

MEDICAL COSTS OF ALPHA-1 ANTITRYPSIN DEFICIENCY: EVIDENCE FROM REAL-WORLD CLAIMS DATA



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PURPOSE

- The goal of this study was to estimate the direct costs associated with Alpha-1 Antitrypsin Deficiency (AATD) to both payers and patients.
- While previous studies have attempted to estimate similar measures, they have relied primarily on surveys.^{1,2}
- This study involved a retrospective analysis of data from a large claims database and is the first to report U.S.-based longitudinal estimates of costs of care in the commercially insured AATD population.

METHODS

- Retrospective analysis of data using the OptumLabs™ Data Warehouse (OLDW). The OLDW is a comprehensive, longitudinal, real-world data asset with de-identified lives across claims and clinical information.³
- Patient identification: first AATD diagnosis or augmentation therapy claim(s) denoted the index date (1993 through 2015):
 - AATD patients ≥ 18 years at index date included.
 - Patients ≥ 65 years of age with Medicare as primary payer excluded.
- Health plan and patient out-of-pocket costs were categorized into the following cost buckets:
 - Physician visits (PV), emergency room visits (ER), inpatients stays (IP), augmentation therapy (AUG), other prescription drugs costs (RX), and other costs (OTH).
- Costs were weighted based on patients' insurance coverage period and adjusted to 2017 U.S. dollars using the medical component of the Consumer Price Index.
- Costs were compared across augmentation use status by Wilcoxon rank sum tests.

AIM 1: TO CALCULATE MEDICAL COSTS OF CARE ASSOCIATED WITH AATD TO THE INSURER AND TO THE PATIENT

RESULT 1

In our cohort, 12.5% of commercially insured AATD patients used augmentation therapy.

Table 1. Demographic and clinical characteristics of commercially insured AATD patients at index date.

Variable	Full Cohort N = 9,117		Augmentation Cohort N = 1,142		Non-Augmentation Cohort N = 7,975		P value*
	Median (Col %)		Median (Col %)		Median (Col %)		
Age categories, n							< 0.001
40<	1,808	19.83	119	10.42	1,689	21.18	
41-53	2,601	28.53	459	40.19	2,142	26.86	
54-64	2,850	31.26	416	36.43	2,434	30.52	
>=65	1,858	20.38	148	12.96	1,710	21.44	
Gender, n							0.252
Male	4,414	48.42	571	50.00	3,843	48.19	
Female	4,703	51.58	571	50.00	4,132	51.81	
Race/Ethnicity, n							< 0.001
White	5,569	61.08	731	64.01	4,805	60.25	
African American	432	4.74	43	3.77	387	4.85	
Hispanic/Asian*	545	5.98	26	2.28	519	6.51	
Unknown/missing*	2,571	28.20	342	29.95	2,264	28.39	
Census region, n							< 0.001
Northeast	1,178	12.92	X ^a	X ^a	X ^a	X ^a	
Midwest	2,421	26.55	345	30.21	2,076	26.03	
South	3,951	43.34	507	44.40	3,444	43.18	
West	1,551	17.01	181	15.85	1,370	17.18	
Unknown/missing	16	0.18	X ^a	X ^a	X ^a	X ^a	
Charlson Comorbidity Score ^b , n							< 0.001
0	2,100	23.03	50	4.38	2,050	25.71	
1	2,863	31.40	545	47.72	2,318	29.07	
2	1,202	13.18	159	13.92	1,043	13.08	
3	841	9.22	134	11.73	707	8.87	
4	536	5.88	X ^a	X ^a	X ^a	X ^a	
≥5	1,554	17.05	155	13.57	1,389	17.42	
Missing	31	0.34	X ^a	X ^a	X ^a	X ^a	
COPD ^b , n							< 0.001
Present	4,963	54.44	1,040	91.07	3,923	49.19	
Moderate/Severe liver disease ^b , n							< 0.001
Present	507	5.56	32	2.80	475	5.96	

Table 1 documents that:

- AATD patients identified in this study are the largest AATD cohort ever studied (9,117).
- About 9% of augmentation users did not have documented COPD.
- Over 5% of AATD patients had moderate/severe liver disease before diagnosis/augmentation therapy treatment.

RESULT 2

Cost drivers for the insurer and the patient were physician visits and prescription drugs.

Table 2. Average Annual Health Plan's and Patient's Out-of-Pocket Cost by Cost Buckets.

Person-years (PY)	FULL COHORT 58,872		AUGMENTATION NON-USERS 50,449				USERS 3,423	
	Mean	SD	Mean ^a	SD	Mean ^a	SD		
Health Plan's Cost Bucket								
Physician Visits	\$5,969	\$19,960	\$5,352	\$18,233	\$15,064	\$35,821		
Emergency Room	\$648	\$4,122	\$619	\$3,966	\$1,072	\$5,949		
Inpatient stays	\$4,912	\$34,092	\$4,506	\$31,827	\$10,902	\$57,668		
Augmentation therapy	\$5,210	\$26,157	\$0	\$0	\$82,002	\$66,873		
Other Rx drugs*	\$2,748	\$9,885	\$2,511	\$9,835	\$6,155	\$9,967		
Other	\$1,613	\$13,075	\$1,197	\$10,599	\$7,741	\$31,548		
Total	\$21,100	\$57,291 ^b	\$14,185	\$45,558 ^b	\$122,936	\$96,036 ^b		
Out-of-pocket Cost Bucket	Mean	SD	Mean ^a	SD	Mean ^a	SD		
Physician Visits	\$731	\$1,362	\$727	\$1,368	\$783	\$1,283		
Emergency Room	\$64	\$286	\$64	\$284	\$56	\$320		
Inpatient stays	\$178	\$886	\$173	\$860	\$257	\$1,210		
Augmentation therapy	\$132	\$941	\$0	\$0	\$2,084	\$3,146		
Other Rx drugs*	\$612	\$928	\$589	\$913	\$940	\$1,066		
Other	\$158	\$873	\$136	\$816	\$481	\$1,452		
Total	\$1,875	\$2,603 ^b	\$1,689	\$2,270 ^b	\$4,601	\$4,541 ^b		

Notes: Data are presented as cost per person-year (SD) in 2017 US Dollars.
*Based on 29,794.8 PY in full cohort; 27,858.8 PY in Non-Users and 1,936.0 PY for Users
a: All cost buckets were statistically different between augmentation users and non-users (p < 0.001)
b: SD for totals were calculated for patients who had both medical and prescription drug insurance

Table 2 documents that:

- The annual cost differed drastically for augmentation users (\$122,936) as compared to non-users (\$14,185).
- The annual out-of-pocket cost was \$4,601 among users versus \$1,689 among non-users.
- All cost buckets were statistically different between augmentation therapy users and non-users (p < 0.001).
- Wide variations in expenditures indicated by large standard deviations relative to means.

AIM 2: TO EXAMINE CHANGES IN COSTS OVER TIME

RESULT 3

Cost buckets increase at fairly consistent rates, even after adjusting for inflation.

Figure 1. Changes in average payer's and patients' out-of-pocket expenditures per person-year and Cost Bucket.

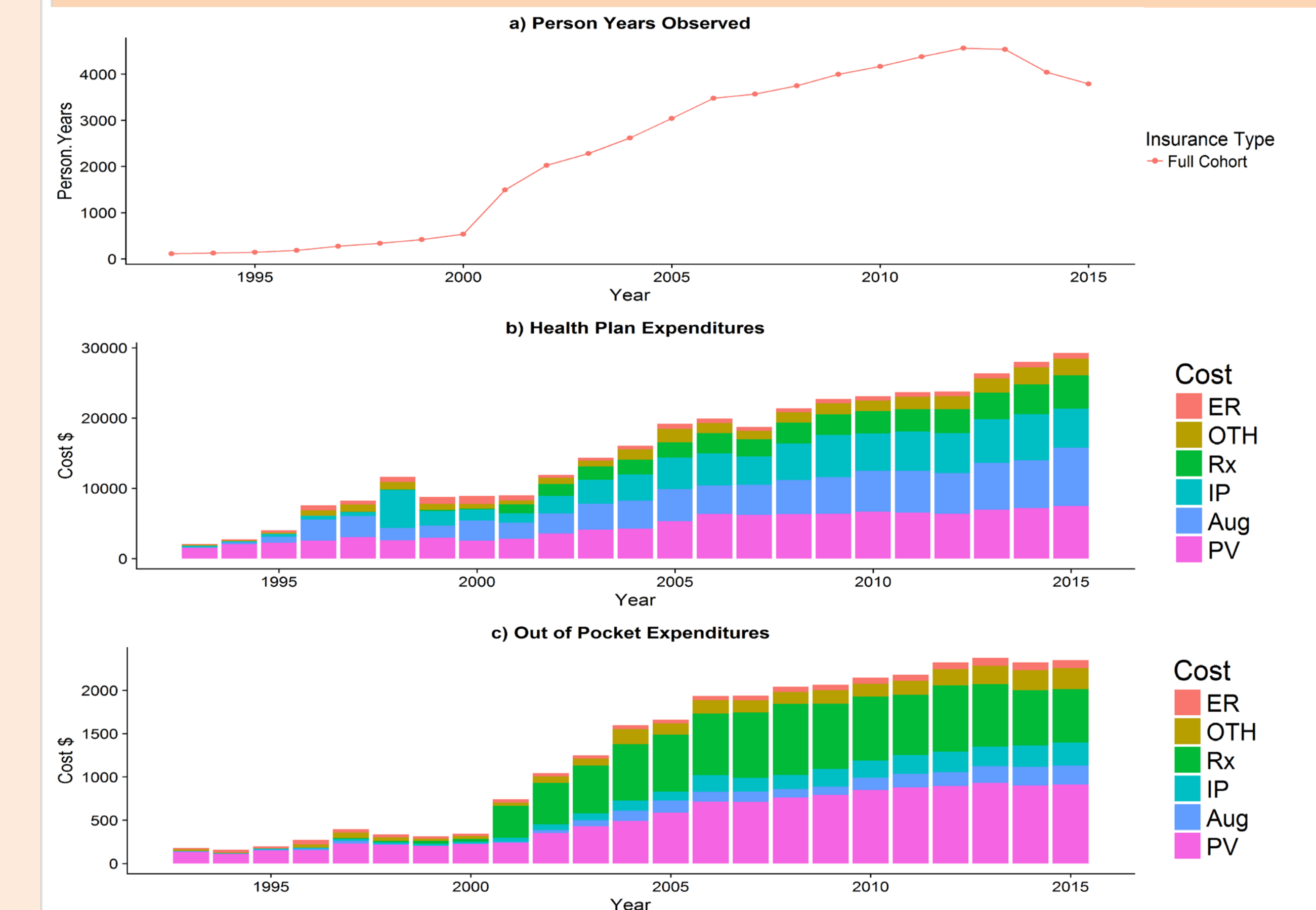


Figure 1 documents that:

- AUG therapy and RX have increased disproportionately to the payer as compared to out-of-pocket costs.
- In 1998 a substantial shortage of augmentation products was noted, possible impacting patients and providers.
- The largest out-of-pocket increase was observed for augmentation therapy.

CONCLUSIONS & CLINICAL IMPLICATIONS

- From the health plan's perspective, the annual costs among AATD patients using augmentation therapy were over 8 times higher as compared to non-users (\$123,000 versus \$14,000, respectively).
- Out-of-pocket expenditures are substantial and were found to be over 270% higher for augmentation users as compared to non-users.
- While further studies are needed to assess the impact of augmentation therapy on health outcomes and cost reductions, AATD patients and insurers may expect to face future increases in their healthcare spending.
- Cost drivers both for the insurer and the patient were physician visits and prescription drugs, which may create barriers to achieving optimal care.

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