Defining Curative Potential: Where Physicians and Payers Align — and Where They Don't

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Background

- Advances in adjuvant/neoadjuvant therapies have improved long-term outcomes in early-stage melanoma, raising the question of when "cure" is appropriate as a concept
- Despite this progress, "cure" remains inconsistently defined in oncology (varying by tumor type and patient/clinical context), and melanoma lacks a well-established cure definition, especially in early-stage disease
- Misalignment across stakeholders (eg, providers vs payers) in terminology and interpretation can create misunderstandings, complicate claims about curative potential, and potentially delay access–highlighting the need for clearer, audience-appropriate definitions to support decision-making and health technology assessment (HTA)/economic modeling

Study objectives

- To compare physician and payer perspectives on what constitutes "cure" and how curative potential should be demonstrated in early-stage melanoma

Methodology

Survey development

- A conceptual framework was developed based on a systematic literature review (SLR), HTA review, and input from a steering committee of clinical and health economic experts
- The framework was translated into a structured online survey for France, Germany, Italy, and the United Kingdom (UK), then validity checked and piloted with the steering committee and select participants
- The steering committee provided detailed feedback to refine the survey content and structure, with equal participant representation across countries to ensure balanced perspectives

Participant recruitment

- Forty participants (24 physicians and 16 payers) from France, Germany, Italy, and the UK were recruited
- Inclusion criteria:
  - Payers
    - Advisors on access decisions for oncology/melanoma
    - Hands-on experience in national pricing/reimbursement decisions
    - Expertise in developing cost-effectiveness models for melanoma therapies where applicable to the country being represented (cost-effectiveness analysis is not applicable for Germany)
  - Physicians
    - Oncologists, surgical oncologists, radiation oncologist dermatologists, melanoma specialists
    - ≥5 years' experience treating early-stage melanoma
    - Personally responsible for prescribing drug treatments
    - Treated ≥2 patients with early-stage melanoma in past 12 months

Survey implementation and analysis

- The survey was disseminated to participants via a link to a secure online survey platform, sent via email by the third-party organization
- Quantitative and qualitative responses were analyzed to evaluate preferred terminology/definitions, evidentiary priorities (RFS, OS, conditional survival, RWE), acceptable follow-up maturity, and modeling acceptability (time-to-cure [TTC], cure fraction [CF])

Results

- Forty participants (24 physicians and 16 payers) from France, Germany, Italy, and the UK were recruited

Terminology and efinitions

- The preferred clinical definition of "cure" for both physicians and payers was "*Complete remission after complete surgical excision of the primary tumor with histologically negative margins, where patient has remained alive and free of local, regional, or distant recurrence for a minimum amount of time*" (**Figure 1**)
- Terminology diverged by stakeholder and audience. Physicians more often used "cured" or "disease-free," while payers favored "long-term survivor" or "recurrence-free" (**Figure 2**)
- "Cured" was most preferred in Germany and Italy, while "recurrence-free" was most preferred in France and "disease-free" in the UK. Germany, Italy, and France also displayed more diversity in terminology (**Figure 2**)
- Most physicians (80%) and about half of payers tailored language to audience
- "Statistically cured" was the preferred term used with colleagues, whereas "disease-free" was preferred when discussing "cure" with payers/hospital managers, followed by "statistically cured," "functionally cured," or "cured"
- Physicians and payers were largely aligned on the foremost challenges to determining "cure" (**Table 1**)

Figure 1. Most preferred clinical definition of cure

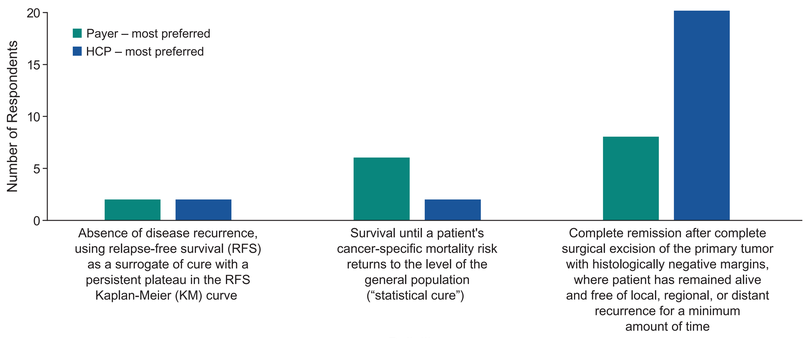
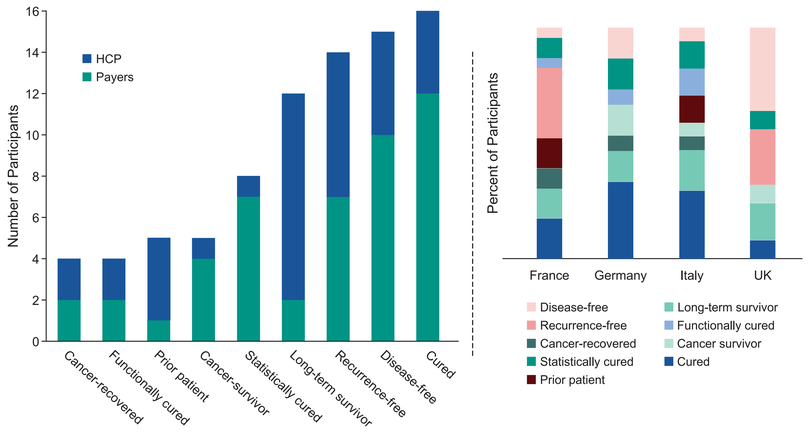


Figure 2. Preferred language/terminology used to represent a "cured" patient who had early-stage melanoma



*Respondents could select up to 3 answers

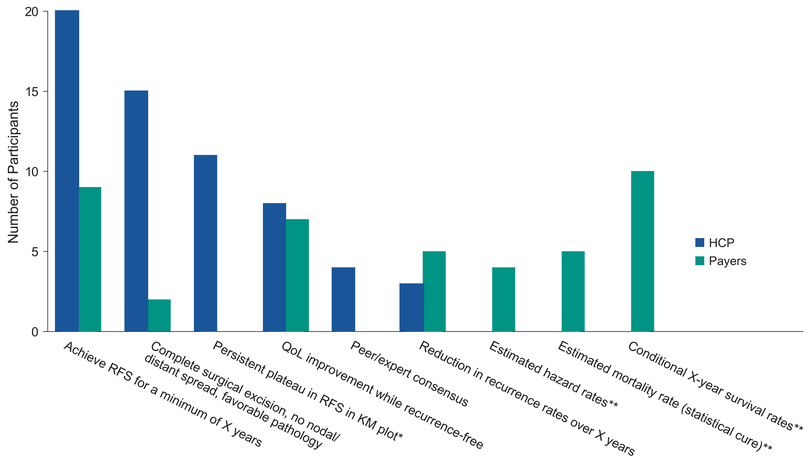
Table 1. Challenges faced by physicians and payers when determining "cure" for early-stage melanoma

Challenges	# HCP	# Payers
The possibility of latent disease recurrence that could emerge years later	**17**	**9**
Variability in patient responses to treatment	14	9
The psychological and well-being consequences that persist despite a cure	13	7
The uncertainty about how to balance recurrence-free with patient safety	8	7
The lack of consensus on what constitutes "cure" in early-stage melanoma	6	5

Curative potential endpoints and benchmarks

- There was agreement across participants and geographies that "achieve RFS for a minimum of X years" was considered the most effective factor in demonstrating the curative potential of a treatment (**Figure 3**)
- Both physicians and payers largely agreed that 5 years of follow-up marks an inflection point as the minimum time needed to demonstrate "cure." Confidence in determining "cure" generally increased with longer follow-up (**Figure 4**)
  - Five years RFS generated higher confidence to demonstrate curative potential, minimum length of follow-up in a clinical trial, demonstrating that patients remain recurrence-free, and as a plateau threshold for determining curative potential (**Figure 4**)
- Across clinical and modeling evidence types, respondents generally expected 3-5 years of underlying data before "cure" estimates are considered credible, with longer durations (≥5 years) required for more complex modeling approaches
- Observed clinical trial data (RFS, OS) were trusted earlier, typically after 2-3 years of follow-up, while model-based projections (eg, hazard rate or conditional survival modeling) required greater data maturity and validation before being accepted
- Physicians and payers both prioritized "long-term clinical trial data from pivotal clinical trials as the most important types of data for determining both time to cure and cure fraction (**Figure 5**)
- Both groups viewed long-term RWE as critical to corroborate trials and inform cure timing

Figure 3. Factors most effective in demonstrating curative potential of a treatment



Note: Answers with "*" were not an option in payers' survey; Answers with "**" were not an option in physicians' survey

Figure 4. RFS duration needed for high confidence of curative potential

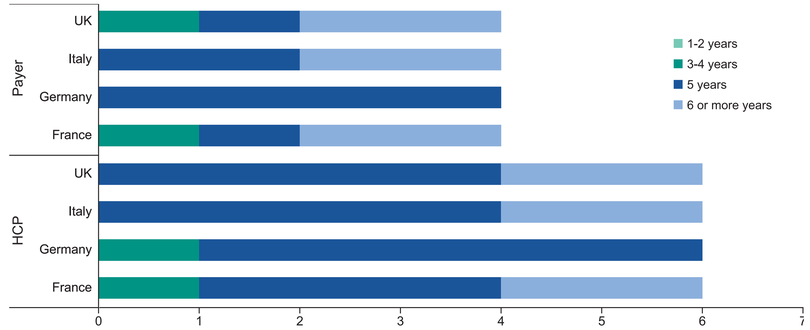
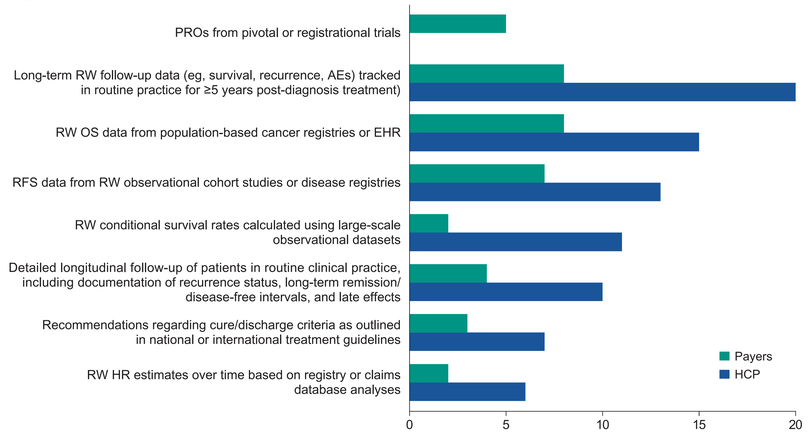


Figure 5. Supportive evidence needed to demonstrate cure timing outside of the clinical trial



Lack of agreement on potential cure proxies

- Discharging a patient was numerically less likely to indicate "cure" according to physicians (41.7%) vs payers (50.0%)
- Physicians were less certain about deeming a patient "cured" if they require ongoing follow-up, with most selecting "No" (37.5%) or "Depends on frequency" (33.3%)
- Payers similarly leaned toward the opinion that needing ongoing follow-up indicates that a patient isn't "cured," with 10 (62.5%) indicating that requiring follow-up negates "cure"
- Almost two-thirds of physicians (62.5%) and payers (62.5%) indicated they would still consider a patient "cured" if they are disease-free but experiencing lingering effects from their diagnosis or treatment
- Patient self-assessment (eg, return to normality) was reported to inform "cure" for half of physicians (50.0%) and almost three quarters (68.8%) of payers

Limitations

- Strengths of this study include the use of a structured survey to provide insights from physicians and payers across four European countries that were previously lacking
- However, the relatively small numbers of participants make it impossible to compare differences between participant types and countries in any statistically meaningful way

Conclusions

- Physicians and payers share core clinical definitions of cure but differ in terminology, confidence thresholds, and evidence needs
- Adopting a standardized lexicon distinguishing clinical remission from statistical cure, explicitly reporting evidentiary maturity, and using transparent modeling can add confidence to cure claims, enhance HTA credibility, and guide pragmatic policies for "cure" and resource planning

https://bit.ly/3P8Ego7

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