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First Results of a Noninterventional, Prospective, Postauthorization Safety Study of Ciltacabtagene Autoleucel in Patients With Relapsed/Refractory Multiple Myeloma

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Key Takeaway

Initial results from this prospective, noninterventional PASS, in which more than half of patients were treated in earlier lines of therapy (1–2 pLOT), demonstrated that the real-world safety and efficacy of cilta-cel in Europe and Brazil were comparable with clinical trial results

Conclusions

Real-world adoption is extending cilta-cel into earlier-line RRMM with more than half of patients receiving it after 1–2 prior lines in this PASS, supporting continued integration earlier in the treatment pathway

Effectiveness outcomes (ORR, 96%; 6-month PFS, 93%; 6-month OS, 95%) were comparable with those reported in clinical trials, including CARTITUDE-1 and CARTITUDE-4[4,7]

The safety profile was consistent with the known profile of cilta-cel, based on clinical trials and other real-world data; in addition, no hematologic SPMs occurred

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Disclosures

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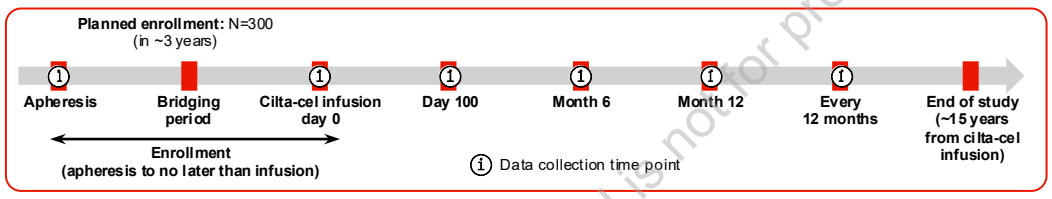
Introduction

- Ciltacabtagene autoleucel (cilta-cel) is approved in Europe and Brazil for patients with lenalidomide-refractory relapsed/refractory multiple myeloma (RRMM) who received ≥1 prior line of therapy (pLOT), including an immunomodulatory drug (IMiD) and a proteasome inhibitor (PI), and showed disease progression on the last therapy.[1,2] As of August 2026, more than 14,000 patients have received cilta-cel worldwide[3]
- The approval of cilta-cel for earlier-line treatment of RRMM was based on results from the phase 3 CARTITUDE-4 trial (NCT04181827)
  - In CARTITUDE-4, cilta-cel significantly prolonged progression-free survival (PFS) and overall survival (OS) in patients with lenalidomide-refractory multiple myeloma after 1–3 pLOT[4,5]
- This Postauthorization Safety Study (PASS; EUPAS49370) is a prospective, noninterventional study to evaluate the safety and effectiveness of cilta-cel in real-world settings in Europe and Brazil[6]
- Here, we present the first publication of results from this ongoing PASS

Methods

- This prospective, noninterventional, ongoing PASS is being conducted in Austria, Germany, Spain, and Brazil, where cilta-cel is a standard of care for patients with RRMM
- The study methodology was presented previously[6]
- **Figure 1** shows the study schema
  - 300 patients will be enrolled
  - Follow-up time is 15 years from cilta-cel infusion
- Key eligibility criteria:
  - Underwent apheresis with the intention of receiving commercial cilta-cel infusion
  - Provided written informed consent
- This analysis included all enrolled patients who had cilta-cel ≥100 days prior to data cut-off
  - Data cut-off: February 27, 2026
- Objectives of the study:
  - The primary objective is to evaluate the short- and long-term safety of cilta-cel, including risk of second primary malignancies (SPMs)
    - Incidence of adverse events (AEs) of interest, including but not limited to SPMs, neurological events, cytopenias, hypogammaglobulinemia, and infections, are reported[6]
  - The secondary objective is to evaluate the effectiveness of cilta-cel, including ORR, PFS, and OS, as reported by investigator

Figure 1: Study design



Results

Patients

- All 106 enrolled patients who were infused with cilta-cel between June 24, 2024, and November 19, 2025 (≥100 days prior to data cut-off), were included
  - 86.8% in Germany, 9.4% in Brazil, 3.8% in Austria
  - No patients from Spain were enrolled during this time period
- At data cut-off, the median follow-up was 8.0 months (range, 1.5–20.2)
- Baseline characteristics are in **Table 1**
  - The median age was 64 years; 29.2% were aged ≥70 years
  - 15/50 (30.0%) patients had high-risk cytogenetics; 29/82 (35.4%) had stage III disease (by International Staging System [ISS] or Revised-ISS)
  - 22 (20.8%) patients received holding therapy
  - Median pLOT was 2 and 57 (53.8%) patients had 1–2 pLOT
  - 99 (93.4%) patients had prior exposure to anti-CD38 antibodies

Table 1: Baseline characteristics

Characteristics		Patients (N=106)
Age	Median (range), years	64.0 (41–81)
	≥70 years, n (%)	31 (29.2)
Sex	Female, n (%)	35 (33.0)
	Male, n (%)	71 (67.0)
ECOG PS	Available, n	98
	0–1, n (%)	88 (89.8)
	≥2, n (%)	10 (10.2)
ISS/Revised-ISS stage[a]	Available, n	82
	I, n (%)	30 (36.6)
	II, n (%)	23 (28.0)
	III, n (%)	29 (35.4)
Cytogenetic risk	Available, n	50
	Standard risk, n (%)	21 (42.0)
	High risk,[b] n (%)	15 (30.0)
	Inconclusive, n (%)	14 (28.0)
EMD[a,c]	Yes, n (%)	2 (1.9)
	No, n (%)	104 (98.1)
Time since diagnosis	Median (range), years	4.8 (0.4–24.0)
Holding therapy	Yes, n (%)	22 (20.8)
pLOT[d]	Median (range)	2 (1–9)
	1–2, n (%)	57 (53.8)
Prior therapy[e]	Triple-class exposure,[f] n (%)	98 (92.5)
	Penta-drug exposure,[g] n (%)	34 (32.1)
	Anti-CD38 antibodies, n (%)	99 (93.4)
	Bispecific antibodies, n (%)	13 (12.3)
	Teclistamab	4 (3.8)
	Talquetamab	11 (10.4)
	ADC (belantamab mafodotin), n (%)	1 (0.9)
	CAR-T cell therapy, n (%)	5 (4.7)
Prior transplantation	Yes, n (%)	87 (82.1)
	Allogeneic	5 (4.7)
	Autologous	83 (78.3)
OOS product use	Yes, n (%)	9 (8.5)
	No, n (%)	97 (91.5)

[a]Most recent status prior to cilta-cel infusion. [b]High cytogenetic risk was defined as having ≥1 of the following: [del(17p) and/or TP53 mutation], [t(4;14), t(14;16), or t(14;20) plus +1q and/or del(1p32)], and/or [monoallelic del(1p32) with +1q, or biallelic del(1p32)]. [c]EMD denotes soft tissue plasmacytoma that was not contiguous with bone. [d]Median number of pLOT was 2 and the n (%) of early-line patients were the same, both with or without considering holding therapy as pLOT. [e]Prior exposures included holding therapy. [f]Exposed to PI, IMiD, and anti-CD38 antibodies. [g]Exposed to at least 2 PIs, 2 IMiDs, and 1 anti-CD38 agent. ADC, antibody-drug conjugate; CAR, chimeric antigen receptor; ECOG PS, Eastern Cooperative Oncology Group performance status; EMD, extramedullary disease; OOS, out of specification.

- 80 (75.5%) patients received bridging therapy (BT); 42 had post-BT response data, 31/42 (73.8%) had partial response (PR) or better to BT (**Table 2**)

Table 2: Bridging therapy

	Patients (N=80)
Duration of BT, median (range), weeks	6.4 (0–24)
Common BT agents	80 (100)
Dexamethasone	54 (67.5)
Carfilzomib	34 (42.5)
Daratumumab	27 (33.8)
Talquetamab	21 (26.3)
Pomalidomide	17 (21.3)
Evaluable response to BT,[a] n	42
PR or better	31 (73.8)

Values are n (%) unless otherwise stated. [a]Most recent assessment prior to cilta-cel infusion.

Safety

- 105 (99.1%) patients had ≥1 AEs
- The most common AEs were cytokine release syndrome (CRS), neutropenia, and hypogammaglobulinemia; additionally, infections and infestations occurred in 50 (47.2%) patients. 38 (35.8%) had serious AEs (**Table 3**)
- 9 (8.5%) patients had grade 5 AEs (**Table 3**), including 1 patient with disease progression reported as a grade 5 AE

Table 3: Most common/frequent AEs

	All Grade (N=106)	Grade 3/4 (N=106)	Grade 5 (N=106)
Patients with ≥1 AEs, n (%)	105 (99.1)	54 (50.9)	9 (8.5)[a]
Most common AEs, n (%)			
CRS	65 (61.3)	1 (0.9)	0
Infections and infestations[b]	50 (47.2)	20 (18.9)	3 (2.8)
Pneumonia[c]	9 (8.5)	7 (6.6)	0
Neutropenia	27 (25.5)	22 (20.8)	0
Hypogammaglobulinemia	21 (19.8)	0	0
Any serious AEs, n (%)	38 (35.8)	–	–
Infections and infestations[b,d]	18 (17.0)	14 (13.2)	3 (2.8)

AEs include new events with onset after cilta-cel infusion. [a]Includes 1 patient with disease progression reported as grade 5 AE. [b]Represents an SOC per Medical Dictionary for Regulatory Activities. [c]Most common infection. [d]Most common SAE. SOC, system organ class.

- Immune effector cell–associated neurotoxicity syndrome (ICANS) was reported in 11 (10.4%) patients and was primarily low grade (**Table 4**)
- A total of 17 cranial nerve palsies (CNPs) occurred in 15 (14.2%) patients (**Table 4**)
  - 2/15 patients experienced 2 separate events of CNP
  - At the time of data cut-off, 82.4% (14/17) of the CNP events were resolved or resolving and 17.6% (3/17) were ongoing
- No hematologic SPMs occurred; 1 gastric carcinoma and 2 nonmelanoma skin cancers were reported (**Table 4**)
- 2 (1.9%) patients had grade 3 immune effector cell (IEC)-parkinsonism (**Table 4**)
  - Both were heavily pretreated with ≥3 pLOT
  - One of the patients had primary refractory disease
  - Both events were ongoing at data cut-off

Table 4: AEs of interest

	All Grade (N=106)	Grade 1/2 (N=106)	Grade ≥3 (N=106)
ICANS, n (%)	11 (10.4)	10 (9.4)	1 (0.9)[a]
Non-ICANS neurologic events, n (%)	19 (17.9)[b]		
IEC-parkinsonism	2 (1.9)[c]	0	2 (1.9)[c]
CNP	15 (14.2)[d]	9 (8.5)	5 (4.7)[e]
Guillain-Barré syndrome	1 (0.9)	0	1 (0.9)[f]
Peripheral neuropathy	3 (2.8)	3 (2.8)	0
Other neurologic disorders, n (%)	14 (13.2)	9 (8.5)	5 (4.7)
SPM, n (%)	3 (2.8)	1 (0.9)	2 (1.9)
Hematologic SPM	0	0	0
Gastric carcinoma	1 (0.9)	0	1 (0.9)[g]
Nonmelanoma skin cancers	2 (1.9)[h]	1 (0.9)	1 (0.9)

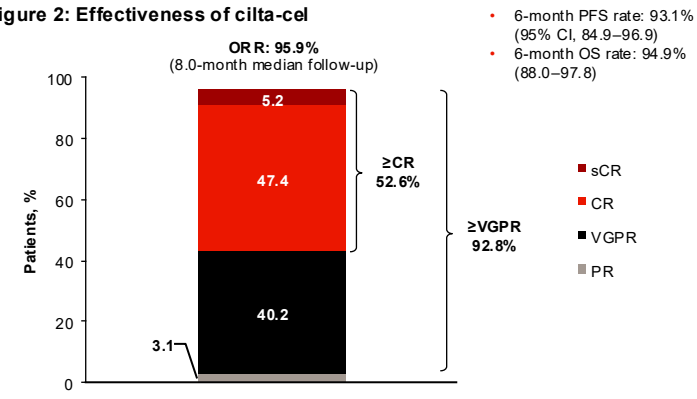
AEs include new events with onset after cilta-cel infusion. [a]Grade 3. [b]Patients could have ≥1 events. [c]Heavily pretreated with ≥3 pLOT for both. Grade 3 for both. [d]1 CNP event was reported without a toxicity grade. [e]All grade 3. [f]Reported as polyradiculoneuropathy. [g]Fatal on day 162. [h]1 grade 2; 1 grade 3.

- 9 (8.5%) patients died
  - 1 death was due to disease progression
  - 8 (7.5%) were due to AEs; 4 (3.8%) due to treatment-emergent AEs
    - 3 due to treatment-related AEs (1 brain stem encephalitis, day 318; 1 multiorgan failure, day 77; 1 bacterial sepsis, day 49)
- Prophylaxis medications were used for 96 (90.6%) patients, including antivirals (74.5%), antibiotics (69.8%), antifungals (39.6%), intravenous immunoglobulin (14.2%), growth factor (9.4%), and corticosteroids (7.5%)

Effectiveness of cilta-cel

- Of 106 patients, 97 had response data available (**Figure 2**)
  - ORR: 95.9% (93/97; 95% CI, 89.8–98.9)
  - Very good partial response (VGPR) or better: 92.8% (90/97; 95% CI, 85.7–97.0)
  - Complete response (CR) or better: 52.6% (51/97; 95% CI, 42.2–62.8)

Figure 2: Effectiveness of cilta-cel



- 6-month PFS rate: 93.1% (95% CI, 84.9–96.9)
- 6-month OS rate: 94.9% (88.0–97.8)

An updated data set with same cut-off date was used for ORR, 6-month PFS, and prophylaxis. sCR, stringent CR.

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Multiple Myeloma

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