on Medical Ethics, UZ Gent (Corneel Heymanslaan 10, 9000 Ghent, Belgium; +32 9 3322111; ethisch.comite@uzgent.be), ref: BC-10401
4. Approved 21/02/2022, The Local Ethics Committee of Medical University of Warsaw (ul. Pawińskiego 3C, 02-106 Warszawa, Poland; +48 22 57 20 303; komisja.bioetyczna@wum.edu.pl), ref: KB/26/2022

5. Approved 16/08/2021, Medical Research Council, Scientific and Research Ethics Committee (ETT TUKEB, 25 Alkotmány u., Budapest, H-1054, Hungary; +36 1 795 1192; attilane.gombos@bm.gov.hu), ref: 40513-5/2021/EÜIG

Primary study design
Interventional

Study design
Interventional randomized controlled trial with mixed methods process evaluation

Study type(s)
Quality of life

Health condition(s) or problem(s) studied
Rare autoimmune liver diseases, i.e. autoimmune hepatitis (AIH), primary sclerosing cholangitis (PSC) and primary biliary cholangitis (PBC)

Interventions
We will conduct a multicenter effectiveness-implementation hybrid trial type 1 (Curran et al., 2021, Med Care) with the primary aim of assessing the effectiveness of the peer-delivered psychosocial support intervention while also analyzing its implementability, identifying implementation barriers, and deriving country-specific implementation strategies.

Design of the effectiveness part of the trial: The effectiveness of the intervention under routine care conditions will be evaluated within a two-armed randomized controlled trial (RCT) comparing structured and peer-delivered psychosocial support plus care-as-usual (CAU) to CAU alone in adult patients with autoimmune rare liver diseases.

Design of the implementation part of the trial: We will conduct a mixed-methods process evaluation assessing implementation outcomes both quantitatively (cross-sectional survey) and qualitatively (focus groups) including different stakeholders.

Using a fixed randomization schedule (allocation ratio 1:1), patients will be randomly assigned to the intervention group or the control group. The intervention group will participate in the 6-week-program in addition to care-as-usual (CAU) while the control group receives CAU only.

The psychosocial support program was developed based on pre-assessed psychosocial support needs of patients with rare diseases (Depping et al., 2021, BMJ Open) and evaluated in a first efficacy trial (Depping et al., 2021, JAMA Psychiatry). The program is based on structured self-help and peer-counseling. Participants receive a manual, which contains six chapters and which is based on Acceptance and Commitment Therapy (ACT). The first chapter includes general information about rare diseases and a reflection exercise on how the disease affects one’s life. The second chapter focuses on dealing with difficult emotions, the third chapter is about disease acceptance, the fourth one about values, the fifth one about setting meaningful goals and the last chapter contains a review and outlook exercise. Patients complete one chapter per week and, in addition, receive a 30-minute telephone-based peer-counseling session to reflect on the content of the manual. Peer-counselors receive a two-day training, consultation guidelines with additional information and exemplary questions for each chapter as well as supervision on demand.

Intervention Type

Behavioural

Primary outcome(s)
1. Effectiveness outcomes will be assessed at baseline, after the program and at a 3-month follow-up. Our primary effectiveness outcome is the baseline-adjusted group difference in mental health-related quality of life directly after the intervention, measured with the Short Form Health Survey (SF-12).
2. Quantitative implementation outcomes will be assessed at post-assessment. Qualitative implementation outcomes after the follow-up assessment. Our primary implementation outcomes are acceptability and feasibility of the program, operationalized by completion rate and subjective rating of appropriateness, helpfulness, and feasibility on numeric rating scales from 0-10.

Key secondary outcome(s)
All effectiveness outcomes will be assessed at baseline, after the program and at a 3-month follow-up.
1. Physical health-related quality of life (Short Form Health Survey (SF-12))
2. Somatic symptom severity (Patient Health Questionnaire-15 (PHQ-15))
3. Depression severity (Patient Health Questionnaire-9 (PHQ-9))
4. Anxiety severity (Generalized Anxiety Disorder Scale-7 (GAD-7))
5. Disease acceptance (Acceptance subscale of the Illness Cognition Questionnaire (ICQ))
6. Helplessness (Helplessness subscale of the ICQ)
7. Social support (Social Support Questionnaire (F-SOZU))
8. Self-management abilities (Appraisal of the Self-Care Agency Scale Revised (ASAS-R))
10. Psychological burden related to somatic symptoms or associated health concerns is measured using the Somatic Symptom Disorder – B Criteria Scale (SSD-12)
11. General self-efficacy is measured using the Self-efficacy scale (SWE)
12. Illness perceptions are measured using the Brief Illness Perception Questionnaire (B-IPQ)

Completion date
31/05/2025

Eligibility

Key inclusion criteria
Effectiveness focus:
1. Autoimmune rare liver disease (AIH, PSC, PBC)
2. Subjective psychosocial support need
3. ≥18 years
4. Written informed consent

Implementation focus:
Patients:
see above

Stakeholders:
1. Involvement in intervention delivery/implementation i.e. healthcare providers, patient representatives, health insurers
2. Written informed consent

Participant type(s)

Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 Years

Sex
All

Key exclusion criteria
Effectiveness focus:
1. Life-threatening health-status
2. Acute suicidality
3. Ongoing psychotherapy
4. Severe cognitive, auditory or visual impairment
5. Inability to complete assessments

Implementation focus:
1. Involvement in outcome assessment or data analysis

Date of first enrolment
31/01/2023

Date of final enrolment
31/01/2024

Locations

Countries of recruitment
Belgium

Canada

Germany

Hungary

Poland

Study participating centre
University Medical Centre Hamburg-Eppendorf, Department of Psychosomatic Medicine and Psychotherapy
Martinistraße 52
Hamburg

Germany
20246

Study participating centre
Toronto Centre for Liver Disease, Toronto General Hospital, University Health Network
200 Elizabeth St.
Toronto
Canada
M5G 2C4

Study participating centre
Ghent University Hospital, Department of Internal Medicine and Pediatrics, Faculty of Medicine and Health Sciences
Corneel Heymanslaan 10
Ghent
Belgium
9000

Study participating centre
Medical University of Warsaw, Liver and Internal Medicine Unit
Banacha 1A
Warsaw
Poland
02-097

Study participating centre
University of Debrecen, Faculty of Medicine, Department of Internal Medicine, Division of Gastroenterology
Nagyerdei krt. 98
Debrecen
Hungary
H-4032

Sponsor information

Organisation
University Medical Center Hamburg-Eppendorf

ROR

https://ror.org/038p55355

Funder(s)

Funder type
Government

Funder Name
European Joint Program on Rare Diseases – Joint Transnational Call 2021

Funder Name
Nation funding agency in Germany: German Federal Ministry for Education and Research (BMBF)

Funder Name
Nation funding agency in Canada: Canadian Institutes of Health Research, Institute of Genetics (CIHR-IG)

Funder Name
Nation funding agency in Belgium: The Research Foundation - Flanders (FWO)

Funder Name
Nation funding agency in Poland: National Centre for Research and Development (NCBR)

Funder Name
Nation funding agency in Hungary: National Research, Development And Innovation Office, Hungary

Results and Publications

Individual participant data (IPD) sharing plan
In accordance with the ethics committee approval and the 2015 German Research Foundation (DFG) guidelines for the handling of research data, quantitative data will be made publicly available in a deidentified form. The times and the conditions of the availability of data will also be in accordance with the 2015 “Recommendations for Sharing Clinical Trial Data” of the Institute of Medicine (IOM). The full data package (i.e. analyzable data set, protocol, statistical

analysis plan, statistical programming code) will be made freely available through a clinical data repository (e.g., Dryad Digital Repository) and saved for at least 10 years. Data sharing will follow the FAIR Data Principles (Findable, Accessible, Interoperable and Reusable) and international naming conventions (e.g. Systematized Nomenclature of Medicine) to maximize transparency and scientific reproducibility.

IPD sharing plan summary

Stored in publicly available repository

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Protocol article	version 1.0	24/03/2023	27/03/2023	Yes	No
Statistical Analysis Plan		22/11/2024	16/01/2025	No	No