

BONAPH1DE: A Prospective, Observational Study of Patients With Primary Hyperoxaluria Type 1 – *Actively Enrolling*

Study Design^{1,2}

- N ~ 200 patient population
- Global, multicenter, prospective, observational, longitudinal study
- Patients managed and treated per routine clinical practice
 - Protocol does not recommend the use of any specific treatments, visits, or procedures
 - No medication provided as part of study participation
- Study data will be collected prospectively and entered into the electronic data capture at least once every 12 months

Objectives²

Primary Objective:

- To characterize the long-term real-world safety of lumasiran in patients of all ages with PH1^a

Secondary Objectives:

- To describe the natural history and progression of patients diagnosed with PH1
- To characterize the long-term real-world effectiveness of lumasiran in patients with PH1^a

Patient Criteria^{1,2}

Inclusion Criteria:

- Diagnosis of PH1, per physician's determination
- Patient consent

Exclusion Criteria:

- Currently enrolled in a clinical trial for any investigational agent

Study Expectations



Study Countries¹

North America

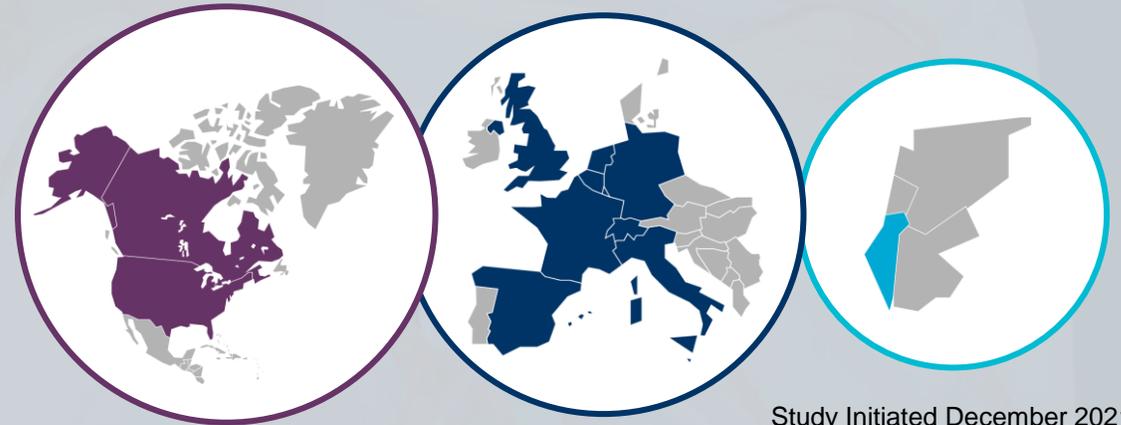
- US
- Canada

Europe

- Belgium
- France
- Germany
- Italy
- Netherlands
- Spain
- Switzerland
- UK

Middle East

- Israel



Study Initiated December 2021¹.

^aIncludes patients with hepatic impairment, severe kidney impairment, or kidney failure, including patients on dialysis, patients <2 years of age, and pregnant or lactating women.

PH1, primary hyperoxaluria type 1; UK, United Kingdom; US, United States.

1. ClinicalTrials.gov identifier: NCT04982393. Updated August 15, 2022. Accessed October 3, 2024. <https://clinicaltrials.gov/ct2/show/NCT04982393> 2. Knauf F, et al. Oral presentation at: the European Society for Paediatric Nephrology; September 16-19, 2021.

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Anticipated Population¹

- Lumasiran-treated
- Untreated
- Treated with other approved therapies
- Liver or combined liver/kidney transplant recipients
- Kidney-only transplant recipients

Key Evidence Outputs Expected¹

- Characterize long-term real-world safety and efficacy of lumasiran in different patient groups including those with renal or hepatic impairment, those under 2 years, as well as pregnant or lactating women.
- Natural history and progression of PH1
- PH1 management with concomitant therapies
- Patient-reported QoL