

# Safety and Efficacy of Ustekinumab in Pediatric Ulcerative Colitis: Results from the Phase 3 UNIFI Jr Study

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Digestive Disease Week®

**MAY 2-5, 2026 | CHICAGO, IL**

EXHIBIT DATES: MAY 3-5, 2026

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# SPEAKER DISCLOSURE

I, **Jeffrey S. Hyams**, disclose the following financial relationships with a commercial interest:

- Served on an advisory board for AbbVie, Johnson & Johnson and Lilly
- Served as a consultant for Boehringer Ingelheim, Genentech, Merck, Roche, and Takeda

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# Background and Objective



Ustekinumab is a fully human IL-12 and IL-23 antagonist indicated for the treatment of moderately to severely active UC in adults<sup>1,2</sup>



Approved pharmacologic treatment options for children with UC remain limited to anti-TNF agents

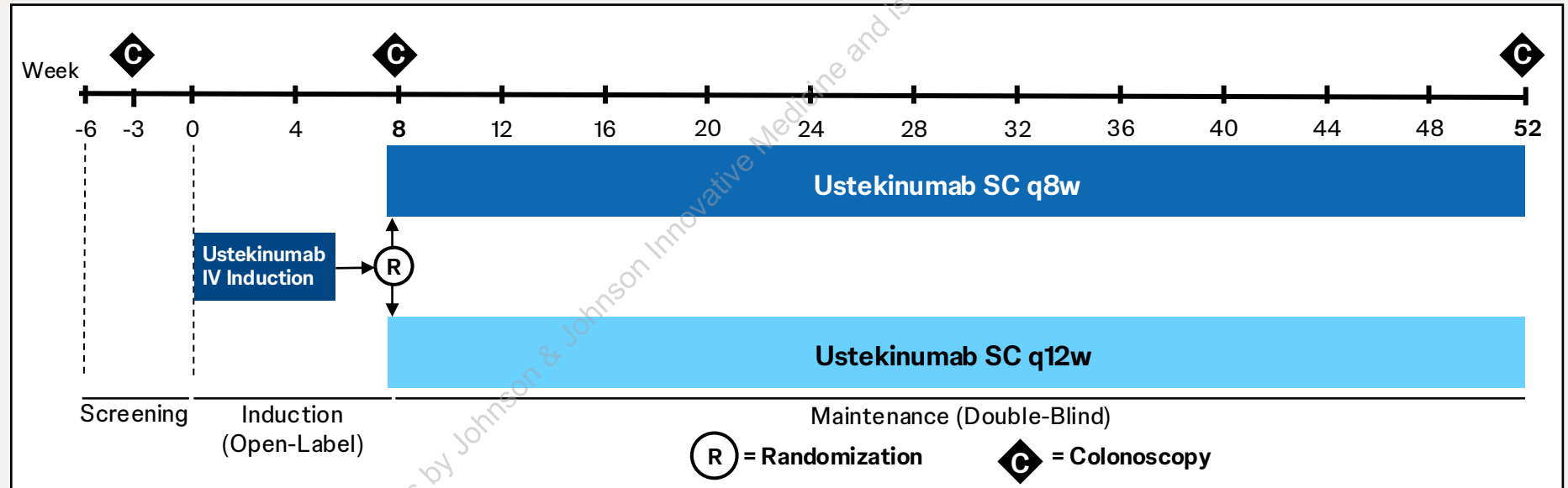


**Objective: The phase 3 UNIFI Jr study evaluated efficacy and safety of ustekinumab in children with moderately to severely active UC**

# UNIFI Jr – Study Design

## Key inclusion criteria:

- Age 2 to <18 years
- Weight  $\geq 10$  kg
- Moderately to severely active UC<sup>a</sup>
- Inadequate response or intolerant to conventional/ biologic therapy or corticosteroid-dependent



## Weight-tiered ustekinumab dosing

### IV induction:

- <40 kg: 250 mg/m<sup>2</sup>
- $\geq 40$  to  $\leq 55$  kg: 260 mg
- >55 to  $\leq 85$  kg: 390 mg
- >85 kg: 520 mg

### SC maintenance:

- <40 kg: 60 mg/m<sup>2</sup>
- $\geq 40$  kg: 90 mg

<sup>a</sup>Baseline Mayo score  $\geq 6$ , Mayo endoscopy subscore  $\geq 2$ . IV=intravenous, q8w=every 8 weeks, q12w=every 12 weeks, SC=subcutaneous, UC=ulcerative colitis.




# Efficacy Endpoints

Primary endpoint	
<b>Clinical remission</b>	Mayo SF subscore of 0 or 1, RB subscore of 0, and endoscopy subscore of 0 or 1 with no friability present on the endoscopy, and no increase from baseline in SF subscore
Key secondary endpoints	
<b>Clinical response</b>	Decrease from baseline in modified Mayo score by $\geq 30\%$ and $\geq 2$ points, with either a decrease from baseline in RB subscore of $\geq 1$ or RB subscore of 0 or 1
<b>Symptomatic remission</b>	Mayo SF subscore of 0 or 1 and RB subscore of 0, with no increase from baseline in SF subscore
<b>Endoscopic improvement</b>	Mayo endoscopy subscore of 0 or 1 with no friability present on the endoscopy
<b>Clinical remission by PUCAI</b>	PUCAI score $< 10$
<b>Corticosteroid-free clinical remission</b>	Clinical remission and not receiving corticosteroids for at least 90 days prior

- Efficacy assessed at Week 8 (Induction) and Week 52 (Maintenance; among induction responders)
- NRI approach was used to account for missing data and intercurrent events<sup>a</sup>
- Data are summarized using descriptive statistics; no statistical testing was performed/no p-values provided

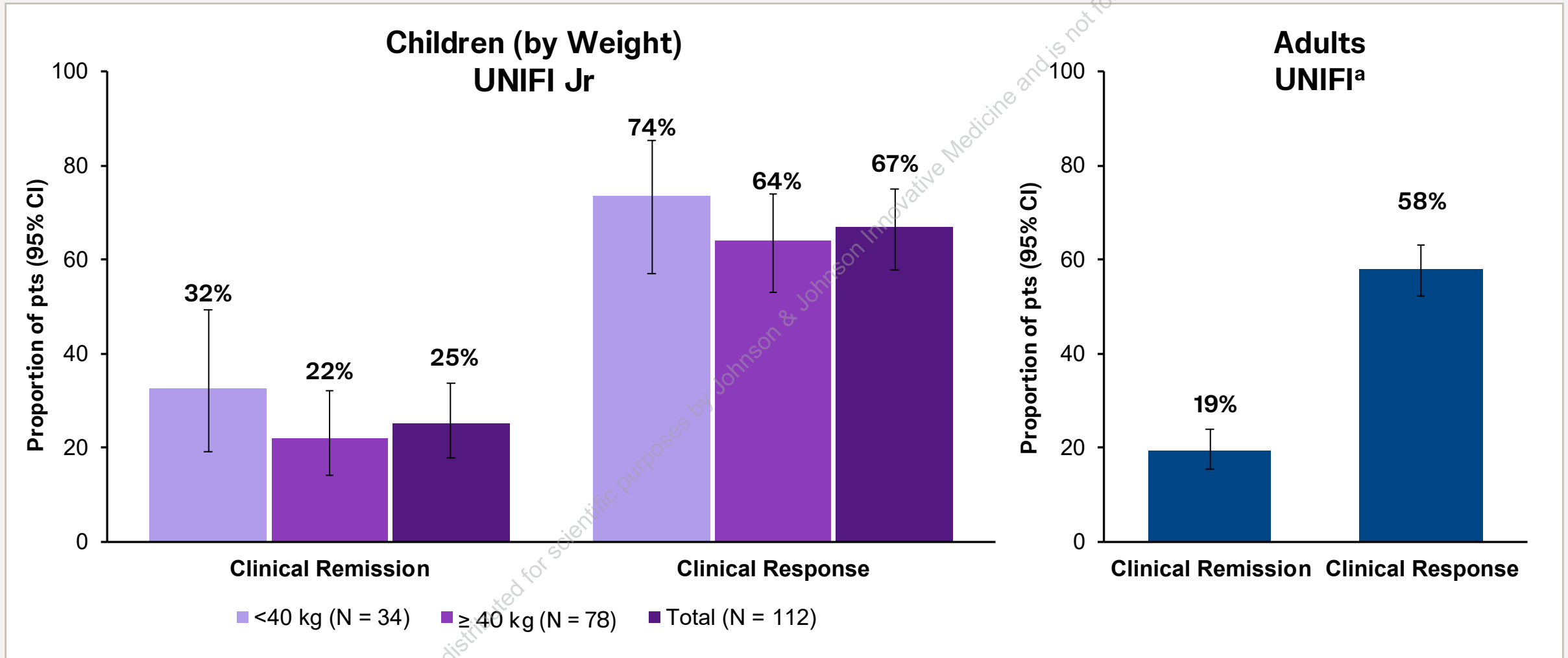
<sup>a</sup>Pts who had a colectomy or ostomy, discontinued study agent due to lack of efficacy, AE of worsening of UC, or other reason (except COVID-19–related reasons other than infection), or who had prohibited changes in UC medications before the analysis timepoint were considered not to have achieved the endpoint. Pts who discontinued study agent due to COVID-19–related reasons (excluding COVID-19 infection) were considered to have missing data from the time of event onwards (hypothetical strategy). Pts who initiated or increased use of rescue medication for treatment loss of response after Week 16/24 (responders/delayed responders) or who dose adjusted after Week 16 were considered not to have achieved the endpoint. After accounting for intercurrent events, pts with missing data were considered non-responders. **AE**=adverse event, **NRI**, non-responder imputation **pts**=participants, **PUCAI**=Pediatric Ulcerative Colitis Activity Index, **RB**=rectal bleeding, **SF**=stool frequency.

# Baseline Characteristics

Baseline Characteristics	Ustekinumab SC			
	q12w (N = 55)	q8w (N = 54)	Total <sup>a</sup> (N = 112)	
<b>Demographics</b>				
	Age, mean (SD) yrs	13.2 (3.4)	13.3 (2.8)	13.2 (3.1)
	Median (IQR) yrs	14.0 (11.0, 16.0)	14.0 (12.0, 15.0)	14.0 (11.0, 15.5)
	Females, %	51%	56%	54%
	Race, % Asian/Black/White <sup>b</sup>	13% / 2% / 84%	11% / 2% / 85%	12% / 2% / 84%
	Weight, mean (SD) kg	47.9 (15.9)	46.6 (13.5)	47.0 (14.6)
	Median (IQR) kg	47.0 (37.2, 57.7)	47.2 (36.0, 57.4)	46.9 (36.1, 57.2)
	Range, kg	14–80	14–76	14–80
<40 kg, %	27%	33%	30%	
<b>Disease Characteristics</b>				
	UC disease duration, mean (SD) yrs	2.3 (2.2)	1.7 (1.6)	2.0 (2.0)
	Extensive UC, %	65%	69%	67%
	Mayo score, mean (SD)	8.4 (1.4)	8.4 (1.5)	8.4 (1.4)
	Moderate (Mayo score 6–10), %	91%	92% <sup>c</sup>	92% <sup>c</sup>
	Severe (Mayo score >10), %	9%	6% <sup>c</sup>	7% <sup>c</sup>
	PUCAI score, mean (SD)	53.5 (13.0)	51.5 (16.1)	52.9 (14.7)
<b>UC-Related Therapies</b>				
	Oral/rectal corticosteroid use at baseline, <sup>d</sup> %	27%	22%	25%
	Immunomodulatory drug use at baseline, <sup>e</sup> %	33%	48%	39%
	History of inadequate response or intolerance to biologic, %	47%	31%	39%

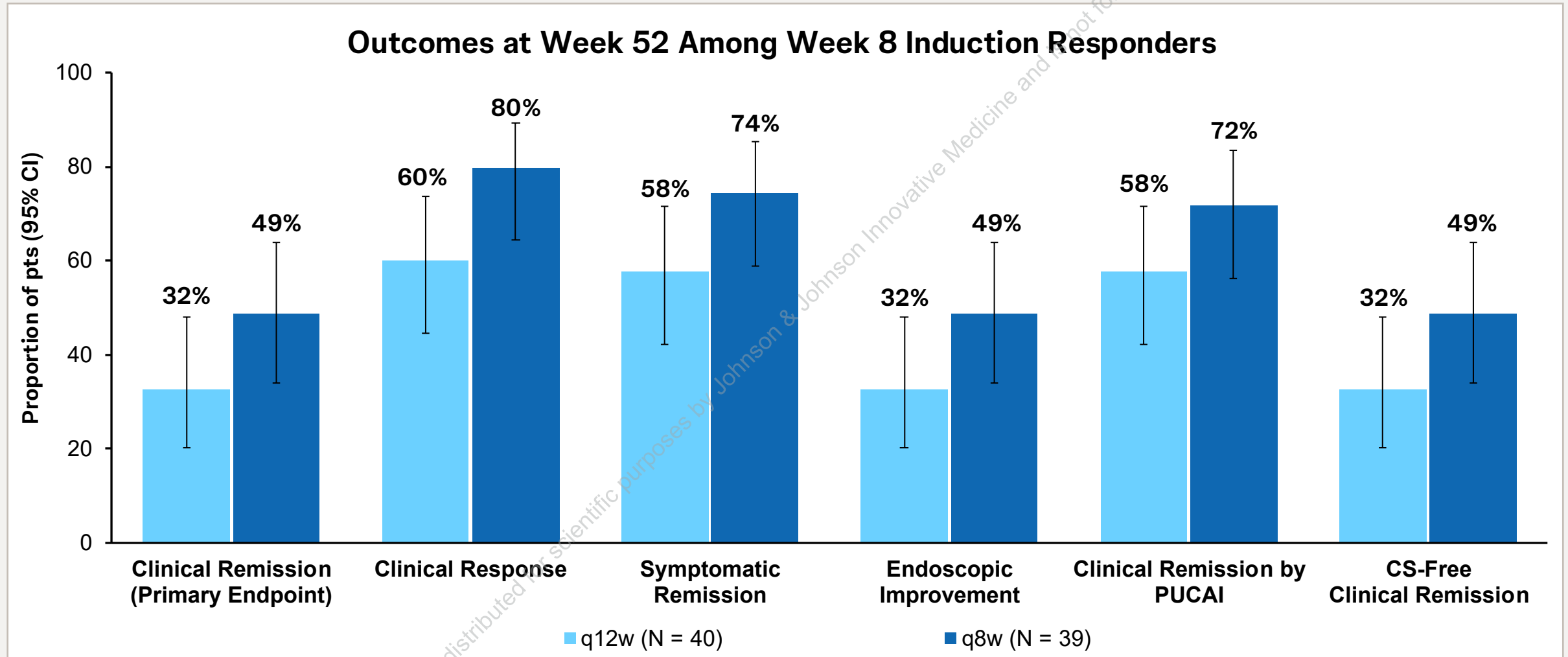
<sup>a</sup>Includes 3 participants who received ustekinumab at induction Week 0 but were not randomized at Week 8 to maintenance treatment. <sup>b</sup>Race not reported for 1 participant (q8w); unknown for 2 participants (q12w and not randomized). <sup>c</sup>1 participant (q8w) had mild disease (Mayo score ≥3 and <6). <sup>d</sup>Excluding budesonide and beclomethasone dipropionate. <sup>e</sup>6-mercaptopurine/azathioprine or methotrexate. IQR=interquartile range, PUCAI=Pediatric Ulcerative Colitis Activity Index, q8w=every 8 weeks, q12w=every 12 weeks, SC=subcutaneous, SD=standard deviation, UC=ulcerative colitis.

# Clinical Response and Remission Rates at Week 8

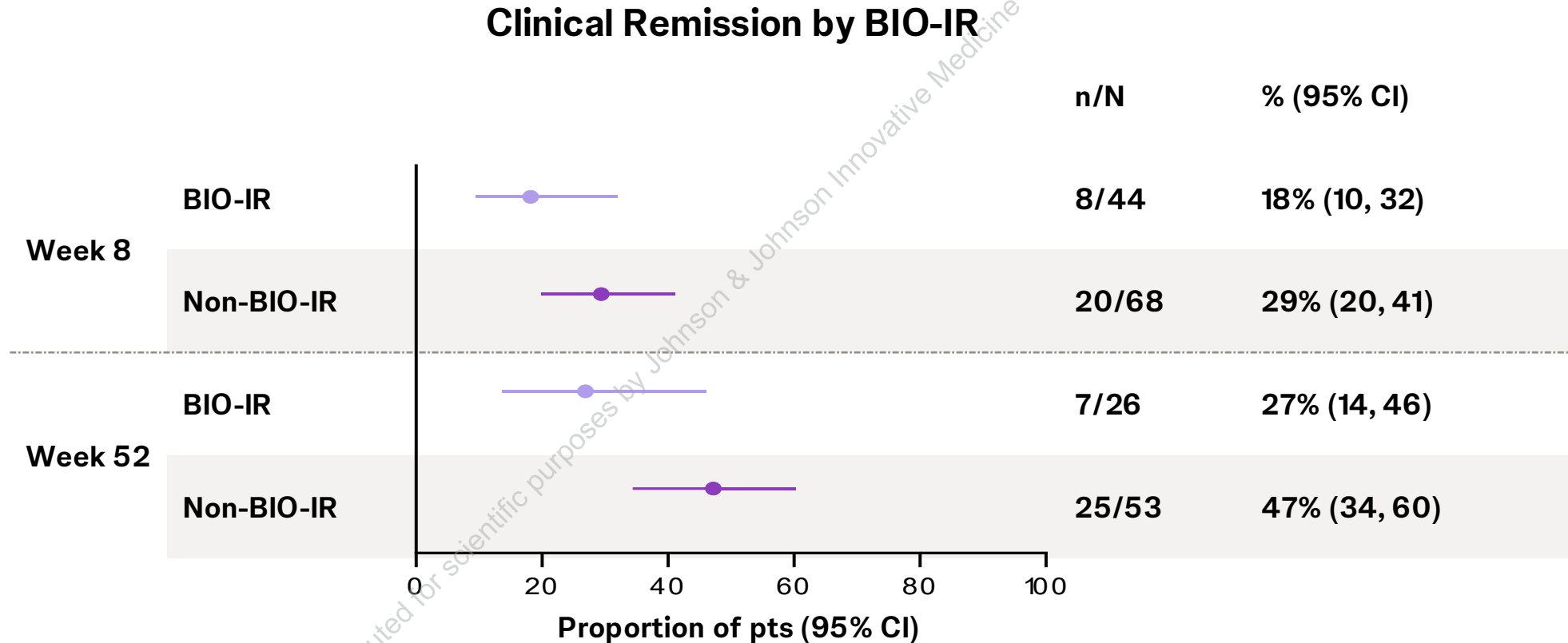


<sup>a</sup>Data are for adults with moderate to severe ulcerative colitis randomized to ustekinumab IV 6 mg/kg in the Phase 3 UNIFI study (Sands BE, et al. *N Engl J Med.* 2019;381:1201-14). CI=confidence interval, IV=intravenous, pts=participants.

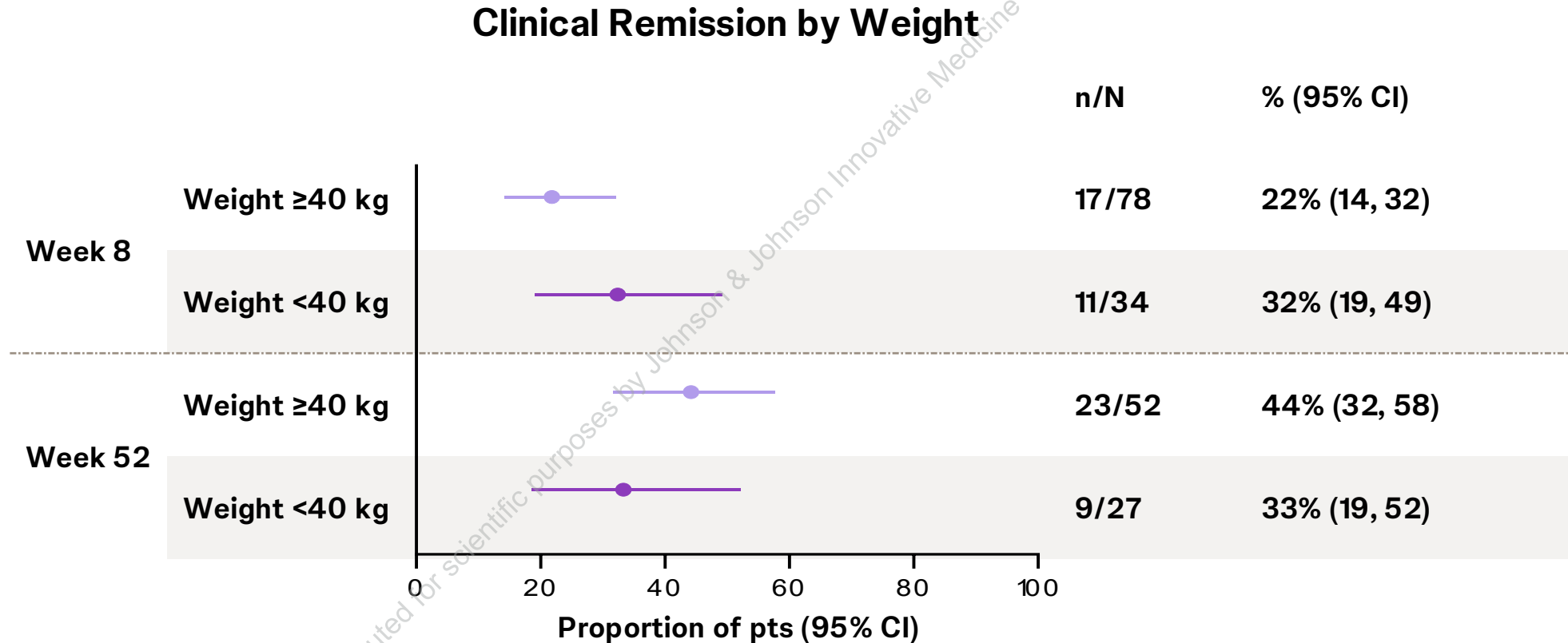
# Primary and Key Secondary Endpoints at Week 52



# Remission Rates by Prior Inadequate Response or Intolerance to Biologic Therapy (BIO-IR)



# Remission Rates by Weight Subgroup



# No New Safety Signals Were Observed (Induction)

## Summary of AEs During the Induction Period (Week 0 to Week 8)

	Ustekinumab IV (N = 112)
<b>Mean weeks of follow-up</b>	8.2
Participants with $\geq 1$ :	
AE	59 (53%)
SAE	6 (5%)
Serious infection <sup>a</sup>	1 (1%)
AE leading to discontinuation	2 (2%)
<b>Most common AEs (<math>\geq 5\%</math>)</b>	
Anemia	12 (11%)
COVID-19	7 (6%)

# No New Safety Signals Were Observed (Maintenance)

## Summary of AEs During the Maintenance Period (Week 8 to Week 52)

	q12w (N = 55)	q8w (N = 54)	Total (N = 109)
<b>Mean weeks of follow-up</b>	36.0	38.1	38.0
Participants with ≥1:			
AE	52 (95%)	48 (89%)	100 (92%)
SAE	2 (4%)	5 (9%)	7 (6%)
Serious infection <sup>a</sup>	0	1 (2%)	1 (1%)
AE leading to discontinuation	3 (5%)	2 (4%)	5 (5%)
<b>Most common AEs (≥5%)</b>			
Ulcerative colitis	21 (38%)	13 (24%)	34 (31%)
Upper respiratory tract infection	13 (24%)	14 (26%)	27 (25%)
Nasopharyngitis	7 (13%)	10 (19%)	17 (16%)
Anemia	5 (9%)	8 (15%)	13 (12%)
Headache	7 (13%)	3 (6%)	10 (9%)
COVID-19	5 (9%)	2 (4%)	7 (6%)
Respiratory tract infection	4 (7%)	2 (4%)	6 (6%)

- No malignancies, deaths, or cases of active tuberculosis were reported during the study
- One opportunistic infection was reported (fungal esophagitis during the maintenance period in the q12w group)

Data are reported for the safety randomized analysis set. Participants are counted only once for given event, regardless of the number of times they experienced the event. Most common AEs by MedDRA version 28.0 Preferred Term. Data from the time of dose adjustment onward are not included. <sup>a</sup>Infections as assessed by the investigator. **AE**=adverse event, **SAE**=serious adverse event, **q8w**=every 8 weeks, **q12w**=every 12 weeks.

# Key Takeaways

- ✓ Ustekinumab induction and maintenance therapy was effective in treating pediatric patients aged 2 to <18 years with moderately to severely active ulcerative colitis
- ✓ Ustekinumab was well tolerated with no new safety signals

# Acknowledgments

UNIFI Jr Investigators	
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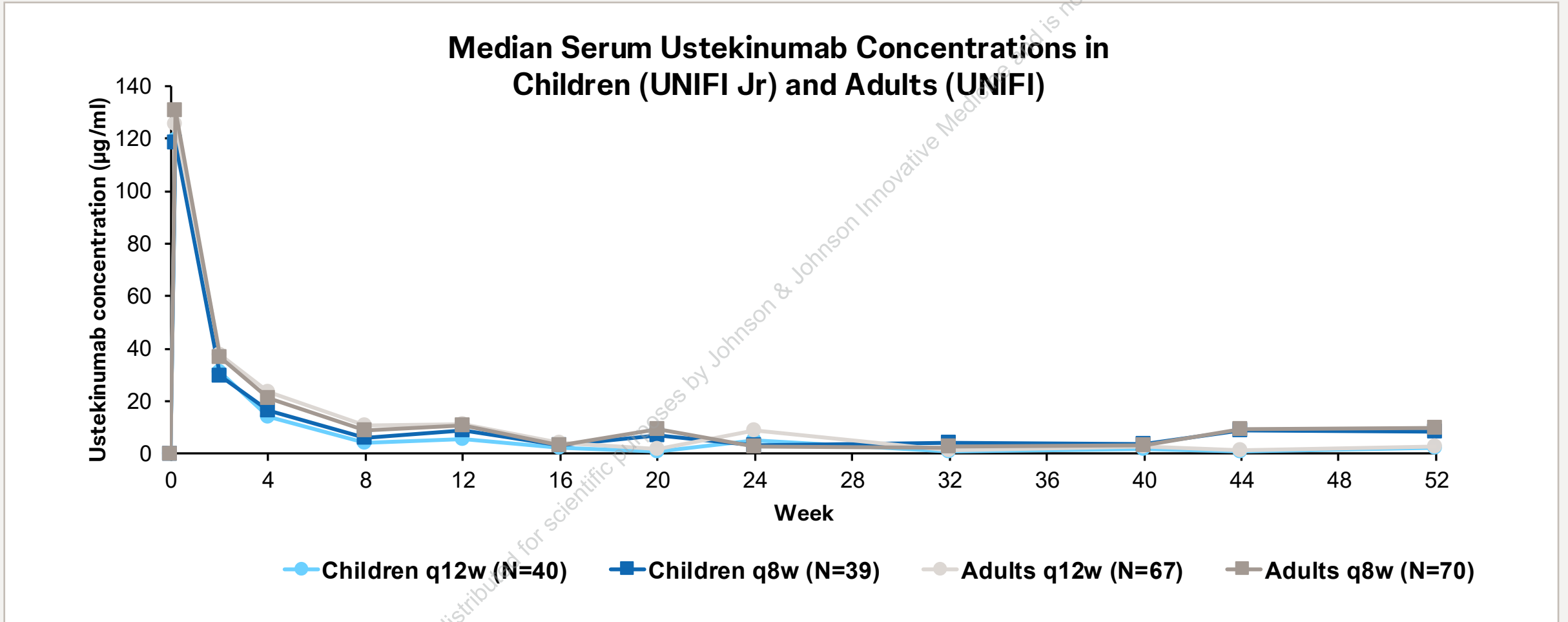


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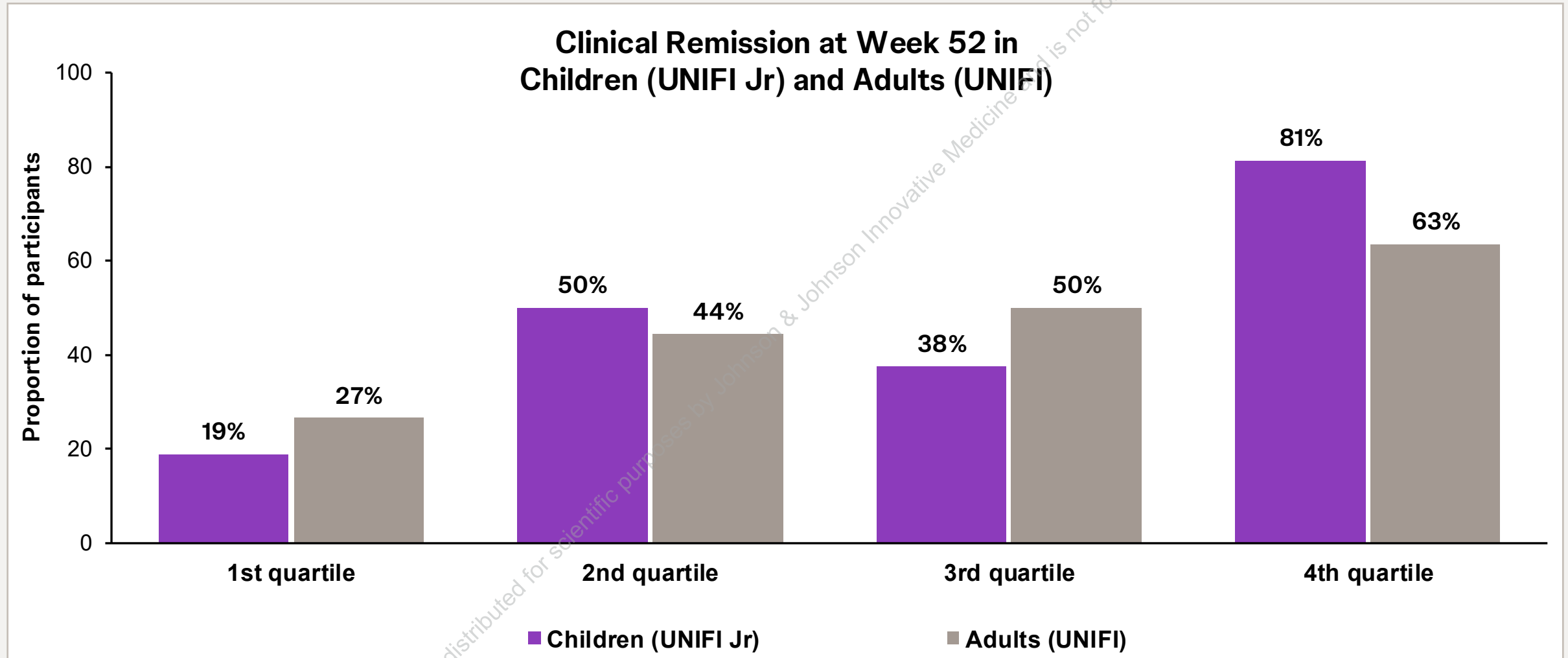
# Back-Up

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# Serum Ustekinumab Concentrations Through Week 52 (Week 8 Clinical Responders)



# Clinical Remission at Week 52 by Pediatric Average Steady-State Trough Ustekinumab Concentration Quartiles



PK clinical responder analysis set.

1st quartile:  $<0.6086175$   $\mu\text{g/mL}$ , 2nd quartile:  $\geq 0.6086175$  and  $<1.7521975$   $\mu\text{g/mL}$ , 3rd quartile:  $\geq 1.7521975$  and  $<3.8968225$   $\mu\text{g/mL}$ ; 4th quartile:  $\geq 3.8968225$ .

# Serum Ustekinumab Concentrations During Maintenance (Randomized Participants; Week 8 through Week 52)

