BRIEF REPORT

blood ICT
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BCMA-targeted T-cell engager therapy induces sustained remission in immune thrombocytopenia

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Immune thrombocytopenia (ITP) is an autoimmune disease mediated by platelet-reactive antibodies, leading to accelerated platelet clearance and an increased risk of bleeding. Despite multiple available therapeutic options, durable treatment-free remissions remain uncommon in patients with refractory disease. Here, we report 3 patients with multidrug refractory ITP treated with a bispecific B-cell maturation antigen (BCMA)–targeting T-cell engager, teclistamab, approved for the treatment of multiple myeloma. Fixed-duration teclistamab therapy–induced platelet response within 4, 9, and 23 days, which was sustained after treatment discontinuation. The entirety of ITP-directed therapies was tapered and discontinued, and the 3 patients remain in treatment-free remission for 8, 6, and 3 months, respectively. Responses were associated with rapid depletion of B cells and plasma cells. Toxicity was manageable and largely limited to low-grade cytokine release syndrome, transient neutropenia, and infections that were readily controlled. These observations highlight BCMA-directed bispecific antibodies as a potential therapeutic strategy in autoimmune hematologic diseases and provide a rationale for prospective clinical trials.

Introduction

Immune thrombocytopenia (ITP) is an acquired autoimmune disorder characterized by isolated thrombocytopenia due to immune-mediated platelet destruction. The condition is characterized by a breakdown of immune tolerance wherein autoreactive B- and plasma cells generate antibodies predominantly targeting platelet membrane glycoproteins. These autoantibodies mediate accelerated platelet clearance through Fc receptor–dependent phagocytosis and complement activation, culminating in thrombocytopenia and increased bleeding risk.[1,2] Although current therapies including

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The data sets generated during and analyzed during the current study are not publicly available due to patient confidentiality but are available from the corresponding author, Marie Luise Hütter-Krönke (luise.huetter-kroenke@charite.de), on reasonable request.

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glucocorticoids, immunosuppressants, anti-CD20 antibodies, splenectomy, spleen tyrosine kinase (SYK) and Bruton tyrosine kinase (BTK) inhibitors, and platelet-stimulating agents can improve platelet counts, many patients fail to achieve sustained, therapy-free remission.[3-5] T-cell engaging strategies, including chimeric antigen receptor (CAR) T cells and bispecific T-cell engager–targeting B cell and plasma cell antigens such as CD19 or B-cell maturation antigen (BCMA), have shown considerable promise in the treatment of autoimmune diseases.[6-9] Whether this success extends to ITP remains an open question, with clinical evidence currently limited to 3 single case reports.[10-12] Teclistamab is a bispecific T-cell engager cotargeting CD3 on T cells and BCMA on plasma cells, approved for the treatment of multiple myeloma. Here, we report a case series of 3 patients with multidrug refractory severe ITP who were successfully treated with teclistamab.

Study design

Three patients with multidrug refractory ITP were treated with off label teclistamab after obtaining written informed consent. Teclistamab was administered subcutaneously using step-up dosing with premedication consisting of antipyretics, H1-antihistamines, and dexamethasone. Response was assessed by serial platelet counts and bone marrow examination before and after therapy. Platelet counts are reported in per nanoliter, which is numerically equivalent to the internationally accepted SI unit $\times 10^9$/L (1/nL = 1 × 10^9/L). Platelet responses were defined according to the International Working Group (IWG) consensus criteria for response in ITP: 1) partial response (PR) was defined as a platelet count ≥30/nL and at least doubled from baseline in the absence of bleeding; 2) complete response (CR) was defined as a platelet count ≥100/nL on at least 2 measurements within 7 days; 3) sustained response was defined as a platelet count ≥100/nL maintained over a minimum of 3 months; and 4) no response was defined as a platelet count that did not meet the criteria for R or CR.[13] Treatment-free remission was defined as a PR maintained over a minimum of 3 months.

Results and discussion

We report 3 patients with multidrug refractory ITP who demonstrated resistance to multiple prior lines of therapy, including glucocorticoids, immunosuppressive agents, thrombopoietin receptor agonists, the anti-CD20 monoclonal antibody rituximab, as well SYK- (fostamatinib), and/or a BTK inhibitor (rilzabrutinib). Splenectomy was either declined by the patient or deemed to carry an

Table 1. Patient characteristics

	Patient 1	Patient 2	Patient 3
Age, y	33	74	74
Sex	F	M	M
Disease	ITP & AIHA (Evans syndrome), rheumatoid arthritis	ITP	ITP
Antiplatelet antibodies	Anti-GP IIb/IIIa	Anti-GP IIb/IIIa, Anti-GP Ib/IX	Not detectable
DAT	IgG, IgM, C3d complement	negative	negative
Prior treatments	Prednisolone Dexamethasone IVIG Azathioprine TPO-RA (eltrombopag, romiplostim, avatrombopag) Rituximab Fostamatinib	Prednisolone Dexamethasone IVIG Cyclophosphamide TPO-RA (romiplostim, avatrombopag) Rituximab Fostamatinib	Prednisolone Dexamethasone IVIG Azathioprine TPO-RA (eltrombopag, romiplostim, avatrombopag) Rituximab Fostamatinib Rilzabrutinib
Bleeding manifestation	Epistaxis; haemorrhagic bullae of oral mucosal	Transfusion dependent gastrointestinal bleeding	Ecchymoses
BM aspiration before therapy	No hematological malignancy; increased megakaryopoiesis	No hematological malignancy; increased megakaryopoiesis	Not performed
BM aspiration after therapy	Normalization of megakaryopoiesis; plasma cell depletion	Normalization of megakaryopoiesis; plasma cell depletion	Not performed
Baseline platelets	2/nL	1/nL	9/nL
Best response	CR	CR	CR
Time to response, d (≥30/nL platelets)	4	9	23
Sustained response >3 mo	CR	CR	PR
CRS	Grade 1 (fever (≥38°C) without hypotension or hypoxia)	None	Grade 1 (fever (≥38°C) without hypotension or hypoxia)
Adverse events	Neutropenia grade 3; hypogammaglobulinemia	Neutropenia grade 3; hypogammaglobulinemia	Hypogammaglobulinemia
Infections	Gastroenteritis; influenza virus infection	Bloodstream infection	None

AIHA, autoimmune hemolytic anemia; anti-GP, antibodies against glycoproteins; BM, bone marrow; F, female; M, male.

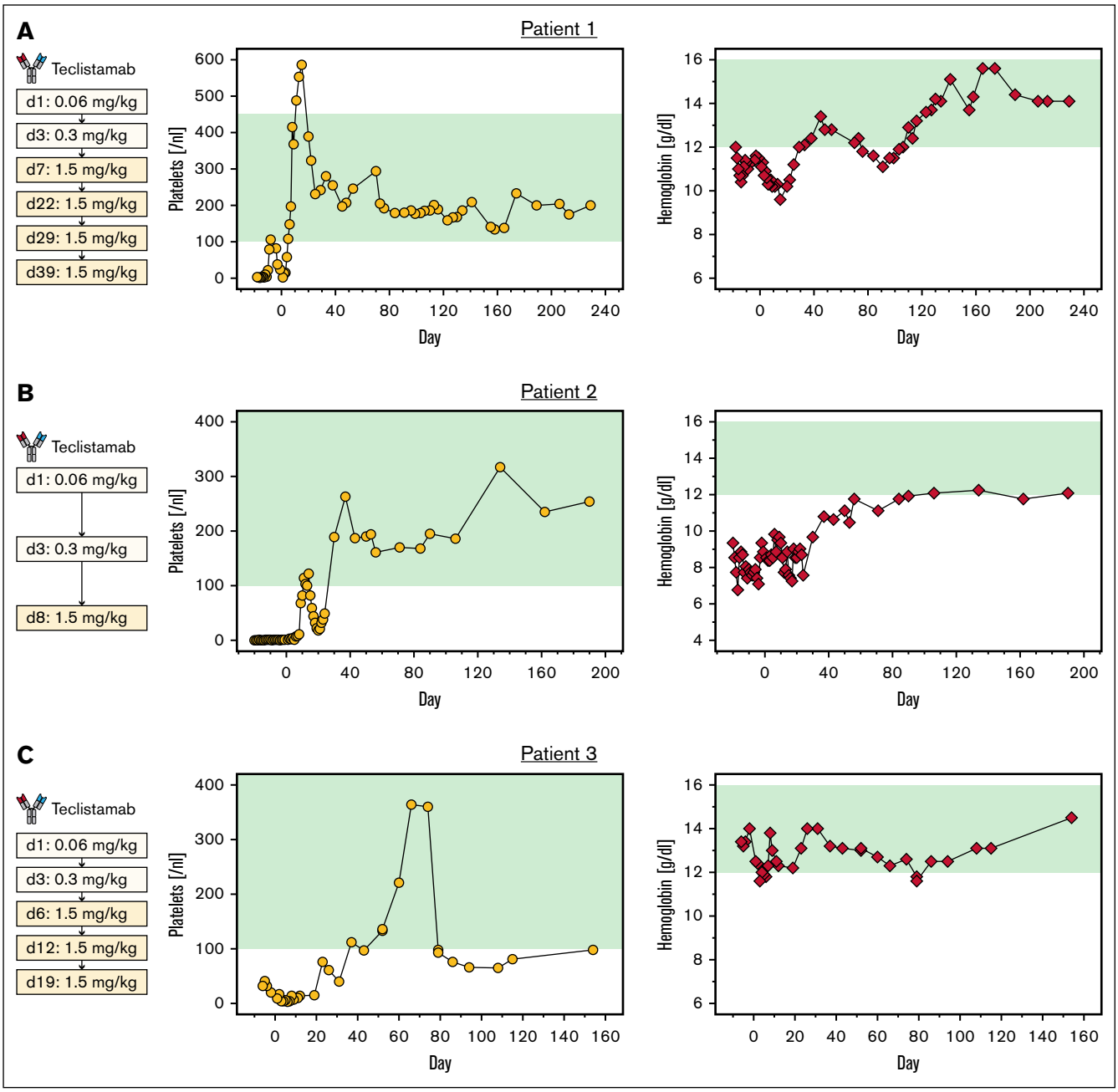

Figure 1. Patient characteristics. Clinical timeline depicting teclistamab administration (left panel), platelet count (central panel), and hemoglobin response (right) for patient 1 (A), patient 2 (B), and patient 3 (C).

unacceptably high procedural risk. All patients last treatment line before initiation of teclistamab consisted of a combination therapy with a thrombopoietin receptor agonist (TPO-RA), recently dosed rituximab and/or fostamatinib (Table 1).

Teclistamab was initiated using a step-up dosing regimen with standard premedication consisting of antipyretics, antihistamines, and dexamethasone. Because the safety, efficacy, required duration, and tolerability of teclistamab in ITP were unknown at the time, all patients received step-up dosing according to standard multiple myeloma protocols. The number of full-dose administrations was individualized based on treatment response and emerging experience across the case series. Each patient received step-up dosing and between 1 and 4 full-dose administrations (1.5 mg/kg), followed by a treatment-free follow-up phase (Figure 1). Teclistamab was generally well-tolerated. Grade 1 self-limiting cytokine release syndrome occurred in patients 1 and 3. Febrile infections were observed in patients 1 and 2, caused by influenza virus infection on day 166 during follow-up phase and a peripheral line–associated bloodstream infection on day 4, respectively, both of which resolved with antiviral/antibiotic therapy. Transient neutropenia of up to CTCAE (Common Terminology Criteria for Adverse Events) grade 3 occurred in 2 patients, which was successfully managed with G-CSF. All patients developed

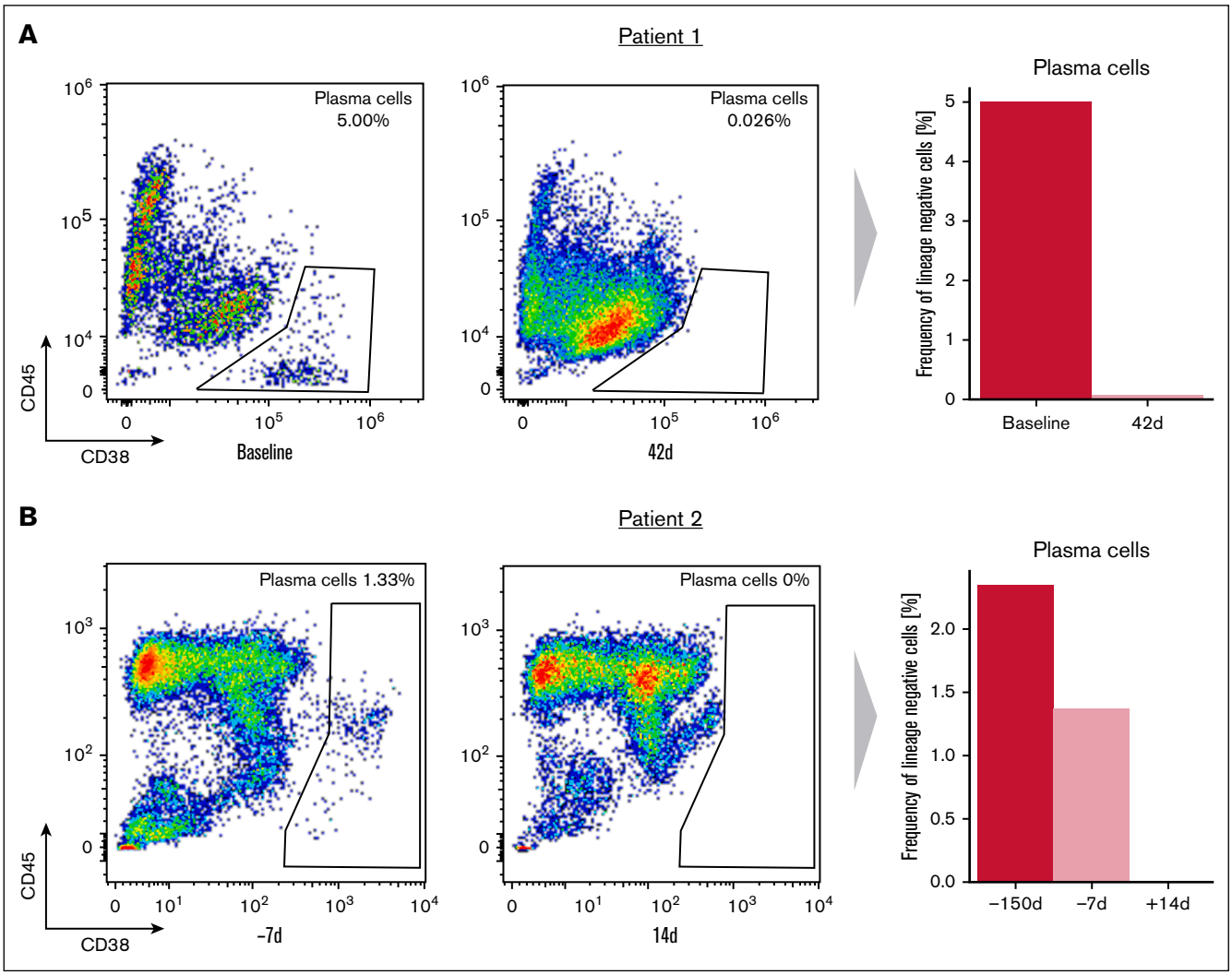

Figure 2. Bone marrow evaluation. Bone marrow flow cytometry analyses for patient 1 (A) and patient 2 (B). Left panels: dot plots demonstrating depletion of plasma cells following teclistamab treatment. Right panel: comparison plasma cell compartments before and after teclistamab treatment. Analyses were performed on lineage-negative cells (neutrophils, erythroid cells, T cells, myeloid cells, natural killer cells, and thrombocytes for patient 1; and erythroid cells and myeloid cells for patient 2).

hypogammaglobulinemia and received prophylactic IV immunoglobulin IgG substitution (IVIG) after the individual teclistamab treatment regimens were completed. No association was observed between platelet response and IVIG treatment. All patients received trimethoprim/cotrimoxazol and aciclovir since initiation of teclistamab treatment.

All 3 patients achieved a complete platelet response, with platelet counts reaching ≥30/nL at days 4, 9, and 23, respectively. Bleeding symptoms resolved promptly in all patients, and bleeding-associated anemia in patient 2 recovered within 30 days. Although patients 2 and 3 were refractory to dexamethasone, the rapid response in patient 1 may in part reflect a contribution from concurrent dexamethasone. We hypothesize, however, that teclistamab rapidly suppressed autoantibody-producing plasma cells, and that the high platelet output driven by TPO-RA therapy in the beginning led to swift consumption of remaining circulating autoantibodies. Patients 1 and 2 achieved complete remission with platelet counts remaining stable through last follow-up at day 229 and day 190, respectively. Patient 3 achieved an initial complete remission but sustained only a partial remission at last follow-up on day 154, with platelet counts ranging from 60/nL to 98/nL without any ITP therapy (Figure 1). This may reflect the greater burden of prior treatment in this patient. ITP-directed therapies were tapered and discontinued in all 3 patients, all of whom remain in treatment-free remission to date for 8, 6, and 3 months, respectively. Notably, in patient 1 with ITP and autoimmune hemolytic anemia (Evans syndrome), hemoglobin normalized and the direct antiglobulin test turned negative for the first time since initial diagnosis by week 5, indicating clearance of erythrocyte-directed autoantibodies and treatment-free remission of both conditions. Bone marrow examination in patient 1 and 2 demonstrated clearance of plasma cells alongside normalization of megakaryopoiesis (Figure 2). Bone marrow examination was not performed in patient 3. In all patients, light chains rapidly disappeared, indicating loss of antibody producing plasma cells.

Clearance of platelet-directed autoantibodies was documented in patient 2 following teclistamab therapy. In patient 1, autoantibodies had been identified during a prior disease episode but were undetectable immediately before teclistamab initiation, most likely due to profound thrombocytopenia (2/nL). In patient 3, platelet autoantibodies were not detectable at any disease stage. Notably, this patient showed a delayed response and sustained only partial remission, which may indicate a more complex, potentially T-cell–mediated cytotoxic component contributing to disease pathogenesis.

Current B-cell–targeting strategies in ITP predominantly rely on CD20-directed therapy with rituximab, which reduces circulating B cells but spares plasma cells.[5] Plasma cell–directed approaches using the anti-CD38 antibody daratumumab have also shown initial response rates of 48% to 95% in multirefractory ITP. However, over 40% of the treated patients experienced an early relapse, suggesting incomplete and nondurable elimination of autoreactive plasma cells.[14,15] CD19-directed CAR T-cell therapy has demonstrated efficacy across multiple antibody-mediated autoimmune diseases.[9] In a patient with multirefractory ITP CAR T-cell therapy led to a durable CR with profound B-cell depletion, followed by timely B-cell reconstitution on day 177. This resulted in immune recovery without the need for IVIG substitution, suggesting a potential advantage over BCMA-targeted therapies, which typically require IVIG replacement.[10] Recently, successful use of blinatumomab, a CD19-directed bispecific T-cell engager, in a patient with combined refractory ITP and antiphospholipid syndrome was demonstrated.[12] Our data showing sustained response in 3 patients by targeting BCMA is in line with a recently published case report demonstrating efficacy of this approach in multirefractory ITP.[11] Consistent with our first 2 patients, a rapid increase in platelet counts within 1 week was observed in a 35-year-old woman. No infections were reported despite the presence of hypogammaglobulinemia, however, follow-up was shorter than in our study.

Our patients are in sustained treatment-free remission for up to 8 months to date. However, longer follow-up will be essential to assess the durability of response and the potential for cure, which would require sustained hematologic remission, absence of autoantibodies, and reconstitution of a normal B-cell and plasma cell repertoire, as has been observed following CAR T-cell therapy in multiple myeloma and autoimmune diseases.[9] The successful and well-tolerated treatment of these 3 patients, together with emerging reports of BCMA-directed T-cell engager therapy in ITP, provides a compelling rationale for prospective clinical trials to establish the safety, efficacy, and optimal dosing of this approach in ITP and related antibody-mediated autoimmune cytopenias.[16]

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Authorship

Contribution: M.K., J.L., M.R.Z., J.K., and M.L.H.-K. wrote the manuscript; M.L.H.-K., J.K., M.K., J.L., V.F., T.T., S.H., and A.S. analyzed the data; and M.L.H.-K., J.K., M.K., J.L., S.S., J.J., D.B., S.B., F.D., L.B., U.K., M.R.Z., A.B., and F.A. provided data.

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References

1. Cooper N, Ghanima W. Immune thrombocytopenia. *N Engl J Med*. 2019;381(10):945-955.
2. Provan D, Semple JW. Recent advances in the mechanisms and treatment of immune thrombocytopenia. *EBioMedicine*. 2022;76:103820.
3. Miltiadous O, Hou M, Bussel JB. Identifying and treating refractory ITP: difficulty in diagnosis and role of combination treatment. *Blood*. 2020;135(7):472-490.
4. Kuter DJ, Ghanima W, Cooper N, et al. Safety and efficacy of rilzabrutinib vs placebo in adults with immune thrombocytopenia: the phase 3 LUNA3 study. *Blood*. 2025;145(24):2914-2926.
5. Ghanima W, Khelif A, Waage A, et al. Rituximab as second-line treatment for adult immune thrombocytopenia (the RITP trial): a multicentre, randomised, double-blind, placebo-controlled trial. *Lancet*. 2015;385(9978):1653-1661.

6. Bucci L, Böltz S, Hagen M, et al. BCMA T-cell engager therapy in patients with refractory autoimmune disease. *N Engl J Med*. 2025;393(15):1544-1547.
7. Alexander T, Krönke J, Cheng Q, Keller U, Krönke G. Teclistamab-induced remission in refractory systemic lupus erythematosus. *N Engl J Med*. 2024;391(9):864-866.
8. Zhang L, Gao Z, Pan H, et al. BCMA-targeted t-cell engager for autoimmune hemolytic anemia after CD19 CAR T-cell therapy. *N Engl J Med*. 2025;392(22):2282-2284.
9. Müller F, Taubmann J, Bucci L, et al. CD19 CAR T-cell therapy in autoimmune disease - a case series with follow-up. *N Engl J Med*. 2024;390(8):687-700.
10. Trautmann-Grill K, von Bonin M, Georgi A, et al. Salvage treatment of multi-refractory primary immune thrombocytopenia with CD19 CAR T cells. *Lancet*. 2025;405(10472):25-28.
11. Duque-Afonso J, Rummelt C, Förster K, Zeiser R, Mougiakakos D. BCMA-CD3 T-cell engager as salvage therapy for multirefractory primary immune thrombocytopenia. *Lancet*. 2026;407(10527):489-491.
12. Gottschlich A, Bücklein V, El-Marouk K, et al. Blinatumomab in combined immune thrombocytopenia and antiphospholipid syndrome. *N Engl J Med*. 2026;394(10):1030-1033.
13. Rodeghiero F, Stasi R, Gernsheimer T, et al. Standardization of terminology, definitions and outcome criteria in immune thrombocytopenic purpura of adults and children: report from an international working group. *Blood*. 2009;113(11):2386-2393.
14. Chen Y, Xu Y, Li H, et al. A novel anti-cd38 monoclonal antibody for treating immune thrombocytopenia. *N Engl J Med*. 2024;390(23):2178-2190.
15. Tsykunova G, Holme PA, Tran HTT, et al. Safety and efficacy of daratumumab in immune thrombocytopenia. *Blood Adv*. 2026;10(1):143-154.
16. Broome C, Hsu D, Choi P, Zhou C, Mozaffarian N. Trial in progress: a phase 1b, open-label, multiple ascending dose study of OM336, a novel BCMAxCD3 T cell engager, in patients with relapsed/refractory autoimmune cytopenias (NCT07083960) [abstract]. *Blood*. 2025;146(suppl 1):2900.