Cost assessment in melanoma clinical trials: A cross-sectional study

To the Editor: Melanoma is the subject of intensive clinical trial investigation, with >600 trials recruiting patients in 2022.[1] Although trials are designed to evaluate the efficacy and safety of novel interventions, costs are critical in determining their feasibility and scalability.[2] Pharmaceutical companies and study sponsors can demonstrate the early economic value of interventions by including financial end points in trials.[3] Despite the recommendation to fully integrate economic data collection into clinical trials,[4] the inclusion of economic end points remains limited.[3,5] Trials assessing specialty oncology medications are particularly underrepresented in trials that include economic end points.[3] To optimize high-value care, we assessed the prevalence and trial characteristics of cost end points in melanoma clinical trials.

A comprehensive search was conducted using ClinicalTrials.gov, a database of trials worldwide, for melanoma studies that mentioned cost using relevant inclusion terms (ie, "melanocytic neoplasm"). Each trial was manually searched for how cost was incorporated (ie, trial description, outcome measure). The data collected included study name, study type, trial start date, duration, completion status, total enrollment, participant age, funding type, country, and cost outcomes.

As of November 2022, 3014 melanoma-focused trials were registered in ClinicalTrials.gov. Fifty-three (1.76%) trials mentioned cost in any capacity (Fig 1). Cost outcomes, measuring the direct and indirect costs of trial interventions, were included in 37 (70%) trials (1.23% of all trials). The remaining 16 studies mentioned cost in the study description alone. Cost was reported as a primary outcome in 7 (13%) trials, secondary outcome in 26 (49%), tertiary/other outcome in 3 (5.7%) (Table I). The trial intervention categories evaluated in terms of their associated costs were prevention (11%), screening (14%), diagnosis (19%), treatment (62%), and health service research (5%). Seventeen (32%) trials included cost-effectiveness analyses, and 98.1% of the studies have not yet reported results.

Less than 2% of the melanoma clinical trials mentioned cost, with only 1.2% including it as an end point. Changes in initially reported outcome measures, exclusion of trials not listed in Clinicaltrials.gov, and unavailable study results, likely influenced by trial stage, hinder the assessment of cost data on ultimate utilization and impact on stakeholders. The limited inclusion of cost end

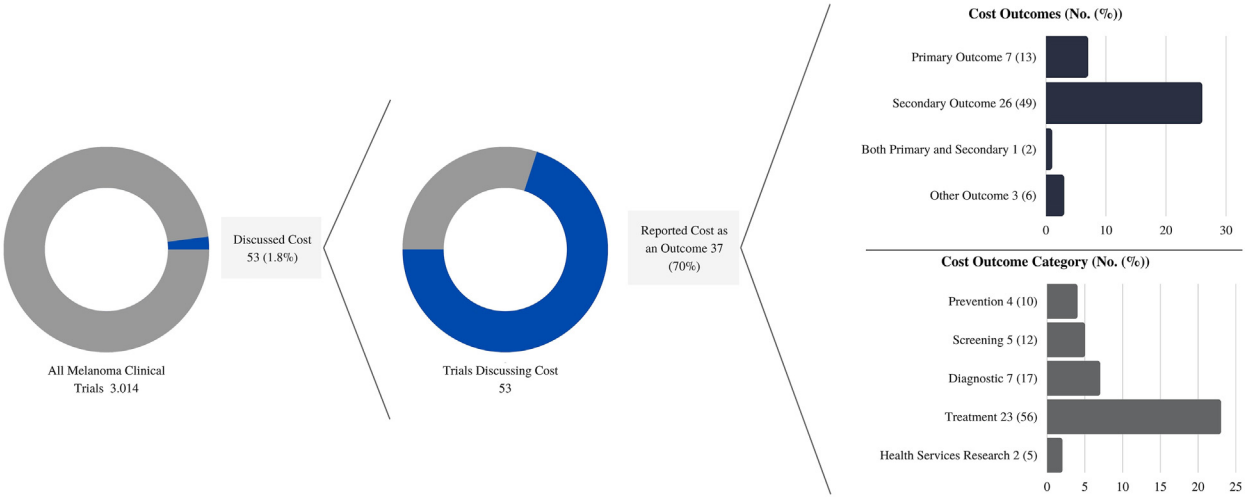

Fig 1. Melanoma clinical trials incorporating cost outcomes of trial interventions. The cost outcome category refers to specific trial intervention categories being evaluated in terms of their associated costs.

Table I. Characteristics of melanoma clinical trials incorporating cost into trial design

Characteristic	*n* (%)
Status	53
Completed	23 (43)
Recruiting	15 (28)
Not yet recruiting	2 (4)
Active/not recruiting	7 (13)
Withdrawn/terminated	3 (6)
Unknown	3 (6)
Results of trial available	1 (2)
Primary intervention	53
Drug	23 (28)
Behavioral	7 (13)
Procedure	6 (11)
Diagnostic test	6 (11)
Device	4 (8)
Screening	4 (8)
Other	3 (6)
Pediatric	7 (13)
Phase	53
Phase 1	1 (2)
Phase 2	6 (11)
Phase 3	6 (11)
Phase 4	1 (2)
Not listed	39 (74)
No. of people enrolled, mean (SD)	3705 (13,327)
No. of people enrolled	53
0-49	8 (15)
50-99	3 (6)
100-499	22 (42)
500-999	10 (19)
>1000	10 (19)
Funding	53
Industry	18 (34)
NIH	6 (11)
Other (academic medical center, non-NIH research institution)	29 (55)
Study type	53
Interventional	35 (66)
Observational	18 (34)
Duration (start date to completion date, mo), mean (SD)*	60.5 (44.3)
Primary country	51
Australia	6 (12)
Belgium	1 (2)
Canada	1 (2)
Cuba	1 (2)
Denmark	1 (2)
France	7 (14)
Germany	3 (6)
Italy	1 (2)
Netherlands	4 (8)
United Kingdom	4 (8)
USA	22 (43)
Cost outcome	53
None	16 (30)
Primary	7 (13)
Secondary	26 (49)
Other	3 (6)
Primary and secondary	1 (2)
Cost outcome category	41†
Prevention	4 (10)
Screening	5 (12)
Diagnostic	7 (17)
Treatment	23 (56)
Drug	18 (44)
Procedure	4 (10)
Other	1 (2)
Health services	2 (5)
Cost-effectiveness analysis performed	17 (32)

NIH, National Institutes of Health; *USA*, United States of America.
*Including ongoing trials.
†Specific trial intervention categories being evaluated in terms of their associated costs. Forty-one cost outcome categories among 37 trials because some trials evaluated the cost of >1 intervention category.

points and availability of cost data in melanoma trials may be reflective of significant practical design challenges. The multifaceted nature of resource utilization is largely driven by predetermined trial-specific protocols optimized for research rather than reflecting real-world utilization. The artificial environment may not provide sufficient information for decision makers (nonrepresentative trial participants, limited follow-up duration, and investigational drugs without well-defined costs). Despite these limitations, cost end points may provide early economic value with high internal validity to payers, offer less biased estimates of key model parameters in resource utilization, and enhance the methodologic quality of cost-effectiveness analyses.[3,4] Early-phase cost evaluations may facilitate timely information for pricing and reimbursement.[4] To develop clinically and economically valuable interventions, it is crucial to prioritize reporting of integrated clinical and economic data, understand barriers, consider other measures of value (quality of life), and further characterize implications.

Rachel S. Goodman, MBA,[a] Desmond C. Garner, BS,[a] Stefan Koester, BS,[a] J. Randall Patrinely, Jr, MD, MBA,[b] Anna K. Dewan, MD,[b] and Douglas B. Johnson, MD, MSCI[c]

From the Vanderbilt University School of Medicine, Nashville, Tennessee[a]; Department of Dermatology, Vanderbilt University Medical Center, Nashville, Tennessee[b]; and Department of

Hematology/Oncology, Vanderbilt University Medical Center, Nashville, Tennessee.[c]

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Correspondence to: Douglas B. Johnson, MD, MSCI, Vanderbilt University Medical Center and Vanderbilt Ingram Cancer Center, 1161 21st Ave S, Nashville, TN 37232

E-mail: douglas.b.johnson@vumc.org

Conflicts of interest

Author Goodman has served on advisory boards or as a consultant for BMS, Catalyst Biopharma, Iovance, Jansen, Mallinckrodt, Merck, Mosaic ImmunoEngineering, Novartis, Oncosec, Pfizer, Targovax, and Teiko; has received research funding from BMS and Incyte; and has patents pending for use of MHC-II as a biomarker for immune checkpoint inhibitor response and abatacept as treatment for immune-related adverse events. Drs Patrinely, Dewan, and Johnson and Authors Garner and Koester have no conflicts of interest to declare.

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