OS-056

Efficacy and Safety of CAM2029 in Patients With Polycystic Liver Disease: Phase 2b POSITANO Study

Presenter: Joost P. H. Drenth

Marie C. Hogan,[1] Frederik Nevens,[2] Arun B. Jesudian,[3] Rajender Reddy,[4] Richard Taubert,[5] Jonel Trebicka,[6] Peter Almgren,[7] Ulrika Axling,[7] Peter Jönsson,[7] Fredrik Tiberg,[7] Joost P. H. Drenth[8]

[1]Mayo Clinic, Rochester, MN, USA; [2]University Hospitals KU Leuven, Leuven, Belgium; [3]Weill Cornell Medicine, New York, NY, USA; [4]University of Pennsylvania, Philadelphia, PA, USA; [5]Medizinische Hochschule Hannover, Hannover, Germany; [6]Universitätsklinikum Münster, Münster, Germany; [7]Camurus AB, Lund, Sweden; [8]Amsterdam University Medical Center, Amsterdam, Netherlands

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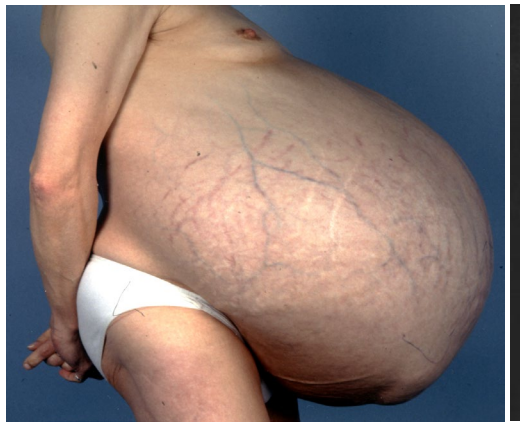
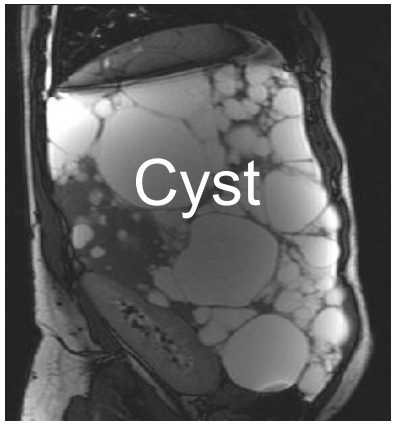
Polycystic Liver Disease (PLD) Is a Rare Disease That Can Significantly Impact Patient Quality of Life

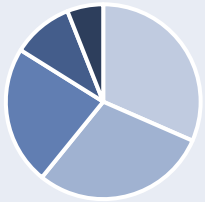
- PLD is a rare genetic disorder defined by the formation of multiple fluid-filled cysts in the liver[1,2]
- In adults, PLD can occur independently in the liver (ADPLD) or as an accompanying condition to ADPKD[1,2]
- Estimated prevalence of clinically significant ADPLD is <1/10,000 (US, data for 1980–2016)[2]



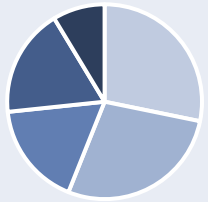


PLD can result in significant hepatomegaly, with patients experiencing symptoms including:[3*]

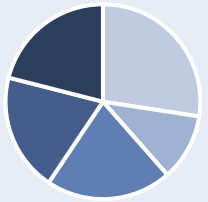
Shortness of breath



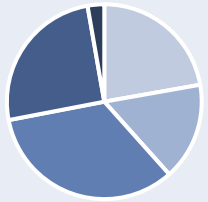
Limited mobility



Dissatisfaction with abdomen size



Fullness



Tiredness

Level of symptoms experienced: None | Mild | Moderate | Moderately severe | Severe

…which can negatively impact patient quality of life

Patient images provided by Prof Joost PH Drenth. *Figure adapted from the PLD-Q, other symptoms recorded include lack of appetite, nausea, problems with intercourse, fear/anxiety for the future, abdominal pain, early satiety, pain or pressure in rib cage and pain in side.
ADPKD, autosomal dominant polycystic kidney disease; ADPLD, autosomal dominant polycystic liver disease; PLD, polycystic liver disease.
1. Cnossen WR, Drenth JPN. *Orphanet J Rare Dis* 2014;9:69; 2. Suwabe T *et al. JHEP Rep* 2020;2:100166; 3. Neijenhuis MK *et al. United European Gastroenterol J* 2018;6:81–88.

SSAs Have Indicated Efficacy in Reducing Liver Volume in PLD

- Currently, there are no approved medical therapies for PLD, representing an unmet need for treatments that can slow cyst growth and reduce liver volume
- Investigator-initiated trials have indicated efficacy of SSAs (octreotide LAR, lanreotide and pasireotide LAR) in reducing liver volume in PLD, and recent guidelines recommend SSAs for symptomatic PLD[1–10]

CAM2029 is a novel, extended-release octreotide depot, based on FluidCrystal® technology[11–14]

- **Ready-to-use autoinjector pen** with a small-gauge needle prefilled with CAM2029 10 mg (0.5 mL)
- **Self-administration** by patients (or by their partners) or administered by a healthcare professional
- **Subcutaneous injection** in the abdomen, thigh or buttock
- Stored at **room temperature** and ready to use
- CAM2029 safety profile is **consistent with that of approved octreotide products**

Pre-filled autoinjector pen

LAR, long-acting repeatable (octreotide)/release (pasireotide); PLD, polycystic liver disease; SSA, somatostatin analogue.

1. European Association for the Study of the Liver. *J Hepatol* 2022;77:1083–108; 2. van Keimpema L *et al. Gastroenterology* 2009;137:1661–8.e2; 3. Hogan MC *et al. J Am Soc Nephrol* 2010;21:1052–61; 4. Caroli A *et al. Clin J Am Soc Nephrol* 2010;5:783–9; 5. Pisani A *et al. Clin Gastroenterol Hepatol* 2016;14:1022–30 e4; 6. van Aerts RMM *et al. Gastroenterology* 2019;157:481–91 e7; 7. Hogan MC *et al. Clin J Am Soc Nephrol* 2020;15:1267–78; 8. Gevers TJ *et al. Liver Int* 2015;35:1607–14; 9. Temmerman F *et al. Aliment Pharmacol Ther* 2013;38:397–406; 10. Torres VE *et al. Kidney Int* 2025;107:234–54; 11. Tiberg F *et al. Br J Clin Pharmacol* 2015;80:460–72; 12. Ferone D *et al. J Clin Endocrinol Metab* 2025;110:1729–39; 13. Camurus AB. Oczyesa® (CAM2029). EU summary of product characteristics, 2025; 14. Spencer-Segal JL *et al.* ESPE/ESE 2025; Poster RC4.4a.

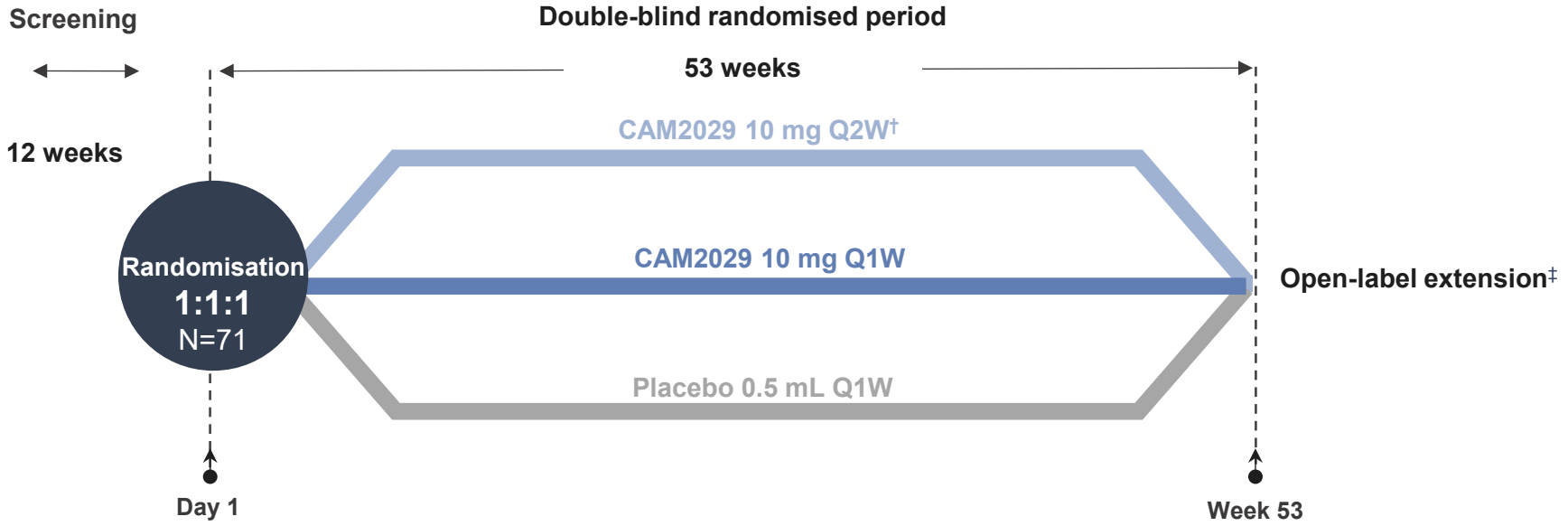
POSITANO: A 53-week, Phase 2b, Double-Blind, Randomised, Placebo-Controlled Trial

Key eligibility criteria:

- Symptomatic PLD (isolated or associated with ADPKD)*
- htTLV ≥1800 mL/m at screening
- Unsuitable for or unwilling to undergo surgical intervention for hepatic cysts

Exclusion criteria:

- SSA treatment or surgical intervention within the 3 months prior to screening



Primary endpoint

- Difference in **htTLV**§ change from baseline to week 53 between CAM2029 (combined doses) and placebo

Key secondary endpoint

- Change in **PLD-S score** (novel PRO tool assessing patient-reported symptoms of PLD) from baseline to week 53 with CAM2029 versus placebo

Other selected secondary endpoints

- Change in total **liver cyst volume**§ from baseline to week 53 with CAM2029 versus placebo
- Change in htTKV§ from baseline to week 53 with CAM2029 versus placebo
- **Safety**

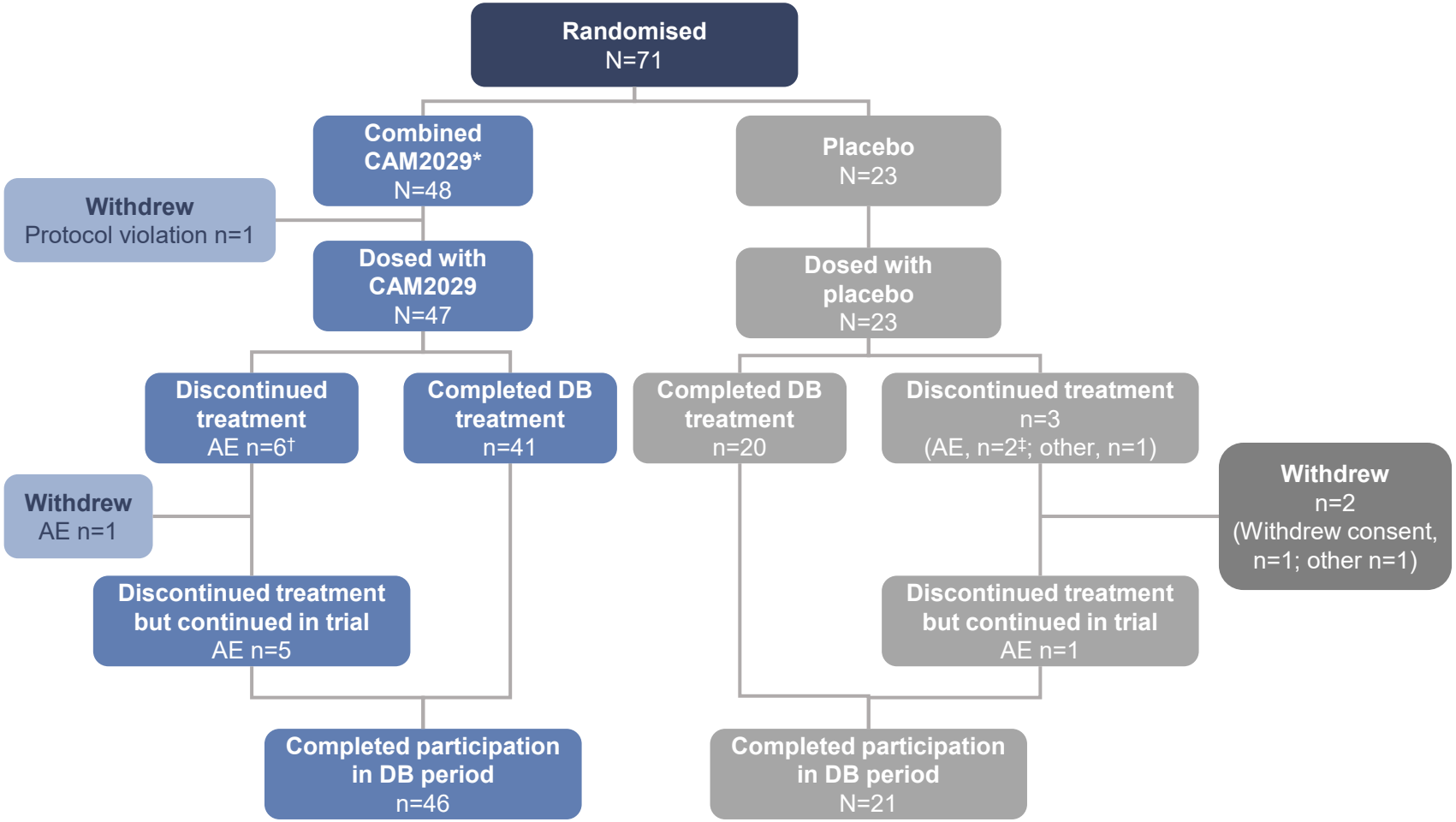
POSITANO (NCT05281328).
Study treatment was received every 7 days ± 2 days and was administered via SC injection in the abdomen, thigh or buttock. *At least one PLD-related symptom within the 2 weeks prior to screening;
†Alternating weekly with placebo 0.5 mL; ‡Upon completing the double-blind randomised period, patients could continue to receive CAM2029 10 mg Q1W or Q2W in a 120-week extension phase;
§Assessed by MRI volumetry with blinded independent central review.
ADPKD, autosomal dominant polycystic kidney disease; htTKV, height-adjusted total kidney volume; htTLV; height-adjusted total liver volume; MRI, magnetic resonance imaging; PLD, polycystic liver disease; PLD-S, Polycystic Liver Disease Symptoms; PRO, patient-reported outcome; Q1W, once weekly; Q2W, once every 2 weeks; SC, subcutaneous; SSA, somatostatin analogue.

Patient Disposition

Patients were enrolled from **11** clinical trial sites

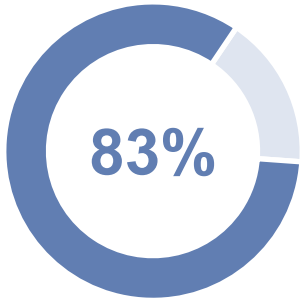
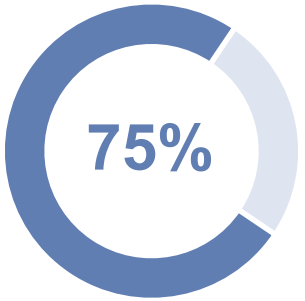
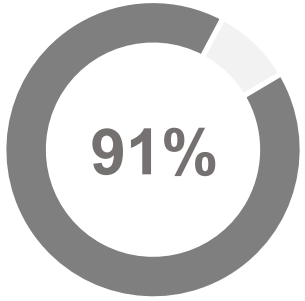
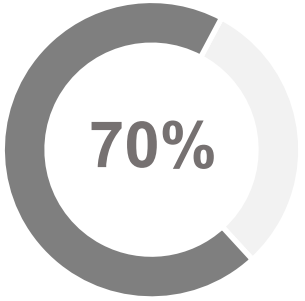
...located across **4** countries

Sites per country:
US n=6, Germany n=3,
Belgium n=1, Netherlands n=1



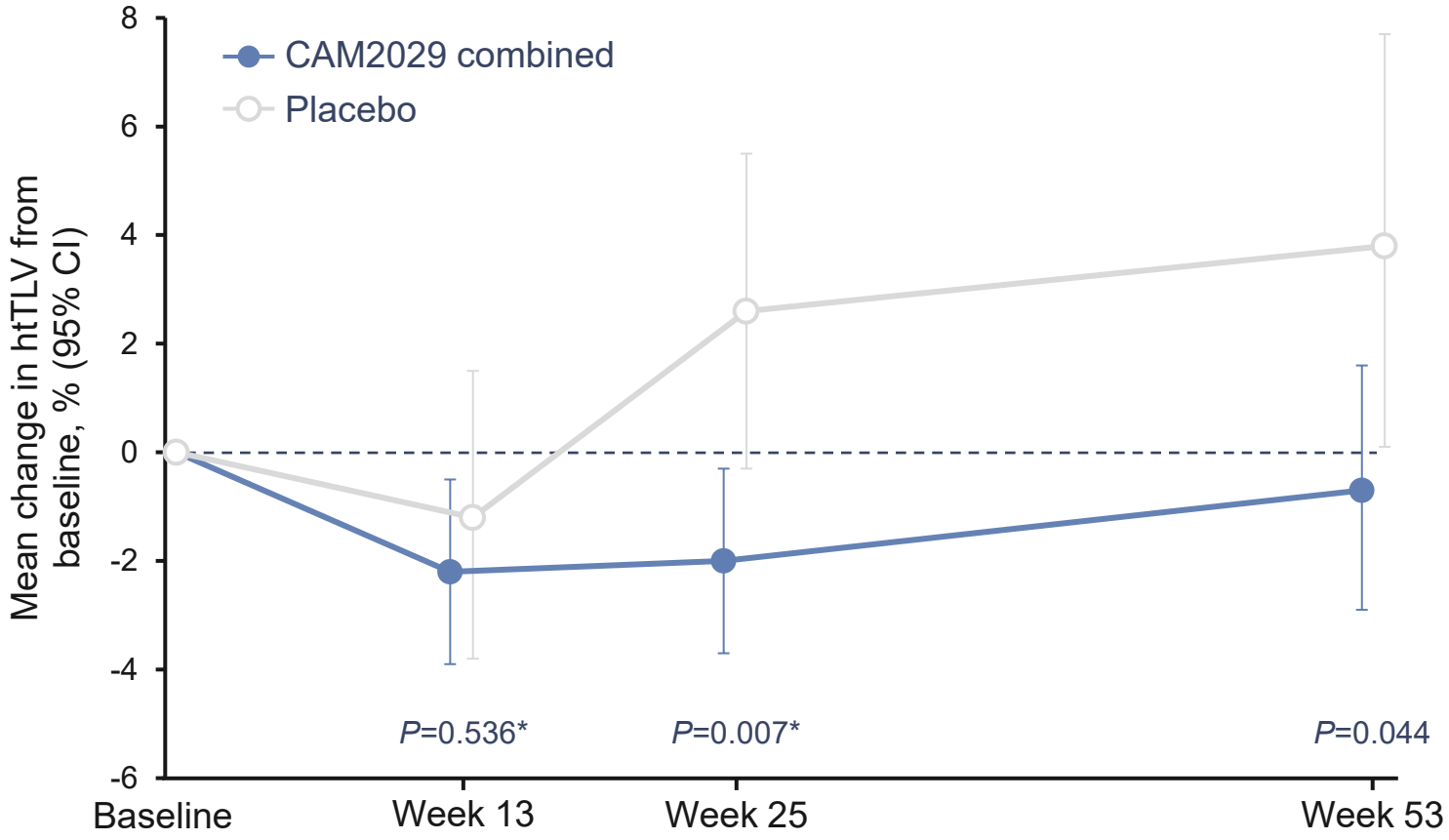
Double-blind, randomised period. *The combined CAM2029 group comprises the CAM2029 Q1W and Q2W groups; †AEs leading to CAM2029 discontinuation: injection site AEs (n=3), PLD (n=1), gastroenteritis (n=1), headache (n=1); ‡AEs leading to placebo discontinuation: injection site AEs (n=1), PLD (n=1).
AE, adverse event; DB, double-blind, SD, standard deviation.

Baseline Demographics and Characteristics Were Similar Between the Groups

	Mean age (SD)	Female	Associated with ADPKD	Mean htTLV (SD)	Mean PLD-S* score (SD)
Combined CAM2029 (N=48)	55 years (8)			3555 mL/m (1842)†	39 (20)‡
Placebo (N=23)	53 years (10)			3307 mL/m (1416)†	40 (20)

ITT analysis set. *The PLD-S is a PRO currently under development to assess patient-reported PLD symptoms; it includes 10 symptoms and assesses the frequency of occurrence of these symptoms in the past week; †Data not available for two patients; ‡Data not available for one patient.
ADPKD autosomal dominant polycystic kidney disease; htTLV, height-adjusted total liver volume; ITT, intention to treat; PLD-D, Polycystic Liver Disease Symptoms; SD, standard deviation.

Primary Endpoint Was Met: CAM2029 Significantly Reduced htTLV Growth Compared With Placebo



CAM2029 combined	48	46	46	46
Placebo	23	21	21	21

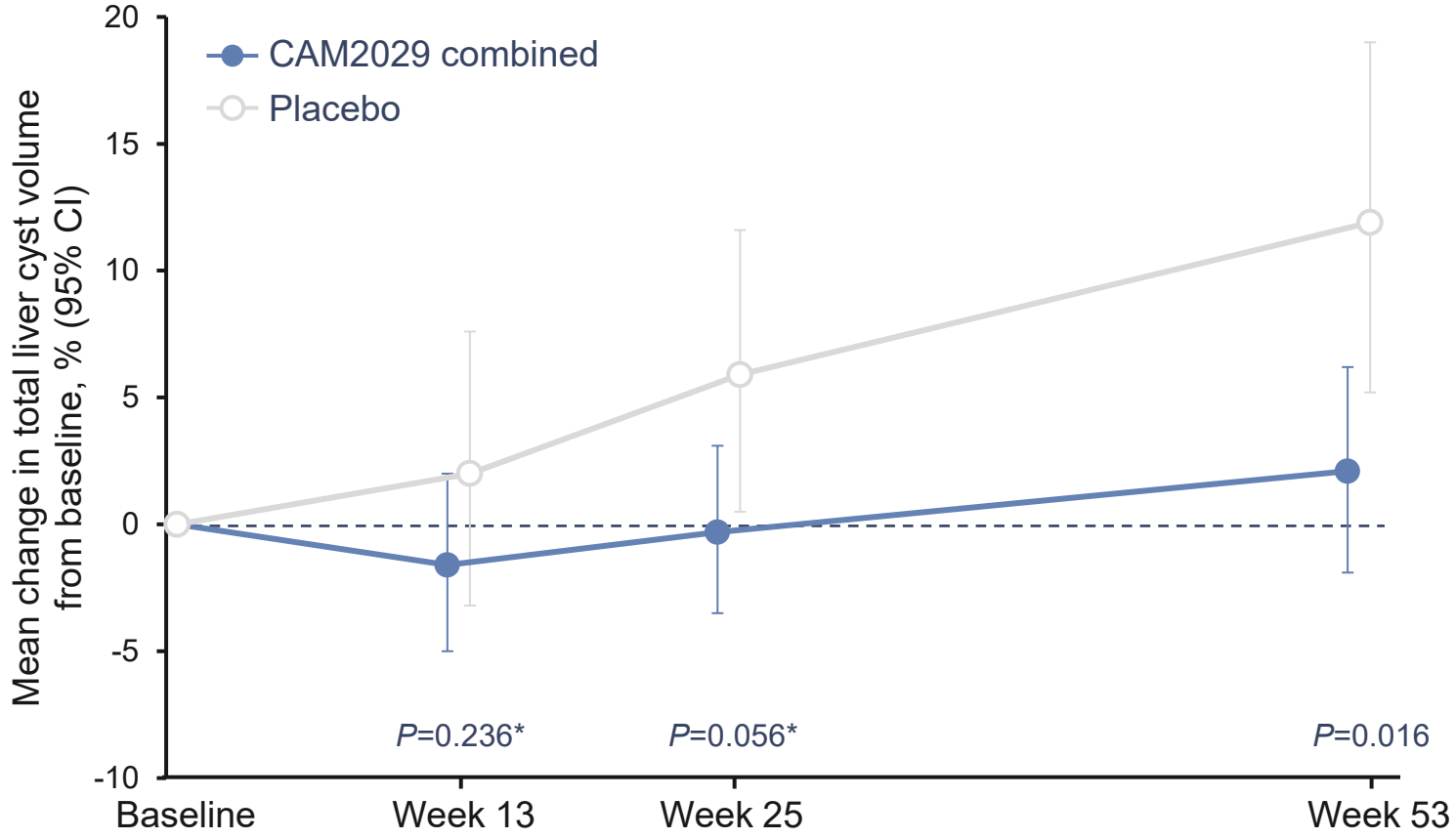
Estimated relative treatment difference between CAM2029 versus placebo from baseline to week 53:

−4.3% (95% CI −8.4, −0.1)

P=0.044

ITT analysis set. htTLV was assessed by MRI volumetry with blinded independent central review. *Nominal p-values, not adjusted for multiple testing.
CI, confidence interval; htTLV, height-adjusted total liver volume; ITT, intention to treat.

Change in Total Liver Cyst Volume Over Time



CAM2029 combined	48	45	46	42
Placebo	23	20	17	17

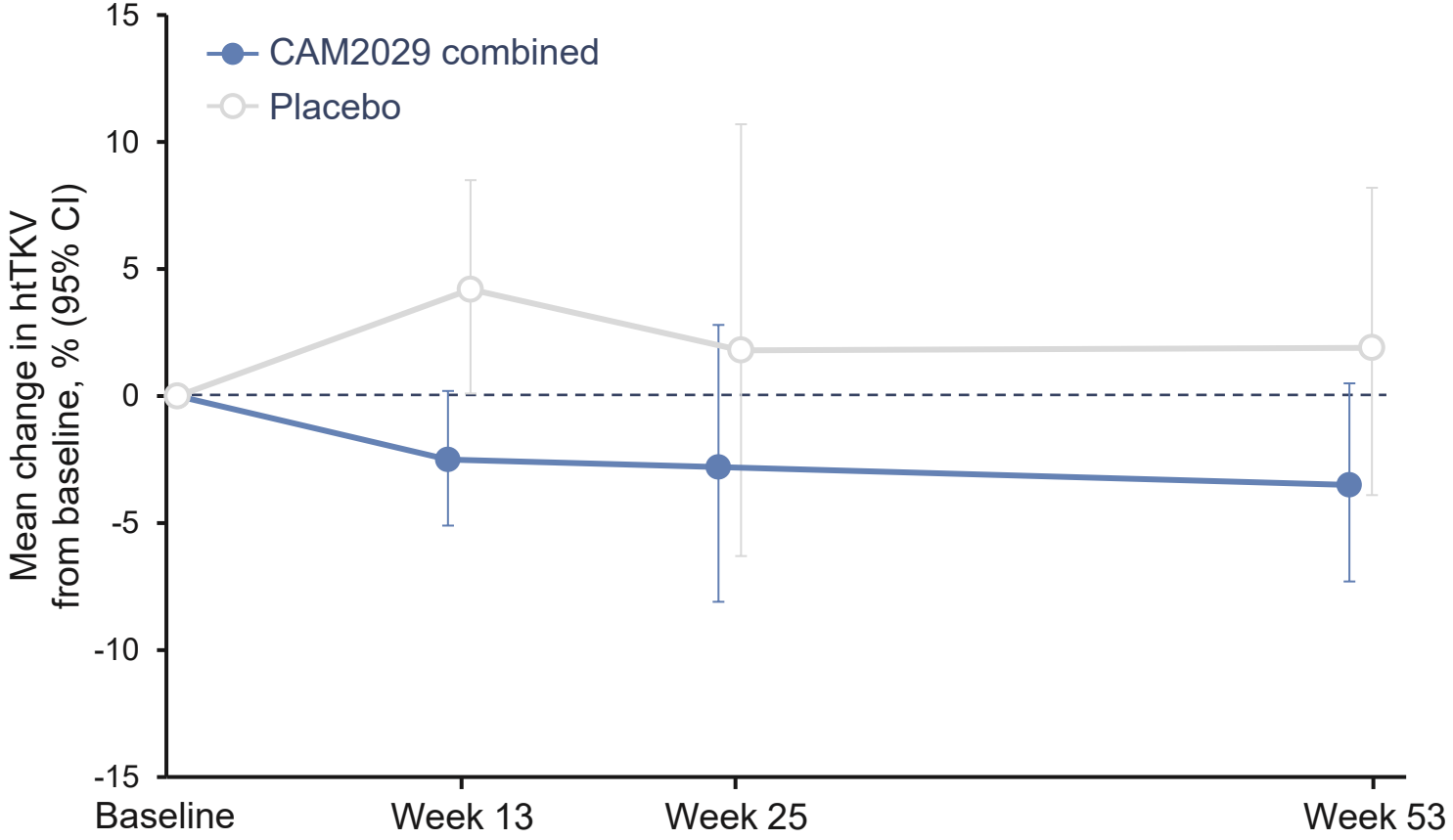
Estimated relative treatment difference between CAM2029 versus placebo from baseline to week 53:

−8.7% (95% CI −15.2, −1.8)

P=0.016

ITT analysis set. Total liver cyst volume was assessed by MRI volumetry with blinded independent central review. *Nominal p-values, not adjusted for multiple testing.
CI, confidence interval; ITT, intention to treat; MRI, magnetic resonance imaging.

Change in htTKV Over Time in Patients With ADPKD



CAM2029 combined	36	31	33	32
Placebo	16	14	15	15

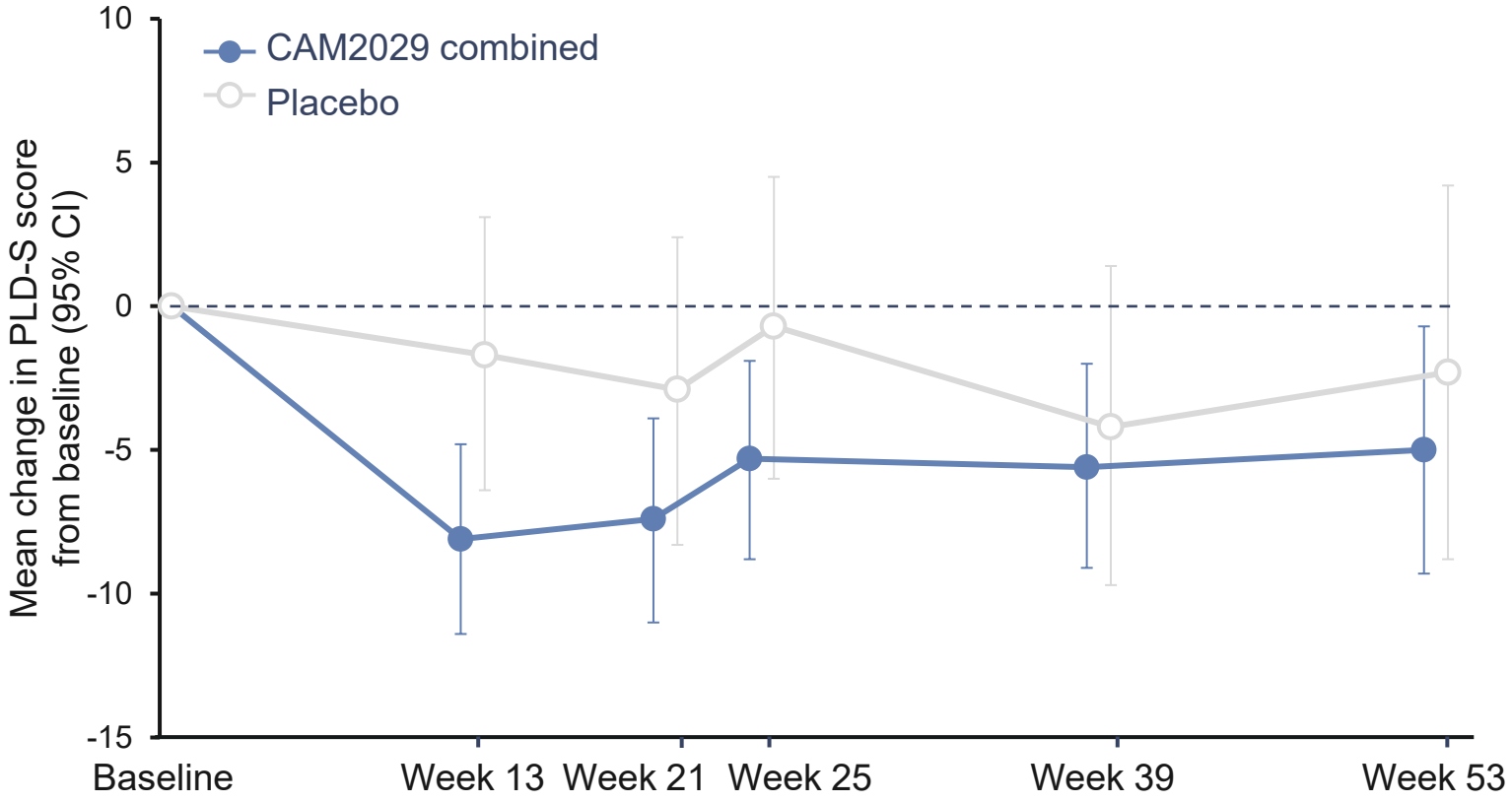
Estimated relative treatment difference between CAM2029 versus placebo from baseline to week 53:

−5.3% (95% CI −11.9,1.7)

P=0.132

htTKV was assessed by MRI volumetry with blinded independent central review.
ADPKD, autosomal dominant polycystic kidney disease; CI, confidence interval; htTKV, height-adjusted total kidney volume.

Change in PLD-S From Baseline Over Time



CAM2029 combined	48	47	47	47	47	47
Placebo	23	23	23	23	23	23

Estimated treatment difference between CAM2029 versus placebo from baseline to week 53:

−2.7 (95% CI: −10.5, 5.2)

P=0.504

ITT analysis set. The PLD-S is a PRO currently under development to assess patient-reported PLD symptoms; it includes 10 symptoms and assesses the frequency of occurrence of these symptoms in the past week. PLD-S score range 0 to 100, with higher score indicating worse symptoms.
CI, confidence interval; ITT, intention to treat; PLD-S, Polycystic Liver Disease Symptoms.

CAM2029 Was Well Tolerated, With No Unexpected Safety Findings

- Most patients experienced treatment-related TEAEs
  - **CAM2029 combined 93.6%**
  - **Placebo 95.7%**
- The most frequently reported TEAEs with CAM2029 were gastrointestinal and injection site related
- Similar proportion of patients discontinued treatment due to TEAEs
  - **CAM2029 combined 12.8%**
  - **Placebo 8.7%**
- Safety profiles were similar for the two CAM2029 doses

TEAEs, n (%)*	CAM2029 combined (N=47)	Placebo (N=23)
Diarrhoea	32 (68.1)	7 (30.4)
Injection site pruritus	20 (42.6)	8 (34.8)
Injection site pain	19 (40.4)	14 (60.9)
Abdominal pain	17 (36.2)	6 (26.1)
Injection site mass	13 (27.7)	7 (30.4)
Injection site erythema	13 (27.7)	1 (4.3)
Nausea	12 (25.5)	6 (26.1)
Injection site bruising	12 (25.5)	3 (13.0)
Flatulence	11 (23.4)	3 (13.0)
Headache	11 (23.4)	3 (13.0)
Steatorrhoea	11 (23.4)	2 (8.7)
Fatigue	10 (21.3)	4 (17.4)
Dizziness	10 (21.3)	0
Abdominal distension	9 (19.1)	5 (21.7)
Constipation	6 (12.8)	7 (30.4)
Back pain	6 (12.8)	5 (21.7)
Hypertension	6 (12.8)	5 (21.7)

Safety analysis set. *TEAEs occurring in ≥20% of patients in either treatment group by preferred term.
SSA, somatostatin analogue; TEAE, treatment-emergent adverse event.

Conclusions

- The **primary endpoint was met**: there was a significant difference in htTLV change from baseline to week 53 between CAM2029 and placebo
- Data suggest a decrease in total liver cyst volume from baseline to week 53 between CAM2029 versus placebo
- CAM2029 was **well tolerated**, with **no unexpected safety findings**

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These results support the further evaluation of CAM2029 as a potential new treatment for PLD

Evaluation of long-term CAM2029 for patients with symptomatic PLD is ongoing in the POSITANO OLE phase

htTLV, height-adjusted total liver volume; OLE, open-label extension; PLD, polycystic liver disease.

Acknowledgements

Thank you to the patients, investigators, nurses and trial coordinators who made this trial possible

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