

Evaluate real-world effectiveness on burden of disease

Cystic Fibrosis



Targeting ~300 patients for enrollment

Retrospective & Prospective

2 yr study

Challenges

- CF is associated with significantly lower quality of life and a high disease burden
- Evaluate the change in patient-reported and caregiver-reported burden of disease after initiating CF treatment.

Solution

- Conducted an observational study to evaluate the real-world effectiveness of treatment on patient and caregivers' burden of illness
- PROs and Caregiver Reporter Outcomes were collected every 2 months
- Described select clinical outcomes

