

Treatment Paradigm, Landscape of Products Used, and Unmet Needs in Pediatric SLE

Ekemini Ogbu MD MSc FAAP

Associate Professor of Pediatrics

Cincinnati Children's Hospital Medical Center

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Objectives

- Review the treatment paradigm for childhood-onset systemic lupus erythematosus
- Review the landscape of products used for treatment of childhood-onset systemic lupus erythematosus
- Describe unmet therapeutic needs in childhood-onset systemic lupus erythematosus

Disclosure

I have no relevant financial relationship(s) with ineligible companies to disclose.

- American College of Rheumatology - Chair, Pediatric Rheumatology Committee
- Pediatric Rheumatology Collaborative Study Group - Advisory Council
- Autoimmune Encephalitis Alliance - Board
- Rheumatology Research Foundation - Board
- Council of Pediatric Subspecialties – Rheumatology Representative

Grants: Lupus Research Alliance, Childhood Arthritis and Rheumatology Research Alliance, Lupus and Allied Diseases Association Inc.

Off-label medications: Will discuss off-label use of therapies for pediatric SLE

Background

- Systemic lupus erythematosus (SLE) is the 2nd most common chronic rheumatic disease in children
- Childhood-onset SLE (cSLE) also called pediatric-onset SLE
- 5-year survival = 90% up from 17.5 - 69% in the 1960's

cSLE still has suboptimal clinical outcomes despite advances in therapeutics and clinical practice, and the increased availability of pediatric rheumatology practices.



Wenderfer SE, et al. Pediatr Res. 2017
Vazzana KM et al. Lupus. 2021

Treatment Paradigm

- Treatment principles, treatment goals and treatment effectiveness
 - Achieve and maintain cSLE disease remission
 - Achieve and maintain low lupus disease activity
 - Reduce cSLE disease-related morbidity and mortality
 - Minimize treatment related toxicities
- Early introduction of immunosuppressive therapies and minimization of corticosteroid use
- Treat to target principles

Smith EMD, et al. Ann Rheum Dis. 2023. Sammaritano LR, et al. Care Res (Hoboken). 2025

Current Landscape of Treatment Products for cSLE

Medication \ Guideline	2025 ACR SLE	2024 ACR Lupus Nephritis	2025 update EULAR lupus nephritis	2023 update EULAR SLE	2024 KDIGO lupus nephritis
Azathioprine	✓	✓	✓	✓	✓
Calcineurin inhibitors	✓	✓	✓	✓	✓
Corticosteroids	✓	✓	✓	✓	✓
Cyclophosphamide	✓	✓	✓	✓	✓
Dapsone	✓				
Hydroxychloroquine	✓	✓	✓	✓	✓
Intravenous gammaglobulin	✓				
Leflunomide					✓
Methotrexate	✓			✓	
Mycophenolic acid analogues	✓	✓	✓	✓	✓

ACR = American College of Rheumatology; EULAR = European Alliance of Associations for Rheumatology

KDIGO - Kidney Disease: Improving Global Outcomes

Scherlinger, M., et al. Nat Rev Drug Discov. 2025; Sammaritano LR, et al. Care Res (Hoboken).2025 Fanouriakis A et al. Ann Rheum Dis. 2026

Current Landscape of Treatment Products for cSLE

Guideline Medication	2025 ACR SLE Guidelines	2024 ACR Lupus Nephritis Guidelines	2025 update 2019 EULAR lupus nephritis	2023 update EULAR SLE	2024 KDIGO Guidelines
Anifrolumab	✓			✓	
Belimumab	✓	✓	✓		✓
Intravenous gammaglobulin	✓				
Obinutuzumab	✓	✓	✓		
Rituximab	✓	✓	✓	✓	✓

ACR = American College of Rheumatology; EULAR = European Alliance of Associations for Rheumatology
KDIGO - Kidney Disease: Improving Global Outcomes

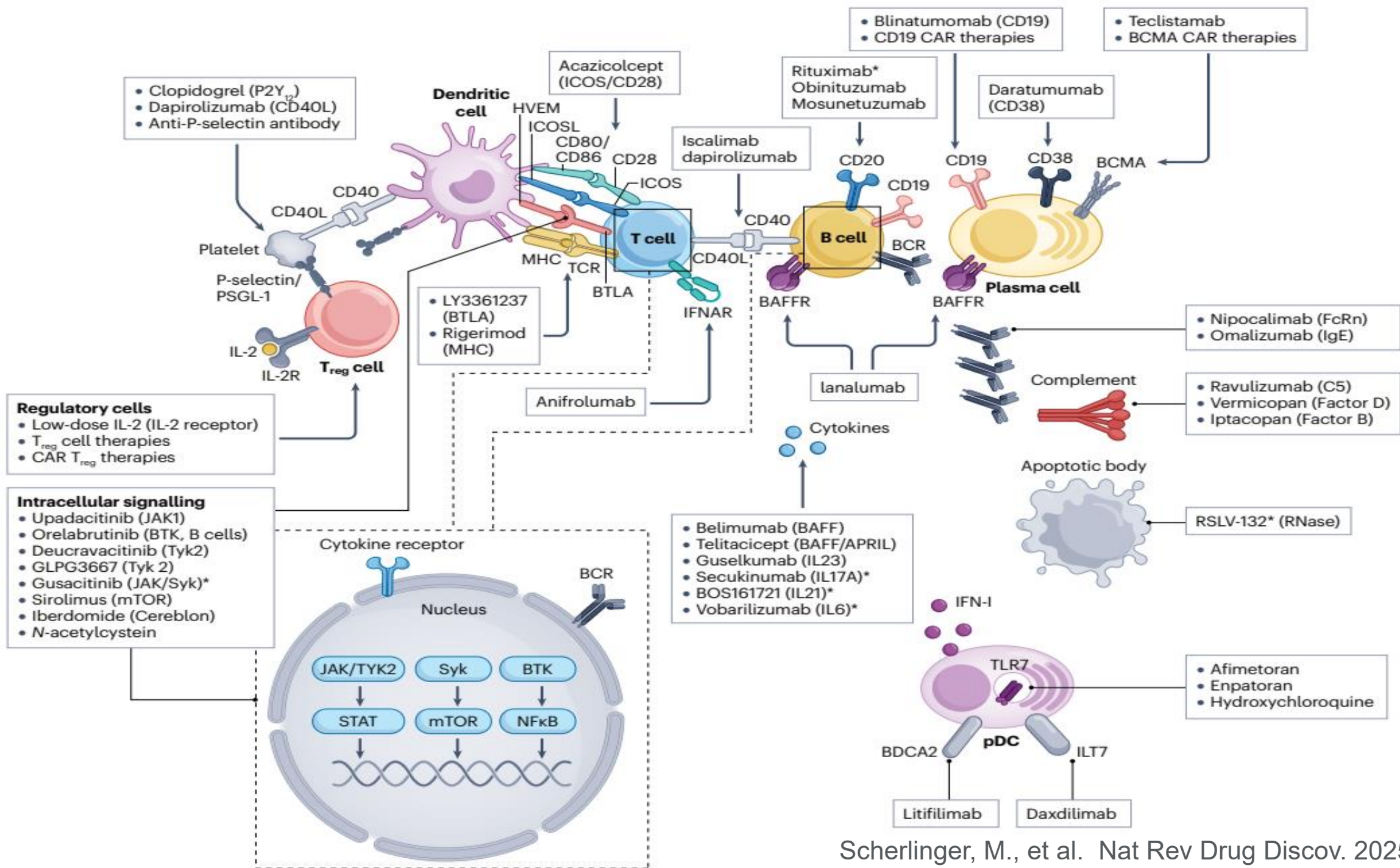
Scherlinger, M., et al. Nat Rev Drug Discov. 2025; Sammaritano LR, et al. Care Res (Hoboken).2025 Fanouriakis A et al. Ann Rheum Dis. 2026

Current Landscape of Treatment Products for cSLE

FDA Approved Medication	Year approved Adult	Year approved Pediatric
Belimumab	2010 (Lupus nephritis), 2011 (SLE)	2019 (≥5years - IV formulation) 2024 (≥5years subcutaneous autoinjector)
Voclosporin	2021 (Lupus nephritis)	None
Anifrolumab	2021	None
Obinutuzumab	2025 (Lupus nephritis)	None

Most therapies for SLE in clinical use are still off-label

Scherlinger, M., et al. Nat Rev Drug Discov. 2025



RESEARCH ARTICLE

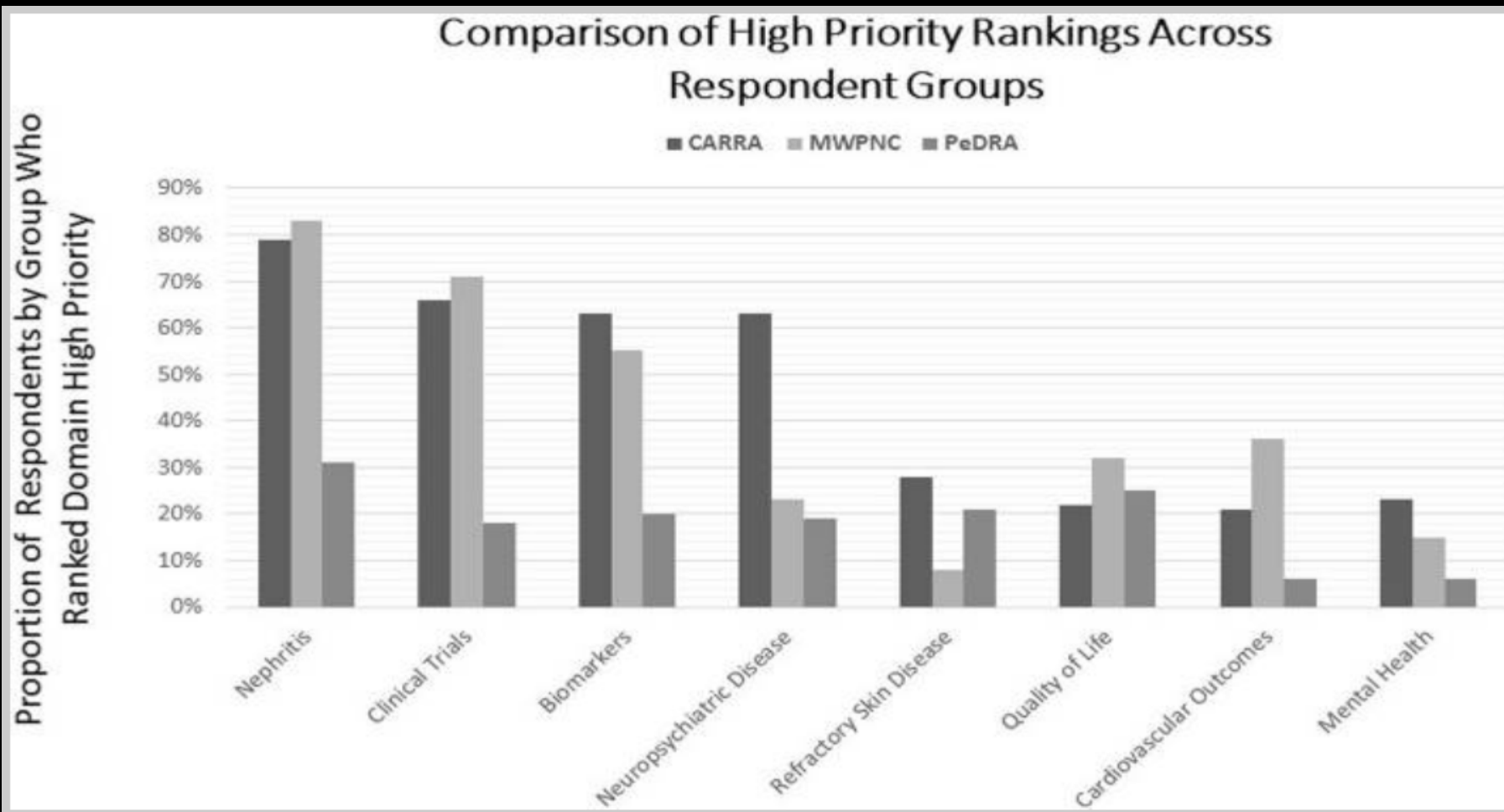
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Research priorities in childhood-onset lupus: results of a multidisciplinary prioritization exercise

Stacy P. Ardoin^{1*}, R Paola Daly², Lyna Merzoug², Karin Tse², Kaveh Ardalan³, Lisa Arkin⁴, Andrea Knight⁵, Tamar Rubinstein⁶, Natasha Ruth⁷, Scott E. Wenderfer⁸, Aimee O. Hersh⁹ and on behalf of the Childhood Arthritis and Rheumatology Research Alliance and Lupus Foundation of America

Unmet needs in cSLE



- Childhood Arthritis and Rheumatology Research Alliance
- Midwestern Pediatric Nephrology Consortium
- Pediatric Dermatology Research Alliance
- Lupus Foundation of America

Unmet needs in cSLE



Treatment of cSLE

Identification of effective therapeutics
Access to effective therapeutics

Unmet Needs in cSLE: Lessons from the Childhood Arthritis and Rheumatology Research Alliance (CARRA) Registry

- 70 active registry sites in North America



Stacy Ardoin



Ekemini Ogbu



Mary Beth Son



Hermine Brunner



Aimee Hersh



Stephen Balevic



Rebecca Sadun



Laura Lewandowski



Edie Weller

Unmet Needs in cSLE: Lessons from the CARRA Registry

- Objective: To quantify unmet therapeutic needs in cSLE
 - ❖ Off-label medication use
 - ❖ Corticosteroid dependence
 - ❖ Inability to achieve low disease activity

Methods:

- Study period
January 2017 - March 2025
- Population:
All patients diagnosed with SLE at age < 18 years +
Medication exposures occurring during childhood (< 18 years)
- 6-monthly registry visits

Methods: Unmet needs definition

Treatment with medications:

CRITERIA 1: Not FDA approved for cSLE

CRITERIA 2: Not considered in ACR lupus nephritis or SLE guidelines

CRITERIA 3: Not used in standard of care for children or adults with SLE

Unmet needs definition:

Treatment with medications

Medication Class	
Corticosteroid	Prednisone, prednisone equivalents
Non-biologic disease modifying anti-rheumatic drugs (nbDMARDs)	hydroxychloroquine, chloroquine, methotrexate, azathioprine, mycophenolate, cyclophosphamide, cyclosporin, voclosporin, and tacrolimus
Biologic disease modifying anti-rheumatic drugs (bDMARDs)	belimumab, rituximab, obinutuzumab, anifrolumab

Methods: Unmet needs definition

CRITERIA 4: Inability to discontinue oral corticosteroids or achieve prednisone-equivalent dose < 7.5 mg/day at 6, 12, 24, and 36 months after diagnosis

CRITERIA 5: Inability to achieve lupus low disease activity state (LLDAS) at 6, 12, 24, and 36 months after diagnosis

Results:

1275 patients = diagnosed with SLE at age < 18 years + medication exposures at age < 18 years

- Sex: Female - 1160 (86.7%)
- Race/Ethnicity: White - 317 (24.9%); Black - 340 (26.7%);
Asian - 143 (11.2%), Hispanic - 277 (21.7%)
- Median age in years at SLE diagnosis: 14.20 [IQR 12.00, 16.02]
- Median age in years at registry enrollment: 15.09 [IQR 12.88, 16.84]
- Disease duration in years: 4.06 [IQR 2.46, 5.88]

1256 patients

Diagnosed with SLE at age < 18 years + Medication exposures at age < 18 years + medication data available

Results:

1256 patients

Diagnosed with SLE at age < 18 years + Medication exposures at age < 18 years + medication data available

- 95.7% - ever treated with hydroxychloroquine
- 85.6% - ever treated with a nbDMARD
- 35.4% - ever treated with a bDMARD.

Figure 1: Use of non-Biologic and Biologic Disease Modifying Anti-Rheumatic Drugs (DMARD) amongst Children with SLE in the CARRA Registry

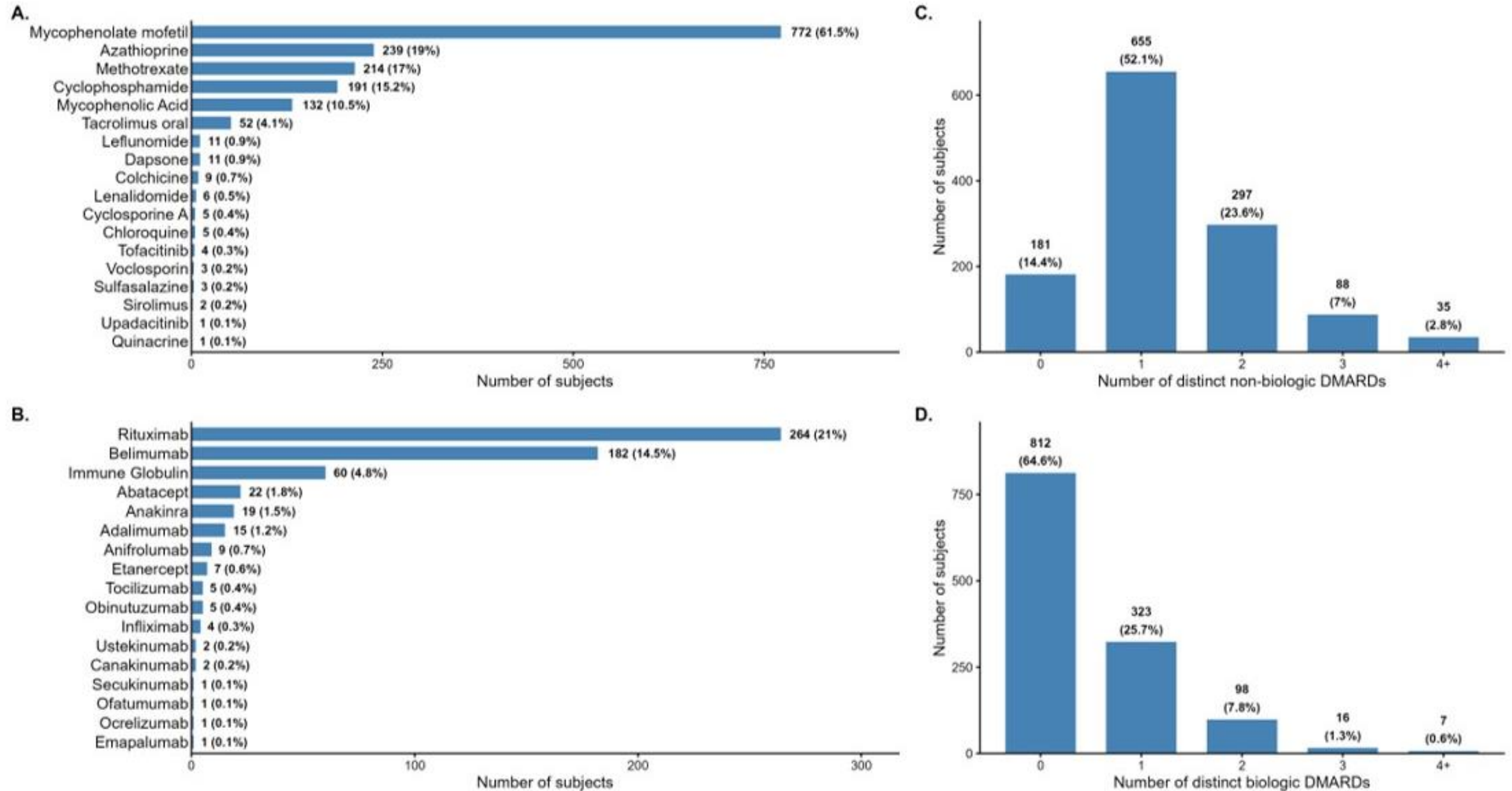


Figure 2: Prednisone Dose by Visit in Patients with cSLE in the CARRA Registry

Dose category ■ Low (≤ 7.5) ■ Medium (7.5-30) ■ High (30+)

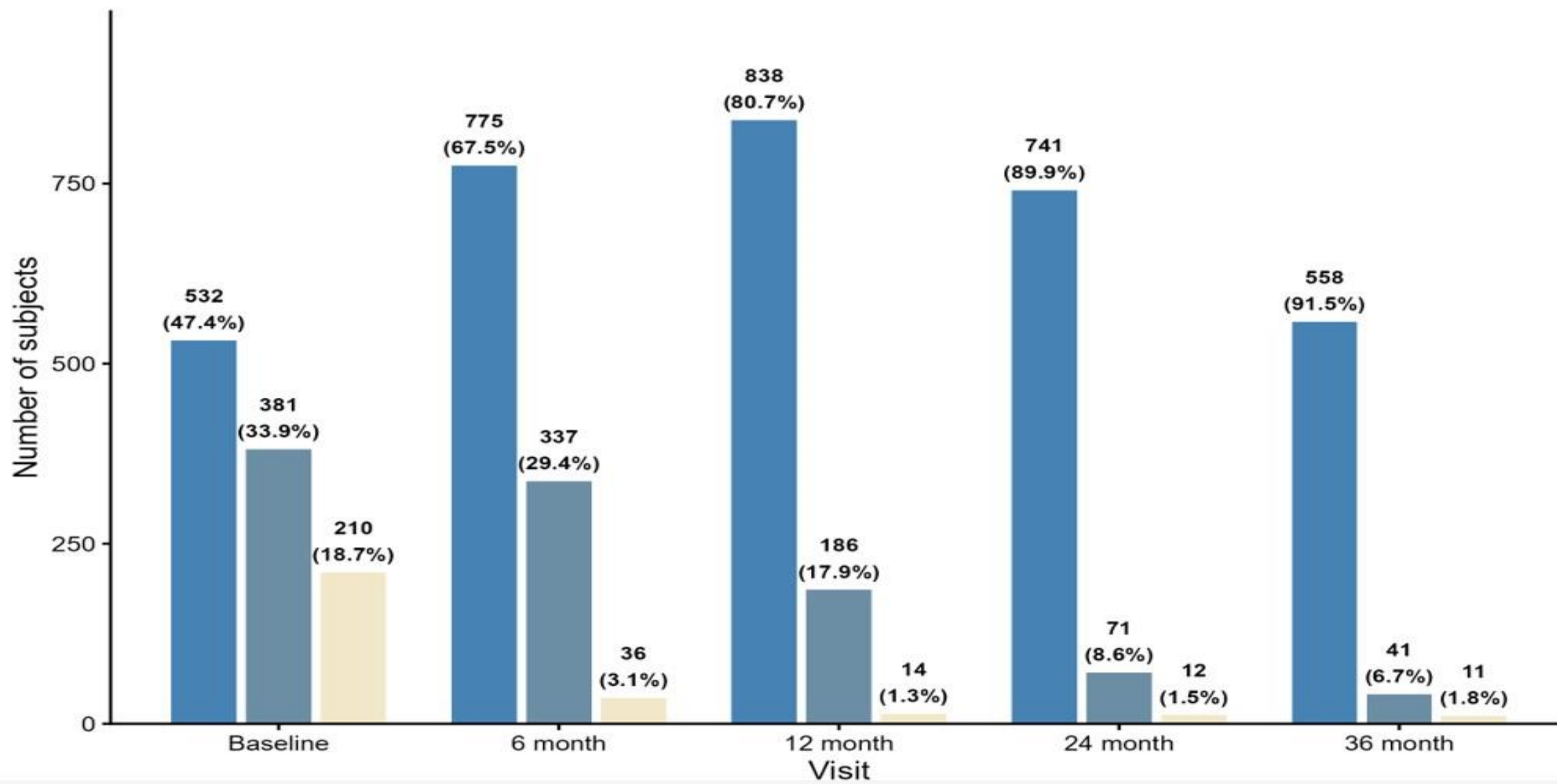
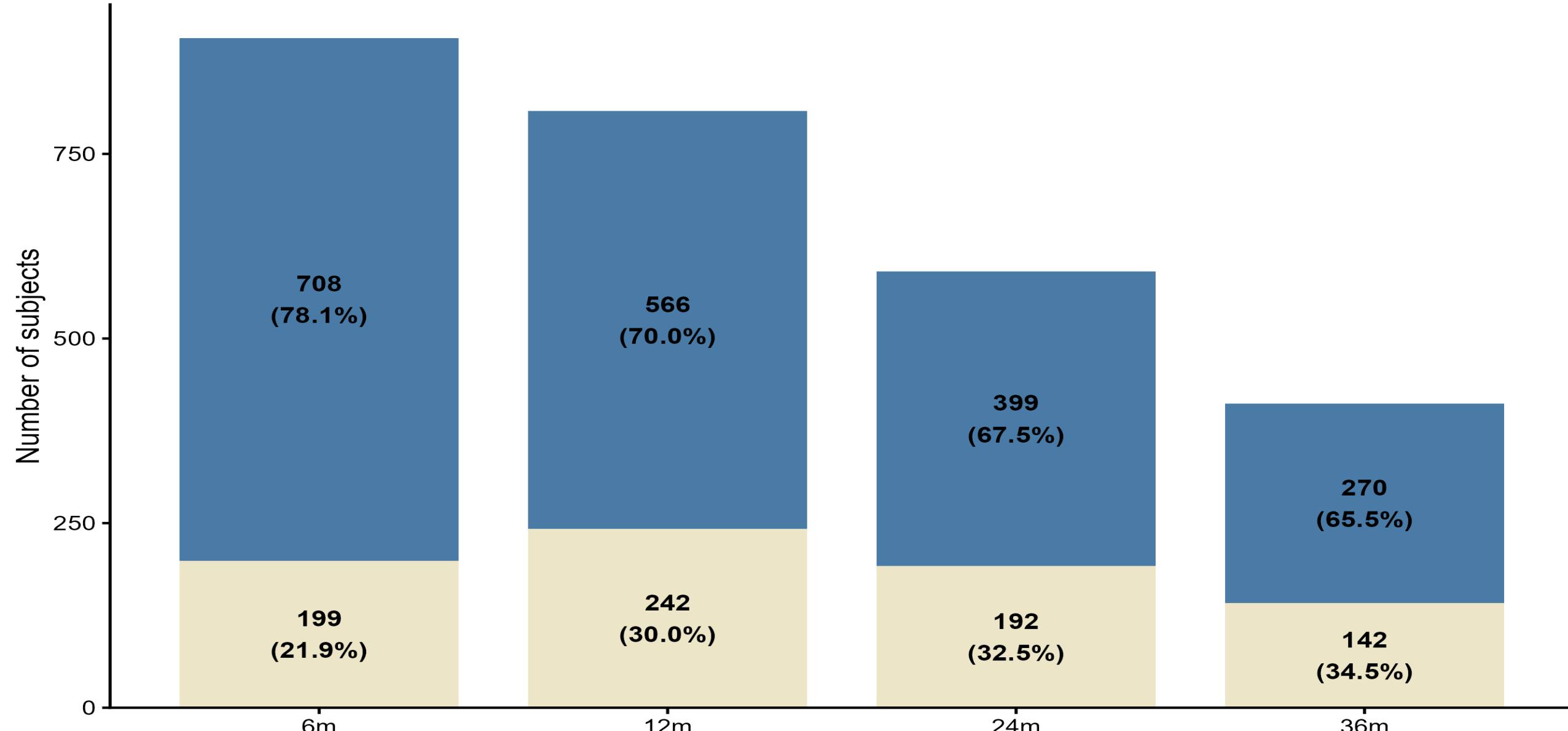


Figure 3: Lupus Low Disease Activity Status in Children with SLE over Three Years in the CARRA Registry

LLDAS met Not met



Results

- Almost all patients with cSLE in the CARRA Registry had substantial unmet therapeutic needs.

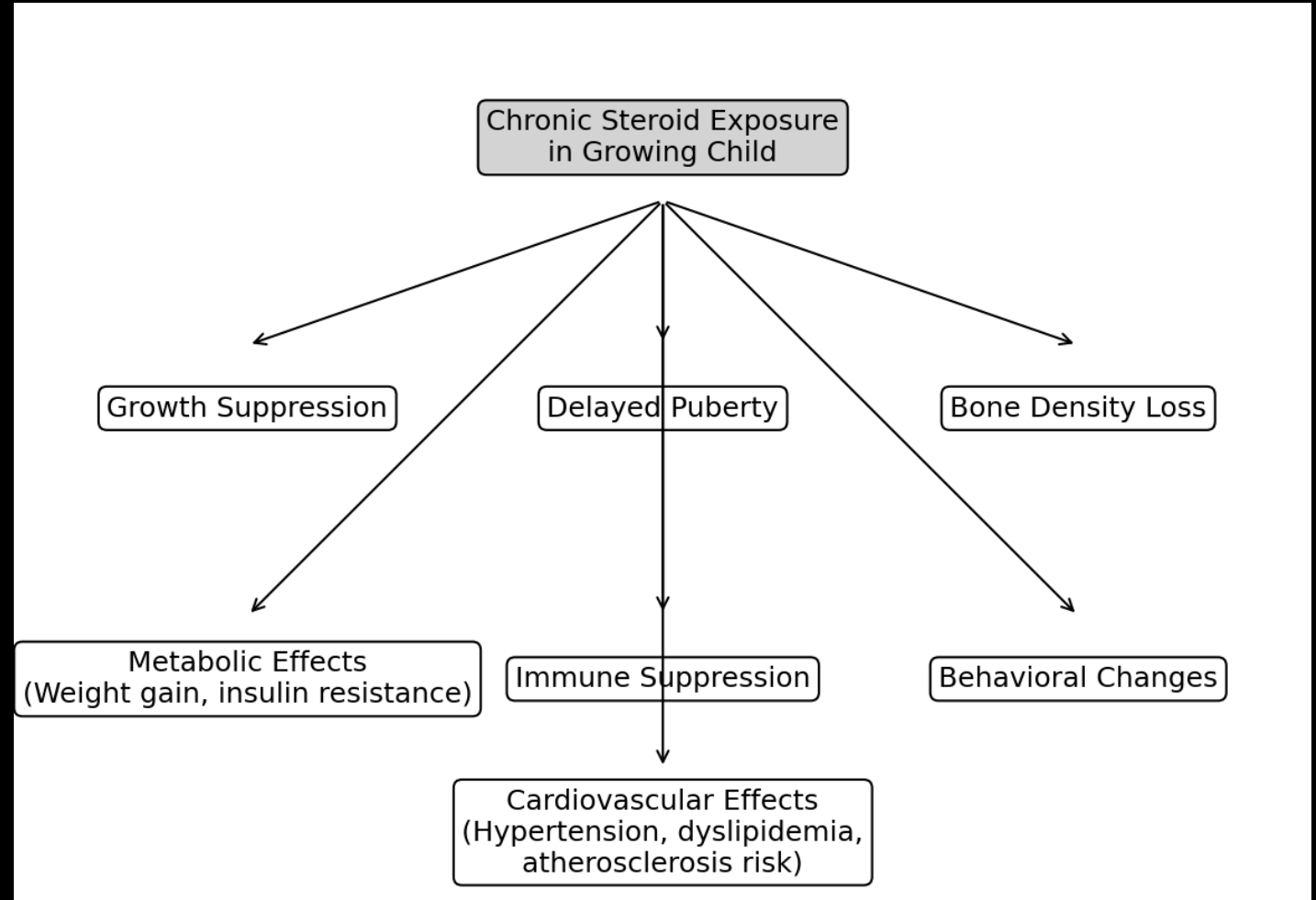
At 12 months after diagnosis:

- ❖ 0.3% of 1256 patients had no unmet needs
- ❖ 26.9% of 1256 patients met 1 - 2 unmet needs criteria
- ❖ 51.9% of 1256 patients met 3 - 4 unmet needs criteria
- ❖ 20.8% of 1256 had 5 or more unmet needs criteria

=>1 in 5 patients have high level of unmet need

Corticosteroid dependency....

- Proportion of patients remaining on corticosteroids:
 - 55.8% at 6 months
 - 43.6% at 12 months
 - 26.9% at 24 months
 - 11.2% at 36 months
- Long-term corticosteroid use.....



Take home message

- Attainment of treatment principles and goals for cSLE hinges on the ready availability of effective therapeutics
- Our study leverages real world data from the largest multi-center prospective registry of cSLE in North America to underscore the limited effectiveness of currently available therapies.
- There is an urgent need for expanded regulatory approval of therapies for children, adolescents and young adults with SLE to improve clinical outcomes.

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Acknowledgements

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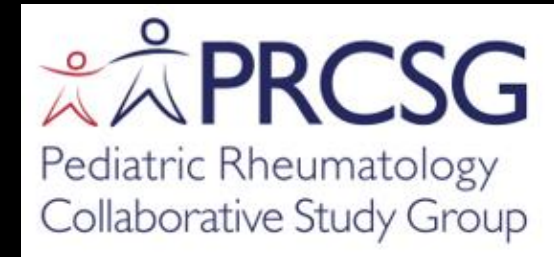
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Childhood Arthritis and Rheumatology Research Alliance Investigators
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Thank you