The Scleroderma Patient-centered Intervention Network: SPIN

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Patient-centered care

- Patient empowerment
- Shared decision-making
- Care plans taking into account patient preferences and values
- Care that meets the needs of individual patients
- Evidence-based
- In chronic disease:

    comprehensive care to reduce disability and improve health-related quality of life

Treatment of scleroderma

EULAR guidelines (Kowal-Bielecka et al. 2009):

“There are also other treatment options for the management of SSc patients, such as physiotherapy, education, new experimental therapies, etc, which were beyond the scope of this project or could not be included because of the lack of expert consensus”

What about rare diseases?

- Typically no access to psychosocial and rehabilitation interventions that are:
  - Specific to needs of people with the disease
  - Adequately tested to determine if useful

What about rare diseases?

- Search for studies across all 6,632 rare diseases listed on NIH Office of Rare Disease Research (July 2011)

- Sought randomized controlled trials (RCT) of psychosocial and rehabilitation interventions with at least 100 patients

- Found only 1 RCT (an exercise intervention for patients recovering from SARS)

Why?

- Few (specialized) centers treat enough patients with a rare disease to:
  - Develop and sustain disease-specific psychosocial and rehabilitation service
  - Conduct large enough trials of disease-specific interventions
- Health care providers in local settings generally have little or no experience with a rare disease and specific needs of patients

Challenges

- To develop supportive interventions (psychosocial, rehabilitation) that are:
  - Accessible to people with scleroderma
  - Low cost for feasible implementation
  - Can be delivered on an ongoing basis

- To conduct high-quality trials to confidentially assess impact interventions (including at least 200-300 people)

SPIN: The Scleroderma Patient-centered Intervention Network

SPIN

- A collaboration of people living with scleroderma, clinicians and researchers

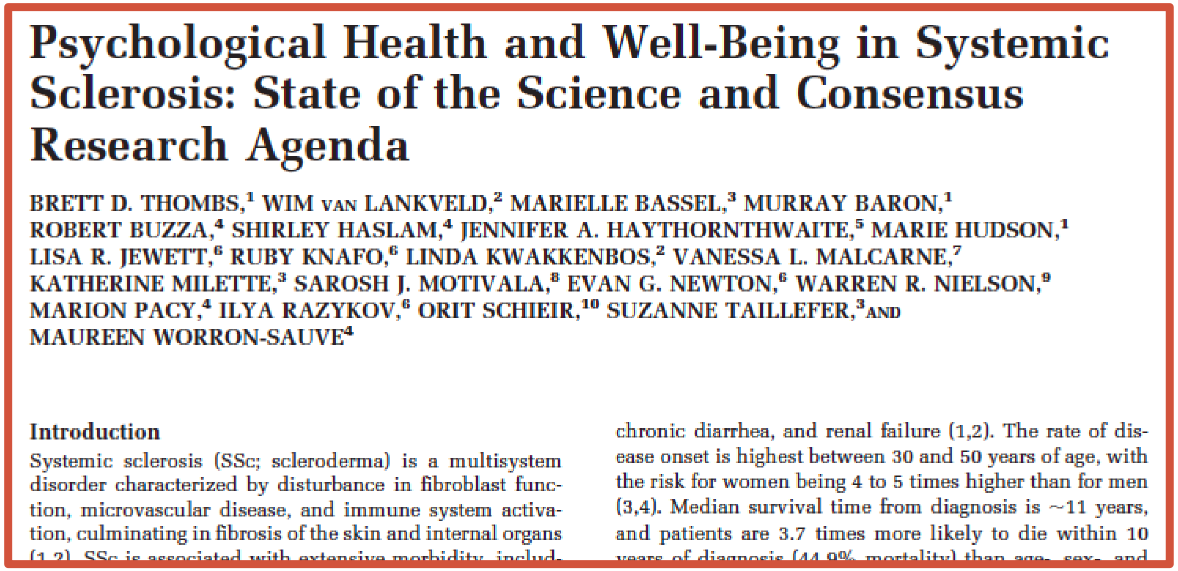
- Aim: To develop and evaluate psychosocial and rehabilitation interventions that are accessible, low-cost, and can be delivered on an ongoing basis to people living with scleroderma

SPIN Background

- Canadian Scleroderma Research Group (CSRG)
- Partnering with scleroderma patient organizations
- 2008: Improving psychological health and well-being in scleroderma (consensus statement)
- 2010: Canadian Patient Survey of Health Concerns and Healthcare Needs

Psychological Health and Well-being: A Consensus Research Agenda

- Fatigue
- Pain
- Depressive symptoms
- Pruritus (Itch)
- Body Image
- Sexual function
- Other areas



Psychological Health and Well-Being in Systemic Sclerosis: State of the Science and Consensus Research Agenda

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Introduction

Systemic sclerosis (SSc; scleroderma) is a multisystem disorder characterized by disturbance in fibroblast function, microvascular disease, and immune system activation, culminating in fibrosis of the skin and internal organs (1,2). SSc is associated with extensive morbidity, includ-

chronic diarrhea, and renal failure (1,2). The rate of disease onset is highest between 30 and 50 years of age, with the risk for women being 4 to 5 times higher than for men (3,4). Median survival time from diagnosis is ~11 years, and patients are 3.7 times more likely to die within 10 years of diagnosis (44.9% mortality) than age-, sex-, and

SPIN Background

- 2010: Consortium for clinical trials of behavioural, psychological and educational interventions
- 2011: SPIN planning meeting

Canadian Institutes for Health Research Team Grant:

  - $1.5 million (2012 – 2017) for operating costs
  - Additional $300,000 in partner funding

  - For SPIN in English and French
  - Other languages: national funding (e.g. the Netherlands)

SPIN – Key components

1) **Patient organization partnerships throughout the research process and as end user**
2) International network of clinical research centers
3) Virtually delivered interventions
4) Cohort multiple RCT (cmRCT) design in rare disease context

Partnering with Scleroderma Patient Organizations

SSC

Scleroderma
Society of Canada

Société Canadienne
de la Sclérodermie

SSO

Scleroderma Society of Ontario

SABC

Scleroderma Association of British Columbia

Celebrating 10 Years of Service 1998-2008

SCLERODERMA
FOUNDATION

SUPPORT · EDUCATION · RESEARCH

Sclérodermie

NVLE

Scleroderma
Society

Supporting people with Scleroderma for over 25 years

FESCA IVZW

Federation of European Scleroderma Associations

SPIN – Key components

1) Patient organization partnerships throughout the research process and as end user
2) **International network of clinical research centers around the world**
3) Virtually delivered interventions
4) Cohort multiple RCT (cmRCT) design in rare disease context

SPIN Members

- Canada (CSRG, McGill)
- USA (UCLA, Michigan, Texas, Johns Hopkins, Utah, Georgetown, Northwestern, Boston University, Stanford)
- France (Paris center)
- The Netherlands (Nijmegen center)
- Australia (Melbourne and Adelaide)
- UK (London center)
- Spanish (Spain, US, Mexico)

SPIN – Key components

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4) Cohort multiple RCT (cmRCT) design in rare disease context

Online self-help interventions

- Increasingly common, for instance:
  - Self-management in diabetes (e.g., Lorig et al, 2010)
  - Depressive symptoms (e.g., Gellatly et al, 2007)
  - Anxiety (e.g., Hirai & Clum, 2006)

- Self-guided online interventions were effective in reducing elevated levels of depressive symptoms (Cuijpers et al, 2011)
    - 7 trials (total N = 1,362)
    - $d=0.28$ ($p<0.001$)

SPIN – Key components

1) Patient organization partnerships throughout the research process and as end user
2) International network of clinical research centers
3) Virtually delivered interventions
4) **Cohort multiple RCT (cmRCT) design in rare disease context**

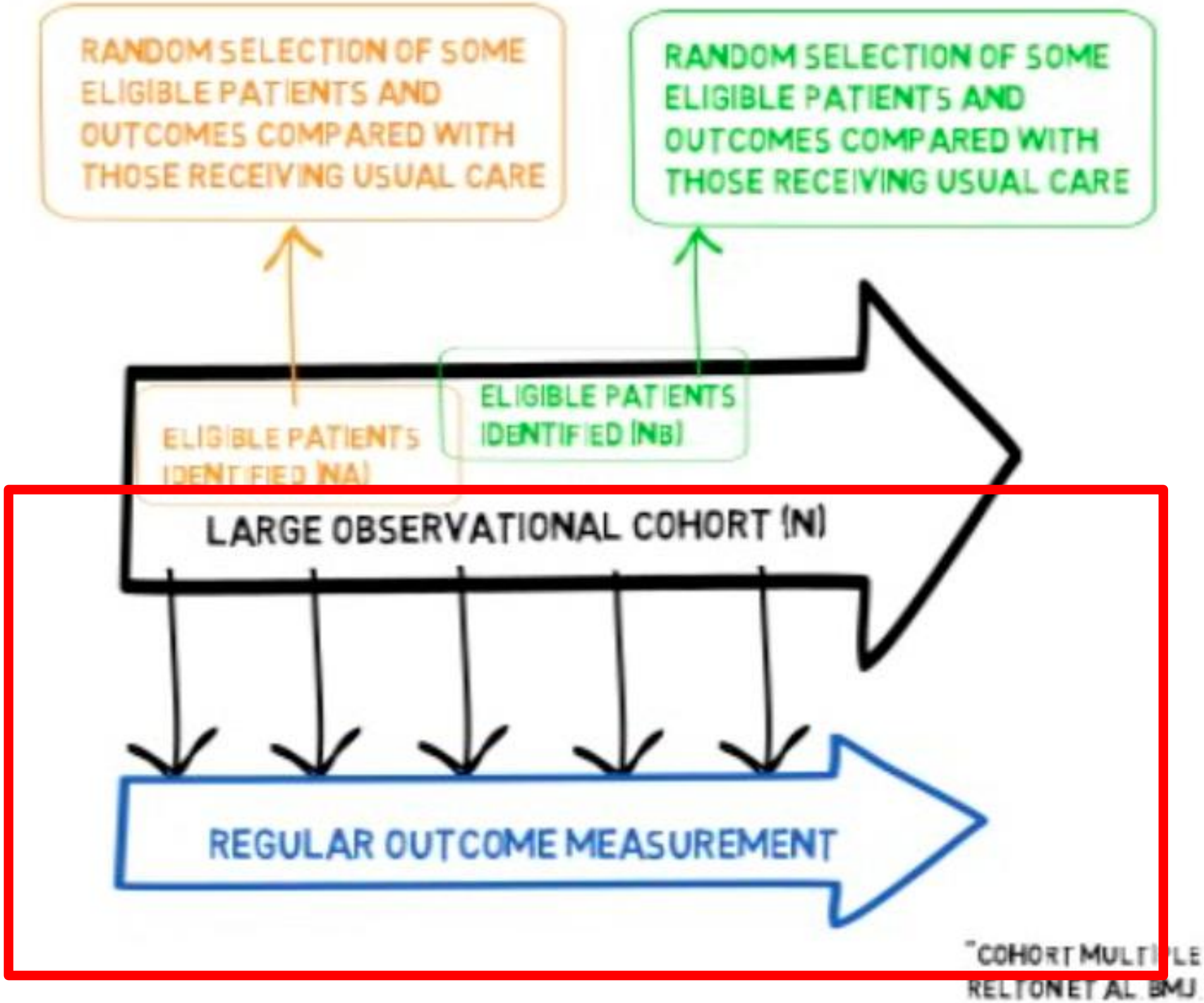
Pragmatic Trials

- Health care decisions: evidence-based medicine
- Explanatory trials: *efficacy*
  - Does an intervention work under ideal circumstances? Can this intervention work?
- Pragmatic trials: *effectiveness*
  - Does an intervention works under real-life conditions? Does it work in terms that matter to the patient?
  - Intervention + treatment as usual vs. treatment as usual

Problems with traditional RCT designs

- Poor recruitment rates – implications for cost, validity, reliability, comparability of the results
- Informed consent barrier to recruitment (Ross, 1999)
- Unrepresentative recruited population
- Patient & clinician treatment experiences altered
- Disappointment bias in control group
- Lack of long term outcomes
- Samples across trials not comparable
- In rare diseases: difficulty of recruiting a sufficiently large patient group for trials

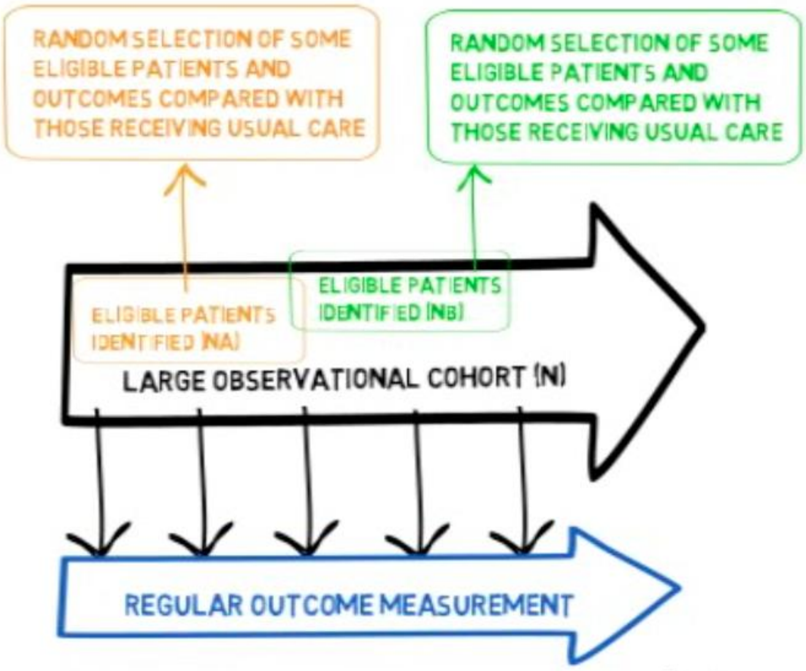
cmRCT design: Cohort



"COHORT MULTIPLE RCT DESIGN"
RELTON ET AL. BMJ 2010

cmRCT design features: Cohort

- Recruitment of large observational cohort
- Regular measurement of outcomes
- Ongoing data collection



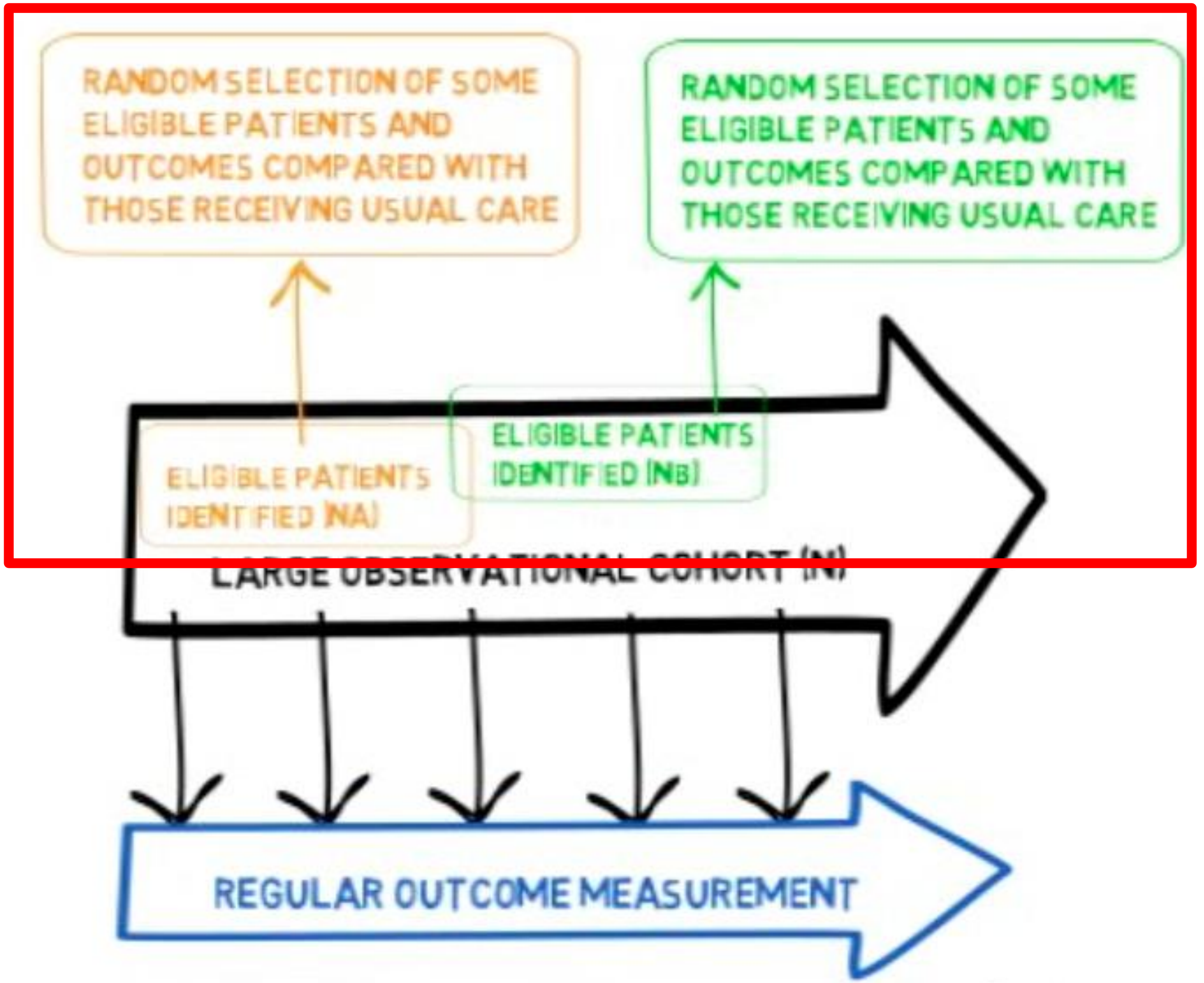
"COHORT MULTIPLE RCT DESIGN"
RELTON ET AL BMJ 2010

cmRCT design: Cohort consent

Consent elements **for inclusion in cohort**:

1) To provide observational data that will be used to better understand problems that may be important to people with scleroderma
2) To use responses to determine if someone is eligible for an intervention, and to be contacted to participate in interventions
3) To compare responses to the responses of people in the cohort who participated in an intervention (in which patient did not participate)

cmRCT design: Trials



"COHORT MULTIPLE RCT DESIGN"
RELTON ET AL. BMJ 2010

cmRCT design features: Random selection

- Capacity for multiple RCTs over time without repeating recruitment
- For each trial:
  - Eligible patients identified, of which some randomly selected to be offered the intervention
  - Outcomes of eligible, randomly selected patients compared to eligible patients not randomly selected

Benefits of cmRCT design

- Recruitment – improved quantity and more representative sample
- Multiple RCT facility
- Long term outcomes as standard
- Ongoing information as to the natural history of the condition and treatment as usual
- Increased comparability between each trial conducted within the cohort
- Less disappointment bias (patients are only told about intervention that is offered to them)

Least suited to…

- ‘Blinded’ trials with placebo arms
- Research questions with hard to measure / hard to collect outcomes (e.g., blood samples)
- Acute or short term conditions

Step 1: The SPIN Cohort

Large international study with people with scleroderma (N=1,500-2,000):

- Online questionnaires every 3 months
- Insight in problems important to patients
- How best to measure these outcomes
- Natural history of scleroderma and treatment as usual

Step 1: The SPIN Cohort

Focus on:

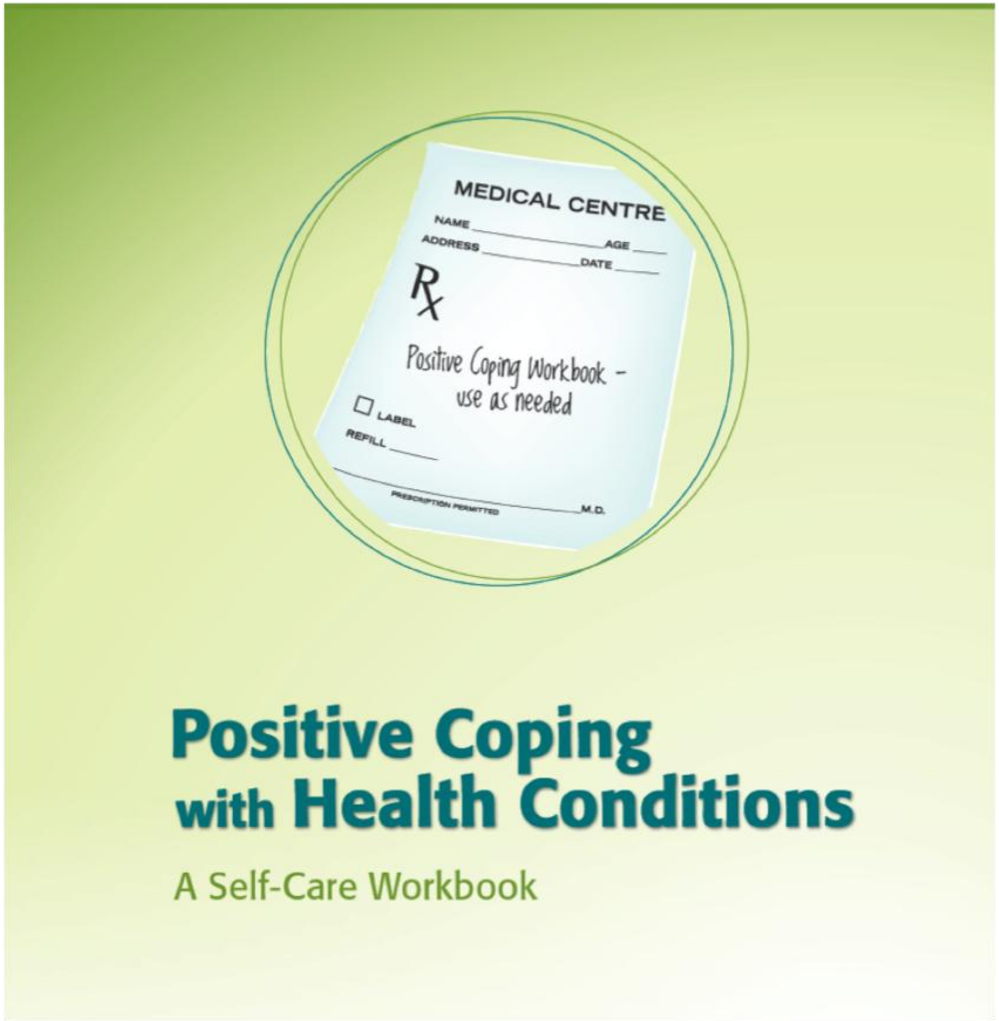
- Physical limitations
- Impact of scleroderma symptoms
- Hand function problems
- Emotions and stress
- Changes in appearance, body image

Step 2: Development of interventions

- Specific to living with scleroderma
- Self-guided (no therapist involved)
- Virtual, online
- Engaging (video, animations)

- Support patients in coping with their disease (e.g., emotional distress, body image)
- Reduce limitations in daily activities (e.g., exercise, hand function)

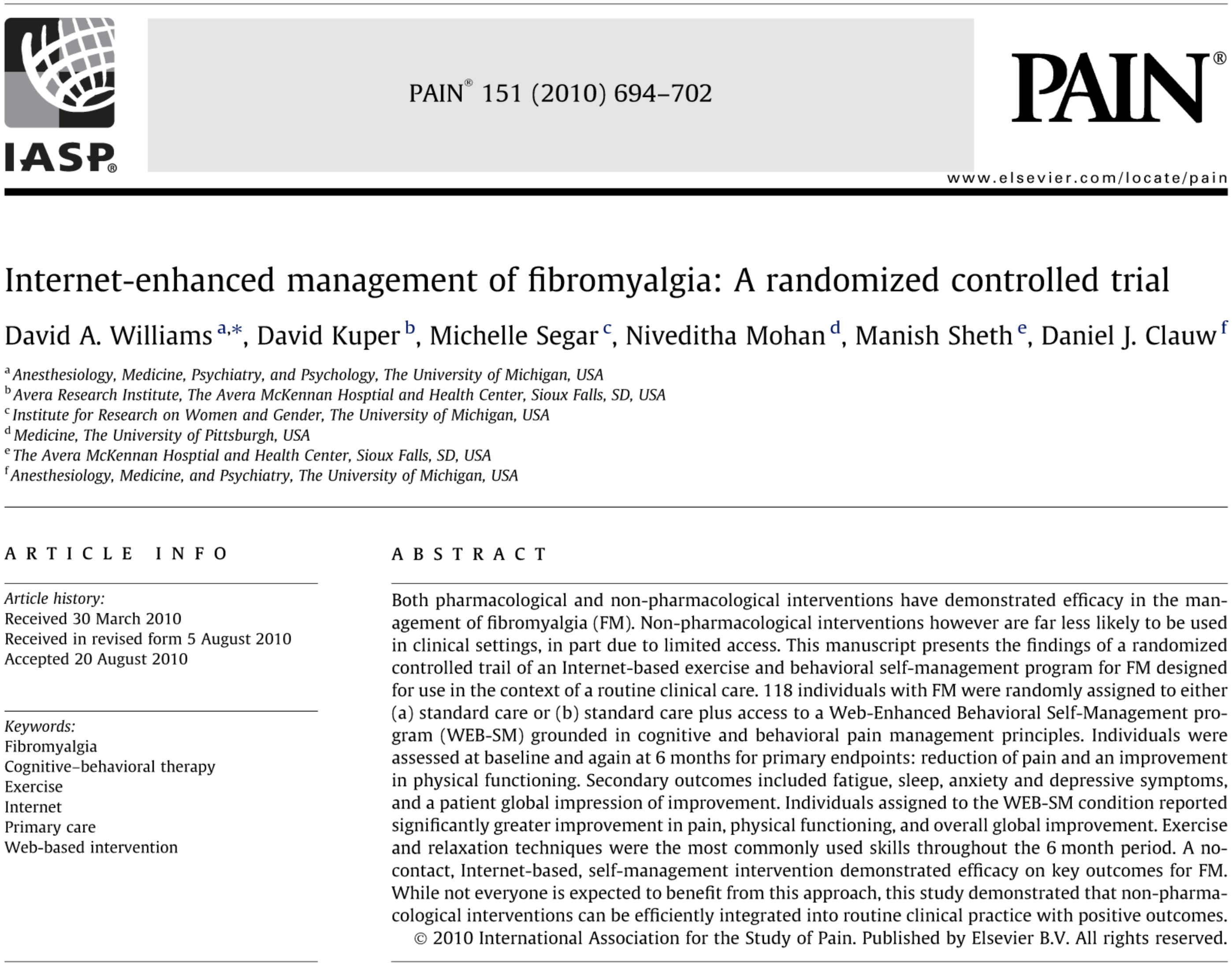
Coping with emotions & stress



Positive Coping
with Health Conditions

A Self-Care Workbook

Scleroderma Self-Management



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Internet-enhanced management of fibromyalgia: A randomized controlled trial

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ABSTRACT

Both pharmacological and non-pharmacological interventions have demonstrated efficacy in the management of fibromyalgia (FM). Non-pharmacological interventions however are far less likely to be used in clinical settings, in part due to limited access. This manuscript presents the findings of a randomized controlled trail of an Internet-based exercise and behavioral self-management program for FM designed for use in the context of a routine clinical care. 118 individuals with FM were randomly assigned to either (a) standard care or (b) standard care plus access to a Web-Enhanced Behavioral Self-Management program (WEB-SM) grounded in cognitive and behavioral pain management principles. Individuals were assessed at baseline and again at 6 months for primary endpoints: reduction of pain and an improvement in physical functioning. Secondary outcomes included fatigue, sleep, anxiety and depressive symptoms, and a patient global impression of improvement. Individuals assigned to the WEB-SM condition reported significantly greater improvement in pain, physical functioning, and overall global improvement. Exercise and relaxation techniques were the most commonly used skills throughout the 6 month period. A no-contact, Internet-based, self-management intervention demonstrated efficacy on key outcomes for FM. While not everyone is expected to benefit from this approach, this study demonstrated that non-pharmacological interventions can be efficiently integrated into routine clinical practice with positive outcomes.

Coping with appearance changes

Changing Faces.

Changing
the way you face
disfigurement

UWE
BRISTOL
University of the
West of England

Online support for young people with a visible difference

about

The website has been designed by young people and uses various activities, illustrations, photographs, videos and advice from young people with visible differences.

read +

get started!

To get started is simple and easy.

If you are under 16 we need your parents' or guardians' permission to join the programme.

discussion forum

This is a place where you can meet and socialise with other young people with a visible difference. You can discuss your experience of YP Face It, talk about any worries you may have or offer support to others.

read +

reviews

" I think YP Face It can really help. It's great that young people are taught skills to improve their confidence in social situations and hear about others with similar problems who have overcome their difficulties. "

Amanda Redman, actress

next →

Improving Hand Function

Scleroderma hand problems:

- Stiffness in hands
  - 81% at least sometimes
  - Of these patients, 73% moderate, severe, or extreme impact on daily activities
- Difficulty making fists (67%, 73%)
- Difficulty holding objects (67%, 76%)

Step 3: Feasibility

How well do our study processes and interventions work?

- Research methodology (process and management)
- Acceptability, utility and practicality of intervention to people with scleroderma
- Interviews, online feedback

→ Adjustments if necessary

Step 4: Randomized Controlled Trials

Does the intervention improve outcomes important to people living with scleroderma?

→ Outcomes of people with scleroderma who received the intervention are compared with outcomes of people who did not receive the intervention (but completed questionnaires in SPIN Cohort)

Step 5: Dissemination

Making interventions available to people with scleroderma around the world through patient organizations

SPIN Innovations

- Leveraging technology to deliver care
- Accessible interventions specific to living with scleroderma
- Partnering with patient organizations to develop and deliver interventions not feasibly provided by any single centre
- Network of major clinical research centers across Canada, the US, and Europe
- Novel research methods

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CIHR IRSC
Canadian Institutes of Health Research
Instituts de recherche en santé du Canada

Fonds de la recherche en santé
Québec