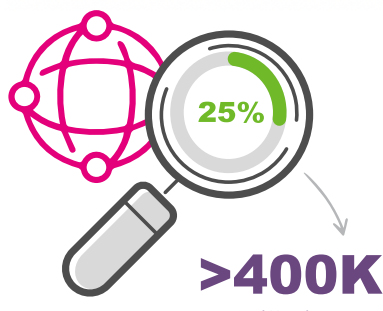




RARE DISEASE DAY 2023

Newsletter #4



- While there is no universal definition, **rare cancers are defined in the US as affecting fewer than 40,000 people** per year. Collectively, rare cancers make up about **25% of all cancers**. Given the low rates of cancer in children, all **children’s cancers are considered rare**.¹
- For many rare cancers, **research to identify causes or develop strategies for prevention and early detection is extremely difficult**. Once diagnosed, finding disease information and effective treatment options is challenging.²

- The FDA established the **Oncology Center of Excellence (OCE) Rare Cancers Program** in 2017 to promote the development of safe and effective new drugs and biologics to treat patients with rare cancers.³ The program works with experts both inside and outside the FDA to identify opportunities to decrease obstacles, better harness scientific knowledge, and strengthen coordination to facilitate efficient drug development.⁴



- To address challenges developing rare disease therapies, **Project Community** and **Project Equity** are 2 of several OCE initiatives that help promote a collaborative, inclusive approach to **clinical trial awareness** and **enrollment for a diverse patient population**.⁴ These programs present opportunities to improve health equity in historically underrepresented communities and drive change in clinical trial diversity.

TKG WORK IN RARE DISEASE

Rare Disease Experience: Lamzede® Launch

Client: Chiesi

Rare Disease: Alpha mannosidosis (AM)

Timing: February 2023

Prevalence: 1 in 500,000-1,000,000 worldwide

Description:

AM is an ultra-rare, genetically inherited, lysosomal storage disorder caused by impaired activity of the alpha-mannosidase enzyme, resulting in a multi-morbid, chronic, progressively worsening, and potentially life-threatening condition

Launch Considerations:

- High unmet need and previously no FDA-approved treatments available
- Low disease awareness leading to delayed diagnosis
- Skepticism about product data due to small patient population, resulting in payers requesting additional information to increase value rating



PATIENT SPOTLIGHT

Vasculitis

- Vasculitis involves inflammation of the blood vessels. The inflammation can cause the walls of the blood vessels to thicken, which reduces the width of the passageway through the vessel. If blood flow is restricted, it can result in organ and tissue damage. Vasculitis might affect just one organ, or several. The condition can be short term or long lasting.
- Kutcher first spoke of his condition in 2022:



Aston Kusher

American actor, producer and entrepreneur



“Two years ago, I had this weird, super rare form of vasculitis that knocked out my vision. It knocked out my hearing. It knocked out all my equilibrium,” he said. “You don’t really appreciate it until it’s gone. Until you go, ‘I don’t know if I’m ever going to be able to see again, I don’t know if I’m ever going to be able to hear again, I don’t know if I’m ever going to be able to walk again”



To learn more about Vasculitis [CLICK HERE](https://www.vasculitisfoundation.org/)

<https://www.vasculitisfoundation.org/>

GET INVOLVED

Register now! Rare Disease Day at NIH

The event aims to raise awareness about rare diseases, the people they affect, and NIH collaborations that address scientific challenges and advance research for new treatments.

Event Details

- Date: Tuesday, February 28, 2023
- Time: 9A-5P EST
- Where: Virtual
- Price: Free
- [Register here](#)



Final Call!

Send in your pictures for Show Your Stripes, Light Up For Rare, and Share Your Colors today, 2/27 to Kerri G

Reference: 1. About rare cancers. National Cancer Institute. <https://www.cancer.gov/pediatric-adult-rare-tumor/rare-tumors/about-rare-cancers>. Accessed February 9, 2023. 2. About rare cancers. National Cancer Institute. <https://www.cancer.gov/pediatric-adult-rare-tumor/rare-tumors/about-rare-cancers>. Accessed February 9, 2023. 3. Commissioner Oof the. Oncology center of excellence. U.S. Food and Drug Administration. <https://www.fda.gov/about-fda/fda-organization/oncology-center-excellence>. Accessed February 10, 2023. 4. Commissioner Oof the. OCE rare cancers program. U.S. Food and Drug Administration. <https://www.fda.gov/about-fda/oncology-center-excellence/oce-rare-cancers-program>. Accessed February 10, 2023.

