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Literature Analysis Characterizing Oncology and Hematology Early Access Program Publications

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Objective:

- We assessed the publication landscape reporting data from oncology and hematology early access programs (EAPs) to characterize several parameters including industry sponsorship, publication type, indication, sample size, data type, and study design

Conclusions:

- Most EAP publications were not industry-sponsored
- Most sponsored publications reported outcomes related to patient characteristics, efficacy, and safety; noted retrospective study designs; and did not mention having a protocol
- We recommend the development of guidelines for publishing outcomes from EAP studies to standardize the parameters reported in these publications

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Introduction

- EAPs, which include expanded access, compassionate use, named patient, and managed access programs, allow access to investigational drugs for patients with high unmet needs who are ineligible for clinical trials
- There is limited guidance from Good Publication Practice (GPP) and regulatory agencies for publishing data from EAPs
  - GPP guidelines stipulate that patient data must be anonymized[1]
  - Food and Drug Administration guidance related to EAP studies focuses on processes for obtaining approval for EAPs and for reporting data to regulatory bodies with no specific guidance on publication of EAP data[2]
- Furthermore, there is limited literature characterizing the outcomes reported in publications from EAP studies[3]
- We assessed the publication landscape reporting data from oncology and hematology EAPs to characterize several parameters including industry sponsorship, publication type, indication, sample size, data type, and study design

Methods

- We searched Embase and PubMed on August 15, 2024, using a predefined search string limited to the last 3 years
  - **Search string:** ("early access program" or "expanded access program" or "compassionate use program" or "managed access program" or "named patient program") AND ("leukemia" or "lymphoma" or "cancer")
- Publications were excluded if they were duplicates, errata, open-label extension trials, or reviews, and if they studied an unrelated disease state or did not mention an EAP term in the abstract
- The abstract from each publication was assessed for the parameters listed in **Table 1**
- A subset of journal articles was analyzed to determine trends in the parameters among the publications with the most common indications from the overall analysis
  - Non-small cell lung cancer (NSCLC, n=27)
  - Multiple myeloma (MM, n=9)
  - Diffuse large B-cell lymphoma (DLBCL, n=6)

Table 1. Parameters assessed

Parameters assessed in all publications	Additional parameters assessed in sponsored publications
Industry sponsorship[a] (yes/no)	Congress
Publication type (manuscript or abstract)	Study drug
Journal	Sample size
Indication	Data type (ie, patient characteristics, efficacy, safety, treatment patterns, PD, PK)
Geographic region	Study design (prospective/retrospective/ambispective[b])

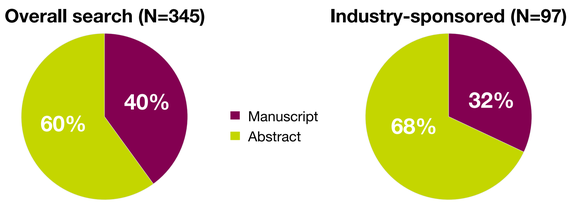
[a]Industry-sponsored publications were defined as those that received funding from a sponsor company related to publication of the manuscript/abstract and/or had authors affiliated with the sponsor company. [b]Both prospective and retrospective. PD, pharmacodynamics; PK, pharmacokinetics.

Results and interpretation

Disposition and publication type

- Of 482 citations retrieved, 345 (60% abstracts; 40% manuscripts) were included overall (**Figure 1**)
- Of the 137 citations that were excluded:
  - 53 reported outcomes in an unrelated disease state
  - 37 were duplicates
  - 22 did not mention an EAP term in the abstract or did not contain data from an EAP
  - 11 were reviews
  - 10 did not report data directly from an EAP
  - 2 were errata
  - 2 were open-label extension trials
- Industry-sponsored publications comprised 28% (97/345) of the results (**Figure 1**)

Figure 1. Overall and industry-sponsored manuscripts and abstracts reporting EAP outcomes



EAP, early access program.

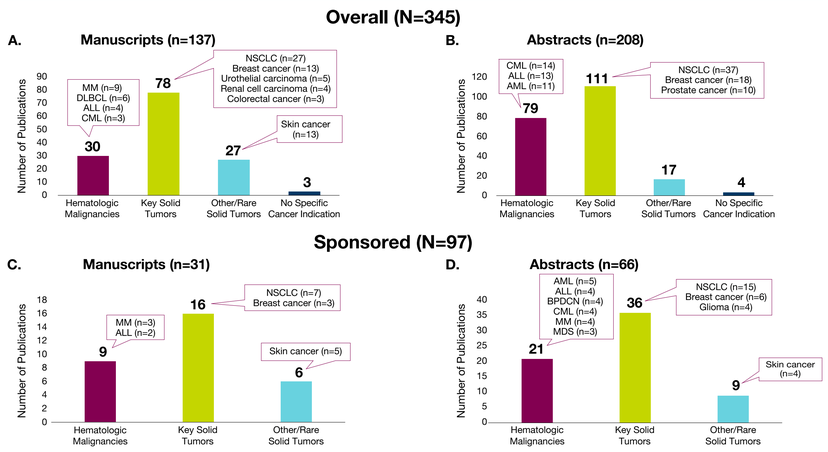
Journals and congresses

- There was a wide variety of journals and congresses represented among the overall and sponsored publications (**Supplemental Figures 1–3**)
  - Most were indication-specific
  - Some of the more common journals represented in the overall search were not represented among the industry-sponsored publications

Indications

- Among all publication types, NSCLC was the most frequent indication (64/345; 19%) (**Figure 2**)

Figure 2. Indications in overall (A and B) and industry-sponsored (C and D) publications reporting EAP outcomes

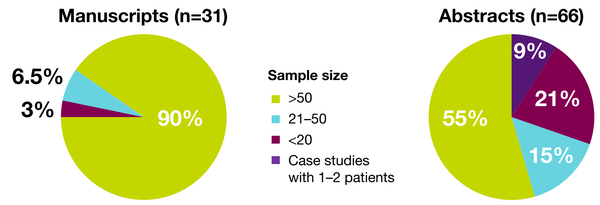


Publications were counted more than once if they included more than one indication category. For the overall analysis (panels A and B), callouts are included for indications with >2 publications for manuscripts and ≥10 publications for abstracts. For the industry-sponsored publications (panels C and D), callouts are included for indications with >1 publication for manuscripts and >2 publications for abstracts.
ALL, acute lymphoblastic leukemia; AML, acute myeloid leukemia; BPDCN, blastic plasmacytoid dendritic cell neoplasm; CML, chronic myeloid leukemia; DLBCL, diffuse large B-cell lymphoma; EAP, early access program; MDS, myelodysplastic syndrome; MM, multiple myeloma; NSCLC, non-small cell lung cancer.

Sample size

- There were no industry-sponsored manuscripts that contained case reports from patients treated in an EAP (**Figure 3**)

Figure 3. Sample sizes in industry-sponsored publications reporting EAP outcomes (N=97)

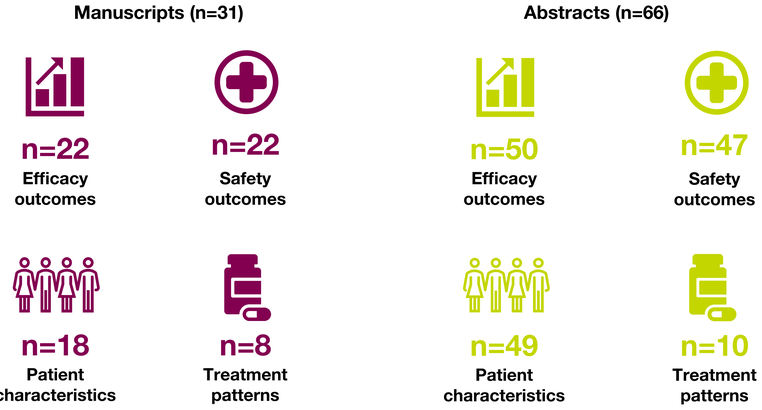


EAP, early access program.

Data type

- Most industry-sponsored publications (manuscripts and abstracts combined) reported efficacy outcomes (72/97; 74%), safety outcomes (69/97; 71%), patient characteristics (67/97; 69%), and treatment patterns (18/97; 19%) (**Figure 4**)
- ≤2% of publications reported data on treatment duration/predictors of treatment duration, genetic profiling, molecular analyses, healthcare resource utilization, biomarkers, pharmacokinetics, or pharmacodynamics

Figure 4. Most common parameters reported from EAP industry-sponsored publications (N=97)



Publications were counted more than once if they included more than one type of data. EAP, early access program.

Study design

- Most publications (52/97; 54%) did not mention having a protocol; most of these "non-protocol" studies were conducted in Europe and the United States (**Table 2**)

Table 2. Study design in industry-sponsored publications reporting EAP outcomes (N=97)

	Manuscripts (n=31)			Abstracts (n=66)		
	Total	Study design	Region[a]	Total	Study design	Region[a]
Protocol	n=9	Retrospective, n=5 Prospective, n=3 Ambispective, n=1	Japan, n=3 Global, n=2	n=18[b]	Retrospective, n=10 Ambispective, n=5 Prospective, n=2 Not specified, n=3	Global, n=8 Europe, n=5 France, n=4
No protocol[c]	n=21	Retrospective, n=11 Prospective, n=9 Ambispective, n=1	Italy, n=5 Europe, n=4 France, n=4	n=31	Retrospective, n=11 Prospective, n=6 Ambispective, n=2 Not specified, n=12	United States, n=9 France, n=8 Global, n=3 Italy, n=3 Spain, n=3 Russia, n=2
Pooled data[d]	n=0	NA		n=11	Retrospective, n=2 Prospective, n=1 Not specified, n=8	
Case reports	n=1[e]	NA		n=6	NA	

Ambispective, both prospective and retrospective. [a]Regions with >1 publication. [b]Two publications reported EAP data from a retrospective and ambispective study and were counted once for each type of study. [c]Publication contained only data collected from an EAP but did not indicate that there was a protocol detailing the study outcomes. [d]Publication contained data from an EAP and another source (ie, postmarketing or clinical trial). [e]Case series including >2 patients. EAP, early access program; NA, not applicable.

Key Trends in NSCLC, MM, and DLBCL

- An in-depth review of manuscripts reporting outcomes in the most common indications found that most publications reported outcomes in relapsed/refractory disease (29/42; 69%) and were not industry-sponsored (32/42; 76%) (**Table 3**)

Table 3. Summary of key trends in NSCLC, MM, and DLBCL

	No. of early access drugs or drug combinations[a]	Line of therapy[b]		No. of publications that obtained data from a registry	No. of industry-sponsored publications	No. of publications citing EAPs with an NCT#
		No. with 1L+ data	No. with 2L+ data			
NSCLC (n=27)	13	8	14	1	7	8
MM (n=9)	4	0	9	1	3	1
DLBCL (n=6)	1	0	6	0	0	0

[a]NSCLC: durvalumab (n=4), lorlatinib (n=4), osimertinib (n=4), pralsetinib (n=3), sotorasib (n=3), afatinib (n=1), alectinib (n=1), amivantamab (n=1), brigatinib (n=1), capmatinib (n=1), mobocertinib (n=1), nivolumab (n=1), selpercatinib (n=1); MM: belantamab mafodotin (n=3), idecabtagene vicleucel (n=2), ixazomib, lenalidomide, and dexamethasone (n=2), ixazomib (n=1), teclistamab (n=1); DLBCL, polatuzumab vedotin, bendamustine, and rituximab (Pola-BR; n=6). [b]The values for 1L+ and 2L+ do not add up to the total n=27 because 1 NSCLC publication reported outcomes with osimertinib as neoadjuvant therapy in a patient with stage IIIA NSCLC and 4 NSCLC publications reported outcomes with consolidation therapy. 1L, first line; 2L, second line; DLBCL, diffuse large B-cell lymphoma; MM, multiple myeloma; NSCLC, non-small cell lung cancer.

Limitations

- Since the parameters assessed in this analysis came from metadata that included only the abstract for each publication, there may have been additional outcomes or study design details reported in the full text manuscripts that were not captured in this analysis
- Based on our criteria for identifying industry-sponsored publications (those that received funding from a sponsor company related to publication of the manuscript/abstract and/or had authors affiliated with the sponsor company), the number of industry-sponsored publications may be underrepresented in our analysis

References

1. DeTora LM, Toroser D, Sykes A, et al. Good Publication Practice (GPP) guidelines for company-sponsored biomedical research: 2022 update. *Ann Intern Med*. 2022;175(9):1298-1304.

2. US Food and Drug Administration. Expanded access, information for industry. https://www.fda.gov/news-events/expanded-access/expanded-access-information-industry#Sponsors. Accessed January 10, 2025.

3. Miller JE, Ross JS, Moch KI, Caplan AL. Characterizing expanded access and compassionate use programs for experimental drugs. *BMC Res Notes*. 2017;10(1):350.

Declaration of Funding

AK: OPEN Health Employee; CG: OPEN Health Employee; JB: AstraZeneca Employee