RESEARCH LETTER

blood neoplasia

TO THE EDITOR:

Somatic *UBA1* mutations in pediatric MDS/AML: first cases suggest distinct pathogenesis from adult VEXAS syndrome

Lili Kotmayer,[1] Ninad Oak,[2] Michael Halyko,[3] Rajat Sharma,[3] Sinisa Savic,[4] Fernanda Gutierrez-Rodrigues,[5] Bhavisha A. Patel,[5] David B. Beck,[6,7] Raul C. Ribeiro,[2] Kim E. Nichols,[2] Geoff Shenton,[8] Anjali Sharathkumar,[3] and Marcin W. Wlodarski[1]

[1]Department of Experimental Hematology, and [2]Department of Oncology, St. Jude Children's Research Hospital, Memphis, TN; [3]Stead Family Department of Pediatrics, Carver College of Medicine, University of Iowa, Iowa City, IA; [4]Leeds Institute of Rheumatic and Musculoskeletal Medicine, University of Leeds, Leeds, United Kingdom; [5]Hematology Branch, National Heart, Lung, and Blood Institute, National Institutes of Health, Bethesda, MD; [6]Center for Human Genetics and Genomics, and [7]Division of Rheumatology, Department of Medicine, New York University School of Medicine, New York, NY; and [8]Department of Children's Oncology, Great North Children's Hospital, Newcastle, United Kingdom

VEXAS (vacuoles, E1 enzyme, X linked, autoinflammatory, somatic) syndrome[1] is a severe autoinflammatory disorder driven by somatic *UBA1* mutations in hematopoietic progenitor cells, typically affecting males aged >50 years (median age, 66-72.1 years).[2-6] VEXAS syndrome has not been reported in children; the youngest documented patient was 23 years old.[7] The disease presents with systemic inflammation, dysfunctional immune responses, venous thromboembolism, macrocytic anemia, bone marrow (BM) failure, and, typically, low-blast-count myelodysplastic syndromes (MDS).[8-12] High-risk MDS and acute myeloid leukemia (AML) are rare (<10%).[13] Here, we report the first 2 pediatric patients with myeloid neoplasms and VEXAS syndrome–defining *UBA1* mutations.

Patient 1 (P1), a 5.5-year-old boy with an unremarkable medical history, presented with pancytopenia (hemoglobin level, 4.7 g/dL; platelet count, 54 × 10^3/μL; white blood cell count, 3.2 × 10^3/μL; absolute neutrophil count, 450/μL; Figure 1A). Initial BM biopsy showed mild dysmegakaryopoiesis without blast excess and a normal male karyotype. Clinical diagnostic sequencing revealed *NRAS*_G13D (variant allele frequency [VAF], 12.8%) and *UBA1*_S56F (VAF, 17.9%) mutations, both confirmed as somatic (supplemental Figure 1). The patient progressed rapidly with worsening pancytopenia, fever, and splenomegaly, and developed a vasculitic rash on his face, arms, and back (Figure 1B). Skin biopsy revealed neutrophilic inflammation and leukocytoclastic vasculitis (Figure 1B). Repeat BM biopsy showed MDS with increased blasts. Despite absent cytoplasmic vacuoles in precursor cells, the copresentation of MDS with a somatic *UBA1* mutation and a characteristic rash suggested a VEXAS syndrome diagnosis (Figure 1C). He received prophylactic triple intrathecal chemotherapy (central nervous system negative). With no response to intravenous immunoglobulin/steroids and rapid disease evolution, P1 received 2 cycles of liposomal cytarabine/daunorubicin (CPX-351) according to the CPX-351 treatment protocol (ClinicalTrials.gov identifier: NCT05656248). CPX-351, previously shown to be effective and well-tolerated in pediatric secondary MDS/AML,[14] induced disease remission (by morphology and flow cytometry) with undetectable *NRAS* and *UBA1* mutations (VAFs <1%) and led to the resolution of the skin rash. After myeloablative conditioning (busulfan/fludarabine/thiotepa), he received a BM graft from an HLA-matched unrelated donor (3.54 × 10^8 total nucleated cells per kg) and achieved full donor myeloid chimerism at day +60. He remains in remission 1-year after transplantation.

P2, a 5-year-old boy, presented with a 1-week history of bleeding symptoms, anemia (hemoglobin level, 8.9 g/dL), and severe thrombocytopenia (platelet count, 5 ×10^3/μL) (Figure 1D). Besides bilaterally enlarged, ulcerated tonsils, no systemic features (including autoinflammation) were identified. Family history was unremarkable. BM aspirate showed ~50% abnormal myeloid blasts with vacuoles. Additional testing identified a complex karyotype with del (5q), trisomy 8 and trisomy

Submitted 9 February 2026; accepted 20 March 2026; prepublished online 2 April 2026. https://doi.org/10.1016/j.bneo.2026.100228.

Raw sequencing data generated in this study have been deposited in the European Genome-phenome Archive under accession number EGAD50000002372.

The full-text version of this article contains a data supplement.

Licensed under Creative Commons Attribution-NonCommercial-NoDerivatives 4.0 International (CC BY-NC-ND 4.0), permitting only noncommercial, nonderivative use with attribution. All other rights reserved.

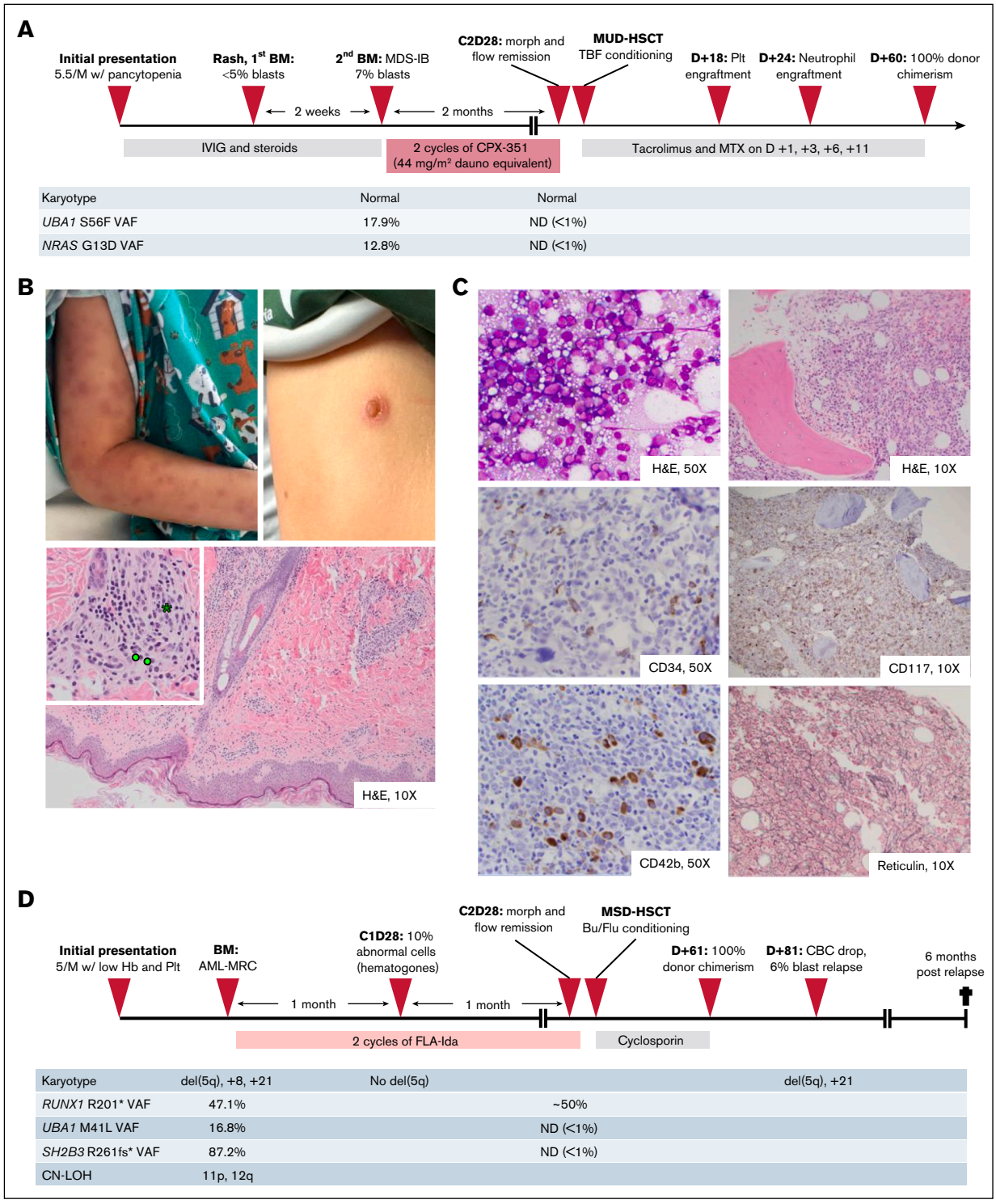
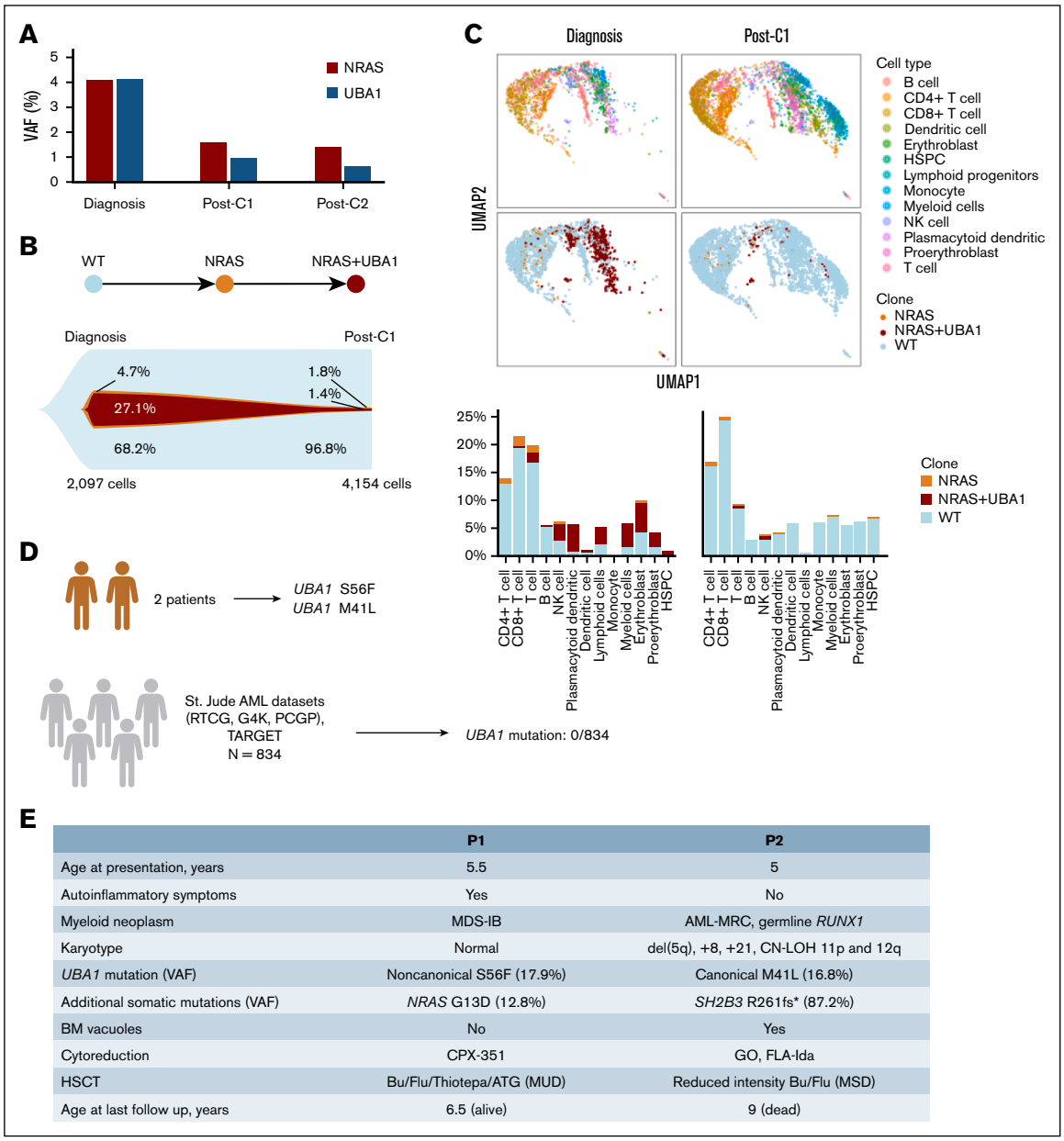


Figure 1. Clinical characteristics of pediatric patients with *UBA1*-mutated myeloid neoplasms. (A) Clinical timeline of P1. (B) Cutaneous manifestations in P1. Top: rash involving the arm (left) and bullous lesion on the upper arm (right). Bottom: skin biopsy of a bullous lesion, hematoxylin-eosin staining (H&E) (original magnification ×10), showing superficial to deep perivascular and periadnexal chronic inflammation with inset (original magnification ×50) showing scattered neutrophils (•) and karyorrhectic debris (*). (C) BM findings in P1. Top: BM touch imprint (H&E, original magnification ×50) and biopsy (H&E, original magnification ×10) showing normocellular BM with left-shifted myeloid maturation with increased blasts. Middle: CD34 (left, original magnification ×50) and CD117 (right, original magnification ×10) immunohistochemistry of the BM, highlighting increased (5%-10%) blasts. Bottom left: CD42b immunohistochemistry (original magnification ×50) demonstrating dysmegakaryopoiesis with hypolobated megakaryocytes. Bottom right: reticulin staining (original magnification ×10) showing moderate fibrosis (grade 2). (D) Clinical timeline of P2. AML-MRC, acute myeloid leukemia with myelodysplasia-related changes; Bu/Flu, busulfan/fludarabine; CBC, complete blood count; CN-LOH, copy-neutral loss of heterozygosity; FLA-Ida, fludarabine/cytarabine/idarubicin; Hb, hemoglobin; IVIG, IV immunoglobulin; M, male; MDS-IB, myelodysplastic syndrome with increased blasts; MTX, methotrexate; MUD-HSCT, matched unrelated donor hematopoietic stem cell transplantation; MSD, matched sibling donor; ND, not detected; Plt, platelet; TBF, thiotepa/busulfan/fludarabine.



E

	P1	P2
Age at presentation, years	5.5	5
Autoinflammatory symptoms	Yes	No
Myeloid neoplasm	MDS-IB	AML-MRC, germline *RUNX1*
Karyotype	Normal	del(5q), +8, +21, CN-LOH 11p and 12q
UBA1 mutation (VAF)	Noncanonical S56F (17.9%)	Canonical M41L (16.8%)
Additional somatic mutations (VAF)	*NRAS* G13D (12.8%)	*SH2B3* R261fs* (87.2%)
BM vacuoles	No	Yes
Cytoreduction	CPX-351	GO, FLA-Ida
HSCT	Bu/Flu/Thiotepa/ATG (MUD)	Reduced intensity Bu/Flu (MSD)
Age at last follow up, years	6.5 (alive)	9 (dead)

Figure 2. Molecular characterization of patient 1. (A) Variant allele frequencies (VAFs) of *NRAS* G13D and *UBA1* S56F from deep sequencing at diagnosis and post-C1 and post-C2. (B) Clonal architecture by single-cell DNA sequencing (scDNA-seq). Top: linear evolution inferred from scDNA-seq; Bottom: fish plot depicting trajectories of mutant and WT cells over time. (C) Integrated genotype and cell surface protein analysis from multiomic scDNA-seq data. Top: uniform manifold approximation and projection (UMAP) analysis based on protein expression clustering showing cell type clustering. Middle: UMAP analysis showing clustering of different clones. Bottom: clone distribution across cell types. (D) Results from *UBA1* mutation screening in 834 childhood AML cases. (E) Summary of clinical and molecular findings. AML-MRC, acute myeloid leukemia with myelodysplasia-related changes; ATG, anti-thymocyte globulin; Bu/Flu, busulfan/fludarabine; CN-LOH, copy-neutral loss of heterozygosity; FLA-Ida, fludarabine/cytarabine/idarubicin; GO, gemtuzumab ozogamicin; HSCT, hematopoietic stem cell transplantation; MDS-IB, myelodysplastic syndrome with increased blasts; MSD, matched sibling donor; MUD, matched unrelated donor; WT, wild type.

21, copy-neutral loss of heterozygosity on 11p and 12q, and pathogenic mutations in *RUNX1*_R201* (VAF, 47.1%), *SH2B3*_R261fs* (VAF, 87.2%), and *UBA1*_M41L (VAF, 16.8%). These findings were consistent with AML with MDS-related changes. The patient received gemtuzumab ozogamicin induction according to the MyeChild01 trial (ClinicalTrials.gov identifier: NCT02724163), but administration was halted when he developed anaphylaxis. Given the high-risk features, he next received 2 cycles of fludarabine/cytarabine/idarubicin, achieving remission by morphology/flow cytometry. *SH2B3* and *UBA1* mutations became undetectable, whereas the *RUNX1* mutation persisted at the heterozygous state, consistent with a germ line origin. After reduced-intensity busulfan/fludarabine conditioning (ClinicalTrials.gov identifier: NCT02724163), he received a BM graft from an HLA-identical matched sibling and achieved prompt engraftment and full donor chimerism. Relapse with 6%

blasts and cytopenia occurred on day +81. Given the adverse prognosis of early relapse, a second transplant was not pursued, and the patient died 6 months after relapse.

P1 harbored the noncanonical *UBA1*_S56F mutation, which reduces global polyubiquitylation by impairing catalytic activity.[15,16] Consistent with his presentation, this mutation is associated with absent BM vacuolization and predominantly hematologic manifestations, with reduced inflammatory features.[17,18] P2's *UBA1*_M41L is a canonical substitution found in ~18% to 20% of VEXAS syndrome cases,[1,6] resulting in a selective loss of the cytoplasmic UBA1b isoform.[6] Among M41 substitutions, M41L is associated with overall milder phenotypes.[6,19,20] M41L has also been identified in reportedly healthy individuals from the National Institutes of Health (NIH) All of Us cohort, albeit with lower VAFs.[21]

Our patients had additional somatic mutations in *NRAS*(P1) and *SH2B3*(P2). Concomitant mutations occur in 27% to 74% of adult VEXAS syndrome,[13,20,22-24] with *DNMT3A* and *TET2* being most common, whereas RAS pathway mutations (*NRAS/KRAS/CBL*) were found only in ~6.25% of cases.[20] NRAS/UBA1 co-occurrence was observed in 3 patients in the NIH VEXAS syndrome cohort.[20] An unbiased screen of 2427 individuals with myeloid malignancy also identified *NRAS/UBA1* in 3 patients without a previous VEXAS syndrome diagnosis.[25] *SH2B3* mutations have not yet been reported in conjunction with *UBA1*.

Notably, both our patients exhibited lower VAFs for *UBA1* than for their co-occurring mutations. A review of published VEXAS syndrome cases showed that 23 patients had lower *UBA1* VAFs relative to co-occurring mutations (across 17 genes). In the NIH cohort, high-VAF *TET2* and *DNMT3A* mutations were identified as ancestral events, with UBA1 as the secondary hit in 2 adults.[20] To test whether *UBA1* similarly arose as a secondary event in P1, we first performed deep sequencing and droplet digital polymerase chain reaction on the BM from P1 at diagnosis and after CPX-351 cycles 1 (post-C1) and 2 (post-C2). At diagnosis, *UBA1* and *NRAS* showed comparable VAFs (4.1% and 4.0%, respectively), decreasing after therapy (post-C1, 0.97% and 1.6%; post-C2, 0.62% and 1.4%, respectively) (Figure 2A), as confirmed by droplet digital polymerase chain reaction (supplemental Figure 2). The greater reduction of *UBA1* VAF challenged its role as the primary driver and prompted us to interrogate the mutation origin. Single-cell DNA sequencing of diagnostic BM and post-C1 revealed *NRAS* as a founder mutation and *UBA1* as a subclone (diagnosis: *NRAS*-only, 4.7%; *NRAS* + *UBA1*, 27.1%) (Figure 2B). Integrated single-cell DNA sequencing cell surface protein analysis showed preserved differentiation capacity with lineage skewing. The *NRAS*-only clone retained multilineage potential with T-cell representation (4.3%), whereas the *NRAS* + *UBA1* clone exhibited marked myeloid/erythroid skewing, with enrichment in myeloid (68.9%), erythroid (57.3%), and natural killer cell (50.4%) compartments (Figure 2C). This supports *UBA1*-driven myeloid/erythroid skewing as previously suggested.[1,20] It is plausible that *UBA1* mutation provides a selective advantage for myeloid/erythroid expansion and clonal progression.

In P2, material was not available to resolve the clonal hierarchy. However, the substantially larger *SH2B3* clone (VAF, 87.2%) compared with *UBA1* (VAF, 16.8%) suggests that *SH2B3* was the primary event. In addition, the high VAF of *SH2B3* reflects mutant allele duplication by uniparental isodisomy 12q, further implying *SH2B3* as a founder mutation. UPD12q, *SH2B3*, and *UBA1* clones were not detected after chemotherapy, suggesting initial chemosensitivity despite ultimately refractory disease. Notably, this patient had germ line RUNX1 disease, which has not been reported with co-occurring *UBA1* mutations; among germ line predispositions, VEXAS syndrome has thus far only been described in DDX41 disorder.[26]

The initial presentation as pediatric-onset high-risk myeloid disease differs from typical adult VEXAS syndrome presentations.[1,2,13,20] This may reflect age-related variation or suggest that *UBA1* mutations represent subclonal events in pediatric malignancies. To assess the prevalence of *UBA1* mutations in pediatric AML, we queried data from the Pediatric Cancer Genome Project,[27] St. Jude Real-Time Clinical Genomics,[28] St. Jude Lifetime Cohort,[29] and the National Cancer Institute's Therapeutically Applicable Research to Generate Effective Treatment (TARGET) dataset (database of Genotypes and Phenotypes [dbGaP] accession: phs000465.v23.p8). Among 834 pediatric AML samples, no somatic mutations in the *UBA1* coding region were identified (Figure 2D). In childhood MDS, a previous study of 32 patients did not report any mutations affecting the *UBA1* coding region.[30] Despite the rarity of *UBA1* mutations, their association with aggressive disease in our patients supports the inclusion of *UBA1* on MDS/AML gene panels for the pediatric population as well.

Although less intensive regimens may be effective in adult VEXAS syndrome,[2,3,31,32] the high-risk neoplasms in our patients necessitated intensive treatment. Both patients were cytoreduced before hematopoietic stem cell transplantation (HSCT); P1 using CPX-351 and P2 with conventional chemotherapy (Figure 2E). P2 experienced early relapse after HSCT, and P1 achieved durable remission. This suggests that CPX-351 may be a sufficient alternative to intensive combination chemotherapy in similar cases. Given the high-risk disease, HSCT was pursued in both patients. In patients with *UBA1* mutations and malignant disease, transplant workup and donor search should be initiated promptly. Several studies have shown the use of HSCT in adult VEXAS syndrome, performed for both myeloid neoplasm (18 patients) and autoinflammatory disorder (16 patients), with a short-term survival rate in the myeloid neoplasm group of ~70% to 80%.[33-36]

In summary, we describe the youngest patients with *UBA1*-mutated myeloid neoplasms, with *UBA1* mutations arising secondary to other leukemia driver mutations (*NRAS* and *SH2B3*). This subtype of MDS/AML is rare and aggressive, requiring intensive treatment and transplantation.

This study was approved by the St. Jude Children's Research Hospital's institutional review board under approval number Pro00006262.

Acknowledgments: The authors thank the patients and their families, Peter Grayson for clinical insights, St. Jude BMF/MDS program team, and the St. Jude Center for Advanced Genome Engineering.

This research was supported by the Intramural Research Program of the National Institutes of Health (NIH) and the National Heart, Lung, and Blood Institute of NIH under award number T32 HL176447. This work was also supported by the Edward P. Evans MDS Foundation (M.W.W.). National Cancer Institute P30 Cancer

Center Support Grant (grant P30CA021765) (M.W.W.), and the Kennedy trust senior fellowship and Medical Research Council (MRC) project grant number MR/Y011945/1 (S.S.).

The findings and conclusions presented in this study are those of the authors and do not necessarily reflect the views of the NIH or the US Department of Health and Human Services.

Contribution: L.K. conducted the genomic experiments and data analysis; N.O. and K.E.N. conducted the data analysis; M.H., R.S., S.S., F.G.-R., B.A.P., D.B.B., R.C.R., G.S., A.S., and M.W.W. provided patient care and conducted the molecular studies; L.K. and M.W.W. wrote the manuscript and prepared the figures; M.W.W. provided overall study supervision; and all authors contributed to the manuscript writing and figure conception and approved the submission.

Conflict-of-interest disclosure: M.W.W. reports consulting for Retro Bio. The remaining authors declare no competing financial interests.

The contributions of F.G.-R. and B.A.P. are considered works of the US Government.

ORCID profiles: L.K., 0000-0002-5089-7659; N.O., 0000-0002-9806-0217; S.S., 0000-0001-7910-0554; F.G.-R., 0000-0003-3116-4588; B.A.P., 0000-0002-2974-7701; R.C.R., 0000-0002-9956-5647; K.E.N., 0000-0002-5581-6555; M.W.W., 0000-0001-6638-9643.

Correspondence: Marcin W. Wlodarski, Department of Experimental Hematology, St. Jude Children's Research Hospital, 262 Danny Thomas Place, Memphis, TN 38105; email: marcin.wlodarski@stjude.org.

References

1. Beck DB, Ferrada MA, Sikora KA, et al. Somatic mutations in *UBA1* and severe adult-onset autoinflammatory disease. *N Engl J Med*. 2020;383(27):2628-2638.
2. Groarke EM, Turturice B, Patel BA, Quinn KA, Fike A, Grayson PC. VEXAS syndrome: a comprehensive review of pathogenesis, clinical spectrum, and therapeutic strategies. *Lancet*. 2026;407(10528):637-648.
3. Mekinian AM, Georgin-Lavaille S, Ferrada MA, et al. American College of Rheumatology guidance statement for diagnosis and management of VEXAS developed by the International VEXAS Working Group Expert Panel. *Arthritis Rheumatol*. 2025;78(3):509-522.
4. van der Made CI, Potjewijd J, Hoogstins A, et al. Adult-onset autoinflammation caused by somatic mutations in UBA1: a Dutch case series of patients with VEXAS. *J Allergy Clin Immunol*. 2022;149(1):432-439.e4.
5. Tsuchida N, Kunishita Y, Uchiyama Y, et al. Pathogenic *UBA1* variants associated with VEXAS syndrome in Japanese patients with relapsing polychondritis. *Ann Rheum Dis*. 2021;80(8):1057-1061.
6. Ferrada MA, Savic S, Cardona DO, et al. Translation of cytoplasmic UBA1 contributes to VEXAS syndrome pathogenesis. *Blood*. 2022;140(13):1496-1506.
7. Sanchez-Hernandez BE, Calderon-Espinoza I, Martin-Nares E. Challenging the paradigm: a case of early-onset VEXAS syndrome. *Rheumatology (Oxford)*. 2024;63(3):e99-e100.
8. Tan IJ, Ferrada MA, Ahmad S, et al. Skin manifestations of VEXAS syndrome and associated genotypes. *JAMA Dermatol*. 2024;160(8):822-829.
9. Kusne Y, Ghorbanzadeh A, Dulau-Florea A, et al. Venous and arterial thrombosis in patients with VEXAS syndrome. *Blood*. 2024;143(21):2190-2200.
10. Gurnari C, Visconte V. From bone marrow failure syndromes to VEXAS: Disentangling clonal hematopoiesis, immune system, and molecular drivers. *Leuk Res*. 2023;127:107038.
11. Zhao LP, Kosmider O, Heiblig M, et al. Characteristics and prognostic significance of myelodysplasia-related features in VEXAS syndrome. *Leukemia*. 2026;40(3):661-665.
12. Mizumaki H, Gao S, Wu Z, et al. In depth transcriptomic profiling defines a landscape of dysfunctional immune responses in patients with VEXAS syndrome. *Nat Commun*. 2025;16(1):4690.
13. Sirenko M, Bernard E, Creignou M, et al. Molecular and clinical presentation of UBA1-mutated myelodysplastic syndromes. *Blood*. 2024;144(11):1221-1229.
14. Hu Y, Caldwell KJ, Onciu M, et al. CPX-351 induces remission in newly diagnosed pediatric secondary myeloid malignancies. *Blood Adv*. 2022;6(2):521-527.
15. Poulter JA, Collins JC, Cargo C, et al. Novel somatic mutations in UBA1 as a cause of VEXAS syndrome. *Blood*. 2021;137(26):3676-3681.
16. Collins JC, Magaziner SJ, English M, et al. Shared and distinct mechanisms of UBA1 inactivation across different diseases. *EMBO J*. 2024;43(10):1919-1946.
17. Al-Hakim A, Kulasekararaj A, Norouzi M, et al. S56F UBA1 variant is associated with haematological predominant subtype of VEXAS. *Br J Haematol*. 2023;203(2):331-335.
18. Rieu JB, Rigolot L. S56F UBA1 variant: a VEXAS syndrome subtype that follows its own path. *Blood*. 2024;144(24):2558.
19. Georgin-Lavialle S, Terrier B, Guedon AF, et al. Further characterization of clinical and laboratory features in VEXAS syndrome: large-scale analysis of a multicentre case series of 116 French patients. *Br J Dermatol*. 2022;186(3):564-574.
20. Gutierrez-Rodrigues F, Kusne Y, Fernandez J, et al. Spectrum of clonal hematopoiesis in VEXAS syndrome. *Blood*. 2023;142(3):244-259.
21. Corty RW, Brogan J, Byram K, Springer J, Grayson PC, Bick AG. VEXAS-defining UBA1 somatic variants in 245,368 diverse individuals in the NIH all of US cohort. *Arthritis Rheumatol*. 2024;76(6):942-948.
22. Gurnari C, Pascale MR, Vitale A, et al. Diagnostic capabilities, clinical features, and longitudinal UBA1 clonal dynamics of a nationwide VEXAS cohort. *Am J Hematol*. 2024;99(2):254-262.
23. Anderson M, Ercelen D, Richardson A, et al. Clinical manifestations of VEXAS syndrome across a broad spectrum of UBA1 mutation burden. *Arthritis Rheumatol*. 2026;78(1):223-230.
24. Li P, Alnoor FNU, Xie W, et al. Rapid growth of acquired UBA1 mutations predisposes male patients to low-risk MDS. *Leukemia*. 2025;39(1):248-256.
25. Sakuma M, Blombery P, Meggendorfer M, et al. Novel causative variants of VEXAS in UBA1 detected through whole genome transcriptome sequencing in a large cohort of hematological malignancies. *Leukemia*. 2023;37(5):1080-1091.

26. Kusne Y, Lasho T, Finke C, Patnaik MM, Badar T. VEXAS syndrome in a patient with DDX41 germline predisposition syndrome. *Leuk Res*. 2024;136:107432.

27. Downing JR, Wilson RK, Zhang J, et al. The pediatric cancer genome project. *Nat Genet*. 2012;44(6):619-622.

28. Newman S, Nakitandwe J, Kesserwan CA, et al. Genomes for kids: the scope of pathogenic mutations in pediatric cancer revealed by comprehensive DNA and RNA sequencing. *Cancer Discov*. 2021; 11(12):3008-3027.

29. Wang Z, Wilson CL, Easton J, et al. Genetic risk for subsequent neoplasms among long-term survivors of childhood cancer. *J Clin Oncol*. 2018;36(20):2078-2087.

30. Schwartz JR, Ma J, Lamprecht T, et al. The genomic landscape of pediatric myelodysplastic syndromes. *Nat Commun*. 2017;8(1): 1557.

31. Al-Hakim A, Trikha R, Phyu Htut EE, et al. Treatment outcomes in patients with VEXAS syndrome: a retrospective cohort study. *Lancet Rheumatol*. 2025;7(7):e472-e484.

32. Hoff FW, Trikha R, Groarke EM, Patel BA. Hypomethylating agents in vacuoles, E1 enzyme, X-linked, autoinflammatory, somatic syndrome (VEXAS): a systematic review. *Br J Haematol*. 2026; 208(3):819-828.

33. Al-Hakim A, Poulter JA, Mahmoud D, et al. Allogeneic haematopoietic stem cell transplantation for VEXAS syndrome: UK experience. *Br J Haematol*. 2022;199(5):777-781.

34. Diarra A, Duployez N, Fournier E, et al. Successful allogeneic hematopoietic stem cell transplantation in patients with VEXAS syndrome: a 2-center experience. *Blood Adv*. 2022;6(3): 998-1003.

35. Mangaonkar AA, Langer KJ, Lasho TL, et al. Reduced intensity conditioning allogeneic hematopoietic stem cell transplantation in VEXAS syndrome: data from a prospective series of patients. *Am J Hematol*. 2023;98(2):E28-E31.

36. Gurnari C, Koster L, Baaij L, et al. Allogeneic hematopoietic cell transplantation for VEXAS syndrome: results of a multicenter study of the EBMT. *Blood Adv*. 2024;8(6):1444-1448.