### Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Modulators: Current and Future Utilization Among 15 Million Commercial Lives

**Background**

Cystic Fibrosis (CF) is a heterozygous, autosomal recessive disorder resulting in one of the most common fatal genetic diseases in the United States and 70,000 worldwide.

- Kalydeco® (ivacaftor) was approved in 2012 for adults and children ages 6 and older with a single F508del mutation, based on improvements in lung function and respiratory exacerbations.
- Orkambi® (lumacaftor/ivacaftor) was approved in 2015 for patients ages 12 to 55 who are homozygous for the F508del mutation.
- Symdeko® (tezacaftor/ivacaftor) was approved in 2018 for patients ages 12 and older who are homozygous for the F508del mutation or who have one F508del and one residual mutation.

**Methods**

- Integrated pharmacy and medical claims data among 15 million commercial members were examined from May 2017 to April 2018 to identify members with a modulator pharmacy claim.
- A modulator’s first claim was identified by a modulator pharmacy claim (ICD-10 code $04 $043 $04) in any field at least 30 days apart.
- A member’s first modulator claim between May 2017 and April 2018 was then used to determine their index date.

**Results**

- Among approximately 15 million commercial members, 2,157 members (14.3 per 100,000) had a modulator claim.
- 1,096 (51.0%) of the 2,157 members were male. Of those, 1,124 (52.0%) were eligible for Kalydeco®. A reanalysis conducted in 2019 identified 1,124 eligible members who could have received Kalydeco®.
- The estimated 1,288 eligible. Our finding is consistent with the Cystic Fibrosis Foundation’s reported prevalence.

**Conclusions**

- Administrative and medical pharmacy claims have the potential to be encoded and include actionable summaries of members’ actual drug use and therapy outcomes.
- Future utilization will depend on future modulator availability and market dynamics.

**References**

- Kalydeco® (ivacaftor) and Orkambi® (lumacaftor/ivacaftor).
- Symdeko® (tezacaftor/ivacaftor).

**Figures**

- Figure 1: Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Modulator Trends in Utilization Among 15 Million Commercial Lives: February 2015 Through November 2018 Among 15 Million Commercial Lives.
- Figure 2: Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Modulator Trends in Utilization Among 15 Million Commercial Lives: February 2015 Through November 2018 Among 15 Million Commercial Lives.
- Figure 3: Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Modulators: Current and Future Utilization Among 15 Million Commercial Lives.