Machine Learning Models to Estimate Disease Activity Measures in Real-World Data Sources: Lessons Learned from Four Autoimmune Diseases

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Objective

Autoimmune diseases such as systemic lupus erythematosus (SLE), rheumatoid arthritis, ankylosing spondylitis, and multiple sclerosis are complex, chronic conditions that require regular monitoring to guide treatment. Validated measures are important tools to consistently track disease activity over time, inform treatment selection, and monitor treatment response and outcomes. Yet, scores for these measures frequently are missing from real-world data (RWD) sources such as electronic medical records (EMRs) due to variations in clinical practice and documentation challenges. This limits the potential for using RWD for research on treatment patterns and outcomes in real-world settings for these diseases.

This effort assessed the feasibility of applying machine learning methods to routinely-recorded unstructured clinical data to estimate scores for validated measures in autoimmune diseases.

Methods

Machine learning (ML) models were developed to estimate scores for four disease activity measures:[1-3]

- Systemic Lupus Erythematosus Disease Activity Index (SLEDAI) for SLE
- Clinical Disease Activity Index (CDAI) for rheumatoid arthritis
- Bath Ankylosing Spondylitis Disease Activity Index (BASDAI) for ankylosing spondylitis
- Expanded Disability Status Scale (EDSS) for multiple sclerosis

For each model, training and validation cohorts were created from RWD sources, and performance metrics were calculated to assess the model performance.

Results

The ML models performed very well when estimating scores. Binarizing the outcome as low versus high at clinically meaningful thresholds yields an area under the receiver-operating-characteristic curve (AUC) of 0.91 (SLEDAI model), 0.88 (CDAI model), 0.82 (BASDAI model), and 0.91 (EDSS model) (Table 1). Application of the models to RWD sources resulted in increased numbers of patients with disease activity scores for inclusion in RWD studies (Figures 1, 2, and 3).

Model development yielded several lessons that are informative for future machine learning efforts. First, the rich detail included in the clinical notes was sufficient to estimate scores for most patient encounters, suggesting that clinicians often document critical information about symptoms, progress, and medication needs even when they do not use a validated instrument to measure disease activity. Second, model features do not entirely overlap with the items on the validated instruments, emphasizing the value of a machine learning approach over simple string searches for phrases.

Successful development of models for four different instruments and diseases suggests this approach is scalable to other instruments and diseases.

Conclusions

- Machine learning models perform very well when estimating disease activity scores.

- Application of these models to RWD sources is useful for addressing missing data and increasing the number of patients available for real-world research studies focused on treatment response and outcomes.

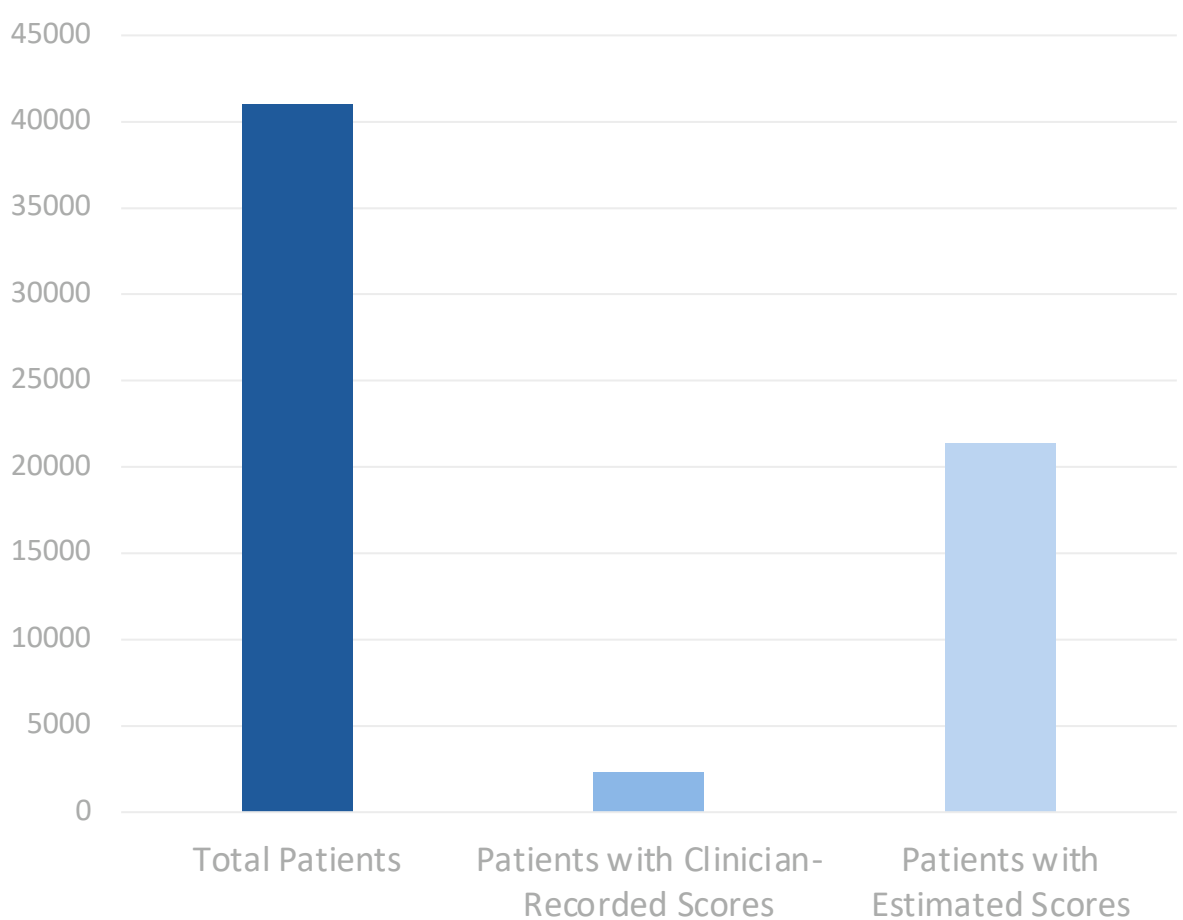Figure 1. SLE Patients and SLEDAI Scores in RWD Source

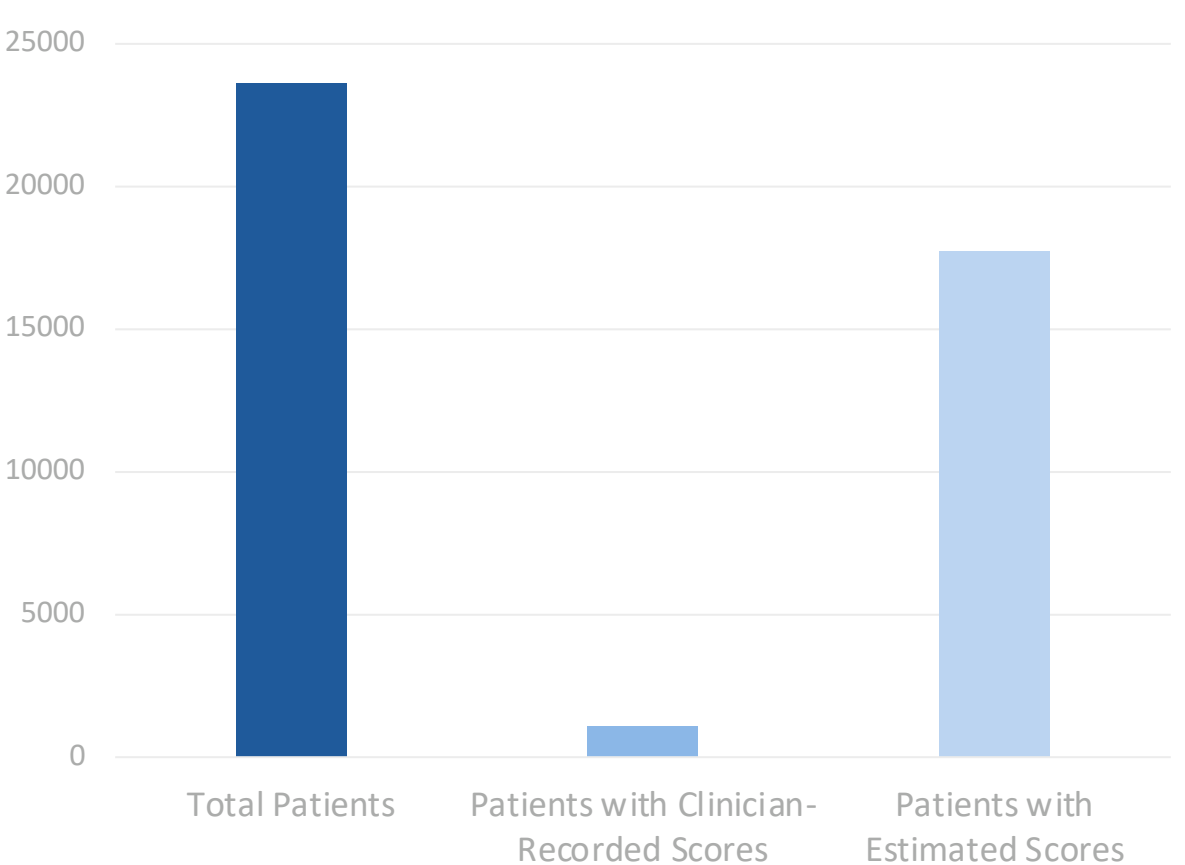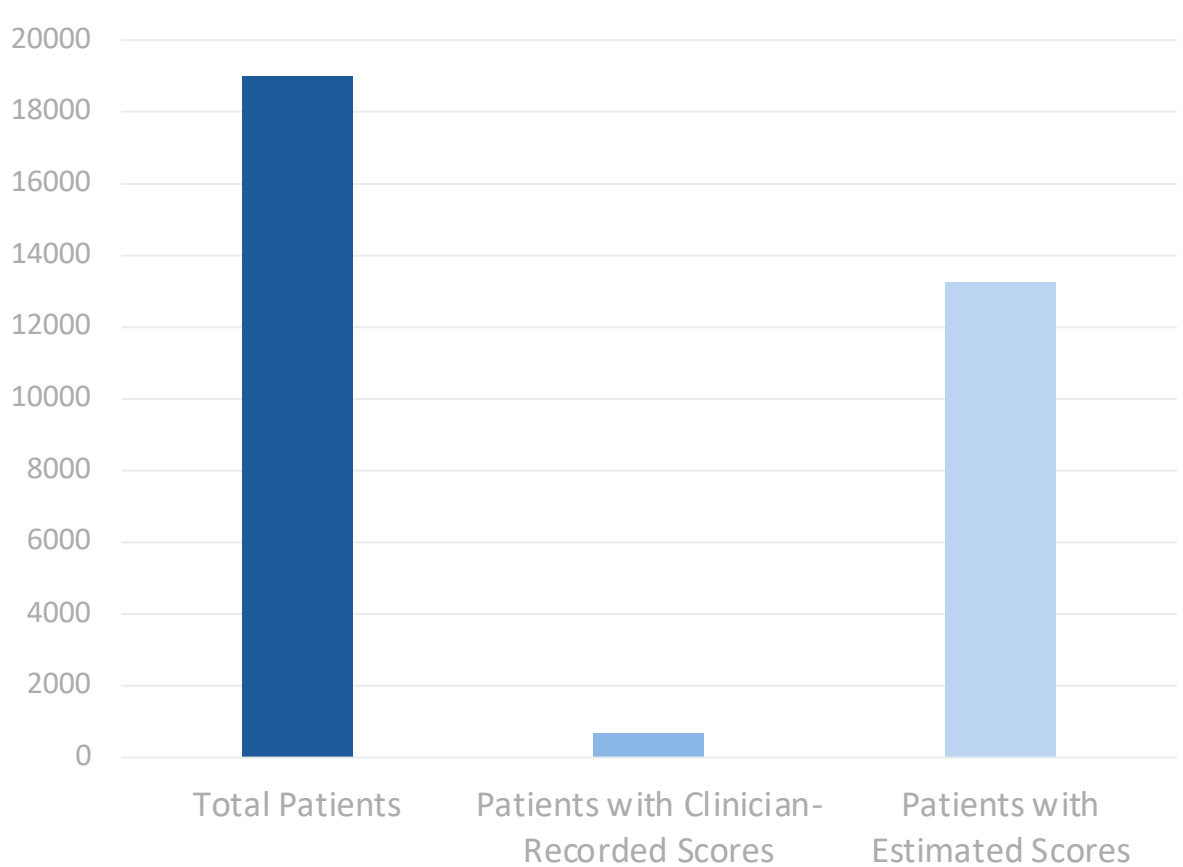Figure 2. Ankylosing Spondylitis Patients and BASDAI Scores in RWD Source

Figure 3. Multiple Sclerosis Patients and EDSS Scores in RWD Source

Table 1. AUC for Estimated Disease Activity Measures

Disease Activity Measure	AUC
SLEDAI model	0.91
CDAI model	0.88
BASDAI model	0.82
EDSS model	0.91

AUC = area under the receiver-operating-characteristic curve; BASDAI = Bath Ankylosing Spondylitis Disease Activity Index; CDAI = Clinical Disease Activity Index; EDSS = Expanded Disability Status Scale; RWD = real-world data; SLEDAI = Systemic Lupus Erythematosus Disease Activity Index

References

1. Alves et al. Validation of a machine learning approach to estimate Systemic Lupus Erythematosus Disease Activity Index score categories and application in a real-world dataset. RMD Open. 2021;7(2).
2. Spencer et al. Validation of a machine learning approach to estimate Clinical Disease Activity Index Scores for rheumatoid arthritis. RMD Open. 2021;7(3).
3. Alves et al. Validation of a machine learning approach to estimate expanded disability status scale scores for multiple sclerosis. Mult Scler J Exp Transl Clin. 2022;8(2).