Oxlumo™ (lumasiran) – Expanded indication

- On October 6, 2022, Alnylam Pharmaceuticals announced the FDA approval of Oxlumo (lumasiran), for the treatment of primary hyperoxaluria type 1 (PH1) **to lower urinary and plasma oxalate levels** in pediatric and adult patients.
    - Oxlumo was previously approved for this indication to lower urinary oxalate levels only.
- The approval of Oxlumo for the expanded indication was based on ILLUMINATE-C, a single-arm study in 21 patients with PH1, including patients on hemodialysis. Cohort A included 6 patients who did not require dialysis at the time of study enrollment. Cohort B included 15 patients who were on a stable regimen of hemodialysis. The primary endpoint was the percent change in plasma oxalate from baseline to month 6 for Cohort A and the percent change in pre-dialysis plasma oxalate from baseline to month 6 for Cohort B.
    - The percent change from baseline to month 6 in plasma oxalate levels in Cohort A was a least-squares (LS) mean difference of -33% (95% CI: -82, 15) and in Cohort B it was -42% (95% CI: -51, -34).
- The recommended dosing regimen of Oxlumo consists of loading doses (monthly for 3 doses) followed by maintenance doses (beginning 1 month after the last loading dose) administered subcutaneously. Dosing is based on actual body weight.
    - Refer to the Oxlumo drug label for complete dosing information.