RARE DISEASE DAY 2023

Newsletter #5

Today is Rare Disease Day!

- In addition to the many challenges patients with rare disease face, ***inequitable access to healthcare is often much worse in the rare disease community*** and the COVID-19 pandemic exacerbated this for many patients. These challenges are compounded for racial and ethnic minorities and marginalized groups, which leads to economic hardship, difficulty accessing care, and poorer outcomes for both patients and caregivers.[1]

- One imperative to addressing ***health equity challenges is outreach and education to patients within minority and ethnic communities and their HCPs***. Often times, HCPs are not knowledgeable on rare diseases and have their own biases, making it difficult to diagnose and treat patients appropriately. It is also critical to engage patients early and frequently to understand their treatment journey and uncover what health inequities they have faced.[2]

- While health equity is becoming more of a priority in rare disease, there is still more work to be done. It is important to ***raise awareness, advocate for the rare community, and learn more about rare diseases*** to improve the lives of patients and their families impacted.

- Rare Disease Day is formally celebrated on February 28th annually to raise awareness but at TKG, we are committed to supporting patients with rare disease year-round. To learn more about a few organizations supporting health equity in rare disease, visit: ***RareX***, ***Rare Disease Diversity Coalition***, and the ***Black Women's Health Imperative***.

TKG WORK IN RARE DISEASE

Rare Disease Experience: Epcoritimab New Product Launch

Client: Genmab/AbbVie

Rare Disease: Relapsed/Refractory Large B-Cell Lymphoma (LBCL)

Timing: PDUFA expected late May

Prevalence: There are an estimated 150,000 new LBCL cases each year globally.1 LBCL includes DLBCL, which is the most common type of NHL worldwide and accounts for approximately 30 percent of all NHL cases

Description:
LBCL is a fast-growing type of NHL, a cancer that develops in the lymphatic system and affects B-cell lymphocytes, a type of white blood cell

Launch Considerations:

- If approved for relapsed/refractory large B-cell lymphoma after two or more lines of systemic therapy, epcoritamab could become the first subcutaneous bispecific antibody available for the treatment of large B-cell lymphoma.
- Competitors are expected to enter market 2 months later

PATIENT SPOTLIGHT

Cleidocranial Dysplasia

- Cleidocranial dysplasia (CCD) affects the development of the bones, skull, and teeth. Signs and symptoms include underdeveloped or absent collarbones, dental abnormalities, and delayed closing of the spaces between the skull bones. Other symptoms may include decreased bone density, hearing loss, bone abnormalities of the hands, and recurrent sinus and ear infections. People with CCD may develop curvature of the scoliosis, osteoporosis, and may be shorter than average.
- When talking about his condition, Matarazzo shared:

Gaten Matarazzo

American Actor known for his roles on Broadway and in film

> "It's one of the biggest reasons why I [hadn't] been getting roles, because of my lisp, and the teeth situation, and my height."

- Thanks to Gaten, public awareness of CCD is rising after being written into the plotline of Stranger Things.

To learn more about CCD CLICK HERE
https://www.vasculitisfoundation.org/

Reference: 1. Milken Institute. Embracing Health Equity for the Rare Disease Community. https://milkeninstitute.org/article/health-equity-rare-disease-community. Accessed February 10, 2023.