Casgevy™ (exagamglogene autotemcel) – New orphan indication

- On January 16, 2024, Vertex announced the FDA approval of Casgevy (exagamglogene autotemcel), for the treatment of patients aged 12 years and older with transfusion-dependent β-thalassemia (TDT).

- Casgevy is also approved for the treatment of patients aged 12 years and older with sickle cell disease (SCD) with recurrent vaso-occlusive crises.

- Casgevy is the second cell-based gene therapy approved for TDT. Bluebird bio's Zynteglo® (betibeglogene autotemcel) was approved for the treatment of adult and pediatric patients with TDT in August 2022.

- The approval of Casgevy for the new indication was based on an ongoing open-label, single-arm study in adult and adolescent patients with TDT. Eligible patients underwent mobilization and apheresis to collect CD34+ stem cells for Casgevy manufacture, followed by myeloablative conditioning and infusion of Casgevy. Thirty-five patients had adequate follow-up to allow evaluation of the primary efficacy endpoint and formed the primary efficacy set (PES). The primary outcome was the proportion of patients achieving transfusion independence for 12 consecutive months (TI12), defined as maintaining weighted average Hb ≥ 9 g/dL without red blood cell (RBC) transfusions for at least 12 consecutive months any time within the first 24 months after Casgevy infusion, evaluated starting 60 days after the last RBC transfusion for post-transplant support or TDT disease management.

    — The TI12 responder rate was 32/35 (91.4%, 98.3% one-sided CI: 75.7, 100). All patients who achieved TI12 remained transfusion-independent, with a median (min, max) duration of transfusion-independence of 20.8 (13.3, 45.1) months.

- The minimum recommended dose of Casgevy is 3×10^6 CD34+ cells/kg, administered as a one-time, single intravenous dose.

    — Refer to the Casgevy drug label for complete dosing and administration recommendations.