 Evaluating Real-World FEV1 Changes Before and After Starting Lumacaftor/Ivacaftor

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BACKGROUND

- Cystic Fibrosis (CF) is a life-threatening disease caused by defective or deficient cystic fibrosis transmembrane conductance regulator (CFTR) protein activity
- Patients with CF experience a progressive decline in lung function, primarily monitored by the percent predicted forced expiratory volume in one second (FEV1)%pred
- FEV1%pred allows for classification of disease severity and has been shown to be related to survival
- In 2015, the median predicted survival was 41 years
- Lumacaftor/ivacaftor clinical trials demonstrated the ability to improve FEV1%pred and reduce exacerbations and hospitalizations

METHODS

- Using CF-Center registry data and specialty pharmacy dispensing data, we identified patients 21 years of age with a baseline FEV1%pred ≥90% when starting lumacaftor/ivacaftor
- Patients were required to have:
  1) initiated lumacaftor/ivacaftor first dispense between 7/20/2015 and 6/1/2016
  2) at least one FEV1%pred measurement available for three time periods: 62 years, 1 year, 1 year post-lumacaftor/ivacaftor initiation
- 3) continued use of lumacaftor/ivacaftor during the 1 year post-lumacaftor/ivacaftor period identified by a dispense between 12/1/2016 and 6/1/2016
- FEV1%pred varies significantly within individuals and can decline substantially when a patient experiences an exacerbation; due to this inter-individual variability, we chose to evaluate average annual change
- The specific outcome of interest was average annual FEV1%pred. The change in average annual FEV1%pred between time periods was used to describe the impact of lumacaftor/ivacaftor

OBJECTIVE

Evaluate real-world FEV1%pred changes after starting lumacaftor/ivacaftor in patients at least 21 years of age with an FEV1%pred baseline of 90% or greater

RESULTS

- Thirteen patients met all inclusion criteria of which 54% were female with an average age of 33 years
- Note: 2 patients were between the age of 51 and 60 years (Figure 1)
- The average baseline FEV1%pred ranged from 91 to 109 and post-lumacaftor/ivacaftor FEV1%pred ranged from 85 to 112(Figure 2)
- The average medication possession ratio (MPR) for the 13 patients during the one year post-lumacaftor/ivacaftor initiation was 84.6%
- Before starting lumacaftor/ivacaftor, 66% patients had declining average annual FEV1%pred, of which 33% were more than -5%, and four patients had improvements of 1% or less (Figure 3)
- After starting lumacaftor/ivacaftor, the change in average FEV1%pred was positive for 11/13 (85%) patients:
  - Two patients had an increase greater than 5%
  - Two patients had an increase that was less than the pre-lumacaftor/ivacaftor change.
  - Two patients had decreased annual FEV1%pred of 0.1% and 3.4%.
- When comparing post- vs pre-lumacaftor/ivacaftor FEV1%pred to the pre-period, 9 of the 13 patients (69%) had a greater improvement in average FEV1%pred (Figure 5)

CONCLUSIONS

- We provide preliminary data to suggest that adults CF patients with FEV1%≥90% benefit from lumacaftor/ivacaftor
- After starting lumacaftor/ivacaftor a greater percentage of patients reported greater lung function gains and negative changes did not exceed 5%.
- Further research is needed to better understand long-term impact to patient outcomes.

REFERENCES

1. Cystic Fibrosis Foundation Patient Registry 2015 Annual Data Report (Booths, Maryland ©2016 Cystic Fibrosis Foundation)