Abstract: P1463

Title: HEALTHCARE RESOURCE USE, ECONOMIC BURDEN AND IN-PATIENT MORTALITY IN PATIENTS WITH ALPHA- AND BETA-THALASSEMIA COMPARED TO MATCHED CONTROLS IN THE REAL-WORLD SETTING

Abstract Type: Poster Presentation

Session Title: Thalassemias

Background:

Thalassemia leads to ineffective erythropoiesis, chronic hemolytic anemia (HA), and associated complications. Alpha- (α) and beta (β)- thalassemia result from diminished synthesis of α- or β-globin, respectively. Limited research has been conducted in the US regarding the healthcare resource use (HCRU) and economic burden of α-thalassemia and non–transfusion-dependent thalassemia (NTDT; both α- and β-thalassemia.

Aims:

To evaluate HCRU and costs in patients with thalassemia compared to controls.

Methods:

Patients with ≥1 inpatient (IP) claim or ≥2 claims in any setting with α- or β-thalassemia International Classification of Diseases (ICD)-9/ICD-10 codes from 1/1/2013–6/30/2021 were selected from the MarketScan® Commercial and Medicare and Multi-State Medicaid claims databases in the US. Index date was defined as the first α- or β-thalassemia ICD code. Adults (≥18 years) with at least 12 months of follow-up from index date to end of enrollment, IP death, or study end were included. Mortality data were reported with variable length follow-up while HCRU and costs were assessed 12 months post-index. Patients were stratified by α- and β-thalassemia and by transfusion-dependent thalassemia (TDT) and NTDT. TDT was defined as ≥8 transfusions within first 12 months post-index, each within 42 days of each other. Controls with no history of thalassemia or other HAs were matched 5:1 to thalassemia cases on age, sex, payer, follow-up time, and race (Medicaid only). Chi-square (categorical variables) and t-tests (continuous variables) were used for outcome comparisons with two-sided significance level of 0.05.

Results:

In the Commercial/Medicare data, 4,183 patients with thalassemia (1,675 [40.0%] α-thalassemia and 2,508 [60%] β-thalassemia) and 20,915 matched controls were analyzed. In the 12 months post-index, 2 (0.1%) patients with α-thalassemia had TDT (1,673 [99.9%] NTDT) and 84 (3.3%) with β-thalassemia had TDT (2,424 [96.7%] NTDT). Mean (SD) age was 46.1 (14.8) years, and 29.7% were male. Compared to matched controls, a significantly higher proportion of patients with α-thalassemia had ≥1 IP admission (19.8% vs 5.6%; p<0.001), emergency room (ER) visit (27.9% vs 17.1%; p<0.001), or outpatient physician visit (99.5% vs 85.7%; p<0.001), and significantly higher mean (SD) total healthcare costs ($21,710 [$55,981] vs $8,641 [$26,570]; p<0.001). Similarly, a significantly higher proportion of patients with NTDT had ≥1 IP admission (20.0% vs 5.7%; p<0.001), ER visit (28.2% vs 17.6%; p<0.001), or outpatient physician visits (99.5% vs 84.4%; p <0.001), and significantly higher total healthcare costs ($21,989 ($53,588) vs $8,686 ($28,949); p<0.001) than matched controls. Similar patterns were seen for TDT and β-thalassemia (Table 1). Eight (0.5%) all-cause IP deaths occurred in patients with α-thalassemia (over mean [SD] follow up of 1,132 [615] days), 7 (0.3%) in patients with β-thalassemia (1,080 [590] days), 13 (0.3%) in patients with NTDT (1,101 [599] days), 2 (2.6%) in patients with TDT (1,091 [690] days) compared to no deaths in any control group (all p<0.05). Medicaid data showed mostly similar trends across outcomes (data not shown).

Summary/Conclusion: Patients with thalassemia, including those with α-thalassemia and NTDT, had significantly higher HCRU, total costs, and higher IP mortality rates than matched controls. This study may underestimate the burden of NTDT since some patients with thalassemia trait/minor may have been included in the NTDT due to

coding errors. Additional therapies are needed to address the underlying pathophysiology of thalassemia to prevent serious complications and reduce HCRU.

Table 1: Healthcare Resource Use and Costs in Commercial/Medicare Database (12-month follow-up)

	α-Thal	α-Thal Control Cohort	β-Thal	β-Thal Control Cohort	NTDT (Any)	NTDT Control Cohort	TDT (Any)	TDT Control Cohort
	N = 1,675	N = 8,375	N = 2,508	N=12,540	N = 4,097	N= 20,485	N = 86	N = 430
	N/Mean (%/SD	N/Mean (%/SD)	N/Mean %/SD	N/Mean %/SD	N/Mean %/SD	N/Mean %/SD	N/Mean %/SD	N/Mean %/SD
Patients with ≥1 inpatient admission (N, %)	**331 (19.8%)***	470 (5.6%)	**501 (20.0%)***	737 (5.9%)	**819 (20.0%)***	1,174 (5.7%)	**13 (15.1%)***	33 (7.7%)
Number of inpatient admissions** (Mean, SD)	1.3 (0.6)^{NS}	1.2 (0.6)	**1.4 (1.0)***	1.2 (0.6)	**1.3 (0.8)***	1.2 (0.6)	1.6 (1.0)^{NS}	1.4 (1.3)
Patients with any outpatient service (N, %)	**1,675 (100.0%)***	7,497 (89.5%)	**2,507 (100.0%)***	11,018 (87.9%)	**4,096 (100.0%)***	18,161 (88.7%)	**86 (100.0%)***	354 (82.3%)
Patients with an ER visit (N, %)	**467 (27.9%)***	1,435 (17.1%)	715 (28.5%)^{NS}	3,674 (29.3%)	**1,157 (28.2%)***	3,603 (17.6%)	**25 (29.1%)***	71 (16.5%)
Patients with an outpatient office visit (N, %)	**1,667 (99.5%)***	7,176 (85.7%)	**2,494 (99.4%)***	10,444 (83.3%)	**4,076 (99.5%)***	17,298 (84.4%)	**85 (98.8%)***	322 (74.9%)
Number of outpatient office visits**, (Mean, SD)	**9.2 (8.0)***	5.7 (5.5)	**9.7 (8.7)***	5.9 (5.7)	**9.4 (8.3)***	5.8 (5.6)	**17.0 (13.1)***	5.2 (6.0)
Outpatient pharmacy								
Patients with any prescription (N, %)	**1,520 (90.7%)***	6,611 (78.9%)	**2,293 (91.4%)***	9,795 (78.1%)	**3,730 (91.0%)***	16,108 (78.6%)	**83 (96.5%)***	298 (69.3%)
Number of prescriptions** (Mean, SD)	**20.6 (22.1)***	18.7 (20.9)	**21.2 (22.4)***	18.6 (21.8)	**20.9 (22.2)***	18.7 (21.5)	**24.2 (25.0)***	14.0 (15.4)
Patients with an iron chelator prescription (N, %)	**17 (1.0%)***	0 (0.0%)	**105 (4.2%)***	0 (0.0%)	**58 (1.4%)***	0 (0.0%)	**64 (74.4%)***	0 (0.0%)
Total costs (Inpatient + Outpatient + Outpatient pharmacy)								
Total costs (Mean, SD), PPPY	**$21,710 ($55,981)***	$8,641 ($26,570)	**$26,918 ($59,471)***	$8,708 ($30,763)	**$21,989 ($53,588)***	$8,686 ($28,949)	**$160,285 ($94,961)***	$8,443 ($37,767)

α-Thal: alpha-thalassemia; β-Thal: beta-thalassemia; NTDT: non-transfusion dependent; PPPY: per patient per year.
TDT: transfusion dependent; *Indicates statistically significant results ($p<0.05$); NS- indicates statistically non-significant results.
** Among patients with event (visit/admission/prescription, etc.)

Keywords: beta thalassemia, Real world data, Cost analysis, Thalassemia