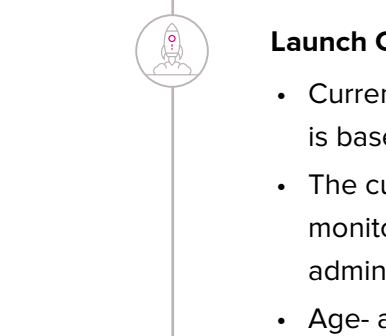
RARE DISEASE DAY 2023

Newsletter #3

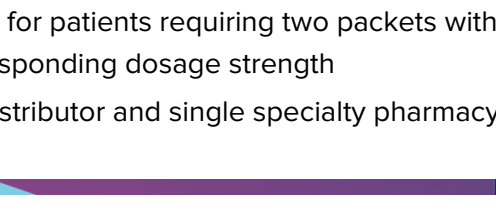
4 Days Until Rare Disease Day!



- Despite the challenges associated with bringing a rare disease therapy to market, pharmaceutical companies are increasingly becoming more involved in the space, evidenced by the ***rising number of orphan drug designation requests*** and ***drugs granted FDA-approval*** every year.

- Prior to the Orphan Drug Act in 1983, there were ***only 10 drugs approved for a rare disease***. An orphan drug designation provides incentives for manufacturers such as ***tax credits***, a ***waiver of the Prescription Drug User Fee*** (currently at ***$3 million for a new drug***), and a ***potential of 7 years of market exclusivity***. Often times, market exclusivity is associated with higher drug pricing and limits patient access.[1]

- Since 2010, the number of orphan drug designations more than doubled compared to the previous decade. It is important to note that an orphan drug designation does not automatically translate into approval. Approximately ***16% of therapies receiving the designation gained FDA approval since the Orphan Drug Act***.[1]



TKG WORK IN RARE DISEASE

Rare Disease Experience: Pradaxa® Pediatric Oral Pellets Launch

Client: Boehringer Ingelheim

Rare Disease: Pediatric venous thromboembolic events (VTE)

Timing: Capsules: June 2022; Oral Pellets: February 2023; Oral Solution: CRL

Prevalence: 1.4 to 11.9 per 100,000 persons aged 0-12 years

Description:
VTE occurs when a blood clot forms in a vein and encompasses deep venous thrombosis (DVT) and pulmonary embolism (PE). Pediatric VTE is associated with significant morbidity and mortality. Hospital-acquired VTE is more common and risk factors include surgery, immobility, inflammation, and critical illness.

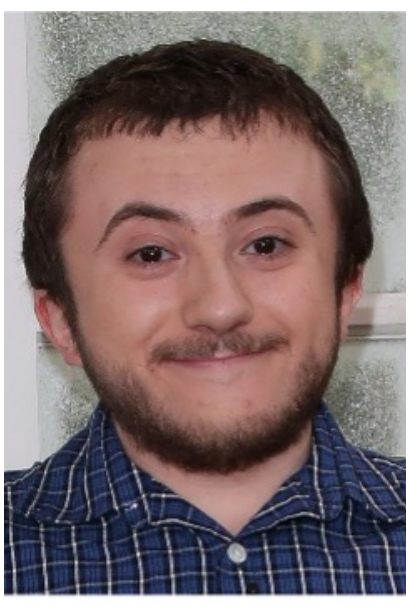
Launch Considerations:

- Currently, no large-scale trials have been completed in pediatric VTE and the standard of care is based on extrapolated data from studies performed in adults
- The current standard of care is associated with daily injections and the need for frequent monitoring of anticoagulation level, Pradaxa Capsules and Oral Pellets offer an oral method of administration
- Age- and weight-based dosing may cause challenges for patients requiring two packets with distinct NDCs – oral pellets have 6 NDCs with a corresponding dosage strength
- Oral Pellets are available through a single specialty distributor and single specialty pharmacy

PATIENT SPOTLIGHT

Osteogenesis Imperfecta

- Osteogenesis imperfecta (OI) is a group of genetic disorders that mainly affect the bones. People with this condition have bones that break easily, often from mild trauma or with no apparent cause. Multiple fractures are common, and in severe cases, can occur even before birth. Milder cases may involve only a few fractures over a person's lifetime.
- Atticus Shaffer suffers from OI type IV. He walks with a limp due to multiple breaks in his legs as a result from his condition. In a 2019 interview, the actor discussed his condition and shared:



Atticus Shaffer

American Actor known for his role as Brick Heck on ABC sitcom The Middle

> "I do know pain. I've had many fractures in my life ... I've had plates, screws, rods put in through all my legs, my back is in an s-curve because of it."

To learn more about OI CLICK HERE
https://oif.org

GET INVOLVED

Register now!

FDA's Rare Disease Day 2023

This year's theme is "Intersections with Rare Diseases – A patient focused event." Participants will have the opportunity to hear directly from the FDA on initiatives to advance medical product development for rare diseases, understand considerations and challenges associated with clinical trials in small populations, and hear from students on rare disease education for medical professionals

Event Details

- Date: Monday, February 27, 2023
- Time: 9A-4:45P EST
- Where: Virtual
- Price: Free
- ***Register here***

Reminder

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

Reference: 1. Maragkou I. Rare disease spotlight – tracing the rise of orphan drug designations over almost 40 years. Pharmaceutical Technology. https://www.pharmaceutical-technology.com/analysis/rare-disease-spotlight-tracing-the-rise-of-orphan-drug-designations-over-almost-40-years/. Published December 23, 2022. Accessed February 9, 2023.