Evrysdi® (risdiplam) – Expanded indication

- On May 30, 2022, Roche announced the FDA approval of Evrysdi (risdiplam), for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.
    - The approval expands the use of Evrysdi to patients under 2 months of age with SMA. Evrysdi was previously approved in patients 2 months of age and older.
- The approval of Evrysdi for the expanded indication was based on a single-arm, open-label study in infants up to 6 weeks of age (at first dose) who have been genetically diagnosed with SMA but do not yet present with symptoms. The efficacy in pre-symptomatic SMA patients was evaluated in 7 patients who had been treated with Evrysdi for at least 12 months: four patients had 2 copies of the *SMN2* gene, 2 patients had 3 copies, and 1 patient had 4 or more copies.
    - The 6 patients with 2 or 3 copies of *SMN2* achieved the following motor milestones as measured by Section 2 of the Hammersmith Infant Neurological Examination (HINE-2) at month 12: 6 patients achieved sitting (5 patients could pivot/rotate and 1 patient achieved stable sit); 4 patients could stand (3 patients could stand unaided and 1 patient could stand with support), and 3 patients could walk independently. All 6 patients were alive at 12 months without permanent ventilation.
- In addition, the Evrysdi drug label was updated to include recent two-year pooled data from Parts 1 and 2 of the FIREFISH study, which demonstrate long-term efficacy in symptomatic infants with Type 1 SMA. A total of 62 patients were enrolled, of which 58 patients received the recommended dose. The median age at enrollment was 5.6 months (range: 2.2 to 6.9 months).
    - At month 24, 40% of patients who received the recommended dose achieved sitting without support for 30 seconds. In addition, 28% of patients achieved a standing measure.
    - The proportion of patients alive without permanent ventilation (event-free survival) was 84% for all patients at month 24. Out of 62 patients, 6 infants died (4 within the first 3 months following study enrollment) and one additional patient withdrew from treatment and died 3.5 months later. Four patients required permanent ventilation by month 24.
- The recommended dose of Evrysdi for patients less than 2 months of age is 0.15 mg/kg orally once daily.
    - Evrysdi powder must be constituted to the oral solution by a pharmacist or other healthcare provider prior to dispensing to the patient.
    - Refer to the Evrysdi drug label for additional dosing information.