

Identifying key health system components associated with improved outcomes to inform the re-configuration of services for adults with rare autoimmune rheumatic diseases: a mixed-methods study



Rosemary J Hollick, Warren R G James, Avril Nicoll, Louise Locock, Corri Black, Neeraj Dhaun, Allyson C Egan, Nicholas Fluck, Lynn Laidlaw, Peter C Lanyon, Mark A Little, Raashid Ahmed Luqmani, Laura Moir, Maureen McBain, Neil Basu



Summary

Background Adults with rare autoimmune rheumatic diseases face unique challenges and struggles to navigate health-care systems designed to manage common conditions. Evidence to inform an optimal service framework for their care is scarce. Using systemic vasculitis as an exemplar, we aimed to identify and explain the key service components underpinning effective care for rare diseases.

Methods In this mixed-methods study, data were collected as part of a survey of vasculitis service providers across the UK and Ireland, interviews with patients, and from organisational case studies to identify key service components that enable good care. The association between these components and patient outcomes (eg, serious infections, mortality) and provider outcomes (eg, emergency hospital admissions) were examined in a population-based data linkage study using routine health-care data obtained from patients with antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis from national health datasets in Scotland. We did univariable and multivariable analyses using Bayesian poisson and negative binomial regression to estimate incident rate ratios (IRRs), and Cox proportional hazards models to estimate hazard ratios (HRs). People with lived experiences were involved in the research and writing process.

Findings Good care was characterised by service components that supported timely access to services, integrated care, and expertise. In 1420 patients with ANCA-associated vasculitis identified from national health datasets, service-reported average waiting times for new patients of less than 1 week were associated with fewer serious infections (IRR 0.70 [95% credibility interval 0.55–0.88]) and fewer emergency hospital admissions (0.78 [0.68–0.92]). Nurse-led advice lines were associated with fewer serious infections (0.76 [0.58–0.93]) and fewer emergency hospital admissions (0.85 [0.74–0.96]). Average waiting times for new patients of less than 1 week were also associated with reduced mortality (HR 0.59 [95% credibility interval 0.37–0.93]). Cohorted clinics, nurse-led clinics, and specialist vasculitis multi-disciplinary team meetings were associated with fewer serious infections (IRR 0.75 [0.59–0.96] for cohorted clinics; 0.65 [0.39–0.84] for nurse-led clinics; 0.72 [0.57–0.90] for specialist vasculitis multi-disciplinary team meetings) and emergency hospital admissions (0.81 [0.71–0.91]; 0.75 [0.65–0.94]; 0.86 [0.75–0.96]). Key components were characterised by their ability to overcome professional tensions between specialties.

Interpretation Key service components associated with important health outcomes and underpinning factors were identified to inform initiatives to improve the design, delivery, and effectiveness of health-care models for rare autoimmune rheumatic diseases.

Funding Versus Arthritis.

Copyright © 2024 The Author(s). Published by Elsevier Ltd. This is an Open Access article under the CC BY 4.0 license.

Introduction

Rare diseases represent a substantial workload for patients and health-care systems, which are designed to manage high volume, low complexity conditions.¹ Service reconfiguration to recognise the disparate needs of patients with rare diseases has been largely confined to genetic paediatric conditions. However, rare autoimmune rheumatic diseases such as systemic vasculitis, systemic lupus erythematosus, myositis, and systemic sclerosis,

comprise an important subgroup of non-genetic rare diseases in adults, with an estimated combined prevalence of 28.8 cases per 10 000 population.²

It is unlikely that a one-size-fits-all model of care would be suitable for patients with rare autoimmune rheumatic diseases due to the different health-care contexts in which care is delivered. However, these clinically heterogeneous conditions share common service delivery challenges.³ These challenges include poor awareness

Lancet Rheumatol 2024;
6: e361–73

Epidemiology Group, Aberdeen Centre for Arthritis and Musculoskeletal Health (R J Hollick PhD, W R G James PhD, L Laidlaw BA, L Moir BSc, M McBain RGN), Health Services Research Unit (A Nicoll PhD, Prof L Locock PhD), and Aberdeen Centre for Health Data Science (Prof C Black MBChB), University of Aberdeen, Aberdeen, UK; NHS Grampian, Foresterhill, Aberdeen, UK (Prof C Black, Prof N Fluck DPhil); British Heart Foundation/University Centre for Cardiovascular Science, University of Edinburgh, Edinburgh, UK (Prof N Dhaun MD); Trinity Health Kidney Centre, Tallaght University Hospital, Dublin, Ireland (A C Egan MRCP); Department of Rheumatology, Nottingham University Hospitals NHS Trust, Nottingham, UK (Prof P C Lanyon DM); Lifespan and Population Health, School of Medicine, University of Nottingham, Nottingham, UK (Prof P C Lanyon); Trinity Kidney Centre, Trinity Translational Medicine Institute, Trinity College Dublin, Dublin, Ireland (Prof M A Little PhD); Nuffield Department of Orthopaedics, Rheumatology and Musculoskeletal Science, University of Oxford, Oxford, UK (Prof R A Luqmani DM); School of Infection and Immunity, University of Glasgow, Glasgow, UK (Prof N Basu PhD)

Correspondence to:
Dr Rosemary J Hollick,
Epidemiology Group, Aberdeen Centre for Arthritis and Musculoskeletal Health, University of Aberdeen, Aberdeen, UK
rhollick@abdn.ac.uk

Research in context

Evidence before this study

Rare autoimmune rheumatic diseases are a clinically heterogeneous group of conditions that share common challenges in service delivery across health-care systems. Substantial variation in care models and outcomes exist but no evidence-based standards or guidelines are available to inform service delivery. To identify empirical studies on experiences and outcomes related to service delivery in rare autoimmune rheumatic diseases, we searched PubMed from database inception to July 10, 2023, without language restrictions, for articles using the search terms "vasculitis", "lupus", "connective tissue disease", "scleroderma", "Sjogren*", "rare autoimmune", "rare disease*", "service*", specific components of vasculitis services ("multidiscip* team", "clinics", "wait*" "quality of care"), professional*", "experience*" and "population-based AND outcome". Our search did not identify any studies that examined the role of specific service components on patient outcomes, with most focusing on individual socioeconomic factors associated with poorer outcomes or diagnostic delays in rare autoimmune rheumatic diseases. One study found that care fragmentation, defined as care delivered at more than one organisation, was associated with increased risk of severe infection, cardiovascular disease, end-stage kidney disease, and stroke in patients with systemic lupus erythematosus. The few qualitative studies that explored patient experiences of care and challenges of service delivery were geographically limited to single centres or region or limited to a single specialty. No studies systematically evaluated in detail, and at a national level, the key aspects of care and outcomes that are important to patients, how patients and staff working across different

specialties caring for rare autoimmune rheumatic diseases experience different patterns of service organisation, and how they believe this impacts on quality of care. Specifically, we found no studies that examined the associations between key components of care, clinical outcomes, and health-care use and the reasons for this across different health-care systems.

Added value of this study

In our mixed methods study, using an exemplar group of rare rheumatic diseases, we have systematically examined how health care is experienced by patients and staff and delivered across diverse health-care systems and identified key service components associated with enhanced clinical outcomes using population-level data. We have explored underpinning factors that explain why key service components are associated with improved outcomes. This includes the ability to overcome jurisdictional boundaries and tensions between specialties, which are rarely acknowledged and actively used to support delivery of rare disease models, and to provide continuity of care.

Implications of all the available evidence

This is the first study to identify key service components that support timely access to services, integrated care, and expertise, and are associated with improved outcomes in an exemplar rare autoimmune rheumatic disease. This provides an evidence base for international strategies to inform development of effective and equitable services across the spectrum of rare rheumatic diseases that will improve patient experiences of care and outcomes.

among health-care professionals; inequalities in timely access to specialist care; shared (and often fragmented) management across multiple medical specialities; and limited access to the wider multi-disciplinary team.⁴

In practice, multiple service delivery models are employed, and clinical outcomes can vary substantially between different centres.⁵ Taken together, there are likely to be service model components that might explain the observed geographical variations in outcomes.⁶ Key components of care organisation and delivery have been shown to be important determinants of clinical outcomes in other long-term conditions, such as stroke⁷ and cancer⁸—eg, reorganisation of care pathways to provide rapid access to expertise, cohorted care (ie, grouping together people with the same condition and treating them in one place) in acute stroke units, and management within multi-disciplinary team meetings. However, the effect of key components of care organisation and delivery on outcomes are unknown in the context of rare autoimmune rheumatic diseases. Specifically, identification of key service elements, qualitatively informed by patient and provider experience, and quantitatively associated with favourable patient and

health service outcomes, have yet to be systemically identified and evaluated at a population level.

Moreover, understanding why key components might be associated with improved outcomes has not been systematically explored. The divergence of health-care structure, policy, and care provision across the four health systems within the UK provides unique opportunities to explore this across different health-care contexts, with internationally transferable lessons for diverse health-care systems.

In this study, we aimed to identify and explain key service components underpinning effective care to inform an optimal service framework for rare autoimmune rheumatic diseases. Our objectives were first, to explore the experiences of patients and document patterns of service components provided across different service contexts (phase 1). Second, to understand from the perspective of a vasculitis service provider, the delivery of service components across different health-care contexts and how these impact on quality of care and outcomes (phase 2). The perspectives of patients and vasculitis service providers were integrated to understand what good care looked like and the key service components that

supported this. Finally, we tested if these key components were associated with improved health outcomes and explored why this might be the case (phase 3).

Methods

Study design and participants

In this mixed methods study, we collected both qualitative and quantitative data (phases 1–3) that were iteratively analysed and integrated at several stages. This approach offers insights to improve care that would not otherwise be possible from the use of either method alone.

Systemic vasculitis served as an exemplar group of rare autoimmune rheumatic diseases since variation in their health care and outcomes is established⁸ and their multi-system nature enhances clinical generalisability to other rare autoimmune rheumatic diseases. To test the association between service components and individual health outcomes, we focused on antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis, a large subgroup of systemic vasculitides that are identifiable with high sensitivity and specificity using International Classification of Diseases (ICD)-10 codes within administrative health-care data. The survey was registered as a service evaluation on the Scottish Research Management Database (reference number 4960). Ethical approval for the patient qualitative interviews was obtained from the Berkshire Research Ethics Committee (12/SC/0495). Ethical approval for case studies and health-care professional interviews was obtained from the University of Aberdeen School Ethics Review Board (CERB/2021/3/2055). Approvals for data linkage were obtained from the Public Benefit and Privacy Panel for Health and Social Care, Scotland (1819-0069; appendix p 14). All survey, patient interview, and case study participants provided electronic, written, or verbal informed consent.

Patient research partners living with systemic vasculitis were involved throughout the study as outlined in the Guidance for Reporting Involvement of Patients and the Public 2 reporting framework (appendix pp 15–17). A core group of three patients were involved at a strategic level within the study management team, while they and a further 13 patients took part in specific tasks either via email or through meetings and workshops. Patient partners contributed to initial conceptualisation and study design, designing patient and vasculitis provider interview guides, advising on themes for analysis, offering sense and sensitivity checks on a freely available web resource for patients, created using patient interview data, reflecting on emerging findings from all work packages, presenting at conferences and identifying implications for practice. Specific examples include two small group online discussions (three patient partners in each) to refine the case study proposals (eg, they highlighted the importance of including pharmacists), and two patient partners who attended three hour-long online meetings with AN to support the focused analysis

of patient data (eg, they identified the importance of ‘feeling safe’).

Procedures

In phase 1 of our study, data were collected in parallel: we used a two-part online survey of vasculitis service providers across the UK and Ireland (appendix p 3) and did narrative interviews with people with systemic vasculitis. The provider survey aimed to describe different service models and components of service drawing on the European Reference Network for Rare Immunodeficiency, AutoInflammatory and AutoImmune Disease criteria for meeting vasculitis centre of excellence standards.⁹ To maximise coverage across the UK and Ireland, the survey was distributed to the service leads of all 87 UK and Ireland Vasculitis Society registered centres (mainly located in England, Wales, and Ireland) and all 14 regional National Health Service (NHS) Health Boards responsible for health-care delivery in Scotland, between Nov 2, 2020, and June 30, 2021. Registration as a UK and Ireland Vasculitis Society centre is voluntary and services are not required to meet specific service criteria.

People with systemic vasculitis across the UK were recruited for interview via charities, social media, and NHS services. The in-depth, narrative interviews took place between June 1, 2020, and June 2, 2021, and aimed to understand experiences of systemic vasculitis and of health care, including the participant’s experience of vasculitis; its impact on their life; and ideas for how services could be improved (appendix pp 4–5).

Vasculitis service provider survey responses provided a list of service components (including their availability and perceptions of importance from a staff perspective). We used this as a framework for an analysis of patient interview data focused on understanding what people valued and what good care looked like. This enabled us to move from an a-priori list of service components (survey questions) to prioritising the key service components from a patient and staff perspective.

Data about vasculitis service components and what good care looked like informed case study site selection for phase 2. We approached sites where we anticipated that at least some of the vasculitis service components were already part of routine practice but might be achieved in different ways. We also ensured that site selection would enable comparison at a macro level (policy and governance in Scotland and England), meso level (organisation of health-care in different areas), and micro level (vasculitis service models).

We conducted six comparative case studies¹⁰ in the UK, which involved interviews with providers in a range of roles contributing to vasculitis care locally. To understand the wider service context, we also identified and approached national leaders in systemic vasculitis for interview. Interviews, which were completed between Oct 11, 2021, and June 22, 2023, explored experiences of different patterns of vasculitis service organisation,

See Online for appendix

For more on the **web resource** see <https://healthtalk.org/introduction/systemic-vasculitis/>

knowledge of how services and their components had developed over time, and understanding of how services are coordinated. We also sought to understand perceptions of how this impacted on quality of care and outcomes and what might improve care further (appendix pp 6–8).

Provider survey data (including list of service components and perceptions of importance), and patient and provider interview data were integrated to refine the list of key service components that supported good care.

In phase 3 of our study, the associations between key service components, clinical outcomes, and health-care utilisation were examined in a population-based cohort of patients with ANCA-associated vasculitis (adults aged ≥ 16 years with ≥ 1 standardised ICD-10 code for granulomatosis with polyangiitis [M31.3], microscopic polyangiitis [M31.7], or eosinophilic granulomatosis with polyangiitis [M30.1]) identified from national hospital admission records in Scotland and linked to other national health datasets:¹¹ outpatient hospital attendances, registries for death and cancer, and records of all medications dispensed in community care (appendix p 9). Records were collected from April 1, 1996, until Oct 30, 2020. Data linkage was conducted by the NHS Scotland electronic Data Research and Innovation Service via deterministic linkage methods using unique personal identification numbers in a process shown to produce highly accurate and complete data.¹² Additional sensitivity analyses were conducted for cohort validation with similar results. In addition to the presence of relevant ICD-10 codes for systemic vasculitis, we identified relevant outpatient department visits (dermatology, ear, nose, and throat, immunology, neurology, ophthalmology, renal medicine, respiratory medicine, rheumatology, and vascular surgery) within the first year after index date, and relevant prescriptions of disease-modifying drugs (including azathioprine, mycophenolate, and methotrexate) and prednisolone from community prescribing data. Prescribing data on biological drugs and intravenous therapies such as rituximab and cyclophosphamide delivered within hospital settings are not currently accessible nationally within routinely collected health-care datasets in Scotland.

The presence or absence of key service components was allocated to individual cases based on their main speciality provider (defined by the study team and clinical advisory group as where $>50\%$ of their outpatient care was received) and corresponding survey responses from their healthboard of treatment. Associations between exposure to key service components and clinical outcomes (eg, serious infection requiring hospital admission, cardiovascular disease, cancer, and death; the most common comorbidities of ANCA-associated vasculitis) and health-care utilisation (eg, emergency hospital admissions) were measured using previously validated ICD-10 codes¹³ (appendix pp 10–11). Patients were excluded if their main speciality provider could not be determined. Outcomes identified before the index date were classified as pre-existing conditions of interest

and excluded from analysis. This so-called look-back period has previously been shown to accurately enable incident morbidities to be distinguished from prevalent morbidities.¹⁴

We then further examined the vasculitis provider survey and interview data to understand the availability of key service components associated with improved outcomes and how these key components made a difference to people's care across different health-care settings.

Statistical analysis

All qualitative interviews with patients and vasculitis service providers were conducted by AN using topic guides created in partnership with our patient partners. Interviews were transcribed verbatim and analysed thematically using a deductive and inductive approach,¹⁵ using NVivo coding software (version 12). AN led the analysis of the interviews, guided by analytic conversations¹⁶ with patient partners and a wider advisory panel of third sector representatives and clinicians. Existing theory was used to enhance understanding. For example, theories about the work patients must do to access and benefit from treatment,¹⁷ the jurisdictions of control created by professional work which, over time, shape what care is and is not provided, and by whom,¹⁸ and the factors influencing sustainability of health-care services.¹⁹

Integration of provider survey and patient and provider interview data used a following a thread strategy.²⁰ A summary of the survey and interview data was presented at a series of meetings with researchers, clinicians, and patient partners. Analytical conversations facilitated a comparative analysis and identification of overarching themes that were important to patients and clinicians to define what good care looks like, what this looked and felt like in practice, and the key service components that supported good care.

The survey data was summarised descriptively with counts and percentages. Baseline characteristics of patients with ANCA-associated vasculitis were descriptively summarised. To investigate the association between service components and key outcomes, an initial univariable analysis involved several models where only one of the self-reported service components was entered as a term adjusted for sociodemographic variables and all two-way interactions. A subsequent multivariable analysis included all service components as terms to control for the presence or absence of other service components and estimate the independent effect of each service component. Additionally, the multivariable analysis included a random intercept by health board of treatment to account for variation due to health boards. The two-way interaction terms were removed from this analysis because they did not improve model fit. Due to the frequent co-existence of nurse-led clinics and nurse-led advice lines within services, these terms were combined into a single variable if at least one was present (appendix p 12).

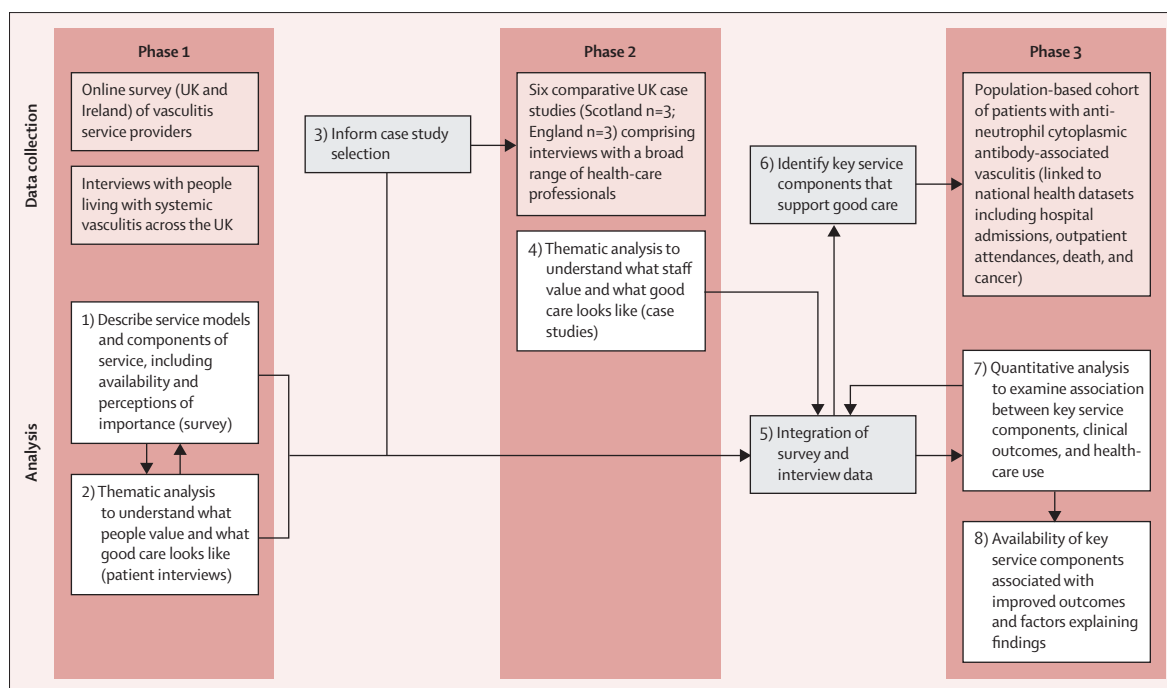


Figure 1: Mixed-method study design

This illustrates the sequential phases (1-3) of data collection and analysis, and where quantitative and qualitative data were integrated. The outputs from each phase are described and how they were used to inform subsequent phases of data collection, where relevant.

All models were adjusted for age at index date, sex, local area measure of deprivation (quintiles), and the Scottish Government urban rural classification. The univariable models included all two-way interactions whereas the multivariable only included main effects. Age was median centred and scaled such that one unit of change corresponded to a decade, and sex was deviation coded so the intercept term represented the average for males and females. Scottish Index of Multiple Deprivation scores were categorised into quintiles (quintile 1 corresponded to the most deprived area and quintile 5 corresponded to the most affluent area) with quintile 3 used as the reference category. Years of follow-up was used as the exposure term to account for varying amounts of follow-up (due to death, leaving Scotland, or no data being collected after Oct 31, 2020).

Both univariable and multivariable analyses used a Bayesian Cox proportional hazards model to estimate mortality. In the initial univariable analysis, rates (total counts accounting for length of time in the study) of serious infection, cardiovascular disease, cancer, and emergency hospital admissions were estimated using a Bayesian Poisson regression. The univariable analysis was initially done to assess the association between key service components and health outcomes. Based on these findings, we reassessed the qualitative data to explore why the key service components were associated with improved health outcomes. However, when we subsequently conducted the multivariable analysis to investigate the independent effect of service components,

we found that the assumptions for the Poisson regression (ie, mean and variance value were the same) was not met. To account for this, a negative binomial regression model was fitted to the data and found to better describe the data (assessed by performing a Leave One Out Cross Validation on models with the same specification). Results are reported as hazard ratios (HRs) and incidence rate ratios (IRRs), with mean posterior value and 95% credibility intervals (CrIs), with 50% CrIs also provided in the multivariable analysis. Bayesian analysis provides an estimate of a distribution of effect rather than the fixed point estimates seen with frequentist methods. Bayesian CrIs do not have the same mathematical interpretation as frequentist CIs and can be interpreted to mean, for example, there is a 95% chance the true value lies within this range given the data. This provides information about the uncertainty, precision, and likely association of the effect as opposed to a dichotomous interpretation of statistical significance. The priors for all coefficients in each model were set to be weakly informative using a Student's *t* distribution with the degrees of freedom set as 3, the mean as 0, and the standard deviation (SD) as 1. The prior for the intercept term used the default behaviour for the brms package, whereby, the Student's *t* mean value was the expected response value if all predictors were set to a value of 0.

All data processing (appendix p 13) was done using R (version 4.3.1) using the tidyverse library (version 2.0.0). All analysis was conducted using the brms library (version 2.20.4).

For the **Urban Rural Classification** see <https://www.gov.scot/publications/scottish-government-urban-rural-classification-2020/pages/2/>

For the **Scottish Index of Multiple Deprivation** see <https://www.gov.scot/collections/scottish-index-of-multiple-deprivation-2020/>

	Vasculitis service providers, n/N (%)
Timely access to services	
Average wait time for new patients with suspected systemic vasculitis within 1 week	46/56 (82%)
Specialist vasculitis nurse in service	29/56 (52%)
Nurse-led advice line	27/56 (48%)
Access to own day-case unit	33/56 (59%)
Urgent access to intravenous treatment within 1 week	42/56 (75%)
ANCA results within 24 h if required	31/33 (94%)
Integrated care delivery	
Cohorted clinics*	43/56 (77%)
Joint or parallel clinics with other specialties	32/56 (57%)
Wait time for returning patients within 1 week	41/56 (73%)
Nurse-led clinic	20/56 (36%)
Vasculitis inpatients managed by dedicated team	16/56 (29%)
Vasculitis inpatients managed by individual specialty	47/56 (84%)
Access to expertise	
Access to any multi-disciplinary team meeting	45/56 (80%)
Access to local speciality-specific or organ-specific multi-disciplinary team only	17/45 (38%)
Access to vasculitis multi-disciplinary team	28/45 (62%)
Data	
Data entry into at least one national registry	43/51 (84%)
Local database of vasculitis patients	23/51 (45%)
ANCA=antineutrophil cytoplasmic antibody. *Patients with systemic vasculitis are grouped together and seen in a dedicated clinic.	
Table 1: Access to vasculitis service components across UK and Ireland	

Role of the funding source

The funder supported interview recruitment by distributing advertisements to people with systemic vasculitis via social media but had no role in data collection, data analysis, data interpretation, or writing of the manuscript.

Results

For this study, we collected both qualitative and quantitative data across 3 phases, that were iteratively analysed and integrated at several stages (figure 1). In phase 1, 56 survey responses were received from 48 trusts or health boards across Scotland (n=11), England (n=30), Wales (n=2), and Ireland (n=5). This included equal numbers of respondents from adult nephrology and rheumatology services working across tertiary referral centres, teaching, and district general hospitals.

The frequency of access to different service components varied (table 1). Timely access to diagnostic services was consistently high, with 46 (82%) of 56 services reporting being able to see new patients with suspected systemic vasculitis on average within 1 week of referral and 31 (94%) of 33 providers offering ANCA results within

24 h. There was variability in access to nurse-led service components, which typically benefit established patients, and access to a dedicated day-case unit for delivery of biologic and cytotoxic treatment (33 [59%] of 56 providers).

45 (80%) of 56 services had the opportunity to discuss patients with vasculitis at a multi-disciplinary team meeting, most commonly local speciality meetings, with only 28 (62%) of 45 services reporting some access to a specialist vasculitis multi-disciplinary team. There was considerable variation in the frequency with which meetings were held (usually monthly or less frequently), activities undertaken, available resources, and administrative support. As an outcome of multi-disciplinary team meetings, 19 (42%) of 45 services reported that they often proposed changes in patient management; 14 (31%) often recommended prescription of biologics; and ten (22%) often requested additional investigations.

The top three components of care prioritised by all specialties were support for specialist nurse-led care (eg, nurse-led advice line and nurse-led clinics), delivery of timely biologic and cytotoxic drug infusions (access to day-case units and urgent access to intravenous treatments within 1 week), and support for multi-disciplinary team meetings (access to any multi-disciplinary team; local speciality-specific or organ-specific multi-disciplinary team; vasculitis multi-disciplinary team; appendix p 18).

We conducted narrative interviews with 32 people (nine men, 23 women) across the UK (Scotland [n=16], England [n=11], Wales [n=3], and Northern Ireland [n=2]) with different forms of systemic vasculitis (60 h of interviews; appendix p 19). Ages ranged from 22 to 81 years, and time since diagnosis from less than 1 year to 18 years. Of the 32 people interviewed, 17 (53%) had a degree level education. At the time of interview, 12 (38%) of 32 people were in full or part-time employment or education and seven (22%) were living alone. We made targeted efforts to recruit people of colour with systemic vasculitis; the resulting interview with a participant from a minority ethnic group reinforced our understanding that wider participation makes research more relevant.

In phase 2, six organisational case studies, three from Scotland and three from England collected data on multiple staff perspectives. We conducted 67 narrative interviews with a broad range of health-care professionals (63 h of interviews; appendix p 20).

From the patient and provider interviews, we identified three overarching themes important to patients and clinicians that defined what good care looks like (timely response to illness, continuity of care and support for shared decision making), what this looked and felt like in practice, and the different types of work patients and clinicians had to do to make this possible (appendix p 21).

The list of survey components assessed was subsequently refined to identify key service components that supported timely response to illness, continuity of care, and support for shared decision making, enabling

patients and staff to be in the loop, combine and connect care, and feel safe (appendix p 22). For example, a key theme was around continuity of care, and this was underpinned by coordination, communication, and relationship work. From the survey data and case studies, service components focused on integrated care delivery were important to achieving this, particularly the ability to see all patients with vasculitis in the same clinic, the role played by specialist vasculitis nurses in joining up care and an awareness of care provided by other specialties for people with vasculitis. This enabled us to identify key service components that supported integrated care delivery (cohorted clinics [ie, the ability to see all patients with vasculitis in a specific clinic], nurse-led clinics, and integrated local and regional vasculitis care).

In phase 3, the association between key service components and health outcomes was measured using national administrative datasets, apart from integrated local and regional vasculitis care and access to out of hours and unscheduled care, which were not possible to accurately quantify from survey data.

In total, 1931 people with ANCA-associated vasculitis (median age at index date 61.2 years [lower and upper 95th percentiles 22.6–84.6]; 965 men, 966 women) were identified and followed up for a median of 6.5 years (lower and upper 95th percentiles 0.1–23.4; table 2). The duration of follow-up reflects patients leaving the study for one of several reasons including: death, no longer registered within NHS Scotland, or study end period (Oct 31, 2020).

Of 1931 people with ANCA-associated vasculitis who were included in the study, we were able to assign care to a specific service for 1420 patients (defined as >50% outpatient care in a relevant specialty). Among the 511 patients for whom we were unable to assign care to a specific service, in most cases this was because the length of follow-up was short (appendix p 23). The number of patients with access to each service component is shown in the appendix (p 24).

We found associations between key service components and emergency hospital admissions (figure 2). In terms of transparent and timely access to services, waiting times of less than 1 week for new patients (IRR 0.78 [95% CrI 0.68–0.92]) and access to a nurse-led advice line (0.85 [0.74–0.96]) were associated with fewer emergency hospital admissions. For integrated care delivery, cohorted clinics (0.81 [0.71–0.91]) and nurse-led clinics (0.75 [0.65–0.94]) were associated with fewer emergency hospital admissions. Access to expertise components, including access to a local specialty or organ-specific multi-disciplinary team meeting (0.75 [0.63–0.88]) and specialist vasculitis multi-disciplinary team meeting (0.86 [0.75–0.96]), were associated with fewer emergency hospital admissions.

We also identified associations between key service components and serious infection (appendix p 25).

	Patients with ANCA-associated vasculitis (n=1931)
Sex	
Male	965 (50.0%)
Female	966 (50.0%)
Age, years	61.2 (22.6–84.6)
Follow-up, years	6.6 (0.09–23.4)
Local area deprivation (quintiles)*	
1 (most deprived)	340 (17.6%)
2	366 (19.0%)
3	376 (19.5%)
4	420 (21.8%)
5 (least deprived)	429 (22.2%)
Location†	
Rural	402 (20.8%)
Urban	1529 (79.2%)

Data are n (%) or median (IQR). ANCA=antineutrophil cytoplasmic antibody.
*Local area deprivation measured using the Scottish Index of Multiple Deprivation. †Location (urban or rural) classified using the Scottish urban rural classification.

Table 2: Baseline sociodemographic characteristics of cohort with ANCA-associated vasculitis identified from national administrative datasets in Scotland

Transparent and timely access to services components such as waiting times of less than 1 week for new patients (IRR 0.70 [95% CrI 0.55–0.88]) and access to a nurse-led advice line (0.76 [0.58–0.93]) were associated with fewer serious infections. Having a specialist vasculitis nurse was also associated with fewer serious infections, but with greater uncertainty around the estimate (0.82 [0.66–1.01]). For integrated care delivery, there were fewer serious infections in patients cared for in services with cohorted clinics (0.75 [0.59–0.96]), joint or parallel clinics (0.75 [0.60–0.95]), and nurse-led clinics (0.65 [0.39–0.84]). Access to expertise, characterised by access to a specialist vasculitis multi-disciplinary team meeting, was associated with fewer serious infections (0.72 [0.57–0.90]).

Some key service components were also associated with a reduction in mortality, such as waiting times of less than 1 week for new patients (HR 0.59 [95% CrI 0.37–0.93]; appendix p 26) and integrated care delivery components such as cohorted (0.64 [0.43–1.05]), joint or parallel (0.72 [0.50–1.10]), and nurse-led clinics (0.61 [0.28–1.05]), however there was greater uncertainty in these estimates. Similar patterns were observed for cancer and cardiovascular disease outcomes (appendix pp 27–28).

The final multivariable models aimed to estimate the independent association between the rate of key health outcomes for each service component. Some service components commonly co-existed within individual services, particularly the presence of nurse-led components of care (nurse-led clinics, specialist nurses, advice lines; appendix p 12), which were combined for this analysis. The overlapping nature of the delivery of

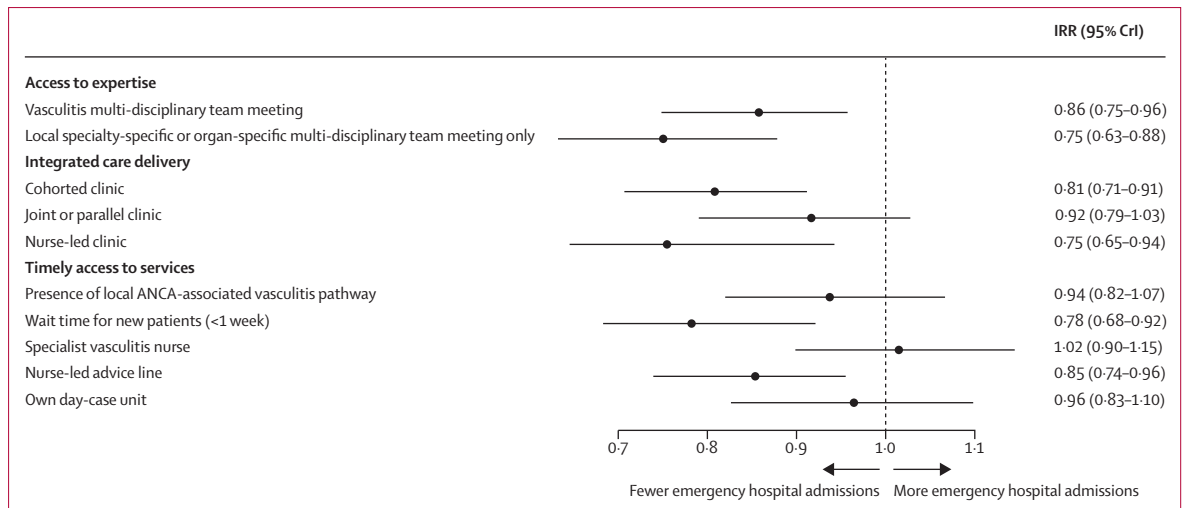


Figure 2: Associations between service components and emergency hospital admissions in patients with systemic vasculitis (n=1420)
 Bayesian Poisson regression, adjusted for age, sex, area-based measure of deprivation, and rural or urban location and all two-way interactions, where IRRs of <1 indicated a reduction in health outcomes of interest. IRR=incidence rate ratio. CrI=credibility interval. ANCA=antineutrophil cytoplasmic antibody.

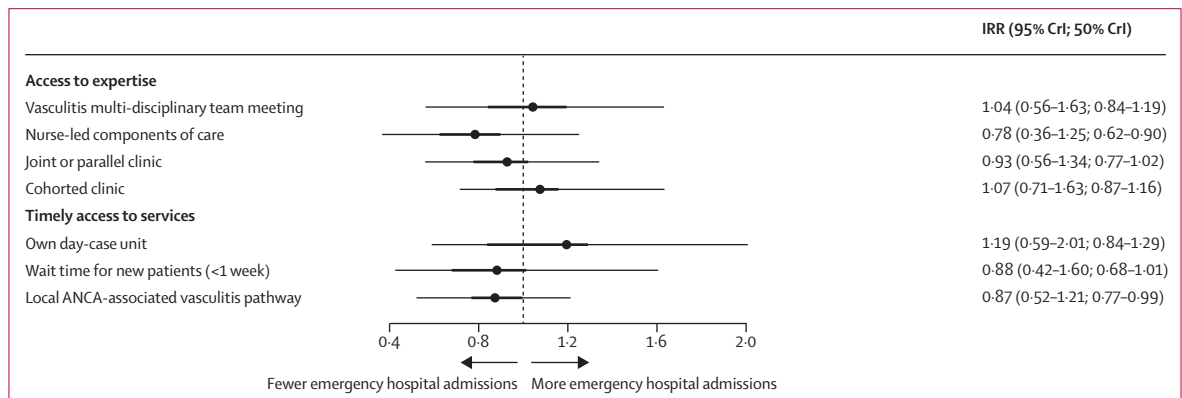


Figure 3: Independent associations between key service components and rate of emergency hospital admissions (n=1420)
 Bayesian negative binomial regression adjusted for age, sex, area-based measure of deprivation, and rural or urban location, with a random intercept term for health board of treatment. The thin horizontal lines represent 95% CrIs, the thick lines show the 50% CrIs, and the points show the mean posterior estimate. CrI=credibility interval. ANCA=antineutrophil cytoplasmic antibody.

service components meant that there was considerable uncertainty in the estimates of effects. However, average new patient wait times less than 1 week were consistent with fewer emergency hospital admissions (IRR 0.88 [95% CrI 0.42-1.60; 50% CrI 0.68-1.01]; figure 3), serious infections (IRR 0.83 [0.40-1.53; 0.62-0.96]; appendix p 29), and reduced risk of mortality (HR 0.50 [95% CrI 0.15-1.10; 50% CrI 0.29-0.57]; appendix p 30). Nurse-led components of care were associated with fewer emergency hospital admissions (IRR 0.78 [0.36-1.25; 0.62-0.90]) and reduced risk of mortality (HR 0.80 [0.26-1.58; 0.53-0.95]; figure 3; appendix p 30).

The estimated rates for cancer and cardiovascular disease were lower for individuals who were cared for within a service with an average waiting time for new

patients of less than 1 week (appendix pp 31-32). To add context to the IRR values presented, an alternative scaling for each of the rate outcomes is included in the appendix (pp 33-36).

The case studies and patient interview data were used to explain why specific service components might be associated with improved clinical and health outcomes (table 3). Average wait times of less than one week for new patients were underpinned by a considerable investment in logistics and a network of relationships to make timely access and treatment a priority: “when somebody is sick ... the wheels move very fast, and decisions are made quickly for patients who need that ... I think the loveliest thing about a service is when you have the availability to go. Now” (nephrologist). However, another nephrologist explained that they had tried to develop a system to reduce

delays in patients being referred to the right speciality, “But the problem was that it was quite person dependent between myself and a rheumatologist, and it didn’t really work.”

Cohorted clinics, whether established as a single speciality or multi-speciality venture, allowed clinicians to think about the particular needs of patients with systemic vasculitis. Such clinics enabled a systematic approach to care and were conducive to “building that long-term trust and relationship for the bumpy bits in the future” (nephrologist). Cohorted clinics also provided a means to make the medical complexity of systemic vasculitis more manageable compared with seeing these patients in general clinics, where “it does just sort of bring you up...I’m not just asking about your joints, and your skin and your [disease-modifying antirheumatic drugs]; I do need to think about your [ear, nose, and throat] system, your respiratory system, [gastrointestinal], neurological” (rheumatologist).

Vasculitis specialist nurses and nurse-led advice lines provided ways to improve patients’ access to services and research, including time and space to explore their perceived needs with someone who “can seem a bit more approachable as a person for the patients” (respiratory consultant). The role of vasculitis specialist nurses in clinical care, supporting mental wellbeing and in organising, coordinating, and escalating care was described in ways that suggested they were “the glue” (ear, nose, and throat registrar) that holds together care that is inherently fragmented, as they are “sort of stitching the whole thing together a bit more” (respiratory consultant).

Vasculitis multi-disciplinary team meetings varied considerably in their set-up and function across different health-care settings. In contexts where they worked well, multi-disciplinary team meetings could provide “that

little bit of focus” on systemic vasculitis, “allow dialogue” between specialties, “improve all our knowledge about what’s current”, and build personal relationships so that “it becomes much easier to then just lift the phone or ping an email to somebody who you have already made a link with” (rheumatologist). Multi-disciplinary team meetings could also be used locally to troubleshoot local service processes, or regionally to facilitate interdisciplinary care.

The key service components were underpinned by effort, often driven by a self-appointed vasculitis champion, to build relationships and address the often unacknowledged jurisdictional tensions between specialties. Nephrology and rheumatology are the two specialties most likely to lead vasculitis care but were likened to “two tribes” with “different views on how to treat and different experiences in the kind of patients they see” (nephrologist).

Where these key components were available, patients also experienced them as an improved relationship with a service that made them feel safe: “I felt really looked after...I really trusted that they were looking after me and they were doing the best they can and they would do everything they can.” This experience of attending cohorted clinics and having access to vasculitis specialist nurses was often in contrast to how patients had felt before their diagnosis.

Discussion

Key service components, grounded in patient and staff experiences and characterised by timely response to illness, continuity of care, and support for shared decision making, are associated with important health outcomes for rare autoimmune rheumatic diseases. Service-reported average waiting times for new patients of less than 1 week were associated with fewer serious infections, emergency

	Patient experiences when key service components present	Professional experiences when key components present
Service-reported average wait times <1 week for new patients	“I had such an incredibly intuitive [general practitioner] who did the bloods that day, and...an amazing team at the [hospital], to quickly read those results and to fast-track that treatment for me. Because I do know that they saved my kidneys.”	“the [general practitioner] might think vasculitis, [and] the [ANCA] result would be emailed to me. The [general practitioner], regardless of the ANCA, might email me directly, or if there’s an [ear, nose, and throat] problem, lung problem, kidney problem, they might email those specialists and those specialists might then reply to the [general practitioner] copying me in.”
Cohorted clinics	“what I do like about the way they go about their business ... every time I go in, they take a urine sample, a blood sample ... and ... they reach out to the [ear, nose, and throat] department, or to maybe the chest department, or speech [and language therapy]...”	“eventually I started to understand more of what was actually happening in the different departments. ... everyone is going to be clear what actually is expected from your department to provide in the care of the patients... those negotiations, as well as sometimes arguments between the clinicians, can be taken out of the equation.”
Vasculitis specialist nurse and nurse-led advice line	“If I’m not sure on anything I ring her and I’ve got a direct number... so if I’m thinking, ‘Hang on, that doesn’t look right...’ Or there was a doctor looked at my bloods and he says, ‘Oh, I think you should stop this [medication]...’...and then the vasculitis nurse rang me and said, ‘No, that figure’s good.’”	“I think a lot of my role ... [is] the more psychological, counselling role and actually, before I did this job, I didn’t foresee that that would be such a major part of the role, but it really is. I think that’s probably I would say a good 80% of what I do in my clinic, is counselling them through their disease. Which is important.”
Vasculitis multi-disciplinary team meeting	“The renal consultants and the rheumatology consultants work as a multidisciplinary team for people with vasculitis. They talk to each other before they make a decision, such as whether I need another rituximab treatment.”	“Often there’ll be four different potential avenues and all may have advantages and disadvantages... we discuss these with the patient either before or after the [multi-disciplinary team]...we also get an [multi-disciplinary team] consensus...and then say to the patients, ‘These are the risks, what feels better to you?’ and sometimes they’ll say, ‘This’, and sometimes they’ll say, ‘Oh, I really don’t know, whatever you say.’ So these are the occasions that it’s even more important to include an [multi-disciplinary team] approach.”

ANCA=antineutrophil cytoplasmic antibody.

Table 3: Understanding why key service components are associated with improved clinical and health outcomes

hospital admissions, and reduced mortality. Nurse-led service components were associated with fewer serious infections and emergency hospital admissions. Key service components were characterised by their ability to support development of relationships and overcome professional tensions between specialties, and fostered a sense of feeling safe for patients.

The role of clinical nurse specialists in facilitating continuity of care has been well described in cancer care, and to a lesser extent rheumatology, however, there has been little evidence to date of the impact of clinical nurse specialists on individual patient outcomes. For example, clinical nurse specialists have been shown to have a key role in developing therapeutic relationships over time, and coordination and so-called brokering roles with a range of different professionals to support timely access to care.²¹ In rheumatology, clinical nurse specialists have been shown to add value to patient care by providing rapid access and high vigilance in relation to patient outcomes and drug management.²² In cancer care, patients who reported having a named clinical nurse specialist were found to have better experiences with care coordination, involvement in treatment decisions, and overall care experiences and improved survival.²³

Patients cared for within services with access to specialist vasculitis multi-disciplinary team meetings had improved outcomes when compared with patients within services without access to these meetings. However, across vasculitis services there were considerable variation in the types of multi-disciplinary team meetings attended, activities undertaken, and degree of administrative support and it was not possible to determine the key components of an effective specialist vasculitis multi-disciplinary team meeting. In contrast to complex medical multi-disciplinary team meetings where evidence is scarce, the function and effectiveness of multi-disciplinary team meetings have been studied extensively within cancer care where the timeliness, cost of care, and adherence to national guidelines is improved for patients managed within an multi-disciplinary team.²⁴ The characteristics of an effective cancer multi-disciplinary team have been clearly defined,²⁵ focused on the team, infrastructure, organisation, logistics, patient centred decision making, and team governance. Nurses also play an important role, particularly in patient advocacy. However, incorporation of patients' views, preferences, and needs into the decision making process, effective communication of decisions to patients, and regular review of multi-disciplinary team decisions were identified as gaps in both cancer and specialist vasculitis multi-disciplinary team meetings.

The key service components associated with improved clinical and health-care use outcomes were underpinned by their ability to overcome jurisdictional tensions and facilitate continuity of care and timely access to expertise. Jurisdictional boundaries and tensions between specialties, notably rheumatology and renal specialties,

have shaped the evolution of vasculitis services in different hospitals. In turn, this has shaped pathways into services, access to expertise, and sustainability. The multicultural nature of health-care organisations, with diverse professions, departments, and teams is a recognised barrier to implementation of evidence-based practices.²⁶ Although social relations and group influences are recognised, improvement efforts are largely focused on organisational infrastructure and processes of care. The role of professional boundaries and so-called jurisdictions of control¹⁸ and the work of staff and patients in coordinating care across these professional boundaries²⁷ is rarely openly acknowledged and actively used to support new collaborative relationships and implementation of rare disease care models. We did not identify any examples of patient involvement in the establishment of vasculitis services or service provision.

Continuity of care also underpinned several key service components, facilitating the communication, coordination, and relationship-building and maintaining work essential to support good care. Continuity of care commonly refers to the ongoing therapeutic relationship between an individual clinician and patient (relational continuity), however, it also describes coherence and consistency of care within and between teams (management continuity) and knowledge of patients' care and situation (informational continuity).²⁸ Relational continuity has been shown to be most valued by patients and staff, particularly for those with chronic conditions²⁹ and is linked to trust and quality of communication,³⁰ including empathy and humanity and receiving information in an understandable format. In other rare autoimmune rheumatic diseases, Sloan and colleagues³¹ found that feeling medically supported was positively correlated with mental wellbeing and perceptions of care.

The importance of time is reflected in all key components associated with improved outcomes: time to see a new referral; and time to access specialist expertise when needed. This is reflected in common conditions, such as stroke, cancer, and heart disease where components of care organisation and delivery shown to be important determinants of clinical outcomes similarly reflect rapid access to services and expertise. These service components are also represented in national service standards and audits, and public and health-care professional messaging for stroke and heart disease (eg, so-called door to needle time); however, to date there has been no similar messaging or national audits in rare autoimmune rheumatic diseases that might support more timely access to expertise.

Despite this study being methodologically rigorous, some limitations exist. Not all services approached responded to the survey, with potential for bias from respondents who represented more well-developed services. However, we aimed to provide a representative sample of patient, health-care professional, and service perspectives as opposed to mapping every vasculitis

service in the UK and Ireland. We received responses from a geographically broad range of service providers across rheumatology and renal specialties, ranging from highly specialised tertiary referral centres to small services based in district general hospitals. Similar to other studies using coded electronic health record data, there is the potential for diagnostic misclassification when using ICD-10 codes for case ascertainment, including lack of granularity and dependence on hospital admission. However, individuals with ANCA-associated vasculitis are highly likely to have had a hospital admission and individual diseases have ICD-10 codes that delineate them from other conditions. ICD-10-coded data has been successfully used as part of a multisourced approach in adult-onset rare rheumatic conditions.³² Furthermore, our aim was not to identify all patients with ANCA-associated vasculitis in Scotland but to obtain a representative sample. Administrative health-care datasets in Scotland have more than 90% population coverage, thus, we were effectively able to correlate exposure to key service components with clinical outcomes and health-care use at a population level.

The provider survey provided a snapshot in time of vasculitis service provision in 2020 and 2021, but the study time frame for population level clinical outcome and health-care use data was 1996 to 2020. Although local services change over time and not every patient will necessarily have been exposed to all service components over their care journey, repeat analysis of patients with an index date of 2015 onwards showed similar associations between key service components and outcomes. Furthermore, there has been no major reconfiguration of vasculitis services in Scotland during the study period. We also recognise that the data are UK-based, which is distinct in terms of funding compared with other countries. However, we have leveraged rarely exploited opportunities for comparative analysis and cross-border learning within a national health-care system, created by the divergence of health-care structure, policy, and care across UK devolved nations. This has highlighted similarities in how key service components work across different health-care contexts and findings that are internationally relevant.

Mixed method research presents several challenges, in terms of articulating the iterative nature and link between phases to an audience who might be less familiar with one or other methodology, and being open to the charge of selective reporting when presenting only extracts of rich and complex qualitative data. However, used appropriately, mixed methods offer explanatory potential and clinical relevance. Our freely available systemic vasculitis online resource illustrates topics of importance to patients with a variety of experiences of systemic vasculitis and health care, making decisions about medication and coordination and organisation of vasculitis care.

These findings inform national and international configuration of vasculitis services. For the first time, identification of key service components associated with improved clinical and health-care provider outcomes provides evidence to inform clinical service standards and guidelines. Certain service components are interdependent and often co-exist together within services, making it difficult to determine their independent effects. However, we have identified specific features, such as waiting times for new patients within 1 week and nurse-led components of care, which are independently associated with fewer serious infections, emergency hospital admissions, and lower risk of mortality. Considering the substantial funding challenges facing health-care systems in the UK and internationally, this evidence will guide prioritisation and delivery of key service components. It also offers opportunities for considerable cost savings for health-care systems in terms of fewer emergency admissions and serious infections. Insights to support integrated care delivery at individual, professional, organisational, and policy level across multiple sites and regions can facilitate creation of sustainable services that manage rare autoimmune conditions, as opposed to disease and organ-specific services. We plan to analyse data from our comparative case studies and use data from patient interviews for a companion paper exploring factors relevant to implementation of key service components.

Reflecting the shared challenges of delivering care to patients with ANCA-associated vasculitis, findings are also likely to be applicable to other rare autoimmune rheumatic diseases. The mixed methodological approach used in this study can be upscaled and applied to a broader range of rare diseases, and more common rheumatic conditions. Incorporating key service and experience outcomes into national administrative health datasets, registries, and rheumatic disease cohorts will provide a framework for rigorous and timely evaluation of different service models across different health-care contexts and identify optimal combinations of service components. Patients must continue to play an integral role going forward, helping to create impactful findings grounded in patient experience to improve care delivery and outcomes.

In conclusion, we have identified key service components supporting timely access to services, integrated care, and access to expertise associated with improved clinical outcomes and health-care utilisation in an exemplar group of rare rheumatic diseases, along with insights into their common underpinning elements across diverse health-care systems. These results might be applicable more widely to health-care services for people living with multisystem rare conditions.

Contributors

NB conceptualised the study, acquired funding, contributed to the methodology, formal analysis, supervision, and writing, reviewing, and editing of the manuscript. CB conceptualised the study, acquired funding, contributed to the methodology, formal analysis, and writing, reviewing, and editing the manuscript. ND, AE, and NF curated data, and contributed to writing, reviewing, and editing the

For more on the systemic vasculitis online resource see healthtalk.org

manuscript. RH conceptualised the study, acquired funding, contributed to the methodology, formal analysis, and supervision, wrote the original draft, and wrote, reviewed, and edited the manuscript. WJ contributed to the methodology, curated data, contributed to data visualisation and formal analysis, wrote the original draft, and wrote, reviewed, and edited the manuscript. LL contributed to methodology, curated data, did formal analysis, and wrote, reviewed, and edited the manuscript. PL curated data and wrote, reviewed, and edited the manuscript. MAL conceptualised the study, acquired funding, contributed to the methodology, and wrote, reviewed, and edited the manuscript. LLo conceptualised the study, acquired funding, contributed to the methodology, formal analysis wrote the original draft, and wrote, reviewed, and edited the manuscript. RL curated data and wrote, reviewed, and edited the manuscript. MM was involved with formal analysis, and writing, reviewing, and editing the manuscript. LM was the project administrator. AN contributed to methodology, curated data, was involved in data visualisation and formal analysis, wrote the original draft, and wrote, reviewed, and edited the manuscript. All authors agreed to the final draft of the manuscript and publication.

Declaration of interest

NB has received speaker fees and research funding from VSL Vifor. RJH has received funding for the present study from Versus Arthritis; is clinical lead of the Scottish Systemic Vasculitis Managed Clinical Network; and has received speaker fees from CSL Vifor. PCL is co-chair of the Rare Autoimmune Rheumatic Disease Alliance (RAIRDA), the joint national clinical lead for the Rheumatology, Getting It Right First Time programme, NHS England, and the clinical lead for Rare Diseases, National Disease Registration Service, NHS England; has received speaker fees from CSL Vifor and Pfizer; and expenses for attending conferences and research funding from CSL Vifor. MAL has received speaker fees from CSL Vifor. LL's research time for this study was supported by the Health Services Research Unit, University of Aberdeen, which received core funding from the Chief Scientist's Office (Scotland). All other authors declare no competing interests.

Data sharing

Patient interviews are archived with the Medical Sociology and Health Experiences Researcher Group, University of Oxford (Oxford, UK); they are all copyrighted to the University of Oxford and available, under licence, to qualitative researchers for secondary analysis (subject to approval and administrative costs). Access to NHS Scotland health data is governed by the NHS Scotland Public Benefit and Privacy Panel for Health and Social Care (HSC-PBPP). Access to the underlying pseudonymised health data used in this study is by application to the HSC-PBPP panel. All code used in the analyses, from data processing to completed results, is available online at https://github.com/warren-james/VOICES_DataProcessingAndAnalysis.

Acknowledgments

Funding for this study was provided by Versus Arthritis (grant number 22088). The authors would like to thank all the patient contributors with lived experience of systemic vasculitis who have been involved in this study. We also wish to acknowledge our collaborators, the UK and Ireland Vasculitis Society, for their support in the conceptualisation and delivery of the study, including development and distribution of the vasculitis provider survey, recruitment to case study sites and for hosting workshops to discuss study findings and inform recommendations. We are also grateful to the European Reference Network for rare immune disorders for their support with development and distribution of the survey to members. We also wish to thank the NHS Scotland electronic Data Research and Innovation Service Team (Public Health Scotland) for their involvement in obtaining approvals, provisioning, and linking data and the use of the secure analytical platform within the National Safe Haven.

References

- 1 Castro R, Senecat J, de Chalendar M, Vajda I, Dan D, Boncz B. Bridging the gap between health and social care for rare diseases: key issues and innovative solutions. In: Posada de la Paz M, Taruscio D, Groft SC, eds. *Rare diseases epidemiology: update and overview*. Cham: Springer International Publishing, 2017: 605–27.
- 2 Leyens J, Bender TTA, Mücke M, et al. The combined prevalence of classified rare rheumatic diseases is almost double that of ankylosing spondylitis. *Orphanet J Rare Dis* 2021; **16**: 326.
- 3 The Lancet Rheumatology. The Lancet Rheumatology. Long-awaited action on rare diseases. *Lancet Rheumatol* 2022; **4**: e229.
- 4 Simpson A, Bloom L, Fulop NJ, et al. How are patients with rare diseases and their carers in the UK impacted by the way care is coordinated? An exploratory qualitative interview study. *Orphanet J Rare Dis* 2021; **16**: 76.
- 5 Tan JA, Dehghan N, Chen W, Xie H, Esdaile JM, Avina-Zubieta JA. Mortality in ANCA-associated vasculitis: a meta-analysis of observational studies. *Ann Rheum Dis* 2017; **76**: 1566–74.
- 6 Action for Rare Disease Empowerment. Making the unseen seen. Rare disease and the lessons learned from the COVID-19 pandemic. <https://www.camraredisease.org/ardent/> (accessed Dec 15, 2023).
- 7 Langhorne P, O'Donnell MJ, Chin SL, et al. Practice patterns and outcomes after stroke across countries at different economic levels (INTERSTROKE): an international observational study. *Lancet* 2018; **391**: 2019–27.
- 8 Adizie JB, Khakwani A, Beckett P, et al. Impact of organisation and specialist service delivery on lung cancer outcomes. *Thorax* 2019; **74**: 546–50.
- 9 European Reference Networks. ERN assessment manual for applicants. Operational criteria for the assessment of healthcare providers. https://ec.europa.eu/research/participants/data/ref/other_eu_prog/hp/guide/pse/hp-asses-manual-ern-op-criteria-hc-providers_en.pdf (accessed Dec 15, 2023).
- 10 Robert K. Yin. Case study research design and methods, 5th edn. Los Angeles, CA: Canadian Journal of Program Evaluation, 2014.
- 11 The Scottish Public Health Observatory. ISD linked database. <https://www.scotpho.org.uk/methods-and-data/overview-of-key-data-sources/scottish-national-data-schemes/isd-linked-database/> (accessed Dec 15, 2023).
- 12 Evans JMM, MacDonald TM. Record-linkage for pharmacovigilance in Scotland. *Br J Clin Pharmacol* 1999; **47**: 105–10.
- 13 Sarica SH, Gallacher PJ, Dhaun N, et al. Multimorbidity in antineutrophil cytoplasmic antibody-associated vasculitis: results from a longitudinal, multicenter data linkage study. *Arthritis Rheumatol* 2021; **73**: 651–59.
- 14 Preen DB, Holman CDJ, Spilsbury K, Semmens JB, Brameld KJ. Length of comorbidity lookback period affected regression model performance of administrative health data. *J Clin Epidemiol* 2006; **59**: 940–46.
- 15 Proudfoot K. Inductive/deductive hybrid thematic analysis in mixed methods research. *J Mixed Methods Res* 2023; **17**: 308–26.
- 16 Looock L, Kirkpatrick S, Brading L, et al. Involving service users in the qualitative analysis of patient narratives to support healthcare quality improvement. *Res Involv Engagem* 2019; **5**: 1.
- 17 May CR, Eton DT, Boehmer K, et al. Rethinking the patient: using burden of treatment theory to understand the changing dynamics of illness. *BMC Health Serv Res* 2014; **14**: 281.
- 18 Abbott A. The system of professions: an essay on the division of expert labour. Chicago, IL: University of Chicago Press, 1988.
- 19 Lennox L, Maher L, Reed J. Navigating the sustainability landscape: a systematic review of sustainability approaches in healthcare. *Implement Sci* 2018; **13**: 27.
- 20 O'Cathain A, Murphy E, Nicholl J. Three techniques for integrating data in mixed methods studies. *BMJ* 2010; **341**: c4587.
- 21 Leary A, Crouch H, Lezard A, Rawcliffe C, Boden L, Richardson A. Dimensions of clinical nurse specialist work in the UK. *Nurs Stand* 2008; **23**: 40–44.
- 22 Oliver S, Leary A. The value of the nurse specialists' role: Pandora initial findings. *Musculoskeletal Care* 2010; **8**: 175–77.
- 23 Alessy SA, Davies E, Rawlinson J, Baker M, Lichtenborg M. Clinical nurse specialists and survival in patients with cancer: the UK National Cancer Experience Survey. *BMJ Support Palliat Care* 2022; published online April 21. <https://doi.org/10.1136/bmjspcare-2021-003445>.
- 24 Freeman RK, Ascoti AJ, Dake M, Mahidhara RS. The effects of a multidisciplinary care conference on the quality and cost of care for lung cancer patients. *Ann Thorac Surg* 2015; **100**: 1834–38.

- 25 National Cancer Registration and Analysis Service. Multi-disciplinary team (MDT) development. The characteristics of an effective multi-disciplinary team (MDT) Development. http://ncin.org.uk/cancer_type_and_topic_specific_work/multidisciplinary_teams/mdt_development (accessed Dec 13, 2023).
- 26 Fitzgerald LDS. Professional boundaries and the diffusion of innovation. In: Dopson S, Fitzgerald L, eds. *Knowledge to action? Evidence-based health care in context*. New York, NY: Oxford University Press, 2005: 104–31.
- 27 Walton H, Hudson E, Simpson A, et al. Defining coordinated care for people with rare conditions: a scoping review. *Int J Integr Care* 2020; **20**: 14.
- 28 Palmer W, Hemmings N, Rosen R, et al. Improving access and continuity in general practice. Nuffield Trust. 2018. <https://www.nuffieldtrust.org.uk/sites/default/files/2019-01/improving-access-and-continuity-in-general-practice-evidence-review-final-update-01-2019.pdf> (accessed Dec 13, 2023).
- 29 Nutting PA, Goodwin MA, Flocke SA, Zyzanski SJ, Stange KC. Continuity of primary care: to whom does it matter and when? *Ann Fam Med* 2003; **1**: 149–55.
- 30 Garrino L, Picco E, Finiguerra I, Rossi D, Simone P, Roccatello D. Living with and treating rare diseases: experiences of patients and professional health care providers. *Qual Health Res* 2015; **25**: 636–51.
- 31 Sloan M, Gordon C, Harwood R, et al. The impact of the COVID-19 pandemic on the medical care and health-care behaviour of patients with lupus and other systemic autoimmune diseases: a mixed methods longitudinal study. *Rheumatol Adv Pract* 2020; **5**: rkaa072.
- 32 Peach E, Rutter M, Lanyon P, et al. Risk of death among people with rare autoimmune diseases compared with the general population in England during the 2020 COVID-19 pandemic. *Rheumatology (Oxford)* 2021; **60**: 1902–09.