

INTRODUCTION

- Cystic fibrosis (CF) is a progressive, genetic disease primarily affecting the respiratory, digestive and reproductive organ systems
- Every year, over 1,000 new CF cases are diagnosed in the United States (US) and in 2017, more than 30,000 individuals were reportedly living with CF in the US
- CFTR modulator therapies, the most advanced CF treatments, act by improving production, intracellular processing, and/or function of the defective CFTR protein. This helps in symptom management and slowing disease progression
- Currently, three approved CFTR modulator therapies are available in the market—ivacaftor, lumacaftor/ivacaftor, and tezacaftor/ivacaftor + ivacaftor with an annual wholesale acquisition cost (WAC) of \$272,886, \$311,719 and \$292,258, respectively
- In patients who were prescribed ivacaftor and lumacaftor/ivacaftor, these drugs accounted for 85% and 74% of their total pharmaceutical spending, respectively
- Thus, there is a need to conduct real-world studies to assess the economic burden of CFTR modulator therapies on both the patients and the specialty pharmacy

OBJECTIVE

To analyze the trends associated with the utilization of CFTR modulator therapies in terms of:

- patient co-pay based on their insurance characteristics, and
- the annual spending of the specialty pharmacy

METHODS

- The study was a retrospective analysis of a national specialty pharmacy prescription refill data for CFTR modulator therapies from January 2015 - August 2018
- Medication name, number of prescriptions, refills, insurance characteristics (primary and secondary insurance types), and patient co-pay values were utilized in estimating the economic burden of CFTR modulator therapies
- Mean co-pay for each CFTR modulator therapy was calculated using the co-pay amount collected during medication refill, which was further categorized based on patient's insurance data
- Insurance data included the insurance type (commercial, government or charitable foundations) and the enrollment of the patient in a secondary insurance plan
- Co-pay amounts were adjusted to 2018 dollar value using the inflation data reported by the Bureau of Labor Statistics
- To estimate the spending of the specialty pharmacy, the number of prescriptions dispensed every year for each CFTR modulator therapy (identified from the refill database) were multiplied with the WAC (price reported in the Red Book) for each CFTR modulator therapy
- Statistical analyses were conducted using Statistical Analysis System University Edition (SAS Institute; Cary, NC)

RESULTS

- A total of 4,444 patients contributed to 57,960 refills of CFTR modulator therapies from January 2015 - August 2018
- Majority of refills (62.95%) were for patients with only primary insurance whereas 37.05% of refills were for patients with both primary and secondary insurances
- Overall, a non-linear trend was observed in average patient co-pay with a high of \$312.70 (2018) and a low of \$182.05 (2016)
- Patients on primary government insurance had a lower co-pay (\$0-\$40) compared to those on commercial insurance (\$20-\$310)
- For patients with both primary and secondary insurances, the secondary co-pay over all the years (January 2015-August 2018) ranged from \$0-\$35
- An upward trend was observed in the spending on lumacaftor/ivacaftor with \$67 million in 2015 and increasing to \$281 million in 2017. However, the spending on ivacaftor remained fairly constant over the years with \$103 million spent in 2015 and \$119 million in 2017
- With the introduction of tezacaftor/ivacaftor + ivacaftor in 2018, the spending was observed to be \$86.06 million (data till August 2018)

RESULTS

Figure 1: Trends in average co-pay (2015- August 2018)

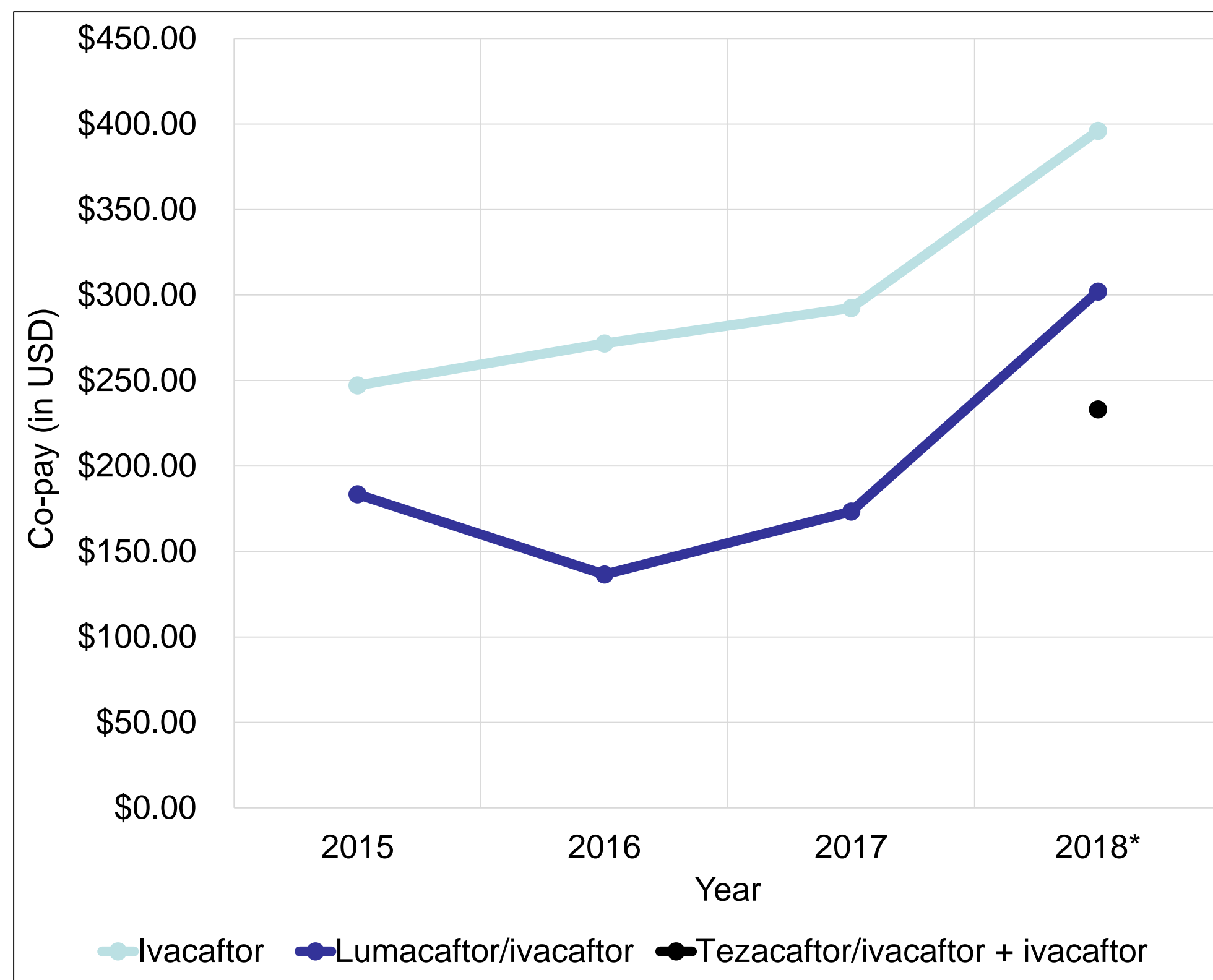


Table 1: Average co-pay from 2015- August 2018

	2015	2016	2017	2018*
Drugs	\$220.01	\$182.05	\$205.60	\$312.70
Ivacaftor	\$247.14	\$271.65	\$292.36	\$396.00
Lumacaftor/ivacaftor	\$183.42	\$136.53	\$173.35	\$302.01
Tezacaftor/ivacaftor + ivacaftor	-	-	-	\$233.16

* Calculated using data only till August 2018

Table 2: Average co-pay for CFTR modulator therapies for patients only on primary insurance from 2015- August 2018

	2015		2016		2017		2018	
	Government	Commercial	Government	Commercial	Government	Commercial	Government	Commercial
Ivacaftor	\$3.82	\$309.42	\$2.31	\$297.13	\$16.82	\$197.58	\$12.21	\$160.75
Lumacaftor/ivacaftor	\$10.88	\$25.63	\$0.80	\$19.98	\$1.76	\$124.16	\$0.55	\$146.06
Tezacaftor/ivacaftor + ivacaftor							\$38.14	\$203.84

Table 3: Average co-pay for CFTR modulator therapies for patients on primary and secondary insurance from 2015- August 2018

	2015		2016		2017		2018	
	Primary Co-pay	Secondary Co-pay	Primary Co-pay	Secondary Co-pay	Primary Co-pay	Secondary Co-pay	Primary Co-pay	Secondary Co-pay
Primary Insurance - Commercial & Secondary Insurance - Commercial								
Ivacaftor	\$321.97	\$22.89	\$407.25	\$13.72	\$583.51	\$12.9	\$747.41	\$21.95
Lumacaftor/ivacaftor	\$564.3	\$14.56	\$320.98	\$13.18	\$409.35	\$18.08	\$603.05	\$15.17
Tezacaftor/ivacaftor + ivacaftor							\$372.78	\$13.41
Primary Insurance - Commercial & Secondary Insurance - Government								
Ivacaftor	\$208.13	\$0.33	\$239.22	\$0.1	\$336.4	\$0.24	\$400.01	\$0.15
Lumacaftor/ivacaftor	\$468.43	\$3.66	\$509.67	\$0.08	\$343.34	\$0.22	\$659.44	\$0.11
Tezacaftor/ivacaftor + ivacaftor							\$84.69	\$0.11
Primary Insurance - Government & Secondary Insurance - Government								
Ivacaftor	\$1016.45	\$16.03	\$1591.47	\$9.96	\$969.77	\$4.12	\$837.33	\$0
Lumacaftor/ivacaftor	\$145.69	\$0	\$218.65	\$0	\$238.5	\$0.25	\$610.29	\$0
Tezacaftor/ivacaftor + ivacaftor							\$266.6	\$0
Primary Insurance - Government & Secondary Insurance - Charitable Foundation								
Ivacaftor	\$1159.45	\$17.37	\$1146.48	\$34.23	\$799.24	\$4.51	\$1153.97	\$45.36
Lumacaftor/ivacaftor	\$962.00	\$4.62	\$843.67	\$3.85	\$429.97	\$7.66	\$595.54	\$0.8
Tezacaftor/ivacaftor + ivacaftor							\$858.87	\$15.9

Table 4: Annual spending of the specialty pharmacy on CFTR modulator therapies

	2015	2016	2017	2018*
Ivacaftor	\$103,876,477	\$117,067,141	\$119,456,754	\$114,558,047
Lumacaftor/ivacaftor	\$67,422,678	\$201,703,216	\$281,342,724	\$183,315,212
Tezacaftor/ivacaftor + ivacaftor				\$86,060,800

*Spending calculated only till August 2018; Symdeko launched in 2018

CONCLUSIONS

- Higher co-pay values have been previously associated with lower patient adherence and adverse clinical outcomes. There is a need to monitor these characteristics in CF as well to understand the impact of co-pays across different insurances and patient outcomes
- Spending on ivacaftor and lumacaftor/ivacaftor has not decreased even with the introduction of tezacaftor/ivacaftor + ivacaftor. This may potentially be due to lower switching rates among patients who have a good response to older CFTR modulator therapies
- Increase in the number of patients enrolled in the CF program at the specialty pharmacy could be a potential reason for the growth in specialty pharmacy spending over the years