

Lumasiran: ILLUMINATE-A Study Overview

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SUMMARY

- ILLUMINATE-A was a phase 3, randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of lumasiran in adults and children ≥ 6 years old with PH1 and an $eGFR \geq 30$ mL/min/1.73m². After the 6-month double-blind treatment period, all patients received lumasiran in an optional 54-month OLE.¹
- The study met the primary endpoint of percent change from baseline in 24-hr UOx excretion corrected for BSA at 6 months. The LS mean percent change from baseline in 24-hr UOx in the lumasiran group was -65.4% compared with -11.8% in the placebo group, resulting in a between-group LS mean difference of -53.5% (95% CI: -62.3 to -44.8; $P < 0.001$).¹
 - At the end of the study at Month 60, the mean (SEM) percent change from baseline in 24-hr UOx was -54% (6%) in the lumasiran/lumasiran group and -54% (8%) in the placebo/lumasiran group.²
- The study met all secondary endpoints that were tested hierarchically, including a significant reduction in POx levels in patients treated with lumasiran compared to placebo at 6 months.¹
- At Month 60, 37 of the 39 patients (95%) experienced an AE. The most commonly reported AE was ISR, which occurred in 14 patients (36%); all were transient and mild in severity. The serious AEs (n=6, 15%), severe AEs (n=4, 10%) and treatment discontinuations (n=1, 3%) reported during the study were considered not related to lumasiran by the investigator.²

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STUDY DESIGN

ILLUMINATE-A was a phase 3, randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of lumasiran in adults and children ≥ 6 years old with PH1. Patients were randomized (2:1) to receive subcutaneous injections of lumasiran 3 mg/kg (N=26) or placebo (N=13) once monthly for 3 loading doses, followed by maintenance doses once every 3 months beginning

1 month after the last loading dose. The primary endpoint was the percent change from baseline in 24-hr UOx excretion corrected for BSA at 6 months (average of visits from Month 3 through 6). After the 6-month double-blind treatment period, all patients received lumasiran in an optional 54-month OLE.¹

Patients randomized to receive lumasiran in the initial 6-month randomized, placebo-controlled period are referred to as the lumasiran/lumasiran group and patients randomized to receive placebo in the initial 6-month randomized, placebo-controlled period are referred to as the placebo/lumasiran group. Of the 39 patients enrolled, 24 of the 26 in the lumasiran/lumasiran group and 13 of the 13 in the placebo/lumasiran group completed treatment in the 54-month OLE.²

Select inclusion and exclusion criteria for ILLUMINATE-A are presented below in **Table 1**.^{1,3}

Table 1. ILLUMINATE-A Inclusion and Exclusion Criteria.^{1,3}

Inclusion Criteria	Exclusion Criteria
<ul style="list-style-type: none"> • Age ≥6 years old • Diagnosis of PH1 confirmed by genetic analysis • eGFR ≥30 mL/min/1.73 m² • Mean UOx ≥0.7 mmol/24hr/1.73m² • For patients taking pyridoxine (vitamin B6) for treatment of PH1, regimen required to have been stable for at least 90 days before randomization and willing to remain on regimen for 12 months from first study drug administration 	<ul style="list-style-type: none"> • Clinical evidence of extrarenal systemic oxalosis • Clinically significant liver function test abnormalities • Known HIV, HCV, or HBV infection • Received an investigational agent within the last 30 days or 5 half-lives • History of kidney or liver transplant

Abbreviations: eGFR = estimated glomerular filtration rate; HBV = hepatitis B virus; HCV = hepatitis C virus; HIV = human immunodeficiency virus; hr = hour; PH1 = primary hyperoxaluria type 1; UOx = urinary oxalate.

PATIENT DEMOGRAPHICS & BASELINE CHARACTERISTICS

Baseline characteristics are shown below in **Table 2**.^{1,4,5}

Table 2. Baseline Demographics and Disease Characteristics.^{1,4,5,a}

Baseline Characteristic ^b	Placebo (n=13)	Lumasiran (n=26)	Overall (N=39)
Median age (range), years	11.0 (6-60)	16.5 (6-47)	14.0 (6-60)
Mean age at informed consent (range), y	17.0 (6-60)	18.7 (6-47)	18.1 (6-60)
Age category, n (%)			
6 to <18 years	8 (62)	14 (54)	22 (56)
18 to <65 years	5 (38)	12 (46)	17 (44)
Female sex, n (%)	5 (38)	8 (31)	13 (33)
Race, n (%)			
Asian	3 (23)	3 (12)	6 (15)
White	9 (69)	21 (81)	30 (77)
Other ^c	1 (8)	2 (8)	3 (8)
Geographic region, n (%)			
Europe	8 (62)	10 (38)	18 (46)
Middle East	3 (23)	5 (19)	8 (21)
North America	2 (15)	11 (42)	13 (33)
Pyridoxine (vitamin B6) use, n (%)	9 (69)	13 (50)	22 (56)
24-hr UOx excretion, mean (SD), mmol/24hr/1.73m ^{2,d}	1.79 (0.68)	1.84 (0.60)	1.82 (0.62)

Baseline Characteristic ^b	Placebo (n=13)	Lumasiran (n=26)	Overall (N=39)
POx level, mean (SD), $\mu\text{mol/L}^e$	19.3 (9.5)	14.8 (7.6)	16.3 (8.4)
Kidney function measures			
eGFR, mean (SD), mL/min/1.73 m ² ^f	78.8 (30.0)	83.0 (25.5)	81.6 (26.8)
eGFR category, n (%)			
≥ 90 mL/min/1.73m ²	4 (31)	9 (35)	13 (33)
60 to <90 mL/min/1.73m ²	6 (46)	13 (50)	19 (49)
30 to <60 mL/min/1.73m ²	3 (23)	4 (15)	7 (18)
Patients reporting history of KSEs, n (%) ^g			
Lifetime	10 (77)	23 (88)	33 (85)
12 months prior to consent	4 (31)	11 (42)	15 (38)
Genotype, n (%) ^h			
PR/*	6 (46)	11 (42)	17 (44)
M/M or M/N	4 (31)	6 (23)	10 (26)
N/N	3 (23)	9 (35)	12 (31)

Abbreviations: BSA = body surface area; eGFR = estimated glomerular filtration rate; hr = hour; KSE = kidney stone event; M = missense; MDRD = Modification of Diet in Renal Disease; N = nonsense; PH1 = primary hyperoxaluria type 1; POx = plasma oxalate; PR = pyridoxine-responsive; SD = standard deviation; ULN = upper limit of normal; UOx = urinary oxalate.

^aPatients remained on background therapies, including hyperhydration, crystallization inhibitors, and/or pyridoxine therapy through Month 12 before adjustments were made to the regimen based on clinical discretion.

^bValues presented as mean (SD) unless otherwise indicated.

^cIncluded 1 patient in the placebo group who reported more than one race and 2 patients in the lumasiran group who reported "other."

^dFor the lumasiran/lumasiran group, baseline is the median of all valid 24-hr urine assessments obtained before the first dose of lumasiran without any non-protocol-related sample issues. For the placebo/lumasiran group, baseline is the median of all valid 24-hr urine assessments at Month 6 without any non-protocol-related sample issues (or, if the patient did not have 2 valid 24-hr urine pharmacodynamic assessments at Month 6, then the baseline was calculated using the latest 3 valid 24-hr urine pharmacodynamic collections obtained before the first dose of lumasiran). The ULN range for 24-hr urinary oxalate is 0.514 mmol/24hr/1.73m² of BSA. To convert values to mg/24 hr/1.73m², multiply by 90.

^eThe ULN range is 12.11 $\mu\text{mol/L}$. The POx analysis set included 23 patients in the lumasiran group and 10 patients in the placebo group.

^feGFR was calculated with the MDRD formula for patients ≥ 18 years of age at screening and the Schwartz Bedside Formula for patients 6 to <18 years of age at screening.

^gA KSE is defined as an event that includes at least one of the following: visit to healthcare provider because of a kidney stone, medication for renal colic, stone passage, or macroscopic hematuria due to a kidney stone.

^hPR was defined as NM_000030.3(AGXT):c.508G>A (p.Gly170Arg) or NM_000030.3(AGXT):c.454T>A (p.Phe152Ile). The asterisk (*) denotes any genotype of PR, M, or N.

EFFICACY RESULTS

A summary of the efficacy results at Month 6 of the double-blind treatment period is presented below in **Table 3**.¹

Table 3. Change from Baseline in the Primary Endpoint and Hierarchically Tested Secondary Endpoints at Month 6.¹

Endpoint	Lumasiran (n=26)	Placebo (n=13)	Difference, Lumasiran- Placebo	P-value
Primary endpoint				
Percent change in 24-hr UOx excretion (95% CI) ^{a,b}	-65.4 (-71.3 to -59.5)	-11.8 (-19.5 to -4.1)	-53.5 (-62.3 to -44.8)	<0.001
Secondary endpoints				

Endpoint	Lumasiran (n=26)	Placebo (n=13)	Difference, Lumasiran-Placebo	P-value
Absolute change in 24-hr UOx corrected for BSA (95% CI), mmol/24hr/1.73 m ^{2a,b}	-1.24 (-1.37 to -1.12)	-0.27 (-0.44 to -0.10)	-0.98 (-1.18 to -0.77)	<0.001
Percent change in 24-hr UOx:Cr ratio (95% CI) ^b	-62.5 (-70.7 to -54.4)	-10.8 (-21.6 to 0.0)	-51.8 (-64.3 to -39.3)	<0.001
Percent change in POx (95% CI) ^{b,c}	-39.8 (-45.8 to -33.8)	-0.3 (-9.1 to 8.5)	-39.5 (-50.1 to -28.9)	<0.001
Percentage of patients with 24-hr UOx excretion ≤1.5×ULN at Month 6 (95% CI) ^{a,d}	84 (64 to 95)	0 (0 to 25)	84 (55 to 94)	<0.001
Percentage of patients with 24-hr UOx excretion ≤ULN at Month 6 (95% CI) ^{a,d}	52 (31 to 72)	0 (0 to 25)	52 (23 to 70)	0.001
Absolute change in POx (95% CI), μmol/L ^{b,d}	-7.5 (-9.0 to -5.9)	1.3 (-1.0 to 3.5)	-8.7 (-11.5 to -6.0)	<0.001

Abbreviations: BSA = body surface area; CI = confidence interval; hr = hour; POx = plasma oxalate; ULN = upper limit of normal; UOx = urinary oxalate; UOx:Cr = urinary oxalate:creatinine.

^aMeasurements of urinary oxalate excretion were corrected for BSA.

^bThe change from baseline to Month 6 was calculated as the mean change or mean percent change across Month 3 through 6. The least squares mean, between group difference in the least squares mean, 95% confidence intervals, and P-value for comparisons of lumasiran and placebo were derived with a mixed model for repeated measures. A difference of less than 0 represents a favorable outcome for lumasiran.

^cThe plasma oxalate analysis set included 23 patients in the lumasiran group and 10 patients in the placebo group.

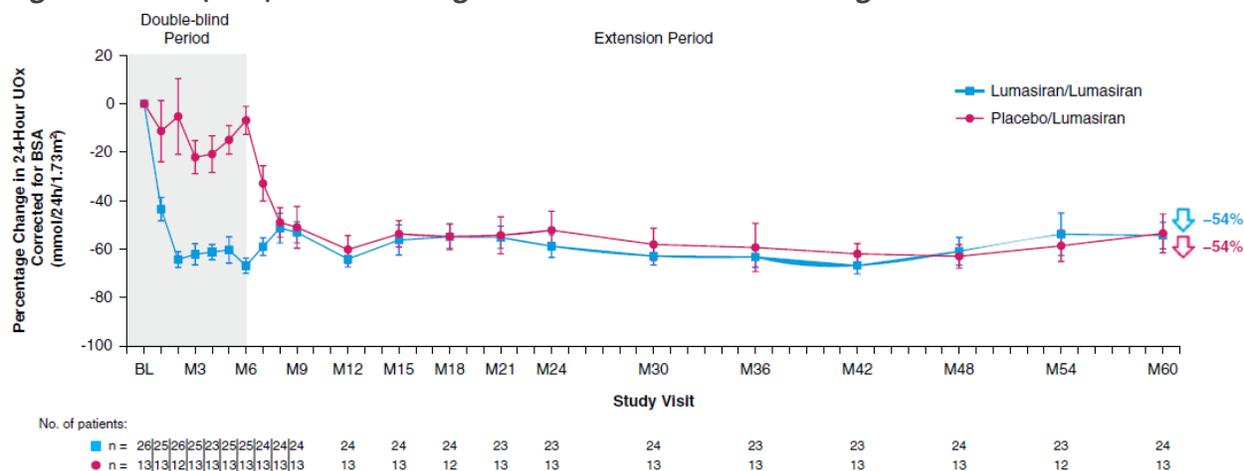
^dData were available for 25 patients in the lumasiran group and 13 patients in the placebo group. The ULN range is 0.514 mmol/24hr/1.73m². The confidence interval is a Clopper-Pearson exact confidence interval.

Urinary Oxalate

In the 6-month double-blind period, the LS mean percent change from baseline in 24-hr UOx in the lumasiran group was -65.4% (95% CI: -71.3 to -59.5) compared with -11.8% (95% CI: -19.5 to -4.1) in the placebo group, resulting in a between-group LS mean difference of -53.5% (95% CI: -62.3 to -44.8; P<0.001).¹

At the end of the study at Month 60, the mean (SEM) percent change from baseline in 24-hr UOx levels was -54% (6%) in the lumasiran/lumasiran group and -54% (8%) in the placebo/lumasiran group. (**Figure 1**). The mean (SEM) 24-hour UOx (mmol/24 h/1.73 m²) values decreased from 1.84 (0.12) at baseline to 0.75 (0.06) at Month 60 in the lumasiran/lumasiran group, and 1.79 (0.19) at baseline to 0.71 (0.05) at Month 60 in the placebo/lumasiran group.²

Figure 1. Mean (SEM) Percent Change 24-Hour in UOx Levels Through Month 60.^{2,a}



Abbreviations: BL = baseline; M = month; SEM = standard error of the mean; UOx = urinary oxalate.

^aBaseline is the median of all valid 24-hour urine assessments collected before the first dose date/time of study drug (lumasiran or placebo) without any non-protocol-related sample issues.

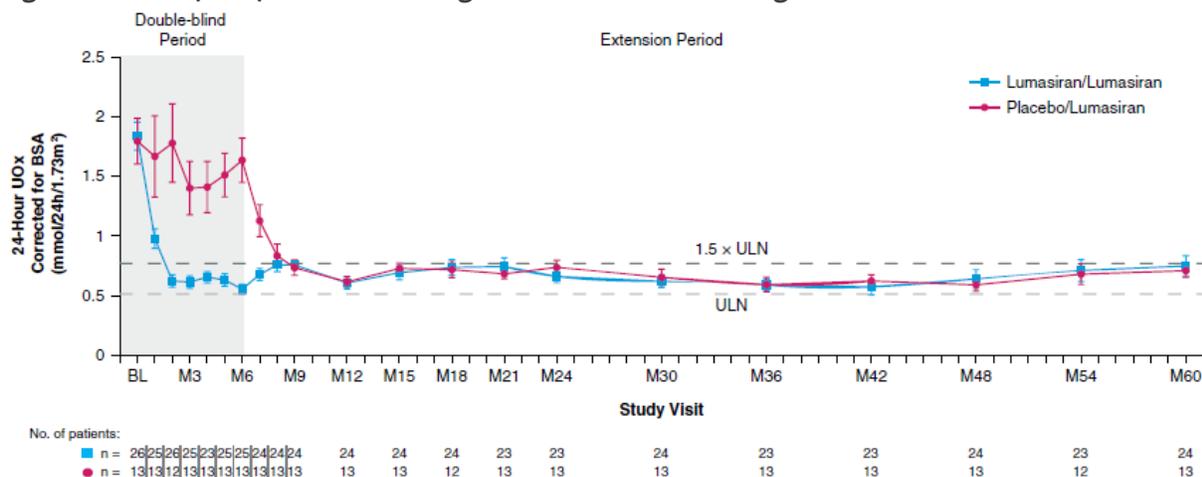
From Frishberg et al.²

Proportion of Patients with 24-Hour UOx Excretion $\leq 1.5 \times \text{ULN}$

In the 6-month double-blind period, 84% of lumasiran-treated patients achieved near normalization or normalization ($\leq 1.5 \times \text{ULN}$) of 24-hr UOx excretion when corrected for BSA, compared to 0% of placebo-treated patients ($P < 0.001$).¹

The mean 24-hour UOx values generally remained between the ULN and $1.5 \times \text{ULN}$ at all time points after approximately 2 to 3 months of lumasiran treatment. The mean (SEM) absolute values of 24-hour UOx corrected for BSA through Month 60 are shown in **Figure 2.**²

Figure 2. Mean (SEM) Absolute Change in 24-Hour UOx Through Month 60.^{2,a}



Abbreviations: BL = baseline; BSA = body surface area; hr = hour; SEM = standard error of the mean; ULN = upper limit of normal; UOx = urinary oxalate.

^aTop dashed line represents 1.5 times ULN of $0.771 \text{ mmol}/24 \text{ hr}/1.73 \text{ m}^2$ ($1 \text{ mmol}/24 \text{ hr}/1.73 \text{ m}^2 = 90 \text{ mg}/24 \text{ hr}/1.73 \text{ m}^2$) for 24-hr UOx. Bottom dashed line represents the ULN of $0.514 \text{ mmol}/24 \text{ hr}/1.73 \text{ m}^2$. Baseline is the median of all valid 24-hour urine assessments collected before the first dose date/time of study drug (lumasiran or placebo) without any non-protocol-related sample issues.

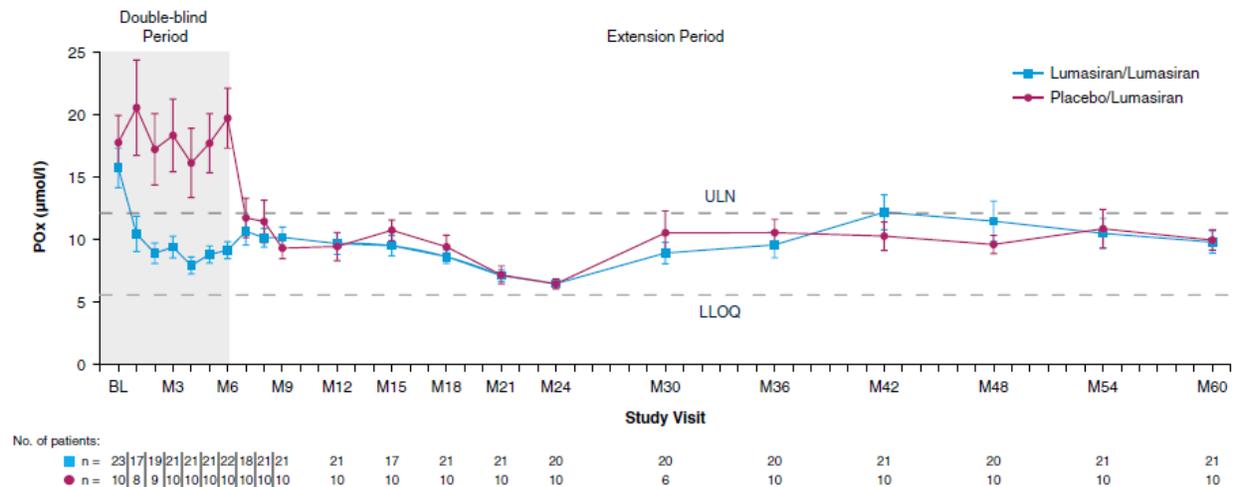
From Frishberg et al.²

Plasma Oxalate

In the 6-month double-blind period, the decline in POx levels was significantly greater in the lumasiran treated group when compared with the placebo group. Among the 33 patients with a baseline POx level of at least 1.5×LLOQ, the LS mean difference in the percent change in POx levels from baseline to Month 6 was -39.5% (95% CI, -50.1 to -28.9; P<0.001).¹

At the end of the study at Month 60, the mean percent change from baseline in POx levels was -35% in the lumasiran/lumasiran group and -38% in the placebo/lumasiran group (**Figure 3**).²

Figure 3. Mean (SEM) POx Levels Through Month 60.^{2,a}



Abbreviations: BL = baseline; LLOQ = lower limit of quantitation; M = month; POx = plasma oxalate; SEM = standard error of mean; ULN = upper limit of normal.

^aBaseline is defined as the mean of all POx measurements before the first dose date/time of study drug (lumasiran or placebo) in the 6-month double-blind period. Top dashed line represents the ULN of 12.11 µmol/L for POx. Bottom dashed line represents the LLOQ of the POx assay at 5.55 µmol/L; values below the LLOQ were assigned a value of 5.55 µmol/L.

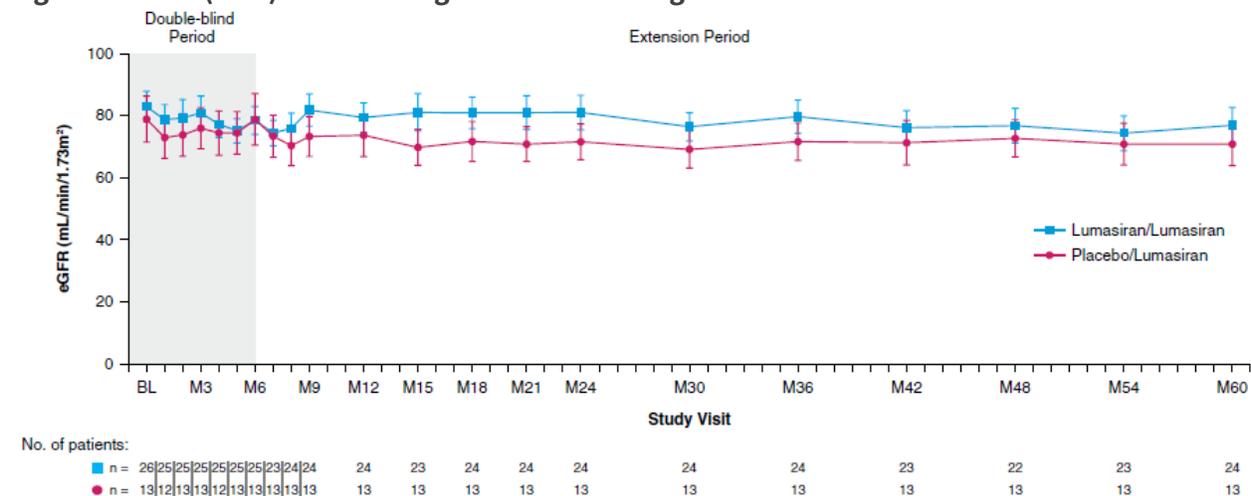
From Frishberg et al.²

Kidney Related Outcomes

eGFR

At Month 60, the mean (SEM) absolute change from baseline in eGFR was -3.40 (2.49) mL/min/1.73m² in the lumasiran/lumasiran group and -8.11 (2.25) mL/min/1.73m² in the placebo/lumasiran group. In a post hoc analysis of all patients in the study during lumasiran treatment, the regression-estimated annual rate of eGFR change over 60 months (slope [SEM]) was -0.6 (0.7) mL/min/1.73m² per year.²

Figure 4. Mean (SEM) eGFR Through Month 60 During Lumasiran Treatment.^{2,a}



Abbreviations: BL = baseline; eGFR = estimated glomerular filtration rate; M = month; MDRD = Modification of Diet in Renal Disease; SEM = standard error of the mean.

^aBaseline is the last assessment before the first dose of study drug (lumasiran or placebo) in the 6-month double-blind period. eGFR was calculated with the MDRD formula for patients ≥ 18 years of age at screening and the Schwartz Bedside Formula for patients 6 to < 18 years of age at screening.

From Frishberg et al.²

Kidney Stone Events

A KSE was defined as ≥ 1 of the following: visit to healthcare provider because of a kidney stone, medication for kidney colic, stone passage, or macroscopic hematuria due to a kidney stone. KSE rates were calculated as the total number of KSEs divided by the total patient exposure time (events per PY).²

During the patient-reported 12-month historical recall period, KSE rates (95% CI) were 3.19 (2.57, 3.96) per PY in the lumasiran/lumasiran group and 0.54 (0.26, 1.13) per PY in the placebo/lumasiran group.⁴

During the study, KSE rates (95% CI) were 0.47 (0.36, 0.62) per PY with 60 months of lumasiran treatment in the lumasiran/lumasiran group (**Figure 5a**) and 0.54 (0.37, 0.78) per PY with 54 months of lumasiran treatment in the placebo/lumasiran group (**Figure 5b**).²

During the final 6 months of lumasiran treatment, KSE rates were 0.09 (0.01, 0.63) per PY in the lumasiran/lumasiran group and 0.68 (0.25, 1.81) per PY in the placebo/lumasiran group. A total of 21 patients (54%) had no KSEs, 7 patients (18%) have 1 KSE, and 11 patients (28%) had more than 1 KSE.²

Figure 5a. Kidney Stone Events in the Lumasiran/Lumasiran Group.²

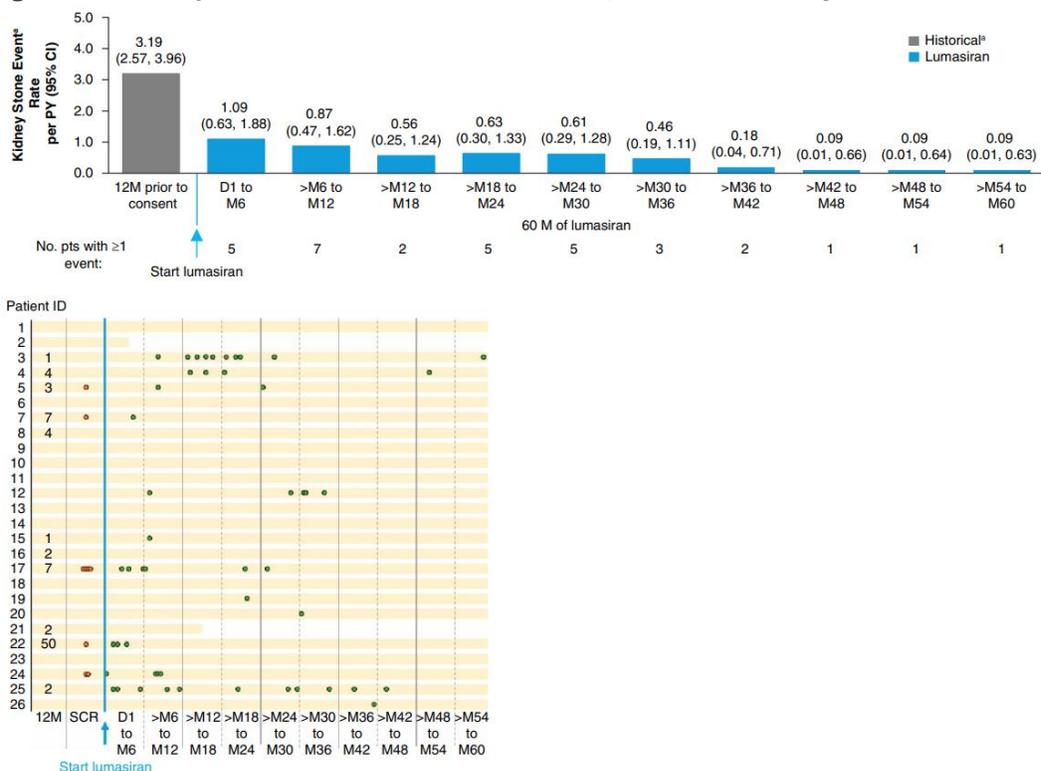
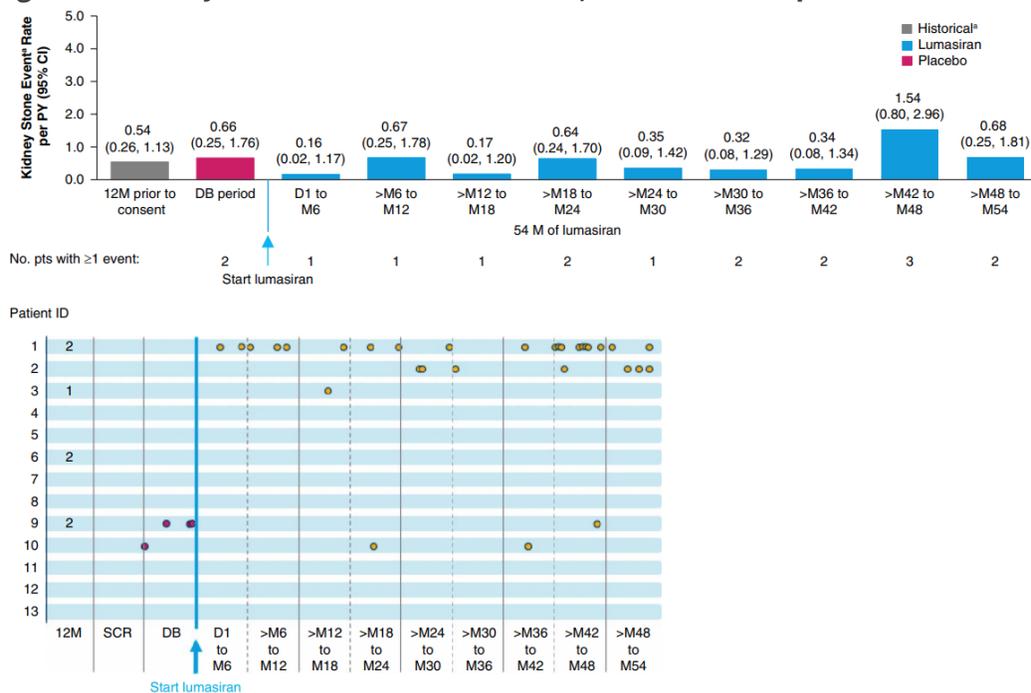


Figure 5b. Kidney Stone Events in the Placebo/Lumasiran Group.²



Abbreviations: CI = confidence interval; D = day; DB = double-blind; KSE = kidney stone event; M = month; PY = patient year.

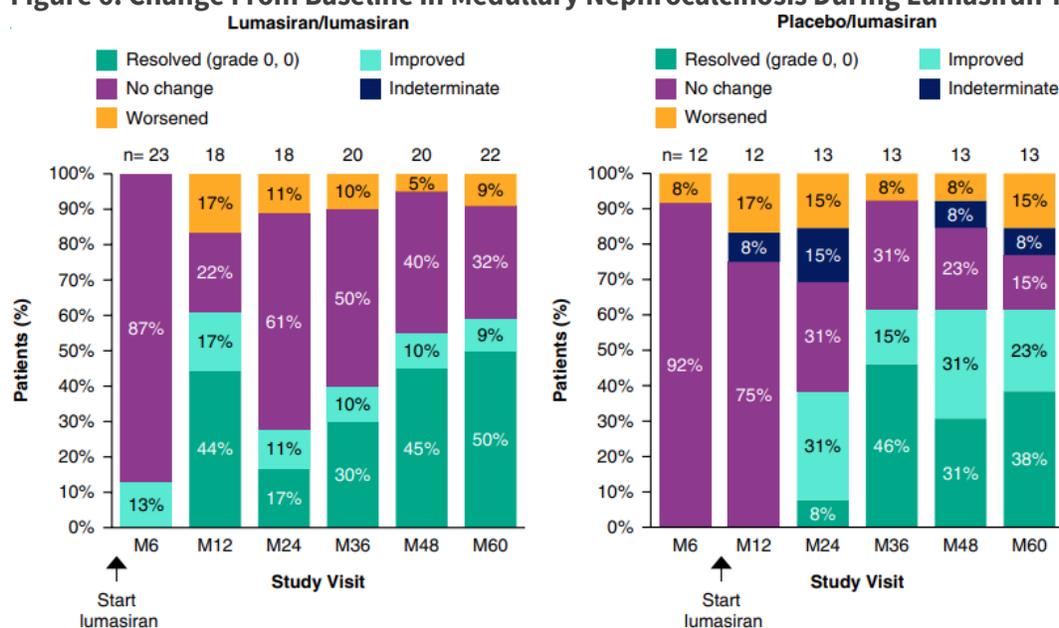
^aThe timing for historical events (before 12 months) was not documented. Historical refers to patient-reported history of KSEs. From Frishberg et al.²

Nephrocalcinosis

Medullary nephrocalcinosis was assessed by ultrasound and graded per kidney on a scale of 0 to 3, with higher grades indicating greater severity. Kidney ultrasounds were reviewed centrally by radiologists who were not investigators or site personnel and who were blinded to treatment and visit.² The degree of medullary nephrocalcinosis in each kidney was graded using a validated 4-point scale: stable (i.e., no change in either kidney), improving (i.e., both kidneys improving, or 1 kidney improving and 1 with no change), worsening (i.e., both kidneys worsening, or 1 kidney worsening and 1 with no change), or indeterminate (i.e., 1 kidney improving and 1 worsening).⁶

A total of 37 patients had kidney ultrasounds at baseline. Eight patients (22%) had bilateral grade 0, 7 patients (19%) had bilateral grade 1, 4 patients (11%) had bilateral grade 2, and 8 patients (22%) had bilateral grade 3 medullary nephrocalcinosis. Medullary nephrocalcinosis grade (left/right or right/left) was 1/0 or 0/1 in 5 (14%) patients, 1/2 or 2/1 in 2 (5%) patients, 2/0 in 2 (5%) patients, and 2/3 in 1 (3%) patient. The change from baseline in medullary nephrocalcinosis at Month 60 relative to study baseline among all patients with available data is shown in **Figure 6**.²

Figure 6. Change From Baseline in Medullary Nephrocalcinosis During Lumasiran Treatment.^{2,a}



Abbreviations: M = month.

^aImproved indicates a lower grade than baseline (defined as the last assessment before the first dose of study drug [lumasiran or placebo] in the 6-month double-blind period. No change indicates the same grade as baseline. Indeterminate indicates one side improved and the other side worsened. Worsened indicates a higher grade than baseline.

From Frishberg et al.²

SAFETY RESULTS

The safety profile of treatment with lumasiran through Month 60 is summarized in **Table 4**. At Month 60, 37 of the 39 patients (95%) experienced an AE. ISRs occurred in 14 patients (36%); all were transient, mild in severity, and resolved without sequelae.²

A majority of the AEs were mild or moderate in severity. Four patients (10%) experienced severe AEs, and 1 patient (3%) experienced an AE leading to discontinuation of study drug. The serious AEs, severe

AEs, and treatment discontinuations reported during the study were considered not related to lumasiran by the investigator.²

Table 4. Safety Profile of Lumasiran Through Month 60.²

Event, n (%)	Placebo/ Lumasiran (n=13)	Lumasiran/ Lumasiran (n=26)	Overall (N=39)
Any AE	12 (92)	25 (96)	37 (95)
AE related to study drug	6 (46)	13 (50)	19 (49)
Serious AE ^a	1 (8)	5 (19)	6 (15)
Severe AE ^b	0	4 (15)	4 (10)
AE leading to discontinuation of study treatment ^c	0	1 (4)	1 (3)
AEs occurring in ≥15% of patients			
ISR ^d	5 (38)	9 (35)	14 (36)
Abdominal pain	1 (8)	8 (31)	9 (23)
COVID-19 infection	4 (31)	4 (15)	8 (21)
Headache	2 (15)	5 (19)	7 (18)
Nasopharyngitis	2 (15)	4 (15)	6 (15)
Death	0	0	0

Abbreviations: AE = adverse event; ISR = injection site reaction.

^aSix patients (15%) experienced a serious AE (abdominal pain [n=2], dysuria [n=1], follicular lymphoma [n=1], postprocedural complication [n=1], postprocedural infection [n=1], kidney impairment [n=1], urinary tract infection [N=1], and urosepsis [n=1]). All were considered not related to lumasiran by the investigator.

^bFour patients (10%) experienced a severe AE (acute pyelonephritis [n=1], follicular lymphoma [n=1], postprocedural complication [n=1], postprocedural infection [n=1], urinary tract infection [n=1], and urosepsis [n=1]). All were considered not related to lumasiran by the investigator.

^cOne patient (3%) discontinued lumasiran treatment due to fatigue and disturbance in attention, considered not related to lumasiran by the investigator, which began during the double-blind period.

^dISRs were transient, considered mild in severity, and resolved without sequelae.

ABBREVIATIONS

AE = adverse event; BL = baseline; BSA = body surface area; CI = confidence interval; D = day; DB = double-blind; eGFR = estimated glomerular filtration rate; HBV = hepatitis B virus; HCV = hepatitis C virus; HIV = human immunodeficiency virus; hr = hour; ISR = injection site reaction; KSE = kidney stone event; LLOQ = lower limit of quantitation; LS = least squares; M = missense; M = month; MDRD = Modification of Diet in Renal Disease; N = nonsense; OLE = open-label extension; PH1 = primary hyperoxaluria type 1; PH1 = primary hyperoxaluria type 1; POx = plasma oxalate; PR = pyridoxine-responsive; PY = patient year; SD = standard deviation; SEM = standard error of the mean; ULN = upper limit of normal; UOx = urinary oxalate; UOx:Cr = urinary oxalate:creatinine.

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