Efficacy, Safety, and Pharmacokinetics of Golimumab in Pediatric Patients With Moderately-to-Severely Active Ulcerative Colitis: Results From the Phase 3 Open-Label PURSUIT 2 Study

Dan Turner,[1] Kathleen G. Lomax,[2] Gigi Veereman,[3] Anne M. Griffiths,[4] Jaroslaw Kierkuś,[5] Ben Kang,[6] Katherine Berezny,[2] Lakshmi Padgett,[2] Gary Mao,[2] Yevgeny Zitser,[2] Richard S. Strauss,[2] Jeroen Verhoeven,[2] Omoniyi J. Adedokun,[2] Jeffrey Hyams[7]

[1]The Juliet Keidan Institute of Pediatric Gastroenterology and Nutrition, Shaare Zedek Medical Center, The Hebrew University of Jerusalem, Jerusalem, Israel; [2]Johnson & Johnson, PA, USA and Belgium; [3]Universitair Ziekenhuis, Vrije Universiteit Brussel, Brussels, Belgium; [4]The Hospital for Sick Children, University of Toronto, Toronto, Canada; [5]The Children's Memorial Health Institute, Warsaw, Poland; [6]Kyungpook National University Chilgok Hospital, Daegu, South Korea; [7]Connecticut Children's Medical Center, Hartford CT, USA

Key Takeaways

- **Among these biologic-naïve pediatric patients with moderately-to-severely active UC receiving SC GLM, nearly one-third achieved clinical remission after 6 weeks induction, and over half of those patients maintained clinical remission at Week 54**
- **Among clinical responders at Week 6, approximately one-third or more achieved clinical and endoscopic outcomes at Week 54 with continued SC GLM treatment**
- **The safety and PK profiles were consistent with the known GLM profiles in adults treated for UC**
- **Overall, these results support GLM treatment in pediatric patients with moderately-to-severely active UC**

Background

- Limited approved treatment options are available for pediatric patients with moderately-to-severely active ulcerative colitis (UC)
- Golimumab (GLM) is a subcutaneously (SC) administered anti-tumor necrosis factor alpha (TNFα) biologic agent[1]
- In the Phase 2/3 PURSUIT-SC study of adults with moderately-to-severely active UC, treatment with SC GLM led to clinically meaningful improvements in the signs and symptoms of UC at induction Week 6[2]
  - SC GLM was approved for the treatment of moderately-to-severely active UC in adults in both the United States and Europe in 2013

Objective

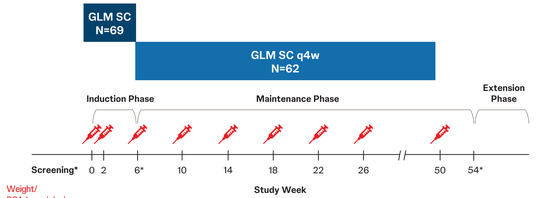
- **To present the efficacy, safety, and pharmacokinetics (PK) of GLM in anti-TNFα-naïve pediatric patients with moderately-to-severely active UC in the PURSUIT 2 study (NCT03596645)**

Methods

- PURSUIT 2 is a Phase 3, multicenter, open-label GLM study in pediatric patients 2 to <18 years of age with moderately-to-severely active UC (**Figure 1**)
  - Patients received SC GLM (weight- or body surface area [BSA]-based doses) at Week 0 and Week 2 during the 6-week induction phase and every 4 weeks (q4w) starting at Week 6 during the 48-week maintenance phase
- Efficacy outcomes at Week 6 and Week 54 (**Table 1**) and safety and PK findings during the induction and maintenance phases are reported
- Although not directly compared in this study, the efficacy and PK results for pediatric patients from PURSUIT 2 are presented alongside an adult reference population from the Phase 2/3 placebo-controlled GLM studies in adults with moderately-to-severely active UC (PURSUIT-SC [NCT00487539] and PURSUIT-M [NCT00488631])[2,3]

Figure 1. Overview of the PURSUIT 2 Study

Study Population:
- 2 to <18 years old (weight ≥10 kg and height >70 cm)
- Moderately-to-severely active UC, defined as baseline Mayo score of 6 to 12 (inclusive) and endoscopy subscore ≥2 assessed by the local endoscopist
- Had not received any biologic anti-TNFα agents
- Current treatment with oral corticosteroids or immunomodulators[a]; OR had an inadequate response, failure to tolerate, or medical contraindication to corticosteroids or immunomodulators[a]; OR had corticosteroid dependence; OR required >3 courses of corticosteroids in the past year



*Endoscopy; [a]Immunomodulators included 6-mercaptopurine, methotrexate, or azathioprine; [b]BSA doses were capped at 200 mg and 100 mg for the 120 mg/m² and 60 mg/m² doses, respectively.

Table 1. Definitions of Clinical and Endoscopic Outcomes

Outcome	Definition
Induction Phase (at Week 6)	
Primary - Clinical remission (Mayo)	A Mayo score of ≤2 points, with no individual subscore >1
Clinical remission (PUCAI)	A PUCAI score of <10
Clinical response	A Mayo score decrease from baseline of ≥30% and ≥3 points, with either a decrease from baseline in the rectal bleeding subscore of ≥1 or a rectal bleeding subscore of 0 or 1
Symptomatic remission	A Mayo stool frequency subscore of 0 or 1 and a rectal bleeding subscore of 0
Endoscopic improvement	A Mayo endoscopy subscore of 0 or 1
Maintenance Phase (at Week 54)	
Maintenance of clinical remission (Mayo)	Clinical remission (Mayo) at Week 54 among patients who were in clinical remission (Mayo) at Week 6
All of the Week 6 outcomes among patients who were in clinical response at Week 6	Same as above
Corticosteroid-free clinical remission among patients who were in clinical response at Week 6	Corticosteroid-free clinical remission (Mayo) at Week 54 among patients who were not receiving corticosteroids for at least 12 weeks before Week 54 (includes oral, parenteral, and rectal routes)

PUCAI=Pediatric Ulcerative Colitis Activity Index.

Analysis Methods

- Patients who had a prohibited change in UC medication, an ostomy or colectomy, used a rescue medication after clinical flare (after Week 6), or discontinued study agent due to lack of efficacy or an adverse event (AE) of worsening of UC prior to the Week 6/Week 54 visit were considered not to have achieved the endpoint
- Data after a discontinuation of study agent due to COVID-19-related reasons were used as available
- Patients who were missing all 4 Mayo subscores (clinical remission [Mayo] or clinical response), >3 PUCAI subscores (clinical remission [PUCAI]), both stool frequency and rectal bleeding subscores (symptomatic remission), all 4 Mayo subscores or endoscopy score (corticosteroid-free clinical remission [Mayo]), or endoscopy score (endoscopic improvement) at Week 6/Week 54 were considered not to have achieved the endpoint at Week 6/Week 54
- For corticosteroid-free clinical remission (Mayo) at Week 54, patients who had a missing value in corticosteroid use had their last value carried forward
- The confidence intervals use the asymptotic formula based on the normal approximation to the binomial distribution

Results

- Most patients were >12 years old (78.3%; mean age, 13.4 ± 3.3 years) or weighed ≥45 kg (75.4%) and had a median (range) duration of disease of 1.43 (0.2-7.8) years (**Table 2**)
- Baseline disease characteristics for the overall GLM pediatric population were representative of pediatric patients with moderate-to-severe UC (**Table 2**)
  - Based on Mayo score, most patients (91.3%) had UC of moderate severity
- At baseline, a majority of patients (97.1%) were receiving UC-related medications: corticosteroids (52.2%), immunomodulators (47.8%), or 5-aminosalicylate (88.4%) (**Table 2**)

Table 2. Patient Characteristics and Medication History at Induction Baseline

	GLM SC N=69
Demographics	
Age in years, mean (SD)	13.4 (3.3)
Age category in years, n (%)	
2 to <6	2 (2.9)
6 to <12	13 (18.8)
12 to <18	54 (78.3)
Female, n (%)	37 (53.6)
Race, n (%)	
White	50 (72.5)
Asian	12 (17.4)
Black or African American	2 (2.9)
Other	5 (7.2)
Weight category, n (%)	
<45 kg	17 (24.6)
≥45 kg	52 (75.4)
Disease characteristics	
UC duration in years, median (range)	1.43 (0.2-7.8)
Mayo score, median (range)	7.0 (5-11)
Mayo score ≥3 to ≤5 (mild), n (%)	2 (2.9)
Mayo score ≥6 to ≤10 (moderate), n (%)	63 (91.3)
Mayo score >10 (severe), n (%)	4 (5.8)
Extensive UC, n (%)	37 (53.6)
C-reactive protein in mg/L, median (range)	1.59 (0.1-92.4)[a]
Fecal calprotectin in mg/kg, median (range)	1590.0 (36-36000)[b]
UC medication history at induction baseline,[c] n (%)	67 (97.1)
Corticosteroid use	36 (52.2)
6-mercaptopurine or azathioprine use	33 (47.8)
Oral 5-aminosalicylate	61 (88.4)

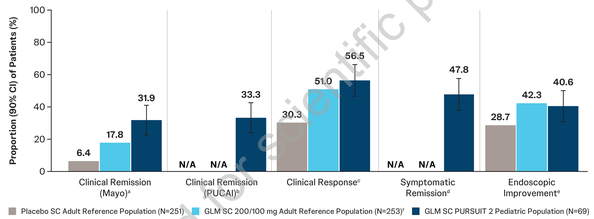
*[a]N=68; [b]N=63; [c]Patients could appear in more than one category. **SD**=standard deviation.*

Patient Disposition

- From Week 0 through Week 6, 7 of 69 (10.1%) of treated patients discontinued GLM, and the most common reason for discontinuation was AE of worsening of UC disease (7.2%)
- From Week 6 through Week 54, 25 of 62 (40.3%) of treated patients discontinued GLM, and the most common reasons for discontinuation were AE of worsening of UC disease and lack of efficacy (14.5% each)

- At Week 6, 31.9% of the GLM-treated pediatric patients were in clinical remission (Mayo) (**Figure 2**)
  - In the PURSUIT-SC adult reference UC population, the remission rates at Week 6 were 17.8% in the GLM group and 6.4% in the placebo group
- Additionally, one-third (33.3%) of the GLM-treated pediatric patients achieved clinical remission by PUCAI, nearly half or more achieved symptomatic remission (47.8%) or clinical response (56.5%), and 40.6% achieved endoscopic improvement (**Figure 2**)
  - In the PURSUIT-SC adult reference UC population, similar proportions of GLM-treated adult patients achieved these outcomes

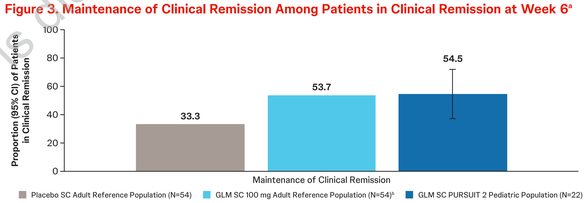
Figure 2. Clinical and Endoscopic Outcomes at Week 6



*[a]A Mayo score ≤2 points, with no individual subscore >1; [b]A PUCAI score <10; [c]A Mayo score decrease from baseline of ≥30% and ≥3 points, with either a decrease from baseline in the rectal bleeding subscore of ≥1 or a rectal bleeding subscore of 0 or 1; [d]A Mayo stool frequency subscore of 0 or 1 and a rectal bleeding subscore of 0; [e]An endoscopy subscore of 0 or 1; [f]In the adult reference study, patients received GLM SC 200 mg at Week 0 and 100 mg at Week 2. CI=confidence interval; **N/A**=not available.*

- Of the 22 pediatric patients who were in clinical remission (Mayo) at Week 6, 54.5% (12 of 22) remained in clinical remission at Week 54 (**Figure 3**)
  - In the PURSUIT-SC adult reference UC population, a similar proportion of GLM-treated patients who were in remission at Week 6 maintained clinical remission at Week 30 (53.7%; 29 of 54)

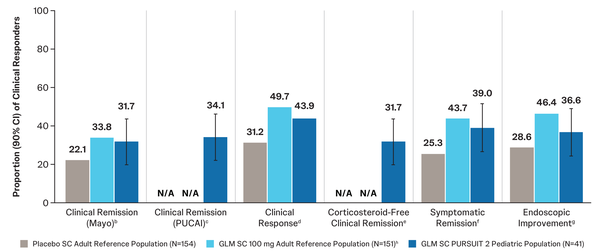
Figure 3. Maintenance of Clinical Remission Among Patients in Clinical Remission at Week 6[a]



[a]Clinical remission was defined as a Mayo score ≤2 points, with no individual subscore >1. Patients who had all 4 Mayo subscores missing at Week 6 (or Week 30/54) or had a missing endoscopy score at Week 30/54 were considered not to be in clinical remission at Week 30/54. [b]In the adult reference study, patients received GLM SC 200 mg at Week 0, 100 mg at Week 2, and 100 mg q4w thereafter.[2]

- At Week 54, of the Week 6 clinical responders, 31.7% of the GLM-treated pediatric patients were in clinical remission (Mayo) and approximately one-third or more achieved the additional clinical and endoscopic outcomes (**Figure 4**)
  - In the PURSUIT-M adult reference UC population, similar proportions of GLM-treated adult patients achieved these outcomes

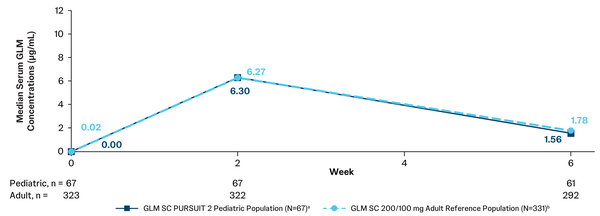
Figure 4. Clinical and Endoscopic Outcomes at Week 54 Among Patients in Clinical Response at Week 6[a]



[a]Patients who were in clinical response at Week 6 and who received ≥1 dose of GLM during the maintenance phase; [b]A Mayo score ≤2 points, with no individual subscore >1; [c]A PUCAI score <10; [d]A Mayo score decrease from baseline of ≥30% and ≥3 points, with either a decrease from baseline in the rectal bleeding subscore of ≥1 or a rectal bleeding subscore of 0 or 1 (note: 90% CI not calculated for this outcome at Week 54); [e]Corticosteroid-free clinical remission (Mayo) at Week 54 among patients who were not receiving corticosteroids for at least 12 weeks before Week 54; [f]A Mayo stool frequency subscore of 0 or 1 and a rectal bleeding subscore of 0; [g]An endoscopy subscore of 0 or 1; [h]In the adult reference study, patients who responded to GLM induction therapy received GLM SC 100 mg q4w from Week 2.[2]

- Through Week 6, serum GLM concentrations observed in the PURSUIT 2 pediatric UC population were comparable to those observed in the PURSUIT-SC adult reference UC population (**Figure 5**)

Figure 5. Median Serum GLM Concentrations



[a]Included patients who received ≥1 GLM dose and had ≥1 valid blood sample drawn for PK analysis during the induction phase (Note: 2 patients were excluded because they received an incorrect GLM SC dose); [b]Included patients in PURSUIT-SC Part 1 and Part 2 who received ≥1 GLM dose at 200/100 mg dose level before Week 6 and had ≥1 valid blood sample drawn for PK analysis before Week 6.

- GLM therapy was well tolerated among pediatric patients, with no new safety concerns identified (**Table 3**)
  - ≥1 treatment-emergent AEs (TEAEs) were reported for 68.1% and 93.5% of patients in the induction and maintenance phases, respectively
  - ≥1 serious AEs were reported for 14.5% and 33.9% of patients in the induction and maintenance phases, respectively, with UC as the most commonly reported serious AE
  - No deaths due to AEs occurred during the study

Table 3. Summary of TEAEs[a]

	Induction Phase (Week 0 to 6) N=69	Maintenance Phase (Week 6 [post-dose] to Week 54) N=62[b]
Average duration of follow-up in weeks	6.3	40
Average exposure (# of administrations)	2.0	9.0
Patients with 1 or more, n (%)[c]		
AEs	47 (68.1)	58 (93.5)
Serious AEs	10 (14.5)	21 (33.9)
AEs leading to death	0	0
AEs leading to discontinuation	6 (8.7)	9 (14.5)
Infections	17 (24.6)	38 (61.3)
Serious infections	1 (1.4)[d]	9 (14.5)[e]
Malignant neoplasms	0	0
Injection-site reactions	2 (2.9)	3 (4.8)

[a]AEs were coded using Medical Dictionary for Regulatory Activities (MedDRA) Version 26.1; [b]Included patients who received ≥1 dose (complete or partial) of golimumab during the maintenance phase; [c]Patients were counted only once for any given event, regardless of the number of times they actually experienced the event; [d]One case of pseudomembranous colitis; [e]Two cases each of cytomegalovirus colitis and pneumonia, and one case each of Clostridium difficile infection, COVID-19, stump appendicitis, and fungal test positive (candida). One case of "UC worsening" was classified as "infection" though the investigator later confirmed no evidence of infection was found.

- Overall, the most frequently-reported AE was UC (60.9%) (**Table 4**)
- The pattern of AEs reported during the maintenance phase was similar to that of the induction phase (**Table 4**)
- Safety findings in pediatric patients with UC were comparable to the adult reference UC populations

Table 4. Common TEAEs (Frequency ≥5% in Either Phase)[a]

	Induction Phase (Week 0 to 6) N=69	Maintenance Phase (Week 6 [post-dose] to Week 54) N=62[b]
Treatment-emergent AE, n (%)[c]		
UC	10 (14.5)	34 (54.8)
Upper respiratory tract infection	-	12 (19.4)
Headache	6 (8.7)	8 (12.9)
COVID-19	5 (7.2)	8 (12.9)
Anemia	-	8 (12.9)
Hematochezia	-	7 (11.3)
Abdominal pain	-	6 (9.7)
Diarrhea	-	5 (8.1)
Influenza	-	5 (8.1)
Pyrexia	4 (5.8)	4 (6.5)
Acne	-	4 (6.5)
Arthralgia	-	4 (6.5)
Nasopharyngitis	-	4 (6.5)
Nausea	-	4 (6.5)

[a]AEs were coded using MedDRA Version 26.1; [b]Included patients who received ≥1 dose (complete or partial) of golimumab during the maintenance phase; [c]Patients were counted only once for any given event, regardless of the number of times they actually experienced the event.

PRESENTED AT: Digestive Disease Week (DDW); May 3-6, 2025; San Diego, CA. **REFERENCES: 1.** Shealy D, et al. *MAbs*. 2010;2:428-439. **2.** Sandborn WJ, et al. *Gastroenterology*. 2014;146:85-95. **3.** Sandborn WJ, et al. *Gastroenterology*. 2014;146:96-109. **ACKNOWLEDGMENTS:** Under the direction of the authors and in accordance with Good Publication Practices, Cindy C. Taylor, PhD, of Certara Synchrogenix, LLC provided writing and editorial assistance. This work was sponsored by Johnson & Johnson. **DISCLOSURES: AMG:** has been a paid consultant for AbbVie, Amgen, Bristol Myers Squibb, Janssen, Lilly, Merck, Organon, Takeda, and Viatris; has received speaker fees from AbbVie, Alimentiv, Janssen, and Takeda; and has received research support for investigator-initiated research from AbbVie. **BK:** has served as a speaker for Celltrion, Janssen, AbbVie, Takeda, and Samsung Bioepis, and has received research funding from Celltrion and Samsung Bioepis. **DT:** reports having received consultation fee, research grant, royalties, or honorarium from Johnson & Johnson, Pfizer, Shaare Zedek Medical Center, Hospital for Sick Children, Ferring, AbbVie, Takeda, Prometheus Biosciences, Lilly, Sorriso Pharmaceuticals, Galapagos, Bristol Meyers Squibb, and Alfasigma. **GM, JV, KB, KGL, LP, OJA, RSS,** and **YZ:** are employees of Johnson & Johnson and own company stock/stock options. **GV:** has served as a medical advisor for the DEVELOP Registry (Johnson & Johnson), Falk Pharma, and Nutricia. **JH:** has served on advisory boards for AbbVie, Janssen, and Lilly and as a consultant for Takeda and Genentech. **JK:** has no conflicts of interest.