

Lumasiran: Kidney Related Outcomes

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SUMMARY

- Assessments of kidney function and other kidney related outcomes measures were evaluated across the lumasiran clinical studies:
 - In the Phase 1/2 and Phase 2 OLE studies, mean eGFR values remained stable through Month 54 of the OLE period among lumasiran treated patients.¹ The rates of kidney stone-related AEs decreased during the Phase 2 OLE compared with Part B of the Phase 1/2 study.²
 - In the ILLUMINATE-A study, mean eGFR values remained stable through Month 54 of the OLE period in the placebo/lumasiran group and the lumasiran/lumasiran group. KSE rates remained low through all months of lumasiran treatment.³
 - In the ILLUMINATE-B study, mean eGFR values remained stable through Month 30 in lumasiran treated patients. Patient-reported historical KSE rates were ≤ 0.25 /person-year through Month 30 of lumasiran treatment.⁴
 - The ILLUMINATE-C study included patients with eGFR ≤ 45 mL/min/1.73m² (Cohort A) and patients on hemodialysis (Cohort B). Mean (SD) eGFR values were 19.8 ± 9.6 mL/min/1.73 m² at baseline and 16.4 ± 9.8 mL/min/1.73 m² at Month 6 for Cohort A.⁵ In the 6-month primary analysis, KSE rates decreased in both Cohort A and Cohort B.⁶
- Across the lumasiran clinical studies, the most common treatment-related AEs were mild ISRs. There were no treatment-emergent deaths or treatment-related severe AEs, serious AEs, or discontinuations.^{1,3,7,8}

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PHASE 1/2 AND PHASE 2 OLE STUDIES

The Phase 1/2 study was a single-blind, placebo-controlled, single and multiple ascending dose study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of subcutaneously administered lumasiran in healthy adult subjects (Part A) and patients with PH1 (Part B). All 20 patients enrolled in the Phase 1/2 Part B study completed the study and enrolled in the Phase 2 OLE study. The data below includes only patients included in Part B of the study. The primary endpoint was incidence of AEs, including kidney stone-related AEs.^{2,9}

Change in eGFR levels over time was evaluated as a secondary endpoint to assess kidney function during the study. GFR was calculated based on the MDRD formula for patients ≥ 18 years of age at screening and the Schwartz Bedside Formula for patients < 18 years of age at screening.¹

Patient Demographics & Baseline Characteristics

Relevant baseline characteristics are shown below in **Table 1**.¹

Table 1. Phase 2 OLE Relevant Baseline Characteristics.^{1,a}

Characteristic	All Treated (N=20)
Median age at screening (range), years	11.5 (6-43)
White race, n (%)	15 (75)
Median eGFR (range), mL/min/1.73 m ²	72.2 (42.5-130.7)

Abbreviations: eGFR = estimated glomerular filtration rate; OLE = open-label extension.

^aBaseline data was derived from the Phase 1/2 parent study.

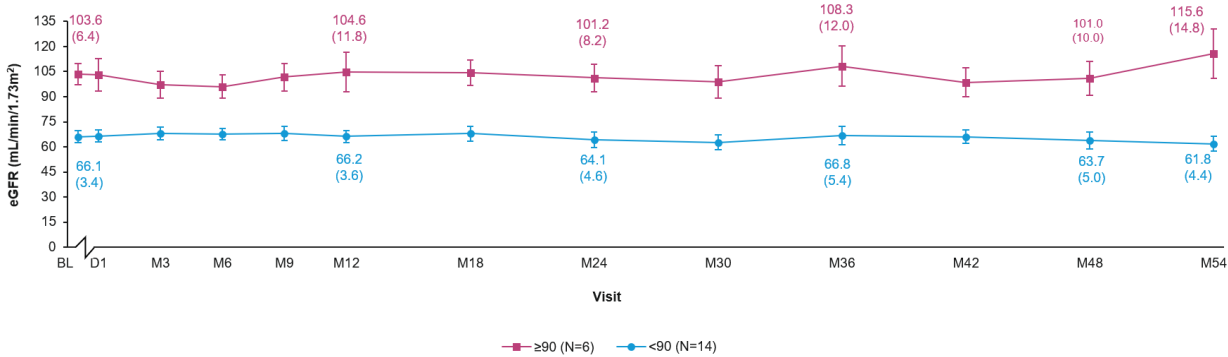
Kidney Function Measures

eGFR

In the Phase 2 OLE, the mean eGFR remained stable over time. The mean absolute change from baseline in eGFR ranged from -3.8 to 1.9 mL/min/1.73m² at Month 54. Over 48 months of follow-up, the mean (SEM) annual rate of change in eGFR value was -0.6 (0.7) mL/min/1.73m².¹

The stability of the eGFR values was consistent over time in a subgroup analysis stratified by an eGFR of ≥ 90 versus < 90 mL/min/1.73 m² at the Phase 1/2 parent study-derived baseline (**Figure 1**).¹

Figure 1. Mean (SEM) eGFR Values Through Month 54 Stratified by Baseline eGFR.¹



Abbreviations: BL = baseline; D = day; eGFR = estimated glomerular filtration rate; M = month; SEM; standard error of mean.

Footnotes: BL is the derived baseline value from the Phase 1/2 parent study.

From Frishberg et al.¹

Safety

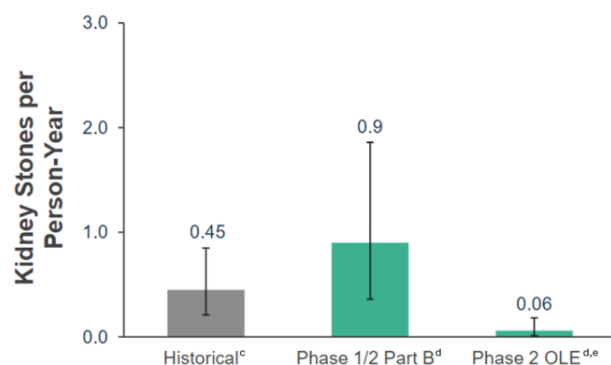
At Month 54 of the Phase 2 OLE, 20 patients (100%) reported AEs, of which 11 patients (55%) experienced treatment-related AEs. The most common treatment-related AEs were ISRs, which occurred in 8 patients (40%). All ISRs were mild in severity, and no ISRs were reported after Month 18 through the end of the study. Serious AEs were reported in 7 patients (35%) and severe AEs were reported in 2 patients (10%), none of which were related to lumasiran. There were no AEs that led to treatment discontinuation, study withdrawal, or death.¹

Kidney Stone-Related AEs

In the 12 months prior to consent (historical), 6 patients (30%) reported ≥ 1 kidney stone. During Part B of the Phase 1/2 study, 4 patients (20%) reported kidney stone-related AEs during lumasiran treatment. After continuing to the Phase 2 OLE, 3 patients (15%) reported kidney stone-related AEs. The rate of kidney

stone-related AEs decreased during the Phase 2 OLE compared with Part B of the Phase 1/2 study (Figure 2).² At Month 54 of the Phase 2 OLE, the rate of kidney stone-related AEs was 0.17 per PY.¹

Figure 2. Kidney Stones Per PY in the Phase 1/2 Part B and Phase 2 OLE Studies.^{2,a,b}



Abbreviations: AE = adverse event; CI = confidence interval; OLE = open-label extension; PY = person-year.

^aError bars represent 95% CI.

^bDuration of follow-up: historical, 20 PYs; Phase 1/2 Part B, 7.8 PYs; Phase 2 OLE, 48.0 PYs.

^cHistorical describes the number of symptomatic kidney stone episodes reported in the 12 months prior to consent for the 001 study.

^dIn the Phase 1/2 Part B and Phase 2 OLE studies, kidney stones were described as kidney stone-related AEs.

^eData presented from Phase 2 OLE with a data cut-off of March 1, 2021.

From Lieske et al.²

ILLUMINATE-A STUDY

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 ≥ 30 mL/min/1.73m². 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-hour 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.¹⁰ Of the 39 patients enrolled, 13 patients (100%) in the placebo/lumasiran group and 24 patients (92%) in the lumasiran/lumasiran group completed treatment in the 54-month OLE.³

The change from baseline in eGFR to Month 6 was evaluated as a secondary endpoint. eGFR was calculated with the MDRD formula for patients ≥ 18 years of age and with the Schwartz Bedside Formula for patients 6 to <18 years of age. The rate of KSEs and change from baseline in nephrocalcinosis grade were evaluated as exploratory endpoints.¹⁰

Patient Demographics & Baseline Characteristics

Relevant baseline characteristics are shown below in Table 2.

Table 2. ILLUMINATE-A Relevant Baseline Characteristics.^{3,11,a}

Characteristic	Placebo/Lumasiran (N=13)	Lumasiran/Lumasiran (N=26)	All Lumasiran ^b (N=39)
Mean age at informed consent (range), y	17.0 (6-60)	18.7 (6-47)	18.1 (6-60)
Male, n (%)	8 (62)	18 (69)	26 (67)

Characteristic	Placebo/Lumasiran (N=13)	Lumasiran/Lumasiran (N=26)	All Lumasiran ^b (N=39)
Race, n (%)			
Asian	3 (23)	3 (12)	6 (15)
White	9 (69)	21 (81)	30 (77)
Other or >1 race	1 (8)	2 (8)	3 (8)
Mean (SD) eGFR, mL/min/1.73m ²	78.8 (30.0)	83.0 (25.5)	81.6 (26.8)
Patients reporting history of KSEs, n (%) ^c			
Lifetime	10 (77)	23 (88)	33 (85)
12 months prior to consent	4 (31)	11 (42)	15 (38)

Abbreviations: eGFR = estimated glomerular filtration rate; KSE = kidney stone event; SD = standard deviation; ULN = upper limit of normal.

^aBaseline is defined as the last non-missing value prior to the first dose of lumasiran.

^bThe all-lumasiran-treated set includes all patients who received any amount of lumasiran.

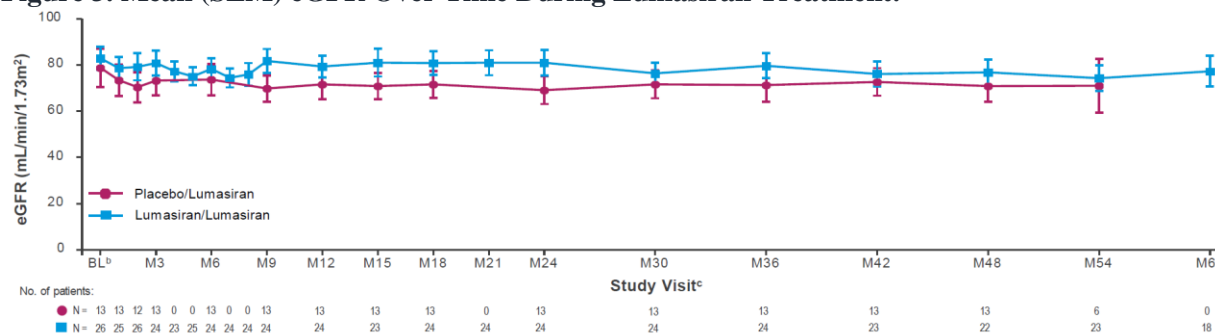
^cA KSE is defined as an event that includes at least one of the following: visit to healthcare provider because of a kidney stone, medication for kidney colic, stone passage, or macroscopic hematuria due to a kidney stone.

Kidney Related Outcomes

eGFR

The mean eGFR remained stable through the end of the study among lumasiran treated patients (**Figure 3**). The mean (SEM) change from baseline was -12.86 (3.89) mL/min/1.73m² in the placebo/lumasiran group and -2.89 (2.75) mL/min/1.73m² in the lumasiran/lumasiran group. In the all-lumasiran-treated set, the mean annual rate of eGFR change per year at Month 48 was -1.19 mL/min/1.73m².³

Figure 3. Mean (SEM) eGFR Over Time During Lumasiran Treatment.³



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

^aeGFR 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.

^bBL is the last assessment collected prior to the first dose date/time of lumasiran.

^cVisit is relative to the first dose of lumasiran.

From Saland et al.³

Kidney Stone Events

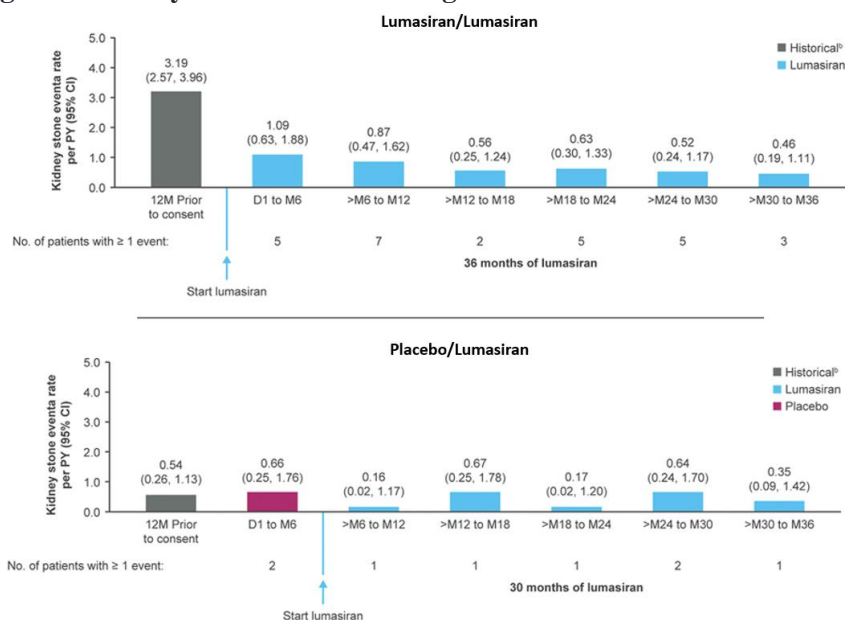
During the patient-reported 12-month historical recall period, KSE rates (95% CI) were 3.19 (2.57, 3.96) per PY in the lumasiran/lumasiran group and 0.54 (0.26, 1.13) per PY in the placebo/lumasiran group.¹²

In the all-lumasiran-treated set (i.e., all patients who received any amount of lumasiran), KSE rates (95% CI) decreased from 2.31 (1.88, 2.84) per PY during the patient-reported 12-month historical recall period to 0.60 (0.46, 0.77) per PY with 36 months of lumasiran treatment.¹¹ KSE rates for the lumasiran/lumasiran group and placebo/lumasiran group are shown in intervals through Month 36 in **Figure 4**.¹²

KSE rates (95% CI) were 0.47 per PY with 60 months of lumasiran treatment in the lumasiran/lumasiran group and 0.54 per PY with 54 months of lumasiran treatment in the placebo/lumasiran group.³

During the final 6 months of lumasiran treatment, KSE rates were 0.09 per PY in the lumasiran/lumasiran group and 0.68 per PY in the placebo/lumasiran group. No KSEs occurred during lumasiran treatment in 13 of the 26 patients (50%) in the lumasiran/lumasiran group and in 8 of the 13 patients (62%) in the placebo/lumasiran group.³

Figure 4. Kidney Stone Events Through Month 36 of Lumasiran Treatment by Treatment Group.¹²



Abbreviations: CI = confidence interval; D = day; KSE = kidney stone event; M = month; PY = person-year.

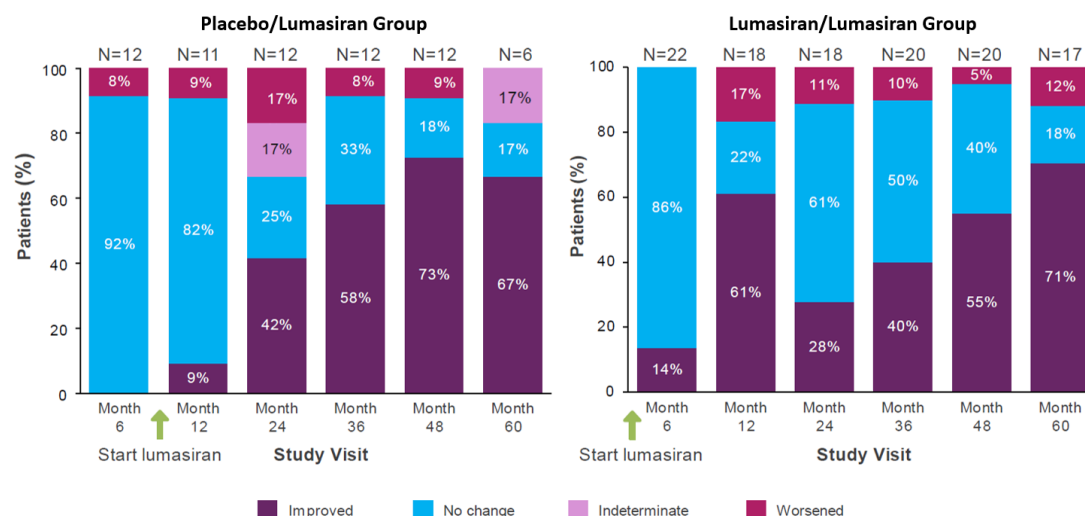
^aA KSE is defined as an event that includes at least one of the following: visit to healthcare provider because of a kidney stone, medication for kidney colic, stone passage, or macroscopic hematuria due to a kidney stone. ^bPatient-reported history of KSEs. From Saland et al.¹²

Nephrocalcinosis

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).³

Medullary nephrocalcinosis generally remained stable or improved at Month 60 (**Figure 5**). Among the 20 patients who had medullary nephrocalcinosis at baseline, medullary nephrocalcinosis grade improved in 16 patients (80%) at Month 60.³

Figure 5. Change From Baseline in Medullary Nephrocalcinosis During Lumasiran Treatment.³



From Saland et al.³

Safety

At Month 60, 37 of the 39 patients (95%) experienced an AE. The most common lumasiran-related AEs were ISRs, which occurred in 14 patients (36%); all were mild in severity. Other AEs occurring in $\geq 15\%$ of patients during lumasiran treatment were abdominal pain (23%), COVID-19 (21%), headache (18%), and nasopharyngitis (15%). AEs related to the study drug were reported in 19 patients (49%) of which 13 (50%) occurred in the lumasiran/lumasiran group. Serious AEs were reported in 6 patients (15%), severe AEs were reported in 4 patients (10%), and AEs leading to discontinuation of study treatment were reported in 1 patient (3%) to be fatigue and difficulty concentrating which began during the double-blind period; all were considered not related to lumasiran treatment by the investigator. There were no deaths in the study.³

ILLUMINATE-B STUDY

ILLUMINATE-B (N=18) was a phase 3, open-label, single-arm study with a 6-month primary analysis period followed by an ongoing 54-month extension period to evaluate the efficacy, safety, pharmacokinetics, and pharmacodynamics of lumasiran in infants and young children < 6 years old with PH1 and an eGFR > 45 mL/min/1.73m² (or normal serum creatinine for infants < 12 months old). Patients received subcutaneous injections of lumasiran as determined by a body weight-based dosing regimen. The primary endpoint was the percent change from baseline in spot UOx:Cr at 6 months.⁴

The change from baseline in eGFR was evaluated as a secondary endpoint to assess kidney function during the study. eGFR was calculated based on the Schwartz Bedside formula in patients ≥ 12 months old. The KSE rates and change from baseline in nephrocalcinosis grade were evaluated as exploratory endpoints.¹³

Patient Demographics & Baseline Characteristics

Relevant baseline characteristics are shown below in **Table 3**.¹²

Table 3. ILLUMINATE-B Baseline Kidney Function Measures.^{4,13}

Characteristic	Initial Weight Group			All Treated (N=18)
	< 10 kg (N=3)	10 to <20 kg (N=12)	≥ 20 kg (N=3)	
Median age at informed consent, months (range)	10.1 (3-14)	50.1 (23-72)	62.2 (54-72)	50.1 (3-72)
Median time from diagnosis to first dose date, months	11.6	28.6	46.4	23.5
Median eGFR ^a (range), mL/min/1.73m ²	135 (135-135)	111 (76-174)	90 (65-135)	111 (65-174)
History of KSEs in past 12 months, n (%)	0	2 (17)	1 (33)	3 (17)
Presence of nephrocalcinosis at baseline, n (%)	3 (100)	10 (83)	1 (33)	14 (78)

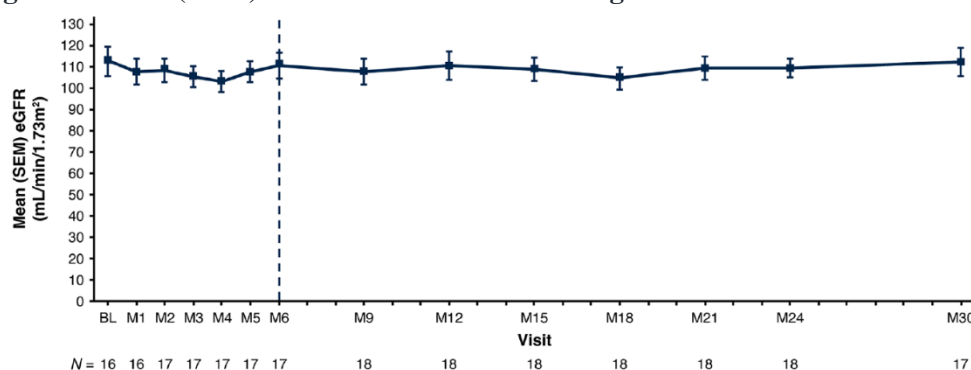
Abbreviations: eGFR = estimated glomerular filtration rate; KSE = kidney stone event.

^aN (all treated)=16. eGFR was not calculated in 2 patients due to their age, which at baseline was <12 months.

Kidney Related Outcomes

eGFR

Mean (SEM) eGFR remained stable from baseline through Month 6 of the primary analysis period and through Month 30 of the extension period (**Figure 6**).^{4,14} The mean (SEM) was 112.8 (6.9) mL/min/1.73m² at baseline and 112.5 (6.7) mL/min/1.73m² at Month 30.⁴

Figure 6. Mean (SEM) eGFR from Baseline Through Month 30.⁴

Abbreviations: BL = baseline; eGFR = estimated glomerular filtration rate; M = month; SEM = standard error of the mean.

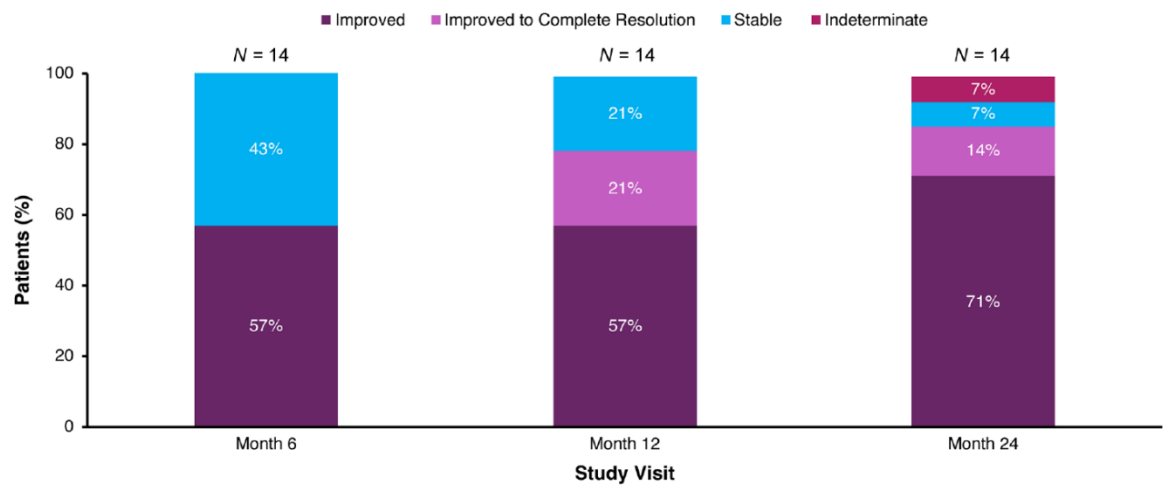
Footnotes: BL value was the last non-missing value collected prior to the first dose of lumasiran. Non-quarterly visits from the extension period are not displayed. Vertical dashed line represents the end of the primary analysis period. eGFR was not calculated for 2 patients, as their age at baseline was <12 months during that time point.

From Frishberg et al.⁴

Nephrocalcinosis

Change in nephrocalcinosis grade was assessed as an exploratory endpoint. Nephrocalcinosis was present in 14 out of 18 patients at baseline. Among these 14 patients, nephrocalcinosis grade was indeterminate in 1 patient (7%), remained stable in 1 patient (7%), and improved in 12 patients (86%), of whom 2 patients (14%) improved to complete resolution. The 4 patients without nephrocalcinosis at baseline remained stable and without nephrocalcinosis at Month 24 (**Figure 7**).⁴

Figure 7. Change in Medullary Nephrocalcinosis Grade in Patients with Nephrocalcinosis at Baseline.^{4,a-c}



^aWorsened = grade higher than baseline; stable = grade same as baseline; improved = grade lower than baseline; indeterminate = one kidney improved and the other kidney worsened.

^bThe 4 patients without nephrocalcinosis at baseline, who remained stable and without nephrocalcinosis at month 24, are not depicted.

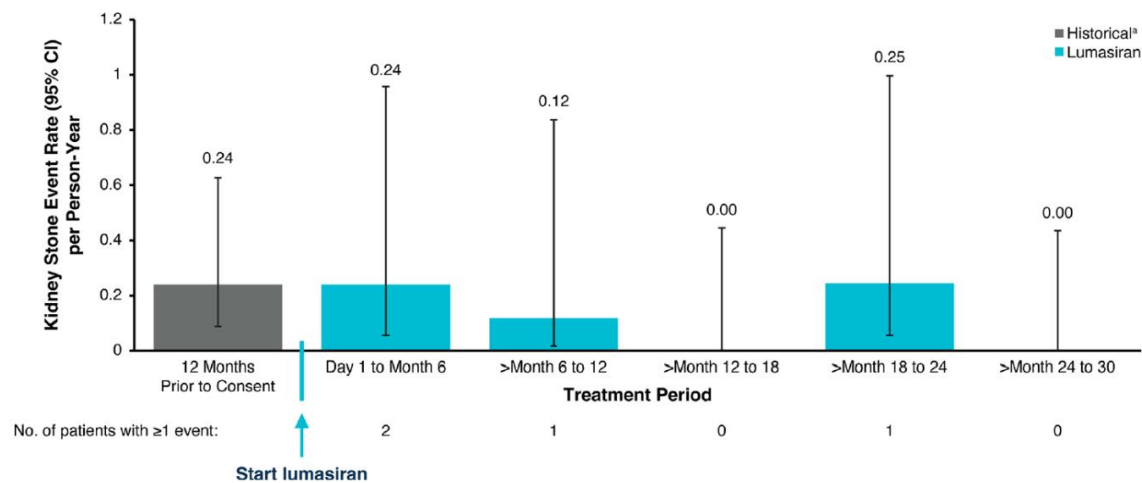
^cKidney ultrasound was not performed at Month 30.

From Frishberg et al.⁴

Kidney Stone Events

A kidney stone event was defined as ≥ 1 of the following (as adjudicated by the Investigator): visit to healthcare provider because of a kidney stone, medication for kidney colic, stone passage, or macroscopic hematuria due to a kidney stone. Patient-reported historical KSE rates were ≤ 0.25 /person-year through Month 30 of lumasiran treatment (**Figure 8**).⁴

Figure 8. Kidney Stone Event Rates from Historical Recall Through Month 30.^{4,a,b}



Abbreviations: CI = confidence interval.

^aHistorical group: patient-reported history of kidney stone events; annualized rate was not calculated for patients age < 6 months.

^bRate is calculated as total number of kidney stone events divided by total person-years during respective period. The 95% CI for the event rate was obtained using a generalized linear model for a Poisson distribution unless the rate was 0, in which case the upper bound of the 95% CI was calculated using the exact Poisson method.

From Frishberg et al.⁴

Safety

The safety profile of lumasiran was evaluated in the 6-month primary analysis and at Month 30 of the extension period, with a total median (range) exposure to lumasiran of 32.6 (27.5-35.3) months. There were 18 patients (100%) who reported an AE, of which five patients (28%) experienced lumasiran-related AEs: ISRs, transient blood bilirubin increase, and headache. The majority of the lumasiran-related AEs were mild, transient ISRs (3 patients [17%] experienced symptoms of erythema, discoloration, and pain at injection site). There were no clinically relevant changes in laboratory measures, vital signs, or electrocardiograms related to lumasiran. No serious AEs reported were considered related to lumasiran. There were no AEs that led to treatment discontinuation, study withdrawal, or deaths from the study.⁴

ILLUMINATE-C STUDY

ILLUMINATE-C was a phase 3, open-label, single-arm study with a 6-month primary analysis period followed by an ongoing 54-month extension period to evaluate the efficacy, safety, pharmacokinetics, and pharmacodynamics of lumasiran in full term infants to adult patients with advanced PH1 with an eGFR ≤ 45 mL/min/1.73m² (or elevated serum creatinine if <12 months old) and POx ≥ 20 μ mol/L. Patients enrolled in the study included those not receiving hemodialysis in Cohort A (N=6) and those receiving hemodialysis in Cohort B (N=15). Patients received subcutaneous injections of lumasiran as determined by a body weight-based dosing regimen. The primary endpoints were the percent change from baseline in POx at 6 months (Cohort A) and percent change from baseline in predialysis POx at 6 months (Cohort B).⁵

The change from baseline in eGFR was evaluated in Cohort A as a secondary endpoint to assess kidney function during the study. eGFR was calculated with the MDRD formula for patients ≥ 18 years of age and with the Schwartz Bedside Formula for patients 1 to <18 years of age. The rate of KSEs and change from baseline in nephrocalcinosis grade were also evaluated as secondary endpoints in the extension period.⁵

Patient Demographics & Baseline Characteristics

Relevant baseline characteristics are shown below in **Table 4**.⁵

Table 4. ILLUMINATE-C Baseline Kidney Function Measures.⁵

Characteristic	Cohort A N=6	Cohort B N=15	All Treated N=21
Median age at consent (range), years	9.0 (0-40)	6.0 (1-59)	8.0 (0-5.9)
Time from diagnosis to first dose, mo	72.2 (4-350)	16.6 (6-440)	21.6 (4-440)
Median eGFR (range), mL/min/1.73 m ²	N=5 ^a 16.5 (8.6-34.1)	NA	N=5 ^a 16.5 (8.6-34.1)
Median number of dialysis therapy sessions per week (range)	NA	6 (3-7)	NA

Abbreviations: eGFR = estimated glomerular filtration rate; NA = not applicable.

^aeGFR value available for 5 patients in Cohort A.

Kidney Related Outcomes

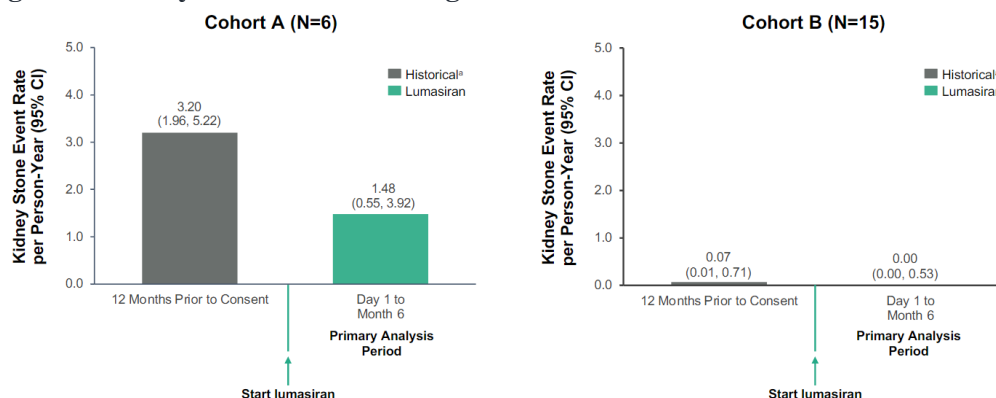
eGFR

The change in eGFR was evaluated for Cohort A only. The mean (SD) eGFR was 19.8 ± 9.6 mL/min/1.73 m² at baseline and 16.4 ± 9.8 mL/min/1.73 m² at Month 6.⁵

Kidney Stone Events

For patients enrolled in Cohort A, the KSE rates (95% CI) were 3.20 (1.96, 5.22) per PY in the 12 months prior to informed consent and 1.48 (0.55, 3.92) per PY in the 6-month primary analysis period. For patients enrolled in Cohort B, the KSE rates were 0.07 (0.01, 0.71) per PY in the 12 months prior to informed consent and 0.00 (0.00, 0.53) per PY in the 6-month primary analysis period (**Figure 9**).⁶

Figure 9. Kidney Stone Events During Lumasiran Treatment.⁶



Abbreviations: CI = confidence interval; KSE = kidney stone event.

^aHistorical group: patient-reported history of KSEs; annualized rate was not calculated for patients <6 months old.

From Groothoff et al.⁶

Nephrocalcinosis

In Cohort A, medullary nephrocalcinosis was present at baseline in 5 of the 6 patients (83%) with kidney ultrasound results. At Month 6, of those 5 patients, the grade of medullary nephrocalcinosis remained stable in 2 patients (40%), worsened in none (0%), and improved in 3 patients (60%) (2 unilateral improvements and 1 bilateral improvement).⁵

In Cohort B, medullary nephrocalcinosis was present at baseline in 2 of the 11 patients (18%) with kidney ultrasound results. At Month 6, an improvement in nephrocalcinosis was observed in both patients (100%) (1 unilateral improvement and 1 bilateral improvement).⁵

Of the 10 patients without nephrocalcinosis at baseline (1 patient in Cohort A, 9 patients in Cohort B), bilateral worsening was observed in the patient in Cohort A (10%), and the grade of nephrocalcinosis remained stable in the 9 patients in Cohort B (90%).⁵

Safety

At Month 24, 21 patients (100%) experienced at least 1 AE. The most frequently reported AEs were pyrexia (38%), diarrhea (29%), and ISRs (24%). Treatment-related AEs were reported in 7 patients (33%). The most common treatment-related AEs were mild ISRs, which occurred in 5 patients (24%). There were no deaths in the study or lumasiran-related severe AEs, serious AEs, discontinuations, or study withdrawals.⁸

ABBREVIATIONS

AE = adverse event; BL = baseline; BSA = body surface area; CI = confidence interval; D = day; eGFR = estimated glomerular filtration rate; ISR = injection-site reactions; KSE = kidney stone event; MDRD = Modification of Diet in Renal Disease; M = month; NA = not applicable; OLE = open-label extension; PH1 = primary hyperoxaluria type 1; POx = plasma oxalate; PY = person-year; SD = standard deviation; SEM = standard error of the mean; UOx = urinary oxalate; ULN = upper limit of normal; UOx:Cr = urinary oxalate:creatinine ratio.

Updated 20 November 2024

REFERENCES

1. Frishberg Y, Groothoff JW, Hulton SA, et al. Long-term treatment with lumasiran: final results from the phase 2 open-label extension study. Presented at: European Renal Association (ERA) Congress; May 23-26, 2024; Stockholm, Sweden.
2. Lieske JC, Garrelfs SF, Michael M, et al. Effect of lumasiran on kidney stones and nephrocalcinosis in patients with primary hyperoxaluria type 1. Presented at: Annual Meeting of the American Urological Association (AUA); September 10-13, 2021; Virtual.

3. Saland JM, Lieske JC, Willey R, et al. Long-term efficacy and safety of lumasiran in patients with primary hyperoxaluria type 1: Final analysis of the ILLUMINATE-A trial. Presented at: American Society of Nephrology (ASN) Kidney Week; October 24-27, 2024; San Diego, CA, USA.
4. Frishberg Y, Hayes W, Shasha-Lavsky H, et al. Efficacy and safety of lumasiran for infants and young children with primary hyperoxaluria type 1: 30-month analysis of the phase 3 ILLUMINATE-B trial. *Front Pediatr*. 2024;12. doi:10.3389/fped.2024.1392644
5. Michael M, Groothoff JW, Shasha-Lavsky H, et al. Lumasiran for advanced primary hyperoxaluria type 1: Phase 3 ILLUMINATE-C trial. *Am J Kidney Dis*. 2023;81(2):145-155. doi:10.1053/j.ajkd.2022.05.012
6. Groothoff JW, Michael M, Shasha-Lavsky H, et al. Lumasiran for patients with primary hyperoxaluria type 1 with impaired kidney function: Data from the 6-month analysis of the phase 3 ILLUMINATE-C trial. Presented at: European Renal Association (ERA) Congress; May 19-22, 2022; Paris, France.
7. Michael M, Magen D, Hayes W, et al. Efficacy and safety of lumasiran for infants and young children with primary hyperoxaluria type 1: 30-month analysis of the phase 3 ILLUMINATE-B trial. Presented at: American Society of Pediatric Nephrology (ASP/N)/Pediatric Academic Societies (PAS) Annual Meeting; April 27-May 1, 2023; Washington, DC, USA.
8. Lieske J, Sellier-Leclerc AL, Shasha-Lavsky H, et al. Lumasiran for primary hyperoxaluria type 1 with impaired kidney function: 24-month analysis of the phase 3 ILLUMINATE-C trial. Presented at: American Society of Nephrology (ASN) Kidney Week; November 2-5, 2023; Philadelphia, PA, USA.
9. Frishberg Y, Deschênes G, Groothoff JW, et al. Phase 1/2 study of lumasiran for treatment of primary hyperoxaluria type 1: A placebo-controlled randomized clinical trial. *Clin J Am Soc Nephrol*. 2021;16(7):1025. doi:10.2215/CJN.14730920
10. Garrelfs SF, Frishberg Y, Hulton SA, et al. Lumasiran, an RNAi therapeutic for primary hyperoxaluria type 1. *N Engl J Med*. 2021;384(13):1216-1226. doi:10.1056/NEJMoa2021712
11. Supplement to: Saland JM, Lieske JC, Groothoff JW, et al. Efficacy and safety of lumasiran in patients with primary hyperoxaluria type 1: results from a phase III clinical trial. *Kidney Int Rep*. 2024. doi:10.1016/j.ekir.2024.04.048.
12. Saland JM, Lieske JC, Groothoff JW, et al. Efficacy and safety of lumasiran in patients with primary hyperoxaluria type 1: results from a phase III clinical trial. *Kidney Int Rep*. 2024. doi:10.1016/j.ekir.2024.04.048
13. Hayes W, Sas DJ, Magen D, et al. Efficacy and safety of lumasiran for infants and young children with primary hyperoxaluria type 1: 12-month analysis of the phase 3 ILLUMINATE-B trial. *Pediatr Nephrol*. 2023;38(4):1075-1086. doi:10.1007/s00467-022-05684-1
14. Sas DJ, Magen D, Hayes W, et al. Phase 3 trial of lumasiran for primary hyperoxaluria type 1: A new RNAi therapeutic in infants and young children. *Genet Med*. 2022;24(3):654-662. doi:10.1016/j.gim.2021.10.024