



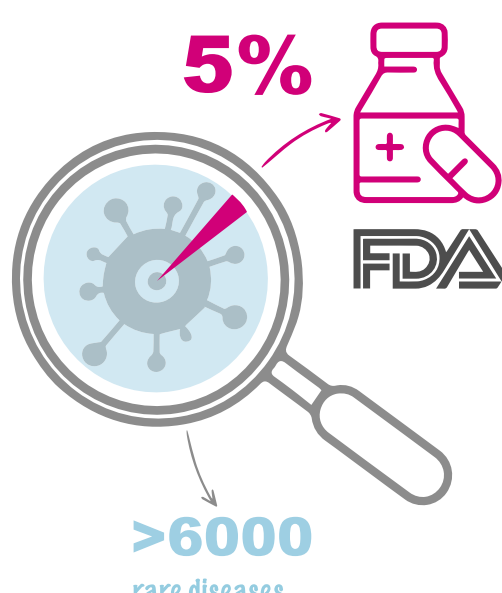
RARE DISEASE DAY 2023

Newsletter #2



5 Days Until Rare Disease Day!

- With over **6,000 rare diseases**, only **5% have at least one FDA-approved treatment** option available.
- This limited number of available treatments is caused by several hurdles, including the length of time to get a drug to market, financial risks in developing a treatment for a limited patient population, and limited knowledge about diagnosis and drug treatment options.¹
- Bringing a new drug or biologic to market can take **over a decade** and **cost more than \$2.5B**, and those numbers are increasing. Weighing the cost, time and risk associated with therapy development, academic and industry researchers and developers have been largely focused on a subset of less than 100 rare diseases, often competing for trial participants and researchers' time while "racing" to be first to achieve marketing authorizations.²



TKG WORK IN RARE DISEASE

Rare Disease Experience: Palovarotene Launch

Client: Ipsen

Rare Disease: Fibrodysplasia Ossificans Progressive (FOP)

Timing: 2023 (CRL received 12/22)

Prevalence: 0.88 in 1M people, approximately 279 people in the US

Description:

A rare musculoskeletal condition where, after birth and progressively through life, muscles and tendons are gradually transformed into bone (a process called ossification). This creates a second "skeleton" of extra bone, which makes movement impossible.

Launch Considerations:

- There are no currently no FDA-approved treatments for FOP
- Messaging considerations around the clinical trial data include:
 - » Trial was issued a partial clinical hold in 2019 due to safety concerns (premature growth plate closure in pediatrics), and there have been several delays that have shifted the anticipated approval date.
 - » Trial size is limited due to small patient population
 - » There is no active comparator, efficacy of palovarotene is compared to data from patients in a 36-month, noninterventional, longitudinal natural history study
- Palovarotene will be a specialty pharmacy product that may be subject to a REMs program, with a sole distributor



PATIENT SPOTLIGHT

Sjögren's Syndrome

- Sjogren's (SHOW-grins) syndrome is a disorder of your immune system identified by its two most common symptoms — dry eyes and a dry mouth. The condition often accompanies other immune system disorders, such as rheumatoid arthritis and lupus.



"Sjögren's is a complex and often invisible disease. As a patient, I was misdiagnosed many times and my symptoms got progressively worse to the point where I couldn't play professional tennis anymore. I understand the daily struggles and the strength it takes to open up about your journey, but I also know the support and education that is created by awareness. I applaud all the patients who shared their stories during Sjögren's Awareness Month and everyone who is living with this disease. Your stories have inspired me, and I encourage all patients to continue to talk about their disease and make their health a priority. You are not alone."



Venus Williams

American professional tennis player



To learn more [CLICK HERE](https://sjogrens.org/)

<https://sjogrens.org/>



GET INVOLVED

Game Time!

Complete the Show Your Stripes, Light Up for Rare, and Share Your Colors activities and be entered for a chance to win a Tango gift card with the winner to be announced on Rare Disease Day!

Light Up For Rare

- Everyone can participate in the **Global Chain of Lights and Share Your Colors!** In an effort of global solidarity, you are invited to light or decorate your home, office, (or desk space!) with the Rare Disease Day colors (**blue, pink, green and purple**)
- You can use garlands, candles, disco lamps, colorful decorations... Let your creativity shine!
- Send in a picture to **Kerri G by February 27th**

Fighting H.A.R.D. Foundation's Run HARD for Rare Kids

- Fighting H.A.R.D. Foundation's Run HARD for Rare Kids was started in 2021 as a way to safely raise funds and awareness for those with rare diseases. This virtual run is held the week leading up to rare disease day. There are multiple events to choose from, ranging from "Relaxing for Rare" to 28 miles over the course of the 8-day event!
- To learn more or register for an event, [click here.](#)

References: 1. Willmer G, Willmer G. The building blocks to make rare disease treatments more common. Horizon Magazine. <https://ec.europa.eu/research-and-innovation/en/horizon-magazine/building-blocks-make-rare-disease-treatments-more-common#:~:text=Indeed%2C%20only%205%25%20or%20fewer,known%20as%20%E2%80%9Corphan%E2%80%9D%20therapies.> Published February 28, 2022. Accessed February 9, 2023. 2. DiMasi JA, Grabowski HG, Hansen RW. Innovation in the pharmaceutical industry: new estimates of F&D costs. J Health Econ. 2016;47:20–33.

