



**KIDNEY
WEEK** 2026

Pegcetacoplan for 76 weeks maintains proteinuria reduction and eGFR stabilization for pediatric patients: VALIANT+VALE subgroup analysis

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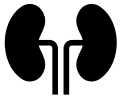
Pediatric patients with C3G and primary IC-MPGN have an unmet clinical need for targeted treatment



C3

C3G and **primary (idiopathic) IC-MPGN** are **rare, complement-mediated diseases** that are often diagnosed in childhood¹

Disease is **driven by C3 dysregulation**, resulting in the **accumulation of downstream effectors** in the glomeruli in C3G, with the addition of immunoglobulins in IC-MPGN, leading to **inflammation and progressive kidney damage** that can ultimately result in **kidney failure**^{2,3}



Despite traditional non-targeted **immunosuppressive therapies** (such as corticosteroids and MMF), approximately 20% of children develop **end-stage kidney disease** within 10–15 years of diagnosis^{3,5,6}



An **early, definitive diagnosis** and **immediate, targeted therapeutic interventions** are needed to **prevent long-term kidney damage**⁴



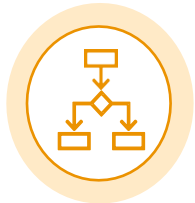
Pegcetacoplan is the only treatment approved to slow the loss of kidney function⁶



1. Patry C, et al. *Pediatr Nephrol.* 2024;39:3569–80. 2. Bomback AS, et al. *Kidney Int Rep.* 2024;10:17–28. 3. Mastrangelo A, et al. *Front Pediatr.* 2020;8:205. 4. Dixon BP, et al. *Clin J Am Soc Nephrol.* 2026;21. 5. Kirpalani A, et al. *Kidney Int Rep.* 2020;5:2313–24. 6. Spartà G, et al. *Clin Kidney J.* 2018;11:479–90. 7. Apellis pharmaceuticals. Prescribing Information. 2026.

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Pegcetacoplan blocks C3 dysregulation and downstream complement activation^{1–7}



In the **phase 3 VALIANT trial** (NCT05067127), adolescents (aged 12–17 years) with C3G or primary IC-MPGN who received pegcetacoplan for 26 weeks had a **significant proteinuria reduction** (74.5% vs placebo) and **stable eGFR** (+9.7 /min/1.73 m² vs placebo)^{8,9}



Efficacy and safety were **sustained with 52 weeks of treatment**¹⁰



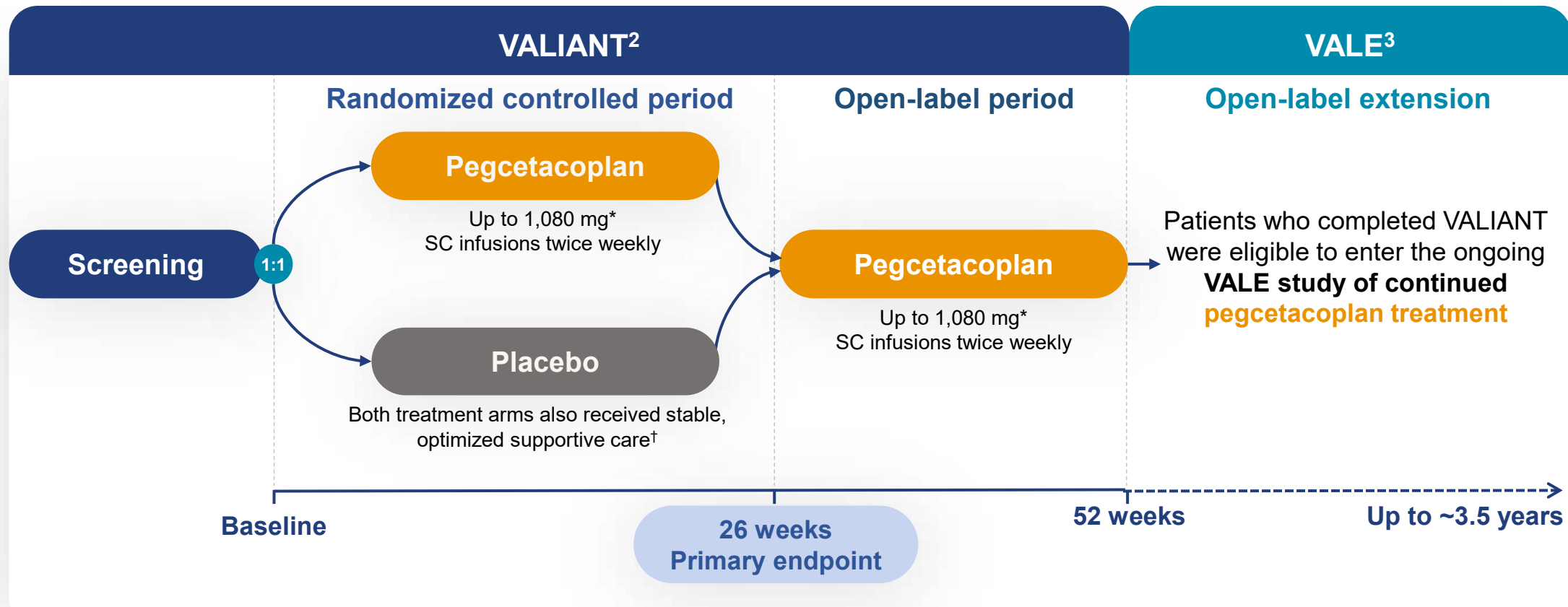
Patients who completed VALIANT were able to enroll in the **VALE open-label extension** (NCT05809531)¹¹



Here, we report **outcomes for adolescents** who received **76 weeks of treatment in VALIANT + VALE**, which provide a **longer-term evaluation** of pegcetacoplan **efficacy and safety**

1. Smith RJH, et al. *Nat Rev Nephrol.* 2019;15:129–43. 2. Zipfel PF, et al. *Front Immunol.* 2019;10:2166. 3. Meuleman MS, et al. *Semin Immunol.* 2022;60:101634. 4. Dixon BP, et al. *Kidney Int Rep.* 2023;8:2284–93. 5. EMPAVELI (pegcetacoplan). Apellis Pharmaceuticals, Inc. 2026. 6. ASPAVELI (pegcetacoplan). Swedish Orphan Biovitrum AB; 2026. 7. Lamers C, et al. *Nat Commun.* 2022;13:5519. 8. Fakhouri F, et al. *N Engl J Med.* 2025;393:2210–20. 9. Vivarelli M, et al. *Clin J Am Soc Nephrol.* 2026. 10. Nester C, et al. IPNA 2025. 11. ClinicalTrials.gov. clinicaltrials.gov/study/NCT05809531.

VALIANT was a double-blind, randomized, placebo-controlled phase 3 study followed by the VALE open-label extension¹



* Adolescents received weight-based dosages. †Stable, optimized antiproteinuric regimens (ACEis, ARBs, SGLT2is) or immunosuppressive therapy (MMF, corticosteroids [prednisone ≤20 mg/d or equivalent]) were permitted provided doses were stable 12 weeks prior to and throughout the randomized controlled period.

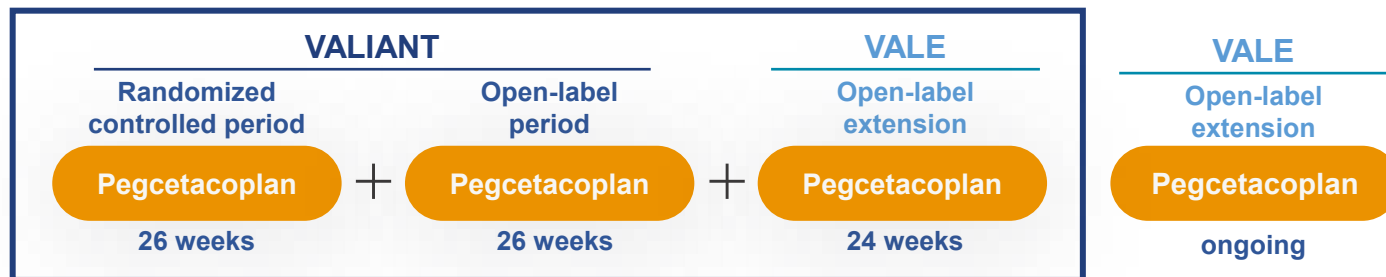
1. Fakhouri et al. *N Engl J Med*. 2025;393:2210–20. 2. ClinicalTrials.gov. VALIANT. clinicaltrials.gov/study/NCT05067127 3. ClinicalTrials.gov. clinicaltrials.gov/study/NCT05809531.

Aim: To evaluate 76 weeks of efficacy and safety of pegcetacoplan in a larger population than previously reported

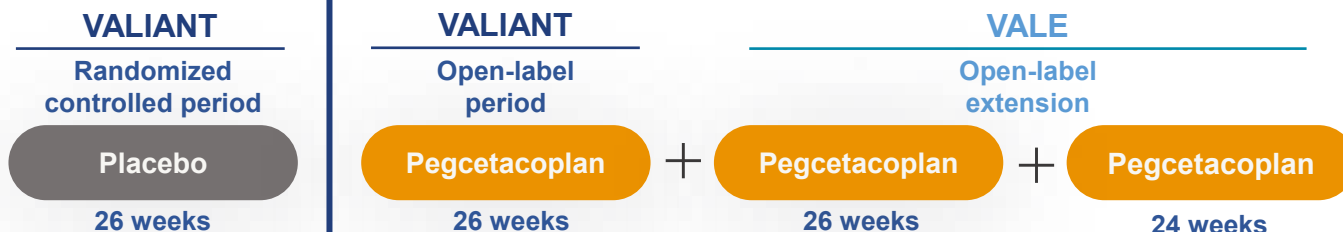
METHODOLOGY FOR THIS ANALYSIS:

- Patients in VALIANT were supplemented with treatment data from the VALE extension study to achieve 76 weeks of pegcetacoplan treatment*
- All data were time-aligned, with baseline defined as the date of pegcetacoplan initiation for all patients

76 weeks of pegcetacoplan treatment in the pegcetacoplan-to-pegcetacoplan arm:



76 weeks of pegcetacoplan treatment in the placebo-to-pegcetacoplan arm:



* No histology data are available for this analysis, as histological assessment was not mandatory at 52 weeks in VALIANT and not included in the VALE study protocol.

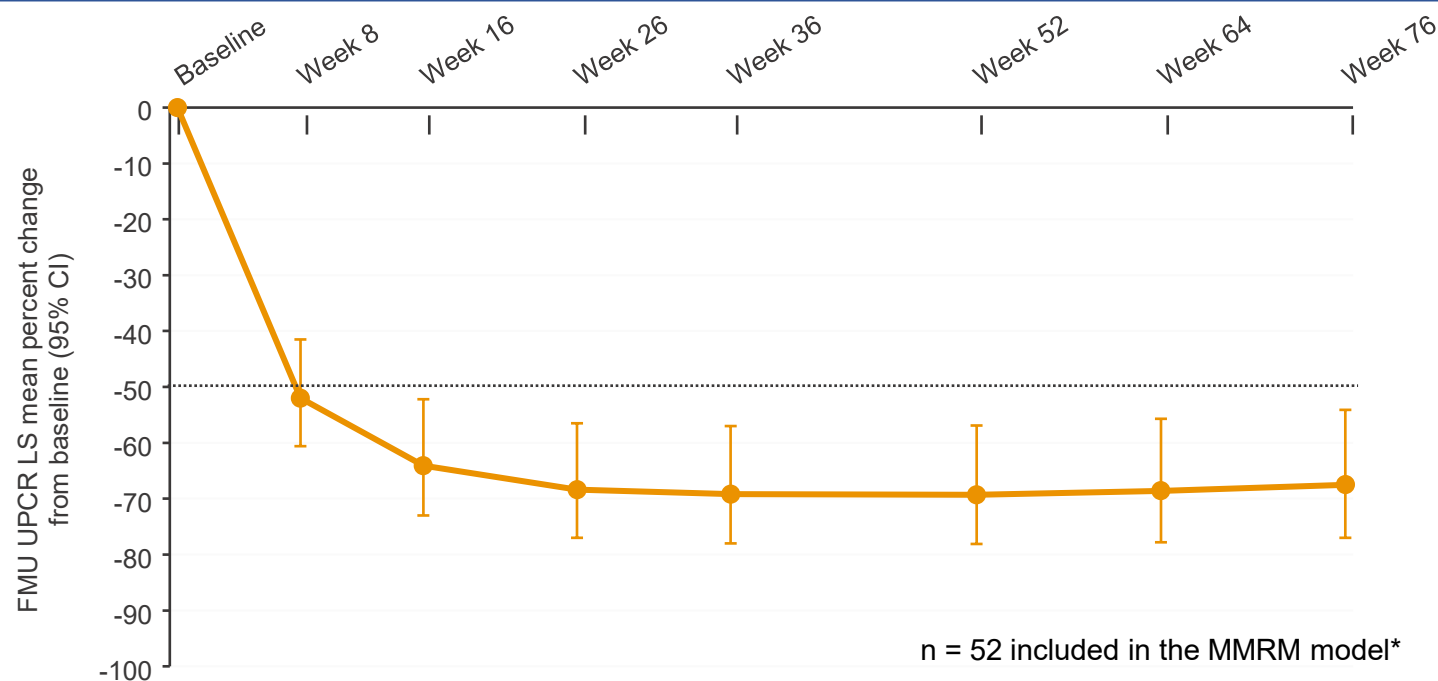
The realigned VALIANT/VALE analysis represents a broad patient population with a large proportion of adolescents

Baseline characteristic	Overall (N = 53)
Age, mean (SD), years	14.7 (1.74)
Sex, female, n (%)	31 (58.5)
Baseline triplicate first-morning spot UPCR, mean (SD), g/g	3.2 (2.90)
Baseline eGFR, mean (SD), mL/min/1.73 m ²	90.1 (32.04)

For participants who received pegcetacoplan during the randomized controlled period of VALIANT, the realigned baseline was defined as the baseline value used in VALIANT. For participants who received placebo during the randomized controlled period of VALIANT, the realigned baseline was defined as the last available non-missing assessment prior to enrolling into the open-label period of VALIANT. Results were based on post-hoc analyses.

Robust proteinuria reductions were maintained through 76 weeks of pegcetacoplan for adolescent patients

Geometric mean change from time-aligned baseline in proteinuria over 76 weeks of pegcetacoplan treatment



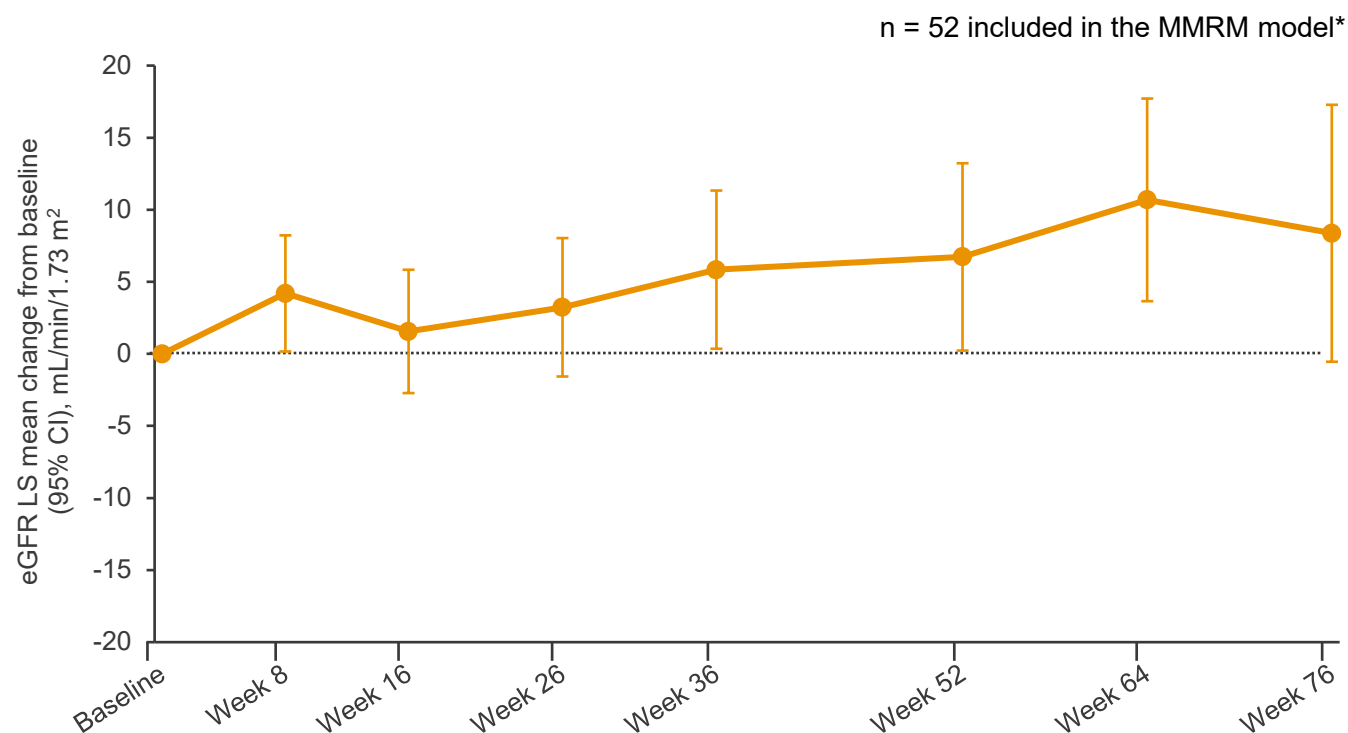
Change from baseline to Week 76:

-67.5%
(95% CI, -77.0 to -54.1)

* Geometric means and % change estimated from exponentiated LS mean differences from an MMRM of log-transformed UPCr. MMRM model including the fixed categorical effect for treatment group, visit, disease type, baseline immunosuppressants use, stratification factors, and the visit-by-treatment group interactions as well as the continuous fixed covariate of baseline log-transformed UPCr, is used to analyze the log-transformed ratio of UPCr at each visit compared with VALIANT baseline. Results were based on post-hoc analyses.

Adolescent patients achieved stable eGFR through 76 weeks of pegcetacoplan

LS mean change from time-aligned baseline in eGFR over 76 weeks of pegcetacoplan treatment



Change from baseline to Week 76:

+8.4
mL/min/1.73 m²
(95% CI, -0.55 to 17.28)

* MMRM model including the fixed categorical effect for treatment group, visit, disease type, baseline immunosuppressant use, stratification factors, and the visit-by-treatment group interactions as well as the continuous, fixed covariate of baseline eGFR, is used to analyze change from baseline eGFR at each visit compared with VALIANT baseline. Results were based on post-hoc analyses. Baseline eGFR was defined as the last eGFR assessment prior to the first dose of pegcetacoplan.

TEAEs in adolescent patients receiving pegcetacoplan through 76 weeks were consistent with the known safety profile for pegcetacoplan

Patients, n (%)				Pegcetacoplan for 76 weeks (N = 53)	
TEAEs				50 (94.3)	
Treatment-related				28 (52.8)	
Severe TEAEs				5 (9.4)	
Serious TEAEs				9 (17.0)	
Encapsulated meningococcal infections				0	
TEAE leading to study discontinuation				1 (1.9)	
Deaths				0	
Common TEAEs (occurring in ≥10% of patients)					
Pyrexia	14 (26.4)	Nausea	9 (17.0)	Influenza	7 (13.2)
Headache	12 (22.6)	Cough	9 (17.0)	Anemia	7 (13.2)
Nasopharyngitis	11 (20.8)	Abdominal pain	8 (15.1)	Fatigue	6 (11.3)
Vomiting	11 (20.8)	Oropharyngeal pain	7 (13.2)	Vitamin D deficiency	6 (11.3)
Diarrhea	9 (17.0)	Pruritus	7 (13.2)	Hypertension	6 (11.3)

TEAEs defined as any new AE that began, or any preexisting condition that worsened in severity, after the first dose of study drug in either VALIANT or VALE and ≤56 days beyond the last dose of study drug. Results were based on post-hoc analyses.

The clinical efficacy of 76 weeks of pegcetacoplan treatment for adolescents was consistent with the overall population of VALIANT



Pegcetacoplan was well tolerated in the adolescent population through up to 76 weeks of treatment, with no new safety signals

Proteinuria reduction maintained after 76 weeks of pegcetacoplan

Adolescents[¶]
(n = 52)

-67.5%
from baseline

Proteinuria
reduction

Results for the overall population will be presented by Dr. Carla Nester on Saturday, October 24

Glomerular C3 clearance after 26 weeks of pegcetacoplan*

74%

achieved C3 staining
reduction ≥ 2 OOM[†]
vs placebo: 12%[‡]

Adults[§]

71%

showed no
detectable deposits
Post-hoc analysis

Histopathology
improvement:
C3 staining

eGFR stabilization maintained after 76 weeks of pegcetacoplan

Adolescents[¶]
(n = 52)

+8.4
mL/min/1.73 m²
from baseline

eGFR
stabilization/
improvement

* Not assessed at Week 76. † Key secondary endpoint. ‡ A fixed sequence testing strategy was used for key secondary endpoints. Key secondary histologic activity score endpoint was not met at week 26; therefore subsequent endpoints including C3 staining reduction and eGFR change were not formally tested and should be interpreted descriptively. § Biopsy was not mandated in adolescents (aged 12-17 years). ¶ All Week 76 endpoints were exploratory and not powered to detect statistically significant differences. 1. Fakhouri F, et al. *N Engl J Med*. 2025;393:2210–20.

Acknowledgements



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Abbreviations

- ACEis, angiotensin-converting enzyme inhibitors; AE, adverse event; ARBs, angiotensin receptor blockers; C3G, C3 glomerulopathy; eGFR, estimated glomerular filtration rate; FMU, first-morning urine; IC-MPGN, immune-complex membranoproliferative glomerulonephritis; IFTA, interstitial fibrosis and tubular atrophy; LS, least squares; MMF, mycophenolate mofetil; MMRM, mixed model for repeated measures; SC, subcutaneous; SE, standard error; SGLT2is, sodium-glucose cotransporter-2 inhibitors; TEAE, treatment-emergent AE; UPCR, urine protein-to-creatinine ratio.