Safety and Efficacy of Ustekinumab in Paediatric Ulcerative Colitis (UC): Results From the Phase 3 UNIFI Jr Study

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Background

- Ustekinumab is a fully human IL-12 and IL-23 antagonist indicated for the treatment of moderately to severely active ulcerative colitis (UC) in adults[1,2]
- Approved pharmacologic treatment options for paediatric patients with UC remain limited

Objective

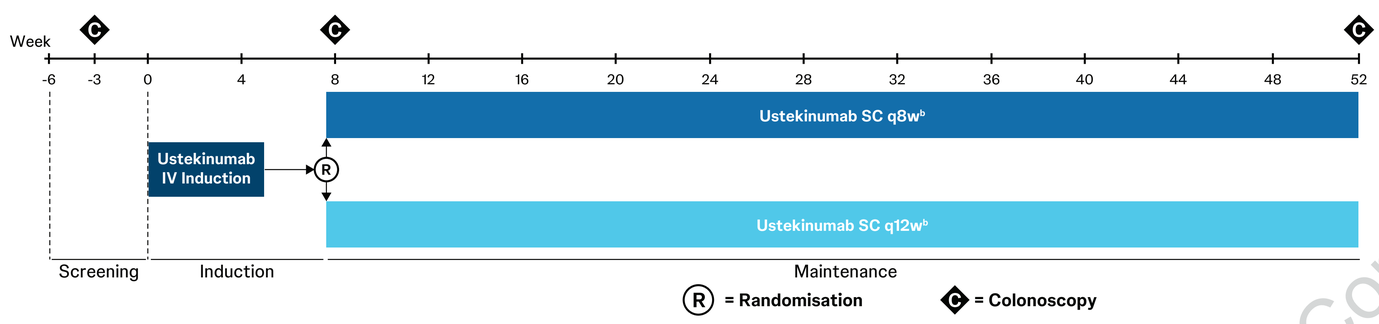
The phase 3 UNIFI Jr study evaluated efficacy and safety of ustekinumab in paediatric participants (pts) with moderately to severely active UC

Methods

UNITI Jr – Study Design

Key inclusion criteria:

- Paediatric pts age 2 to <18 years; weight ≥10 kg
- Moderately to severely active UC[a]
- Inadequate response or intolerant to conventional/ biologic therapy or corticosteroid-dependent



Weight-tiered IV dosing:
- <40 kg: 250 mg/m²
- ≥40 to ≤55 kg: 260 mg
- >55 to ≤85 kg: 390 mg
- >85 kg: 520 mg

Phase 3, multicenter, interventional, treat-through study consisting of an open-label induction period with a single IV ustekinumab induction dose followed by randomisation of all participants at W8 (1:1, double-blind, parallel group), stratified by weight (<40 kg/≥40 kg) and induction W8 clinical response status, to receive blinded SC ustekinumab maintenance therapy q8w or q12w for 44 weeks (to W52)

Primary endpoints:
- Clinical remission at W8
- Clinical remission at W52 in pts with induction response at W8

[a]Baseline Mayo score ≥6, Mayo endoscopy subscore ≥2. [b]Ustekinumab 90 mg SC for ≥40 kg body weight; 60 mg/m² for <40 kg body weight. ***IV****=intravenous,* ***q8w****=every 8 weeks,* ***q12w****=every 12 weeks,* ***SC****=subcutaneous,* ***W****=week.*

Key Takeaways

- **Ustekinumab induction and maintenance therapy was effective in treating paediatric patients aged 2 to <18 years with moderately to severely active UC**
- **Ustekinumab was well tolerated with no new safety signals**

Endpoints & Statistical Considerations

Endpoints

- **Clinical response:** decrease from baseline in modified Mayo score by ≥30% and ≥2 points, with either a decrease from baseline in RB subscore of ≥1 or RB subscore of 0 or 1
- **Clinical remission:** 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
- **Symptomatic remission:** Mayo SF subscore of 0 or 1 and RB subscore of 0, with no increase from baseline in SF subscore
- **Endoscopic improvement:** Mayo endoscopy subscore of 0 or 1 with no friability present on the endoscopy
- **Clinical remission by PUCAI:** PUCAI score <10
- **CS-free clinical remission:** clinical remission and not receiving CS for ≥90 days prior

Handling of intercurrent events and missing data

- Pts who had a colectomy (partial or full) or ostomy, discontinued study intervention due to lack of efficacy or an AE of worsening of UC or other reasons (except COVID-19 reasons other than infection), or had prohibited changes in UC medications were considered not to have achieved the endpoint
- Pts who discontinued study intervention due to COVID-19 reasons (excluding COVID-19 infections) were considered to have missing data from the time of the event onwards (hypothetical strategy)
- Pts who had rescue medication used (initiation or increase above baseline) for treatment of loss of response after W16 for responders and W24 for delayed responders, or had dose adjusted after W16 were considered not to have achieved the endpoint
- After accounting for the above intercurrent events, pts with missing data were considered non-responders

AE*=adverse event,* ***CS****=corticosteroid,* ***PUCAI****=Pediatric Ulcerative Colitis Activity Index,* ***RB****=rectal bleeding,* ***SF****=stool frequency.*

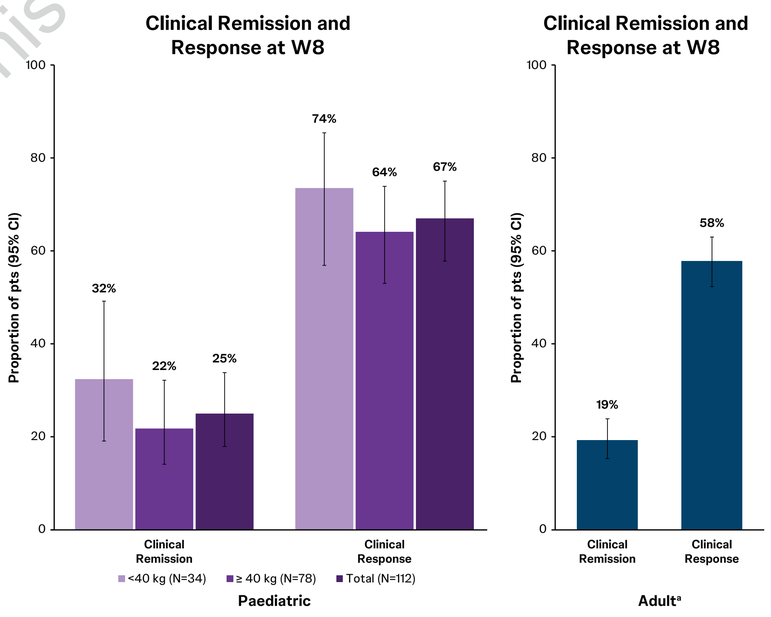
Results

Baseline characteristics

Baseline Characteristics	Ustekinumab SC q12w (N=55)	Ustekinumab SC q8w (N=54)	Ustekinumab SC Total[a] (N=112)
Demographics			
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[b]	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%
Disease Characteristics			
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%[c]	92%[c]
Severe (Mayo score >10), %	9%	6%[c]	7%[c]
PUCAI score, mean (SD)	53.5 (13.0)	51.5 (16.1)	52.9 (14.7)
UC-Related Therapies			
Oral/rectal corticosteroid use at baseline,[d] %	27%	22%	25%
Immunomodulatory drug use at baseline,[e] %	33%	48%	39%
History of inadequate response or intolerance to biologic, %	47%	31%	39%

[a]Includes 3 pts who received ustekinumab at induction W0 but were not randomised at W8 to maintenance treatment. [b]Race not reported for 1 pt (q8w); unknown for 2 pts (q12w and not randomised). [c]1 pt (q8w) had mild disease (Mayo score ≥3 and <6). [d]Excluding budesonide and beclomethasone dipropionate. [e]6-mercaptopurine/azathioprine or methotrexate. ***IQR****=interquartile range,* ***SD****=standard deviation.*

Clinical response and remission rates were similar across weight groups at W8

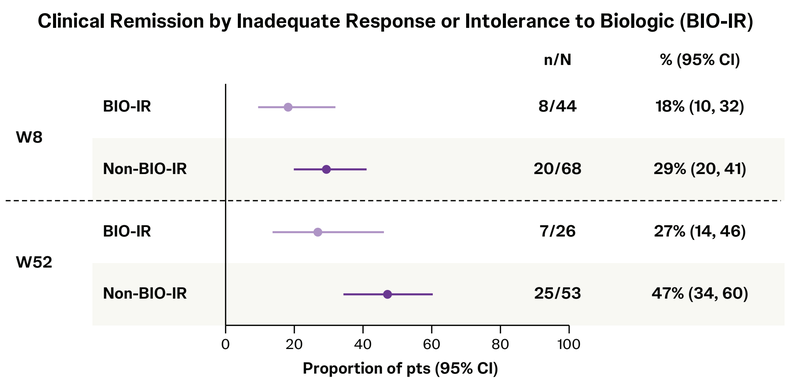


Data summarised using descriptive statistics; no statistical testing was performed/no p-values provided. [a]Data are for ustekinumab-randomised adults with moderate to severe UC from the Phase 3 UNIFI study.[3] ***CI****=confidence interval.*

Primary and key secondary endpoints at W52

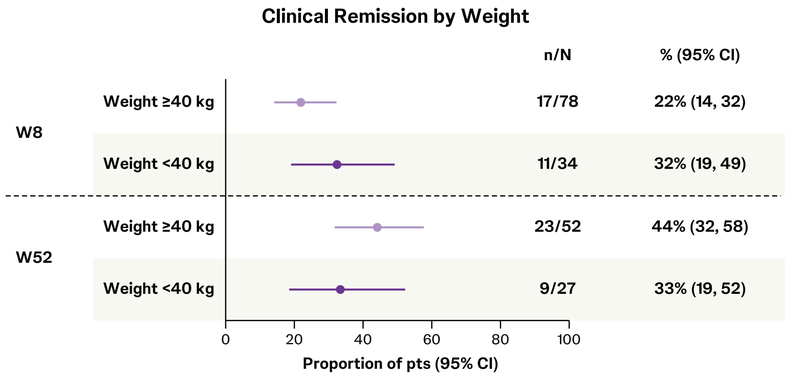
Data summarised using descriptive statistics; no statistical testing was performed/no p-values provided.

Remission rates at W8 and W52 were higher in pts without prior inadequate response or intolerance to biologic therapy



Data summarised using descriptive statistics; no statistical testing was performed/no p-values provided. ***BIO-IR****=inadequate response or intolerance to biologic.*

Remission rates were similar from W8 to W52 in all weight subgroups



Data summarised using descriptive statistics; no statistical testing was performed/no p-values provided.

No new safety signals were observed

Summary of AEs During the Induction Period (W0 to W8)

	Ustekinumab IV (N=112)
Mean weeks of follow-up	8.2
Pts with ≥1:	
AE	59 (53%)
SAE	6 (5%)
Serious infection[a]	1 (1%)
AE leading to discontinuation	2 (2%)
Most common AEs (≥5%)	
Anaemia	12 (11%)
COVID-19	7 (6%)

Data are reported for the safety analysis set. Pts 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.
[a]Infections as assessed by the investigator. ***SAE****=serious adverse event.*

Summary of AEs During the Maintenance Period (W8 to W52)

	q12w (N=55)	q8w (N=54)	Total (N=109)
Mean weeks of follow-up	36.0	38.1	38.0
Pts with ≥1:			
AE	52 (95%)	48 (89%)	100 (92%)
SAE	2 (4%)	5 (9%)	7 (6%)
Serious infection[a]	0	1 (2%)	1 (1%)
AE leading to discontinuation	3 (5%)	2 (4%)	5 (5%)
Most common AEs (≥5%)			
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%)
Anaemia	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%)

Data are reported for the safety randomized analysis set. Pts 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.
[a]Infections as assessed by the investigator.

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

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