

Outcome Measures, Disease Outcomes & Trajectory in Childhood and Adult-onset SLE

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A decorative arc of approximately 20 small, light blue butterflies is positioned at the top of the slide, curving from the left towards the right.

Disclosures

- I receive research support from Luminary Therapeutics
- I consult for the Childhood Arthritis and Rheumatology Research Alliance

A decorative arc of various colored butterflies (pink, purple, blue, yellow) flying across the top of the slide.

Objectives

- Compare adult and pediatric instruments for measuring disease activity and damage
- Evaluate use of these outcome tools in adult and pediatric lupus trials
- Understand similarities and differences in lupus disease outcomes and concerns across the lifespan

SLE Disease Activity & Flare Measures

	 Adult-onset SLE	 Childhood-onset SLE
Disease Activity	SLEDAI BILAG Physician Global Assessment SLE-DAS	SLEDAI BILAG Physician Global Assessment
Flare	SELENA-SLEDAI Flare Index BILAG	SELENA-SLEDAI Flare Index BILAG ACR Provisional Criteria for Global flares in cSLE

Lupus Low Disease Activity Definitions

	 LLDAS	 cLLDAS
Disease Activity	Clinical SLEDAI = 0	Clinical SLEDAI = 0
PGA (0-3)	≤ 0.5	≤ 0.5
Glucocorticoid Threshold	Prednisone ≤ 5 mg/day	Prednisone ≤ 0.1 mg/kg/day (max 5 mg/day)
Maintenance Therapy	Stable medications	Stable medications

SLE Remission Definitions

	 DORIS	 cSLE Clinical Remission
SLEDAI	≤ 4 , no major organ activity	≤ 4 , no major organ activity
New Activity	None vs prior visit	None vs prior visit
PGA (0-3)	≤ 1	≤ 1
Glucocorticoid Threshold	Prednisone ≤ 7.5 mg/day	Prednisone ≤ 0.17 mg/kg/day (max 7.5 mg/day)
Maintenance Therapy	Stable medications	Stable medications

SLE Flare, Response, Damage Measures

	 Adult SLE	 Childhood-onset SLE
SLE Flare	SELENA-SLEDAI Flare Index SLE-DAS flare tool	SELENA-SLEDAI Flare Index ACR Provisional Criteria for Global flares in cSLE
Treatment Response	SLE Responder Index-4 BICLA	SLE Responder Index-4 BICLA PRINTO/ACR 50 Childhood Lupus Improvement Index
SLE Damage	SLICC/ACR Damage Index	SLICC/ACR Damage Index

SLE Flare, Response, Damage Measures

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SLE Damage	SLICC/ACR Damage Index	SLICC/ACR Damage Index

Patient Reported Outcome Measures

	 Adult SLE	 Childhood-onset SLE
General	PROMIS Patient Global Assessment SF-36	PROMIS Patient/Parent Global Assessment Peds-QL CHAQ
Lupus specific	Lupus QoL Lupus PRO SLE QoL L-QoL Lupus Impact Tracker	SMILEY

Use of Disease Activity Measures in aSLE and cSLE Trials

	Belimumab		Anifrolumab	
	BLISS-1,2	PLUTO	TULIP-2, SC	BLOSSOM
Eligibility	SLEDAI ≥ 6	SLEDAI ≥ 6	SLEDAI ≥ 6 ≥ 1 BILAG A / ≥ 2 BILAG B PGA ≥ 1	SLEDAI ≥ 6 ≥ 1 BILAG A / ≥ 2 BILAG B PGA ≥ 1

Efficacy Outcome Measures in aSLE and cSLE Trials

	Belimumab		Anifrolumab	
	BLISS-1,2	PLUTO	TULIP-2, SC	BLOSSOM
Primary Efficacy	SRI-4	SRI-4	BICLA	BICLA
Secondary Efficacy	SFI BICLA	PRINTO/ACR response	SRI-4 CLASI	SRI-4 PRINTO/ACR response
PRO	SF-36	Patient/Parent Global	Patient Global	Patient/Parent Global PedsQL

Disease Activity



Disease activity at diagnosis

cSLE



aSLE



Prevalence of lupus nephritis



At diagnosis

37-67%

7-31%

Lifetime

50-80%

20-40%



Prevalence of neuropsychiatric lupus



SLE Disease Survival and Damage

	cSLE	aSLE
Survival (10 year)		
 High-income country	99%	92%
Low-income country	79%	85%
Standardized mortality ratio	18.8	2.9
 ESKD	↑↑	↑

SLE Concerns Across the Lifespan

mental health
pubertal delay*
education*
growth*
reproductive health
malignancy
impact on family
bone health
vocation
cardiovascular disease



Take-Away Points

- **The same primary eligibility and efficacy measures are utilized for aSLE and cSLE clinical trials**
 - Weight-based adjustments for glucocorticoid dosing
- **SLE disease activity and damage accrual are higher in cSLE**
- **Lifespan concerns are similar with addition of growth, puberty and education for children**