Sobi's Aspaveli® and Phase 3 VALIANT data in Nephrology 52-week update

Conference call for investors and analysts

12 June 2025

Forward-looking statements

This presentation contains certain forward-looking statements with respect to certain of the Company's current expectations and projections about future events. These statements, which sometimes use words such as "intend," "proposed," "plan," "expect," and words of similar meaning, reflect management's beliefs and expectations and involve a number of risks, uncertainties and assumptions that could cause actual results and performance to differ materially from any expected future results or performance expressed or implied by the forward-looking statement. Statements contained in this presentation regarding past trends or activities should not be taken as a representation that such trends or activities will continue in the future. The information contained in this presentation is subject to change without notice and, except as required by applicable law, the Company does not assume any responsibility or obligation to update publicly or review any of the forward-looking statements contained in it. You should not place undue reliance on forward-looking statements, which speak only as at the date of this presentation.

Agenda

Introduction C3G and IC-MGN

Nicholas Webb
VP, Head of Clinical Strategy Immunology

VALIANT 52-week data

Fadi Fakhouri
MD PhD Professor of Nephrology

Summary and Q&A

Guido Oelkers
Chief Executive Officer

Lydia Abad-Franch
Head of R&D and Chief Medical Officer

Pegcetacoplan in C3G and primary IC-MPGN

Nicholas Webb
VP, Head of Clinical Strategy Immunology

C3G and primary IC-MPGN are rare, chronic and heterogeneous kidney diseases[1–5]

A

Clinical background

A group of complement driven renal diseases that typically present with proteinuria and/or haematuria, with symptoms overlapping with other glomerulopathies

B

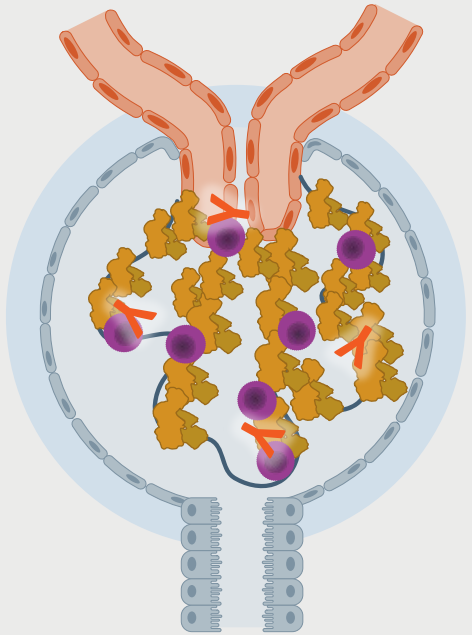
Pathophysiology

C3 overactivation drives accumulation of C3 breakdown products in the glomeruli

This leads to progressive damage that can result in ESKD if left untreated

Key

- Myeloid cell
- C3 deposit
- Immune deposit

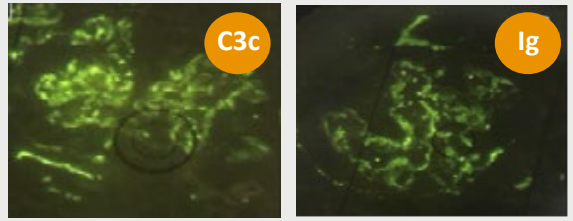


C

Diagnosis

C3 glomerulopathy (C3G)
No or few Igs | **C3 dominant***

C3/Ig deposits (IC-MPGN)
Ig positive | **Not C3 dominant**



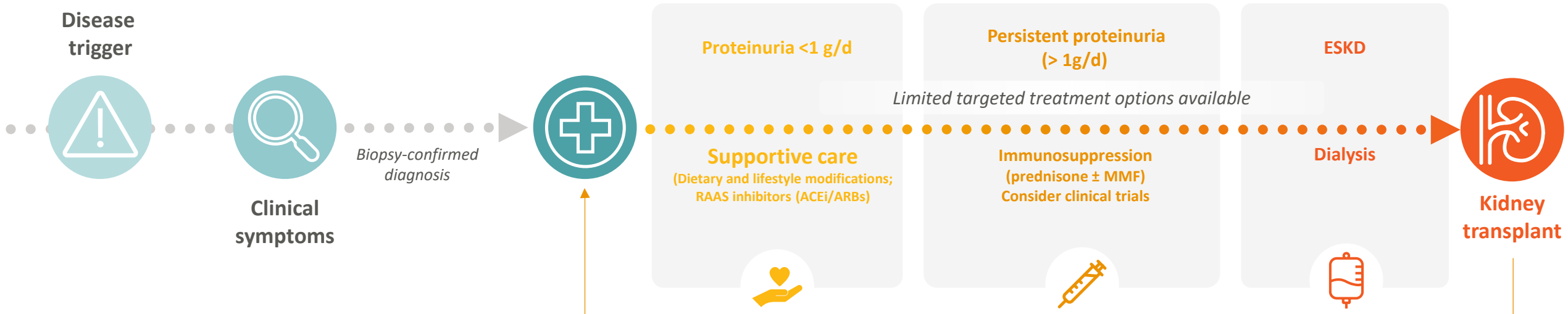
*C3 dominant: C3 is ≥2 orders of magnitude stronger than for any other common immune reactant.

C3/3c, complement 3/3c; ESKD, end-stage kidney disease; IC-MPGN, immune complex-mediated membranoproliferative glomerulonephritis; Ig, immunoglobulin.

References: 1. Smith RJH, et al. Nat Rev Nephrol 2019;15:129–43. 2. Zipfel PF, et al. Mol Immunol 2015;67:21–30. 3.Cook HT & Pickering MC. Nat Rev Nephrol 2015;11:14–22. 4. Noris M & Remuzzi R. Nephrol Dial Transplant 2024;39:202–14. 5.Mastrangelo A, et al. Front Pediatr 2020;8:205.

Despite the current treatment algorithm in C3G and primary IC-MPGN, patients continue to progress to ESKD[1–6]

50% of patients progress to ESKD within 10 years of diagnosis despite treatment



Post-transplant patients have impaired eGFR and require further immunosuppressive treatment

Up to 80% of patients experience clinical disease recurrence after transplantation, with a **76% greater risk of mortality** vs other nephropathies

ACEi, angiotensin-converting enzyme inhibitor; ARB, angiotensin II receptor blocker; C3G, complement 3 glomerulopathy; d, day; eGFR, estimated glomerular filtration rate; ESKD, end-stage kidney disease; IC-MPGN, immune complex-mediated membranoproliferative glomerulonephritis; MMF, mycophenolate mofetil; RAAS, renin-angiotensin-aldosterone system.

References: 1. Caravaca-Fontán F, et al. Nephron 2020;144:272–80. 2. Michels MAHM, et al. Pediatr Nephrol 2022;37:601–12. 3. Vivarelli M, et al. Pediatr Nephrol 2021;37:521–35. 4.Jefferson JA. Clin J Am Soc Nephrol 2018;13:1264–75. 5. O'Shaughnessy MM, et al. J Am Soc Nephrol 2017;28:632–44. 6. Heiderscheit AK, et al. Am J Med Genet C Semin Med Genet 2022;190C:344–57.

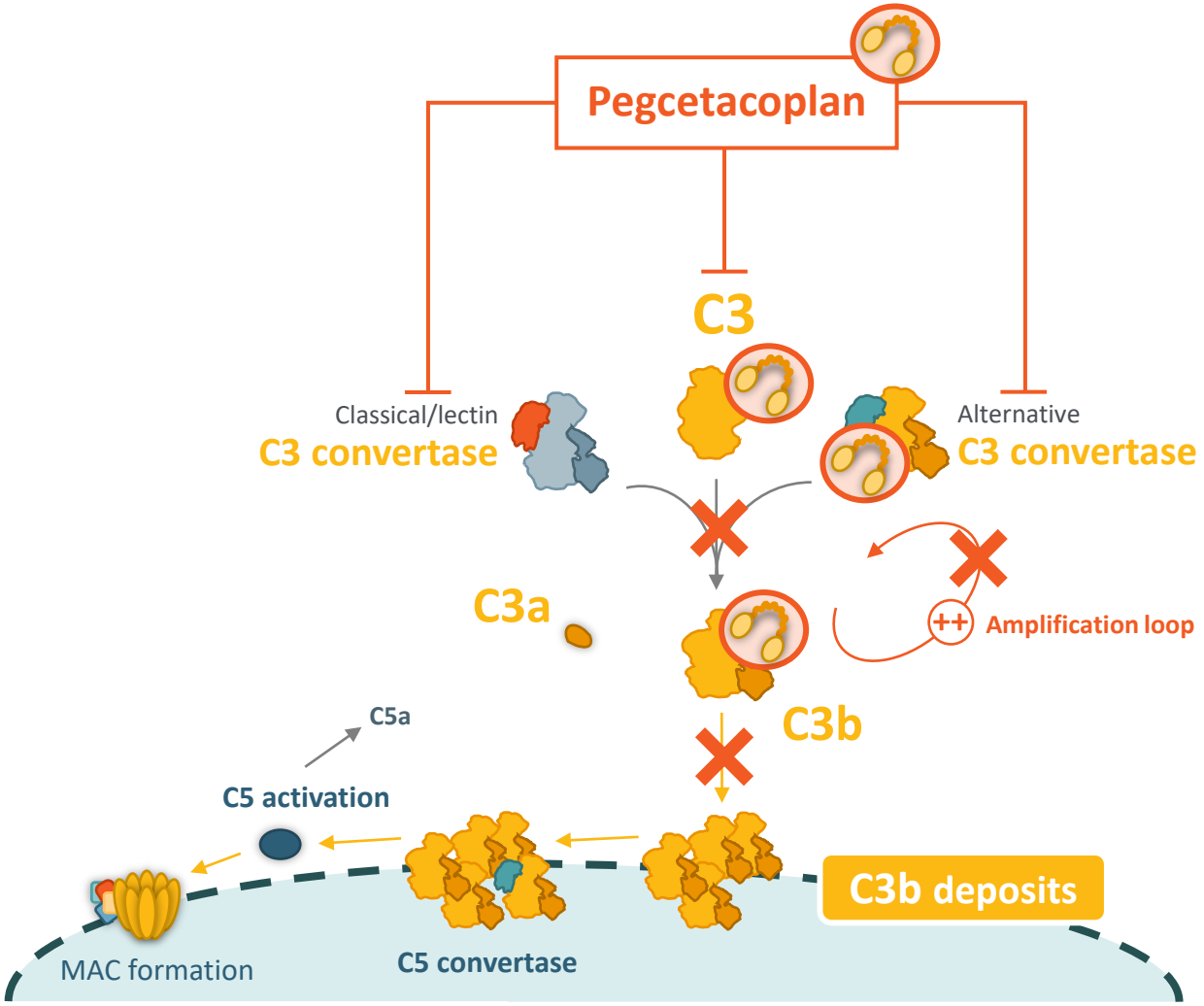
Pegcetacoplan, a C3 and C3b inhibitor, targets C3 dysregulation to preserve kidney function and prevent disease progression

Pegcetacoplan

Selectively binds to **C3** and **C3b**, blocking C3 cleavage by all convertases and downstream effectors of complement activation[1–5]

Assessed in patients with C3G and IC-MPGN in Phase 2 studies[6,7]

Under **Phase 3 investigation in adults and adolescents with C3G and primary IC-MPGN**, either in native kidneys or post-transplant[8,9]



Pegcetacoplan in C3G and primary IC-MPGN is investigational and has not been reviewed or approved for C3G/primary IC-MPGN by any regulatory authority.

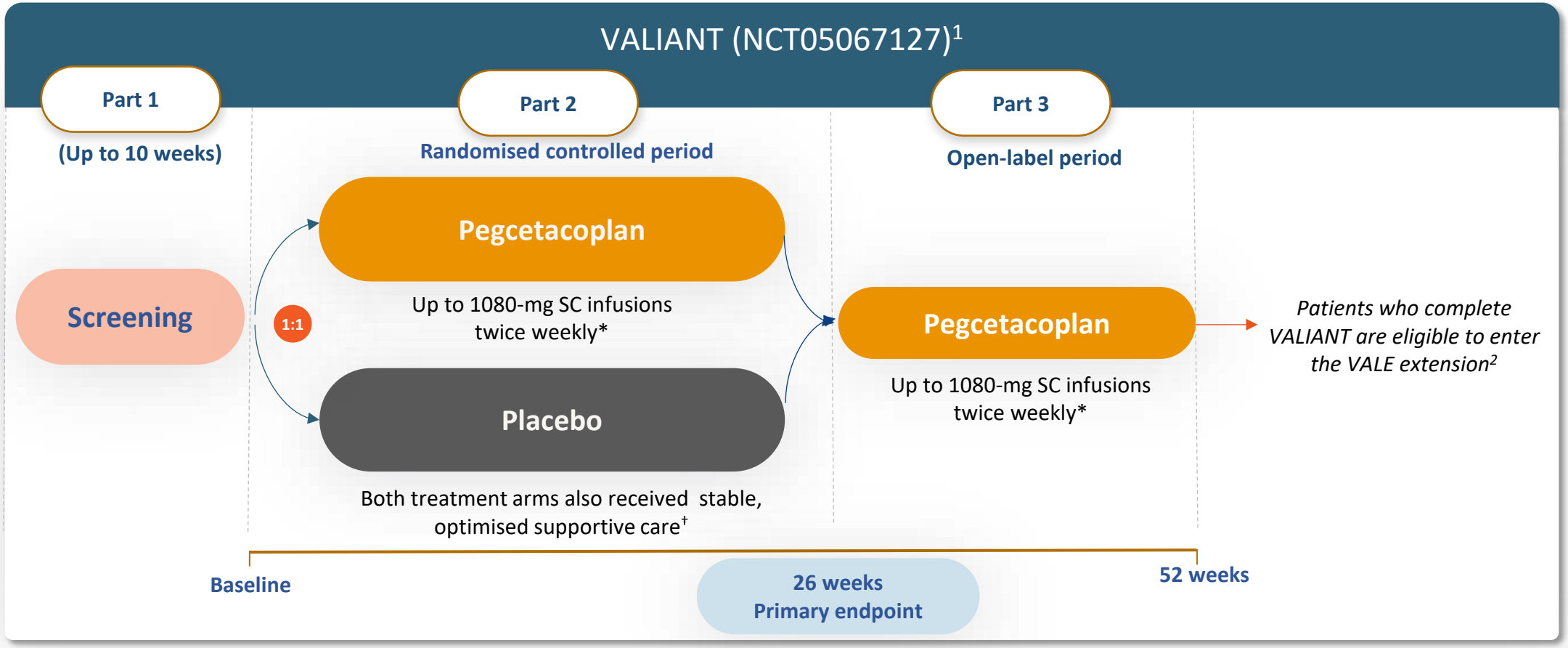
C3/3a/3b/5/5a, complement 3/3a/3b/5/5a; C3G, C3 glomerulopathy; IC-MPGN, immune complex-mediated membranoproliferative glomerulonephritis; MAC, membrane attack complex.

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. US Prescribing Information: EMPAVELI® (pegcetacoplan) injection, for subcutaneous use, 02/2024. Accessed 12 September 2024; 5. EMA Summary of Product Characteristics: ASPAVELI 1 080 mg solution for infusion, 12/2021. Accessed 12 September 2024; 6. Dixon BP, *et al. Kidney Int Rep* 2023;8:2284–93; 7. Bomback A, et al. Presented at American Society of Nephrology Kidney Week 2023 (Poster SA-PO923); 8. Dixon BP, et al. Presented at American Society of Nephrology Kidney Week 2023 (Poster 048); 9. ClinicalTrials.gov identifier: NCT05809531. Last update posted 12 March 2024. Accessed 12 September 2024.

Pegcetacoplan for C3G and primary (idiopathic) IC-MPGN: 52-week results from the Phase 3 VALIANT trial show sustained efficacy

Fadi Fakhouri, MD PhD
Professor of Nephrology
Centre Hospitalier Universitaire Vaudois
Lausanne, Vaud, Switzerland

VALIANT: Double-blind Phase 3 study



* All adults and adolescents weighing ≥50 kg self administered 1080 mg/20 mL. Adolescent patients weighing 30–34 kg received 540 mg/10 mL for the first 2 doses, then 648 mg/12 mL. Adolescent patients weighing 35–49 kg received 648 mg/12 mL for the first dose, then 810 mg/15 mL.

† Stable, optimized antiproteinuric regimens: ACEis, ARBs, SGLT2is, MMF, and corticosteroids (prednisone ≤20 mg/d or equivalent) were permitted.

1. Dixon BP, et al. ASN Kidney Week 2023. Nov. 2–5, 2023. Abstract INFO12-SA. **2.** https://clinicaltrials.gov/study/NCT05809531. Accessed May 20, 2025.

VALIANT: Eligibility criteria

Key eligibility criteria

Inclusion

- **Adolescents** (12–17 y) **or adults** (≥18 y)
- **Diagnosis of primary C3G or IC-MPGN** (with or without previous renal transplant)
- **MMF and corticosteroids** (prednisone ≤20 mg/d or equivalent) permitted

Exclusion

- **>50% global glomerulosclerosis or interstitial fibrosis on renal biopsy**

Other eligibility criteria

Inclusion

- Evidence of active disease*
- ≥1 g/d of proteinuria on screening urine collection and UPCR ≥1 g/g in ≥2 first-morning spot urine samples
- eGFR ≥30 mL/min/1.73 m^{2}†
- Mandatory vaccination against *Streptococcus pneumoniae*, *Neisseria meningitidis* (types A, C, W, Y, and B), and *Haemophilus influenzae* (type B)
- Stable, optimized antiproteinuric regimens: ACEis, ARBs, SGLT2is

Exclusion

- Evidence of transplant rejection
- Diagnosis of secondary C3G or IC-MPGN
- Severe infection within 14 days prior to first dose
- Recurrent or chronic severe infections or history of meningococcal disease
- Previous exposure to pegcetacoplan or another complement inhibitor
- Evidence of improving renal disease

*In adults or adolescents with a baseline kidney biopsy collected during screening or a historic biopsy collected within 28 weeks prior to randomisation, ≥2+ C3 staining in kidney biopsy. In adolescents without a baseline kidney biopsy, ≥1 of the following: plasma sC5b-9 level > ULN during screening, serum C3 < LLN during screening, active urine sediment during screening, presence of C3 nephritic factor within 6 months of screening.

† Calculated using the CKD-Epi equation for adults or the Bedside Schwartz equation for adolescents.

1. Nester CM et al. Presented at American Society of Nephrology Kidney Week 2024 (Oral SA-OR92) **2.** Nester C, et al. *Clin J Am Soc Nephrol* 2024;19:1201–8.

VALIANT: Primary and key secondary endpoints

Primary (week 26)

- **Log-transformed ratio of UPCR** compared with baseline

Key Secondary (week 26)

- Proportion of participants **achieving a composite renal endpoint**
- Proportion of participants with **a reduction of ≥50% in UPCR** from baseline
- **Change in the activity score of the C3G histologic index score** from baseline
- **Decreased C3 staining on renal biopsy** from baseline
- **Change in eGFR** from baseline

Exploratory (week 52)

- Primary and secondary endpoints

Safety

- **Incidence and severity of TEAEs** over 52 weeks
- **Graft rejection and loss**

1. Nester CM et al. Presented at American Society of Nephrology Kidney Week 2024 (Oral SA-OR92) **2.** Nester C, et al. *Clin J Am Soc Nephrol* 2024;19:1201–8.

VALIANT included a **broad patient population**

Baseline characteristic	Pegcetacoplan	Placebo
Patients, n (%)	63 (100.0)	61 (100.0)
Age, mean (SD), y	28.2 (17.1)	23.6 (14.3)
Sex, female, n (%)	37 (58.7)	33 (54.1)
Race, white, n (%)	45 (71.4)	46 (75.4)
Baseline 24-h UPCR, mean (SD), g/g	4.0 (2.9)	3.3 (2.4)
Baseline triplicate first morning spot UPCR, mean (SD), g/g	3.1 (2.4)	2.5 (2.0)
Baseline eGFR,* mean (SD), mL/min/1.73 m^2	78.5 (34.1)	87.3 (37.2)
Underlying disease based on screening biopsy, n (%)		
C3G	51 (81.0)	45 (73.8)
C3GN	45 (71.4)	41 (67.2)
DDD	4 (6.3)	4 (6.6)
Undetermined	2 (3.2)	0
Primary IC-MPGN	12 (19.0)	16 (26.2)
Time since diagnosis, mean (SD), y	3.6 (3.5)	3.8 (3.6)
Post-transplant recurrent disease, n (%)	5 (7.9)	4 (6.6)

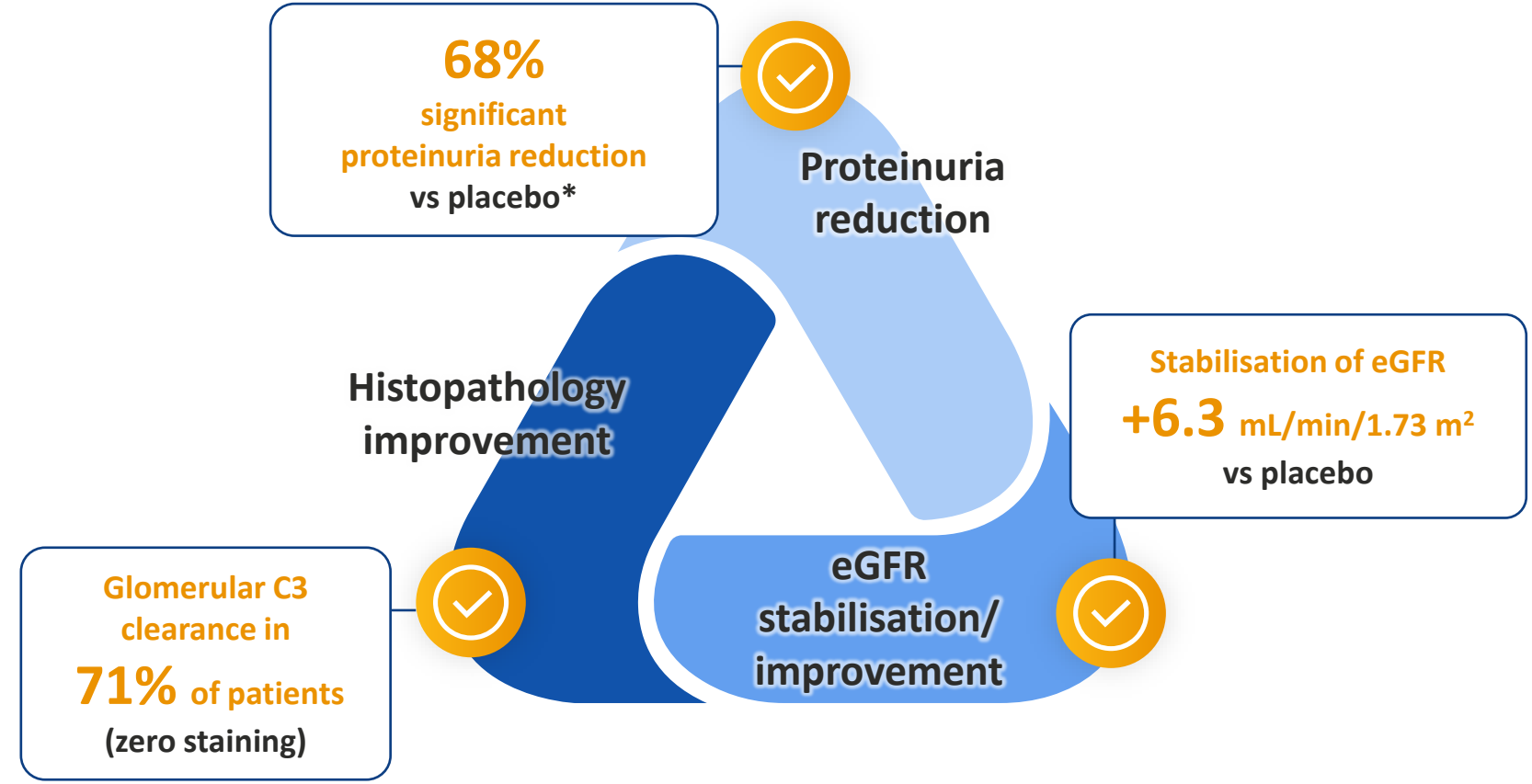
Calculated using the CKD-Epi equation for adults or the Bedside Schwartz equation for adolescents.

1. Nester CM et al. Presented at American Society of Nephrology Kidney Week 2024 (Oral SA-OR92) **2.** Nester C, et al. *Clin J Am Soc Nephrol* 2024;19:1201–8.

VALIANT overall study results (26 weeks): **pegcetacoplan's efficacy** in C3G and primary IC-MPGN[1]

Kidney Health Initiative (KHI) consensus[2]:

Favorable treatment effect on **histopathology, proteinuria and eGFR**



* Consistent across subgroups (age, disease type, transplant status).

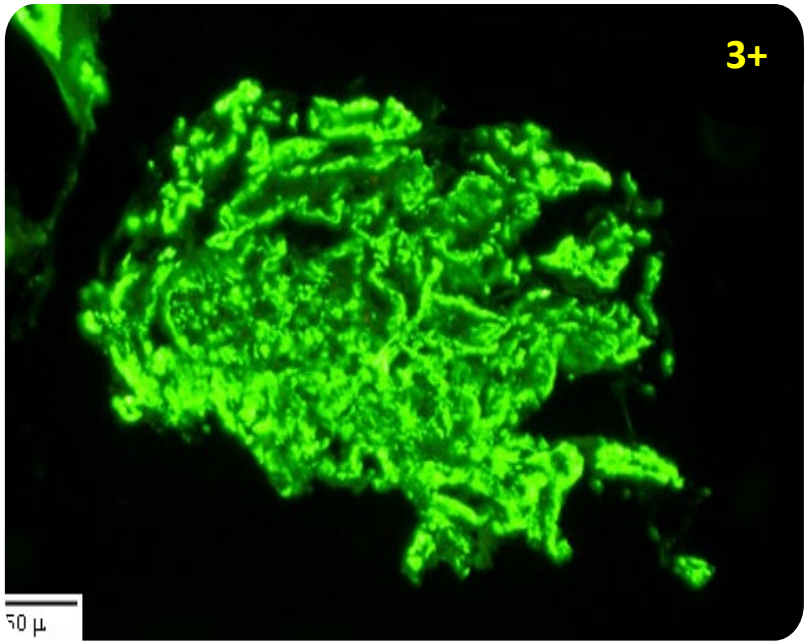
C3G, C3 glomerulopathy; eGFR, estimated glomerular filtration rate; IC-MPGN, immune complex membranoproliferative glomerulonephritis.

1. Nester CM et al. Presented at American Society of Nephrology Kidney Week 2024 (Oral SA-OR92) **2.** Nester C, et al. *Clin J Am Soc Nephrol* 2024;19:1201–8.

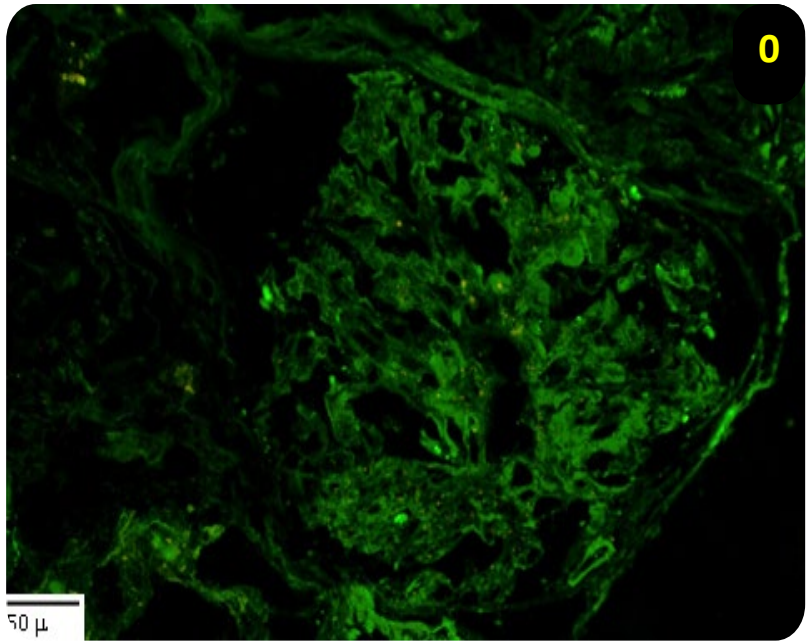
VALIANT (26 weeks): pegcetacoplan's glomerular C3 clearance

Biopsies from C3G native kidney patient who received pegcetacoplan

Baseline



Week 26



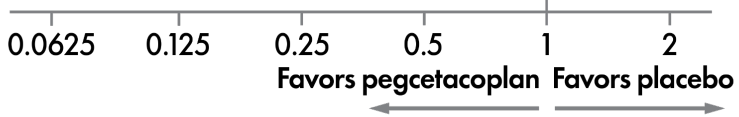
71.4% (25/35) of pegcetacoplan-treated patients

achieved 0 intensity staining

1. Nester CM et al. Presented at American Society of Nephrology Kidney Week 2024 (Oral SA-OR92) **2.** Nester C, et al. *Clin J Am Soc Nephrol* 2024;19:1201–8.

VALIANT (26 weeks): pegcetacoplan's **consistent efficacy** in proteinuria reduction in **broad patient subgroups**

		Geometric mean ratio (95% CI)	Relative reduction (95% CI)	
Overall				
Overall	Pegcetacoplan (N = 63) vs placebo (N = 61)		68.1% (57.3, 76.2)	*P*<.0001
Age Group				
Adolescents (12–17 yr)	Pegcetacoplan (n = 28) vs placebo (n = 27)		74.5% (58.5, 84.3)	*P*<.0001
Adults (≥18 yr)	Pegcetacoplan (n = 35) vs placebo (n = 34)		62.5% (47.1, 73.4)	*P*<.0001
Disease type				
C3G	Pegcetacoplan (n = 51) vs placebo (n = 45)		65.8% (54.0, 74.6)	*P*<.0001
IC-MPGN	Pegcetacoplan (n = 12) vs placebo (n = 16)		73.7% (38.5, 88.7)	*P*=.002
Transplant status				
No transplant	Pegcetacoplan (n = 58) vs placebo (n = 57)		67.5% (55.8, 76.1)	*P*<.0001
Posttransplant recurrence	Pegcetacoplan (n = 5) vs placebo (n = 4)		64.9% (12.9, 85.9)	*P*=.024
Baseline FMU UPCR group				
<3 g/g	Pegcetacoplan (n = 39) vs placebo (n = 45)		64.8% (51.3, 74.6)	*P*<.0001
≥3 g/g	Pegcetacoplan (n = 24) vs placebo (n = 16)		72.1% (51.7, 83.9)	*P*<.0001
Baseline eGFR				
<60 mL/min/1.73 m^2	Pegcetacoplan (n = 21) vs placebo (n = 20)		70.1% (47.8, 82.8)	*P*<.0001
≥60 mL/min/1.73 m^2	Pegcetacoplan (n = 42) vs placebo (n = 41)		66.1% (51.6, 76.2)	*P*<.0001
IS treatment*				
Yes	Pegcetacoplan (n = 48) vs placebo (n = 42)		70.3% (58.3, 78.8)	*P*<.0001
No	Pegcetacoplan (n = 15) vs placebo (n = 19)		64.5% (36.6, 80.1)	*P*=.0005



IS treatment was based on "immunosuppressants" and/or "corticosteroids for systemic use" per Anatomical Therapeutic Chemical level 2.

1. Vivarelli M, et al. Presented at ERA 2025. 2. Kavanagh DC, et al. Presented at ERA 2025.

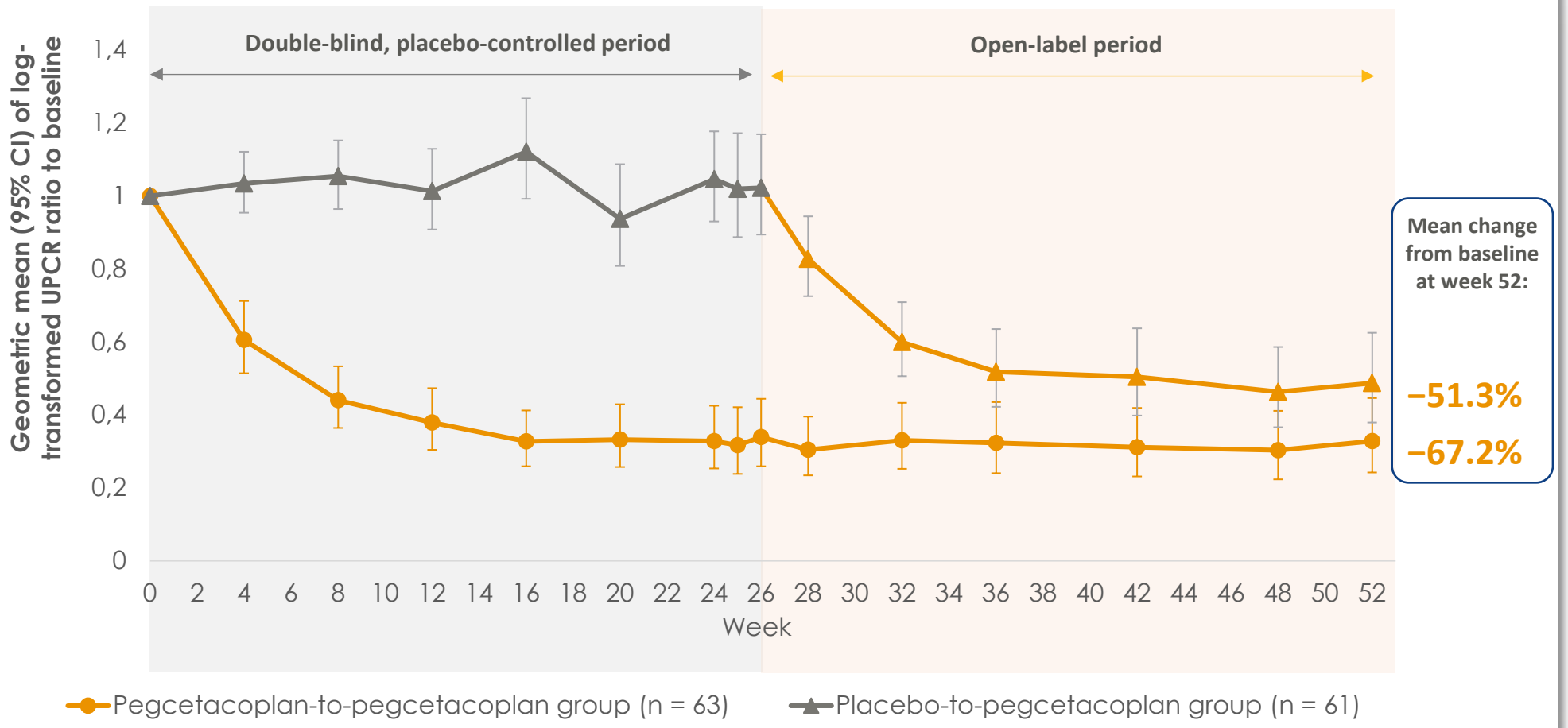
VALIANT (26 weeks): pegcetacoplan's **efficacy** in key populations

	Proteinuria reduction vs placebo	**eGFR stabilisation/ improvement** vs placebo	**Histopathology improvement** C3 clearance ≥2 OOM
Nephrotic range UPCR ≥3 g/g	**72.1%** reduction	**+16.2** mL/min/1.73 m^2	**84.6%** of patients
Concomitant immunosuppression	**70.3%** reduction	**+6.8** mL/min/1.73 m^2	**69.2%** of patients
Adolescents 12–17 years	**74.5%** reduction	**+9.7** mL/min/1.73 m^2	biopsies not mandatory for adolescents

1. Vivarelli M, et al. Presented at ERA 2025. 2. Kavanagh DC, et al. Presented at ERA 2025.

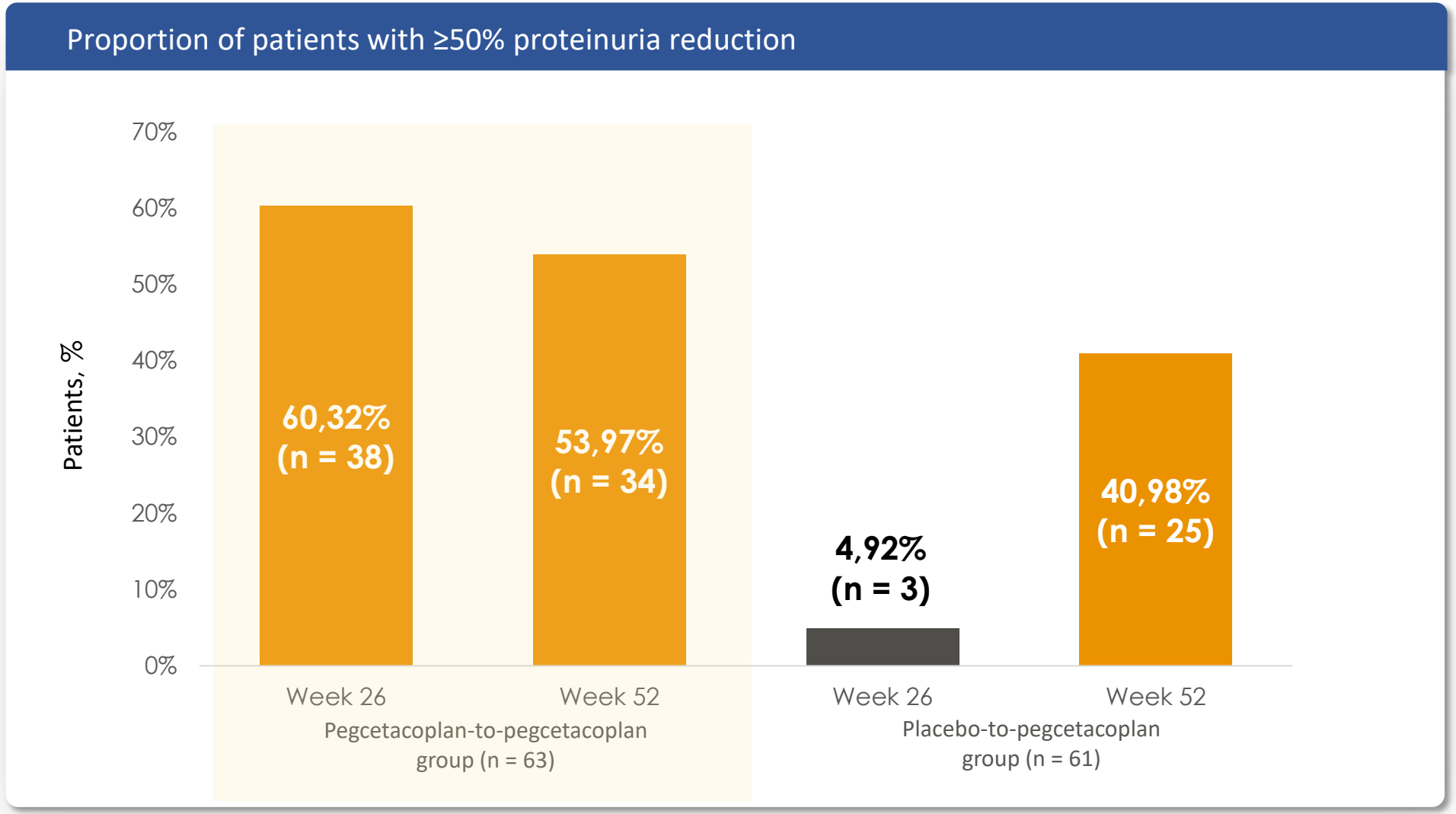
Robust **proteinuria reductions** at week 26 were **maintained** through week 52

Change in proteinuria according to treatment group



1. Fakhouri F, et al. Presented at ERA 2025.

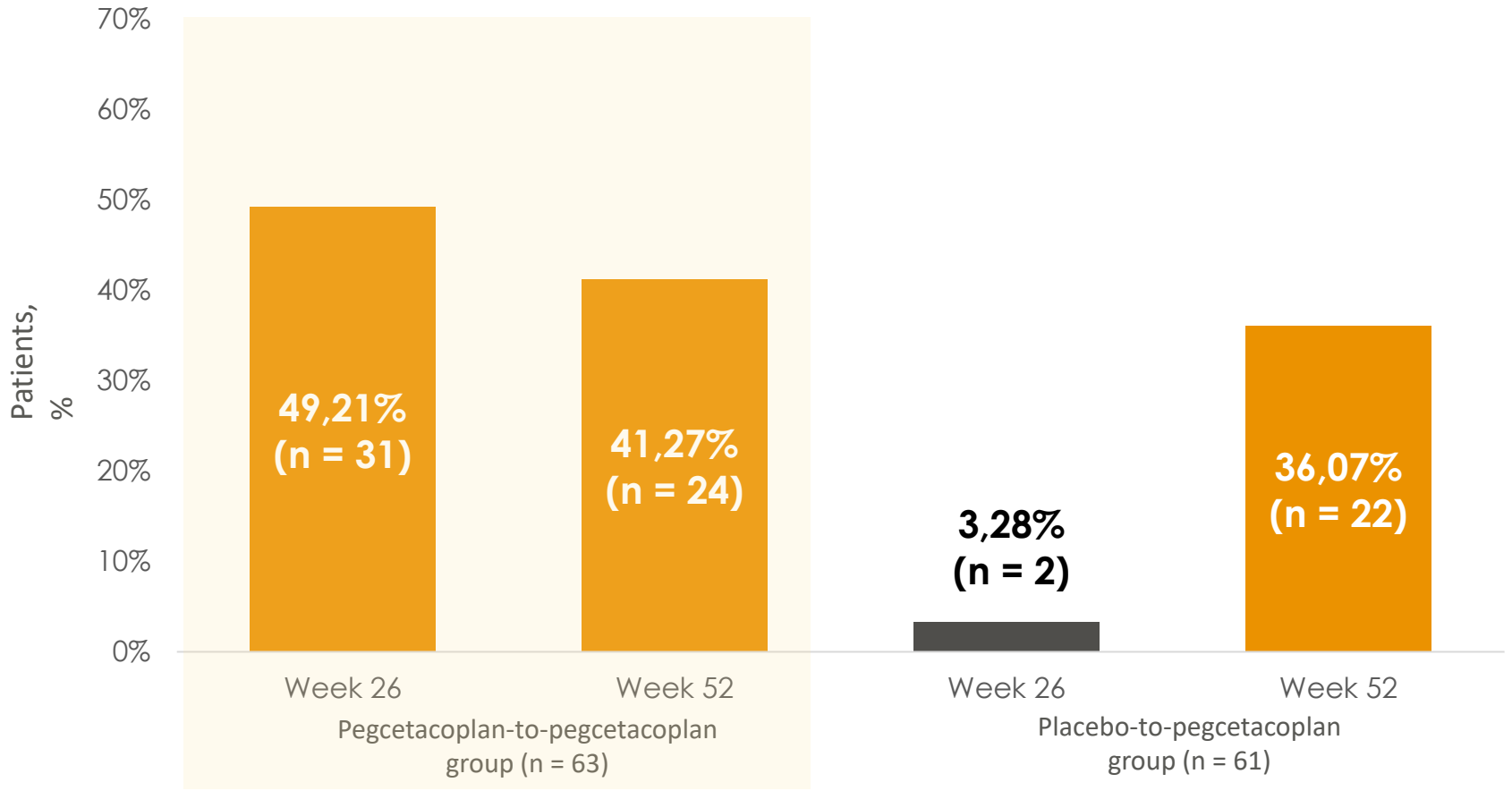
Similar proportions of patients in each group achieved **≥50% proteinuria reduction at week 52**



1. Fakhouri F, et al. Presented at ERA 2025.

Similar proportions of patients in each group achieved the **composite renal endpoint at week 52**

Proportion of patients who met the composite endpoint*

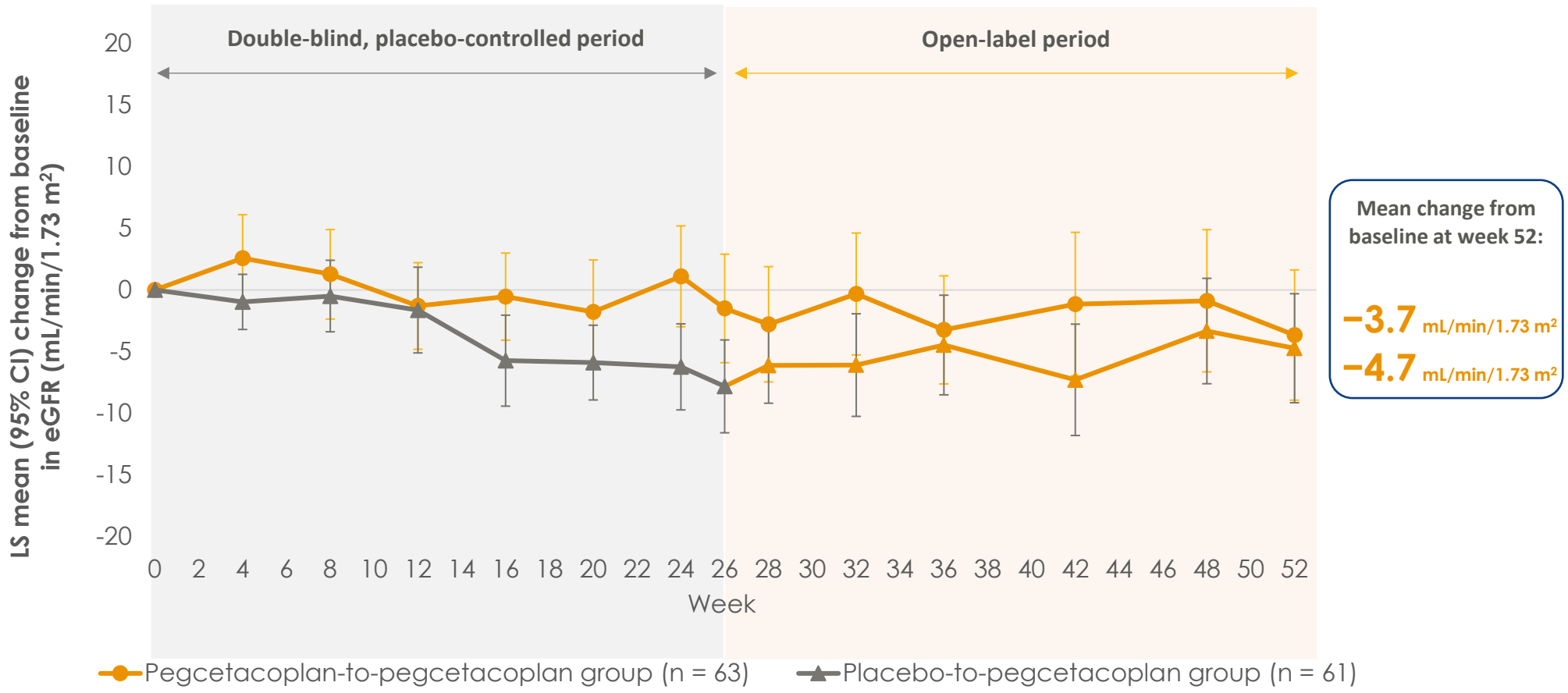


* ≥50% reduction in UPCR and ≤15% reduction in eGFR.

1. Fakhouri F, et al. Presented at ERA 2025.

eGFR remained stable for both groups for the duration of the study

Change in eGFR according to treatment group



1. Fakhouri F, et al. Presented at ERA 2025.

TEAEs **consistent** with previous safety profile

Event, n (%) of patients	Double-blind, placebo-controlled period	Open-label period	
	Pegcetacoplan group (n=63)	Pegcetacoplan-to-pegcetacoplan group (n=61)	Placebo-to-pegcetacoplan group (n=57)
Any TEAE	54 (85.7)	47 (77.0)	42 (73.7)
Maximum severity			
Mild	26 (41.3)	25 (41.0)	20 (35.1)
Moderate	25 (39.7)	19 (31.1)	17 (29.8)
Severe	3 (4.8)	3 (4.9)	5 (8.8)
Treatment-related TEAE	27 (42.9)	10 (16.4)	19 (33.3)
Infusion-related TEAE	21 (33.3)	6 (9.8)	12 (21.1)
Serious TEAE	6 (9.5)	6 (9.8)	4 (7.0)
TEAE leading to treatment withdrawal	2 (3.2)	2 (3.3)	2 (3.4)
TEAE leading to dose interruption	8 (12.7)	7 (11.5)	6 (10.5)
TEAE leading to study discontinuation	1 (1.6)	2 (3.3)	2 (1.8)
TEAE leading to death	1 (1.6)	0	0
Rejection episodes	0	1 (1.6)	0
Graft loss	0	0	0

1. Fakhouri F, et al. Presented at ERA 2025.

Adherence was high and the **safety profile was consistent** with previous reports

During the OLP, most patients had **adherence ≥90%**
- Pegcetacoplan-to-pegcetacoplan group: 59 (96.7%)
- Placebo-to-pegcetacoplan group: 55 (96.5%)

No infections caused by **encapsulated bacteria** were reported **during the RCP**

Four infections caused by **encapsulated bacteria** were reported **during the OLP**: two cases of pneumococcal pneumonia, one case of streptococcal pharyngitis, and one urinary tract infection caused by *Escherichia*

One of the cases of pneumococcal pneumonia met seriousness criteria

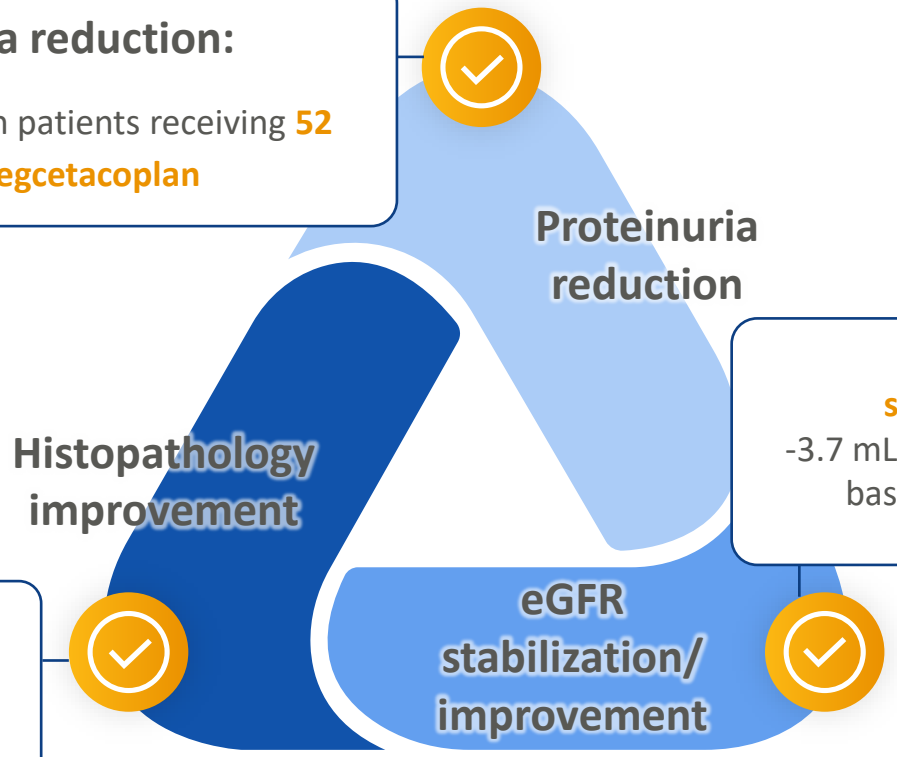
1. Fakhouri F, et al. Presented at ERA 2025.

With **1 year** of treatment, **pegcetacoplan** led to **robust and sustained proteinuria reductions** and **stable eGFR** for patients with C3G and primary IC-MPGN

VALIANT study in patients aged ≥12 years with native or post-transplant recurrent C3G/ primary IC-MPGN

Proteinuria reduction:

67% **reduction** in patients receiving **52 weeks of pegcetacoplan**



eGFR:

stabilization of eGFR

-3.7 mL/min/1.73 m^2 change from baseline with **52 weeks of pegcetacoplan**

Histopathology improvement:

glomerular C3 clearance in 71% of patients (zero staining) at week 26

Pegcetacoplan was well tolerated with no new safety signals

1. Fakhouri F, et al. Presented at ERA 2025.

Summary and concluding remarks

Guido Oelkers

CEO Sobi

On track for launch in early 2026 in C3G and primary IC-MPGN in Europe

- **EMA** filing validated in February
  - Expect CHMP opinion by year-end
- **PMDA** (Japan) filing planned by year-end
  - Incorporating 52-week data into package
- **enFuse® Injector** for subcutaneous delivery on track
  - Aim to be available in Europe for PNH, C3G and primary IC-MPGN
- **Launch preparation and country launch readiness** ongoing with commercial and medical build up

Empaveli Injector® commercialised by Apellis Pharmaceuticals, Inc. in the US

enFuse is a trademark of Enable Injections, Inc.

Exciting potential for Aspaveli/Empaveli in Nephrology

➢ We are confident in pegcetacoplan's blockbuster potential

- Diagnosed patient population today of approximately **8k patients with C3G or primary IC-MPGN in Europe** with an addressable population of approximately 4-5K
- Additional potential opportunity in selected international markets, **total current diagnosed population in Sobi territories approximately 16K** with an addressable population of approximately 6K
- Unlocking the full potential requires understanding the individual complete patient journey and ensuring every patient with kidney disease receives a diagnosis

➢ Market research indicates high enthusiasm on pegcetacoplan's potential

" Data suggests that pegcetacoplan is effective across many subpopulations, including some of the more fragile patients like transplant recipients."
Spanish HCP

"Histological evidence is very important to me, and the ability for most patients to have no staining is impressive*. This potentially completes the biological story: inhibiting C3 addresses the C3 deposits and the disease."
Japanese HCP

"At 26 weeks, it is very favorable that we are already seeing this level of placebo-adjusted eGFR stabilization."
German HCP

Sobi Market Research, completed Q1 2024 &2025
*Refers to Valiant trial histological data showing 71% of patients with glomerular C3 staining clearance from baseline at week 26.

Aspaveli/Empaveli a key near term building block of the future

Investment in 2025 for multiple launches in 2025/26

2

Major launches

1. Altuvoct
2. Vonjo

3

Key filing

1. Gamifant - HLH/MAS
2. **Aspaveli - C3G/IC-MPGN**
3. NASP - uncontrolled gout

4

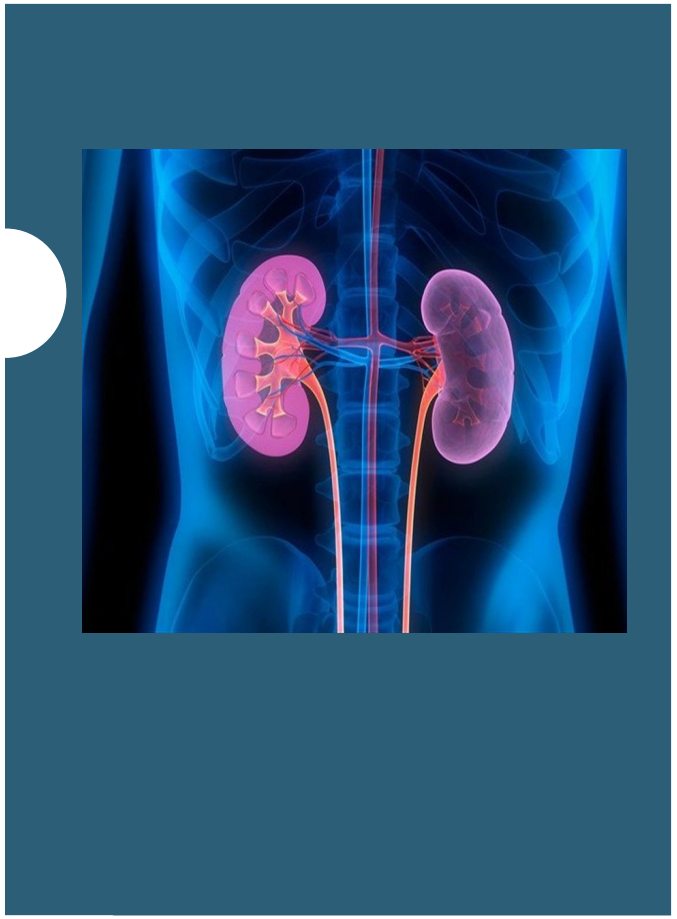
Priority development projects in area of high unmet medical need

1. Gamifant - IDS
2. Vonjo - VEXAS
3. Vonjo - CMML
4. Altuvoct - synovitis

NASP: nanoencapsulated sirolimus plus pegadricase, CMML: Chronic myelomonocytic leukaemia, IDS: Interferon-gamma driven sepsis

Aspaveli/Empaveli a potential new treatment in C3G and primary IC-MPGN

- Devastating diseases with limited target treatment options
- Large market potential – 8K diagnosed patients in Europe
- Pegcetacoplan results at week 52 show potential best in class efficacy in C3G and primary IC-MPGN
- Launch in Europe is on track for early 2026




Q&A