225

Treatment Patterns, Healthcare Resource Utilization, and Costs in Adolescent and Young Adult Patients With Acute Lymphoblastic Leukemia

Amy Nguyen,[1] Eric Burgess,[1,*] Arthur Sillah,[1] Yong Zhu,[2] Lisa Le,[2] Kristin J. Moore,[1] Melisa Stricherz[1]

[1]Jazz Pharmaceuticals plc, Dublin, Ireland; [2]Optum Life Sciences, Eden Prairie, MN, USA

*Presenting author.

Background

- Acute lymphoblastic leukemia/lymphoblastic lymphoma (ALL/LBL), clonal malignancies of hematopoietic stem cells, are prevalent pediatric cancers representing up to one-third of all pediatric malignancies[1]
- The treatment landscape for ALL has evolved significantly over the past decade[2,3] with protocol updates and newer therapies[3]
- While treatment advances have significantly improved outcomes for pediatric ALL/LBL patients,[4,5] adolescent and young adult ([AYA] within age range of 15-39 years old[6]) and adult populations have not experienced the same degree of improvement[7]
- Survival rates in ALL/LBL decline with age, particularly around the transition to adult treatment settings, suggesting that treatment approaches (pediatric vs adult regimens) significantly impact outcomes,[8] with AYA patients responding better to pediatric-inspired regimens (PIRs) than with traditional adult protocols[9-13]
- Asparaginase-containing PIRs have shown improved survival and decreased relapse rates over conventional cytotoxic regimens such as hyper-fractionated cyclophosphamide, vincristine, doxorubicin, and dexamethasone (hyper-CVAD)[9,12,13]
- Despite improved outcomes and guideline recommendations, asparaginase-containing PIRs are not widely adopted[6,14,15]

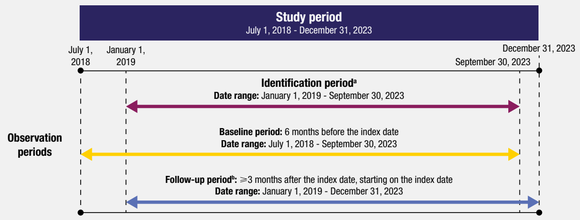
Objective

- To examine treatment patterns, healthcare resource utilization (HCRU), and costs for AYA patients with de novo or relapsed ALL in the United States stratified by treatment type

Methods

- This retrospective descriptive analysis used the deidentified Optum Market Clarity claims and electronic heath records database from July 1, 2018, to December 31, 2023 as the study period for this analysis (**Figure 1**)
- The index date (ID) was defined as the earliest non-diagnostic medical claim for ALL between January 1, 2019, to September 30, 2023
- Patients were followed from the ID until earliest of disenrollment/end of study (minimum 3 months) or death (whichever was earliest)
- Eligible patients with de novo ALL or with relapsed ALL aged 15-39 years had ⩾2 non-diagnostic claims for ALL on separate days between January 1, 2019, and September 30, 2023
  - Patients initiated treatment within 60 days of ID, had continuous enrollment during a 6-month baseline (prior to ID) and during the follow-up period, had no history of oncology medication or ALL diagnosis prior to ID (for those with de novo ALL), and had no evidence of another primary cancer or pregnancy during the baseline and follow-up periods
  - Patients with relapsed ALL could not have prior claims for relapsed ALL during the 6-month baseline period, however this criterion did not apply to the de novo ALL group
- Patients were grouped by diagnosis (de novo ALL and relapsed ALL) and subgroups were based on treatment: asparaginase-containing regimens, hyper-CVAD, nelarabine-containing regimens, additional regimens without PIR (reduced regimen), or other regimens
- Patient data, including patient characteristics, time to diagnosis and duration of treatment, and HCRU and costs were descriptively analyzed and reported

Figure 1: Study Period (Identification, Baseline, and Follow-Up Periods)



[a]Patient index date was during the identification period; [b]Treatment and outcomes were assessed during the follow-up period.

Results

- Between January 1, 2019, and September 30, 2023, records for 19,650 patients diagnosed with de novo ALL and 2288 patients with relapsed ALL were identified
- After applying exclusion criteria, 157 AYA patients with de novo ALL and 34 patients with relapsed ALL were included in this analysis
  - Of patients with de novo ALL, 43 received asparaginase-containing regimens, 29 received hyper-CVAD, 8 received nelarabine-containing regimens, 55 received reduced regimens, and 22 received other regimens
  - Patient characteristics at diagnosis of de novo ALL were generally consistent across treatment groups (except for age and sex of hyper-CVAD–treated patients) with an overall median age (interquartile range [IQR]) of 19 (16, 27) years and 66% male sex (**Table 1**)
    - The hyper-CVAD group had a younger median age (IQR) of 16 (15, 22) years, and a higher proportion of female patients (48%)

Table 1. Patient Baseline Characteristics

	Overall de novo ALL Cohort (n=157)[a]	PIR	Non-PIRs			Overall Relapsed Cohort (n=34)
		Asparaginase (n=43)	Hyper-CVAD (n=29)	Nelarabine (n=8)	Reduced Regimen (n=55)	
Age on ID, median (IQR), years	19 (16, 27)	19 (17, 27)	16 (15, 22)	20 (16, 30)	19 (16, 26)	22 (18, 32)
Sex, n (%)						
Female	53 (34)	12 (28)	14 (48)	<5	16 (29)	15 (44)
Male	104 (66)	32 (74)	15 (52)	>5	39 (71)	19 (56)
Race, n (%)						
White	99 (63)	24 (56)	15 (52)	7 (88)	35 (64)	23 (68)
Black or African American	12 (8)	<5	<5	<5	7 (13)	<5
Hispanic	10 (6)	<5	<5	<5	5 (9)	<5
Other/unknown/missing	36 (23)	15 (35)	8 (28)	<5	8 (15)	8 (24)
US Region, n (%)						
Northeast	30 (19)	⩾5	9 (31)	<5	11 (20)	5 (15)
Midwest	69 (44)	17 (40)	12 (41)	5 (63)	24 (44)	17 (50)
South	25 (16)	6 (14)	<5	<5	10 (18)	<5
West	27 (17)	14 (33)	<5	<5	>5	<5
Other/unknown	6 (4)	<5	<5	<5	<5	<5
Insurance status, n (%)						
Commercial	85 (54)	24 (56)	13 (45)	7 (88)	30 (55)	21 (62)
Medicare	7 (4)	<5	<5	<5	<5	<5
Medicaid	65 (41)	>5	>5	<5	>5	>5
Baseline Charlson Comorbidity Index						
Median (IQR)	0 (0, 2)	0 (0, 2)	0 (0, 2)	0 (0, 2)	0 (0, 2)	2 (2, 2)

[a]In the overall de novo ALL cohort, 22 patients received other regimens (information not shown).

ALL, acute lymphoblastic leukemia; hyper-CVAD, hyper-fractionated cyclophosphamide, vincristine, doxorubicin, and dexamethasone; ID, index date; IQR, interquartile range; n, number of patients; PIR, pediatric-inspired regimen; US, United States.

- Median (IQR) time from diagnosis to treatment was 22 (10, 34) days in overall patients with de novo ALL and 29 (15, 40), 17 (13, 24), 15 (11, 26), and 17 (9, 34) days with asparaginase-containing regimens, hyper-CVAD, nelarabine-containing regimens, and reduced regimens, respectively (**Table 2**)
- Median (IQR) time on treatment was 12 (5, 24) months overall and similar across treatments for de novo ALL

Table 2. Time From Diagnosis to Treatment Initiation and Time on Treatment

	Overall de novo ALL Cohort (n=157)[a]	PIR	Non-PIRs			Overall Relapsed Cohort (n=34)
		Asparaginase (n=43)	Hyper-CVAD (n=29)	Nelarabine (n=8)	Reduced Regimen (n=55)	
Time from diagnosis to treatment initiation						
Median (IQR), days	22 (10, 34)	29 (15, 40)	17 (13, 24)	15 (11, 26)	17 (9, 34)	16 (7, 26)
Time on treatment[b]						
Median (IQR), months	12 (5, 24)	14 (7, 27)	12 (7, 25)	10 (5, 22)	12 (3, 22)	11 (6, 19)

[a]In the overall de novo ALL cohort, 22 patients received other regimens (information not shown); [b]Time between start date of first line of therapy to end of treatment/data capture.

ALL, acute lymphoblastic leukemia; hyper-CVAD, hyper-fractionated cyclophosphamide, vincristine, doxorubicin, and dexamethasone; IQR, interquartile range; n, number of patients; PIR, pediatric-inspired regimen.

- Overall, most patients with de novo ALL had ambulatory visits (90% office visits and 98% outpatient visits) and most patients were admitted to the emergency department (93%) and/or had ⩾1 inpatient stay during follow-up (**Table 3**)
- Per-patient per-month (PPPM) number of visits (mean [standard deviation; SD]) were generally similar across treatments, with the mean (SD) PPPM ambulatory visits being 6 (4) in patients with de novo ALL
- Asparaginase-treated patients had fewer PPPM inpatient days (3 [3]) compared with the other 3 treatment groups (hyper-CVAD, 4 [4]; nelarabine, 4 [4]; reduced regimens, 5 [7])

Table 3. All-Cause Healthcare Resource Utilization During Follow-Up

	Overall de novo ALL Cohort (n=157)[a]	PIR	Non-PIRs		
		Asparaginase (n=43)	Hyper-CVAD (n=29)	Nelarabine (n=8)	Reduced Regimen (n=55)
Prevalence, n (%)					
Ambulatory visits	156 (99)	43 (100)	29 (100)	8 (100)	54 (98)
Office visits	141 (90)	39 (91)	26 (90)	8 (100)	50 (91)
Outpatient visits	154 (98)	43 (100)	29 (100)	8 (100)	53 (96)
Emergency department visits	146 (93)	42 (98)	29 (100)	6 (75)	49 (89)
Inpatient stays	145 (92)	42 (98)	28 (97)	8 (100)	47 (85)
Pharmacy fills	150 (96)	41 (95)	29 (100)	7 (88)	53 (96)
Mean per-patient per-month counts (SD)					
Ambulatory visits	6 (4)	6 (3)	6 (2)	8 (5)	6 (4)
Office visits	2 (2)	1 (1)	2 (2)	3 (3)	2 (2)
Outpatient visits	4 (3)	5 (3)	4 (2)	6 (4)	4 (3)
Emergency department visits	0.3 (0.3)	0.3 (0.2)	0.3 (0.2)	0.4 (0.5)	0.3 (0.3)
Inpatient stays	0.4 (0.3)	0.3 (0.2)	0.4 (0.3)	0.3 (0.2)	0.4 (0.4)
Inpatient days	4 (5)	3 (3)	4 (4)	4 (4)	5 (7)
Pharmacy fills	5 (3)	5 (3)	6 (2)	5 (4)	5 (3)

[a]In the overall de novo ALL cohort, 22 patients received other regimens (information not shown).

ALL, acute lymphoblastic leukemia; hyper-CVAD, hyper-fractionated cyclophosphamide, vincristine, doxorubicin, and dexamethasone; n, number of patients; PIR, pediatric-inspired regimen; SD, standard deviation.

- PPPM all-cause total costs (mean [SD]) were lowest with asparaginase-containing regimens ($44,381 [$28,456]) and highest with nelarabine-containing regimens ($86,270 [$67,338]) as shown in **Table 4**
- PPPM pharmacy costs (mean [SD]) were higher with asparaginase-containing regimens ($1940 [$5073]) and nelarabine-containing regimens ($1928 [$4141]) compared with hyper-CVAD ($1506 [$2623]) and reduced regimens ($1426 [$2824])

Table 4. All-Cause Healthcare Costs During Follow-Up

	Overall de novo ALL Cohort (n=157)[a]	PIR	Non-PIRs			Overall Relapsed Cohort (n=34)
		Asparaginase (n=43)	Hyper-CVAD (n=29)	Nelarabine (n=8)	Reduced Regimen (n=55)	
Mean per-patient per-month costs in follow-up (SD)						
Total costs (medical + pharmacy)	$50,213 ($39,850)	$44,381 ($28,456)	$47,233 ($27,288)	$86,270 ($67,338)	$50,896 ($44,510)	$67,036 ($66,397)
Medical costs	$48,130 ($38,878)	$42,441 ($28,226)	$45,727 ($27,154)	$84,341 ($65,394)	$49,470 ($44,345)	$59,447 ($58,732)
Ambulatory	$5233 ($17,127)	$3344 ($7304)	$4584 ($8460)	$35,983 ($65,210)	$3706 ($7897)	$7895 ($21,131)
Office visits	$687 ($2293)	$403 ($1002)	$1197 ($4606)	$553 ($591)	$704 ($1518)	$2179 ($5776)
Outpatient visits	$4546 ($16,984)	$2941 ($7160)	$3388 ($7213)	$35,430 ($65,194)	$3002 ($7756)	$5716 ($20,664)
Emergency department visits	$354 ($928)	$247 ($241)	$466 ($1281)	$314 ($321)	$427 ($1221)	$268 ($451)
Inpatient stays	$27,877 ($32,007)	$20,701 ($21,126)	$24,488 ($20,068)	$25,275 ($24,324)	$33,602 ($41,580)	$30,231 ($30,035)
Other medical costs	$14,666 ($14,228)	$18,149 ($15,249)	$16,189 ($11,521)	$22,769 ($21,238)	$11,735 ($11,468)	$21,052 ($39,663)
Pharmacy costs	$2083 ($4267)	$1940 ($5073)	$1506 ($2623)	$1928 ($4141)	$1426 ($2824)	$7589 ($15,869)

[a]In the overall de novo ALL cohort, 22 patients received other regimens (information not shown).

ALL, acute lymphoblastic leukemia; hyper-CVAD, hyper-fractionated cyclophosphamide, vincristine, doxorubicin, and dexamethasone; n, number of patients; PIR, pediatric-inspired regimen; SD, standard deviation.

Conclusions

- In this descriptive analysis of real-world data, we observed <30% of patients receiving asparaginase-containing regimens, despite guideline recommendations and published studies demonstrating survival in AYA
- Although the mean PPPM number of ambulatory and emergency department visits and the mean PPPM inpatient stays were similar between asparaginase-treated patients and hyper-CVAD–treated patients, the mean number of inpatient days in the hospital was fewer among asparaginase-treated patients
- This analysis also suggests that asparaginase-treated patients have lower all-cause treatment costs compared to other treatments
- These findings support the adoption of asparaginase-containing PIRs in AYA patients, suggesting benefits beyond improved survival outcomes. Specifically, total all-cause mean costs were lower among asparaginase-treated patients during the full follow-up, and there was no increase in all-cause HCRU

References: 1. Kaseb H, et al. Lymphoblastic Lymphoma, In: StatPearls [Internet]. 2023. Accessed July 7, 2025. Available at: https://www.ncbi.nlm.nih.gov/books/NBK537237/. **2.** Su W, et al. *Am J Hematol.* 2025;100(5):847-859. **3.** Jabbour E, et al. *J Hematol Oncol.* 2023;16(1):22. **4.** Raetz EA, et al. *Pediatr Blood Cancer.* 2023;70(suppl 6):e30585. **5.** Pui C-H, et al. *N Engl J Med.* 2009;360(26)2730-2741. **6.** Reference with permission from the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Acute Lymphoblastic Leukemia V.2.2025. © National Comprehensive Cancer Network, Inc. 2025. All rights reserved. Accessed October 3, 2025. To view the most recent and complete version of the guideline, go online to NCCN.org. NCCN makes no warranties of any kind whatsoever regarding their content, use or application and disclaims any responsibility for their application or use in any way. **7.** Terwilliger T and Abdul-Hay M. *Blood Cancer J.* 2017;7(6):e577. **8.** Stock W, et al. *Leuk Lymphoma.* 2011;52(12):2237-2253. **9.** Stock W, et al. *Blood.* 2008;112(5):1646-1654. **10.** Muffly L and Curran E. *Hematology Am Soc Hematol Educ Program.* 2019;2019(1):17-23. **11.** Siegel SE, et al. *JAMA Oncol.* 2018;4(5):725-734. **12.** Huguet F, et al. *J Clin Oncol.* 2009;27(6):911-918. **13.** DeAngelo DJ, et al. *Leukemia.* 2015;29(3):526-534. **14.** Bhagnani T, et al. *Blood.* 2023;142(1):3704-3705. **15.** Muffly LS, et al. *JCO Oncol Pract.* 2023;19(12):1190-1198.

Support and Acknowledgments: This study was supported by Jazz Pharmaceuticals. Medical writing support, under the direction of the authors, was provided by Heyam Hayder, PhD, of CMC Connect, a division of IPG Health Medical Communications, with funding from Jazz Pharmaceuticals, in accordance with Good Publication Practice (GPP 2022) guidelines.

Disclosures: A Nguyen, **E Burgess**, **A Sillah**, **K Moore**, and **M Stricherz** are employees of and hold stock ownership/options in Jazz Pharmaceuticals. **Y Zhu** and **L Le** are employees of Optum Life Sciences, a company that received funding from Jazz Pharmaceuticals for the current study. **Y Zhu** holds stock ownership with UnitedHealth Group, the parent company of Optum Life Sciences.

Poster presented at the Academy of Managed Care Pharmacy (AMCP) Nexus; October 27-30, 2025; National Harbor, MD, USA.