Integrated Safety Analysis of Tagraxofusp, a CD123-Directed Targeted Therapy, in Patients With Hematologic Malignancies

AML-397

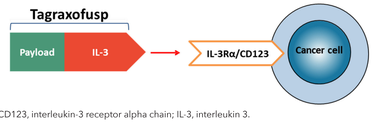
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BACKGROUND

- The interleukin-3 receptor alpha chain (CD123), a cell-surface target aberrantly expressed in various hematologic malignancies, plays multiple biologic roles and is involved in the control of malignant hematopoiesis, innate as well as adaptive immunity, and the inflammatory response[1]
- CD123 is an attractive therapeutic target for blastic plasmacytoid dendritic cell neoplasm (BPDCN), acute myeloid leukemia (AML), chronic myelomonocytic leukemia (CMML), myelofibrosis (MF), and multiple myeloma due to its differential overexpression in these malignancies[1,2]
- Tagraxofusp (TAG), a first-in-class CD123-targeted agent,[3] is the only treatment approved by the US Food and Drug Administration (December 2018)[4,5] and European Medicines Agency (January 2021)[6] for patients with BPDCN
  - TAG is a recombinant human interleukin-3 fused to a truncated diphtheria toxin payload, which is internalized in CD123-expressing cells and irreversibly blocks protein synthesis, inducing cell death

Figure 1. Tagraxofusp – Novel CD123-Targeted Therapy



CD123, interleukin-3 receptor alpha chain; IL-3, interleukin 3.

(**Figure 1**)

- In the pivotal study of TAG in patients with BPDCN, substantial efficacy and a manageable safety profile were demonstrated[7], leading to FDA approval of the biologics license application
  - An overall response (partial response or better) was achieved in 75% of first-line patients; complete response (CR)/clinical CR (CRc, CR with residual skin abnormality not indicative of active disease): 57%
  - Fifty-one percent of patients who achieved CR/CRc successfully bridged to stem cell transplant
  - The most commonly reported adverse events (AEs; >50%) were increased alanine aminotransferase (ALT), increased aspartate aminotransferase (AST), and hypoalbuminemia
  - Capillary leak syndrome (CLS), was reported in 21% of patients but was manageable with risk-mitigation strategies and early intervention
- TAG has also been investigated in several phase 1/2 clinical trials for AML, CMML, and MF[8]
- Herein, we report the aggregated safety data of TAG monotherapy from trials in BPDCN, AML, CMML, and MF

METHODS

- An integrated safety analysis was performed for patients who received TAG monotherapy in three phase 1/2, multicenter clinical studies: Study 0114 (NCT02113982; BPDCN/AML), Study 0214 (NCT02270463; AML), and Study 0314 (NCT02268253; CMML/MF)
  - The study design and characteristics of these clinical trials are described in **Table 1**

Table 1. Design and Characteristics of TAG Clinical Studies

Study Identifier/ Patient Diagnosis	Type of Study	Dosage Regimen
Study 0114 BPDCN or AML	▶ Open label ▶ Multistage ▶ Dose escalation/ expansion	**Stage 1 (dose escalation):** Patients treated at 7, 9, 12, or 16 mcg/kg (16 mcg/kg evaluated only in patients with AML) **Stage 2 (expansion):** Patients treated at 12 mcg/kg **Stage 3 (pivotal cohort):** Patients (first-line BPDCN) treated at 12 mcg/kg **Stage 4 (continued access):** Patients (BPDCN) treated at 12 mcg/kg Enrollment and long-term follow-up completed; study is closed All stages: 21-d therapy cycle; 15-minute IV infusion d1-5
Study 0214 AML	▶ Nonrandomized ▶ Open label ▶ 2-stage dose escalation	**Stage 1 (dose escalation):** Patients treated at 7, 9, or 12 mcg/kg **Stage 2 (expansion):** Additional patients treated at 12 mcg/kg Enrollment discontinued for practical (not safety-related) reasons; study is closed 28-d therapy cycle; 15-minute IV infusion for 5 consecutive d
Study 0314 CMML or MF		**Stage 1 (dose escalation):** Patients treated at 7, 9, or 12 mcg/kg **Stage 2 (expansion):** Additional patients with MF and CMML treated at 12 mcg/kg **Stage 3a:** Additional patients with CMML will be treated at 12 mcg/kg 21-day (cycles 1–4) or 28-day (cycles ≥5*) therapy cycle 15-minute IV infusion d1-3 of 21/28-d cycle

*As of a recent protocol amendment.
AML, acute myeloid leukemia; BPDCN, blastic plasmacytoid dendritic cell neoplasm; CMML, chronic myelomonocytic leukemia; CR, complete response; d, day; IV, intravenous; MF, myelofibrosis; minimal residual disease; TAG, tagraxofusp.

- Patients who received the recommended phase 2 dose of 12 mcg/kg IV on days 1–3 (MF and CMML) or days 1–5 (BPDCN and AML) were analyzed for incidence and timing of treatment-related AEs (TRAEs), AEs of interest, and discontinuations
  - AEs (≤30 days after the last TAG infusion) were summarized by Medical Dictionary for Regulatory Activities (MedDRA) version 13.1 (or higher) System Organ Class and preferred term with the incidence and percentages of patients experiencing each AE summarized with descriptive statistics
  - AEs were also summarized in accordance with National Cancer Institute Common Terminology Criteria for Adverse Events version 4.03 (or higher) grade and relationship to study drug
- Safety data for TAG monotherapy, reported as individual case study reports (ICSRs) postapproval (US) and from an early access program (EAP; Europe), were also collected

RESULTS

- In total, 201 patients receiving TAG monotherapy (12 mcg/kg) in a clinical trial setting were included: BPDCN, n=86; AML, n=46; CMML, n=33; MF, n=36
- As of July 2021, 11 (6%) patients discontinued treatment with TAG monotherapy due to TRAEs of any grade
- Of the overall 201 patients, 86% (n=173) reported at least 1 TRAE (**Table 2**)
  - Most common (any-grade; ≥25%) included hypoalbuminemia (n=82; 41%), increased ALT (n=81; 40%), increased AST (n=79; 39%), and thrombocytopenia (n=53; 26%)
- More than half of the total patients (54%; 109/201) reported at least 1 grade ≥3 TRAE; most common (≥15%) were thrombocytopenia (n=41; 20%), increased AST (n=40; 20%), and increased ALT (n=35; 17%)

TAG has a predictable and manageable safety profile in patients with BPDCN, AML, CMML and MF

Table 2. Most Common Treatment-Related AEs (≥15%, Any-Grade; ≥5%, Grade ≥3) Summary: Overall and by Indication/Study – TAG Monotherapy 12 mcg/kg (Safety Population)

MedDRA Preferred Term, n (%)	Study 0114 BPDCN (n=86)		Study 0114 AML (n=36)		Study 0214 AML (n=10)		Study 0314 CMML (n=33)		Study 0314 MF (n=36)		Total (N=201)	
	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3
Any	**77** (90)	**54** (63)	**31** (86)	**18** (50)	**10** (100)	**9** (90)	**26** (79)	**16** (49)	**29** (81)	**12** (33)	**173** (86)	**109** (54)
Hypoalbuminemia	**40** (47)	**1** (1)	**19** (53)	**1** (3)	**6** (60)	**0**	**9** (27)	**0**	**8** (22)	**1** (3)	**82** (41)	**3** (2)
Increased ALT	**44** (51)	**24** (28)	**20** (56)	**6** (17)	**5** (50)	**5** (50)	**6** (18)	**0**	**6** (17)	**0**	**81** (40)	**35** (17)
Increased AST	**43** (50)	**25** (29)	**22** (61)	**9** (25)	**5** (50)	**4** (40)	**4** (12)	**1** (3)	**5** (14)	**1** (3)	**79** (39)	**40** (20)
Thrombocytopenia	**30** (35)	**22** (26)	**5** (14)	**5** (14)	**5** (50)	**4** (40)	**8** (24)	**7** (21)	**5** (14)	**3** (8)	**53** (26)	**41** (20)
Pyrexia	**24** (28)	**0**	**7** (19)	**0**	**5** (50)	**0**	**5** (15)	**0**	**6** (17)	**0**	**47** (23)	**0**
Nausea	**19** (22)	**0**	**10** (28)	**0**	**4** (40)	**0**	**9** (27)	**1** (3)	**4** (11)	**0**	**46** (23)	**1** (1)
Increased weight	**23** (27)	**0**	**5** (14)	**0**	**2** (20)	**0**	**6** (18)	**0**	**2** (6)	**0**	**38** (19)	**0**
Capillary leak syndrome	**18** (21)	**6** (7)	**6** (17)	**1** (3)	**2** (20)	**2** (20)	**6** (18)	**3** (9)	**3** (8)	**2** (6)	**35** (17)	**14** (7)
Chills	**17** (20)	**1** (1)	**5** (14)	**0**	**4** (40)	**0**	**3** (9)	**0**	**5** (14)	**0**	**34** (17)	**1** (1)
Fatigue	**16** (19)	**1** (1)	**8** (22)	**2** (6)	**2** (20)	**0**	**3** (9)	**0**	**3** (8)	**1** (3)	**32** (16)	**4** (2)
Hypotension	**15** (17)	**3** (4)	**7** (19)	**1** (3)	**2** (20)	**0**	**4** (12)	**3** (9)	**4** (11)	**0**	**32** (16)	**7** (4)
Anemia	**13** (15)	**6** (7)	**3** (8)	**2** (6)	**1** (10)	**1** (10)	**8** (24)	**5** (15)	**2** (6)	**2** (6)	**27** (13)	**16** (8)
Neutropenia	**10** (12)	**7** (8)	**2** (6)	**2** (6)	**2** (20)	**1** (10)	**2** (6)	**2** (6)	**1** (3)	**0**	**17** (9)	**12** (6)

AEs were coded using MedDRA version 19.0. If a patient experienced >1 event with a given preferred term, patient was counted only once for that preferred term.
AE, adverse event; ALT, alanine aminotransferase; AML, acute myeloid leukemia; AST, aspartate aminotransferase; BPDCN, blastic plasmacytoid dendritic cell neoplasm; CMML, chronic myelomonocytic leukemia; d, day; Gr, grade; MedDRA, Medical Dictionary for Regulatory Activities; MF, myelofibrosis; TAG, tagraxofusp.

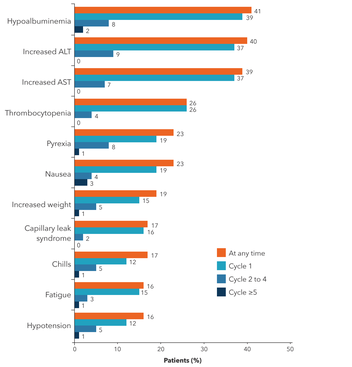
- The most common hematologic TRAE (any-grade and grade ≥3) was thrombocytopenia (**Table 3**)
  - All events were transient in nature; prolonged bone marrow suppression was not observed
- Overall, 23% (47/201) of patients experienced ≥1 treatment-related serious AE
- Irrespective of grade, the onset of most TRAEs commonly occurred in cycle 1 and resolved by cycle 2 (**Figure 2**)
  - The incidence rates of common TRAEs substantially decreased in subsequent cycles, although the number of patients in cycles ≥2 was lower than in cycle 1
  - Tumor lysis syndrome was infrequent and occurred in 5% (n=10) of patients and 2% (n=3) had infusion-related reactions
- CLS occurred in 17% (n=35) of the total 201 patients (**Table 4**)
- Onset of CLS was usually within the first week of cycle 1 and resolved quickly; no patients experienced the first onset after cycle 2
- The majority of all CLS events were nonsevere (grade ≤2 **Figure 3**); 2 of 201 (1%) patients with BPDCN experienced a grade 5 event
- Only 1 of the 15 patients who resumed treatment after an initial CLS event experienced a recurring event
- CLS, disease progression, pyrexia, blood albumin decreased, and liver function test increased were the most common AEs reported as ICSRs postapproval and from the EAP (December 2018 to September 2021)
- No new safety signals that would alter the safety profile were observed

Table 3. Any-Grade and Grade ≥3 Hematologic Treatment-Related AEs Summary: Overall and by Indication/Study – TAG Monotherapy 12 mcg/kg (Safety Population)

MedDRA Preferred Term, n (%)	Study 0114 BPDCN (n=86)		Study 0114 AML (n=36)		Study 0214 AML (n=10)		Study 0314 CMML (n=33)		Study 0314 MF (n=36)		Total (N=201)	
	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3	Any Gr	Gr ≥3
Thrombocytopenia	**30** (35)	**22** (26)	**5** (14)	**5** (14)	**5** (50)	**4** (40)	**8** (24)	**7** (21)	**5** (14)	**3** (8)	**53** (26)	**41** (20)
Anemia	**13** (15)	**6** (7)	**3** (8)	**2** (6)	**1** (10)	**1** (10)	**8** (24)	**5** (15)	**2** (6)	**2** (6)	**27** (13)	**16** (8)
Neutropenia	**10** (12)	**7** (8)	**2** (6)	**2** (6)	**2** (20)	**1** (10)	**2** (6)	**2** (6)	**1** (3)	**0**	**17** (9)	**12** (6)
Leukopenia	**9** (11)	**7** (8)	**2** (6)	**2** (6)	**0**	**0**	**2** (6)	**1** (3)	**1** (3)	**0**	**14** (7)	**10** (5)
Febrile neutropenia	**2** (2)	**2** (2)	**3** (8)	**3** (8)	**0**	**0**	**0**	**0**	**1** (3)	**1** (3)	**6** (3)	**6** (3)
Lymphopenia	**4** (5)	**3** (4)	**0**	**0**	**1** (10)	**1** (10)	**1** (3)	**0**	**0**	**0**	**6** (3)	**4** (2)
Leukocytosis	**1** (1)	**0**	**0**	**0**	**0**	**0**	**4** (12)	**1** (3)	**0**	**0**	**5** (3)	**1** (1)

AEs were coded using MedDRA version 19.0. If a patient experienced >1 event with a given preferred term, patient was counted only once for that preferred term.
AE, adverse event; AML, acute myeloid leukemia; BPDCN, blastic plasmacytoid dendritic cell neoplasm; CMML, chronic myelomonocytic leukemia; d, day; Gr, grade; MedDRA, Medical Dictionary for Regulatory Activities; MF, myelofibrosis; TAG, tagraxofusp.

Figure 2. Most Common Treatment-Related AEs (≥15%, Any-Grade) by Cycle: Overall, N=201 – TAG Monotherapy 12 mcg/kg (Safety Population)



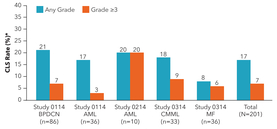
Adverse events were coded using MedDRA version 19.0. If a patient experienced >1 event with a given preferred term, patient was counted only once for that preferred term.
AE, adverse event; ALT, alanine aminotransferase; AST, aspartate aminotransferase; d, day; MedDRA, Medical Dictionary for Regulatory Activities; TAG, tagraxofusp.

Table 4. Summary of Treatment-Related CLS Events: Overall and by Indication/Study – TAG Monotherapy 12 mcg/kg (Safety Population)

	Study 0114 BPDCN (n=86)	Study 0114 AML (n=36)	Study 0214 AML (n=10)	Study 0314 CMML (n=33)	Study 0314 MF (n=36)	Total (N=201)
CLS any-grade, *n* (%)	**18** (21)	**6** (17)	**2** (20)	**6** (18)	**3** (8)	**35** (17)
Resumed treatment, *n* (%)*	**9** (50)	**1** (17)	**1** (50)	**3** (50)	**1** (33)	**15** (43)
Recurring events†	**0**	**0**	**1** (100)	**0**	**0**	**1** (7)
Median time to any-grade, days (range)	**6.0** (3-51)	**5.0** (4-29)	**9.0** (1-41)	**5.5** (2-26)	**5.0** (2-8)	**5.5** (1-51)
Time to resolution	**5.0** (2-69)	**7.0** (5-28)	**4.0** (3-4)	**6.0** (2-9)	**7.0** (5-12)	**5.0** (2-69)
Median time to grade ≥3, days (range)	**8.0** (4-12)	**4.0** (4-4)	**25.0** (9-41)	**7.0** (4-26)	**6.5** (5-8)	**8.0** (4-41)
Time to resolution	**7.0** (2-69)	**8.0** (8-8)	**3.5** (3-4)	**4.0** (4-4)	**9.5** (7-12)	**6.0** (2-69)

*Percentages are based on number of patients who experienced a CLS any-grade event.
†Percentages are based on total number of patients who resumed treatment.
AML, acute myeloid leukemia; BPDCN, blastic plasmacytoid dendritic cell neoplasm; CLS, capillary leak syndrome; CMML, chronic myelomonocytic leukemia; d, day; MF, myelofibrosis; TAG, tagraxofusp; TRAE, treatment-related adverse event.

Figure 3. Summary of Treatment-Related Any-Grade and Grade ≥3 CLS Events: Overall and by Indication/Study – TAG Monotherapy 12 mcg/kg (Safety Population)



*Percentages are based on total number of patients.
AML, acute myeloid leukemia; BPDCN, blastic plasmacytoid dendritic cell neoplasm; CLS, capillary leak syndrome; CMML, chronic myelomonocytic leukemia; d, day; MF, myelofibrosis; TAG, tagraxofusp; TRAE, treatment-related adverse event.

CONCLUSIONS

- This integrated analysis represents the largest collation of safety data following treatment with TAG monotherapy in the 3 years following US approval
- TAG has an established and manageable safety profile, with the vast majority of AEs reported as transient and decreasing in frequency/ severity with increasing cycles
- No myelosuppression or cumulative toxicity were observed over multiple treatment cycles
- CLS onset was usually within cycle 1 (no patients experienced first onset after cycle 2) and median time to resolution was 6 days using early intervention measures including holding treatment
- The safety profile observed postapproval and in the EAP was consistent with reported clinical trial data, reflecting successful adoption of risk-management strategies in real-world practice

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Biomedicines. 2019;7(1):6. **Author DISCLOSURES: Naveen Pemmaraju:** Consultancy: Pacylex Pharmaceuticals, ImmunoGen, Inc., Bristol Myers Squibb Co., Blueprint Medicines, ClearView Healthcare Partners, Protagonist Therapeutics, Inc., Affymetrix, Incyte, Novartis Pharmaceuticals, LFB Biotechnologies, Stemline Therapeutics, Inc., Celgene Corporation, AbbVie Pharmaceuticals, MustangBio, Roche Diagnostics, DAVA Oncology, Aptitude Health, CareDx, Inc.; Other: Research Support and Research Funding: Novartis Pharmaceuticals, Research Funding: Affymetrix, Membership on an entity's Board of Directors or advisory committees: Stemline Therapeutics, Inc., AbbVie Pharmaceuticals, ADH Communications, Blasts Hematology, Springer Science+Business Media, SagerStrong Foundation, **Hagop M. 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