Harliku™ (nitisinone) – New indication, new formulation

- On June 10, 2025, the FDA approved Cycle Pharmaceuticals' Harliku (nitisinone) tablets, for the **reduction of urine homogentisic acid (HGA) in adult patients with alkaptonuria (AKU)**.

- Nitisinone tablets are also approved under the brand name Nityr® for the treatment of adult and pediatric patients with hereditary tyrosinemia type 1 in combination with dietary restriction of tyrosine and phenylalanine.

- The approval of Harliku was based on an open-label, randomized, no-treatment controlled trial in 40 adult patients diagnosed with AKU. Patients received either Harliku or no treatment for three years.

    — Harliku was effective at reducing levels of urinary HGA. The Harliku group had an average percent reduction from baseline of 88% (95% CI: 79, 97) after 1 year of treatment, which was sustained through three years of treatment with an average percent reduction from baseline of 91% at year 3 (95% CI: 85, 97). In contrast, the untreated controls had an average increase of 107% from baseline to year 1 (95% CI: 0, 216) and 108% from baseline to year 3 (95% CI: 19, 198).

- The most common adverse reactions (> 1%) with Harliku use were elevated tyrosine levels, keratitis and thrombocytopenia.

- The recommended dosage of Harliku is 2 mg administered orally, once daily.

- Cycle Pharmaceuticals' launch plans for Harliku are pending. Harliku will be available as a 2 mg tablet.