Understanding treatment needs to improve care and outcomes for children and adults with rheumatic conditions and their caregivers.

Dr Amy Helen Kelly

BMed Sci(Hons) MBBS BSci (Med) FRACP

Supervisor: A/Prof. Davinder Singh-Grewal

Associate Supervisor: Prof. Allison Jaure

A thesis submitted to fulfill the requirements for the degree of Doctor of Philosophy

Faculty of Medicine and Health

The University of Sydney Australia

2026

Table of contents

Declaration

This is to certify that the content of this thesis is my own work. This thesis has not been submitted for any other degree or purpose.

I certify that the intellectual content of this thesis is the product of my own work, and that all assistance received in preparing this thesis and all sources have been acknowledged.

Dr Amy Helen Kelly

14th November 2025

Acknowledgements

My purpose in completing this Doctorate was to develop my research skills to better understand what really matters to patients. Throughout my clinical work, I had observed that the health system is constructed in a way that favours the health care workforce over patients and their families. As clinicians working in the system we often fail to comprehend what our patients go through before they see us, including the financial burden of days off work or school, taking leave, often hours of travel and overnight stays, car parking, not to mention finding a car space or walking from the car park, all in order to diagnose their condition and manage their symptoms. Too often our appointments are constrained by time, and the hospital or clinic setting is overwhelming. When I saw patients as a trainee, it struck me that that they did not really understand what I was trying to explain to them. It made me also wonder if they felt that I did not really understand what they were trying to tell me! Thus, understanding the patient's perspectives, was the motivation behind this thesis.

Paediatric rheumatology came to me out of my own ignorance; realising I knew very little about childhood diseases in rheumatology I set out to learn more, knowing that I would at some stage be referred paediatric patients or adults whose disease had begun in childhood. For the "start" in paediatric rheumatology I am very grateful to my supervisor Associate Professor Davinder Singh Grewal, who provided me with the opportunity to attend clinics and suggested I attempt this doctorate. My second supervisor, Professor Allison Jaure, thankyou also, I respect and admire the enormous body of work you have completed; you provided the ideas behind research, and I have learnt so much from you. To Dr Jeffery Chaitow and Ann Senner, your work to sustain paediatric rheumatology services in New South Wales is extraordinary and I thank you for your support and allowing me to access your clinics. Thankyou also to the team at Campbelltown Hospital, every time I have completed a research paper, I realise there are still many more avenues to explore, and I am looking forward to having more time to pursue this further at Campbelltown Hospital.

Finally, to the most important people in my life, my family. The last nearly 8 years has seen the size of our family double, from just three of us to now 6. At times I wondered why I was doing this and placing extra strain on my family. To my husband Andrew, thank you for all your support, without it I could never have dreamt of completing this. To Bill, Freddie, Archie and Arabella thank you for understanding when Mummy had to escape to her office or disappear for a night in Sydney to attend more clinics. To Mum, thank you for your support also. This research began when we still had Sandy, he is gone now and it's hard to believe that this research began when he was still with us. To Dad and Belinda, thank you for allowing me to crash at your place when I needed a bed in Sydney, even when I was heavily pregnant with twins and desperate to finish a first draft on one of the papers, before our lives changed forever! Thankyou.

It has been a team effort!

Attributions:

Chapter 1 of this thesis has been published as, *Range and consistency of outcome measures reported in randomised trials in dermatomyositis: a systematic review.* Kelly AH, Singh-Grewal D, Sumpton D, Hasset G, Manera KE, Tong A. Clin Exp Rheumatol. 2022 Feb;40(2):358-365. I designed the study, analysed the data and wrote the drafts of the manuscript.

Chapter 2 of this thesis has been published as, *Perspectives and experiences of parents of children with juvenile dermatomyositis: a semistructured interview study.* Kelly AH, Kelly A, Singh-Grewal D, Chaitow J, Jaure A. Pediatr Rheumatol Online J. 2025 Mar 28;23(1):34. I designed the study, analysed the data and wrote the drafts of the manuscript.

In addition to the authorship attribution statements above, in cases where I am not the corresponding author of a published item, permission to include the published material has been granted by the corresponding author.

Dr Amy Helen Kelly

14th November 2025

As supervisor for the candidature upon which this thesis is based, I can confirm that the authorship attribution statements above are correct.

Associate Professor Davinder Singh Grewal

14th November 2025

No content produced by generative AI tools has been used in the preparation of this thesis.

Dr Amy Helen Kelly

14th November 2025

This research was supported by and Australian Government Research Training Program (RTP) Scholarship.

Dr Amy Helen Kelly

14th November 2025

Works Arising from this Thesis

Original Articles in Print

Range and consistency of outcome measures reported in randomised trials in dermatomyositis: a systematic review. Kelly AH, Singh-Grewal D, Sumpton D, Hasset G, Manera KE, Tong A. Clin Exp Rheumatol. 2022 Feb;40(2):358-365.

Perspectives and experiences of parents of children with juvenile dermatomyositis: a semistructured interview study. Kelly AH, Kelly A, Singh-Grewal D, Chaitow J, Jaure A. Pediatr Rheumatol Online J. 2025 Mar 28;23(1):34.

Original articles for Submission

Priorities for outcomes among parents of children with Juvenile Dermatomyositis. A mixed methods study. Kelly A.H, Kelly A, Chaitow J, Guha C, Jaure A, Singh-Grewal D.

Telemedicine in Australia and its role in the provision of rheumatology services: A narrative review. Kelly A.H.

The experiences of using telemedicine in patients with rheumatic disease: A group interview study. Kelly A.H, Goon K, Yoon J, Singh-Grewal D.

Presentations

Annual ARA NSW-ACT Branch Meeting: Oral presentation*, Preliminary Themes; Outcome Measure reported in Dermatomyositis Trials,* November 2017

Centre for Kidney Research weekly meetings

> *Range and consistency of outcome measures reported in randomised trials in dermatomyositis: a systematic review,* 2017.
>
> *Preliminary themes: Perspectives and experiences of parents of children with juvenile dermatomyositis,* 2023.
>
> *Preliminary Themes: Priorities for outcomes among parents of children with juvenile dermatomyositis,* 2025.

Westmead Hospital weekly rheumatology meeting:
Perspectives and experiences of parents of children with juvenile dermatomyositis: a semi structured interview study, 2023.

East Coast paediatric rheumatology meeting:
Perspectives and experiences of parents of children with juvenile dermatomyositis: a semi structured interview study, 2023.

Abstract

Background

Increasingly, the importance of involving the patient in making decisions about their health care and in turn for clinicians to understand the patient's perspective is recognised. This has extended to the mandating of patient reported outcomes across many health jurisdictions in clinical trials. Attempts have been made to identify the treatment needs of rheumatic disease patients in the existing literature and many of these tools are already being utilised in current rheumatology practice. Rheumatic diseases are chronic diseases, often involving long term management over many months to years. Paediatric rheumatic disease may also require long term management with improved health outcomes seen when patients are placed at the centre of their care, promoting self-management of their chronic disease(1). As well as disease chronicity, many rheumatic diseases are rare, including Juvenile Dermatomyositis, making them often difficult to study, with small numbers; frequently data is extrapolated between conditions and different patient cohorts. Building on the existing literature, within this thesis a systematic review of outcome measures reported in myositis randomised control trials was conducted and identified that the majority of outcomes reported were surrogate markers and there were few patient reported outcome measures (PROMs). It was also identified that there was very limited data about patient and caregiver experiences in Juvenile Dermatomyositis (JDM) research and to further investigate this, two qualitative research projects were conducted. The first explains the experiences and perspectives of parents who have children diagnosed with JDM and the second study examines parents' perspectives on the outcome measures important to them.

With the onset of the COVID-19 pandemic during the researching of this thesis, it became clear that the health care system would undergo fundamental changes, particularly with regards to the delivery of health care services. As such, part 2 of this thesis evolved to investigate the current landscape of telemedicine in Australia and the patient's perspective of utilising these services, where telemedicine is now recognised as an integral part of health care delivery in Australia. A narrative review was conducted examining how telemedicine is utilised in health care in Australia and the benefits and the disadvantages, in the management of chronic disease and more specifically rheumatic diseases. A qualitative study was then carried out, investigating the experiences of rheumatic disease patients in a metropolitan centre, that provides insights into the patient's perspective when using telemedicine, to help inform clinicians and administrators as to how to best use this modality to improve health outcomes.

Part 1

Systematic review of the outcome measures reported in dermatomyositis randomised control trials.

A systematic review was completed examining the scope and consistency of outcomes reported in randomized trials in dermatomyositis. Twenty randomised control trials were included in the review including 3 trials that reported on JDM; it was identified that most outcome measures reported in dermatomyositis randomised control trials were clinical and surrogate markers and were not patient reported outcomes. There were wide variation and

heterogeneity in the outcomes reported and it was determined that there was a need for further trials to include more standardised reporting of outcome measures.

Qualitative study examining the experiences and perspectives of parents of children diagnosed with Juvenile Dermatomyositis

In this study nineteen parents were interviewed whose child had a confirmed diagnosis of JDM, at varying stages of their child's treatment journey, with the majority being within 5 years of their child's diagnosis. Following data analysis, six themes were identified, including rapid crescendo of fear and desperation, lost and unsupported in the health system, disrupting family routines, grieving what has been lost, managing an uncertain future and gaining confidence and motivation. It was concluded that the diagnosis of JDM is often delayed with parents experiencing confusion, distress and disruption to theirs and their families' lives throughout their treatment journey.

Mixed methods study identifying priority outcomes for parents of children with JDM.

In this study nineteen parents of children with JDM were interviewed and were asked to rank outcome measures in order of priority. The data was analysed and themes identified that explained the reasons behind their rankings. The highest ranked outcomes were; mortality/death, physical function and muscle weakness. Four themes were identified, experiential relevance of symptoms, confronting mortality and navigating symptoms and side effects, towards recovery and finding strength to participate and navigating uncertainty and confusion. The earlier systematic review in this thesis identifies some of the measures prioritised by parents however this mixed method study also identifies novel outcomes that should potentially be included in future JDM trials.

Part 2

A narrative Review into Telemedicine in Australia and its role in the provision of rheumatology services

In this narrative review, the literature was reviewed and summarised to determine the current utilisation of telemedicine services in Australia. The advantages and disadvantages of telemedicine within different patient populations and from a rheumatologist's perspective were examined and summarised, suggesting there are benefits to the utilisation of telemedicine including convenience and the provision of health care services in areas that have limited access to healthcare. Negative impacts, however, were also identified including patients' desire to build a relationship with their health care practitioner and placing a high value on in-person care and the physical examination.

Qualitative study examining the experience of rheumatic disease patients accessing telemedicine.

In this study, group interviews were conducted with a small number of participants (n=8) asking participants to describe their experiences and perspectives of telemedicine. Patterns and themes were identified within the data using thematic analysis. It was identified that participants prioritised the face-to-face consultation over telemedicine in certain circumstances; with priority given to the physical examination, accuracy and timely diagnosis of their rheumatic disease. Telemedicine was valued where convenience was required, such as obtaining a prescription from their General Practitioner, however a face-to-face consultation was preferred with their Rheumatologist. Despite

the small number of participants, the themes identified generally concurred with the existing literature and provided an Australian context, with it being concluded that there are circumstances where telemedicine is a valuable option to meet patient's health care needs, however patient preference for face-to-face consultations should not be ignored if patient centred rheumatology care is prioritised.

Foreword

To assess the impact of chronic disease and its treatment, the importance of a patient centred approach has been recognised(2). At the centre of the patient centred approach is the acknowledgment that patients bring expertise to their experience of illness, social background, attitude to risk, values and preferences(3). In contrast clinicians contribute knowledge about a disease including its diagnosis, treatment, prognosis and likely outcomes(3). Combining this patient expertise and clinician knowledge is vital to the planning and delivery of medical care(3). Traditionally there has been little interest in understanding the patient's experience, it has been recognised more recently, however, that a more detailed understanding of the patient's perspective, which can determine their feelings, thoughts and behaviours can influence clinical outcomes(4) and provide the platform for patient self-management in chronic diseases(1) and address the wider treatment needs of rheumatic disease patients. Treatment needs can be defined as addressing the needs of the patient to effectively achieve long-term clinical remission, with no ongoing symptoms and to optimise quality of life(5).

Rheumatic disease patients, both adult and paediatric potentially face a lifetime of chronic disease management and, as in other chronic diseases, understanding their perspective is vital to achieving high-quality care(6)and improving health outcomes. Paediatric rheumatology comprises several diseases including Juvenile Idiopathic Arthritis, Systemic Lupus Erythematosus and Juvenile Dermatomyositis and these diseases can be the cause of significant short- and long-term disability(7). Outcomes in both paediatric and adult rheumatologic conditions have dramatically improved over the last 20-30 years with the use of conventional DMARDS and biological DMARDS(8, 9) new and emerging treatments are continuing to be investigated. Children and adults diagnosed with rheumatological conditions can now enjoy improved quality of life(8, 9). Less is understood, however, about the rheumatic disease patient's and caregiver's perspective and more specifically in those diagnosed with Juvenile Dermatomyositis. More broadly there is limited knowledge about the experience of rheumatic disease patients utilising telemedicine services. This thesis aims to explore this aspect of rheumatic disease management and care, with a particular focus on the patient's and their caregiver's perspective.

Juvenile Dermatomyositis (JDM) is an inflammatory autoimmune disease in children, affecting 2-3 per million children per year(10). Clinical manifestations include; myopathy, characteristic rash, arthritis and calcinosis(11). The current 5 year survival rate of children with JDM is >95%(10), however 70%-80% of patients have ongoing disease activity(10) resulting in significant morbidity, including irreversible skeletal, muscular and cutaneous damage(12), with the potential to have a negative impact on health related quality of life of patients and their care-givers(13). One of the major aims of treating JDM is to enable the child to grow up to have the best possible life(12). However despite advances in treatments, studies of rheumatic diseases in children have shown patients to be at risk of poor social development, reduced educational and vocational achievement and reduced health related quality of life(12). These complications can have lasting impacts on both patients and their families. Much of the clinical data for JDM comes from evidence is greater than 15 years old, highlighting the desperate need for further, up to date research(12, 14).

In medical research there has been a move towards patient public involvement (PPI), where increasingly there is collaboration between researchers and patients or members of the public, to include the lived experience of the patient in medical research. More and more there are also

specific requirements placed on researchers by regulatory authorities to include PPI in their research(15). In Australia, leading patient advocacy group Arthritis Australia is actively involved in expanding PPI and supporting initiatives that prioritise consumer involvement(16). The practical application of PPI in research is the utilisation of patient reported outcome measures (PROMs) in trials, defined as …"any report of the status of a patient's health condition that comes directly from the patient without interpretation of the patient's response by a clinician or anyone else"(17).

Many of the outcome measures used in trials are problematic; principally because of the use of unvalidated surrogates, outcomes of little or no relevance to patients, limited comparability due to variable outcome selection and bias reporting of outcomes(18). PROMs have undergone some validation in rheumatic disease, specifically in dermatomyositis and increasingly juvenile dermatomyositis with evidence supporting their reliability(19). Included in this thesis is a systematic review of outcome measures in juvenile dermatomyositis and adult dermatomyositis, identifying a lack of patient reported outcome measures in existing randomised controlled trials(20).

Risk factors for poor adjustment in children with a chronic illness include characteristics of the condition, functional independence and psychosocial stress(21). These risk factors also impact on care-givers quality of life. Qualitative research can provide an in-depth insight into beliefs and attitudes of patients and their care-givers relative to these recognised risk factors(22), it can "facilitate a holistic and contextualised exploration of the multiple factors and processes involved in the chronic illness experience and thus highlight opportunities for enhanced patient care"(4). Optimal health outcomes are achieved when health care services recognise patient and caregiver's values, priorities and experiences (21). Included in this thesis is a qualitative study describing the experience of caregivers of Children with Juvenile Dermatomyositis and a second qualitative study, investigating outcome measures prioritised by parents of children with Juvenile Dermatomyositis. Ethics approval was granted by the Sydney Childrens' Hospital Network Ethics Committee under project number 2021/ETH00053.

The COVID-19 pandemic changed health care systems around the world. This thesis was developed during and after the pandemic when it became apparent that the rapidly shifting health care landscape of COVID-19 could not be ignored. Steps were therefore taken to include an aspect of pandemic healthcare that was being rapidly integrated into rheumatology care, telemedicine and hence part 2 of this thesis was realised. Given that understanding the patient's perspective is pivotal to achieving better outcomes for patients(23), it became apparent that there was a need to better understand how rheumatic disease patients experienced telemedicine services. Telemedicine is defined by the World Health organisation as "the delivery of health care services, where distance is a critical factor, by all health care professionals using information and communication technologies for the exchange of valid information for diagnosis, treatment and prevention of disease and injuries, research and evaluation, and for the continuing education of health care providers, all in the interest of advancing the health of individuals and their communities"(24). In the US up to 15% of all physicians have used telemedicine services(25) and in Australia it has been reported that up to 118.2 million telehealth services have been delivered to 18 million patients, and more than 95,000 practitioners have now used telehealth services up to July 2022(26). Telemedicine has been identified by health administrators as a cost-effective way of delivering health care services(25) and remains a pivotal aspect of health care in Australia, now

permanently embedded in both public and private medical practice with the implementation of specific telemedicine Medicare rebates(27). There have been few studies exploring the experience of rheumatic disease patients using telemedicine services in Australia. Part 2 of this thesis includes a narrative review that summarises the utilisation of telemedicine in Australian health care, its benefits and disadvantages for rheumatologists, adult and paediatric patients. Further, a qualitative study that explores the rheumatic disease patient's perspective and experience of telemedicine in a post-pandemic world is also included in part 2 of this thesis.

This research will provide insights specifically into the outcome measures reported in myositis randomised control trials, the perspectives of Juvenile Dermatomyositis patients and their caregivers and help to identify areas of priority for stakeholders to focus limited health resources both in clinical practice and in research. These areas include the implementation and uptake of PROMs in research and clinical practice as well as addressing the needs of parents of children with JDM as they navigate their child's diagnosis, management and ideally, eventual disease remission. More broadly the current telemedicine landscape in health care services in Australia will be examined and synthesised as it has evolved from the COVID-19 pandemic. Specifically, the experiences of Australian adult rheumatology patients when utilising telemedicine services will also be studied; their priorities and preferences for the delivery of health care services via telemedicine or in-person, as telemedicine remains a pivotal part of the Australian health care landscape. This thesis provides novel information that can be utilised by both health care providers and administrators, placing the patient at the centre of their health care, to improve health outcomes in rheumatic disease patients as navigate their chronic illness.

Range and consistency of outcome measures reported in randomised trials in dermatomyositis: a systematic review

A.H. Kelly[1-3], D. Singh-Grewal[1,2], D. Sumpton[2,4,5], G. Hasset[6,7], K.E. Manera[2,4], A. Tong[2,4]

[1]Department of Paediatric Rheumatology, The Children's Hospital Network, Sydney, NSW; [2]Sydney School of Public Health, The University of Sydney, NSW; [3]Department of Medicine, Campbelltown Hospital, Sydney, NSW; [4]Centre for Kidney Research, The Children's Hospital at Westmead, Sydney, NSW; [5]Rheumatology Department, Concord Repatriation General Hospital, Concord, NSW; [6]Department of Rheumatology, Liverpool Hospital, Liverpool, NSW; [7]South Western Sydney Clinical School, University of New South Wales, Sydney, NSW, Australia.

Abstract

Objective

Dermatomyositis (DM) and juvenile dermatomyositis (JDM) are idiopathic inflammatory myopathies, which can be resistant and unresponsive to initial treatments, leading to severe complications and impaired quality of life. There are few randomised trials in dermatomyositis and the outcomes reported may not be consistent, which can limit decision-making. The aim of this study is to assess the scope and consistency of outcomes reported in randomised trials in dermatomyositis.

Methods

MEDLINE, Embase, PsycINFO and clinicaltrials.gov were searched from 1993-2020 for randomised trials in children and adults with dermatomyositis. The frequency and characteristics of the outcomes reported were analysed and classified.

Results

20 trials were included. Across these trials, a total of 743 outcome measures were reported, which were grouped into 34 outcome domains; of which 17 were clinical, 13 were surrogate/biochemical, and 4 were patient-reported outcomes. The top five most frequently reported outcome domains were muscle inflammation (15 trials, 46 outcome measures), physical function (14 trials, 16 outcome measures), muscle strength (13 trials, 30 outcome measures), global health (12 trials, 33 outcome measures) and immunologic marker (11 trials, 91 outcomes).

Conclusion

The majority of outcomes reported in trials in people with dermatomyositis and JDM are clinical and surrogate outcomes rather than patient-reported outcomes. The outcomes reported are very inconsistent across trials, with wide heterogeneity in the measures used. Standardised reporting of critically important outcomes is needed to strengthen the value of trials for decision-making.

Key words

dermatomyositis, juvenile dermatomyositis, outcome measures, outcome domains

Amy Helen Kelly, MD, FRACP
Davinder Singh-Grewal, MD, PhD, FRACP
Daniel Sumpton, MD, PhD, FRACP
Geraldine Hasset, MD, PhD, FRACP
Karine E. Manera, PhD
Allison Tong, PhD

Please address correspondence to:
Amy H. Kelly,
Department of Medicine,
Campbelltown Hospital,
Therry Road,
Campbelltown,
NSW 2560, Australia.
E-mail: amy.kelly@health.nsw.gov.au

Received on July 15, 2021; accepted in revised form on January 19, 2022.

Competing interests: none declared.

Introduction

Dermatomyositis (DM) and juvenile dermatomyositis (JDM) are rare connective tissue diseases that make up the majority of the idiopathic inflammatory myopathies (1). They can present with a range of symptoms including skin rash, poikiloderma, muscle weakness and elevated muscle enzymes (1). Both DM and JDM may also cause significant end-organ damage, which can be life-threatening. DM has also been associated with depression, anxiety and fatigue, with patients reporting worse quality of life compared to healthy individuals (2). Despite a lack of evidence for their use and significant side effects, glucocorticosteroids remain the mainstay of first line treatments (3). Given the proportion of patients whose disease remains refractory to steroid treatment, new, targeted treatments are being proposed to treat these diseases (3).

There are few randomised control trials for interventions for DM and JDM. Of the trials that exist, outcome measures reported often have limited relevance to patients and their caregivers, with few patient-reported outcome measures (PROMs) used (4). With the development of new treatments there is a critical need for consistent reporting of relevant outcome measures to ensure comparability across studies and improve the interpretation of the evidence base for interventions (5). Efforts have been made by the International Myositis Assessment and Clinical Studies Group (IMACS) and Paediatric Rheumatology International Trials Organisation (PRINTO) in 2011 to standardise reporting of outcome measures in DM and JDM (6-9). However, there has not been detailed assessment of the outcomes and measures reported in trials in dermatomyositis and JDM. OMERACT clearly stipulates the development of outcome measure sets should start with a review of existing measures used in the literature (10).

The aim of this study was to review, determine the scope and consistency of outcome measures reported in randomised trials for dermatomyositis and juvenile dermatomyositis, to inform strategies for further development of outcome measures that are important to patients, caregivers, clinicians and policy makers (11).

Methods

Search and study selection

We searched MEDLINE, Embase, PsycINFO and clinicaltrials.gov up to 30th May 2020 for randomised trials in children and adults with dermatomyositis/juvenile dermatomyositis (see Supplementary index). Citations were limited to those in the English language and had to include more than 50% participants with a diagnosis of DM and/or JDM in the intervention group (Fig. 1). We used searched terms related to dermatomyositis and juvenile dermatomyositis (Suppl. Index). Where applicable, PRISMA guidelines (2009) were followed within the scope of a systematic review providing a descriptive summary of the type of outcomes reported in trials (12). Co-authors (AT and DS) verified the search strategies and search results.

Data extraction

We extracted the following characteristics from each trial: year published, participating countries, study duration, intervention type, number of participants, number of male/female participants and all outcomes reported. Outcome measures were defined as any measures reported separately from any trial arm (13). We extracted details of the outcome measures including specific metric and time point of measurement (the time frame from trial commencement to when the outcome was measured) (13).

Analysis

All outcomes were extracted by the first author (AHK) and classified into domains. The extracted outcome measures and domain classifications were checked by a second reviewer (DS). A third reviewer, (AT) further checked the domain classifications until consensus was reached. The outcome measures identified as core set measures were classified according to published domain names by IMACS/PRINTO wherever possible (4). Appropriate and descriptive domain classifications were developed where there was no existing

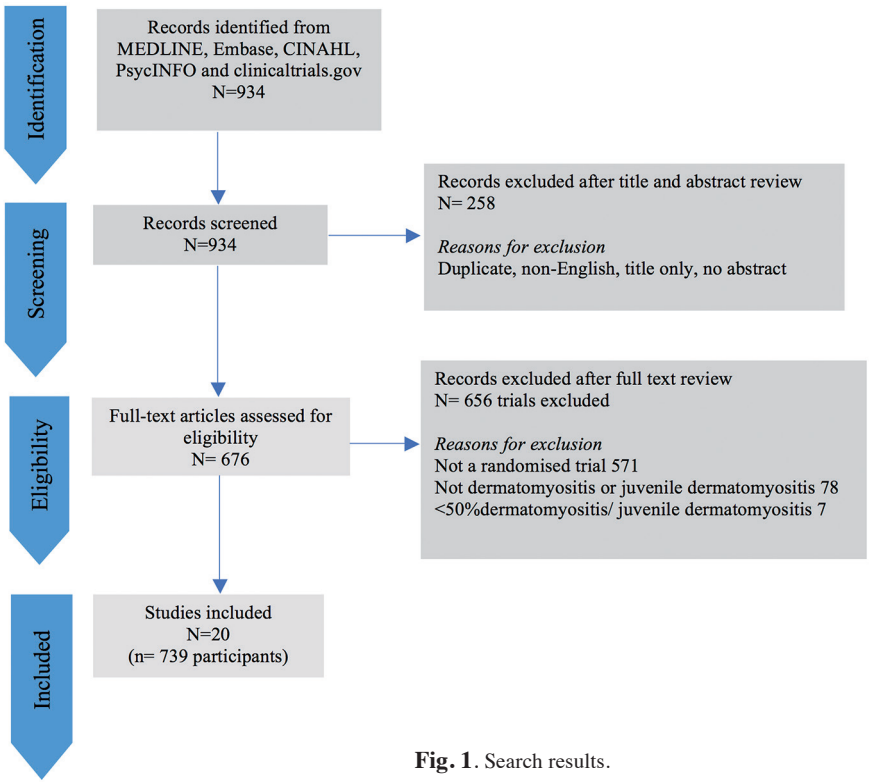


Fig. 1. Search results.

domain name for the outcome measures reported (Suppl. index).

All outcome domains were further classified into surrogate (*e.g.* biochemical markers, imaging or measures used as a substitute for a clinical outcome) (14), clinical (composite scores that included clinical evaluation, or medical outcome of a treatment or disease) and patient-reported (outcome measures reported by patients or caregivers; including quality of life or symptoms) (15). The number of trials that reported each outcome domain was then recorded.

We then conducted a detailed analysis of the muscle strength domain and deconstructed the components of the composite scores that were classified within the global health domain. For the purpose of this review, Visual Analogue Scales (VAS) were considered as their own individual composite score comprising of one component.

Results

Trial characteristics

From 934 citations (Fig. 1), we included 20 randomised control trials published between the years 1993 and 2019 (Table I). Three trials included children. Thirteen (65%) trials were of pharmacological interventions. The number of participants ranged from 14 to 200, with the majority of participants being female. The trial duration ranged from 4 to 130 weeks. The number of outcome measures reported by each trial ranged from 3 to 49.

Outcome domains

There were a total of 743 different outcome measures reported across all 20 trials. These were grouped into 34 outcome domains which were identified and classified into clinical (17 outcomes), surrogate (13 outcomes) and patient-reported outcome domains (4 outcomes) (Fig. 2). The top ten most frequently reported outcomes domains were muscle inflammation (15 trials), physical function (14 trials), muscle strength (13 trials), global health (12 trials), immunologic markers (11 trials), haematologic (9 trials), cardiovascular (8 trials), inflammation (5 trials), adverse event not specified (5 trials), skin (5 trials) and treatment efficacy (5 trials). The number of times an outcome domain was reported across all the trials ranged from 1–90. The outcome measures used for each of the top five outcome domains are described in the following.

Outcome measures

– Muscle inflammation

Within the muscle inflammation domain there was a total of 11 different measures reported across 15 trials. The majority of measures (n=7) were reported without units of measurement. The timepoints measurements were made at ranged from 1 to 96 weeks. The most commonly reported outcome was creatinine kinase, further examples of measures in this domain include AST, muscle MRI (T2 STIR) signal and other novel markers of muscle inflammation as described within a specific trial.

– Physical function

Within the physical function domain there were 9 different measures reported across 14 trials, over timepoints ranging from 1 to 130 weeks. The most frequently reported outcome measure was the Health Assessment Questionnaire (HAQ) which was reported across six trials. The next most reported measure was Activity of Daily Living scale, reported across three trials. Five of the 14 trials reported measures that were not reported in any other trials, these measures included; the McMaster Toronto Arthritis Patient preference disability questionnaire, the Physical Activity Enjoyment scale, Convery Assessment scale, the CMAS and the 6 minutes walking distance (6-MWD) test.

– Global health

Within the Global Health Domain there were 15 composite scores identified, with 75 individual components of all the composites (Fig. 4). Physician global activity was the most frequently reported composite score across the trials (n=7). There were 42 different components that were reported in only one trial. The five most frequent components used in the composite measures included physician-assessed Visual Analogue Scales (VAS) (7 trials), patient-reported VAS (6 trials), CPK (6 trials), myositis treatment (5 trials) and other muscle enzymes (5 trials). For the purpose of this review Visual Analogue Scales were considered as their own individual composite score comprising of one component.

Table I. Characteristics of included trials (n=20).

Trial	ID	Country	Disease	Females	Males	Number of participants	Trial duration	Intervention	Comparator	Outcome	Number of outcome measures reported
1	Amato 2011 (25)	USA	DM	10	6	16	52 weeks	Etanercept	placebo (prednisone)	no major safety concerns, evidence of a steroid sparing effect.	22
2	Munters 2013 (26)	Sweden	DM and PM	16	5	21	12 weeks	exercise	No exercise control	endurance exercise maybe beneficial	10
3	Munters 2013 (27)	Sweden	DM and PM	15	2	17	12 weeks	exercise	No exercise control	endurance exercise maybe beneficial	14
4	Alexanderson 2014 (28)	Sweden	DM and PM	14	5	19	24 weeks	exercise	exercise	safety of resistive exercise, but no difference between goups	6
5	Chung 2007 (29)	United Kingdom/Sweden	DM and PM	31	6	37	24 weeks	oral creatine	placebo	benefit	15
6	Dalakas 1993 (30)	USA	DM	N/A	N/A	15	12 weeks	IVIg	placebo	benefit	13
7	Guo 2014 (31)	USA	DM and PM	N/A	N/A	48	49 weeks	Sifalimumab	placebo	benefit	24
8	Habers 2016 (32)	Netherlands	JDM	N/A	N/A	26	36 weeks	exercise	no exercise (waiting)	benefit	25
9	Ito/Ibi 2011 (33)	Japan	DM/mitochondrial myopathy	N/A	N/A	22	8 weeks	hydrogen enriched water	placebo	benefit	17
10	Miller 1992 (34)	USA	PM and DM	28	11	39	4 weeks	plasma exchange and leukapheresis	placebo	no benefit	9
11	Miyasaka 2012 (35)	Japan	DM and PM	20	6	26	8 weeks	IVIg	placebo	no benefit	3
12	Oddis 2013 (36)	USA	DM, PM and	146	54	200	8 weeks	Rituximab	placebo	benefit	11
13	Ruperto 2016 (37)	Europe	JDM	82	47	129	96 weeks	Prednisone and cyclosporin or prednisone and methotrexate	prednisone	benefit	19
14	Solis 2016 (38)	Brazil	JDM	10	5	15	20 weeks	Creatine	placebo	no benefit	35
15	Vencoskv 2000 (39)	Czech Republic	PM and DM	23	13	36	12 weeks	Cyclosporine and prednisone	methotrexate and prednisone	benefit	10
16	Wiesinger 1998 (40)	Austria	PM and DM	9	5	14	6 weeks	exercise	control	benefit	4
17	Higgs 2014 (41)	USA	PM and DM	29	10	39	24.5 weeks	Sifalimumab	placebo	possible benefit	32
18	Tjarnlund 2015 (42)	Europe and USA	PM and DM	13	7	20	24 weeks	Abatacept	placebo (delayed start)	possible benefit	22
19	Idera Pharmaceuticals 2019 (43)	United States, Hungary and United Kingdom	DM	7	23	30	28 weeks	IMO-8400	placebo	N/A	41
20	Novartis Pharmaceuticals 2019 (44)	United States, Czech Republic and Japan	DM and PM	4	13	17	130 weeks	BAF 312	placebo/ BAF312	no safety concern	23

– Muscle strength

Within this domain a total of 19 outcomes were reported. Manual Muscle testing-8 (MMT-8) was the most commonly reported outcome reported in 5 trials, followed by MMT (Medical Research Council extended 0–15 scale) in 3 trials and MMT-8 (0–80 scale) in 2 trials (Fig. 3). The remaining 16 outcome measures were all only reported in one trial each. The timepoints at which each outcome was measured ranged from 3–27 weeks. The majority of outcomes (n=11) were measured at 12 weeks.

– Immunologic markers

Outcome measures classified into the immunologic domain included surrogate biomarkers such as ANA, ENA and other autoantibodies. Within this domain, there were 69 different measures reported across 11 trials. Immunologic measures were by far the most frequently reported when compared to other surrogate markers, including haematologic (n=9), other measures of inflammation (n=7) and metabolic (n=3).

Discussion

Even within the small number of randomised trials in children and adults with DM, the outcomes reported are varied and inconsistent, with a range of different measures used to report the same outcome. Across the 20 trials, there were 34 outcome domains, consisting of 743 different outcome measures. The majority of outcome domains

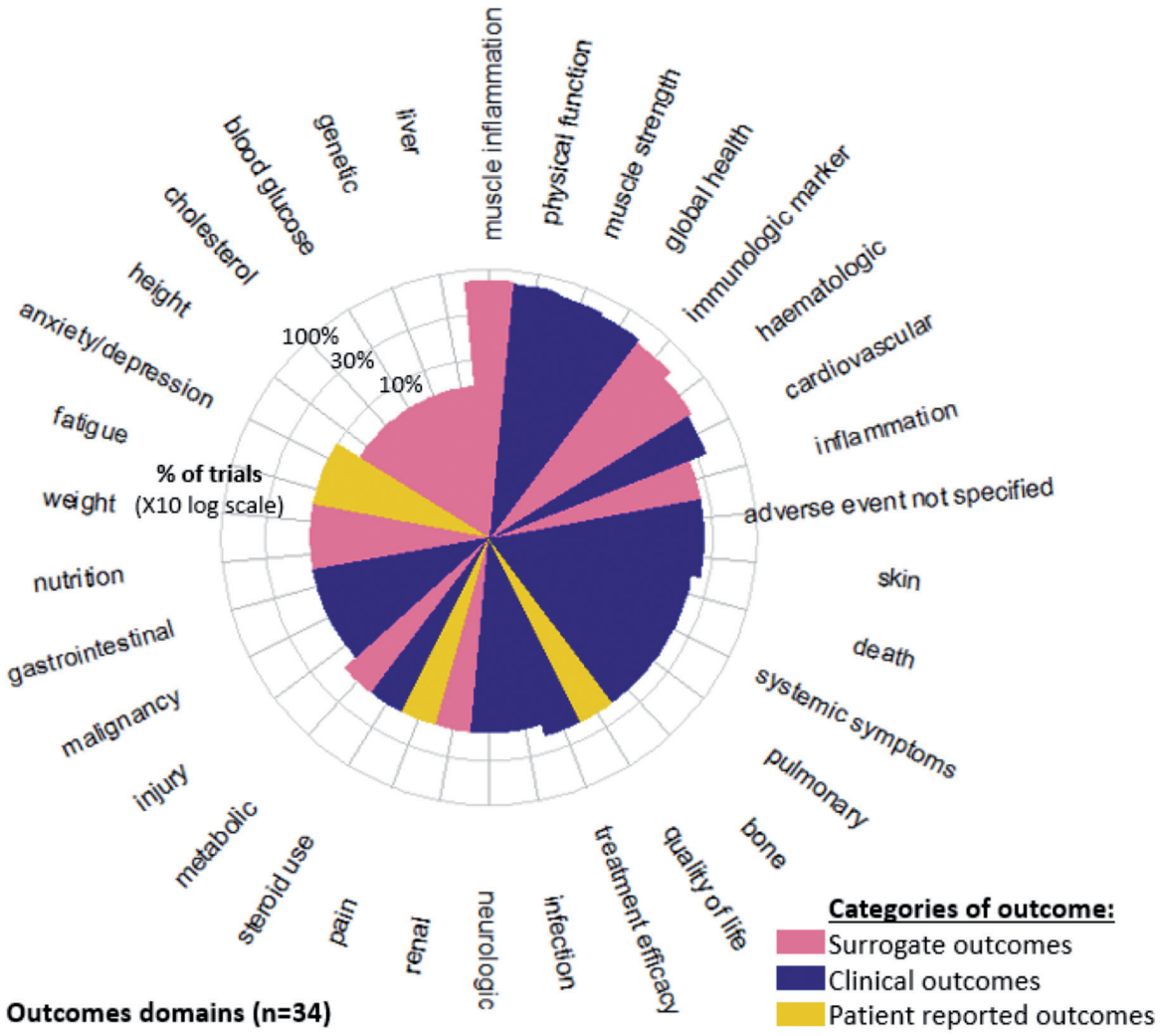


Fig. 2. Number of trials reporting each outcome domain (total 20 trials) (total 34 outcome domains).

were surrogate and clinical endpoints. Only four outcome domains (pain, anxiety/depression, fatigue and quality of life), included patient-reported outcome measures. The top five most frequently reported outcome domains were muscle inflammation (16 trials, 46 outcome measures), physical function (13 trials,16 outcome measures), muscle strength (13 trials, 30 outcome measures), global health (12 trials, 33 outcome measures) and immunologic markers (11 trials, 91 outcomes).

The global health domain (Fig. 4) included 15 composite scores where a number of these scores reported the same components. These overlapping components detail specific disease manifestations, which to complete, require a high degree of experience, clinical knowledge and are vulnerable to different interpretation between investigators (4). They may also be time consuming to complete. For example, the MITAX (a measure of the physicians intention to treat) and MYOACT (a measure of disease activity within the last 4 weeks) are two overlapping tools, differing by only one component (16) (Fig. 4). Both include information relating to the extent of involvement in the constitutional, articular, cardiac, pulmonary, gastrointestinal, cutaneous and skeletal muscle organ/systems (16), totalling 29 and 30 components respectively. The utility of the clinical meaning of a composite score that differs by one component, we would argue, is limited. Complex composite scores have been criticised as potentiating the misinterpretation of the magnitude of the effect of an intervention (17). Complex composite scores may also be difficult to utilise in the busy clinical setting.

Improvements in consistency of outcomes reported by adopting core outcome sets has been demonstrated in rheumatoid arthritis, psoriatic arthritis and ankylosing spondylitis (14). However, IMACS and PRINTO have developed core outcome measures that include clinical and surrogate measures (4) and there are currently only two recommended and defined as patient-reported outcome measures, Short Form36 (SF36) for adult patients with IIM and the Child Health Questionnaire–Parent Form 50 (CHQ–PF50) for patients with juvenile dermatomyositis (4). Neither of these have been validated in patients with IIMS (4) and measure patient reported physical function only. We identified only four outcome

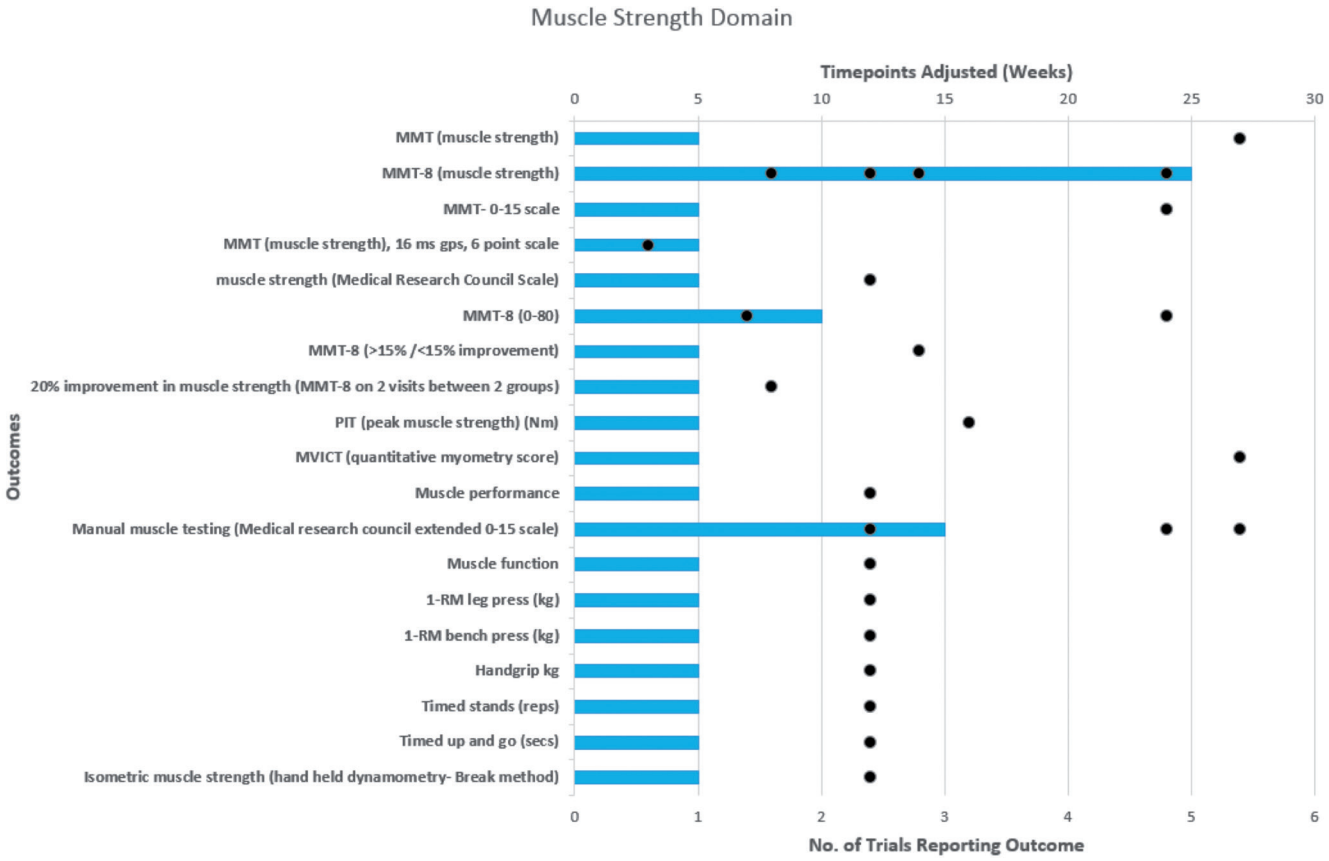


Fig. 3. Number of outcome measures in muscle strength domain, their timepoints and the number of trials reporting that outcome, noting the number of muscle groups or scale reported.

domains (Fig. 2) that described patient reported outcome measures, illustrating that there has been limited inclusion of patient reported outcome measures within dermatomyositis and juvenile dermatomyositis trials to date.

There is strong evidence to support the inclusion of the patient's perspective in determining disease activity (18). Tory *et al*. (18) found patient reported outcome measures in JDM were associated with greater discordance between the patient's perception of their disease and their treating physician, concluding that patients/families may place a greater emphasis on patient reported outcomes (18). In adult DM, poorer quality of life scores are associated with worse muscle strength (18). Patient-reported outcomes are vital to inform physicians assessments of disease activity, as the patient experience is recognised as central to achieving high quality, high value care (19). Without including patient reported outcomes, trials are potentially missing a vital component of the patient experience of their disease.

To better capture the patient's perspective, the OMERACT Myositis Special Interest Group identified five themes as being essential to include in myositis-specific PROMs; symptoms, activity/participation, strategies, knowledge of disease, self-management and emotional factors (4). Outcome measures that reflect how patients feel or function were underreported in the twenty trials we identified, with only four trials reporting quality of life; three trials reporting pain, two trials reporting fatigue, and two reporting anxiety/depression. The development of validated PROMs, inclusive of the patient's perspective (symptoms, activity/participation, strategies, knowledge of disease, self-management and emotional factors) in dermatomyositis and JDM is urgent and the paucity of PROMs in this review demonstrates that the choice of outcomes reported has not always been those that are most relevant to patients (13). Patient reported outcome measures have demonstrated similar reliability in trials compared to other surrogate measures, such as diastolic blood pressure and blood glucose levels in (20).

One unpublished, qualitative study found that caregivers value knowledge of surrogate measures of muscle inflammation, as they provided an easy measure for them to understand their own child's response to treatment (21). Creatinine kinase can be used as a measure of disease activity and damage assessment and is included in the IMACS core set measures for DM and JDM (4). However, in our study, there were 11 different measures of muscle inflammation reported across 16 trials. The effectiveness of surrogate markers is lost where too many are used, or their relationship to response to the intervention is obscure (11).

Randomised clinical trials should report consensus determined outcome measures to better gauge the impact of treatment interventions (6). With the development of new therapeutic interventions, trials will need to report replicable, meaningful outcome measures so that interventions can be

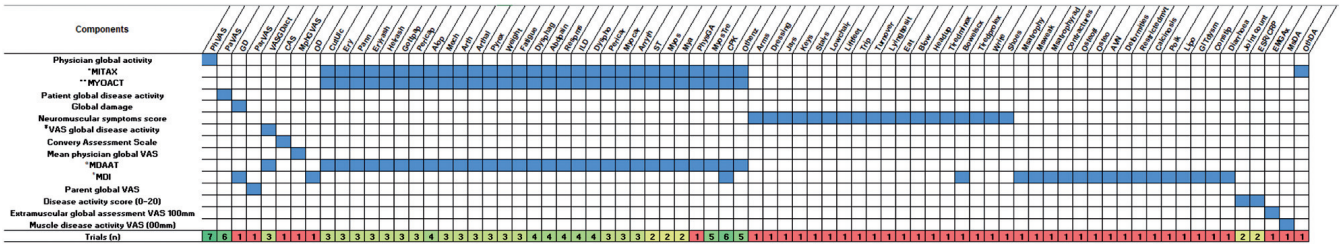


Fig. 4. Composite matrix of Global Health Domain measures showing breakdown of composite scores (far left column) into their components (top row, see Supplementary index). The bottom row represents the number of trials (n) that report each component.
*Myositis Intention to Treat Activity Index (MITAX);
**Myositis Disease Activity Assessment Visual Analogue Scales (MYOACT);
ɣVisual Analogue Scale (VAS);
αMyositis Disease Activity Assessment Score (MDAAT);
Myositis Damage Index (MDI).

compared across trials (11). The ability for clinicians to apply research findings to everyday practice is limited if outcome measures reported are varied, inconsistent(15) or lacking important clinical information. We note that skin is an important element (often), in the presentation of DM and JDM, however, outcome measures reporting skin disease, such as the Cutaneous Dermatomyositis Disease Area and Severity Index (CDASI), a validated tool to assess cutaneous manifestations in DM (22), were not universally reported across all trials (Fig. 2). A validated skin outcome measure should be considered as an important outcome measure to be reported across DM/JDM trials.

Our study showed that historically there is great variation and inconsistency in how outcomes are reported across DM and JDM. At every level, including the domain, outcome reported, timepoint and metric of measurement there was a lack of uniformity in reporting. Within the physical function domain, for example, 5 of the 14 trials reported measures that were not reported in any other trial. Previous studies in nephrology and cardiovascular disease have reported similar problems (15). Even amongst similar outcome measures, the way in which they are measured and reported varies across trials. The muscle strength domain (Fig. 3) reported 19 different measures, all measuring muscle strength, with varying groups of muscles being tested, using different scales (Suppl. Table S3). The timepoints over which these outcomes were measured varied from 5 weeks to 27 weeks. Manual Muscle Testing is purported to be a surrogate of muscle function (23) (and recently proposed as a validated, core outcome measure, by IMACS/PRINTO) (4) is one example where there were nine different metrics reported across the trials that we identified. It has been reported in other diseases that reporting inconsistent outcome measures can result in reporting bias(15, 24), whereby trialists selectively report outcomes that show an effect. We acknowledge that efforts have been made to improve the reporting of these measures. However, in the future it will be vital for trials to include standardised, validated measures.

Our findings provide systematic and detailed evidence of the inconsistencies in the reporting of outcome measures. However, there are some potential limitations. Only 20 randomised trials were included reporting predominantly on drug interventions. Inevitably because of the rarity of DM and JDM, trials may include other inflammatory myopathies which may necessitate reporting additional outcome measures. We acknowledge that there may be differences in the outcomes reported in early-stage trials, where surrogate markers may be preferentially reported. However, we decided it was not possible to exclude these trials given the small number of trials identified. Being limited to only 20 trials, we were not able to apply a meaningful, in-depth analysis of the uptake of the published core outcome measures sets. Our review was also limited to including trials that reported in English and all the trials identified were from high income countries, which may imply publication bias.

There is wide heterogeneity and lack of consistency in the reporting of outcome measures across trials in DM and JDM. The findings highlight the need to revise and implement core measures set and draws attention to improving the use of patient-reported outcome measures. Rare diseases such as DM and JDM with already few randomised trials in the literature, offer an opportunity to develop cohesive, uniform and most importantly patient relevant outcome measures that can be reported across all future trials and ultimately improve patient outcomes.

References

1. STROWD LC, JORIZZO JL: Review of dermatomyositis: Establishing the diagnosis and treatment algorithm. *J Dermatol Treat* 2013; 24: 418-21.
2. POULSEN KB, ALEXANDERSON H, DALGARD C, JACOBSEN S, WEILE L, DIEDERICHSEN LP: Quality of life correlates with muscle strength in patients with dermato- or polymyositis. *Clin Rheumatol* 2017; 36: 2289-95.
3. CHEN KL, ZEIDI M, WERTH VP: Recent advances in pharmacological treatments of adult dermatomyositis. *Curr Rheumatol Rep* 2019; 21: 53.
4. RIDER LG, AGGARWAL R, MACHADO PM *et al*.: Update on outcome assessment in myositis. *Nat Rev Rheumatol* 2018; 14: 303-18.
5. TUNIS SR, MAXWELL LJ, GRAHAM ID *et al*.: Engaging stakeholders and promoting uptake of omeract core outcome instrument sets. *J Rheumatol* 2017; 44: 1551-9.
6. BENVENISTE O, RIDER LG: 213th ENMC International Workshop: Outcome measures and clinical trial readiness in idiopathic inflammatory myopathies, Heemskerk, The Netherlands, 18-20 September 2015. *Neuromuscul Disord* 2016; 26: 523-34.
7. REGARDT M, MECOLI CA, PARK JK *et al*.: Omeract 2018 modified patient-reported outcome domain core set in the life impact area for adult idiopathic inflammatory myopathies. *J Rheumatol* 2019; 46: 1351-4.

8. MECOLI CA, PARK JK, ALEXANDERSON H *et al*.: Perceptions of patients, caregivers, and healthcare providers of idiopathic inflammatory myopathies: An international OMERACT study. *J Rheumatol* 2019; 46: 106-11.
9. RIDER LG, WERTH VP, HUBER AM *et al*.: Measures of adult and juvenile dermatomyositis, polymyositis, and inclusion body myositis: Physician and patient/parent global activity, manual muscle testing (MMT), health assessment questionnaire (HAQ)/ childhood health assessment questionnaire (C-HAQ), childhood myositis assessment scale (CMAS), myositis disease activity assessment tool (MDAAT), disease activity score (DAS), short form 36 (sf-36), child health questionnaire (CHQ), physician global damage, myositis damage index (MDI), quantitative muscle testing (QMT), myositis functional index-2 (FI-2), myositis activities profile (MAP), inclusion body myositis functional rating scale (IBMFRS), cutaneous dermatomyositis disease area and severity index (CDASI), cutaneous assessment tool (CAT), dermatomyositis skin severity index (DSSI), skindex, and dermatology life quality index (DLQI). *Arthritis Care Res* 2011; 63 (Suppl. 11): S118-57.
10. BOERS M, KIRWAN JR, TUGWELL P *et al*.: The OMERACT handbook. *OMERACT* 2017.
11. CHONG LSH, SAUTENET B, TONG A *et al*.: Range and heterogeneity of outcomes in randomized trials of pediatric chronic kidney disease. *J Pediatr* 2017; 186: 110-7.e11.
12. LIBERATI A, ALTMAN DG, TETZLAFF J *et al*.: The prisma statement for reporting systematic reviews and meta-analyses of studies that evaluate healthcare interventions: Explanation and elaboration. *BMJ* 2009; 339: b2700.
13. WILLIAMSON PR, ALTMAN DG, BAGLEY H *et al*.: The COMET handbook: Version 1.0. Trials 2017; 18: 280.
14. SUMPTON D, BIGOT A, SAUTENET B *et al*.: The scope and consistency of outcomes reported in trials in patients with systemic sclerosis. *Arthritis Care Res* 2020; 72: 1449-58.
15. SAUTENET B, TONG A, WILLIAMS G *et al*.:Scope and consistency of outcomes reported in randomized trials conducted in adults receiving hemodialysis: A systematic review. *Am J Kidney Dis* 2018; 72: 62-74.
16. ISENBERG DA, ALLEN E, FAREWELL V *et al*.: International consensus outcome measures for patients with idiopathic inflammatory myopathies. Development and initial validation of myositis activity and damage indices in patients with adult onset disease. *Rheumatology* 2004; 43: 49-54.
17. O'LONE E, VIECELLI AK, CRAIG JC *et al*.: Cardiovascular outcomes reported in hemodialysis trials. *J Am Coll Cardiol* 2018; 71: 2802-10.
18. TORY H, ZURAKOWSKI D, KIM S: Patient and physician discordance of global disease assessment in juvenile dermatomyositis: Findings from the Childhood Arthritis & Rheumatology Research Alliance Legacy Registry. *Pediatr Rheumatol Online J* 2020; 18: 5.
19. BARTON JL, KATZ P: The patient experience: Patient-reported outcomes in rheumatology. *Rheum Dis Clin North Am* 2016; 42: xv-xvi.
20. BLACK N: Patient reported outcome measures could help transform healthcare. *BMJ* 2013; 346: f167.
21. KELLY AH, CHAITOW J, SINGH-GREWAL D, TONG A: Perceptions of caregivers of children with juvenile dermatomyositis. Unpublished 2020.
22. TIAO J, FENG R, BIRD S *et al*.: The reliability of the cutaneous dermatomyositis disease area and severity index (cdasi) among dermatologists, rheumatologists and neurologists. *Br J Dermatol* 2017; 176: 423-30.
23. ZANFRAMUNDO G, TRIPOLI A, COMETI L *et al*.: One year in review 2020: Idiopathic inflammatory myopathies. *Clin Exp Rheumatol* 2021; 39: 1-12.
24. DWAN K, GAMBLE C, WILLIAMSON PR, KIRKHAM JJ: Systematic review of the empirical evidence of study publication bias and outcome reporting bias - an updated review. *PLoS One* 2013; 8: e66844.
25. AMATO AA, TAWIL R, KISSEL J *et al*.: A randomized, pilot trial of etanercept in dermatomyositis. *Ann Neurol* 2011; 70: 427-36.
26. ALEMO MUNTERS L, DASTMALCHI M, ANDGREN V *et al*.: Improvement in health and possible reduction in disease activity using endurance exercise in patients with established polymyositis and dermatomyositis: a multicenter randomized controlled trial with a 1-year open extension followup. *Arthritis Care Res* 2013; 65: 1959-68.
27. ALEMO MUNTERS L, DASTMALCHI M, KATZ A *et al*.: Improved exercise performance and increased aerobic capacity after endurance training of patients with stable polymyositis and dermatomyositis. *Arthritis Res Ther* 2013; 15: R83.
28. ALEXANDERSON H, ALEMO MUNTERS L, DASTMALCHI M *et al*.: Resistive home exercise in patients with recent-onset polymyositis and dermatomyositis - a randomized controlled single-blinded study with a 2-year followup. *J Rheumatol* 2014; 41: 1124-32.
29. CHUNG YL, ALEXANDERSON H, PIPITONE N *et al*.: Creatine supplements in patients with idiopathic inflammatory myopathies who are clinically weak after conventional pharmacologic treatment: Six-month, double-blind, randomized, placebo-controlled trial. *Arthritis Care Res* 2007; 57: 694-702.
30. DALAKAS MC, ILLA I, DAMBROSIA JM *et al*.: A controlled trial of high-dose intravenous immune globulin infusions as treatment for dermatomyositis. *N Engl J Med* 1993; 329: 1993-2000.
31. GUO X, HIGGS BW, REBELATTO M *et al*.: Suppression of soluble t cell-associated proteins by an anti-interferon-alpha monoclonal antibody in adult patients with dermatomyositis or polymyositis. *Rheumatology* 2014; 53: 686-95.
32. HABERS GEG, JOYCE BOS GJF, VAN ROYEN-KERKHOF A *et al*.: Muscles in motion: A randomized controlled trial on the feasibility, safety and efficacy of an exercise training programme in children and adolescents with juvenile dermatomyositis. *Rheumatology* 2016; 55: 1251-62.
33. ITO M, IBI T, SAHASHI K, ICHIHARA M, ITO M, OHNO K: Open-label trial and randomized, double-blind, placebo-controlled, crossover trial of hydrogen-enriched water for mitochondrial and inflammatory myopathies. *Med Gas Res* 2011;1: 24.
34. MILLER FW, LEITMAN SF, CRONIN ME *et al*.: Controlled trial of plasma exchange and leukapheresis in polymyositis and dermatomyositis. *N Engl J Med* 1992; 326: 1380-4.
35. MIYASAKA N, HARA M, KOIKE T, SAITO E, YAMADA M, TANAKA Y: Effects of intravenous immunoglobulin therapy in japanese patients with polymyositis and dermatomyositis resistant to corticosteroids: a randomized double-blind placebo-controlled trial. *Mod Rheumatol* 2012; 22: 382-93.
36. ODDIS CV, REED AM, AGGARWAL R *et al*.: Rituximab in the treatment of refractory adult and juvenile dermatomyositis and adult polymyositis: A randomized, placebo-phase trial. *Arthritis Rheum* 2013; 65: 314-24.
37. RUPERTO N, PISTORIO A, OLIVEIRA S *et al*.: Prednisone versus prednisone plus ciclosporin versus prednisone plus methotrexate in new-onset juvenile dermatomyositis: a randomised trial. *Lancet* 2016; 387: 671-8.
38. SOLIS MY, HAYASHI AP, ARTIOLI GG *et al*.: Efficacy and safety of creatine supplementation in juvenile dermatomyositis: A randomized, double-blind, placebo-controlled crossover trial. *Muscle Nerve* 2016; 53: 58-66.
39. VENCOVSKY J, JAROSOVA K, MACHACEK S *et al*.: Cyclosporine a versus methotrexate in the treatment of polymyositis and dermatomyositis. *Scand J Rheumatol* 2000; 29: 95-102.
40. WIESINGER GF, QUITTAN M, ARINGER M *et al*.: Improvement of physical fitness and muscle strength in polymyositis/dermatomyositis patients by a training programme. *Br J Rheumatol* 1998; 37: 196-200.
41. HIGGS BW, ZHU W, MOREHOUSE C *et al*.: A phase 1b clinical trial evaluating sifalimumab, an anti-ifn-alpha monoclonal antibody, shows target neutralisation of a type i ifn signature in blood of dermatomyositis and polymyositis patients. *Ann Rheum Dis* 2014; 73: 256-62.
42. TJARNLUND A, DASTMALCHI M, MANN H *et al*.: Abatacept in the treatment of adult dermatomyositis and polymyositis: Artemis, a randomized, treatment delayed-start trial. *Ann Rheum Dis* 2015; 74: 817-8.
43. PHARMACEUTICALS I: Trial of imo-8400 in adult patients with dermatomyositis. Unpublished 2019.
44. PHARMACEUTICALS N: A double blind, randomized, placebo-controlled study to evaluate, safety, tolerability, efficacy and preliminary dose-response of BAF312 in patients with active dermatomyositis (DM). Unpublished 2019.

Reflections on Chapter 1

The purpose of this systematic review was to establish the outcome measures used in existing myositis research, specifically randomised control trials and categorise them to develop an understanding of the distribution of and uptake of outcome measure groups including, surrogate and patient reported outcome measures. It was noteworthy that although OMERACT(10) recommends that a review of existing measure should occur before a universal set of outcome measures are developed, at the time of this study this had not occurred in myositis research. The process of reviewing the literature and identifying outcome measures provided a foundation of knowledge, not just of outcome measures used but also more specifically knowledge about juvenile dermatomyositis and the treatments used to manage the disease. This knowledge subsequently informed the qualitative interview questions that were then directed to research participants in papers 2 and 3.

It was a time-consuming process to identify the studies to be included in the work and then to systematically identify the outcome measures reported in those studies. Due to the very limited number of randomised controlled trials in myositis there were relatively few papers included. The process did certainly highlight the paucity of research in myositis and also more broadly how clinical rheumatology is often forced to rely upon treatments where there is little or no evidence or evidence is sometimes extrapolated from other rheumatic conditions. The lack of historical research into dermatomyositis highlights how difficult it is to recruit patients or caregivers who have experienced rare diseases and this became an emerging theme throughout the development of this thesis and would impact later papers.

RESEARCH ARTICLE Open Access

Perspectives and experiences of parents of children with juvenile dermatomyositis: a semi-structured interview study

Amy Helen Kelly[1,6*], Ayano Kelly[2,3,4], Davinder Singh-Grewal[5], Jeffrey Chaitow[5] and Allison Jaure[6,7]

Abstract

Background Juvenile Dermatomyositis (JDM) is a rare, childhood inflammatory disease and its management can be challenging and confronting for both clinicians and caregivers. Little is known about the perspectives of parental caregivers of children with JDM. This study aimed to describe the experiences of parents of children with JDM to inform person-centred care.

Methods Semi-structured interviews (face-to-face, telephone) were conducted with parents of children with JDM from three centres in Australia. Transcripts were analysed thematically.

Results Nineteen parents (15 mothers) of 17 children aged 8 to 21 with JDM participated. Six themes were identified. Rapid crescendo of fear and desperation (alarming deterioration, sudden realisation of seriousness, desperate for a diagnosis), lost and unsupported in the health system (at the mercy of the medical team, frustrated at the lack of services, neglected priorities, protracted and painful search for answers), disrupting family routines (sibling neglect and loss, overloaded with a medicalised schedule, always on standby, burdened by financial strains), grieving what has been lost (missing the sunlight, struggling with the loss of physical function, disrupted schooling, changes in their child from steroid side effects), managing an uncertain future (bound to chronicity, fearing relapse, insecurity with transition to adult care), gaining confidence and motivation (strengthening partnerships with clinicians, growing maturity and independence, gaining hope from shared experiences).

Conclusions The diagnosis of JDM is often delayed and caregivers of children with JDM report distress, disruption and uncertainty throughout their treatment journey with their child. Addressing these fears and establishing support mechanisms that help parents navigate their way through the medical system and support changing family dynamics are vital to optimise health outcomes for children diagnosed with JDM.

Keywords Juvenile dermatomyositis, Qualitative methods, Interviews, Patient-centred care, Caregivers

*Correspondence:
Amy Helen Kelly
Amy.Kelly@health.nsw.gov.au
[1]Department of Rheumatology, Campbelltown Hospital, Campbelltown, NSW, Australia
[2]School of Clinical Medicine, University of New South Wales, South West Sydney Clinical Campuses, Liverpool, NSW, Australia
[3]Department of Rheumatology, Liverpool Hospital, Liverpool, NSW, Australia
[4]Ingham Institute of Medical Research, Liverpool, NSW, Australia
[5]Department of Paediatric Rheumatology, Westmead Children's Hospital, Westmead, NSW, Australia
[6]Sydney School of Public Health, The University of Sydney, Sydney, NSW, Australia
[7]Centre for Kidney Research, Westmead Children's Hospital, Westmead, NSW, Australia

Introduction

Juvenile Dermatomyositis (JDM) is a rare, chronic, childhood autoimmune disease, characterised by inflammation of small blood vessels of the tissues and organs, leading to a characteristic rash, muscle weakness, elevated muscle enzymes and sometimes involvement of vital organs with potential environmental triggers and genetic predisposition thought to play a role in its aetiology [1]. Better outcomes are achieved with early diagnosis and early aggressive treatment, often with significant side effects [2]. Due to the heterogeneity in presentation and the rarity of the disease, the diagnosis of JDM is often delayed [3], enhancing anxiety, confusion and a sense of isolation for caregivers of children with JDM.

Caregivers of children with JDM have reported increased levels of stress, higher levels of anxiety and poorer quality of life [3] and parenting stress may adversely affect child health-related outcomes as it could potentially interfere with the management of the child's chronic illness [4]. The challenges for parents in caring for a child diagnosed with JDM may be amplified by the limited knowledge of JDM among non-paediatric rheumatology clinicians, difficulty accessing appropriate services, prolonged treatment, complexities in managing the condition, the burden of side effects of treatment and impacts on the family unit.

There are few studies examining the perspectives of parents or caregivers of children with JDM. This study aimed to describe the experiences of caregivers of children with JDM to inform strategies and interventions to address their needs.

Methods

Participant selection

Participants were eligible if they were a parent or caregiver of a child (aged 0 to 18 years) with clinician diagnosed JDM and were English speaking. Both parents were offered the opportunity to participate. Participants were recruited through the only public paediatric rheumatology centre in New South Wales, Australia, the Sydney Children's Hospital Network, which includes paediatric rheumatology clinics at The Children's Hospital Westmead, The Children's Hospital at Randwick and John Hunter Hospitals. Potential participants were identified by the paediatric rheumatology team as a family with a child diagnosed with JDM. AHK independently approached the family after they had received an introductory letter to the study from their treating team. AHK had no involvement with their child's clinical care. Purposive sampling was used to capture a broad range of perspectives based on socioeconomic status, geographic location, ethnicity, their child's disease course, sex and age. All participants consented to de-identified data being recorded and transcribed and included in the final paper. The project was approved by the Sydney Childrens' Hospital Network Ethics Committee under project number 2021/ETH00053.

Data collection

A preliminary interview guide was developed based on the literature of the experiences and perspectives of caregivers of children with other paediatric rheumatic diseases [3, 5, 6]. AHK conducted a semi-structured interview, approximately 40 minutes in duration, either face to face at a hospital clinic or by telephone, depending on participants preference or COVID- 19 requirements. Recruitment ceased once data saturation was reached (when no new themes or new concepts were emerging in the data). Interviews were recorded and then transcribed verbatim.

Data analysis

Transcripts were entered into HyperRESEARCH version 4.0 to assist with the coding, storage and searching of the data. Using thematic analysis, as described by Braun and Clark (2006) [7] the first author (AHK) coded the transcripts, line by line, conceptualising and categorizing the data and assigning codes to inductively identified concepts. Relationships between common concepts were explored in the data to develop analytical themes, according to Braun and Clark's [7] definition of thematic analysis, where a theme captures *something important about the data in relation to the research question, and represents some level of patterned response or meaning within the data set* [7]. A thematic schema was mapped to demonstrate the connection between themes. A second and third investigator AJ and AK, read and reviewed the preliminary themes to ensure that all experiences and perspectives of participants were included. AHK identified quotes that best captured the themes and AK and AJ reviewed the quotes and consensus was reached as to their appropriateness. All participants consented to de-identified quotes from their interviews being included in the final paper.

Results

The characteristics of the participants are shown in Table 1. Nineteen of 27 (70%) caregivers who were contacted agreed to participate. The majority of interviews were conducted via telephone ($n = 13$, 72%). The majority of participants were female (15, 79%) and all participants identified as their child's biological parent. Table 2 details the characteristics of the children of participants, including their initial presentation, treatments they received, duration of their illness at the time of interview and the number of specialists they had seen prior to a diagnosis being made.

Table 1 Characteristics of participants

Characteristic	No. (%)
Biological Mother	15(79)
Biological Father	4(21)
Age (years)	
40s	14(74)
50s	4(21)
60 and over	1(5)
Education	
Secondary	1(5)
Certificate/Diploma	2(11)
Bachelors/Higher	16(84)
Marital Status	
Married/defacto	19(100)
Employment	
Casual	1(5)
Full time	9(47)
Part time	5(26)
none	4(21)
Geographical Location	
Metropolitan	14(74)
Rural	5(26)
Religion	
Religious affiliation	10(53)
Ethnicity	
Caucasian	15(79)
Greek	1(5)
Fijian Indian	1(5)
Japanese	2(11)

Table 2 Characteristics of the children of participants

Characteristics	No. (%) *n* = 17
Females	9 (53)
Males	8 (47)
Country of birth	
Australia	16 (94)
Other	1 (6)
Religion	
Religious affiliation	10 (59)
Ethnicity	
Caucasian/Australian	13 (76)
CALD	4 (24)
Clinical presentation of illness	
Muscle weakness	8 (47)
Skin rash	11 (65)
Lethargy	1 (6)
Pain	4 (24)
Falls	2 (12)
Age at diagnosis	
1–5 years	8 (47)
6–11 years	4 (24)
12–15 years	5 (29)
15–20 years	0 (0)
Current treatment	
Prednisone	6 (33)
Methotrexate	11 (61)
IVIG	3 (17)
Other DMARD	1 (5)
Tofacitinib	2 (11)
None	4 (22)
Previous treatment	
None	3 (17)
Methylprednisone	4 (22)
Prednisone	10 (56)
IVIG	1 (5)
Methotrexate	7 (39)
Other DMARD	1 (5)
Comorbidities	
None	11 (61)
Perthes disease	1 (5)
Asthma	1 (5)
Coeliac disease	1 (5)
ADHD	1 (5)
Osteoporosis	1 (5)
Skin striae	2 (11)
No. of doctors initially referred to	
1	4 (22)
2	11(61)
3	2(11)
4	1(6)
Duration of illness (years)	
1–2	7 (40)
3–5	7 (40)
6–10	3 (20)

We identified six themes: Rapid crescendo of fear and desperation, lost and unsupported in the health system, disrupting family routines, grieving what has been lost, managing an uncertain future, gaining confidence and motivation. The respective subthemes are described below and selected quotations are illustrated in Table 3. Figure 1 illustrates the relationship between these themes in a thematic schema.

Rapid crescendo of fear and desperation

Alarming deterioration

Parents felt frightened and panicked at the rapid decline they saw in their child's physical abilities. They were very concerned to witness their child being unable to do up their seat belt, falling over at the supermarket, or being unable to get out of bed, *"... we went away bushwalking with some friends, and I had to carry her in the backpack"* (mother).

Sudden realisation of seriousness

Parents initially were dismissive of child's symptoms as a minor illness, part of normal development, or even growing pains, *"I just thought it might be a cold. I just thought it was growing pains."* (mother). However, they gradually began to realise the seriousness of their condition as they

could see their child was not recovering and, in many cases, deteriorating.

Desperate for a diagnosis

Parents felt an increasing desperation when numerous clinicians were unable to find a diagnosis for their child's symptoms – *"The hardest part was the long wait to diagnosis"* (mother). They felt disappointed and let down because of the delayed diagnosis, particularly when they felt general practitioners did not know about the condition– *"our GP had not even heard of it"* (mother). They found it difficult to understand that there was no clear answer as to why their child developed JDM and described feeling stupid when they repeatedly asked the clinicians why this happened to their child. They felt a sense of relief once the diagnosis of JDM was made.

Lost and unsupported in the health system

At the mercy of the medical team

Parents felt intimidated and disempowered by the use of medical terms that were difficult to understand. Protocols for administration of medications, including weekly dosing and the regular blood monitoring for medications also contributed to a sense of being overwhelmed with a rigid medical schedule. Parents relied on the medical team to educate them about JDM and its treatments. However, many parents described feeling lost, *"I do feel a little in the dark…. sometimes I wonder if there are things we are just not being told"* (father). Parents from culturally and linguistically diverse backgrounds (CALD), who were not familiar with the health system found it even more difficult to understand. Parents felt overwhelmed by the complexity of the disease including the varying way the disease could initially present and its response to treatment. They understood that treatments were being determined by an individual clinician's experience, leading them to feel like there was no standard treatment protocol being followed, *''There seems to be so much happening with the disease… it's all very subjective, I feel like we are 100% reliant on the* (Doctor's) *gut feel"* (mother).

Frustrated at lack of services

Parents were shocked by the limited services once their child was diagnosed with JDM. They described feeling forgotten and alone when they discovered there was no single point of contact to coordinate their child's care. Parents in rural or remote areas felt vulnerable as it was difficult to travel long distances to medical appointments in a large metropolitan city, *"it was scary being 5 hours away from the specialist, it's hard when we are the only ones in a rural town* (with the disease)" (mother).

Neglected priorities

Parents reported being disillusioned and upset when they perceived that clinicians were focussed on the treatment of muscle weakness and did not address other symptoms, including pain, and mental health issues, *"I can't localise it, it's pretty broad. It's either muscle pain, back pain, stomach pain, it comes in different forms. I don't think that has really been addressed or understood in a detailed manner….it's something that should've been teased out a little bit more" (father).*

Protracted and painful search for answers

Parents were desperate for the treatments for their children to work, they were prepared to try anything, and some sought non-pharmacological ways to manage the condition including seeking out alternative therapies "*We did try a few elimination diets"* (mother) and, "*We were prepared to try anything" (mother)*, knowing that these were not recommended therapies for children with JDM. Parents described spending many hours researching online and struggled with the lack of specific information relevant to the Australian health care system. Parents discovered that treatment practices varied internationally depending on the availability of treatments and the local health care system. Variation in treatment practices internationally coupled with discovering worst-case scenarios online, including finding examples of children with lifelong disabilities, was both alarming and frightening.

Disrupting family routines

Sibling neglect and loss

In focussing their attention on their child with JDM, some parents felt that their relationship with their other children, or among siblings, suffered. For example, previously siblings were able to play together, this was not possible because of disease symptoms or medication side effects, *''she basically lost a little friend*' (mother). Participants were frustrated that their other children were missing out on school and important life events because of the time taken caring for their child with JDM. Parents were also concerned for future potential mental health problems for siblings, *"we* [the whole family, including siblings] *were semi-suicidal for a lot of last year…"* (mother).

Overloaded with a medicalised schedule

Attending multiple medical appointments was time consuming and parents described the changes they had made to their family's routine *''our family has made Saturday a rest day; that is the day she takes medications"* (mother). Parents spoke of having to accommodate the burden of responsibility for managing their child's medications, including minimising family activities because of side effects from medications, monitoring for their child's

Table 3 Selected quotations supporting each theme

Themes and Subthemes	Quotations
Rapid crescendo of fear and desperation	
Alarming deterioration	*She never goes to the doctor because she's never sick, and then one day she said to me 'can you take me to the doctor, it's really hurting', and that's when I thought oh, God, this must be not growing pains. (mother).*
Sudden realisation of seriousness	*…she needed help wiping herself and things like that, and we were like oh God, what's going to happen, are we going to have to become carers, are we going to have to, one of us resign our jobs, things like that. (mother).*
Desperate for a diagnosis	*…then we couldn't get him to see the neurologist for I think two months. We had this terrible time where we didn't know what it was and we didn't know what to do. (mother).*
Lost and unsupported in the health system	
At the mercy of the medical team	*I do feel a little in the dark….sometimes I wonder if there are things we are just not being told (father).*
Frustrated at lack of services	*Because JDM is so specialised, you didn't really fit anywhere (mother).*
Neglected priorities	*Having a teenager is pretty stressful as you may know…but the combination of a teenager and then someone who's unwell, the compounding can impact mental health (mother).*
Protracted and painful search for answers	*We probably saw six or seven doctors. Only one had heard of it, but didn't know much about it (father)*
Disrupting family routines	
Sibling neglect and loss	*…during that time, she basically lost a little friend…That was emotionally quite difficult for her and obviously for him, because he just wasn't able to do anything (mother).*
Overloaded with a medicalised schedule	*Generally, you find that when you come into the hospital and you see the rheumatologist, it's busy. It's chaos…I'd like to be able to ask a question and have a bit more time (mother).* *I guess the medication is probably the biggest responsibility, just ensuring that that's all okay and making sure his scripts, monitoring that (mother)*
Always on standby	*If he needs to take a day off school because he's just not up to it, you know, I make sure I work from home that day…(father).*
Burdened by financial strains	*Our family was based on my single income and (my wife) wasn't working at the time. I just had to find a way to somehow keep it together (father).*
Grieving what has been lost	
Missing the sunlight	*I know she shouldn't be out in the sun, and I know that that's not great, but I don't know to what extent…I was like, does that mean no lunch time at school, no sport? (mother).*
Struggling with the loss of physical function	*…he was walking with someone who had a broken arm, and he was feeling just as sick as this kid that had a broken arm, and people were saying to him 'well, why aren't you helping that boy with the broken arm, because you're fine?' and he was like I'm not fine, I'm really struggling to walk up and down the stairs. I think because you can't see it, people are not as empathetic (mother).*
Disrupted schooling	*… there are days when he's got a headache or a cold now… I'm a bit more lenient. If he needs a day off here or there, I'm all right (mother).*
Changes in their child from steroid related side effects	*Once he started the prednisone it completely interrupted his sleep, so there's some days he's completely exhausted from waking up continuously through the night (mother).*
Managing an uncertain future	
Bound to chronicity	*…what's it going to do, will it go away, will it get worse? You know…it's a very unpredictable disease, it can be chronic (mother).*
Fearing relapse	*I think it's always there and present for him, because he knows it will never go away. So yes, I think maybe that's the uncertainty around the disease coming back (mother).*
Insecurity with transition to adult care	*The longer-term impacts for that person with JDM, because I believe the JDM will always be a juvenile autoimmune related condition, it won't move into an adult type– it may or may not, but it's always going to be treated like a JDM. Just understanding maybe a little bit more about that in an adult, or that transition to adulthood. Maybe that's what could be improved, actually, that whole transition to the adult world (mother).*
Gaining confidence and motivation	
Strengthening partnership with clinicians	*They relied on our observation to report anything that's happening on a day to day basis and how the treatment is going. We felt quite involved in the treatment in that way, which was good (father)*

Table 3 (continued)

Themes and Subthemes	Quotations
Growing maturity and independence	*He is conscious of eating properly now. Whether this is due to the experiences that he had with this supposed dietary intervention or whether he's doing it because he just doesn't want to appear overweight, he's conscious about what he eats. I think it's in a positive space at the moment (father)*
Gaining hope from shared experiences	*But she's scoring goals, she's getting awards, she's loving it. Socially, she's loving it, and the teams are fantastic. That's one of the joys now that we're out the other end (father).*

response to treatment as well as being the central coordinators of their child's care, made them feel that they were different to other families; *"we are not a normal family anymore"* (mother).

Always on standby

Some participants described having to give up all other parts of their lives, including their job, travel, social events and career aspirations to constantly be there for their child, *"I sat outside the school for hours, knowing that he might need me to come and get him at any point"* (mother). This was described as frustrating, emotionally demanding and time consuming, particularly for mothers who felt they carried the majority of the caring load for their child.

Burdened by financial strains

Parents were stressed juggling their work or business, for example, time taken attending multiple medical appointments was time they had to take off work, which was particularly burdensome when they ran their own businesses. Families carried the burden of costs of travelling to multiple appointments (particularly for regional families), including self-funding allied health therapies, because there were no publicly available services. Finances were a source of tension in many households, "*the only thing we fight about every month is the credit card bill*" (mother).

Grieving what has been lost

Missing the sunlight

Parents understood it was important to avoid the sun to prevent worsening their child's skin disease. It saddened them to have to reconsider family outings and avoid the hottest time of the day, however it also provided an opportunity to proactively manage part of their child's disease. Sun exposure did cause anxiety in parents, and they were confused and worried about managing their child's exposure to the sun particularly when they were not with them. They felt sorry for their child when they missed out on activities to avoid the sun; "*You've got a little boy who just wants to be like every other kid and doesn't want to be out of the sun*" (father).

Struggling with the loss of physical function

Parents were upset at the manifestations of the disease in their child and felt compelled to protect their children from the negative comments of others, knowing that their child's symptoms were only visible to those that knew them well. They felt vulnerable and defensive towards those that did not appreciate the suffering of their child, *''He was walking with someone who had a broken arm, and he was feeling just as sick as the kid with the broken arm and people were saying, why aren't*

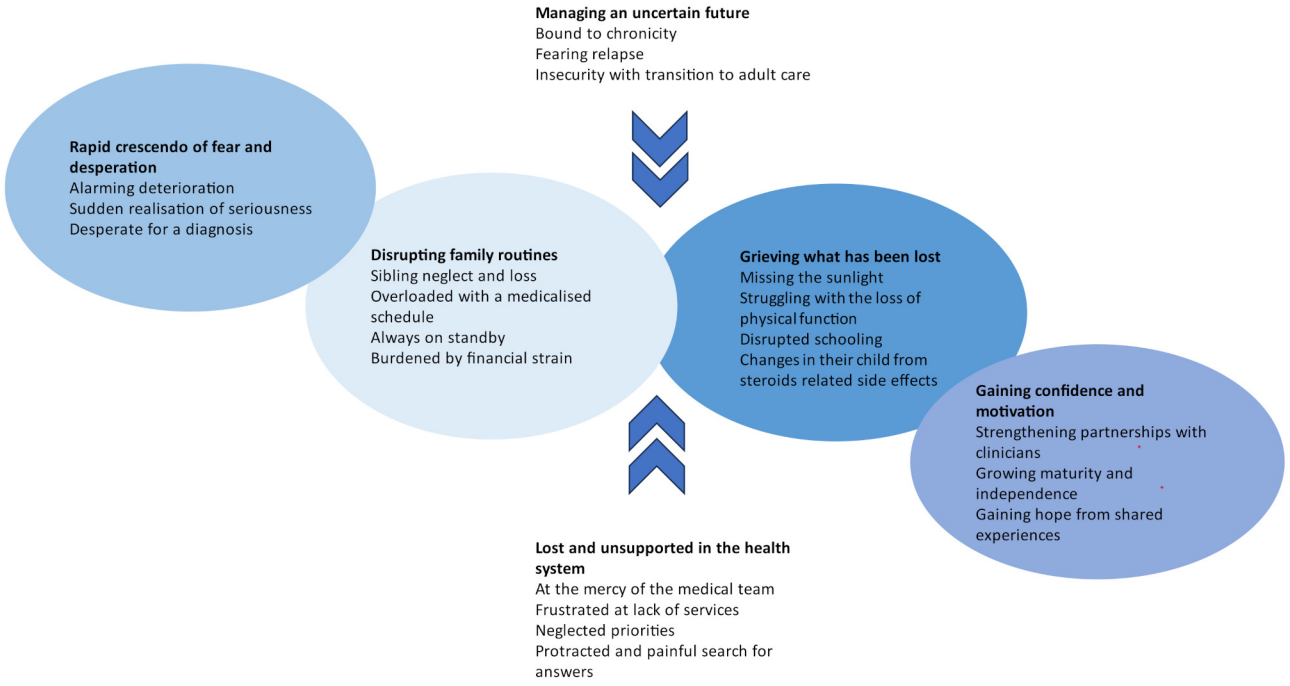


Fig. 1 Schematic diagram of themes and subthemes. Rapid crescendo of fear and desperation disrupted family routines, which contributed to parents' grief at the loss of their normal family life. Feeling lost and unsupported in the medical system and managing an uncertain future also contributed to disrupted family routines and parents grieving what they had lost. Parents fears, struggles with services, changes in family routine, navigating a complicated health system, over time, evolved into them gaining confidence and motivation

you helping that boy with the broken arm?" (mother). Parents wanted their child to do more, hoping that exercise would improve their symptoms, and they felt guilty when they saw their child struggling or in pain.

Disrupted schooling

Parents were despondent to see their child struggle to attend school because of their illness and suffer from side effects of treatment; "*my child is a primary school drop out"* (mother). The majority of parents felt supported by the child's school, however some reported having to justify and negotiate more support services from their school, "*school says he will catch up....but this disease isn't going anywhere*" (mother). Paradoxically, parents reported that COVID remote learning helped their child focus on improving their health whilst maintaining connection with their schoolwork, in a safe environment, *''We had a COVID safe bubble*" (mother). Parents described a sense of relief and satisfaction that their child was not missing out on school.

Changes in their child from steroid related side effects

Participants described feeling very despondent by the changes they saw in their child from the side effects of treatments, particularly steroid side effects. They saw their child gain weight and become self-conscious *"every couple of months we go up a size, he is starting to feel different to other kids"* (mother). Parents were upset by memories of their child's struggles and were sensitive to their self-image, which was amplified when their child moved into their teenage years, *"'She won't even go back and look at photos of herself when she was like that* (mother). Other steroid side effects, including emotional lability was very alarming; "*I give my son toxins every day that make him feel like he wants to die, and then have to spend the rest of the day telling him that it's worth living and there is hope"* (mother).

Managing an uncertain future

Bound to chronicity

Parents did not expect JDM to take so long to achieve remission, nor did they initially appreciate the need for ongoing treatment, "*we didn't realise that it's not fixed overnight"* (mother). Parents became aware that their child may cycle in and out of remission and many parents were shocked and confused by this, they had not expected the disease to behave in this way.

Fearing relapse

Participants described their greatest fear was a relapse of their child's illness, requiring further treatment and the potential side effects that may follow. They felt anxious about what the future might hold for their family; to the extent that some "*tried not to think about the future"* (mother). Parents were unsure as to how they or

their child would cope if they experienced a relapse of the disease.

Insecurity with transition to adult care

Parents were concerned about how their child would transition from paediatric care to adult medical services, including worrying about how their child would navigate the complexity of their disease such as sun avoidance and know if their disease had relapsed,… *I think the transition to adult and the adult rheumatology area, that's probably where we're a bit nervous at the moment because we know next year when he finishes school, he has to move into the adult world* (mother).

Gaining confidence and motivation

Strengthening partnerships with clinicians

Parents reported wanting to know about the objective markers of their child's disease and to understand if there had been improvements with treatment. *If there was more of a… some type of tracking system where we could put in how we think the skin's doing, blood results, those types of things could be monitored all together. Rather than just having a look at him every couple of months. I think that would really help with our anxiety around what is happening* (father).

Parents appreciated feeling involved in their child's treatment. This was aided by clinicians sharing pathology results with them, such as creatinine kinase (CK) to monitor response to treatment. At regular follow up appointments, they waited anxiously for their child's results and celebrated when they saw improvements in objective markers, such as CK, "*We always looked at the CK*" (father).

Growing maturity and independence

With the intense focus on their child's physical health and managing side effects, such as weight gain from steroids or worsening skin disease from sun exposure, parents saw their child become more aware of healthy habits and avoiding sunlight. Parents felt a sense of pride when they observed how their child matured and learned to navigate their condition with their support, "*I know that [he has an] awareness that he doesn't want in be in the sun"* (mother).

Gaining hope from shared experiences

Parents found it helpful to talk to other parents affected by JDM, they wanted to understand other's experiences. This enabled them to visualise what disease remission looked like. It was their way "…. of *looking for the light at the end of the tunnel"* (father), and it gave them hope for the future.

Discussion

Previous studies have indicated that parents caring for children with JDM experiences higher rates of psychological disturbance including anxiety and emotional distress [8]. Our study confirmed similar findings. In our study parents of children with JDM experienced a sudden fear when they realised that their child was seriously unwell and become desperate for a diagnosis. They felt frustrated at the lack of knowledge by many in the medical community about JDM. Once the diagnosis had been made and their child was receiving treatment for JDM, parents described feeling overwhelmed by the rigid medical schedule that took over their family life. They felt isolated because of the lack of services to support their child, finding it challenging to access allied health services with adequate knowledge of the disease. Parents found it difficult to process the diagnosis because their doctors were unable to determine the cause of JDM. Parents also reported experiencing increased financial strain. Complicated treatment regimes, including high doses of steroids, sun avoidance measures and the requirement for regular blood monitoring led parents to describe a sense of feeling overwhelmed by the "medicalisation" of their lives and they grieved the disruption in their family's routine. Parents were saddened to see their child have their schooling disrupted and feared relapse, often because they had seen online worse case scenarios of children with severe disease, yet they gained hope and confidence when they witnessed positive progress in their child. Parents were pleased when they saw the development of independence and confidence in their child's self-management.

Parents of children with chronic illness experience greater levels of stress and lower quality of life [3]. A study examining the experiences of caregivers of children with JDM in the United States, reported that parents' quality of life was reduced and their mood adversely affected [3]. Our study identified similar stress amongst parents. Another childhood rheumatic disease, Juvenile idiopathic arthritis has been described as a family disease [6], similarly our study also identifies direct impacts on parents and their families. Parents described their sadness at the disruption to their normal family routine, changes in relationships between siblings, demands of attending medical appointments and the need to drop everything to support their child. These burdens were especially carried by mothers in our cohort and existing evidence in the literature confirms the major caregiving role to the mother [9] in childhood chronic diseases. The demands of caregiving and effects on financial resources can have a negative impact on women's professional and social lives [9].

Our study provides broad insights into the perspectives of parents of children with JDM. Purposive sampling was

used to include a diverse group of participants including men, women, culturally and linguistically diverse participants and those from regional and metropolitan areas, however, there are some potential limitations. Most of the participants were mothers with a high level of educational attainment, aged in their 40's or over and only approximately 20% of participants were from culturally and linguistically diverse backgrounds (CALD). The transferability of our findings to fathers, young parents, less educated or non-English speaking populations is thus uncertain. We acknowledge that we may have recruited those individuals highly motivated to participate in research, which may bring some inherent bias into the study. We note however that there was reasonable diversity in the duration of the children's disease duration at the time of interview (see Table 2), which may have documented the perspectives of parents with years of experience versus those with newly diagnosed children. Further limitations to our study include that we may not have captured an accurate representation of children's experiences of their disease, by interviewing their parents. Previous studies have suggested that using parents as a "proxy" for their child's experience may underestimate the child's experience, specifically regarding health-related quality of life [11]. The majority of the interviews were via telephone ($n = 13$) and it is acknowledged that this may have influenced participants responses to the questions asked.

We identified specific areas of concern for parents with implications for clinical practice, including delayed diagnosis, lack of JDM specific services, monitoring treatment response, sun avoidance strategies and disruption to schooling. Limited awareness of JDM, its heterogenous presentation [5] and limited paediatric rheumatology services across Australia all contribute to the likelihood of a delayed diagnosis. Previous studies have indicated that the mean time to diagnosis is 8.5 months [3]. In our cohort, there was a high likelihood of delayed diagnosis, with 78% of participants reported seeing at least 2 specialists (after being referred by their General Practitioner) prior to the diagnosis of JDM being made (Table 2). In other chronic diseases, delayed access to specialist care results in deteriorating health, more frequent hospital admissions and poorer health outcomes [10]. This is particularly true for rural and regional patients, who may have to travel long distances with the associated time and the financial demands that entails [10]. It may not be possible to educate every health practitioner specifically about JDM, however understanding abnormal musculoskeletal and/or dermatological presentations in children is integral to reducing the time to diagnosis, in addition to the urgent need to expand access to paediatric rheumatology services across Australia.

We identified further implications for clinical practice, including treating teams recognising the importance of involving parents and children in their disease's management. Parents felt a sense of success [12] when "celebrating" a normal creatinine kinase level or seeing improvement in their child's CMAS (Childhood Myositis Assessment Score). Many reported that closely following sun avoidance recommendations disrupted family routines, however managing their child's sun exposure also gave parents a sense of control over an aspect of their child's disease. Clinicians should focus on effectively communicating the goals and measurements of treatment, recognising the importance of communicating when those goals have been met and providing a clear framework for sun avoidance measures, to avoid confusion and empower parents. Further, parents understand the importance of minimising disruption to their child's schooling [13], with current research suggesting that school disruptions during the COVID-19 era had adverse effects on child health and wellbeing [14]. A previous study suggested that families of children with JDM were worried and anxious during the COVID-19 pandemic relating to disruptions in their treatment or isolation from their school and usual support structures [15]. Our study overlapped with the COVID-19 pandemic and participants reported a sense of relief that their child was not missing out on educational opportunities when their school was in lockdown, when compared to their peers. Multidisciplinary management of families with JDM could therefore include working more collaboratively with educational partners to ensure minimal disruption to schooling. Pandemic home-schooling models may provide a template for JDM patients to better engage in schoolwork.

Our study identified rationalising the use of steroids as an important area for future research. In our cohort, parental stress was impacted by steroid related side effects. Rheumatic diseases are often complex in their management and often require long term management [16]. Similarly JDM may also require complex medication regimes, often lacking evidence base and usually relying on the experience of the treating paediatric rheumatologist [17]. It may be difficult to predict an individual patient's response to treatment [2] and high dose steroids may be required [18], with the potential for significant side effects including; weight gain, stunted growth, bone loss [19], mood disorders and psychosis [20]. Children have reported they often fear the side effects of medications such as steroids and methotrexate [11]. The distress and morbidity caused by side effects of high dose steroids (which can in part fuel the fear of relapse for parents) highlights the urgency to find new, less toxic treatments.

Conclusion

Our study provides a unique insight into the experience of caregivers of children with juvenile dermatomyositis as they navigated their child's initial presentation, diagnosis and response to treatment. Key areas of concern include delays in diagnosis, lack of access to services, response to treatment, fear of relapse, toxic side effects from medications and the unknown causes of the disease. This study provides an important platform for understanding how to better support families with JDM, providing clinicians and policy makers the evidence they need to improve the comprehensive management of this rare disease, to enhance treatment strategies and better inform future research directions.

Abbreviations

JDM	Juvenile Dermatomyositis
AHK	Dr Amy H Kelly
AJ	Professor Allison Jaure
AK	Dr Ayano Kelly
CK	Creatinine Kinase
CMAS	Childhood Myositis Assessment Score
CALD	Culturally and linguistically diverse

Supplementary Information

The online version contains supplementary material available at https://doi.org/10.1186/s12969-025-01079-2.

Supplementary Material 1

Acknowledgements
Not applicable.

Author contributions

AHK devised the project, collected data, analysed data and was the major contributor to writing the final manuscript. AK contributed to analysing the data and contributed to writing the final manuscript. DSG contributed to the collection of data and to the writing of the final manuscript. JC contributed to the collection of data. AJ contributed to analysing the data and writing the final manuscript.

Funding
Dr A.H Kelly received a University of Sydney postgraduate research scholarship that contributed to funding this project.

Data availability

The datasets used and/or analysed during the current study are available from the corresponding author on reasonable request.
Competing interests: The authors declare that they have no competing interests.

Declarations

Ethics approval and consent to participate

The project was approved by the Sydney Childrens' Hospital Network Ethics Committee under project number 2021/ETH00053.

Consent for publication

Not applicable.

Competing interests

No conflicts of interest recorded.

Received: 28 August 2024 / Accepted: 2 March 2025
Published online: 28 March 2025

References

1. Pachman LM, Hayford JR, Hochberg MC, Pallansch MA, Chung A, Daugherty CD, et al. New-onset juvenile dermatomyositis: comparisons with a healthy cohort and children with juvenile rheumatoid arthritis. Arthritis Rheum. 1997;40:1526–33.
2. Wu JQ, Lu MP, Reed AM. Juvenile dermatomyositis: advances in clinical presentation, myositis-specific antibodies and treatment. World J Pediatr. 2020;16:31–43.
3. Kountz-Edwards S, Aoki C, Gannon C, Gomez R, Cordova M, Packman W. The family impact of caring for a child with juvenile dermatomyositis. Chronic Illn. 2017;13:262–74.
4. Cousino MK, Hazen RA. Parenting stress among caregivers of children with chronic illness: A systematic review. J Pediatr Psychol. 2013;38:809–28.
5. Kountz-Edwards S. The psychosocial impact of juvenile dermatomyositis on pediatric patients and parents. Dissertation Abstracts International: Section B: The Sciences and Engineering. 2016;77:No Pagination Specified.
6. Grazziotin LR, Currie G, Twilt M, MJ IJ, Kip MMA, Koffijberg H, et al. Factors associated with care- and health-related quality of life of caregivers of children with juvenile idiopathic arthritis. Pediatr Rheumatol Online J. 2022;20:51.
7. Braun V, Clarke V. Using thematic analysis in psychology. Qualitative Res Psychol. 2006;3:77–101.
8. Ardalan K, Adeyemi O, Wahezi DM, Caliendo AE, Curran ML, Neely J, et al. Parent perspectives on addressing emotional health for children and young adults with juvenile myositis. Arthritis Care Res (Hoboken). 2021;73:18–29.
9. Simşek IE, Erel S, Simşek TT, Atasavun Uysal S, Yakut H, Yakut Y, et al. Factors related to the impact of chronically disabled children on their families. Pediatr Neurol. 2014;50:255–61.
10. Job J, Nicholson C, Calleja Z, Jackson C, Donald M. Implementing a general practitioner-to-general physician econsult service (econsultant) in Australia. BMC Health Serv Res. 2022;22:1278.
11. Livermore P, Gray S, Mulligan K, Stinson JN, Wedderburn LR, Gibson F. Being on the juvenile dermatomyositis rollercoaster: A qualitative study. Pediatr Rheumatol Online J. 2019;17:30.
12. Smith J, Cheater F, Bekker H. Parents' experiences of living with a child with a long-term condition: A rapid structured review of the literature. Health Expect. 2015;18:452–74.
13. Thornton CP, Ruble K, Jacobson LA. Education for children with chronic illness: moving forward in online and virtual learning. JAMA Pediatr. 2022;176:341–2.
14. Rajmil L, Hjern A, Boran P, Gunnlaugsson G, Kraus de Camargo O, Raman S. Impact of lockdown and school closure on children's health and well-being during the first wave of covid-19: A narrative review. BMJ Paediatr Open. 2021;5:e001043.
15. Wilkinson MGL, Wu W, O'Brien K, Deakin CT, Wedderburn LR, Livermore P. A survey to understand the feelings towards and impact of covid-19 on the households of juvenile dermato myositis patients from a parent or carer perspective. Rheumatol Adv Pract. 2021;5:rkab058.
16. Bakker MM, Putrik P, Dikovec C, Rademakers J, Vonkeman HE, Kok MR, et al. Exploring discordance between health literacy questionnaire scores of people with Rmds and assessment by treating health professionals. Rheumatology (Oxford). 2022;62:52–64.
17. Bellutti Enders F, Bader-Meunier B, Baildam E, Constantin T, Dolezalova P, Feldman BM, et al. Consensus-based recommendations for the management of juvenile dermatomyositis. Ann Rheum Dis. 2017;76:329–40.
18. Wu Q, Wedderburn LR, McCann LJ. Juvenile dermatomyositis: latest advances. Best Pract Res Clin Rheumatol. 2017;31:535–57.
19. Ferrara M, Borrelli B, Greco N, Coppola L, Coppola A, Simeone G, et al. Side effects of corticosteroid therapy in children with chronic idiopathic thrombocytopenic purpura. Hematology. 2005;10:401–3.
20. Buchman AL. Side effects of corticosteroid therapy. J Clin Gastroenterol. 2001;33:289–94.

Publisher's note

Reflections on Chapter 2

This paper was designed to inform the clinician and policy makers of the perspectives and experiences of the parents of children with JDM throughout their child's illness. At the time of conducting the research there had been no similar study and it was determined that a qualitative paper exploring these issues would provide valuable information for treating clinicians and policy makers, similar to that which had been published in other childhood diseases such as juvenile arthritis and childhood kidney disease. The interview questions were developed using knowledge gained from paper 1, the principal investigator's own clinical experience and A/Professor Davinder Singh-Grewal's as well as the qualitative research skills of and Professor Allison Tong. The pool of parents that were determined to be eligible for the study was estimated across NSW to be no greater than 70, reflecting the rarity of juvenile dermatomyositis. It was not financially feasible nor practical for the principal investigator to extend the study Australia wide or indeed, internationally and as such there was an understanding that there would be constraints on the number of participants agreeing to take part in the studies that formed chapters 2 and 3. This geographical constraint was also highlighted by the necessity on the part of the researcher to conduct both face to face and telephone interviews as there were a number of participants that lived many hours away from a major metropolitan centre. As the interviews progressed, it became apparent that the approximate time taken was 40 minutes. Up to an hour was put aside to conduct the interview, with there being individual variability as to how accurate the time taken was. There were some participants that were very eager to go into detail about their experiences, and some also seemed to enjoy the opportunity contribute to the research of a disease which had so fundamentally changed their family's life. By comparison, some participants were very keen to move through the questions quickly, they may have been distracted or had limited time available. Some however, had relatively straight forward experiences, where their child had been diagnosed, treated and the disease resolved within months, and this also have influenced the time taken to interview them. Initially it had been planned that the qualitative interview study would form the one research paper and would include the qualitative data detailing the parent's experiences and perspectives and then as a separate result include the ranked outcomes. It soon became apparent however that there was rich qualitative data arising from the ranked outcomes on their own and that this could form the basis of a second research paper. The interviews were divided into 2 separate parts, the first discussing the perspectives and experiences of having their child diagnosed with JDM and the second part explaining the concept of research outcomes and asking them to rank them. A list of outcomes was provided to participants prior to the interview via email, and they were asked to refer to the list during the interview.

Chapter 3

Priorities for outcomes among parents of children with Juvenile Dermatomyositis. A mixed methods study.

Authors

Dr Amy Helen Kelly FRACP[1, 6] (Orcid ID 0000-0002-7963-8159)

Dr Chandana Guha MA, PhD[6,]

Dr Ayano Kelly FRACP, PhD[2, 3, 4]

Dr Jeffrey Chaitow FRACP[5]

Professor Allison Jaure PhD[6,7]

Associate Professor Davinder Singh-Grewal FRACP, PhD[5]

Affiliations

„Department of Rheumatology, Campbelltown Hospital, New South Wales, Australia.

†University of New South Wales, School of Clinical Medicine, South West Sydney Clinical Campuses, Liverpool, New South Wales, Australia

[3]Department of Rheumatology, Liverpool Hospital, Liverpool, New South Wales, Australia.

[4]Ingham Institute of Medical Research, Liverpool, New South Wales, Australia

[5]Department of Paediatric Rheumatology, Westmead Children's Hospital, Westmead, New South Wales, Australia.

[6]The University of Sydney, School of Public Health, Sydney, New South Wales, Australia.

[7]Centre for Kidney Research, Westmead Children's Hospital, Westmead, New South Wales, Australia.

No conflicts of interest recorded.

Correspondence to:

Dr Amy H Kelly

Department of Medicine, Campbelltown Hospital

Therry Road, Campbelltown, New South Wales, Australia

Phone: +61246343000 email: Amy.Kelly@health.nsw.gov.au

Abstract

Background

Juvenile dermatomyositis (JDM) is the most common of the childhood inflammatory myopathies. Outcomes reported in trials may not be those that are relevant to parents of children with JDM. This study aims to identify outcomes that are important to parents of children with JDM and the reasons for their choices.

Methods

Semi-structured face-to-face and telephone interviews were conducted with parents of children (aged 8-21 years) with JDM from three centres in Australia. Participants were asked to rank 21 outcomes and to explain the reasons for their choices. A relative importance score (0, lowest importance, and 1, highest importance) was calculated and the qualitative data was analysed thematically.

Results

Nineteen participants (15 mothers) of 17 children with JDM participated. The top 10 highest ranked outcomes were mortality/death (importance score 0.5), physical function (0.49), muscle weakness (0.28), muscle inflammation (0.25), emotional wellbeing (0.22), inflammation (0.19), pain (0.18), skin problems (0.16), fatigue (0.14), and breathing difficulties (0.11). We identified four themes: experiential relevance of symptoms, navigating symptoms and side effects, towards recovery and finding strength to participate and navigating uncertainty and confusion

Conclusions

Parents of children with JDM prioritise death and mortality, muscle-related outcomes, physical function and debilitating symptoms. Including these outcomes in research in paediatric JDM may help to inform shared decision-making based on outcomes that are meaningful to patients and caregivers.

Introduction

Juvenile Dermatomyositis (JDM) is the most common of the childhood inflammatory myopathies(28). JDM can present with a variety of symptoms including characteristic rash of the hands and face, symmetrical proximal weakness, elevated muscle enzymes and also involvement of other, vital organs(29). Due to its heterogeneity, the diagnosis of JDM may be delayed, treatments are often prolonged and can have significant side effects(28) resulting in increased

stress, confusion and anxiety placed on family units(30)and parents of children with JDM have reported increased levels of stress, anxiety and poor quality of life(28).

Relying on evidence based, clinically meaningful outcome measures historically has been challenging in JDM as JDM trials often don't report outcomes that are meaningful to patients and their families(20) making it difficult to involve the patient or their caregivers in shared decision making(31). Current recommendations suggest that trials should include patient reported outcome measures (PROMs) and many existing clinical outcome measures for assessment of rheumatic diseases have not been developed with patient or caregiver input(31). Attempts have been made more recently to include the patient or caregiver's perspective when reporting in JDM trials(32, 33) with the publication of a core set of outcome measures to be used in JDM trials(34). This study aims to build on earlier work to identify and prioritise outcomes of importance to caregivers of children with JDM and to describe the reasons for their choices.

Methods

Participant selection and recruitment

Parents of children (aged 8-21years) diagnosed with juvenile dermatomyositis were identified and asked to participate in either individual face to face or telephone interviews and were recruited through the public hospital paediatric rheumatology service, within the Sydney Children's Hospital Network, NSW, Australia. Both parents were offered the opportunity to participate. The principal author, AHK approached participants after they had received an introductory letter to the study from their treating team. AHK had no involvement with their child's clinical care. Purposive sampling was used to capture a broad range of perspectives based on socioeconomic status, geographic location, ethnicity, their child's disease course, sex and age. All participants provided informed consent. The project was approved by the Sydney Childrens' Hospital Network Ethics Committee under project number 2021/ETH00053.

Data collection

A preliminary interview guide was developed based upon input from a multidisciplinary team. Participants were first asked to identify and rank an initial 21 outcomes (with 1 being the most important), which were identified from trials and a systematic review of outcomes measures used in dermatomyositis trials(35-37) as well as feedback from earlier interviews. AHK conducted a semi-structured interview, lasting for approximately 40 minutes either face to face or by telephone, depending on participants preference or COVID- 19 requirements, where they were asked to express reasons for their outcome ranking choices Recruitment ceased once data

saturation was reached (when no new themes or new concepts were emerging in the data). Interviews were recorded and then transcribed verbatim.

Data analysis

Priority scores

The importance score for each outcome was based on the frequency of the ranks that were attributed to each outcome and the relative importance of the rank that was calculated based on reciprocal rankings. Reciprocal ranking is defined as one over the rank assigned by each participant in the group(38). For example, if physical function was ranked as first by one and fifth by another participant, the reciprocal ranks that was assigned to the ranks would be 1 and one fifth. Respectively. If the outcome was not ranked by the participant, it was given a 0 as the reciprocal ranking. A higher reciprocal ranking indicates higher priority of the outcome(38). This score takes into account the importance given to the outcome by the ranking and the consistency of being nominated by the participants in each separate interview. A more detailed explanation is given in Appendix part D.

Thematic analysis of qualitative data

Transcripts were entered into HyperRESEARCH version 4.0 to assist with the coding, storage and searching of the data. Using thematic analysis, as described by Braun and Clark (2006)(39)

the first author (AHK) coded the transcripts, line by line, conceptualising and categorizing the data and assigning codes to inductively identified concepts. Relationships between common concepts were explored in the data to develop analytical themes to reflect the reason for their priority ranking of outcomes. A thematic schema was mapped to demonstrate the connection between themes. A second and third investigator AJ and AK, read and reviewed the preliminary themes to ensure that all experiences and perspectives of participants were included. AHK identified quotes that best captured the themes and AK and AJ reviewed the quotes and consensus was reached.

Results

Nineteen of 27 (70%) caregivers who were contacted agreed to participate. The majority of interviews were conducted by telephone (n=13, 72%). Most participants were female (15, 79%) and all participants identified as their child's biological parent. The characteristics of the participants are shown in table 1. Table 2 details the characteristics of the children of participants, including 4/17 (22%) children not on any current treatment.

In total participants ranked 21 outcomes, shown in table 3 and figure 1. The top 10 highest ranked outcomes were mortality/death (importance score 0.53), physical function (0.52), muscle weakness (0.30), muscle inflammation (0.26), emotional wellbeing (0.24), inflammation (0.20), pain (0.19), skin problems (0.17), fatigue (0.15), and breathing difficulties (0.12).

In the qualitative data we identified four relevant themes depicting the experience of participants and their individual experience of their child's illness; Experiential relevance of symptoms; confronting mortality and navigating symptoms and side effects; towards recovery and finding strength to participate; and navigating uncertainty and confusion. The themes and respective subthemes are described below and selected quotations in table 4 and Figure 2 illustrates the relationship between these themes in a thematic schema.

Experiential relevance of symptoms

Tangible and distressing skin symptoms

Participants gave high priority to skin-related symptoms as they could see their child's characteristic rash and could visualise it as a marker of disease activity. "*The only things that are relevant to me are anything to do with her skin, really. Muscle weakness, breathing difficulty she never had.*" (mother). They understood that their child's skin rash had to be managed by avoiding sunlight and maintaining strict sun protection, which became a burden and forced changes to their family's routine. In contrast those whose children did not have skin symptoms, skin symptoms were not prioritised. Participants described monitoring their child's symptoms as distressing, given the unpredictability of their child's symptoms, "*I just don't know if this is going to keep coming back. It's going to be a long process though, because I imagine if this Methotrexate now fixes her skin again, then we're going to be a bit more worried about going off that"* (mother).

Muscle weakness and decline in physical function

Participants reflected that physical function was an important outcome as it was a decline in physical function that prompted them to initially seek medical help for their child. They also understood that physical function was a marker of disease activity and its improvement indicated if their child was responding to treatment, "*physical function… that I understand, that's very important* (father) and, … *that is the definition of function, it's based on how the muscles respond. For my doctor, they would probably look at inflammation first but that's not as a parent of such importance",* (mother). It was challenging for participants to see their child become incapacitated and unable to do basic tasks such as doing up their seatbelt or being unable to stand up from the

floor. Some explained that their child had to depend on their peers for help, … "*he finds it hard to put his socks on at this age…. in the past he's had to ask a friend to do it for him",* (mother).

Some participants identified breathing difficulties as being another marker of disease activity and indicating a decline in physical function, … "*and breathing difficulties, did you say? He had a little bit of that. [That's] when it's actually at its' worst",* (mother). Participants understood muscle weakness to be an outcome that was associated with a measure, such as the CMAS (Childhood Myositis Assessment Scale), as their child had experience of this measure being recorded in clinic appointments to determine the effectiveness of treatment – "*the only one I think was really important was the one that* [the physiotherapist] *did",* (mother).

Debilitating fatigue

Participants felt helpless as they saw their child suffer from severe fatigue, some wondered if this was…*driven by the disease and the treatments and whether there's some psychological component* (mother)? Participants rated fatigue as an outcome when it impacted their child's daily routine … "*He slept and he slept for three hour chunks in the middle of the day because we know it's a drug that creates drowsiness",* (mother). Participants reported inevitable fatigue from the disease and the medications used to treat it.

School engagement, wellbeing and craving normality

Participants wanted their child to be engaged at school and felt alarmed when they saw a loss of focus in their child. Some identified this loss of focus in hindsight as an early indicator of JDM in their child – … "*then once she got diagnosed and they put her on treatment, then she was much more engaged at school. Then her teacher was saying to us, wow. We were thinking she just wasn't really applying herself as much. But in retrospect, she was really lethargic",* (mother).

Participants indicated that their child's social wellbeing improved when the disease began to respond to treatment, and they identified school participation and engagement with their peers as a reflecting a return to what "normal" children of a similar age were doing. Participants were desperate for their child's life to return to what it had been prior to the diagnosis of JDM. "*School is still incredibly, because of the social aspect of that, it's really important* (mother)".

Suffering pain in silence

Participants worried that they couldn't recognise if their child was in pain. They didn't know what they should be looking out for and therefore an objective marker of pain was important to them. "*Pain, I think once you know your child is in pain… I think this is important…"* (mother). Participants prioritised pain over other outcomes that they felt were the medical team's focus…. "(The) *Second thing is managing pain. Then I'm thinking next one is fatigue. These* (other outcome measures) *are all doctor stuff".* (mother).

Navigating symptoms and side effects

Life threatening consequences

Many participants rated death as the most important outcome; it was the outcome they feared the most, "*I hate to put… mortality or death is probably your first outcome, isn't it? You'd hate for that to happen*", (mother). They understood that many scientific trials will rate death in the context of it being a serious adverse event, yet despite this understanding, participants were shocked and scared to learn that death could occur with juvenile dermatomyositis… *"Death. I don't know whether that was even on our mind,* (mother)?... *I mean, mortality I wouldn't… they're very hopeful for a positive outcome where he goes into remission, so that would be the last thing",* (mother).

Participants were not initially aware that JDM could be fatal as they had heard about improved outcomes for JDM and that most parents did not witness serious, life-threatening complications in their child… "*Oh, goodness. Mortality, death. That's probably the most important you want to know. Is this deadly or whatever"* (mother)?

Individualising side effects

Reporting outcomes that described side effects their child experienced was important to some participants as it was the distressing side effects of medications that they had to deal with on a daily basis... *"Yes. For me, yes.* [steroid side effects are important] (mother) and…. [it was the steroids], …*it was because she swelled up. Everywhere, she would have a big belly and a big swollen face"* (mother).

For some participants side effects were not a priority as they felt they had accessible information… *"I think a lot of that information is actually available, to be honest. Particularly around gastrointestinal, like a lot of that's side effects more than the actual disease, at least from what I understand. I think that that information actually is probably quite accessible. If I wanted to know, for example, on Saturday she was a bit off, I can literally just read the Methotrexate information sheet and it will say that nausea and vomiting are common symptoms",* (mother). For many it was a very individual experience of side effects, and this influenced how the related outcomes to these side effects were ranked.

Towards recovery and finding strength to participate

Managing self-consciousness

Weight gain and effect on growth from steroids, often affected children in their adolescent and teenage years and participants described their children becoming very sensitive to physical

changes in their appearance, … *"his growth has been stunted and he gets very embarrassed about that because he's the smallest",* (mother). It deeply troubled participants that their child became self-conscious, embarrassed and lost confidence amongst their peers... *"He then became very anxious, he wasn't comfortable looking at himself in the mirror. He's never been like that before",* (mother).

Weight was also an important marker of general health, and this was relevant to participants as a way to counteract the symptoms of JDM… *"Even the ability to exercise comes to that weight, equal to the discomfort of the physical body.* [Weight] *creates a lot of the health issues and the mobility issues",* (mother). Maintaining a healthy weight was regarded as vital to their child's recovery from JDM, … *"*[His] *appearance. We see that's linked directly to mental health for kids. My son is dressing, he's ten years old. He was always just a regular sized kid, a beautiful kid. He looks like the Incredible Hulk and he wears a men's extra-large", (*mother). Self-consciousness was described as impacting on their child's mental health and potentially delaying their recovery from JDM.

Developing and maintaining emotional resilience

Once their child's disease had gone into remission, participants reflected on what they could have understood better about their child's journey with JDM and this reflection directed them to prioritise certain outcomes, such as emotional wellbeing, … *"I think his emotional wellbeing…He's been quite… it's a big thing for a 12-year-old boy to carry on his shoulders, the uncertainty of it and all of that",* (mother). They recognised in hindsight that their child's emotional well-being was just as important as physical function and required intervention… *"She had a really lovely teacher who just helped her to navigate …what's happening and the rest of the year group. The school counsellor saw* (her) *regularly particularly when her emotions were all over the place and feeling overwhelmed and everything",* (mother).

Participants reflected on the importance of a physical recovery, supporting an emotional recovery, which aided in developing resilience and returning to the life they had before JDM… *"She did sport quite reluctantly, whereas for the two years after she got diagnosed, she did a sport every single term...I think that was really nice as well, because it just made her be more physical because movement's really important for your body"* (mother). Participants reported that their child had a new found focus on their health, which was a positive consequence of JDM and this influenced the outcomes they valued.

Navigating uncertainty and confusion

Unpredictable cause and prognosis

Participants acknowledged that there was so much that was not understood about JDM and this motivated parents to prioritise the outcomes that might help explain the cause of JDM, … *"No, I*

think it's just spontaneous combustion. I think it's just unlucky, I don't know. There's no known reason why it happened. I find the doctors don't want to talk about it, they don't want to… it's not about why it happened, it's kind of about just treating it", (mother).

Many participants made clear that there were outcomes that were important to them and there were outcomes that they recognised would be the focus of the medical team that might determine their child's prognosis; they understood that there would be a difference of priorities of outcomes between the medical team and parents … "*It's really interesting, because it's more perception than actual… because these* [outcomes] *are all relevant to the doctor",* (father).

Participants described their own guilt with the lack of knowledge about JDM and they questioned their own actions and asked themselves if they had contributed to the disease in their child, … "*I remember one family…their boy was diagnosed with the condition in a very similar time. They were eating well, they thought they were doing everything right, but this happened. We also heard about a family whose children both were diagnosed with the condition, so we wondered if it's something to do with the way we live, what we eat, the environment we live in. This sort of thing is very relevant to the mental health of the parents. It's ongoing, we always worry about this happening again" …* (father).

Searching for markers of disease activity

Participants knew how important it was to find new markers of disease that could be potential targets for medical therapies, and they hoped that there would be future biomarkers, yet unknown, that might prove to be vital to understand JDM to improve that outcomes for future sufferers of the disease, … *"Also with the research, why do these kids get it? Do you know what I mean? It's not genetic, you know, what triggers it? Because they don't know anything, really", (*mother).

Participants speculated (particularly those with children with active disease) that there may be new treatment options that could spare their child some of the side effects of current treatments. Similarly, if there was a trigger they perceived as a cause for their child's disease, such as anxiety they prioritised it as an outcome, … *"I don't know the research, maybe, but people suggest that anxiety is a trigger for autoimmunes, in terms of adding to the disease, the possibility of the disease flaring"* (mother).

Confusing medical terms for inflammation

Parents learnt very early in the disease course of their child the importance of "inflammation" as a tool to monitor response to treatment; however, despite the term "inflammation" being used frequently by the medical team, there was little understanding of the meaning of the terms ESR

(erythrocyte sedimentation rate) and CRP (C- reactive protein), … *"When we go for the blood test, we want to know that the inflammation has come down….Understanding the numbers. I didn't quite understand them. I've got two friends who are haematologists and they explained the two important things here…",* (father).

Participants described sometimes feeling like the medical team were speaking a different language when they failed to understand the different terms of inflammation. Interestingly, a more specific marker of muscle inflammation, CK (creatinine kinase), parents better understood the importance of because it directly related to muscle inflammation which they knew was a centrepiece of the effects of JDM on their child's body. It was talked about at medical appointments by the medical team and many described waiting anxiously for the CK result, it was understood to be a helpful marker of their child's response to treatment …" (CK)…*Yeah, for us that was the first indicator that was medically based", (mother).*

Discussion

Outcomes important to participants of children with JDM included mortality/death, physical function, muscle weakness, muscle inflammation, emotional wellbeing, inflammation, pain, skin problems, fatigue and breathing difficulties. Mortality and death was the outcome parents feared the most. Physical function, muscle weakness and skin problems were important as they were often the symptoms that had initiated their child's diagnosis with JDM. Emotional wellbeing, pain and fatigue were prioritised when reflecting on their child's disease journey. Surrogate markers of muscle inflammation and inflammation were important as participants recognised these were a focus for the medical team.

Mortality/death was the highest priority ranked outcome that we identified. Participants described its importance relating to fear of losing their child and their expectation that their children will outlive them(40). Despite it being given highest priority, many participants reported being surprised that JDM could result in death and this may be because were not aware that JDM could be fatal and hence they prioritised it highly when they realised it was potentially an outcome of JDM. With advances in medical treatments, death from JDM has become rare and in a high-income country like Australia, childhood deaths are a very small percentage of total deaths(40). For JDM it may be that treatment advances make conversations around death seem less relevant for both parents and the medical teams(40) but death is still prioritised by parents as it is their most feared outcome for their child. Participants prioritised outcomes that were relevant to their own child's experience of their disease, including when they noticed a change in their child's physical function. Participants understood the loss of physical ability as muscle weakness and focussed on an objective measure of muscle weakness such as the CMAS (Childhood Myositis

Assessment Score), a recognised and frequently used tool reported in juvenile dermatomyositis trials(31, 41). Skin problems were identified in the top ten of prioritised outcomes, reflecting the importance parents place on this outcome and as is reflected in the qualitative data, the demands managing their child's exposure to the sun put on their family's daily routine. Surrogate markers such as CK (muscle inflammation) and CRP/ESR (inflammation) were prioritised by participants as they recalled these were measures documented by the medical team to demonstrate their child's response to treatment. Participants recounted anxiously waiting for the next "CK" result and if there was an improvement it was something that they celebrated(30). Despite this, many participants did not know what the specific measure of CK or CRP and ESR represented and how they related to their child's disease. Patient reported outcomes (PROMs) are under reported in JDM trials(31) and emotional wellbeing, fatigue and pain were all patient reported outcomes that were important to parents whose children had progressed through treatment for JDM. Often it was only with hindsight that participants saw the importance of these outcomes. They reflected that fatigue may have been one of the initial signs that their child was unwell, and they felt guilty that they had not been more aware of when their child was in pain. Breathing difficulties were described as a marker of their child being very unwell and interestingly there was no linking of this outcome to other surrogate outcomes such as blood pressure or heart rate, rather it was understood as a symptom of the disease or a side effect of a medication.

It has previously been reported that parents of children with JDM experience high levels of stress(36). Traditionally when clinicians have focussed on surrogate markers of disease activity, the effects of stress and anxiety on families may have been under appreciated(35). Stress and anxiety may impact on the outcomes of treatments in JDM, particularly where the role of the parent is vital in administering medications, attending medical appointments and providing emotional and social support to their child. Rarely does a chronic illness occur in isolation to other psychosocial factors for the patient and their families, hence the importance of understanding the patient's perspective. We are not aware of a similar study that has focussed on both ranking of outcome measures and the qualitative data explaining these rankings, asking caregivers which outcome measures are important to them in JDM research.

The US Food and Drug Administration defines PROMs as important aspects of how individuals are "feeling and functioning"(42) and PROMs are vital to optimising treatments for paediatric rheumatic conditions, providing information to treating clinicians informing their understanding of a child's and their families full experience of their disease journey(42), for example, patients and their caregivers of children with paediatric systemic lupus erythematosus may describe important symptoms such as pain and fatigue even when surrogate markers of disease activity have normalised. Within the top 10 of the outcome measure prioritised by parents in our study, (table 3), 3 of them were PROMs. PROMs are vital to future JDM research and without their inclusion, important treatment effects and barriers to treatment may not be well understood by

treating clinicians. In rheumatoid arthritis, for example, fatigue has been proposed as reliable measure given it is frequently a symptom that is important to patients and can provide additional information that is not usually obtained from outcomes currently used(43). Pain has also been recognised as one of the top five domains as essential to capture in myositis specific PROMs (31).

Traditionally outcome measure reported in paediatric rheumatic conditions are typically composite outcome measures, which often include laboratory results(44). Previous research examining which outcomes are important to patients has documented the perceived importance of outcomes was influenced by what clinicians regularly measured in routine care(45). We saw a similar phenomenon, where parents prioritised surrogate markers such as CK and CRP/ESR, knowing that these were important to the medical team to assess disease activity. Following markers of inflammation may have assisted participants' understanding of the importance of controlling inflammation and provided them with a tangible marker of treatment success or failure. JDM-specific surrogate markers such as CK may have been favoured over other surrogate markers (e. blood pressure or heart rate). In other diseases such as kidney disease, blood pressure is more closely monitored by patients and their carers, is more relevant to disease activity and therefore more highly ranked(46). Surrogate outcome measures importance, therefore, is likely to be disease specific.

There were some limitations to our study. Our aim was to purposefully select participants from a broad range of backgrounds given evidence suggests that low health literacy is associated with negative health outcomes(47). We may, therefore, have selected for higher educated, English-speaking participants, motivated research participants, with higher levels of health literacy, where their child's disease course may have been less complex. This may have resulted in not capturing the full breadth of themes in the data. It was also noted to be challenging to explain to participants not familiar with scientific research the definition of an outcome measure in a way in which they could understand and rank outcomes. It could also be argued, however, that this may have resulted in "grounded" data, resulting in a more accurate representation of participants personal experiences than if parents had been educated about outcomes prior to the collection of data. Furthermore, some participants found it difficult to rank all 21 outcomes and one participant did not rank the outcomes at all, which may have impacted on the quantitative results, however not ranking an outcome may also signify its lack of importance to that participant. It was also noted that some of the measures' reciprocal rankings had a higher standard error, for example, mortality 0.5 (SE 0.11), which may have reduced their reliability. It is also recognised that paediatric studies focussing on parent's perspectives may not capture themes important to paediatric patients(48) and in our study by collecting data only from parents we may have missed an opportunity to understand the child's perspective more directly.

Shared decision making is an evidence-based approach to healthcare that is essential to promoting patient centred care(49). Identifying outcome measures important to parents of

children with a chronic illness is critical to employing this model both in the research and clinical setting. For the first time our study time identifies outcome measures that are prioritised by parents of children with JDM. Priority outcomes include, death/mortality, markers of muscle inflammation and inflammation, physical function, muscle weakness, pain, fatigue, emotional wellbeing, skin problems and breathing difficulties. We would urge key stakeholders and policy makers to continue to strive to incorporate PROMs and JDM relevant surrogate outcomes into future research to better inform the management of children with JDM.

Table 1. Characteristics of participants

Characteristic	**No. (%)**
Biological Mother	15(79)
Biological Father	4(21)
Age (years)	
40s	14(74)
50s	4(21)
60 and over	1(5)
Education	
Secondary	1(5)
Certificate/ Diploma	2(11)
Bachelors/Higher	16(84)
Marital Status	
Married/defacto	19(100)
Employment	
Casual	1(5)
Full time	9(47)
Part time	5(26)
none	4(21)
Geographical Location	
Metropolitan	14(74)
Rural	5(26)
Religion	
Religious affiliation	10(53)
Ethnicity	
Caucasian	15(79)
Greek	1(5)
Fijian Indian	1(5)
Japanese	2(11)

Table 2. Characteristics of the children of participants

Characteristics	**No. (%) n=17**
Females	9 (53)
Males	8 (47)
Country of birth	
Australia	16 (94)
Other	1 (6)
Religion	
Religious affiliation	10 (59)
Ethnicity	
Caucasian/Australian	13 (76)
CALD	4 (24)
Clinical presentation of illness	
Muscle weakness	8 (47)
Skin rash	11 (65)
Lethargy	1 (6)
Pain	4 (24)
Falls	2 (12)
Age at diagnosis	
1-5 years	8 (47)
6-11 years	4 (24)
12-15 years	5 (29)
15-20 years	0 (0)
Current treatment	
Prednisone	6 (33)
Methotrexate	11 (61)
IVIG	3 (17)
Other DMARD	1 (5)
Tofacitinib	2 (11)
None	4 (22)
Previous treatment	

None	3 (17)
Methylprednisone	4 (22)
Prednisone	10 (56)
IVIG	1 (5)
Methotrexate	7 (39)
Other DMARD	1 (5)
Comorbidities	
None	11 (61)
Perthes disease	1 (5)
Asthma	1 (5)
Coeliac disease	1 (5)
ADHD	1 (5)
Osteoporosis	1 (5)
Skin striae	2 (11)
No. of doctors initially referred to	
1 4 (22)	
2 11(61)	
3 2(11)	
4 1(6)	
Duration of illness (years)	
1-2	7 (40)
3-5	7 (40)
6-10	3 (20)

Table 3. Ranks and importance scores of participants

Outcomes	Ranking	Score	SE*	CI (95% lower)	CI (95% upper)
Mortality death	1	0.50	0.11	0.35	0.65
Physical function	2	0.49	0.11	0.30	0.61
Muscle weakness	3	0.28	0.04	0.20	0.33
Muscle inflammation (CK, AST)	4	0.25	0.05	0.20	0.34
Emotional wellbeing	5	0.22	0.04	0.16	0.27
Inflammation (CRP, ESR)	6	0.19	0.04	0.14	0.25
Pain	7	0.18	0.04	0.12	0.24
Skin problems	8	0.16	0.06	0.09	0.27
Fatigue	9	0.14	0.03	0.10	0.18
Breathing difficulties	10	0.11	0.02	0.08	0.14
Social interaction	11	0.11	0.01	0.09	0.13
Exercise endurance	12	0.10	0.02	0.07	0.12
Cardiovascular disease	13	0.09	0.02	0.06	0.14
Weight	14	0.09	0.01	0.06	0.10
Educational school performance	15	0.08	0.01	0.06	0.10
Ability to travel	16	0.07	0.02	0.04	0.09
Heart rate	17	0.07	0.02	0.04	0.11
GIT problems	18	0.07	0.01	0.05	0.09
Height	19	0.05	0.01	0.04	0.07
Cosmetic appearance	20	0.04	0.02	0.02	0.06
Blood pressure	21	0.04	0.00	0.03	0.05

*Standard error

Figure 1. Prioritised Outcomes

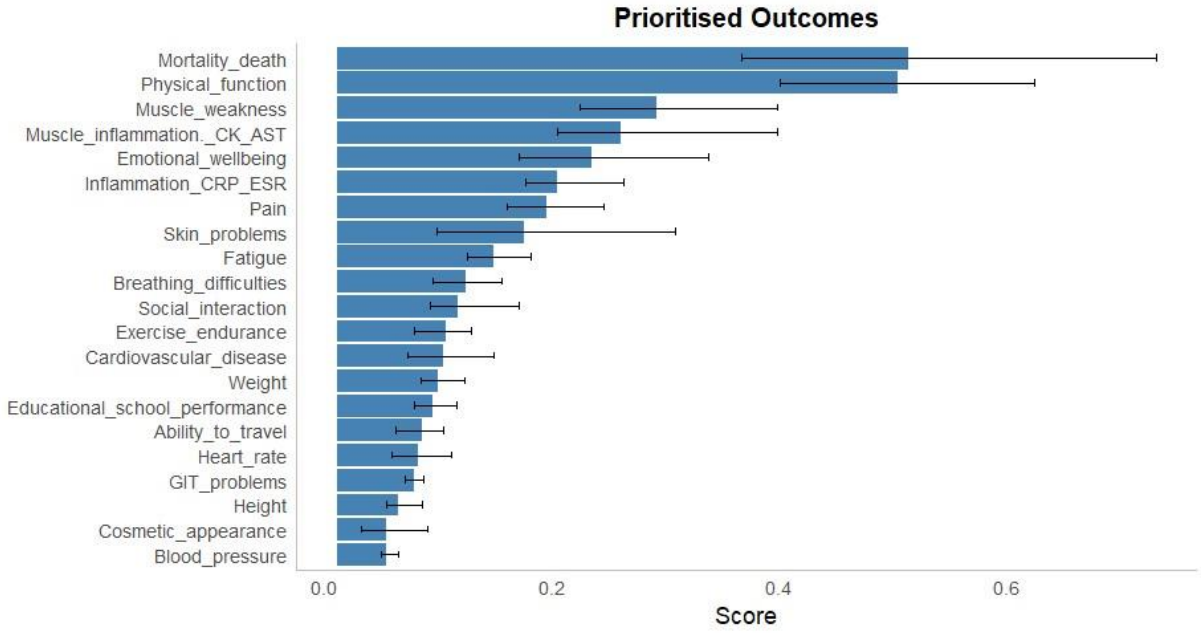


Table 4. Selected quotations supporting each theme

Themes and Subthemes	**Selected Quotations**
Experiential relevance of symptoms	
Tangible and distressing skin symptoms	*Her face was a bit puffy as well, I noticed back in the photo, quite puffy. It was basically just this rash, mainly on her joints, the eyelids, the cheeks* (mother).
Muscle weakness and decline in physical function	*The muscle weakness and physical function are kind of, I would tie them in…because that is the definition of function, it's based on how the muscles respond. For my doctor, they would probably look at inflammation first but that's not as a parent of such importance* (mother).
Debilitating fatigue	*Fatigue, again, I think that she's going to feel tired, she's going to feel like crap* (mother).
School engagement, well-being and craving normality	*We made it, she had to continue with her swimming lessons because swimming is really good. Actually I thought oh, I really don't like soccer, and we trialled it, she loved it. She was also like mummy I'm keen on basketball, tried it and she loved it* (mother).
Suffering pain in silence	*At the beginning I was quite worried about the pain that she was experiencing and was it a normal level of pain? Is it a, I guess, acceptable level of pain in terms of is this normal in terms of the disease and stuff like that? I guess those kinds of things, from a parent's perspective at least, how to interpret the symptoms that we were seeing* (mother).
Navigating symptoms and side effects	

Life threatening consequences	*I don't think this is a condition where death is something that we're dealing with as a possibility* (father).
Individualising side effects	[Anxiety]… *So much causes anxiety that anything that contributes to a better sense of wellbeing has to really valuable as well* (mother).
Towards recovery and finding strength to participate	
Managing self-consciousness	*The weight gain is having an impact on his emotional wellbeing and his interaction with his peers, so that would be clumped in, what I would say is the next batch of important outcomes* (father).
Developing and maintaining emotional resilience	*…in retrospect, probably emotional wellbeing, I think that's something we should've been thinking about earlier and we weren't. That's something I think that is actually more important that I realised* (mother).
Navigating uncertainty and confusion	
Unpredictable cause and prognosis	*When he first got diagnosed, we didn't think at the time – not that we didn't consider it, but he was just such a little kid. He had to go to hospital, he went to hospital, he didn't really question it* (mother).
Searching for markers of disease activity	*Even before that, no one knows what causes it. Indicators coming from medical things like heart rate or whatever, they're going to be more indicative as to what the cause of the disease is than whether she's socially okay…?*(mother)

Confusing medical terms for inflammation	*I think the physical function… it's a bit tricky, actually, because the outcome of inflammation I guess the cause of the physical function and the breakdown around that is probably inflammation and muscle inflammation. I guess importance, it's sort of that muscle inflammation, because I think that will then influence your*
	ability to do physical functions. Aren't they connected? Isn't one a cause and one an outcome?

Figure 2. Schematic diagram of themes, subthemes and their relationship to participant ranked outcomes

Reflections on Chapter 3

As discussed earlier at the end of chapter 2, data for chapter 2 and 3 was acquired form a single interview. There were challenges for chapter 3, including the concept of research outcome measures. There was considerable variability on the concept of understanding outcomes within the cohort of participants. Effort was made to explain that research was conducted with measures that were pre-determined and then evaluated for a particular intervention (with a medications intervention being the easiest method to explain this concept). It became apparent as the interviews progressed that when the participants understood what each outcome was they then better understood concept of research outcomes, hence why it seemed to be more effective to have a pre-determined list. Participants were also given the opportunity to add any other outcomes that weren't on the list and then the list was refined further however, Interestingly there were almost no additional outcome measures suggested and often after discussion with the participant it was agreed that there was an outcome already listed that adequately explained their suggested outcome.

After having finished writing up the paper and having presented it to research colleagues, some suggested perhaps in the future ranked outcome studies should begin with an education session explaining research outcome measures for potential participants in the research, to enhance their understanding. This approach may have some relevance however, it was also discussed that this may have biased participants to a particular view about outcomes, or potentially remove "real-world" patient perspective, which traditionally has been lacking in medical research, this may have led to "trained patients". Hence on reflection despite the difficulties in explaining challenging research concepts to parents the result is more grounded research data in the patient perspective.

When a list of outcomes was provided, inevitably, they have to be listed in order. Effort was made to educate parents that they were not ranked already as they saw listed. It was by chance that mortality was listed higher than other outcomes. This may have led to parents to focus more on mortality and hence rank it higher. During the interviews it was observed that priority was given to certain outcomes that were relevant to their child's experience of JDM, for example if their child had experienced predominantly skin disease, then skin outcomes were prioritised. None of the participants had experience with the death of their child or even life-threatening complications, however the investigators determined it was an important outcome to include, given how universally it is included in clinical trials.

Part 2: Chapter 4

Telemedicine in Australia and its role in the provision of rheumatology services: A narrative review

"*We expect that teleconsultations will become so common as to be unremarkable, that the prefix tele- will disappear, and that all telemedicine work will be considered as part of usual practice*" (50).

Telemedicine is defined by the World Health Organisation (WHO) as, "the provision of health services by health professionals, where distance is a critical factor, using information and communication technologies to exchange valid information for the purposes of diagnosis, treatment and prevention of disease and injury, research and evaluation, and to facilitate the continuing education of health professionals, with the aim of safeguarding the health of individuals and communities"(51). The WHO and UNICEF (United Nations Children's Fund) report multiple benefits from the use of telemedicine including, enhancing access to health care for those living in remote areas and promoting better management of health care for vulnerable groups, including the elderly and those with chronic diseases (51). Telemedicine and the provision of telehealth services in rheumatology may also enhance care however the data is somewhat conflicting, with some of the evidence suggesting a benefit, including improved access to health care services for isolated patients whilst others reporting negative impacts such as increased rates of investigations and maintaining medications for longer(52). Regardless, telemedicine is now ingrained in the Australian Health care landscape and has become an integral part of the delivery of rheumatology care in Australia. It is therefore, vital that further studies examine its benefits and disadvantages so that telemedicine can best meet the health care needs of Australians. Telemedicine and telehealth it has been noted to be used interchangeably and have considerable "overlap in scope"(53). Telemedicine is considered to be … "under the umbrella of telehealth and refers specifically to clinical services"(54). For the purpose of this review and thesis, telemedicine will be used henceforth.

A snapshot of telemedicine services

According to the Australian government it is estimated that between 13 March 2020 and 31 July 2022, 118.2 million telehealth services have now been delivered to 18 million patients, and more than 95000 practitioners have now used telehealth services(26). In the twelve months between

2023-2024, 23.6% (a decline of 3.9% from 2022-2023) of Australians had at least one telehealth consultation for their own health. Those most likely to use telemedicine were those with a chronic health care condition, females, the elderly and those from the least socio-economic disadvantage (55). The COVID-19 pandemic saw an increased uptake of telemedicine consultations when the Australian Government listed enhanced Medicare item numbers and medical practitioners were encouraged to defer face to face consultations to minimise any potential transmission of the COVID- 19 virus, to protect themselves and vulnerable patients, including rheumatic disease patients. Government made temporary changes to the Medicare Benefits Scheme (MBS), which enabled general practitioners, specialists and other health care professionals to subsidise telemedicine consultations for their patients(56). The MBS item numbers have since evolved and have now been made permanent, where video is the preferred mode of delivery, however some phone services can be used(27). In general there is also a requirement for an "established clinical relationship" within the last 12 months for the patient to be eligible for the rebate(27). Existing research identifies concerns about the ability of telemedicine to replace in-person medical consultations(57), specifically with respect to the severity of the disease(57) and the requirements of some specialities for a thorough clinical examination. It has been identified that patients confidence in telehealth is increased when they report having an existing clinical relationship with a clinician, with a previous cross-sectional study (n=390) reporting moderate confidence in the use of telemedicine, with a mean score of 3.3 on a 5 point Likert scale (SD=0.713) (57). Patients report more confidence in telemedicine consultations when they are used for general consultations and lower confidence with telemedicine when it is used by emergency specialists(57). In Australia, data from 2023-2024 suggests a high level of satisfaction with telemedicine consultations, with 89.3% describing they would be willing to use telemedicine again(58). The use of any form of telemedicine varies amongst different medical specialties. In the US data from the American Medical Association's 2016 Physician Benchmark Survey concluded; allergy and immunology have one of the lowest rates of telemedicine uptake at 6.1% and cardiology (24.1%), emergency medicine (22.3%), radiology (39.5%) and psychiatry (27.8%) have some of the highest rates of telemedicine use(59). Telemedicine is an established mode of delivering health care, particularly in the management of chronic diseases and remains an integral part of the health care landscape in Australia.

The rheumatologist's perspective

Following the onset of the pandemic, rheumatic diseases were increasingly managed via telemedicine consultations(53). As an example, the management of rheumatoid arthritis, which, often in the early stages of diagnosis and treatment, may require close monitoring for adjustment of medications was facilitated effectively with telemedicine consultations. It was quickly identified that telemedicine could reduce the burden on an already stretched health care system. As in other parts of the world, rheumatology services in Australia are limited and an alternative method of

health care delivery, that is more cost effective may include telemedicine(60). An Australian study identified that prior to the COVID-19 pandemic, over three quarters of respondents reported using telemedicine with <5 patients per week(56) and Australian rheumatologists were comfortable to prescribe DMARDS (disease modifying anti-rheumatic drugs) and BDMARDS (biologic disease modifying anti-rheumatic drugs) via a telemedicine consultation(56), perhaps more so when the diagnosis is a relatively straightforward, such as a rheumatoid arthritis patient requiring methotrexate. The decision, however, becomes more complex, when relevant clinical findings such as features of interstitial lung disease and liver cirrhosis are missed in the absence of a clinical examination(60) . Rheumatologists have in previous studies identified the lack of a physical examination a significant barrier to them offering telemedicine to their patients and further identified other concerns, including dissatisfaction with joint counts, limited access to technology and administrative tools as well as a lack of financial incentives (56). Studies have also indicated that rheumatology patients are less likely to be advised to de-escalate their immunosuppressant medications during a telemedicine consultation(52), which may be the result of lack of clinician confidence without physically examining the patient or fear of inducing a flare of the disease (52). A critical interpretive synthesis, published in 2024, identified a total of 94 studies examining the unintended consequences of telehealth in Australia(61). The study found that the safety of care was impacted by telehealth, including the risk of misdiagnosis and delayed treatment(52, 61). Specifically in rheumatology, factors such as interpersonal or social clues, poor preparation by the patient for a telemedicine consult and a lack of a physical examination, may all be relevant factors that impact on the safety of telemedicine (61) and the confidence a rheumatologist have to engage in the medium. Other concerns maybe the medicolegal implications of providing care across state borders and also the lack of local services to deal with complications of treatments initiated via telemedicine(61). Another Australian study examined the perspective of clinicians utilising telemedicine to deliver health services to remote communities and described clinician's preference to have an existing clinical relationship with patients to optimise the use of telemedicine(56), noting that rheumatologists preference may be to conduct only follow up appointments via telemedicine and that initial consults should be conducted in person to encompass a thorough physical examination and develop the patient-physician relationship.

The patient's perspective

There have been several studies exploring the perspectives of certain populations engaging in telemedicine and one of the groups often described as the population that can most benefit from telemedicine, are rural and remote populations. In remote areas, it was observed that more commonly the patient was located at a remote clinic for the telemedicine consult to occur, necessitating that these remote clinics were adequately resourced to facilitate telemedicine(56). This may be in contrast to an urban setting, where the patient is usually based at home(56) during a telemedicine consultation. Patient characteristics including health literacy may also influence

their ability to utilise telemedicine services and other factors such as English and digital technology proficiency(56). This disadvantage may also be seen in ethnic minorities in metropolitan centres. Patients' ability to describe their symptoms and use medicines as prescribed are all potential barriers to using telemedicine(56). Warr et al(62) argues that there are "blind spots" that hinder the broad implementation of telemedicine services for rural (and potentially metropolitan) populations, this may include health administrators and clinicians paying little attention to the social determinants of health amongst certain communities. Numerous studies have noted that elderly population, with lower digital literacy levels are at a disadvantage utilising telemedicine services and younger patients are more receptive to the technology(52, 63, 64). Elderly patients would seem to favour in-person consultation to telemedicine, believing that their health needs are better met face to face(64). It has been suggested that elderly patients have less familiarity with digital technologies, hence their more likely negative perceptions of telehealth. Data from the Australian Communications and Media Authority confirms that 95% of Australians use mobile phones to access the internet, with 95% of younger age groups (18-24 year olds) accessing the internet with their mobile phone and 77% of those age over 75(65). Interestingly, a high level of satisfaction with telemedicine use has been reported when a webcam is used (66). When any new technology is developed and it is identified as a method to improve health outcomes, there is a degree of "hype" around its application, which is often influenced by the financial benefits. Warr et al reports that the commercial potential of telehealth was noted in 2019 to be $USD 226.8 billion by 2026(62), which may lead to complex ethical issues as to financial gains versus appropriate medical care and this "hype" may in fact mask when problems arise with a new technology which requires further study and investigation. For telemedicine, problems have emerged, including that it may actually isolate vulnerable groups, including the elderly population, disrupt existing service models and shift power and responsibilities(62); such as shifting the responsibility of managing chronic conditions further onto the patient, where those that do not have a high level of health literacy may be disadvantaged by telemedicine. Other unintended consequences may include depriving rural areas of necessary expertise, threatening rural medical practice, where telemedicine detracts from on the ground in-person medical practice in rural and remote communities(61).

Paediatric populations

Telemedicine may be a successful modality to engage younger age groups, that readily use digital devices, in the management of their healthcare(67). It may also promote improved cost savings for families, avoiding travel, avoidance of missing days off school and increased health related quality of life(68). Telemedicine consultations may encounter problems with technology, with one mixed methods study noting that out of 40 recorded consultations, 17.5% had technical issues(68). Despite these hurdles, the majority of participants described being satisfied with the consultation(68). Within a paediatric telemedicine consultation there may be additional challenges

such as engaging in a patient led versus parent /caregiver led consultation(68) and perceived importance placed by clinicians and patients on the in-person physical examination. An Australian study found that in general practice over 95% of the consultations with a paediatric patient required a physical examination suggesting, therefore that in the paediatric population, telemedicine may not be readily translatable as a replacement for an in-person consultation(69). In paediatric consultations, carers are recognised as critical to the consultation, with up to 90% of consultations with children being led by the carer(69). For rheumatic disease paediatric patients, promoting and maintaining patient led involvement in their care is vital, especially as paediatric patients transition to adult rheumatology services and telemedicine may make this more difficult if the consultation is predominantly led by the parent or caregiver. Furthermore, paediatric telemedicine may be limited by maintaining the attention span of young children, where there are other distractions in the room and with limited visualisation of the child via a webcam and certain behaviours or movements may not be able to be accurately assessed. Conversely telemedicine has been lauded as a method that promotes self-management, where self-management of a chronic disease may be vital to enhancing self-efficacy and confidence in managing their own chronic condition(70). This may be more beneficial to adolescents where protecting confidentiality is vital to the delivery of health care to this sometimes-challenging group(71). Adolescents value the patient- clinician relationship, which enables their confidence in telemedicine, and they may also value not having to travel to appointments(71). Younger patients may also find comfort in describing their thoughts and feelings via telemedicine instead of in person(71). Familiarity with telemedicine likely enhances paediatric patients and their families satisfaction with the technology(66) and increasingly it is seen as an acceptable method of care for patients, already known to clinicians, for management of their chronic condition.

Conclusion

As with all new health technologies there are likely to be patient populations that will benefit and those that are disadvantaged. Structuring telemedicine services within the Australian health care system will require ongoing monitoring, research and reviewing of health outcomes so that it remains fit for purpose. The rapid uptake of telemedicine during the COVID-19 pandemic was remarkable for both patient and clinician adaptability, with government providing the necessary framework for it to become a viable and realistic option for 100 000's of Australians to access health care. The pandemic, however, was a particularly unusual and difficult time and should not form the blue print for telemedicine services in the years to come. Telemedicine has a role to play in the provision of care for those who suffer from a rheumatic disease, enabling more timely access to care and access to scarce rheumatology services. The health care landscape is rapidly changing and in the future health care models maybe vastly different from the models we utilise now. New, emerging technologies, such as Artificial Intelligence will also impact all aspects of health care in the coming years and it is likely that telemedicine will also not be spared.

Chapter 5

The experiences of using telemedicine in patients with rheumatic disease: A group interview study

Authors

Dr Amy Helen Kelly FRACP[1, 3] (Orcid ID 0000-0002-7963-8159)

Dr Kara Goon[1]

Dr Michael Yoon[1]

Associate Professor Davinder Singh-Grewal FRACP, PhD[2]

Acknowledgements

Professor Allison Jaure PhD[3,4]

Affiliations

[1]Department of Rheumatology, Campbelltown Hospital, New South Wales, Australia.

[2]Department of Paediatric Rheumatology, Westmead Children's Hospital, Westmead, New South Wales, Australia.

[3]The University of Sydney, Sydney School of Public Health, Sydney, New South Wales, Australia.

[4]Centre for Kidney Research, Westmead Children's Hospital, Westmead, New South Wales, Australia.

Abstract

Background

Telemedicine may enhance access to medical care for patients with rheumatic disease, however there is limited information as to how rheumatic disease patients perceive telemedicine consultations with their general practitioner (GP) and rheumatologist. This study aims to describe rheumatic disease patients' perspective of telemedicine, to improve patient centred care.

Methods

Three group interviews were conducted from January 2023 to August 2024 with adults (>18 years of age) diagnosed with a rheumatic disease, recruited from a centre in Australia. The interviews were recorded and transcribed. The qualitative data was then analysed using thematic analysis.

Results

Thirty participants were contacted to participate, with only 8 (7 female) participants completing the group interviews. We identified four themes: enabling control and convenience (providing efficiency, minimising burden of time and travel, allowing for flexibility in location, avoiding infection), enhancing healthcare accessibility (removing barriers, developing trust, confusing referral pathways, cost of living pressures), detracting from personal and comprehensive care (fear of a delayed diagnosis, valuing the physical examination, inhibiting communication) and lacking familiarity (restrictive access, struggling with technology and unknown regulation).

Conclusions

Despite challenging recruitment, rheumatic disease patients' preference face to face medical consultations over telemedicine in certain circumstances; prioritising the physical examination, accuracy and timely diagnosis of their rheumatic disease. Telemedicine was favoured for convenience with their General Practitioner but a more detailed consultation in person was preferred with their rheumatologist. As rheumatology health services become insufficient for a growing population, telemedicine may bridge the gap, however patient preference for face-to-face consultations should not be ignored if patient centred care in rheumatology is prioritised.

Introduction

International experience has demonstrated widespread uptake of telemedicine during and since the COVID pandemic that has revolutionised access to healthcare for rheumatic disease patients, where scarce health resources are becoming more and more in demand and the management of rheumatic diseases is increasingly utilising intense treat to target management regimes(72). There is a growing contrast between the requirements of optimal rheumatic disease management and available health resources(73). The provision of rheumatology

services in Australia is also experiencing similar challenges, and telemedicine has been proposed as a method to bridge this divide. Telemedicine is defined as an electronic tool to communicate medical information and connect the patients and providers in distant locations and it can enable remote access to a General Practitioner and a Specialist rheumatologist for patients with rheumatic disease and has been shown to benefit patients who suffer from chronic disease, such as rheumatoid arthritis(74),(75). The use, however, of telehealth is dependent on the understanding and willingness of patients to utilise it (75). Previous international studies have identified a number of factors that influence a patient's willingness to utilise telemedicine, these factors include; age, elderly patients are less willing to use telehealth, familiarity with technology, where socioeconomic status may preclude access to digital technology and geographic location, where paradoxically patients living in rural and remote locations maybe less likely to access telemedicine (75).

Specifically, the benefits of using telemedicine in rheumatology has been mixed, with some studies suggesting a benefit, including enhanced patient satisfaction(76) and providing a cost effective method of managing patients with a chronic medical condition(74), whilst other studies identify telemedicine as resulting in a delayed diagnosis, increased reliance of investigations and reduced likelihood of changing existing medications(52). There are few examples of the patient's experience with telemedicine within an Australian context exploring their willingness to engage with the technology and their preference for telemedicine versus and in-person consultation with either GP or rheumatologist. This study aims to explore rheumatic disease patient's experiences with telemedicine; specifically, their experiences with their GP and rheumatologist and identify patients' key concerns or satisfaction with the technology within an Australian metropolitan context.

Methods

Participant Recruitment and Selection

Participants were identified from a public rheumatology clinic at Campbelltown Hospital. Participants had to speak English, be over the age of 18 years and be diagnosed with a clinician determined rheumatic disease. Participants were approached initially by their treating rheumatologist and if agreeable to participate, were then contacted by AHK who made arrangements for group interview times. AHK had no involvement with the participant's clinical care. Purposive sampling was used when participants were initially approached to participate in the study, with the intent to capture a broad range of perspectives based on socioeconomic status, ethnicity, sex, age and disease diagnosis. All participants provided informed consent. The project was approved by the Sydney Childrens' Hospital Network Ethics Committee under project number 2021/ETH00053.

Data Collection

Group interviews were conducted between January 2023 and January 2024, facilitated by a moderator (AHK) and assistant moderators (KG and MY) focusing discussion on telemedicine and how it was utilised by their GP and rheumatologist, directed by questions which had been derived following a review of the literature. Interview times were for approximately 1 hour. Prior to the group interviews, participants were asked to complete a series of demographic questions (see appendix). There were four planned group interview times, with only 3 eventually taking place due to low participation rates.

Data Analysis

The group interviews were recorded, transcribed and then analysed. Using thematic analysis, as described by Braun and Clark (2006)(39) the first author (AHK) coded the transcripts, line by line, conceptualising and categorizing the data and assigning codes to inductively identified concepts. A thematic schema was mapped to demonstrate the connection between themes. A second investigator AJ, reviewed the preliminary themes, AHK identified quotes that best captured the themes and AHK and AJ reviewed the quotes until consensus was reached.

Results

Participant characteristics

Despite contacting 30 potential participants, a total of 8 participants completed the group interviews. With participant numbers in each group ranging from 1 to 4. The majority of participants were female (7) and 6 out 8 participants were over the age of 60. Fifty percent (4/8) had rheumatoid arthritis and 6/8 had completed education up to secondary school only. All reported being of Caucasian ethnicity and 3/8 were currently prescribed a DMARD, 2/8 were prescribed a targeted synthetic DMARD and 2/8 prescribed a biological DMARD. The characteristics of participants are summarised in table 2.

Themes

Four themes were identified and are described below. Selected quotations relating to each theme are shown in table 1 and figure 1 illustrates the relationship between these themes in a thematic schema.

Enabling control and convenience

Providing efficiency

Participants described directing the consultation according to their needs; if they needed a script or a referral a telemedicine consultation would facilitate this, and they would specifically

ask for this in the consultation. Telemedicine was preferred but only if this was for a short consult for a specific reason, such as a script or a referral. … *"I forgot I had to have Prolia, the injection on Wednesday….so I just made a phone consult to see if she can give me a prescription* (female).

Minimising burden of time and travel

Patients valued telemedicine consultations as it enabled them to have minimal disruption to their day. They could take phone calls at work or in the car. They found it convenient that they no longer had to wait in a crowded waiting room… *"I didn't want to go and book a consult when there's people waiting and it was only for a prescription…"* (female). Telemedicine allowed patients quick access to their GP, without the wait times.

Allowing for flexibility in location

Participants described being able to access telemedicine from any location, they could be at their workplace and speak with their doctor, or they could avoid days off work to go to the doctor. Some participants however, prioritised their privacy and reported only using telemedicine consultations from home… "*No, it's always at home…and I'll sit there and I'll wait…. I've got the phone there, because I don't want to leave her hanging there as well"* (female).

Avoiding infection

Participants understood they were at increased risk of infections, and this informed their willingness to use telemedicine consultations to avoid a doctor's waiting room, which they believed to be a potential source of infection…. *"So, I do understand everyone being really cautious and being careful to protect everybody else as well. But for people that have got diseases like us, it made it really hard…." (*female).

Enhancing healthcare accessibility

Removing barriers

Telemedicine was viewed as a way to quickly access their GP, there was less wait time than a face-to-face consultation,…"*there's a shortage of GPs, and you can't get in straight away"…* (female). If they had a telemedicine consultation, they could discuss their concerns over the phone and then their GP could determine if they needed to see them face to face "…. *yeah….and then he tells me if he wants to see me …and squeezes me in"* (female).

Developing trust

Participants valued being able to access their regular GP, who they trusted and believed understood their medical history. Participants were keen to build a relationship with a… "good GP"…(female) and they felt that they could only build a good relationship via a face-to-face

medical appointment. They did not believe that telemedicine contributed to building the patient-doctor relationship.

Confusing referral pathways

Some participants described being confused by the complex pathways to access specialist appointments. They might be required to have a telemedicine consultation with a specialist, but they had to see their GP in person to receive the referral to the specialist, *"so I had a telemedicine consult with the cardiologist, but just to have that, then I had to have a referral and then I had to go to the GP and get a referral and then they didn't want to do that on the phone and then I had to wait for that and it's just been a mess"* (female).

Cost of living pressures

Participants reported noticing that their GP and specialist were harder to access following the pandemic, particularly if they bulk billed. They described it as being a financial imperative to seek out a bulk billing GP… "*It's definitely harder if you're just a single person on your own, you're paying rent, you're on a pension and then you've got to pay X amount to go and see the doctor on that day…"* (female).

They were not prepared to pay for a telemedicine consultation as they did not see that it carried as much value for them in comparison to a face-to-face appointment. By contrast many described being happy to pay for a face-to-face rheumatology appointment privately as they saw value in that consultation, "*…I'm happy to keep paying. I see value in it. I'm not concerned*" (female).

Detracting from personal and comprehensive care

Fear of a delayed diagnosis

Participants described how close friends, and family did not truly appreciate their symptoms "*….half the time they didn't believe me…but you are living it and feeling it all the time……that's…why it affected me in the joints and people don't see that…."* (female). This created their fear that their symptoms would be dismissed by their rheumatologist as well if the consultation was via telemedicine. Participants regarded accuracy of assessment and validation of their symptoms in person as being vital to promoting a person's trust in their rheumatologist "…. *So, if I hadn't have gone in and had a physical exam, they wouldn't have picked up half of what they have picked up now…"* (female).

Valuing the physical examination

Participants valued a physical examination by their rheumatologist, and they knew there were times when they could not accurately describe their symptoms in the absence of a physical examination. They were anxious for their rheumatologist to examine their joints, "…*Sometimes when my fingers are swollen, on days like that and I can't bend them, you can't describe that over the phone…"* (female). Participants were not confident that their rheumatologist could

accurately assess their joints over the phone, nor were they confident they could locate where their pain was, hence their preference for seeing their rheumatologist face to face "…. *Some days it's not that bad. Other days it's really bad and unless you're actually there, they can't assess how bad it really is…"* (female).

Inhibiting communication

Many felt uncomfortable describing their feelings via a telemedicine consultation with their GP. Participants placed priority on being able to observe and relay facial expressions and body movements when explaining how they were feeling "…. *It's harder over the phone to try and say what is wrong with you…",* (female).

Lacking familiarity

Restrictive access

Participants described being denied a face-to-face consultation when it was considered they were "unsafe" to see their GP in person; they were only offered a telemedicine appointment. It made them angry when it was assumed they would knowingly infect others with a respiratory infection, by not declaring symptoms such as a chronic cough, when they knew they were not infectious. It frustrated participants that this exclusion was instigated by non-medical employees such as receptionists before they could see a doctor, symptoms were always assumed to be infectious *…"so they say, ''Go and have a COVID test'', as you know yourself and your own health that you haven't got COVID, go there and wait in a line for an hour or so…"* (male). Participants were frustrated they were… … *"being treated like a leper…"* (female).

Struggling with technology

Participants had some experience with webcam technology from their COVID experiences "…*we can actually ring telemedicine and we get on the camera and everything for them…"* (female). None of the participants described using a video call or webcam to facilitate a telemedicine consultation, they reported they had never been offered a video call as an alternative to a face-to-face appointment, they had only ever been offered a telephone call. Some participants reported that they would not have been comfortable to use a webcam, "*…I don't have cameras and things on me…. I don't like using technology. I don't keep up with it. I hate it. I hate the things when they break down…"* (female) and … "[telemedicine]…*I couldn't get it on my phone at that time…"* (male).

Unknown regulation

Participants recalled never having heard of telemedicine consultations prior to the COVID pandemic. They believed it was reserved for GPs only and what they perceived to be simple medical problems "…. *I've heard of telemedicine, but I've never realised what it was really…what it was for. I just assumed it was for a GP visit or something, had a sore throat*… (female) and… *"I realised it's probably meant for mental advice…*" (female). Patients were unaware when they

were being billed by their doctor during a telehealth consultation*, "… I assumed they were getting paid somewhere*…" (female). They described being on the phone with their doctor, but it was not explained that their doctor would receive a payment for the consult*, "Obviously it was on the telephone, but I just never associated that to telemedicine…"* (female).

Discussion

Our study identified themes that describes when rheumatic disease patients prioritised telemedicine consultations over face-to-face consultations. The themes identified illustrate the motivations behind these preferences including, enabling control and convenience (providing efficiency, minimising burden of time and travel, allowing for flexibility in location and avoiding infection), enhancing healthcare accessibility (removing barriers, developing trust, confusing referral pathways and cost of living pressures). Other themes identified describe the reasons participants preferred face to face consultations including, detracting from personal and comprehensive care (fear of a delayed diagnosis, valuing the physical examination, inhibiting communication) and lacking familiarity (restrictive access, struggling with technology and unknown regulation).

Participants in our study preferred face-to-face appointments with both their GP and rheumatologist, believing that a face-to-face consultation enabled a more accurate physical examination and diagnosis and promoted the development of trust between the patient and clinician. Participants valued the physical examination, describing the skill of the experienced clinician to localise the cause of their physical pain, which they felt was impossible to do over the phone, this was particularly important to their experience with their rheumatologist. A British study(77), similarly identified rheumatology patients' preference for face-to-face consultations, concluding that telemedicine could reduce clinical accuracy and detract from the trust relationship between clinicians and patients.

Participants in our study believed there was a role for telemedicine, where it could enable efficient access to routine medical care, for example, receiving existing care such as medication prescriptions from their GP. Previous studies have reported that timeliness maybe an important factor in patient outcomes, where telemedicine is used(52). In the context of general practice participants regarded this convenience as a tool for them to request from the doctor what they wanted or needed from the medical consultation. Telemedicine promoted convenience allowing them to avoid taking a day off work and avoid exposure to other patients and their infections in the doctors waiting room; telemedicine maybe an attractive option from a cost perspective, where it could reduce hospital or clinic visits, travel time and provide economic benefits for both the patient and the health service provider(73). Interestingly, participants in our study reported resenting non-medical staff making decisions as to whether they could see either their GP or rheumatologist face to face or via telemedicine. They described feeling stigmatised for symptoms which they felt were not related to infection. Studies have noted previously that participant's negative experiences during the COVID-19 pandemic, may negatively impact their

perceptions of telemedicine(77) and medical administrative protocols, including defaulting to telemedicine where there is a perceived risk from that patient being seen in person may promote barriers to obtaining the necessary medical care, including inappropriate triaging and difficulties navigating medical administration systems(77). Telemedicine may also unknowingly enhance the existing power and influence of medical receptionists in general practice and specialty practices, where a receptionist may act as a gatekeeper with whom a patient needs to negotiate to see a doctor(78), whether that be face to face or via telemedicine.

An existing systematic review (76) examining the effectiveness of telemedicine when compared to standard of care in rheumatic disease patients found that there was no significant difference between patient reported outcome measures and patient satisfaction, there was however, some evidence to suggest improved disease remission, functional impairment and radiographic joint damage(76). Specifically, none of our participants described a desire nor had they been offered rheumatological consultations via telemedicine. An earlier systematic review from 2017, notable to be before the COVID-19 pandemic, suggested that most studies reported a favourable benefit for telemedicine, however they were at significant risk of bias and one study reported the potential for telemedicine to cause harm (79). An Australian study (conducted during the COVID 19 pandemic) identified that telemedicine appointments in rheumatology patients were associated with improved attendance, diagnostic delay, reduced likelihood of changing or ceasing medications, earlier requirement for review and patient's less likely to be discharge from the clinic(52).

Telemedicine may promote additional barriers to accessing health care especially for those patients that struggle with digital technology. Existing literature in rheumatic disease patients describes consultations via telephone (not with the aid of a webcam) as insufficient and often result in further appointments being required(80). In our study, all participants reported engaging in telemedicine appointments via telephone only and none of them were offered the use of webcam or cameras during their telemedicine consultations. This may have been the result of clinicians under or overestimating the difficulty their patients may have using webcam technology or possibly reflects the clinician's own difficulty using the technology(77).

There were several limitations to our study, including the most obvious, sub optimal recruitment of participants. We were able to conduct only three interview group meetings, with an additional two being cancelled due to no participants being present. Due to time constraints, we sourced participants from the one local health district within a large metropolitan city, which may have selected for a socio-economic group less likely to be motivated or economically able to participate in research. Of those that did participate, all were over the age of 50 and given their metropolitan location, they may have faced fewer barriers to accessing different modes of health care compared to rural patients, which may have influenced some of their responses. According to an Australian Rheumatology Workforce report, published in 2023, the majority of rheumatology patients in Australia are older than 75 and live in inner regional areas(81), hence our study may not have captured all themes across an Australian rheumatology patient demographic and factors such as difficulty accessing face to face medical services could have

been greater amongst a regional population, potentially resulting in a more favourable attitude to telemedicine. We sourced participants from a public hospital rheumatology clinic which may also have been a limitation, given the majority of rheumatic disease patients in Australia are seen in private practice and private patients may prefer face to face consultations given they are paying for a service. As we describe in this study, patients may place a higher value on a face-to-face consultation. Due to the limited demographic backgrounds of participants, we may not have fully encapsulated the breadth of themes of the topic, however we would note that many of the themes identified were similar to those identified in the existing literature, which may in some part validate our research. The timing of the research, emerging from the COVID-19 pandemic, may have influenced participants willingness to participate, perhaps being influenced by a "health fatigue" towards their medical care or indeed, medical practitioners in general. Other participant characteristics including gender and disease where the majority of participants were female and reported suffering from an inflammatory arthritis may also have influenced their opinion of telemedicine, comparatively we had very few males and connective tissue disease patients, which may have provided a different perspective. Due to the small numbers in our study, it is not possible to draw reliable conclusions as to whether disease remission and /or medications prescribed influences the participants attitude towards telemedicine.

Our study provides an Australian perspective of rheumatic disease patients attitudes towards telemedicine health services as the health system emerged from the COVID-19 pandemic and provides a useful perspective on how to better utilise telemedicine services in the health care system into the future. These include participants' preferences for face-to-face consultations over telemedicine, placing value on the physical examination. Addressing important barriers to accessing medical care, such as struggling with digital technology and assumptions being made about patient symptoms which were used to determine the mode of medical consultation they had access too. As new technologies emerge, the traditional medical consultation will evolve and change, however the importance of encompassing the patient's perspective cannot be underestimated, where understanding the patient's perspective promotes the best health outcomes for rheumatic disease patients.

Table 1. Selected quotations supporting each theme

Themes and Subthemes	**Selected Quotations**
Enabling control and convenience	
Providing efficiency	*Well, I didn't want to go and book a consult when there's people waiting and it was only for a prescription* (female).
Minimising burden of time and travel	*I think it's easier from a work perspective and I was off the road for 12 months due to epilepsy, so that made* it [difficult] (female).
Allowing for flexibility in location	*No* [telehealth] *it's always at home. I always book here* (female).
Avoiding infection	*He just said, well, okay Mum, you've got to be careful just watch what you're doing and stay safe virtually* (female).
Enhancing healthcare accessibility	
Removing barriers	*There's a shortage of GPs and you can't get in straight away*…[it's worse since the pandemic]…*because they're very hesitant to see people* (female).
Developing trust	[Face to face consultations are preferred]…*yeah because that way you can get that personal interaction with one another* (female).
Confusing referral pathways	*You have to get all these referrals and then they want to see you face to face… but then the referral for a telehealth, so it didn't make sense in your mind* (female).
Cost of living pressures	[Telemedicine consultations]*… Because I had to pay first and then I was connected…* (male).
Detracting from personal and comprehensive care	
Fear of a delayed diagnosis	*It took way too long to get a diagnosis, finally…and it wasn't until I was actually in the hospital and I had the attacks in here, that they went, "Oh my God, wow. We finally see"* (female).

Valuing the physical examination	*I do prefer to see them* [face to face] *because then I can tell them and show them where it hurts* (female).
Inhibiting communication	*It's not that easy, because you forget what you want to say over the phone* (female).
Lacking familiarity	
Restrictive access	*As I said, you get these forms that you have to fill in, have you had a cold? (female).*
Struggling with technology	[Telemedicine]…*yes but I think I couldn't get it on my phone at that time* (male).
Unknown regulation	*Very rarely did I have to do telehealth and it was mostly for scripts and things like that. It wasn't actually a consultation* (female).

Figure 1. Schematic diagram of themes and subthemes. Figure 1 illustrates the role of telemedicine in enabling control and convenience, by avoiding infection, minimising the burden of travel time and creating flexibility in location. Whilst removing barriers and enhancing health care accessibilities and providing efficiencies, telemedicine can lack familiarity. Participants value the physical examination and without it they can develop a fear of a delayed diagnosis, inhibiting communication with their doctor. This difficulty communicating, including developing trust with their doctors can detract from personal and comprehensive care.

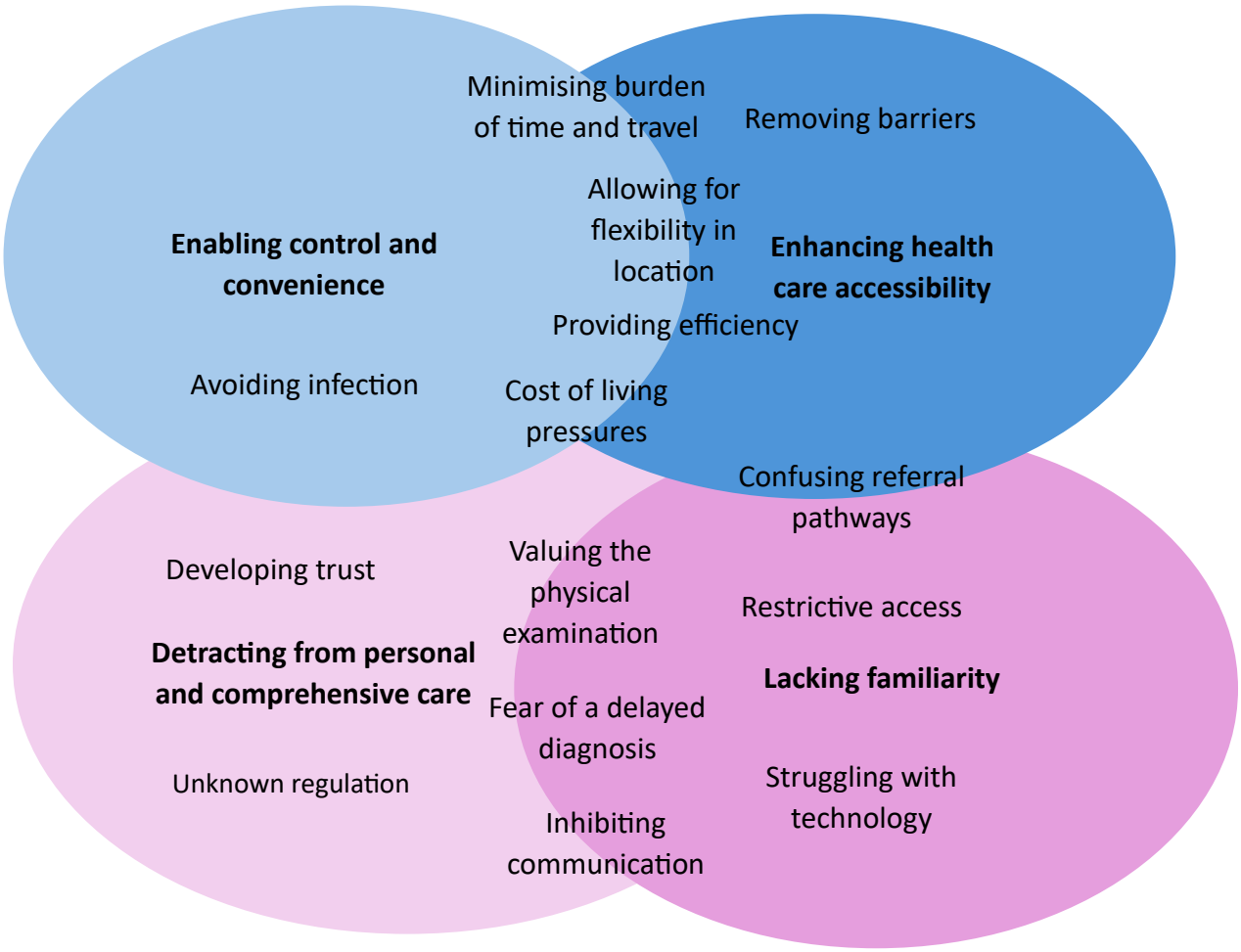


Table 2: Participant characteristics

No. of participants invited to participate	30
No. of participants	8 (%)
Female	7 (88)
Male	1 (13)
Age (years)	
50s	2 (25)
60s	3 (38)
70s	1 (13)
80 and over	2 (25)
Education	
Primary	1 (13)
Secondary	6 (75)
Certificate/ Diploma	0
Bachelors/Higher	1 (13)
Marital Status	
Married/defacto	5 (63)
Single	3 (37)
Employment	
Retired	7 (87)
Full time	1 (13)
Disease	
Rheumatoid arthritis	4 (50)
Scleroderma	1 (13)
Vasculitis	1 (13)
Psoriatic arthritis	2 (25)
Osteoarthritis	3 (38)
Medication	
Prednisone	2 (25)
DMARD	3 (37)
Biologic DMARD	1 (13)
Targeted synthetic DMARD	1 (13)
none	2 (25)
Ethnicity	
Caucasian	8 (100)

Reflections on Chapters 4 and 5

Part two of this thesis evolved as a direct impact of the COVID-19 pandemic. It soon became apparent that the provision of health care services in Australia has shifted and that this thesis was well placed to study some of those changes and clinical practice there had been a huge uptake of telemedicine services during the lockdown periods in Australia that has persisted and remains permanently enshrined into health services. Hence part 2 of this thesis was developed.

There 2 main linking themes between part 1 and part 2 of this thesis. Firstly, both sections contribute to knowledge about rheumatic disease patients in Australia and their experiences of the health care system. Secondly, the technology of telemedicine was experienced by many of the participants in the study that comprises chapters 2 and 3 and the technology itself, ie. the use of phone interviews, was a practical way to conduct research during the pandemic and post pandemic months. The aim of the narrative review in Chapter 4 was to gain a better understanding of the evidence for (and against) telemedicine and to specifically gain a better understanding of the Australian context with respect to patients and their rheumatologists to build the necessary knowledge base to conduct research for chapter 5.

Chapter 5 was surprisingly difficult to complete with adequate numbers. As has been mentioned there were planned group interview sessions where none of the participants organised presented for the session. This was despite calling and confirming prior to the schedule session and offering to pay for parking or transport to the hospital. Time then became a deciding factor, with a deadline for thesis submission looming and after analysing the data it was felt there was adequate material to proceed to writing up the study. It was reassuring that the international literature reviewed for chapter 4, largely supported the findings in an Australian context for chapter 5.

Some of the barriers to people presenting for their group interview session included being unable to take time off work, financial constraints and physical disability. Suspected fatigue with the medical system was also a possibility where these patients with a chronic condition were already required to follow up with their rheumatologist or GP on a regular basis and they were not inclined to present to the hospital to engage in a research project. This attitude could be further researched on its own (but may also suffer from a similar problem!) or the study itself could be replicated in a different patient cohort, for example in a private rheumatology practice. Another way to circumnavigate the problem could have been to offer virtual group interviews online or even a financial incentive to attend, however, both these options may also have biased the selection of participants motivated to attend research conducted in this way.

Following the inception of part 2 of this thesis, it has emerged that telemedicine is only a small part of the health care revolution occurring this century and that artificial intelligence is

developing as the greatest influence on the provision of health care services society has ever seen. Whilst conducting the research to chapter 5 it became apparent that this older cohort of patients could potentially be left behind if there was increased reliance on digital technologies such as AI by health departments, creating further health disparities. This reality makes it an urgent priority to research further the uptake, application and utilisation of AI by patients and clinical providers.

Conclusions

In the introduction of this thesis the importance of understanding the patient's perspective was highlighted, whereby greater knowledge of the patient's perspective can enhance the medical care they receive and improve clinical outcomes. The patient perspective is enhanced by the considerable breadth of rheumatic disease patients that these thesis focuses on, from parents of children with JDM to adults living with rheumatoid arthritis. The aim of part one of this thesis was to specifically investigate the existing literature and then to explore the perspectives of patients and their families. Part two of this thesis evolved due to external influences; it became apparent that the COVID-19 pandemic would have lasting impacts on the healthcare system, particularly with the increased uptake of telemedicine, and it was decided therefore to further investigate the role telemedicine plays in the Australian health care system with the focus remaining on patient experiences and perspectives. Anchoring this thesis on the patient's perspective has been the guiding statement that has driven the 5 separate research projects. This thesis has aimed to address the following:

1. Identifying existing outcome measures used in dermatomyositis research.
2. Understanding the perspectives and experiences of parents of children with JDM to inform future research and clinical practice.
3. Understand parent's prioritisation of outcome measures reported in juvenile dermatomyositis research; to provide clarity about which outcome measures should be included in future JDM research.
4. Summarise telemedicine utilisation in Australia and understand the advantages and disadvantages of utilising telemedicine as a method of health care delivery.
5. Understand the patients experience and utilisation of telemedicine services within an Australian context.

Part 1

Identifying existing outcome measures used in dermatomyositis research.

Efforts have been made by the International Myositis Assessment and Clinical Studies group (IMACS) and the Paediatric Rheumatology International Trials Organisation (PRINTO) to standardised outcome measures reported in dermatomyositis trials(41, 82-84), with much of the research into dermatomyositis being derived from disease registries and observational studies. There are a limited number of randomised control trials in

dermatomyositis, highlighting its rarity and the difficulty investigating interventions and despite these efforts to develop core outcome sets, no prior systematic reviews examining the outcome measures reported in dermatomyositis control trials exists. OMERACT (Outcome Measures in Rheumatology) recommends a systematic review is conducted to identify outcome domains in the existing literature, which informs the development of core outcomes(85) and the systematic review examining dermatomyositis randomised control trials within this thesis aims to fill this evidence gap.

It was identified in this review, the most reported outcome measures including muscle inflammation, physical function, muscle strength global health and immunologic markers. It was determined that these outcomes were predominantly clinical or surrogate measures with few patient reported outcome measures being reported. Firstly, this review identified the lack of robust randomised control trials investigating treatment outcomes in dermatomyositis patients, which was particularly notable given the increasing number of novel therapeutic agents being made available for the treatment of dermatomyositis. Only 20 trials were identified that met the criteria to be included in the review, with the criteria including, citations that were limited to being published in the English language and containing more than 50% participants with a diagnosis of dermatomyositis and/or JDM in the intervention group. Secondly, it was confirmed that there were few PROMs being used in DM clinical trials and thirdly, of the clinical and surrogate outcomes being used, there was marked heterogeneity in their calculation and reporting.

Implications for policy, practice and research:

Multiple groups (IMACS, PRINTO, OMERACT) have promoted the use of core set of outcome measures to be utilised in rheumatic disease research. In stark contrast to the studies included in the systematic review in this thesis, evidence now confirms that up to 80% of rheumatoid arthritis clinical trials include OMERACT developed core set of outcome measures(85). The benefit of using a core set of measures includes providing uniform measures enabling meta-analysis and potentially reducing the risk of reporting bias(86). They also promote a breadth of involvement of all participants in research, including patients, their families and clinicians(86). In dermatomyositis trials, PROMs primarily focus on functional ability, quality of life, disease activity from a patient's perspective and skin symptoms with some of the more prevalent PROMs including patient global assessment of disease activity and HAQ-DI, as endorsed by IMACS, the Medical Outcomes Study Short Form-36 (SF-36), the Nottingham Health Profile (NHP), the Arthritis Impact Measurement Scale-2 (AIMS2), the Patient Reported Outcomes Measurement Information System-29 (PROMIS-29), and the Myositis Activity Profile (MAP) amongst others(87).

The impact of not including PROMs in DM trials maybe substantial, with the Food and Drug Administration and the European Medicines Agency describing PROMs as providing evidence of treatment efficacy(88). Some authors, however, argue that there are potential limitations of including PROMs in trials including potential biases such as subjective influences of mood, social context, treatment expectations and socioeconomic status(88).

It has been reported that patients can overestimate the benefit of interventions and underestimate the risk of harm(89). With PROM's being more subjective in nature, they may be more susceptible to this effect. This effect may influence patients to be more accepting of treatment interventions(89) and overstate their effect, which ultimately could impact the reliability and reproducibility of clinical trials in real world medical practice. Conversely it can be argued that PROM's are an integral part of delivering patient centred medical care, where quality of health care is defined by the patient and not just by the clinician or health administrator(90). PROMs can also enhance communication between the clinician and the patient, provide information about the differences between treatment interventions and provide guidance on informing policy making at a population level(90).

It is clear there is a need in dermatomyositis research to have a clear set of well defined, reproducible outcome measures and there have been gains made in developing these(31, 41, 91) however it is also clear that there is a paucity of PROM's and this systematic review highlights the urgent need for the utilisation and definition of a core set of PROMs to be used in dermatomyositis research. By highlighting the inconsistencies in the reporting of outcome measure in dermatomyositis trials, evidence is provided to promote further refinement of core set measures in dermatomyositis and to encourage the development and validation of dermatomyositis PROMs.

Understanding the perspectives and experiences of parents of children with JDM to inform future research and clinical practice.

The first qualitative study in this thesis aimed to identify the relevant perspectives and experiences of parents with children diagnosed with juvenile dermatomyositis. After interviewing 19 parents of 17 children, six themes were identified, including, rapid crescendo of fear and desperation, lost and unsupported in the health system, disrupting family routines, grieving what has been lost, managing an uncertain future and gaining confidence and motivation. Parents spoke of their experience often, of a delayed diagnosis, dramatic changes to their family's daily routines, fear of their child relapsing and the implications that may have for the need for further treatments as well as treatment side effects experienced by their child and how this impacts their lives and the lives of their families.

It was identified that there were often multiple medical doctors who had seen their child before the diagnosis of JDM was made, leading to frustration and despair at the deterioration they were witnessing in their child. Parents described their naivety at the duration of treatment, the rollercoaster of medical appointments, difficulty accessing appropriate allied health therapists and their great fear of their child relapsing. Principally, the fear of their child relapsing was related to parent's horror at some of side effects of the medications, in particular, steroids, with heartbreaking accounts being given of children dreading looking at themselves in the mirror because of weight gain, or emotional lability

that had led some children to threaten self-harm. Parents confirmed the dramatic change in their family activities and the interaction between siblings, including how simple requirements for the management of their skin disease, such as avoiding the sunlight, led to their once active, outdoor family no longer visiting the beach.

Implications for policy, practice and research:

It's reported in the existing literature on JDM that parents of children with JDM experience increasing levels of stress and anxiety(92). Parental stress may also adversely affect the management of a child's chronic illness (92)and this qualitative study identifies in more detail the possible reasons behind parents' stress and anxiety, gaining important insights which can inform clinicians. As an example, it is reasonable to assume that medical doctors outside of rheumatology have little experience in diagnosing JDM and this study points to potential gains in reducing the likelihood of a delayed JDM diagnosis with better education of the non-rheumatology workforce of what is **not** normal in the childhood musculoskeletal examination.

It is noted that this study provided only the parents perspective and that the next, valuable area of research would be to conduct a similar study to understand the child's perspective as they navigate their disease. The need for sun avoidance created significant anxiety amongst parents and it may be that in the future; sun protection measures could be better communicated to families at the time of diagnosis and strategies identified that manage their anxiety around this aspect of their child's care. Further research into rationalising the use of high doses of steroids in children with JDM is also warranted following parent's articulation of the serious side effects their children experienced and how they perceived it to affect themselves and their families. There is some evidence that a reduced steroid tapering dose and early introduction of steroid sparing agents may provide adequate disease control in children with JDM(93), but to date, there are limited randomised control trials confirming this. Lastly, better access to multidisciplinary care and supports for parents at a health administration level may also promote better outcomes for children and their families with JDM.

Understanding parents' prioritisation of outcome measures reported in juvenile dermatomyositis research; to provide clarity about which outcome measures should be included in future JDM research.

The second qualitative research paper in this thesis, focussed on examining the outcome measures that were important to parents of children with JDM. Participants were also asked to rank in order of importance 21 different outcome measures. A relative importance score was calculated, and the qualitative data was analysed. The top 5 highest ranked outcome measures were mortality/death (importance score 0.5), physical function (0.49), muscle weakness (0.28), muscle inflammation (0.25), emotional wellbeing (0.22). Four themes were identified in the qualitative data; experiential relevance of symptoms,

navigating symptoms and side effects, towards recovery and finding strength to participate and navigating uncertainty and confusion.

Parents described feeling surprised that death could be associated with JDM, yet they prioritised it as the most important outcome. Parents recognised that the death of their child was their most feared complication, yet they were unaware of the potential seriousness of JDM and the potential complications from the therapies used to treat it. Physical function and muscle weakness were the next ranked outcomes, reflecting the initial diagnosis of the child, with many presenting with physical weakness, manifesting as an inability to carry out daily activities such as putting on their own seat belt. Muscle inflammation, specifically measurement of creatinine kinase (CK) was also prioritised by parents which probably reflected their experiences in the clinic where the medical team monitored the CK to assess their child's response to treatment. Emotional wellbeing was also prioritised and some parents reflected that this was only a priority in retrospect, that initially they had not considered their child's emotional wellbeing as being important, but as their journey with JDM progressed they came to understand that improving their child's emotional wellbeing was just as important as their physical recovery.

The qualitative data in this study confirmed parents prioritised outcomes that reflected the symptoms that their own child had experienced. JDM can be heterogeneous in its presentation, and not all patients experience the classic skin and muscle symptoms together, some may predominantly experience skin symptoms which led parents to prioritise skin as an outcome. The roller coaster journey of JDM and the sometimes-harsh side effects from treatments, in particular steroids, led many parents to prioritise weight as an example of a steroid related side effect which was reflected in the theme; navigating symptoms and side effects. Towards recovery and finding strength to participate explained a parent's reflection on their child's journey with JDM and the return of their lives to what it had been prior to their diagnosis. Navigating uncertainty and confusion indicated the many unknowns that parents described with their understanding of JDM, such as their desperation to find out what had caused the disease in the first place. Biomarkers were outcome measures that were prioritised by parents as they were related to their search for a cause of their child's disease. This theme also explained their confusion when they were asked to discriminate between different markers of inflammation, such ESR and CRP, identifying there was little knowledge about what these represented in the context of their child's disease.

Implications for policy, practice and research:

There does not appear to be any prior study in the literature that investigates parent's priorities for outcomes in JDM. This paper directly relates to one of the core aims of this thesis, to explain and describe the patient/parent's perspective and experiences of a disease. This research informs on the perspective of families which in the first instance provides practical information to the medical team that can enhance patient care. Families are the primary source of support and care for children with a chronic illness(36) and by understanding family functioning and identifying areas of need, medical adherence to

medications, clinic appointments and implementing necessary lifestyle recommendations can improve(92). This paper helps identify potential areas of need, including communicating potential side effects of JDM and improving their management, improving the explanation of surrogate markers and the reasons for their use, in particular ESR and CRP and understanding the importance placed on emotional wellbeing by parents and addressing this early in the child's disease course.

On a broader level this paper provides insights into outcome measures perceived by parents to be important in JDM research and implies the importance of PROMs in a core set of measures for JDM research, enhancing future research and creating more reliable and comparable understanding of interventions for the management of JDM. The inclusion of parent prioritised outcomes promotes the relatability of clinical research into everyday clinical practice and reduces research waste(94) and further research should be conducted into PROMs (eg. the patient global assessment of disease activity measure), examining the specific outcome measures meaningful to parents and patients of JDM. Part 1 of this thesis provides an important platform for policy makers and clinicians to help build sustainable health care systems to better manage the children and families who suffer from this rare and little understood disease.

Part 2

Summarise telemedicine utilisation in Australia and understand the advantages and disadvantages of utilising telemedicine as a method of health care delivery.

The review first identifies the increased uptake of telemedicine consultations in Australia following the COVID-19 pandemic and the evolution of the Australian government's subsidy of the digital modality. The review identifies those medical specialities internationally (eg. psychiatry) that have moved quickly to utilise telemedicine and those that have the lowest rates of use (e. immunology)(59). The implications of using telemedicine for the rheumatologist are also examined, suggesting that there is a preference for an in-person initial consultation amongst both overseas and Australian rheumatologists, this may be the result of lacking clinical confidence in making a diagnosis when using telemedicine(56, 63). From the patient's perspective the review determines that there are groups that may be disadvantaged by telemedicine, including the elderly, lower socioeconomic groups and increased responsibility for their healthcare on the patient. Paediatric patients and their families may enjoy the convenience and potential cost savings of telemedicine but also prioritise an in-person consultation that promotes more accurate clinical assessments.

Implications for policy, practice and research:

This narrative review confirms that there is a role for telemedicine consultations within the Australian health care system, however its application must be tempered by the acknowledged limitations of the modality, including the vital role the physical examination plays in the practice of rheumatology. Telemedicine may in some part bridge the gap between scarce health resources and the increasing demands of an overburdened health

care system, however it's use must not further marginalise disadvantaged groups. There are important ethical considerations when telemedicine is used, where telemedicine could result in further exacerbating equity of access for already disadvantage groups(95). Patients most perceived to benefit from telemedicine, such as elderly and rural patients, may seek out telemedicine as a way to enhance their access to health care, but they may also be the group of patients less likely to use the required technology to gain the most benefit from a telemedicine consultation(95). There may also be further un-intended consequences where telemedicine promotes financial reimbursement over quality of care(95). Further research into the health and social outcomes related to telemedicine is vital to inform how telemedicine and other digital health technologies are integrated into the Australian health care landscape.

Understanding rheumatic disease patients experience and utilisation of telemedicine services within an Australian context.

The final paper of this thesis was motivated by increased uptake of telemedicine services following the COVID-19 pandemic and specifically how rheumatic disease participants perceived and experienced telemedicine with both their General Practitioner and Rheumatologist. It was a rapidly evolving space, with this study having to adapt to a changing healthcare environment. Where initially the plan had been to include patient's experiences accessing their rheumatologist and general practitioners, their understanding of COVID-19, vaccinations and how it might impact their rheumatic disease during the COVID-19 pandemic, however, with government restrictions easing and delays due to difficult recruitment, this approach was no longer relevant. The decision was made therefore, to focus more specifically on telemedicine. It was a difficult study to recruit for due to time constraints and participant motivation, reflected in only eight final participants. Four main themes were identified in the data; enabling control and convenience, enhancing healthcare accessibility, detracting from personal and comprehensive care and lacking familiarity.

The first theme, based around convenience, was identified by participants as their main motivation for seeking a telemedicine consult. They found telemedicine consults convenient; they could tell their GP what they wanted, often requesting a prescription, and they were not hampered by having to wait in a waiting room, or interrupt their daily activities for any prolonged duration. The second theme describes how telemedicine could be used to access their GP in a timelier manner. Health care accessibility, however, was not always described as being easier with telemedicine. Participants reported that telemedicine didn't always promote the development of trust between themselves and their GP who they mostly preferred to see in person. Participants also noted administrative barriers were sometimes created by clinicians themselves. For example, when planning to see a specialist via telemedicine, they required a face-to-face referral from their GP. Cost of living pressures were also explained as being a barrier to accessing health care, though not

specifically for telemedicine. Overwhelmingly participants prioritised and valued a face-to-face appointment with their rheumatologist, enabling a physical examination. They described it as being the only way they could have their symptoms validated, the correct diagnosis made, and in their view, the appropriate treatment prescribed. Participants also described other barriers to accessing health care including struggling with technology and being forced to have a telemedicine consultation in place of a face-to-face consultation after being triaged by nonmedical administrative staff.

Implications for policy, practice and research:

Whilst there were small numbers recruited for this study, making it difficult to claim a saturation of themes from the data, the themes that were identified broadly align with similar studies on telemedicine, which gives them some relevance within an Australian context. Understanding patient's perspective regarding telemedicine is vital if health models continue to include telemedicine as a method health care delivery. Principally telemedicine is proposed to enable those living in isolated areas, those with limited access to health care services and those with infections who are required to isolate, access to medical care(96). Despite the participant group living in a metropolitan centre, the themes identified describe the relative isolation that many Australians both within rural and metropolitan areas experience from rheumatology services due to the limited availability of rheumatologists in Australia and highlights the need for rheumatology services across Australia to be increased following changes in workforce demographics and population increases(81). Scarcity of GPs and difficulty building a relationship with a GP is also made relevant by this study and confirms the urgent need for policy makers to address widespread shortages across general practice. This research confirms the importance placed by patients on the physical examination, particularly from their rheumatologist and building trust with their GP through in-person conversation. This finding could be inferred to have significant relevance in the evolving context of artificial intelligence (AI) in health care, where human contact is replaced by computers and indirectly this study highlights the urgent need to explore AI technology and it's potential to be used in medical consultations. Anecdotally in clinical practice patients are reporting using AI services to self-diagnose or direct where and what they seek treatment for. Patient use of AI is only possible by those that can access this technology with adequate digital literacy or financial means. Difficulty accessing technology was described by participants as a reason why they did not engage in the use of a web cam telemedicine consult, identifying this barrier may reveal groups of patients (or indeed, clinicians) that will not be engaged in an "AI revolution" in medicine and may in fact be disadvantaged by it. Interestingly, recent reports by the University of NSW suggests that 75% of health care providers are experimenting with AI solutions(97), this is a rapidly moving sphere and as suggested by this telemedicine study, rapid uptake of technology does not always equate to patient satisfaction. There may be a disconnect between the need for these technologies to ease the financial burden of an ever-growing health system and the preferences of patients; it could therefore be argued that by not considering the patient perspective, we may be inadvertently increasing the burden on the health care system.

Summary

When trying to view this thesis through an objective lens, there are some changes that with hindsight could have improved it. There are the obvious improvements such as increasing recruitment numbers and asking research colleagues for more assistance with recruitment. If COVID had never happened, another qualitative study, interviewing paediatric patients would likely have gone ahead and remains an important future research goal that would complement the studies in this thesis. Another area for improvement could have been to include questions relating to telemedicine with the qualitative study that comprises chapter 2 and 3. This could have provided an interesting perspective that may have strengthened the link between part 1 and part 2.

Reflecting on all the chapters of this thesis I am conscious that I bring inherent bias as a clinician. Every effort was made to ensure that none of the participants were patients of mine, however given the relatively small community of paediatric and adult rheumatology in Australia, the principal investigator being a rheumatologist may have affected some participants willingness to participate in the studies or potentially influenced their responses. Even for chapter 4, my own bias for or against telemedicine could have influenced the literature that I was drawn too that helped comprise chapter 4. The COVID pandemic may also have changed my own perspective and the perspective of participants towards telemedicine and all aspects of healthcare, where there may be for example, heightened concerns for the transmission of infections, particularly in an immunocompromised cohort or different attitudes towards treatments? It is interesting to consider whether telemedicine would be as foremost in patient's and clinicians minds if the pandemic had never happened? How would this then have prepared rheumatic disease patients for AI?

This thesis provides detailed insights into the deficiencies of the outcome measures reported in dermatomyositis trials, the experiences and perspectives of rheumatic disease patients and their carers and how they engage with the health care system through the medium of telemedicine. By focussing on rheumatic disease patients this thesis helps identify areas of concerns for patients, where they were satisfied and where services or communication could be improved. Most importantly this thesis reinforces the importance of placing the patient and their caregivers at the centre of the medical care they receive, with the expectation this will improve medical outcomes.

Appendix Part A: Letter detailing ethics approval

The Sydney children's Hospitals Network
care, advocacy, research, education

Dr Amy Kelly
Department of Medicine
Campbelltown Hospital

3 March 2021

Phone: (02) 9845 1253
Facsimile: (02) 9845 1317
Email: SCHN-ethics@health.nsw.gov.au

Contact for this correspondence:
Research Ethics Office
Research Ethics Support Officer
Corner Hawkesbury Road and Hainsworth Street Locked Bag 4001
Westmead NSW 2145
Sydney Australia
DX 8213 ParramaΣa Tel +61 2 9845 0000 Fax +61 2 9845 3489
http://www.schn.health.nsw.gov.au/
ABN 53 188 579 090

Dear Dr Kelly, **HREC Reference:** **2021/ETH00053**

Project title: **Understanding treatment needs to improve care and outcomes for children and adults with rheumatic conditions and their caregivers.**

Sites: **The Children's Hospital at Westmead**
Sydney Children's Hospital, Randwick
Campbelltown Hospital
Camden Hospital

Thank you for submitting the above project for single ethical and scientific review. This project was first considered by the Sydney Children's Hospitals Network Human Research Ethics Committee ("the Committee") at its meeting **21 January 2021** and subsequently by the Executive of SCHN HREC on the **1 March 2021**.

The HREC has been accredited by the NSW Department of Health as a lead HREC under the model for single ethical and scientific review, and by the National Health and Medical Research Council as a certified committee in the review of multi-centre clinical research projects.

This HREC is constituted and operates in accordance with the National Health and Medical Research Council's *National Statement on Ethical Conduct in Human Research* and *CPMP/ICH Note for Guidance on Good Clinical Practice.*

I am pleased to advise that the Committee has granted ethical approval of this research project. Your approval is valid for five (5) years, effective the date of this letter.

This application has been assessed in accordance with, and meets the requirements of the National Statement on Ethical Conduct in Human Research (2007).

J:\PROJECT FILES - Ethics & Governance\Ethics\LNR\2021\2021.ETH00053\5. Correspondence Out\Ethics approval letter - 3 Mar 2021 - Exec Officer 1 Mar 2021.docx

The documents reviewed and approved by the Committee are:

Document	*Version*	*Date*
REGIS Project Registration		Rec'd 13 Jan 2021
HREA	V3	23 Feb 2021
Adult consent form – Project 2	V3	21 Feb 2021
Adult consent form – Project 3	V3	08 Feb 2021
Adult consent form-HCW – Project 3	V1	08 Feb 2021
Adult PIS – Project 1	V3	08 Feb 2021
Child PIS - Project 2	V2	08 Feb 2021
Ethics protocol	V3	08 Feb 2021
Ethics response Feb 2021 checklist form	-	25 Feb 2021
Healthcare worker- PIS – Project 3	V2	08 Feb 2021
Interview guide - Project 3	V3	08 Feb 2021
Interview questions - Project 2	V3	09 Feb 2021
JDM Interview guide revised - Project 1	V3	09 Feb 2021
Letter of introduction - Project 1	V2	08 Feb 2021
Letter of introduction - Project 2	V2	08 Feb 2021
Letter of introduction - Project 3	V2	08 Feb 2021
Parent/Guardian consent form - Project 1	V3	08 Feb 2021
Parent/Guardian consent form - Project 2	V3	08 Feb 2021
Parent/Guardian PIS – project 2	V3	08 Feb 2021

Patient and caregiver PIS – project 3	V2	08 Feb 2021
Young adult PIS - project 2	V3	08 Feb 2021

Please note the following conditions of approval:

1. The Coordinating Investigator will immediately report anything which may warrant review of ethical approval of the project in accordance with the SCHN adverse event reporting policy.

2. All proposed changes to the research protocol, including the conduct of the research, changes to site or personnel, or an extension to HREC approval, are to be provided to the HREC or its delegate for review before those changes can take effect.

3. The HREC will be notified, giving reasons, if the project is discontinued at a site before the expected date of completion.

4. The co-ordinating investigator will provide an annual report to the HREC on the anniversary of this approval letter, and a final report on completion of the study.

5. Your approval is valid for five (5) years from the date of the final approval letter. If your project extends beyond that five year period and you are still actively recruiting you will be required to resubmit your application incorporating any amendments within six (6) months of that approval expiry date. If your project is in follow up on, or analysis, please submit and application for amendment to extend the approval period. Ethics approval can be extended for a period of twelve (12) months at a time.

6. In the event of a project **not having commenced** within 12 months of its approval, the approval will lapse and reapplication to the HREC will be required.

Should you have any queries about the HREC's consideration of your project please contact the Research Ethics Support Officer on (02) 9845 1253.

You are reminded that this letter constitutes ethical approval only. You must not commence this research project at a site until separate authorisation from the Chief Executive or delegate of that site has been obtained. A copy of this letter must be forwarded to all site investigators for submission to the relevant Research Governance Officer.

The SCHN HREC wishes you every success in your research.

Yours faithfully

Associate Professor Sarah Garnett
Chair, Sydney Children's Hospitals Network Human Research Ethics Committee Sydney Children's Hospitals Network Human Research Ethics Committee

***NB: All clinical trials must now be registered on a publicly accessible registry such as the Australian New Zealand Clinical Trials Registry. For further information please go to www.anzctr.org.au*. Please provide this office with a copy of your registration number for our records if you have not already done so.**

J:\PROJECT FILES - Ethics & Governance\Ethics\LNR\2021\2021.ETH00053\5. Correspondence Out\Ethics approval letter - 3 Mar 2021 - Exec Officer 1 Mar 2021.docx

Supplementary Table S1. Search filters.

MEDLINE	Embase	Clinicaltrials.gov	PsychINFO
20th February 2017-8th May 2020	20th February 2017-8th May 2020	20th February 2017-8th May 2020	20th February 2017-8th May 2020
1. randomised controlled trial.pt. 2. controlled clinical trial.pt.	1. randomised controlled trial.pt. 2. controlled clinical trial.pt.	dermatomyositis	dermatomyositis.mp
3. pragmatic clinical trial.pt.	3. pragmatic clinical trial.pt.		
4. randomised.ab.	4. randomised.ab.		
5. placebo.ab.	5. placebo.ab.		
6. clinical trials as topic/	6. clinical trials as topic/		
7. randomly.ab.	7. randomly.ab.		
8. trial.ti.	8. trial.ti.		
9. or/1-8 9.	9. or/1-8 9.		
10. exp dermatomyositis/	10. exp dermatomyositis/		
11. dermatomyosit$.tw.	11. dermatomyosit$.tw.		
12. 10 or 11	12. 10 or 11		
13. 9 and 12	13. 9 and 12		
14. animals/ not (humans/and animals/) 15. 13 not 14	14. animals/ not (humans/and animals/) 15. 13 not 14		

Supplementary Table S2. Description of outcome domains.

Treatment ef cacy	Unspecified definition of improvement
Global health	Clinical composite scores encompassing disease activity
Muscle strength	Variable measures of muscle strength
Physical function	Measures of physical function
Quality of life	Measures of quality of life
Skin	Adverse events of skin, changes in composite score measuring skin disease activity
Muscle in ammation	Specific markers of muscle in ammation *e.g.*, CK, AST
Immunologic marker	Autoantibodies, novel immune markers
Haematologic	Cell counts, haematologic disorders
Metabolic	"complete metabolic profile", electrolytes
Bone	Clinical endpoints for disease states related to bone
Cardiovascular	Cardiac disorders, measures of cardiac function
Pulmonary	Pulmonary disorders, measures of pulmonary function
Steroid use	Steroid treatment, +/- dose
Pain	Pain VAS
Anxiety/depression fatigue	Anxiety/depression Fatigue scores
Adverse event not speci ed	
Renal	Measures of renal function
Liver	Measures of liver function
In ammation	CRP, ESR
Genetic	Genetic markers
Blood glucose	Blood sugar level, HBAIc
Cholesterol	Measures of lipids
Weight	BMI, weight measures
Height	Height measures
Nutrition	Diet
Systemic symptoms	Temperature
Neurologic	Neurologic disorders
Death	Death
Infection	Sepsis
Gastrointestinal	Gastrointestinal disorders
Injury	Iatrogenic adverse event
Malignancy	Neoplasms

Supplementary Table S3. Reported muscle strength outcomes, the number of reported muscle groups tested and the scale used.

Muscle Strength Outcome Measure	No. of muscle groups tested	Scale
MMT	Not specified	Not specified
MMT-8	8	Not specified
MMT	Not specified	0-15
MMT	16	0-6
Medical Research Scale	6	0-60
MMT-8	8	0-80
MMT-8	8	>15%/<15% improvement
MMT-8	8	20% improvement in muscle strength on 2 visits

PIT (peak isometric torque) peak muscle strength	Not specified	Not specified
MVICT (maximal voluntary isometric contraction testing) quantitative myometry score	10	Not specified
Muscle performance	Not specified	Not specified
Medical Research Scale	6	0-15
Muscle function	Not specified	Not specified
1-RM leg press	Not specified	Not specified
1-RM bench press	Not specified	Not specified
Handgrip	Not specified	Not specified
Timed stands	Not specified	No. of repetitions
Timed up and go	Not specified	No. of seconds
Isometric muscle strength (handheld dynometry break method)	Not specified	Not specified

Fig. 4 legend.

PhVAS: physician VAS; PaVAS: patient VAS; GD: global damage; ParVAS: parent VAS; VASGDact: VAS global disease activity; CAS: Convery Assessment Scale; MphGVAS: mean physician global VAS; OD: other damage (death not specified); CutUlc: cutaneous ulceration; Ery: erythroderma; Pann: panniculitis; Eryrash: erythematous rashes; Helrash: heliotrope rash; Gottpap: Gottran's papules; Pericap: periungal capillary changes; Alop: alopecia; Mech: mechanic's hands; Arth: arthritis; Arthral: arthralgia; Pyrex: pyrexia; Weight: weight loss; Fatigue: fatigue; Dysphag: dysphagia; Abdpain: abdominal pain; Respms: respiratory muscle weakness; ILD: acute reversible interstitial lung disease; Dyspho: dysphonia; Pericar: pericarditis; Myocar: myocarditis; Arryth: arrythmia; ST: sinus tachycardia; Myos: myositis; Mya: myalgia; PhysGA: physician global assessment; MyosTre: myositis treatment; CPK: creatinine phosphokinase; Othenz: other muscle enzymes; Arms: lift arms; Dressing: dress; Jars: open jars; Keys: use keys; Stairs: go up stairs; Lowchair: sit on low chair; Lift feet: lift feet up; Trip: trip over; Turnover: turn over; Lyingtosit: lying to sit; Eat: eat; Blow: blow through mouth; Headup: lift head up; Tiredminex: tired with minimal exertion; Bowelscx: bowel complications; Tiredprolex: tired with prolonged exertion; Write: write; Shoes: put on shoes; Msatrophy: muscle atrophy; Msweak: muscle weakness; Msatrophyrad: muscle atrophy radiographically; Contractures: contractures; Osteo#: osteoporosis with fracture; Osteo: osteoporosis; AVN: avascular necrosis; Deformities: deformities; Restrictedmvt: restricted movement; Poik: poikiloderma; Lipo: lipodystrophy; GITdysm: GIT dysmotility; Constip: constipation; Diarrhoea: diarrhoea; Joint Count: joint count; EMGAx: extramsucular global assessment; MsDA: muscle disease activity VAS (100mm); OthDA: other disease activity.

Appendix Part C: Supplementary material for Chapters 2 and 3, interview guide.

Participant and child demographics and clinical characteristics

A. Demographics of caregiver

1. Name of participant: _____
2. Date of birth: |__|__| / |__|_ |||__ |__|
3. Gender: male □ female □ unspecified □
4. Interview location/date: _____
5. Ethnicity: _____
6. Religion: _____
7. Postcode: |__|__|__ |__|
8. Employment status caregiver: □ FT □ PT or casual □ Student □ Not employed □ Other _____
9. Highest level of education of caregiver:
   - □ Primary school
   - □ School certificate
   - □ HSC/equivalent
   - □ Diploma
   - □ University degree
10. Marital status: □ Married □ Living with partner □ Partner (not living with) □ Divorced □ Separated □ Widower □ Single
11. Living arrangement: _____
12. No. of children/ages: _____
13. Relationship to child/patient: _____

B. Demographics of child

1. Name of child:: _____
2. Gender: male □ female □ unspecified □
3. Date of birth of child: |__|__| / |__|_ |||__ |__|

4. Country of birth of child: _____

5. Religion: _____

6. Ethnicity: _____

C. Clinical characteristics

1. Initial presentation of illness:_____

2. Age at diagnosis: _____

3. Current treatment: □ none

□ prednisone dose; duration: _____

□ immunosuppression; dose; duration _____

□ IViG; duration _____

□ Other; duration _____

4. Previous treatment: □ none

□ prednisone dose; duration: _____

□ immunosuppression; dose and duration _____

□ IViG; duration _____

□ Other; duration _____

5. Comorbidities: □ No □ Yes
_____

6. Other Medications: □ No □ Yes
_____

D. Access to healthcare services

1. General Practitioner: □ Yes □ No

2. Specialist::

□ Paediatric Rheumatologist

□ Derrmatologist

□ Paediatrician

□ Neurologist

□ Other

3. Allied Health:

□ Physiotherapist

□ Occupational therapist

□ Other

4. Other: (please specify)

Interview guide

1. Introduction

- Explanation of the study, confidentiality, obtain informed consent, questions.
- Can you tell me about how you first found out your child had JDM?
- Thinking from the time your child was diagnosed up until now, what areas of your life have been most impacted by JDM; what has been most challenging - why?

2. Personal and role

- In what way has caring for a child with JDM impacted you – personally?
- What are the added responsibilities and do you feel about these/cope with these? (medications, school)

3. Relationships (family/social) and financial

- Has it impacted on your spouse/family – in what way and how do you cope with this? (relationships)
- Did it have any impact financially – in what way?

4. Healthcare and treatment management

- On a scale of 1-10 (where 1 is not confident at all and 10 is very confident) how confident do you feel about managing your child's health/treatment – why?
- How accessible are health care services for you and your child?
- How involved do you feel you are in making treatment decisions? Can you give some examples? *(access, communication)*
- How would you describe your relationship with your child's health care team?
- Are there any other support groups or alternative health care services that you access?

5. Outlook and prognosis

- What do you consider to be the long term outlook for your child– challenges? *(health, career, education, social)*

6. Outcomes

- Researchers conduct clinical trials (research) to look at effectiveness of treatments and the findings are meant to inform decisions that are made between the doctor, parent and child. However, the outcomes (what they measure) are often chosen by the researchers – not by the parents/children. I want to know what outcomes are important to you, that you think should be included in research. Here is a list of outcomes that researchers have used in clinical trials (show item 1). Feel free to ask me any questions about these. And as you go through, you can talk about what you are thinking as you are making the choices.
- Then there is a space for you to add other outcomes. What other outcomes are important for researchers to include in their studies to help you make decisions about treatment – why?

7. Support

- Do you get support from family/school/community? – details

- How could support be improved for parents caring for a child with JDM
- What kind of information or support do you think is important for families living with a child diagnosed with JDM?
- What advice would you give to families who have just found out their child has JDM?

8. Close: Is there something else that you think is important to add?

Item 1. Outcomes

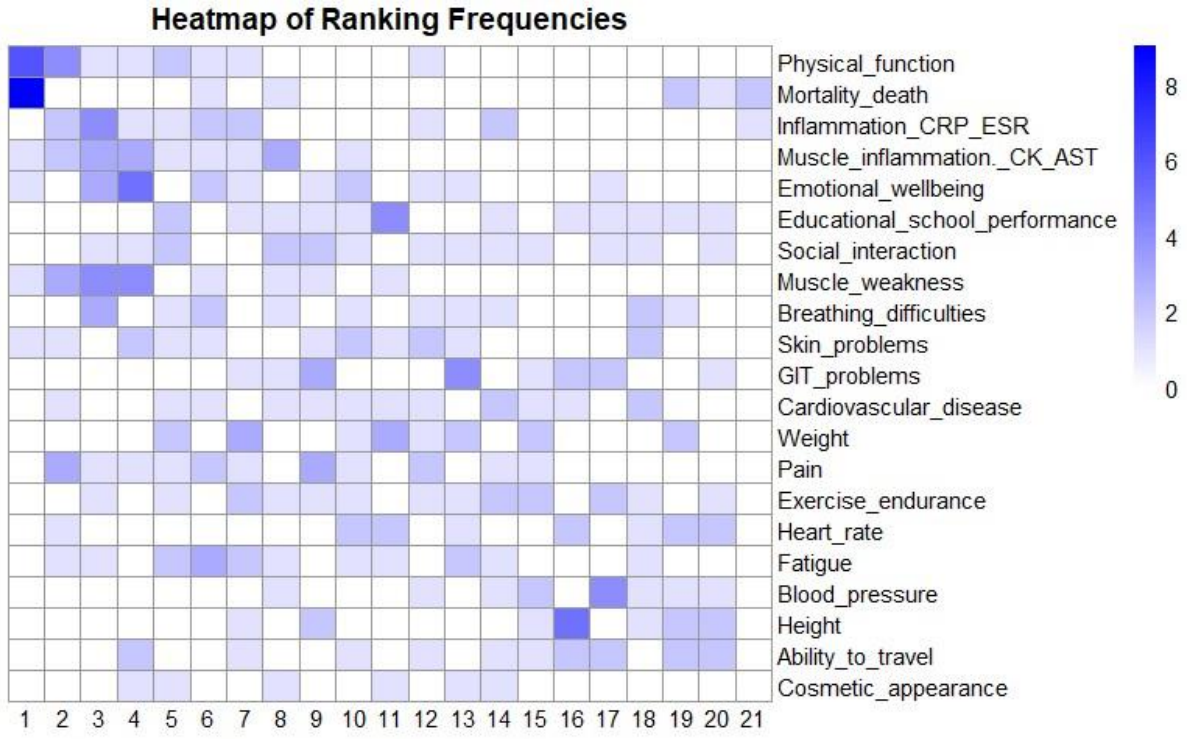
Rank	Outcomes
	Physical function (ability to do daily tasks eg dress, eat, shower)
	Mortality / death
	Inflammation (CRP, ESR)
	Muscle inflammation (AST, CK)
	Emotional wellbeing (including mood, anxiety, depression)
	Educational/ school performance
	Social wellbeing (interaction with peers, relationships)
	Muscle weakness
	Breathing difficulties
	Skin problems
	Gastrointestinal problems (eg. nausea, vomiting)
	Cardiovascular disease
	Weight
	Pain
	Exercise endurance (ability to play sport)
	Heart rate
	Fatigue (feeling tired with no energy)
	Blood pressure
	Height
	Cosmetic appearance
	Ability to travel (visit family, friends)

What other outcomes do you think are important to measure in research?

_____

_____

Appendix Part D: Supplementary material for Chapter 3, additional results, depicting a heatmap of ranking frequencies.



Supplementary material for Chapter 3: Mathematical formula for nominal group technique ranking

The rankings from the nominal group technique produced ordinal data. We used a measure of importance (i.e. importance score) for each outcome to prioritize the outcomes, based on the attributed rankings. To calculate this measure, the distribution of the ranking for each outcome was obtained by calculating the probability of each rank for each outcome. Using mathematical notation, this is written as $P($ in rank $)$, i.e., the probability of the outcome being assigned the rank i . Thus, for each outcome, we obtained the probability of being ranked in first place, in second place, and so on. By the total law of probabilities, these probabilities were decomposed as:

$$P(O_j \text{ in rank } i) =$$

$$= P(O_j \text{ in rank } i \,|O_j \text{ is nominated}) \times P(O_j \text{ is nominated})$$

$$+ P(O_j \text{ in rank } i \,|O_j \text{ not nominated}) \times P(O_j \text{ not nominated})$$

where "nominated" meant the outcome was given a rank by the participant. We assumed that the $P($ in rank | not nominated) was 0 , because if the participant did not rank the outcome , then the probability of any rank was 0 . Therefore, the expression above simplified to:

$$P(O_j \text{ in rank } i) = P(O_j \text{ in rank } i \,|O_j \text{ is nominated}) \times P(O_j \text{ is nominated})$$

We therefore observed that the probability had two components: 1) the importance given to the outcome by the ranking and 2) the consistency of being nominated by the participants. We then used these probabilities and computed the weighted sum of the reciprocal ranking ( ) to obtain the importance score (IS):

$$IS = \sum_{i=1}^{nr\ of\ outcomes} P(O_j\ in\ rank\ i) \times \frac{1}{i}$$

The importance score can be interpreted as a summary measure of importance of the outcome that incorporates the consistency of being nominated and the rankings given by participants. The ranks were inverted to give more weight to higher ranks and less to lower ranks. Scores ranged between zero and one, and higher scores identified outcomes that were more valued by participants. This measure had a similar motivation to the Expected Reciprocal Rank Evaluation Metric proposed in a different context.

[24] The importance scores were also calculated separately by country, gender, age, and for patients and caregivers. The analysis was conducted using the software package R version 3.2.3 (R Foundation for Statistical Computing, Vienna, Austria).

Appendix Part E: Supplementary material for chapter 5, interview guide.

DEMOGRAPHICS Date: __ __ __ __ / __ __ / __ __ __ __ Location: _____

Sex:	□ Male	□ Female	□ Unspecified	□ Prefer not to say	
Age:	_____	Date of birth:			
Highest level of education:	□ Primary school	□ High/secondary Professional school before 10th Grade	□ High/secondary school up to 10th grade (School grade/HSC	□ High/secondary school (12th vocational schoo certificate)	□ certificate, e.g. Diploma
□	Undergraduate/Bachelor's Degree	□ Postgraduate Degree (Masters/PhD)			
Employment status:	□ Full Time	□ Part time/casual	□ Student	□ Not employed	□ Voluntary
Type of work?	_____				
Marital status:	□ Married	□ Living with partner/de facto	□ Partner (not living with)	□ Divorced	□ Separated
	□ Widowed	□ Single			
Who do you live with?	□ Housemates	□ Parents	□ Family	□ Partner	□ No one
Number of children/dependents:	_____				
(For patients/caregivers only) What is the rheumatic disease you or your child has been diagnosed with?	□ Rheumatoid Arthritis	□ Osteoarthritis	□ Ankylosing Spondylitis	□ Polymyalgia Rheumatica	□ I don't know
	□ other				
Age when diagnosed?	□ 0-10 years	□ 11-18 years	□ 18- 25years	□ 26-40 years	
	□ 41-55 years	□ 56-65 years	□ 66+ years		
Are you/they on any medications?	□ prednisone	□ anti-inflammatories	□ methotrexate	□ plaquenil	□ sulfasalazine
	□azathioprine	□ biologic (eg. Adalimumab, etanercept, certolizumab…?)			

Interview Guide COVID-19 AND Telehealth:

Introduction

- Could you all, individually, briefly tell me about your experience with a rheumatic disease/ treating patients with rheumatic disease prior to the pandemic.
- How does your rheumatic disease usually affect your life? Before the pandemic?
- What services did you access for care of your rheumatic disease pre COVID-19? Eg Telehealth?

Access to Medical Services During the COVID-19 Pandemic:

1. How did the health services you had previously accessed or provided change with the onset of the Pandemic?
    - Has there been any difficulty accessing this care?
    - What were the restrictions? Ie. Needed COVID test prior to accessing, wearing masks….
2. Have you used telehealth services?
    - Telephone
    - Webcam
    - How? At home, at GPs, at a relatives/friend's, hospital (patient and HCP)

Satisfaction with Services:

Were you satisfied with the services available?

- Have these services changed following the pandemic?

2. Do you think these services impacted on the care you received or provided (for better or worse?)
    - How?

Knowledge of Social Distancing measures

1. Are you following social distancing recommendations (it is ok if you're not, this is not a trick question!)
    - If yes/ no; why?
    - How careful/ relaxed are you with these measures? Why?

Knowledge of Rheumatic Disease and COVID-19

1. What do you understand your risk is or the risk generally for patients with rheumatic disease and COVID-19 is?

- Has your willingness to access medical services been affected by your knowledge of rheumatic disease and COVID-19? If so, why?

2. What do you understand your risk is or the risk generally for patients with rheumatic disease on medications (including immune suppressants) is?

- Have these medications also affected your willingness to access medical services?

3. What resources have you accessed regarding information about Rheumatic diseases and COVID-19?

4. What do you think is the role of vaccinations in rheumatic diseases? Do you have any concerns regarding vaccinations and yourself, your loved ones or your patients?

COVID-19 Pandemic and mood

1. Has the COVID-19 pandemic and/ or the restrictions affected your mood or your patients' generally?
    - Why? Not seeing family, socially isolated, poorer physical health
    - Has your mood changed throughout the pandemic?
    - Hope fo future? Vaccinations?

Close

Is there anything else that you think might be important to mention?

References

1. Harris JG, Bingham CA, Morgan EM. Improving care delivery and outcomes in pediatric rheumatic diseases. Curr Opin Rheumatol 2016;28:110–6.
2. Forestier B, Anthoine E, Reguiai Z, Fohrer C, Blanchin M. A systematic review of dimensions evaluating patient experience in chronic illness. Health Qual Life Outcomes 2019;17:19.
3. Phillips RL, Short A, Kenning A, Dugdale P, Nugus P, McGowan R, et al. Achieving patient-centred care: The potential and challenge of the patient-as-professional role. Health Expect 2015;18:2616–28.
4. Loesken C, Maehder K, Buck L, Hartl J, Löwe B, Schramm C, et al. Understanding illness experiences of patients with primary sclerosing cholangitis: A qualitative analysis within the soma.Liv study. BMC Gastroenterol 2023;23:12.
5. Chauhan K, Jandu JS, Brent LH, Al-Dhahir MA. Rheumatoid arthritis. Statpearls. Treasure Island (FL): StatPearls Publishing Copyright © 2026, StatPearls Publishing LLC.; 2026.
6. Barton JL, Katz P. The patient experience: Patient-reported outcomes in rheumatology. Rheum Dis Clin North Am 2016;42:xv–xvi.
7. Coda A, Jones J, Grech D, Grewal DS. Survey of parent and carer experiences and expectations of paediatric rheumatology care in new south wales. Aust Health Rev 2017;41:372–7.
8. Sawhney S, Magalhães CS. Paediatric rheumatology--a global perspective. Best Pract Res Clin Rheumatol 2006;20:201–21.
9. Alivernini S, Firestein GS, McInnes IB. The pathogenesis of rheumatoid arthritis. Immunity 2022;55:2255–70.
10. McCann LJ, Kirkham JJ, Wedderburn LR, Pilkington C, Huber AM, Ravelli A, et al. Development of an internationally agreed minimal dataset for juvenile dermatomyositis (jdm) for clinical and research use. Trials 2015;12.
11. Ramanan AV, Feldman BM. Clinical outcomes in juvenile dermatomyositis. Curr Opin Rheumatol 2002;14:658–62.
12. Tollisen A, Sanner H, Flatø B, Wahl AK. Quality of life in adults with juvenile-onset dermatomyositis: A case-control study. Arthritis Care & Research 2012;64:1020–7.
13. Apaz MT, Saad-Magalhaes C, Pistorio A, Ravelli A, de Oliveira Sato J, Marcantoni MB, et al. Health-related quality of life of patients with juvenile dermatomyositis: Results from the paediatric rheumatology international trials organisation multinational quality of life cohort study. Arthritis & Rheumatism: Arthritis Care & Research 2009;61:509–17.
14. Ramanan A. Therapeutic advances in juvenile idiopathic arthritis. Rheumatology 2011;50:iii25.
15. Botto-van Bemden A, Adebajo AO, Fitzpatrick CM. Patient and public involvement in rheumatic and musculoskeletal research: An idea whose time has firmly come. BMC Rheumatol 2023;7:12.
16. Australia A. Enhancing research through meaningful consumer engagement. 2026 [updated 2026; cited]; Available from: https://arthritisaustralia.com.au/programs-research/national-research-program/consumer-engagement/.
17. Cochrane. Chapter 18: Patient reported outcome measures. [cited]; Available from: https://www.cochrane.org/authors/handbooks-and-manuals/handbook/current/chapter-18#:~:text=A%20patient%2Dreported%20outcome%20(PRO,(Powers%20et%20al%202017).
18. Tong A, Crowe S, Chando S, Cass A, Chadban SJ, Chapman JR, et al. Research priorities in ckd: Report of a national workshop conducted in australia. American journal of kidney diseases : the official journal of the National Kidney Foundation 2015;66:212–22.
19. Ardalan K, Marques MC, Cella D, Curran ML, Gray EL, Lee J, et al. Psychometric properties of patient-reported outcomes measurement information system (promis) fixed short forms in juvenile myositis. Semin Arthritis Rheum 2025;71:152649.

20. Kelly AH, Singh-Grewal D, Sumpton D, Hasset G, Manera KE, Tong A. Range and consistency of outcome measures reported in randomised trials in dermatomyositis: A systematic review. Clin Exp Rheumatol 2022;40:358–65.
21. Tong A, Jones J, Craig JC, Singh-Grewal D. Children's experiences of living with juvenile idiopathic arthritis: A thematic synthesis of qualitative studies. Arthritis Care Res (Hoboken) 2012;64:1392–404.
22. Jamieson N, Fitzgerald D, Singh-Grewal D, Hanson CS, Craig JC, Tong A. Children's experiences of cystic fibrosis: A systematic review of qualitative studies. Pediatrics 2014;133:e1683–97.
23. Taylor PC, Moore A, Vasilescu R, Alvir J, Tarallo M. A structured literature review of the burden of illness and unmet needs in patients with rheumatoid arthritis: A current perspective. Rheumatol Int 2016;36:685–95.
24. Shende V, Wagh V. Role of telemedicine and telehealth in public healthcare sector: A narrative review. Cureus 2024;16:e69102.
25. Pylypchuk Y, Barker W. Use of telemedicine among office-based physicians, 2021. Astp health it data brief. Washington (DC): Office of the Assistant Secretary for Technology Policy; 2012. p. 1–16.
26. Agency ADH. digitalhealthgovau 2025.
27. Australia S. 2025 [updated 2025; cited]; Available from: https://www.servicesaustralia.gov.au/telehealth-billing-codes-for-mbs-items?context=20#:~:text=Medicare%20Benefit%20Schedule%20(MBS)%20telehealth%20(video%20and%20phone),have%20different%20MBS%20item%20numbers.
28. Leung AKC, Lam JM, Alobaida S, Leong KF, Wong AHC. Juvenile dermatomyositis: Advances in pathogenesis, assessment, and management. Curr Pediatr Rev 2021;17:273–87.
29. Wu JQ, Lu MP, Reed AM. Juvenile dermatomyositis: Advances in clinical presentation, myositis-specific antibodies and treatment. World J Pediatr 2020;16:31–43.
30. Kelly AH, Kelly A, Singh-Grewal D, Chaitow J, Jaure A. Perspectives and experiences of parents of children with juvenile dermatomyositis: A semi-structured interview study. Pediatric rheumatology online journal 2025;23:34.
31. Rider LG, Aggarwal R, Machado PM, Hogrel JY, Reed AM, Christopher-Stine L, et al. Update on outcome assessment in myositis. Nat Rev Rheumatol 2018;14:303–18.
32. Oddis CV, Reed AM, Aggarwal R, Rider LG, Ascherman DP, Levesque MC, et al. Rituximab in the treatment of refractory adult and juvenile dermatomyositis and adult polymyositis: A randomized, placebo-phase trial. Arthritis and Rheumatism 2013;65:314–24.
33. Giancane G, Lavarello C, Pistorio A, Oliveira SK, Zulian F, Cuttica R, et al. The printo evidence-based proposal for glucocorticoids tapering/discontinuation in new onset juvenile dermatomyositis patients. Pediatric rheumatology online journal 2019;17:24.
34. McCann LJ, Pilkington CA, Huber AM, Ravelli A, Appelbe D, Kirkham JJ, et al. Development of a consensus core dataset in juvenile dermatomyositis for clinical use to inform research. Ann Rheum Dis 2018;77:241–50.
35. Kountz-Edwards S. The psychosocial impact of juvenile dermatomyositis on pediatric patients and parents. Dissertation Abstracts International: Section B: The Sciences and Engineering 2016;77:No Pagination Specified.
36. Kountz-Edwards S, Aoki C, Gannon C, Gomez R, Cordova M, Packman W. The family impact of caring for a child with juvenile dermatomyositis. Chronic Illn 2017;13:262–74.
37. Grazziotin LR, Currie G, Twilt M, MJ IJ, Kip MMA, Koffijberg H, et al. Factors associated with care- and health-related quality of life of caregivers of children with juvenile idiopathic arthritis. Pediatric rheumatology online journal 2022;20:51.
38. Chapelle O, Metlzer D, Zhang Y, Grinspan P. Expected reciprocal rank for graded relevance. Proceedings of the 18th ACM conference on Information and knowledge management; 2009; Hong Kong, China. Association for Computing Machinery; 2009. p. 621–30.
39. Braun V, Clarke V. Using thematic analysis in psychology. Qualitative Research in Psychology 2006;3:77–101.
40. Campbell S, Moola FJ, Gibson JL, Petch J, Denburg A. The unspeakable nature of death & dying during childhood: A silenced phenomenon in pediatric care. Omega (Westport) 2024;89:88–107.
41. Rider LG, Werth VP, Huber AM, Alexanderson H, Rao AP, Ruperto N, et al. Measures of adult and juvenile dermatomyositis, polymyositis, and inclusion body myositis: Physician and patient/parent global activity, manual muscle testing (mmt), health assessment questionnaire (haq)/childhood health assessment questionnaire (c-haq),

childhood myositis assessment scale (cmas), myositis disease activity assessment tool (mdaat), disease activity score (das), short form 36 (sf-36), child health questionnaire (chq), physician global damage, myositis damage index (mdi), quantitative muscle testing (qmt), myositis functional index-2 (fi-2), myositis activities profile (map), inclusion body myositis functional rating scale (ibmfrs), cutaneous dermatomyositis disease area and severity index (cdasi), cutaneous assessment tool (cat), dermatomyositis skin severity index (dssi), skindex, and dermatology life quality index (dlqi). Arthritis Care Res (Hoboken) 2011;63 Suppl 11:S118–57.
42. Zigler CK, Randell RL, Reeve BB. Assessing patient-reported outcomes in pediatric rheumatic diseases: Considerations and future directions. Rheum Dis Clin North Am 2022;48:15–29.
43. Kirwan JR, Minnock P, Adebajo A, Bresnihan B, Choy E, de Wit M, et al. Patient perspective: Fatigue as a recommended patient centered outcome measure in rheumatoid arthritis. The Journal of rheumatology 2007;34:1174–7.
44. Ringold S, Consolaro A, Ardoin SP. Outcome measures in pediatric rheumatic disease. Rheum Dis Clin North Am 2021;47:655–68.
45. Manera KE, Johnson DW, Craig JC, Shen JI, Ruiz L, Wang AY, et al. Patient and caregiver priorities for outcomes in peritoneal dialysis: Multinational nominal group technique study. Clin J Am Soc Nephrol 2019;14:74–83.
46. Hanson CS, Gutman T, Craig JC, Bernays S, Raman G, Zhang Y, et al. Identifying important outcomes for young people with ckd and their caregivers: A nominal group technique study. American journal of kidney diseases : the official journal of the National Kidney Foundation 2019;74:82–94.
47. Carvajal Bedoya G, Davis LA, Hirsh JM. Patient-reported outcomes in rheumatology patients with limited english proficiency and limited health literacy. Arthritis Care Res (Hoboken) 2020;72 Suppl 10:738–49.
48. Livermore P, Gray S, Mulligan K, Stinson JN, Wedderburn LR, Gibson F. Being on the juvenile dermatomyositis rollercoaster: A qualitative study. Pediatric rheumatology online journal 2019;17:30.
49. Boland L, Graham ID, Légaré F, Lewis K, Jull J, Shephard A, et al. Barriers and facilitators of pediatric shared decision-making: A systematic review. Implement Sci 2019;14:7.
50. Mago A, Aggarwal V, Gupta L. Telerheumatology and its interplay with patient-initiated care. Rheumatol Int 2021;41:1883–4.
51. (UNICEF) Neculau AEea. A guide to telelmedicine in primary healthcare. 2022.
52. Zhu W, De Silva T, Eades L, Morton S, Ayoub S, Morand E, et al. The impact of telerheumatology and covid-19 on outcomes in a tertiary rheumatology service: A retrospective audit. Rheumatology (Oxford) 2021;60:3478–80.
53. Barlas N, Barlas SB, Basnyat S, Adalier E. Telemedicine in rheumatoid arthritis: A review of the pubmed literature. Mediterr J Rheumatol 2023;34:16–23.
54. Gajarawala SN, Pelkowski JN. Telehealth benefits and barriers. J Nurse Pract 2021;17:218–21.
55. Statistics ABo. Patient experiences. 2024.
56. Mathew S, Fitts MS, Liddle Z, Bourke L, Campbell N, Murakami-Gold L, et al. Telehealth in remote australia: A supplementary tool or an alternative model of care replacing face-to-face consultations? BMC Health Serv Res 2023;23:341.
57. Jabour AM. Assessing patient confidence in telehealth: Comparing across 17 medical specialties. Digit Health 2025;11:20552076251330486.
58. Thomas E, Lee CMY, Norman R, Wells L, Shaw T, Nesbitt J, et al. Patient use, experience, and satisfaction with telehealth in an australian population (reimagining health care): Web-based survey study. J Med Internet Res 2023;25:e45016.
59. Rangachari P, Mushiana SS, Herbert K. A narrative review of factors historically influencing telehealth use across six medical specialties in the united states. Int J Environ Res Public Health 2021;18.
60. Nash P. Telemedicine and rheumatology. Joint Bone Spine 2022;89:105439.
61. Osman S, Churruca K, Ellis LA, Luo D, Braithwaite J. The unintended consequences of telehealth in australia: Critical interpretive synthesis. J Med Internet Res 2024;26:e57848.
62. Warr D, Luscombe G, Couch D. Hype, evidence gaps and digital divides: Telehealth blind spots in rural australia. Health (London) 2023;27:588–606.
63. Venuturupalli S, Peck A, Jinka Y, Fortune N, Davuluri N, Nowell WB, et al. Home-based telemedicine in rheumatology-a scoping review. ACR Open Rheumatol 2024;6:312–20.

64. Kong SS, Otalora Rojas LA, Ashour A, Robinson M, Hosterman T, Bhanusali N. Ability and willingness to utilize telemedicine among rheumatology patients-a cross-sectional survey. Clin Rheumatol 2021;40:5087–93.
65. (ACMA) ACaMA. Communications and media in australia series: How we use the internet, executive summary and key findings 2024.

66. Legaspi DMD, Tee CA, Dans LF. Telemedicine usability and satisfaction among pediatric rheumatology patients and their caregivers during covid-19 pandemic. Acta Med Philipp 2024;58:103–9.
67. Alnasser Y, Proaño A, Loock C, Chuo J, Gilman RH. Telemedicine and pediatric care in rural and remote areas of middle-and-low-income countries: Narrative review. J Epidemiol Glob Health 2024;14:779–86.
68. Pooni R, Pageler NM, Sandborg C, Lee T. Pediatric subspecialty telemedicine use from the patient and provider perspective. Pediatr Res 2022;91:241–6.
69. Chan S, Khandaker T, Li Y, Jackson TM, Rahimi-Ardabili H, Lau AY. Translating primary care to telehealth: Analysis of in-person paediatric consultations and role of carers. BJGP Open 2025;9.
70. Chua V, Koh JH, Koh CHG, Tyagi S. The willingness to pay for telemedicine among patients with chronic diseases: Systematic review. J Med Internet Res 2022;24:e33372.
71. Rankine J, Kidd KM, Sequeira GM, Miller E, Ray KN. Adolescent perspectives on the use of telemedicine for confidential health care: An exploratory mixed-methods study. J Adolesc Health 2023;73:360–6.
72. Ward IM, Schmidt TW, Lappan C, Battafarano DF. How critical is tele-medicine to the rheumatology workforce? Arthritis Care Res (Hoboken) 2016;68:1387–9.
73. Bernard L, Valsecchi V, Mura T, Aouinti S, Padern G, Ferreira R, et al. Management of patients with rheumatoid arthritis by telemedicine: Connected monitoring. A randomized controlled trial. Joint Bone Spine 2022;89:105368.
74. Ma Y, Zhao C, Zhao Y, Lu J, Jiang H, Cao Y, et al. Telemedicine application in patients with chronic disease: A systematic review and meta-analysis. BMC Med Inform Decis Mak 2022;22:105.
75. Muehlensiepen F, Petit P, Knitza J, Welcker M, Vuillerme N. Factors associated with telemedicine use among patients with rheumatic and musculoskeletal disease: Secondary analysis of data from a german nationwide survey. J Med Internet Res 2023;25:e40912.
76. Hormaza-Jaramillo A, Arredondo A, Forero E, Herrera S, Ochoa C, Arbeláez-Cortés Á, et al. Effectiveness of telemedicine compared with standard care for patients with rheumatic diseases: A systematic review. Telemed J E Health 2022;28:1852–60.
77. Sloan M, Lever E, Harwood R, Gordon C, Wincup C, Blane M, et al. Telemedicine in rheumatology: A mixed methods study exploring acceptability, preferences and experiences among patients and clinicians. Rheumatology (Oxford) 2022;61:2262–74.
78. Arber S, Sawyer L. The role of the receptionist in general practice: A 'dragon behind the desk'? Soc Sci Med 1985;20:911–21.
79. McDougall JA, Ferucci ED, Glover J, Fraenkel L. Telerheumatology: A systematic review. Arthritis Care Res (Hoboken) 2017;69:1546–57.
80. Spinelli FR, Govoni M, Iannone F, Mosca M, Cauli A, Frediani B, et al. Telemedicine for rheumatological consultation: The new semeiotics for rheumatic examination. Clin Exp Rheumatol 2023;41:993–6.
81. Association AR. Ara rheumatology workforce report february 2023. 2023 [updated 2023; cited]; Available from: https://rheumatology.org.au/Portals/2/Documents/Public/About%20the%20ARA/News%20and%20media/ARA%20Workforce%20Doc_DIGITAL_compressed.pdf.
82. Benveniste O, Rider LG. 213th enmc international workshop: Outcome measures and clinical trial readiness in idiopathic inflammatory myopathies, heemskerk, the netherlands, 18-20 september 2015. Neuromuscul Disord 2016;26:523–34.
83. Regardt M, Mecoli CA, Park JK, de Groot I, Sarver C, Needham M, et al. Omeract 2018 modified patient-reported outcome domain core set in the life impact area for adult idiopathic inflammatory myopathies. The Journal of rheumatology 2019;46:1351–4.
84. Mecoli CA, Park JK, Alexanderson H, Regardt M, Needham M, de Groot I, et al. Perceptions of patients, caregivers, and healthcare providers of idiopathic inflammatory myopathies: An international omeract study. The Journal of rheumatology 2019;46:106–11.
85. (OMERACT) OMiR. Protocol template for a systematic review of measurement properties. March 2025.

86. Webbe J, Sinha I, Gale C. Core outcome sets. Arch Dis Child Educ Pract Ed 2018;103:163–6.
87. DiRenzo D, Bingham CO, 3rd, Mecoli CA. Patient-reported outcomes in adult idiopathic inflammatory myopathies. Curr Rheumatol Rep 2019;21:62.
88. Kluzek S, Dean B, Wartolowska KA. Patient-reported outcome measures (proms) as proof of treatment efficacy. BMJ Evid Based Med 2022;27:153–5.
89. Hoffmann TC, Del Mar C. Patients' expectations of the benefits and harms of treatments, screening, and tests: A systematic review. JAMA Intern Med 2015;175:274–86.
90. Williams Kathryn SJ, Morris Darcy, Grootemaat Pam and, Cristina T. Patient reported outcome measures literature review. Australian Commission on Safety and Quality in Health Care 2016.
91. Rider LG, Yip AL, Horkayne-Szakaly I, Volochayev R, Shrader JA, Turner ML, et al. Novel assessment tools to evaluate clinical and laboratory responses in a subset of patients enrolled in the rituximab in myositis trial. Clinical and Experimental Rheumatology 2014;32:689–96.
92. Cousino MK, Hazen RA. Parenting stress among caregivers of children with chronic illness: A systematic review. J Pediatr Psychol 2013;38:809–28.
93. Orandi AB, Fotis L, Lai J, Morris H, White AJ, French AR, et al. Favorable outcomes with reduced steroid use in juvenile dermatomyositis. Pediatric rheumatology online journal 2021;19:127.
94. Postma L, Luchtenberg ML, Verhagen AAE, Maeckelberghe ELM. The academic impact of paediatric research agendas: A descriptive analysis. Res Involv Engagem 2024;10:97.
95. Hull SC, Oen-Hsiao JM, Spatz ES. Practical and ethical considerations in telehealth: Pitfalls and opportunities. Yale J Biol Med 2022;95:367–70.
96. Ageing Do. 2025 [updated 2025; cited]; Available from: https://www.health.gov.au/topics/health-technologies-and-digital-health/about/telehealth.
97. Wales UoNS. How ai in healthcare is reshaping the future of medicine in australia. 2025 [updated 2025; cited]; Available from: https://www.unsw.edu.au/study/your-future/ai-in-healthcare#:~:text=AI%20is%20revolutionising%20healthcare%20by,with%20data%20and%20AI%20solutions.