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Plain Language Summary

- People with systemic lupus erythematosus experience symptoms that can be disabling and even life-threatening
- Regulators responsible for approving new medications (such as the US Food and Drug Administration) recommend that studies testing new treatments for the disease include suitable questionnaires that ask patients about relevant disease-related symptoms and their impact on their lives
- We looked at recently published studies where adults with systemic lupus erythematosus talked about their symptoms and the impact of the disease on their lives, and found 82 different symptoms (most commonly tiredness, joint and muscle pain, and skin rash) and 41 different impacts on patients’ lives (most commonly how well they can move around, how they feel emotionally, and their relationships with others)
- We used this information to create a model of the patient experience of systemic lupus erythematosus, which will be used to test the suitability of questionnaires for use in clinical studies

Conclusions

- A CDM of the patient experience of SLE that includes 14 symptom dimensions, three proximal impact dimensions, and seven distal impact dimensions was developed based on a literature review
- This CDM is being used to evaluate the suitability of PROs for supporting, in combination with clinical outcomes, the approval of new SLE therapies, as per FDA guidance on patient-focused drug development (poster presenting these data [PCR205] is available via the QR code)

Introduction

- Systemic lupus erythematosus (SLE) is a heterogenous, multisystemic autoimmune disease<sup>1</sup> characterized by symptoms such as fatigue, fever, weight loss, rashes, and joint and muscle pain, as well as life-threatening complications that affect the kidneys, heart, lungs, and central nervous system<sup>2</sup>
- The importance of incorporating patient-reported outcome (PRO) measures in SLE clinical trials is recognized,<sup>3,4</sup> with the US Food and Drug Administration (FDA) recommending the use of a conceptual disease model (CDM) to determine whether existing PRO measures capture the entirety of a concept of interest<sup>5</sup>

Objective

- To develop a CDM of the symptoms and impacts of SLE

Methods

Identification of Patient-Reported Symptoms and Impacts

- A targeted literature review (TLR) was conducted in Embase and Medline to identify English-language full-text publications (published 2018–2023) detailing patient-reported symptoms and the impacts experienced by adults with SLE
- Publications were screened for eligibility using predefined inclusion criteria based on population, intervention/comparator, outcomes, and study design (**Table**)
- Data on patient characteristics, and symptoms and impacts of SLE were extracted from selected qualitative and quantitative publications

CDM Development

- A CDM of SLE symptoms and impacts was developed based on the concepts identified from the TLR
- Thematic analysis was used to group health concepts by common themes into dimensions capturing symptoms and impacts
- Impacts were categorized as ‘proximal’ if they were directly related to SLE or ‘distal’ if they occurred because of a proximal impact

Results

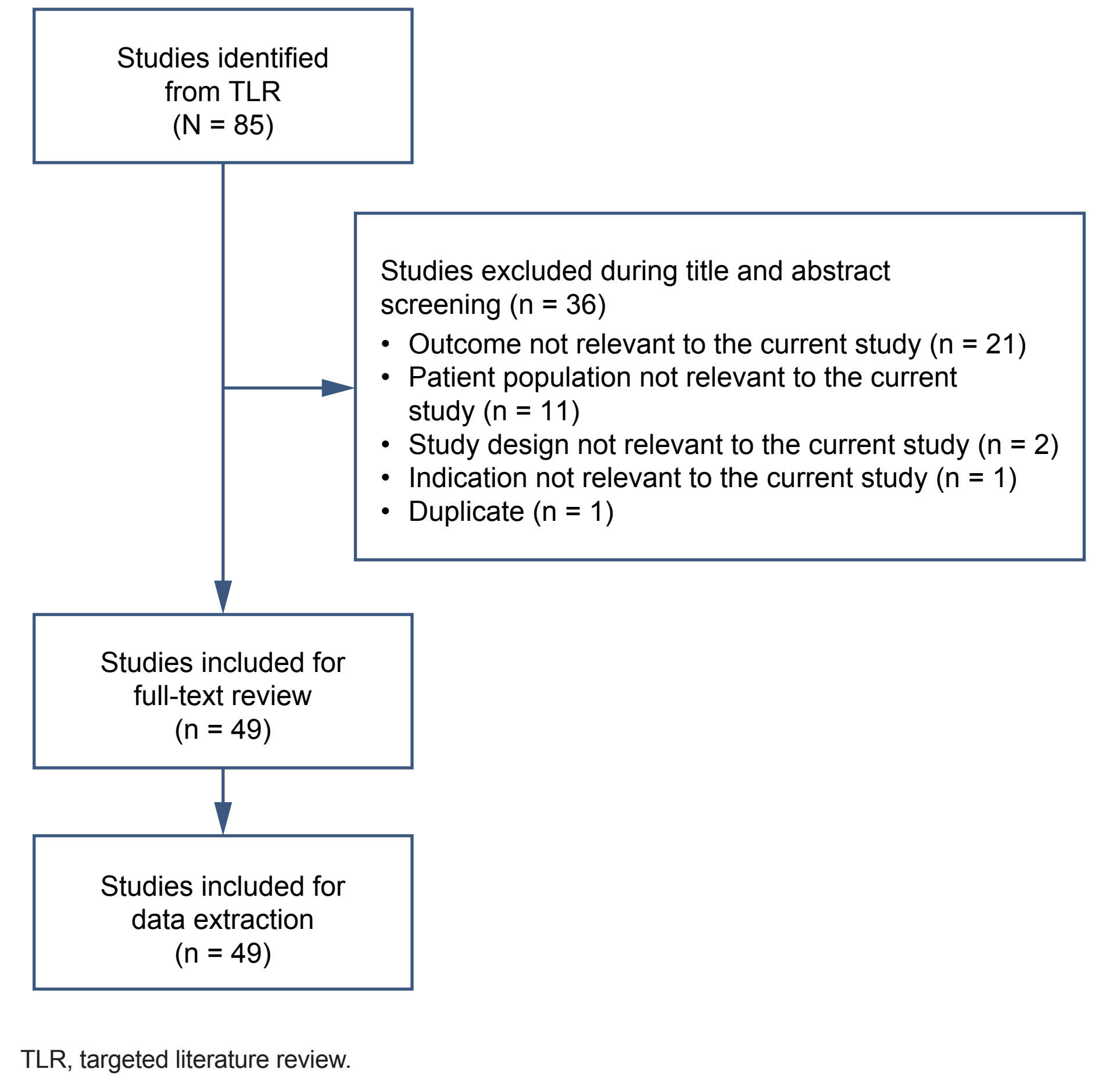
Patient-Reported Symptoms and Impacts

- The TLR identified 49 publications for data extraction (**Figure 1**) describing experiences of patients from the USA (n = 19), the UK (n = 6), Italy (n = 3), France (n = 3), China (n = 2), and Singapore (n = 2)
- In total, 82 symptoms and 41 impacts of SLE were identified
  - The most frequently reported symptoms (in ≥ 10 publications) were fatigue (n = 76), joint pain (n = 38), muscle pain (n = 36), rash (n = 36), and bodily pain (n = 30)
  - The most frequently reported impacts (in ≥ 10 publications) were physical functioning (n = 20), emotional health (n = 16), intimate relationships (n = 11), and cognitive impairment/dysfunction (n = 10)

CDM of SLE

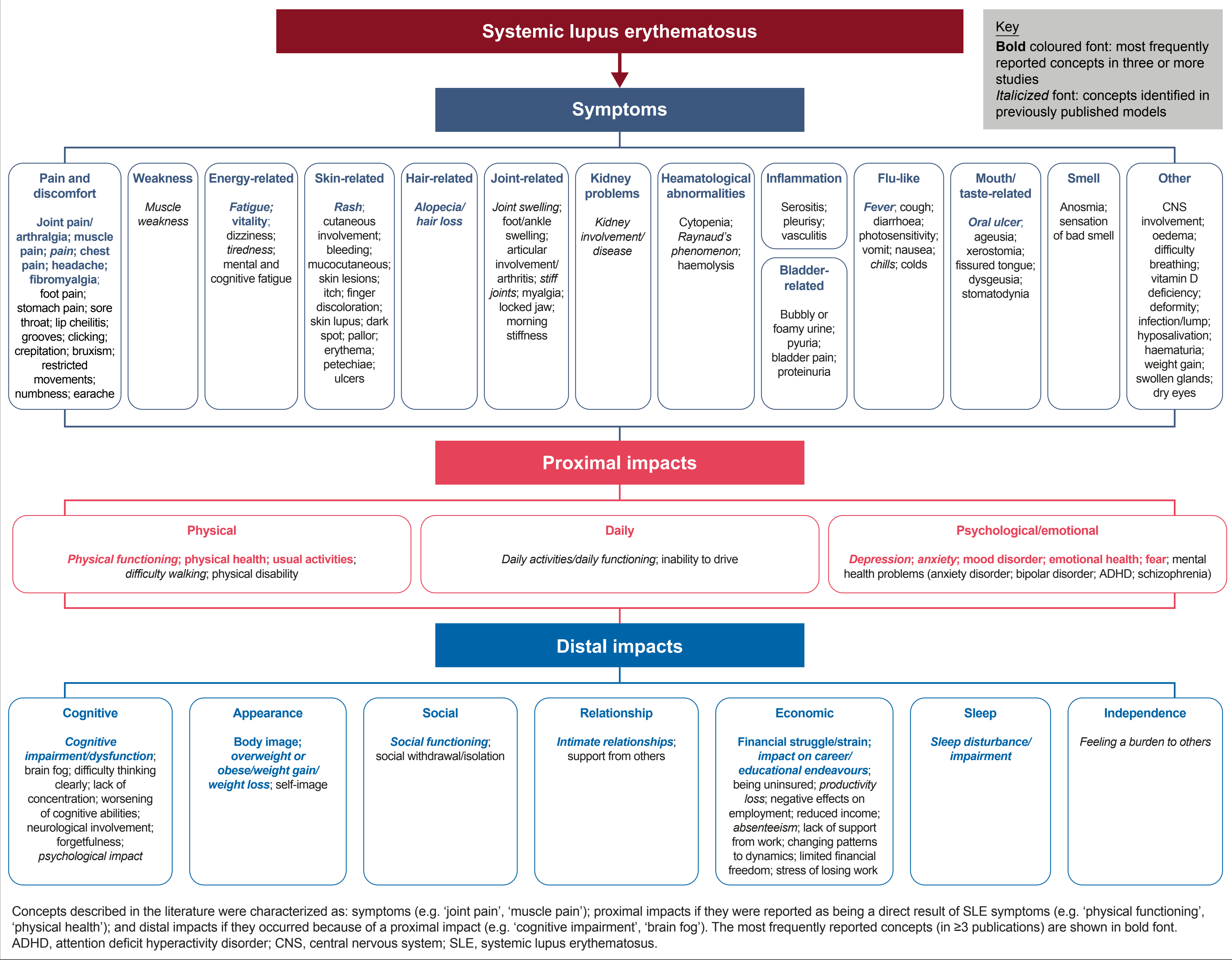
- A CDM of the patient experience of SLE was developed (**Figure 2**)

Figure 1. PRISMA Flow Diagram for the TLR



- The CDM grouped the 82 identified symptoms into 14 dimensions, the 13 impacts proximal to SLE into three dimensions, and the 28 impacts distal to SLE into seven dimensions

Figure 2. Conceptual Model of SLE



| Table. PICOS Inclusion Criteria for Screening and Selection of Publications Reporting Patient-Reported Symptoms and Impacts of SLE |   |
|--|---|
| PICOS category   | Inclusion criteria  |
| Population   | Adult patients with SLE   |
| Intervention/comparator  | No limits   |
| Outcomes   | Symptoms/signs and impacts of SLE <ul style="list-style-type: none"><li>• Burden of disease</li><li>• Functional impacts</li><li>• Physical impacts</li><li>• Psychological/emotional impacts</li><li>• Social impacts</li><li>• Behavioural impacts</li><li>• Short- and long-term complications</li></ul>   |
| Study design   | <b>Qualitative studies</b> <ul style="list-style-type: none"><li>• Qualitative interviews</li><li>• Focus group discussions</li><li>• Targeted/narrative literature review</li></ul> <b>Quantitative studies</b> <ul style="list-style-type: none"><li>• Cross-sectional survey studies</li><li>• Prospective and retrospective cohort studies</li><li>• Randomized controlled trials</li><li>• Observational cohort studies</li><li>• Epidemiological studies</li><li>• Systematic reviews</li></ul> |

PICOS, population, intervention/comparator, outcomes, study design; SLE, systemic lupus erythematosus.

**References:** 1. Hoi A, et al. *Lancet* 2024;403:2326–38; 2. Vaillant AAJ, et al. *StatPearls*. Updated 2023. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK535405/> (Accessed 5 September 2024); 3. FDA. 2010. Available from: <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/systemic-lupus-erythematosus-developing-medical-products-treatment> (Accessed 5 September 2024); 4. EMA. 2015. [https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-clinical-investigation-medical-products-treatment-systemic-lupus-erythematosus-and-lupus-nephritis\\_en.pdf](https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-clinical-investigation-medical-products-treatment-systemic-lupus-erythematosus-and-lupus-nephritis_en.pdf) (Accessed 5 September 2024); 5. FDA. *Health Qual Life Outcomes* 2006;4:79

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