HSD118

HEALTHCARE RESOURCE UTILIZATION WITH RECOMBINANT ADAMTS13 IN PATIENTS LIVING WITH CONGENITAL TTP: RESULTS FROM A PHASE 3 RANDOMIZED CLINICAL TRIAL

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INTRODUCTION

- cTTP is an ultra-rare, inherited deficiency of the VWF-cleaving enzyme ADAMTS13, characterized by thrombocytopenia and microangiopathic hemolytic anemia. It can present as acute, life-threatening TTP events or with chronic symptoms; in both cases, microthrombi can cause organ ischemia, resulting in stroke, myocardial infarction, and kidney injury[1-5]
- Although generally effective, PBTs used to replace ADAMTS13 (the historical standard of care in cTTP) remain insufficient to restore ADAMTS13 activity. Consequently, this treatment is associated with a risk of acute TTP events and organ damage, and with the burden of large infusion volumes and potential allergic reactions[6-14]
- TAK-755 (rADAMTS13; Takeda Pharmaceuticals USA, Inc.) is an approved replacement therapy for ADAMTS13 deficiency for prophylactic and on-demand treatment in patients with cTTP in multiple jurisdictions[15-19]
- We present here an assessment of HRU from the phase 3 clinical trial comparing rADAMTS13 with PBT[20,21]

METHODS

Study design

PATIENTS

Ethics committee approval was obtained, and all participants/legally authorized representatives provided informed consent prior to study entry

Patients 0–70 years old with genetically confirmed cTTP and ADAMTS13 activity <10% and no history or presence of functional ADAMTS13 inhibitor at screening

TREATMENT

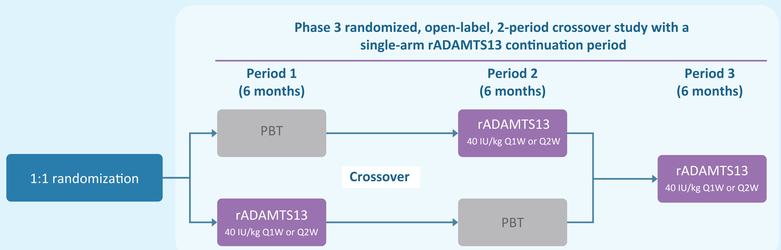
Patients were randomized 1:1 to receive rADAMTS13 40 IU/kg Q1W or Q2W or PBT prophylaxis, as determined by the participant's usual PBT prophylaxis schedule

- PBTs could include fresh frozen plasma, pooled solvent/detergent-treated plasma, or plasma-derived factor VIII/VWF concentrates

Participants received treatment for 6 months (Period 1) and then crossed over to the alternate treatment for 6 months (Period 2); all participants then received rADAMTS13 prophylaxis for 6 months (Period 3)

ENDPOINT

HRU was a secondary endpoint of the trial (NCT03393975), assessing the number and duration of hospital visits overall and for acute TTP events, and the number of acute care and emergency department visits



RESULTS

PATIENT DEMOGRAPHICS

- Baseline demographics were similar between the treatment arms, although the median and mean age of participants in the arm who initiated with rADAMTS13 were somewhat older

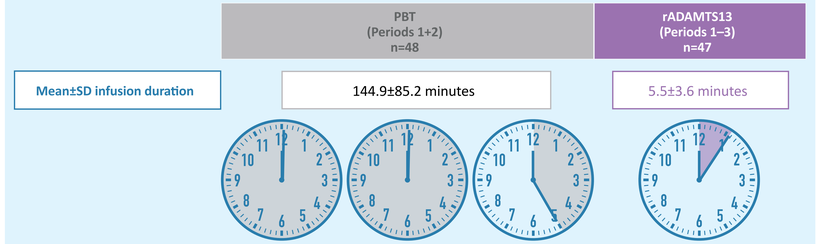
	PBT→rADAMTS13 (n=27)	rADAMTS13→PBT (n=21)	Total (N=48)
Age, years			
Mean±SD	29.1±16.9	33.5±16.5	31.0±16.7
Median (range)	27.0 (5–68)	42.0 (3–54)	32.5 (3–68)
Age group, years, n (%)			
<6	1 (3.7)	3 (14.3)	4 (8.3)
6 to <12	3 (11.1)	1 (4.8)	4 (8.3)
12 to <18	3 (11.1)	1 (4.8)	4 (8.3)
≥18	20 (74.1)	16 (76.2)	36 (75.0)
Sex, n (%)			
Male	11 (40.7)	9 (42.9)	20 (41.7)
Female	16 (59.3)	12 (57.1)	28 (58.3)

Number of participants who received ≥1 dose (safety analysis set).

TREATMENT EXPOSURE AND INFUSION DURATION

Mean rADAMTS13 infusion duration in Periods 1–3 was observed to be shorter than PBT infusions in Periods 1 and 2

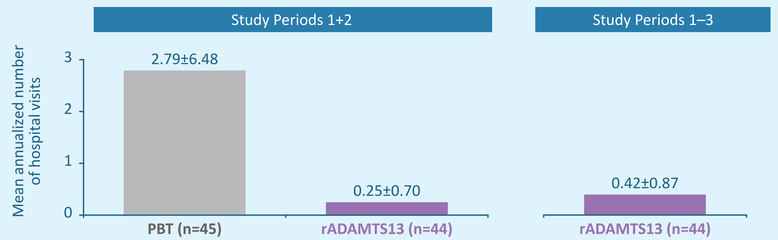
- Similar differences in infusion duration were seen between rADAMTS13 and PBT in Period 1 alone and Period 2 alone
- Forty-seven patients received a mean±SD of 35.0±12.36 infusions of rADAMTS13 during Periods 1–3, while 48 patients received 16.9±6.48 infusions of PBT during Periods 1 and 2*



*More rADAMTS13 infusions were administered than PBT infusions, reflecting the study design. Number of patients is based on the number of participants who received ≥1 dose (safety analysis set).

MEAN ANNUALIZED NUMBER OF HOSPITAL VISITS FOR ANY REASON

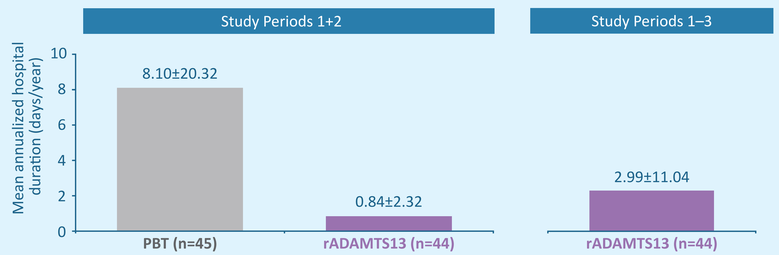
- Mean annualized hospital visits remained below 1 per participant-year with rADAMTS13 across the study periods evaluated



Number of participants with confirmed cTTP diagnosis who received ≥1 dose; modifications applied to the analysis dataset as specified in the protocol for participants enrolled prior to November 2017 (modified full analysis set). Values above columns represent the mean±SD.

MEAN ANNUALIZED HOSPITAL DURATION

- Mean annualized hospital duration was numerically shorter with rADAMTS13 compared with PBT during Periods 1 and 2; patients receiving rADAMTS13 also had shorter hospital durations in the combined Periods 1–3



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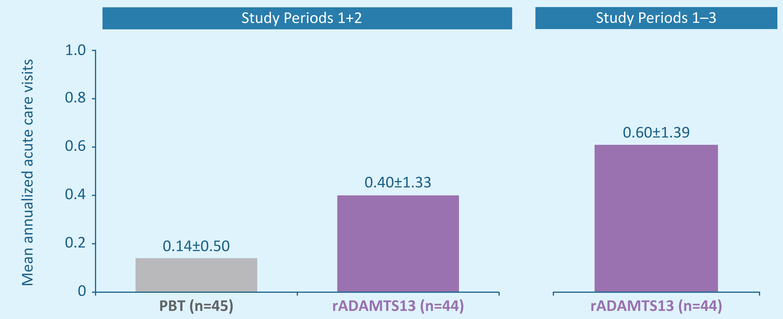
MEAN ANNUALIZED NUMBER OF HOSPITAL VISITS AND HOSPITAL DURATION FOR ACUTE TTP EVENTS

- No acute TTP events were observed in patients treated with rADAMTS13; consequently, both annualized hospital visits for these events and hospital duration were zero per year
- One patient receiving PBT experienced an acute TTP event, resulting in a mean±SD of 0.04±0.26 annualized visits for acute TTP events during Periods 1 and 2 and annualized hospital duration of 0.08±0.51 days/year

MEAN ANNUALIZED ACUTE CARE VISITS

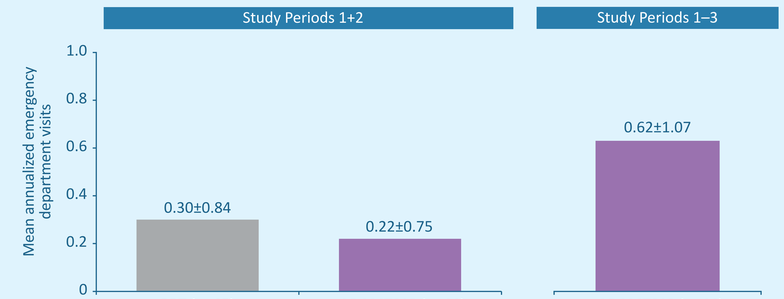
- The mean annualized number of acute care visits was low in both arms (<1 visit per participant-year); among patients treated with rADAMTS13, the mean±SD rate was 0.40±1.33 during Periods 1 and 2 and 0.60±1.40 during all 3 treatment periods, while patients treated with PBT experienced 0.14±0.50 acute care visits during Periods 1 and 2



Number of participants with confirmed cTTP diagnosis who received ≥1 dose; modifications applied to the analysis dataset as specified in the protocol for participants enrolled prior to November 2017 (modified full analysis set). Values above columns represent the mean±SD.

MEAN ANNUALIZED EMERGENCY DEPARTMENT VISITS

- Few emergency department visits occurred in either arm; rADAMTS13 prophylaxis was associated with a mean±SD annualized 0.22±0.75 emergency department visits during Periods 1 and 2 and 0.62±1.07 visits across Periods 1–3, compared with 0.30±0.84 emergency department visits during Periods 1 and 2 among patients treated with PBT



Number of participants with confirmed cTTP diagnosis who received ≥1 dose; modifications applied to the analysis dataset as specified in the protocol for participants enrolled prior to November 2017 (modified full analysis set). Values above columns represent the mean±SD.

LIMITATIONS

- These results should be interpreted with caution given the limitations of a small sample size in the context of the rare nature of cTTP, and the fact that the study was not powered to demonstrate statistical significance in comparing rADAMTS13 with PBT
- Reasons for acute care visits were not collected, limiting the ability to assess the association between visits and treatment

CONCLUSIONS

- **The lower rate of hospitalization, length of hospital stay, and infusion duration associated with rADAMTS13 use may indicate lower patient burden and reduced HRU compared with PBT**
  - **Mean annualized rates of hospital visits and hospital duration, overall and due to acute TTP events, were numerically lower for rADAMTS13 prophylaxis than for PBT during the crossover phase (Periods 1 and 2) and remained low across the study periods evaluated**
  - **Overall mean annualized rates of acute care visits were low (<1 visit per participant-year). During the crossover phase, rates were numerically higher with rADAMTS13 prophylaxis than with PBT**
  - **Low annualized rates of emergency department visits were observed for both rADAMTS13 and PBT prophylaxis during the crossover phase and for rADAMTS13 across Periods 1–3**
- **Long-term outcomes across these endpoints are being evaluated in an ongoing phase 3b (NCT04683003) open-label extension**

ABBREVIATIONS

ADAMTS13, a disintegrin and metalloproteinase with thrombospondin motifs 13; cTTP, congenital thrombotic thrombocytopenic purpura; HRU, healthcare resource utilization; PBT, plasma-based therapy; Q1W, once weekly; Q2W, once every 2 weeks; rADAMTS13, recombinant ADAMTS13; TTP, thrombotic thrombocytopenic purpura; VWF, Von Willebrand factor.

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FUNDING

The study was funded by Takeda Development Center Americas, Inc., Cambridge, MA, USA.

ACKNOWLEDGMENTS

The authors thank Hamid Ferdosi for presenting this work. The authors also thank the patients and their families for participating in this trial as well as the Study TAK-755-281102 (NCT03393975) investigators and study staff for their contributions to this trial. Under the direction of the authors, medical writing support for this poster was provided by James Borwick, BS, and was funded by Takeda Development Center Americas, Inc., Cambridge, MA, USA.

DISCLOSURES

MS: Speaker fees and advisory board honoraria from Takeda; and research grants from Baxalta (a Takeda company) and Shire (a Takeda company). PK: Honoraria for consultations, advice and presentations, and financial support for scientific research and travel grants from Ablynx/Sanofi, Alexion, Biotest, CSL Behring, Novo Nordisk, Roche, Takeda, and Technoclone. PC: Speaker fees and advisory board honoraria from Alexion, Sanofi, and Takeda. W-AH: Speaker fees and advisory board honoraria from Chugai/Roche, CSL Behring, Novo Nordisk, Sobi, and Takeda. SH: Honoraria from Bayer Yakuhin, Chugai, CSL Behring, Novo Nordisk, Pfizer Japan, Sanofi K.K., and Takeda. SB: Nothing to disclose. SI: Advisory board honoraria from AbbVie, Acrotech Biopharma, ADC Therapeutics, argenx, Ipsen, and Sobi. SX, TMR, and DR: Employees of Takeda Development Center Americas, Inc., and Takeda stockholders. JW: Grant/research support and honoraria for lectures from Alnylam, Amgen, AstraZeneca, Baxalta/Shire (a Takeda company), Bayer, CSL Behring, LFB, Novartis, Novo Nordisk, Octapharma, Pfizer, Roche, Sanofi, Siemens, Sobi, and Werfen.

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Presented at the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Congress; May 17–20, 2026; Philadelphia, PA, USA