Xromi (hydroxyurea) – New drug approval

- On April 4, 2024, the FDA approved Rare Disease Therapeutics' Xromi (hydroxyurea), to reduce the frequency of painful crises and reduce the need for blood transfusions in pediatric patients aged 6 months of age to less than 2 years, with sickle cell anemia with recurrent moderate to severe painful crises.
- Hydroxyurea is also available in a tablet and capsule formulation for sickle cell anemia.
- The effectiveness of Xromi was established based on an adequate and well-controlled study of hydroxyurea capsules in adult patients with sickle cell anemia with recurrent moderate to severe pain crises and additional pharmacokinetic data from a single-arm, open-label study of Xromi in pediatric patients aged 10 months to less than 2 years with sickle cell anemia, who were treatment naïve or had not received hydroxyurea in the 6 months prior to enrollment.
- Xromi carries a boxed warning for myelosuppression and malignancies.
- Additional warnings and precautions for Xromi include hemolytic anemia; embryo-fetal toxicity with unapproved use in adolescents and adults; vasculitic toxicities; live vaccinations; risks with concomitant use of antiretroviral drugs; macrocytosis; pulmonary toxicity; and laboratory test interference.
- The most common adverse reactions (> 33%) with Xromi use were neutropenia and thrombocytopenia.
- The recommended initial dose of Xromi is 15 mg/kg orally once daily. The dose may be increased by 5 mg/kg/day every 8 to 12 weeks until a maximum tolerated dose of 35 mg/kg/day is reached if blood counts are in an acceptable range.
    - Refer to the Xromi drug label for complete dosing and administration recommendations.
- Rare Disease Therapeutics' launch plans for Xromi are pending. Xromi will be available as a 100 mg/mL oral solution.