**Supplementary Materials**

Supplementary Table 1 – Number and percentage of adult and pediatric HSCT recipients who met each number of modified published TMA diagnostic criteria to be classified as patients with Suspected TMA

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | **Adultsa** | | **Pediatricsb** | |
|  | **Number of patients meeting the criteria (n)** | **Percentage of all HSCT recipients (N=16,809; n/N)** | **Number of patients meeting the criteria (n)** | **Percentage of all HSCT recipients (N=901; n/N)** |
| Cases who met at least 1 criterion | 16,681 | 99% | 859 | 95% |
| Cases who met at least 2 criteria | 15,876 | 94% | 744 | 83% |
| Cases who met at least 3 criteria | 12,573 | 75% | 365 | 41% |
| **Cases who met at least 4 criteriac** | 3029 | 18% | 94 | 10% |
| Cases who met at least 5 criteria | 229 | 1% | 0 | 0% |
| Cases who met at least 6 criteria | 8 | <0.1% | 0 | 0% |
| Cases who met all 7 criteria | 0 | 0% | 0 | 0% |

Abbreviations: HSCT, hematopoietic stem cell transplant; TMA, thrombotic microangiopathy

aCriteria were required to be met concurrently within 14 days for adult patients; bCriteria were required to be met concurrently within 28 days for pediatric patients; patients were excluded if they received a diagnosis code for TMA or HUS within 12 months of HSCT procedure, as these patients were classified as having Confirmed TMA. cOnly patients who met ≥4 of the diagnostic criteria were classified as patients with Suspected TMA.

Supplementary Table 2 – Frequency of HSCT codes for cases with a conditioning agent within the span of ≤15 days before through ≤5 days after HSCT in the adult and pediatric populations

|  |  |  |  |
| --- | --- | --- | --- |
| **Code** | **Description** | **Frequency of Codes in the Adult Population (N=16,809)** | **Frequency of Code in the Pediatric Population (N=901)** |
| 38241 | Hematopoietic progenitor cell (HPC); autologous transplantation | 4120 (24.5%) | 148 (16.4%) |
| 30243Y0 | Transfusion of Autologous Hematopoietic Stem Cells into Central Vein, Percutaneous Approach | 2769 (16.5%) | 90 (10.0%) |
| 38240 | Hematopoietic progenitor cell (HPC); allogeneic transplantation per donor | 2438 (14.5%) | 290 (32.2%) |
| 4104 | Autologous hematopoietic stem cell transplant without purging | 2283 (13.6%) | 117 (13.0%) |
| 4105 | Allogeneic hematopoietic stem cell transplant without purging | 1622 (9.6%) | 61 (6.8%) |
| 30243Y3 | Transfusion of Allogeneic Unrelated Hematopoietic Stem Cells into Central Vein, Percutaneous Approach | 756 (4.5%) | 38 (4.2%) |
| 30243Y2 | Transfusion of Allogeneic Related Hematopoietic Stem Cells into Central Vein, Percutaneous Approach | 746 (4.4%) | 34 (3.8%) |
| 30233Y0 | Transfusion of Autologous Hematopoietic Stem Cells into Peripheral Vein, Percutaneous Approach | 574 (3.4%) | 23 (2.6%) |
| 30243Y1 | Transfusion of Nonautologous Hematopoietic Stem Cells into Central Vein, Percutaneous Approach | 292 (1.7%) | 9 (1.0%) |
| 30243G0 | Transfusion of Autologous Bone Marrow into Central Vein, Percutaneous Approach | 147 (0.9%) | 5 (0.6%) |
| 4103 | Allogeneic bone marrow transplant without purging | 144 (0.9%) | 21 (2.3%) |
| 30243Y4 | Transfusion of Allogeneic Unspecified Hematopoietic Stem Cells into Central Vein, Percutaneous Approach | 126 (0.7%) | 1 (0.1%) |
| 4101 | Autologous bone marrow transplant without purging | 124 (0.7%) | 1 (0.1%) |
| 30233Y3 | Transfusion of Allogeneic Unrelated Hematopoietic Stem Cells into Peripheral Vein, Percutaneous Approach | 113 (0.7%) | 3 (0.3%) |
| 30233Y2 | Transfusion of Allogeneic Related Hematopoietic Stem Cells into Peripheral Vein, Percutaneous Approach | 105 (0.6%) | 6 (0.7%) |
| 4106 | Cord Blood Stem Cell Transplant | 97 (0.6%) | 19 (2.1%) |
| 30243X4 | Transfusion of Allogeneic Unspecified Cord Blood Stem Cells into Central Vein, Percutaneous Approach | 90 (0.5%) | – |
| 30233Y4 | Transfusion of Allogeneic Unspecified Hematopoietic Stem Cells into Peripheral Vein, Percutaneous Approach | 45 (0.3%) | – |
| 4107 | Autologous hematopoietic stem cell transplant with purging | 29 (0.2%) | 10 (1.1%) |
| 30243X0 | Transfusion of Autologous Cord Blood Stem Cells into Central Vein, Percutaneous Approach | 25 (0.1%) | 2 (0.2%) |
| 30233Y1 | Transfusion of Nonautologous Hematopoietic Stem Cells into Peripheral Vein, Percutaneous Approach | 24 (0.1%) | – |
| 30243G4 | Transfusion of Allogeneic Unspecified Bone Marrow into Central Vein, Percutaneous Approach | 19 (0.1%) | 7 (0.8%) |
| 30233G0 | Transfusion of Autologous Bone Marrow into Peripheral Vein, Percutaneous Approach | 19 (0.1%) | 1 (0.1%) |
| 30243G1 | Transfusion of Nonautologous Bone Marrow into Central Vein, Percutaneous Approach | 18 (0.1%) | 3 (0.3%) |
| 30233X4 | Transfusion of Allogeneic Unspecified Cord Blood Stem Cells into Peripheral Vein, Percutaneous Approach | 16 (0.1%) | – |
| 4108 | Allogeneic hematopoietic stem cell transplant with purging | 15 (0.1%) | – |
| 38243 | Hematopoietic progenitor cell (HPC); HPC boost | 7 (<0.1%) | 4 (0.4%) |
| 30233G4 | Transfusion of Allogeneic Unspecified Bone Marrow into Peripheral Vein, Percutaneous Approach | 7 (<0.1%) | 2 (0.2%) |
| 30233X0 | Transfusion of Autologous Cord Blood Stem Cells into Peripheral Vein, Percutaneous Approach | 7 (<0.1%) | – |
| 4100 | Bone marrow transplant not otherwise specified | 6 (<0.1%) | 1 (0.1%) |
| 30240Y0 | Transfusion of Autologous Hematopoietic Stem Cells into Central Vein, Open Approach | 6 (<0.1%) | – |
| 4102 | Allogeneic bone marrow transplant with purging | 4 (<0.1%) | – |
| 30263Y0 | Transfusion of Autologous Hematopoietic Stem Cells into Central Artery, Percutaneous Approach | 3 (<0.1%) | – |
| 30263Y1 | Transfusion of Nonautologous Hematopoietic Stem Cells into Central Artery, Percutaneous Approach | 2 (<0.1%) | 3 (0.3%) |
| 30233G1 | Transfusion of Nonautologous Bone Marrow into Peripheral Vein, Percutaneous Approach | 2 (<0.1%) | 1 (0.1%) |
| 30240Y1 | Transfusion of Nonautologous Hematopoietic Stem Cells into Central Vein, Open Approach | 2 (<0.1%) | – |
| 30253Y0 | Transfusion of Autologous Hematopoietic Stem Cells into Peripheral Artery, Percutaneous Approach | 2 (<0.1%) | – |
| 30263G0 | Transfusion of Autologous Bone Marrow into Central Artery, Percutaneous Approach | 1 (<0.1%) | 1 (0.1%) |
| 30230Y0 | Transfusion of Autologous Hematopoietic Stem Cells into Peripheral Vein, Open Approach | 1 (<0.1%) | – |
| 30230Y2 | Transfusion of Allogeneic Related Hematopoietic Stem Cells into Peripheral Vein, Open Approach | 1 (<0.1%) | – |
| 30240Y2 | Transfusion of Allogeneic Related Hematopoietic Stem Cells into Central Vein, Open Approach | 1 (<0.1%) | – |
| 30253Y1 | Transfusion of Nonautologous Hematopoietic Stem Cells into Peripheral Artery, Percutaneous Approach | 1 (<0.1%) | – |

Abbreviations: HSCT, hematopoietic stem cell transplant

Supplementary Table 3 – HUS and TMA codes

|  |  |  |  |
| --- | --- | --- | --- |
| **LOINC /CPT/ ICD-10-PCS/ 1CD-9-PCS Codes** | **Description** | **Adult, n (%)** | **Pediatric, n (%)** |
| M31.1 | Thrombotic microangiopathy | 77 (61.6) | 25 (83.3) |
| 446.6 | Thrombotic microangiopathy | 34 (27.2) | 3 (10.0) |
| D59.3 | Hemolytic uremic syndrome | 10 (8.0) | 1 (3.3) |
| 283.11 | Hemolytic-uremic syndrome | 4 (3.2) | 1 (3.3) |

Abbreviations: HUS, hemolytic uremic syndrome; TMA, thrombotic microangiopathy

Supplementary Table 4 – Conditioning agentsa

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| **Code System** | **Code** | **Code description** | **Frequency of codes in the adult population (n=16,809)** | **Frequency of codes in the pediatric population (n=901)** |  |
|  |
| RxNorm | 6718 | Melphalan | 10,029 (59.7%) | 198 (22.0%) |  |
| ICD-10-PCS | J9245 | Injection, melphalan hydrochloride, 50 mg | 5749 (34.2%) | 112 (12.4%) |  |
| RxNorm | 24698 | Fludarabine | 4768 (28.4%) | 126 (14.0%) |  |
| RxNorm | 3002 | Cyclophosphamide | 3365 (20.0%) | 343 (38.1%) |  |
| ICD-10-PCS | J9185 | Injection, fludarabine phosphate, 50 mg | 2937 (17.5%) | 100 (11.1%) |  |
| RxNorm | 1828 | Busulfan | 2901 (17.3%) | 188 (20.9%) |  |
| RxNorm | 2105 | Carmustine | 2868 (17.1%) | 30 (3.3%) |  |
| RxNorm | 3041 | Cytarabine | 2591 (15.4%) | 60 (6.7%) |  |
| ICD-10-PCS | J9070 | Cyclophosphamide, 100 mg | 1814 (10.8%) | 164 (18.2%) |  |
| ICD-10-PCS | J9050 | Injection, carmustine, 100 mg | 1555 (9.3%) | 17 (1.9%) |  |
| ICD-10-PCS | J0594 | Injection, busulfan, 1 mg | 1507 (9.0%) | 93 (10.3%) |  |
| ICD-10-PCS | J9100 | Injection, cytarabine, 100 mg | 1448 (8.6%) | 32 (3.6%) |  |
| RxNorm | 1011 | Lymphocyte immune globulin, anti-thymocyte globulin | 613 (3.6%) | 52 (5.8%) |  |
| RxNorm | 10473 | Thiotepa | 400 (2.4%) | 267 (29.6%) |  |
| RxNorm | 121191 | Rituximab | 384 (2.3%) | 35 (3.9%) |  |
| ICD-10-PCS | DW051ZZ | Beam radiation of whole body using photons 1 - 10 MEV | 158 (0.9%) | 16 (1.8%) |  |
| ICD-10-PCS | J9340 | Injection, thiotepa, 15 mg | 152 (0.9%) | 135 (15.0%) |  |
| RxNorm | XW043B3 | Introduction of cytarabine and daunorubicin liposome antineoplastic into central vein, percutaneous approach, new technology group 3 | 103 (0.6%) | – |  |
| ICD-10-PCS | J9310 | Injection, rituximab, 100 mg (deprecated 2018) | 59 (0.4%) | 7 (0.8%) |  |
| ICD-10-PCS | DW052ZZ | Beam radiation of whole body using photons >10 MEV | 59 (0.4%) | 1 (0.1%) |  |
| RxNorm | 117055 | Alemtuzumab | 40 (0.2%) | 46 (5.1%) |  |
| ICD-10-PCS | DW050ZZ | Beam radiation of whole body using photons <1 MEV | 31 (0.2%) | 4 (0.4%) |  |
| ICD-10-PCS | DWY57ZZ | Contact radiation of whole body | 28 (0.2%) | 1 (0.1%) |  |
| RxNorm | 968804 | Cytarabine liposome (deprecated 2020) | 24 (0.1%) | 11 (1.2%) |  |
| ICD-10-PCS | DWY5FZZ | Plaque radiation of whole body | 12 (0.1%) | – |  |
| ICD-10-PCS | J9098 | Injection, cytarabine liposome, 10 mg | 10 (0.1%) | – |  |
| ICD-10-PCS | DW053ZZ | Beam radiation of whole body using electrons | 8 (<0.1%) | 2 (0.2%) |  |
| RxNorm | XW033B3 | Introduction of cytarabine and daunorubicin liposome antineoplastic into peripheral vein, percutaneous approach, new technology group 3 | 7 (<0.1%) | 1 (0.1%) |  |
| ICD-10-PCS | J0202 | Injection, alemtuzumab, 1 mg | 6 (<0.1%) | 11 (1.2%) |  |
| ICD-10-PCS | J9010 | Injection, alemtuzumab, 10 mg (deprecated 2015) | 6 (<0.1%) | 11 (1.2%) |  |
| ICD-10-PCS | DW054ZZ | Beam radiation of whole body using heavy particles (protons, ions) | 5 (<0.1%) | – |  |
| ICD-10-PCS | J9312 | Injection, rituximab, 10 mg | 5 (<0.1%) | 2 (0.2%) |  |
| ICD-10-PCS | J8530 | Cyclophosphamide; oral, 25 mg | 4 (<0.1%) | – |  |
| ICD-10-PCS | D7031ZZ | Beam radiation of neck lymphatics using photons 1 - 10 MEV | 3 (<0.1%) | – |  |
| ICD-10-PCS | D7041ZZ | Beam radiation of axillary lymphatics using photons 1 - 10 MEV | 3 (<0.1%) | – |  |
| ICD-10-PCS | D7051ZZ | Beam radiation of thorax lymphatics using photons 1 - 10 MEV | 3 (<0.1%) | – |  |
| ICD-10-PCS | D7061ZZ | Beam radiation of abdomen lymphatics using photons 1 - 10 MEV | 3 (<0.1%) | – |  |
| ICD-10-PCS | D7071ZZ | Beam radiation of pelvis lymphatics using photons 1 - 10 MEV | 3 (<0.1%) | – |  |
| ICD-10-PCS | D7081ZZ | Beam radiation of inguinal lymphatics using photons 1 - 10 MEV | 3 (<0.1%) | – |  |
| ICD-10-PCS | D7001ZZ | Beam radiation of bone marrow using photons 1 - 10 MEV | 2 (<0.1%) | – |  |
| ICD-10-PCS | DV011ZZ | Beam radiation of testis using photons 1 - 10 MEV | 2 (<0.1%) | – |  |
| ICD-10-PCS | DW055ZZ | Beam radiation of whole body using neutrons | 2 (<0.1%) | – |  |
| ICD-10-PCS | DW061ZZ | Beam radiation of pelvic region using photons 1 - 10 MEV | 2 (<0.1%) | – |  |
| ICD-10-PCS | J8510 | Busulfan; oral, 2 mg | 2 (<0.1%) | 2 (0.2%) |  |
| ICD-10-PCS | D0000ZZ | Beam radiation of brain using photons <1 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | D0061ZZ | Beam radiation of spinal cord using photons 1 - 10 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | D7000ZZ | Beam radiation of bone marrow using photons <1 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | D7004ZZ | Beam radiation of bone marrow using heavy particles (protons, ions) | 1 (<0.1%) | – |  |
| ICD-10-PCS | D7011ZZ | Beam radiation of thymus using photons 1 - 10 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | D7021ZZ | Beam radiation of spleen using photons 1 - 10 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | D7022ZZ | Beam radiation of spleen using photons >10 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | D7034ZZ | Beam radiation of neck lymphatics using heavy particles (protons, ions) | 1 (<0.1%) | – |  |
| ICD-10-PCS | D7043ZZ | Beam radiation of axillary lymphatics using electrons | 1 (<0.1%) | – |  |
| ICD-10-PCS | DW011ZZ | Beam radiation of head and neck using photons 1 - 10 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | DW033ZZ | Beam radiation of abdomen using electrons | 1 (<0.1%) | – |  |
| ICD-10-PCS | DW041ZZ | Beam radiation of hemibody using photons 1 - 10 MEV | 1 (<0.1%) | – |  |
| ICD-10-PCS | D0001ZZ | Beam radiation of brain using photons 1 - 10 MEV | – | 2 (0.2%) |  |
| ICD-10-PCS | D7064ZZ | Beam radiation of abdomen lymphatics using heavy particles (protons, ions) | – | 1 (0.1%) |  |
| ICD-10-PCS | J9153 | Injection, liposomal, 1 mg daunorubicin and 2.27 mg cytarabine | – | 1 (0.1%) |  |

NOTE: In the adult population, 0.03% (5/16,809) patients had site-specific beam radiation without another type of conditioning agent ≤15 days prior to, or ≤5 days after, the HSCT procedure code date. In those pediatric patients who received site-specific beam radiation (0.33%; 3/901), they also received another type of conditioning agent.

Abbreviations: HSCT, hematopoietic stem cell transplant

aPatients could have had more than one treatment. Frequency based on 14,698 unique adult patients and 654 unique pediatric patients.

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Adult** | | | **Pediatric** | | |
|  | **Confirmed TMAa**  **N=125** | **Suspected TMAb**  **N=3029** | **Non-TMA**  **N=13,655** | **Confirmed TMAa**  **N=30** | **Suspected TMAb**  **N=94** | **Non-TMA**  **N=777** |
| **Coexisting conditions, n (%)** |  |  |  |  |  |  |
| Myocardial infarction | 6 (4.8) | 151 (5.0)\* | 536 (3.9) | 0 (0) | 0 (0) | 1 (0.1) |
| Congestive heart failure | 27 (21.6)\* | 402 (13.3)\* | 1319 (9.7) | 6 (20) | 22 (23.4) | 124 (16) |
| Peripheral vascular disease | 14 (11.2) | 310 (10.2)\* | 1234 (9.0) | 3 (10) | 15 (16) | 84 (10.8) |
| Cerebrovascular disease | 10 (8.0) | 212 (7.0)\* | 614 (4.5) | 5 (16.7) | 9 (9.6) | 71 (9.1) |
| Chronic pulmonary disease | 29 (23.2) | 623 (20.6)\* | 2273 (16.6) | 8 (26.7) | 15 (16) | 131 (16.9) |
| Connective tissue/rheumatic disease | 7 (5.6) | 102 (3.4) | 390 (2.9) | 1 (3.3) | 1 (1.1) | 3 (0.4) |
| Peptic ulcer disease | 6 (4.8)\* | 51 (1.7) | 220 (1.6) | 0 (0) | 0 (0) | 10 (1.3) |
| Liver disease | 16 (12.8) | 492 (16.2)\* | 1449 (10.6) | 7 (23.3) | 20 (21.3) | 111 (14.3) |
| Mild | 16 (12.8) | 456 (5.1)\* | 1382 (10.1) | 5 (16.7) | 18 (19.1) | 92 (11.8) |
| Moderate or severe | 3 (2.4) | 69 (2.3)\* | 167 (1.2) | 3 (10) | 3 (3.2) | 32 (4.1) |
| Renal disease | 24 (19.2) | 506 (16.7)\* | 1804 (13.2) | 4 (13.3) | 6 (6.4) | 42 (5.4) |
| Diabetes | 18 (14.4) | 564 (18.6)\* | 2213 (16.2) | 2 (6.7) | 5 (5.3) | 20 (2.6) |
| Diabetes without chronic complications/mild to moderate | 18 (14.4) | 551 (18.2)\* | 2164 (15.8) | 2 (6.7) | 5 (5.3) | 20 (2.6) |
| Diabetes with chronic complications | 3 (2.4) | 161 (5.3)\* | 648 (4.7) | 0 (0) | 0 (0) | 0 (0) |
| Metastatic solid tumor | 14 (11.2) | 363 (12.0)\* | 1400 (10.3) | 15 (50) | 29 (30.9) | 257 (33.1) |

Supplementary Table 5 – medical history of adult and pediatric patients in the confirmed, suspected and non-TMA groups at baseline, prior to HSCT

Abbreviations: HSCT, hematopoietic stem cell transplant; TMA, thrombotic microangiopathy

\*Denotes a statistically significant difference compared with the non-TMA group, p<0.05

aConfirmed TMA defined as cases with TMA and/or HUS diagnosis codes

bSuspected TMA defined as cases without TMA and/or HUS diagnosis codes, but that met the modified published criteria

Supplementary Table 6 – Ongoing HSCT-TMA clinical studies currently listed on clinicaltrials.gov

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| **Title** | **NCT number** | **Study type and phase** | **Treatment(s)** | **Primary Endpoint(s)** | **Status** | **Completion date** |
| Study in Adult and Pediatric Patients With HSCT-TMA | NCT04970004 | Observational, Retrospective | No intervention | Proportion of patients achieving TMA response | Withdrawn | April 2022 |
| Study of Ravulizumab in Pediatric Participants with HSCT-TMA | NCT04557735 | Phase 3, Open-label, Single Arm, Multicenter | Ravulizumab  Best Supportive Care | TMA response | Recruiting | Primary:  December 2024  Study: June 2025 |
| Ravulizumab in Thrombotic Microangiopathy After Hematopoietic Stem Cell Transplant | NCT04543591 | Phase 3, Randomized, Double-Blind, Placebo-Controller, Multicenter | Ravulizumab  Placebo  Best Supportive Care | TMA response | Recruiting | Primary:  December 2024  Study: July 2025 |
| Single Patient Expanded Access Treatment Plan For The Investigational Product Naroplimab | NCT04247906 | Expanded Access Treatment Plan | Nasroplimab | N/A | Available | N/A |
| Efficacy and Safety Study of Naroplimab in Pediatric Patients with High-Risk Hematpoietic Stem Cell Transplant TMA | NCT05855083 | Phase 2, Uncontrolled, Single-Dosing Regimen | Narsoplimab | 100-day survival rate following high-risk HSCT-TMA diagnosis | Recruiting | Primary:  December 2025  Study:  December 2025 |
| Evaluate the PK Efficacy Safety and Tolerability of Pegcetacoplan in Patients with Thrombotic Microangiopathy | NCT05148299 | Phase 2, Open-label, Single-Arm, Multicenter | Pegcetacoplan | Pegcetacoplan pharmacokinetics (AUC0-tau, Cmax, Tmax, Ctrough) | Recruiting | Primary: January 2026  Study: January 2026 |
| Safety and Efficacy Study of OMS721 in Patients With Thrombotic Microangiopathies | NCT02222545 | Phase 2, Uncontrolled, Ascending-Dose-Escalation | OMS721 | Assess the safety and tolerability of multiple-dose administration of OMS721 in subjects with TMA  Evaluate the response rate to OMS721 in patients with HSCT-TMA | Completed | Primary:  January 2020  Study:  August 2020 |
| Defibrotide TMA Prophylaxis Pilot Trial | NCT03384693 | Phase 2, Open-Label, Single-Arm | Defibrotide | Percent of Total Doses of Defibrotide That Were Missed [Feasibility]  Participants With Reportable Serious Adverse Events [Safety] Per CTACAE v5 Grade 3 or Higher  Participants With Clinically Significant Bleeding Requiring Discontinuation of Therapy [Safety]  Participants With Hypersensitivity Reaction Requiring Discontinuation of Therapy [Safety] | Completed | Primary: July 2020  Study: July 2020 |
| OMS721 Compassionate Use in Patients With Thrombotic Microangiopathy | NCT02355782 | Open-Label, Expanded Access | OMS721 | N/A | Available | N/A |
| Identification of Plasma Biomarkers for Early Diagnosis of Transplant-associated Thrombotic Microangiopathy | NCT06102694 | Observational | N/A | The frequency of TA-TMA  Risk factors for TA-TMA  Levels of sC5b-9 at regular times | Recruiting | Primary:  January 2025  Study: December 2025 |
| Eculizumab to Treat Thrombotic Microangiopathy/Atypical Hemolytic Uremic Syndrome-Associated Multiple Organ Dysfunction Syndrome in Hematopoietic Stem Cell Transplant Recipients | NCT03518203 | Phase 2, Prospective, Single-Arm, Multicenter | Eculizumab | Survival | Completed | Primary:  April 2022  Study:  June 2022 |
| A Safety and Efficacy Study of NAC in Patients With TA-TMA | NCT03252925 | Phase 3, Interventional | N-Acetylcysteine  Placebo |  | Completed | Primary:  October 2021  Study: October 2021 |