Administration of Rivogenlecleucel (Rivo-cel, BPX-501) Cells Following αβ- and B-Cell–Depleted HLA-Haploidentical HSCT in Children With Fanconi Anemia

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BACKGROUND

- Allogeneic hematopoietic cell transplantation (HSCT) is curative for some children with Fanconi anemia (FA);
- Long-term survivors in patients with FA who undergo HSCT can be complicated by the occurrence of squamous cell carcinoma, which is associated with skin cancer.

OBJECTIVES

- To evaluate the safety and efficacy of rivo-cel administered after αβ- and B-cell–depleted haplo-HSCT in pediatric patients with FA.

METHODS

- In 2 multicenter, prospective phase 1/2 trials (US [NCT03301168] and EU [NCT02065869]), αβ- and B-cell–depleted haplo-HSCT was followed by infusion of a fixed number of donor lymphocytes genetically modified with the iC9 safety switch (rivo-cel) in patients with malignant and nonmalignant diseases.

RESULTS

- For patients with FA, the conditioning regimen included:
  - Cyclophosphamide 1000 mg/m² over 4 days (days -4 to -1) + Fludarabine 180 mg/m² over 4 days (days -4 to -1) + Fludarabine 180 mg/m² over 4 days (days -4 to -1) + Fludarabine 180 mg/m² over 4 days (days -4 to -1) + Fludarabine 180 mg/m² over 4 days (days -4 to -1)
  - Rabbit anti-thymocyte globulin (ATG) was administered for 3 consecutive days (days -1 to 0) to reduce the incidence and severity of possible aGvHD.
- Rivo-cel was administered on day -1 for prevention of Epstein-Barr virus–related post-transplant lymphoproliferative disorder (PTLD) via protocol, rivo-cel cells were to be injected only on day 21 ± 14 following the allograft.

- No post-transplant pharmacologic GVHD prophylaxis was used.
- Patients who developed visceral GvHD or were refractory to SoC treatment were included in the efficacy-evaluable population (EEP).

- All 6 AEs were grade 1 or 2:
  - Alopecia, maculopapular rash

- There were no relevant conflicts of interest to disclose.