



Date: July 13, 2022

To: Alport Syndrome Community of Patients and Families

From: Alport Syndrome Foundation (ASF)

For Immediate Release:

It is with sadness and disappointment that Alport Syndrome Foundation (ASF) must inform our community and membership that Sanofi's HERA Study in Alport syndrome is being halted.

Though the study drug, lademirsen, demonstrated efficacy in Alport syndrome mouse models, a meaningful clinical improvement in kidney function versus placebo was not observed in patients during Phase 2 of the study. Sanofi noted to ASF that the decision to stop the program was not driven by safety concerns and "...the overall safety profile of lademirsen remains unchanged." Principal Investigators in the study have been notified by Sanofi, and they will in turn inform the study participants.

On behalf of the ASF board and staff, and our entire organizational membership, we extend our gratitude to the patients that participated in the HERA Study. Without patient participation, exploration of potential new therapies would not be possible. We are deeply grateful.

Though unexpected and disappointing, ASF must remain vigilant in our efforts to work with the research community and pharmaceutical industry to support a pipeline for potential new therapies and/or a cure for Alport syndrome. Science must move forward. This news reiterates the importance of ASF's recent major investment in a patient registry (NKF Patient Network – Alport Syndrome) and a natural history study with biosamples (NEPTUNE) to support scientific collection of patient data. It is this kind of critical collective data that will lead to a better understanding of disease progression in all types of patients, and to finding the key underlying mechanism of action that will allow for a safe and effective treatment for Alport syndrome.

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