Can Real-World Evidence (RWE) Drive European, UK, and US Payer Decisions in the Reassessment of Oncology Therapies?

M. Bharmal,[1] I. Katsoulis,[2] J. Chang,[3] A. Graham,[2] R. Palencia,[4] A. Stavropoulou,[2] C. Pashos[5]

[1]EMD Serono Research & Development Institute, Inc., Billerica, MA, USA, an affiliate of Merck KGaA; [2]Market Access Transformation, Fleet, UK; [3]Pfizer, New York, NY, USA; [4]Merck Healthcare KGaA, Darmstadt, Germany; [5]Genesis Research, Winchester, MA, USA

Disclosures

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IK: Ioannis Katsoulis, an employee of Market Access Transformation, was a consultant to Merck.

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RP: Roberto Palencia was a full-time employee of Merck Healthcare KGaA, Darmstadt, Germany, at the time this research was conducted.

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Background

- New innovative oncology therapies may receive marketing authorization with evidence deemed insufficient by HTA bodies and payers: single-arm trials, short trial duration, or surrogate/composite endpoints[1]

- At **reassessment**, HTA bodies and payers have an opportunity to evaluate evidence that allows them to **mitigate financial risk while ensuring patient access** to therapies with appropriate effectiveness and risk profiles[2-4]

- Advancements in real-world data and analytics requirements allow drug developers to better understand and generate the types of information HTA bodies and payers are looking at or need to know[2-4]

HTA, health technology assessment.

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Scope

- To understand how HTA bodies and payers perceive the **use and value** of RWE when reassessing oncology therapies and their perceptions on RWE's **most desired attributes**
- Multinational assessment of HTA bodies and payers at the national or regional level from the US, France, Germany, Spain, and the UK (England)
- Markets were selected to represent a range of different payer archetype markets

Country					
Number of respondents	**10**	**5**	**5**	**5**	**5**
Respondent type	• 4x national PD • 2x national MD • 2x IDNs • 2x PBMs	• 3x ex-TC • 2x ex-CEPS	• 3x ex–G-BA • 2x SHI (with GKV-SV experience)	• 2x ex-national HTA decision makers • 3x regional HTA decision makers	• 3x ex-NICE • 2x NHS England

CEPS, Comité Économique des Produits de Santé; G-BA, Gemeinsamer Bundesausschuss; GKV-SV, Gesetzliche Krankenversicherung; HTA, health technology assessment; IDN, integrated delivery network; MD, medical director; NHS, National Health Service; NICE, National Institute for Clinical Excellence; PBM, pharmacy benefit manager; PD, pharmacy director; RWE, real-world evidence; SHI, Social Health Insurance; TC, transparency committee.

Methods

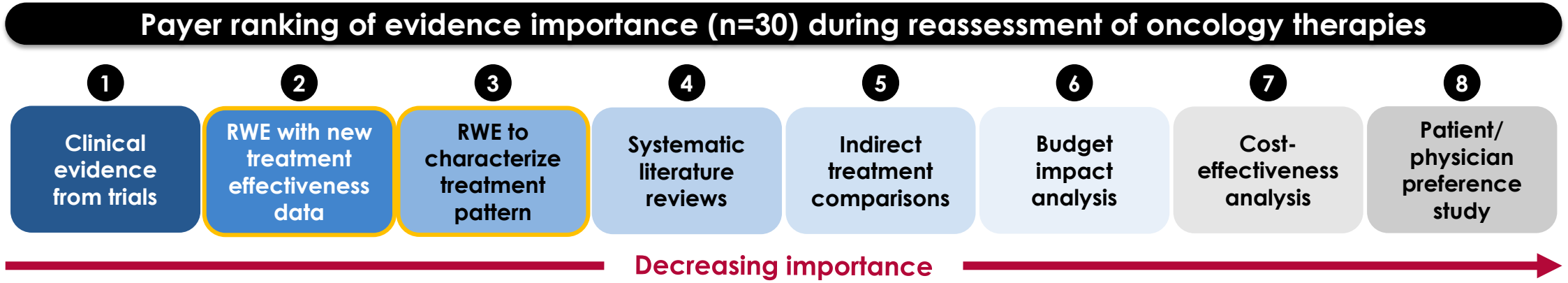
- A **web-based survey** was administered via the **Market Access Transformation RPR™ online portal** in May 2022 among 30 national and regional payer decision-makers and influencers from the US, France, Germany, Spain, and the UK

- Responses were collected through the RPR™ portal in 5 days and analyzed using descriptive statistics

- Question types:
  - Rating and ranking scale to **quantitatively** evaluate perceptions on RWE use and attributes
  - Open-text **qualitative** questions to contextualize perceptions of value and recommendations to manufacturers for the optimization of the evidence

RPR, Rapid Payer Response; RWE, real-world evidence.

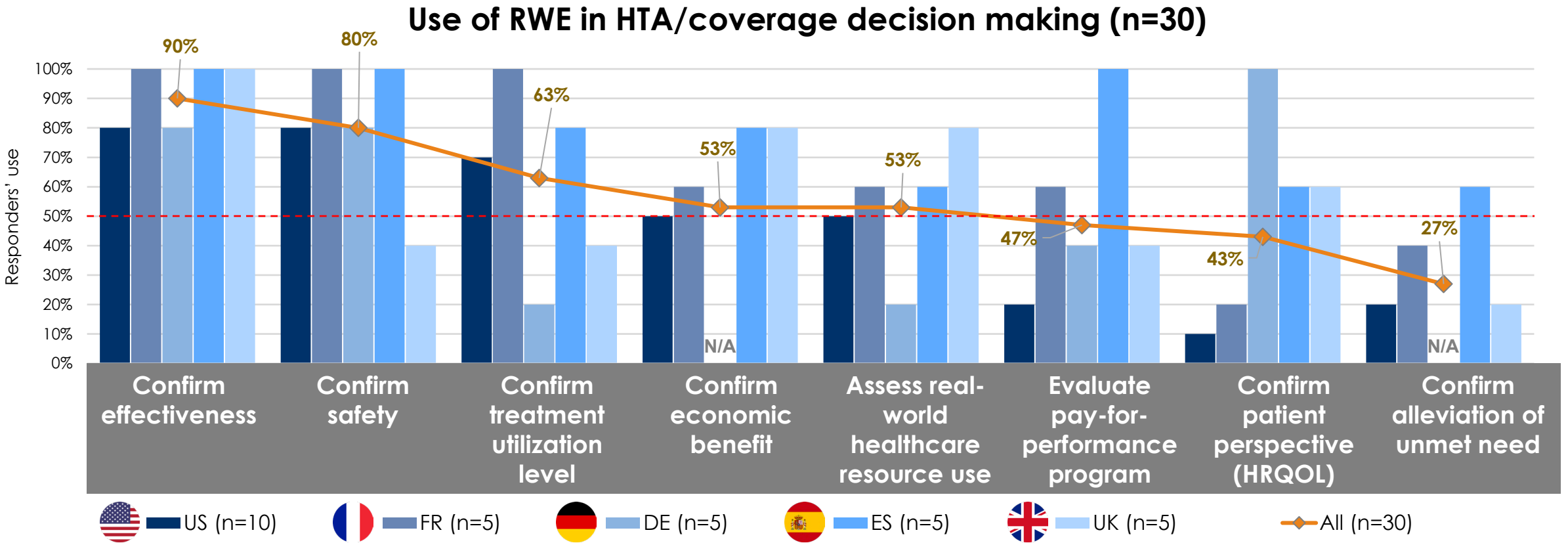
After clinical trial evidence, RWE on effectiveness and treatment patterns is perceived to be of high importance



	1	2	3	4	5	6	7	8
(n=10)	Clinical evidence	RWE effectiveness	SLR	RWE treatment pattern	ITC	BIA	CEA	Patient/physician preference
(n=5)	Clinical evidence	RWE effectiveness	RWE treatment pattern	ITC	SLR	BIA	Patient/ physician preference	CEA
(n=5)	Clinical evidence	ITC	RWE effectiveness	SLR	RWE treatment pattern	Patient/physician preference	BIA	CEA
(n=5)	Clinical evidence	BIA	RWE effectiveness	RWE treatment pattern	CEA	ITC	SLR	Patient/physician preference
(n=5)	CEA	Clinical evidence	RWE effectiveness	SLR	RWE treatment pattern	ITC	BIA	Patient/physician preference

BIA, budget impact analysis; CEA, cost-effectiveness analysis; ITC, indirect treatment comparison; RWE, real-world evidence; SLR, systematic literature review.

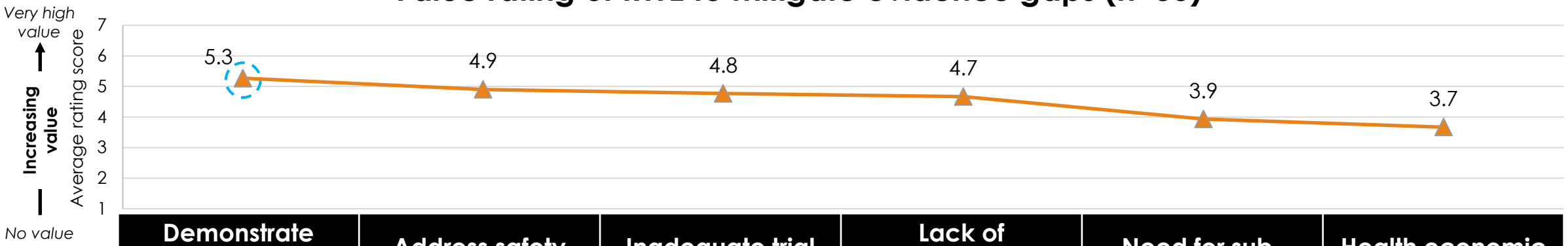
RWE at the reassessment stage is used to confirm the efficacy, safety, and level of utilization of oncology therapies



HRQOL, health-related quality of life; HTA, health technology assessment; RWE, real-world evidence.

Demonstrating comparative clinical benefit with mature data from the real-world vs the SOC is key in the reassessment stage

Value rating of RWE to mitigate evidence gaps (n=30)



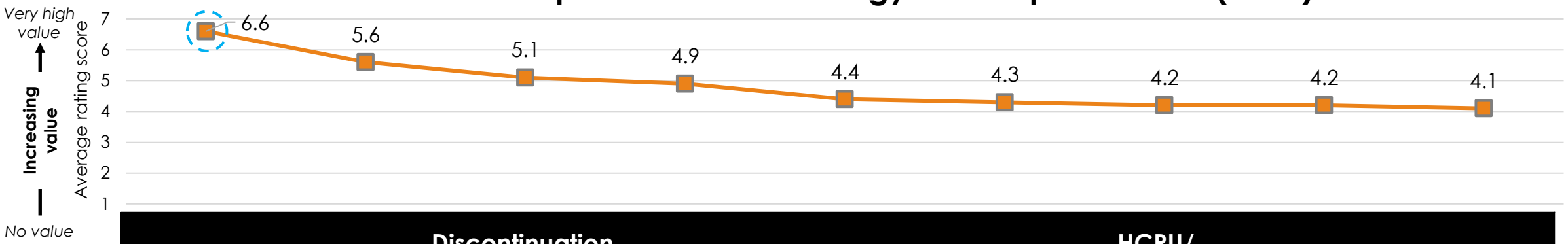
	Demonstrate longer-term effectiveness	Address safety concerns	Inadequate trial population	Lack of comparative clinical data	Need for sub-population analysis	Health economic requirements
(n=10)	**5.6**	5.0	4.7	4.4	4.2	4.0
(n=5)	5.4	**5.8**	4.8	4.4	3.8	3.4
(n=5)	4.6	3.8	4.6	4.2	2.6	2.4
(n=5)	5.4	5.4	**5.2**	5.0	4.2	3.8
(n=5)	5.0	4.4	4.6	**5.6**	**4.6**	**4.4**

High value, score 5-7 | Moderate value, score 3-4.9 | Low value, score 1-3

RWE, real-world evidence; SOC, standard of care.

Due to limitations of registrational trials, payers see value in survival and safety from RWE at reassessment

Value of endpoints in RWE oncology studies post launch (n=30)



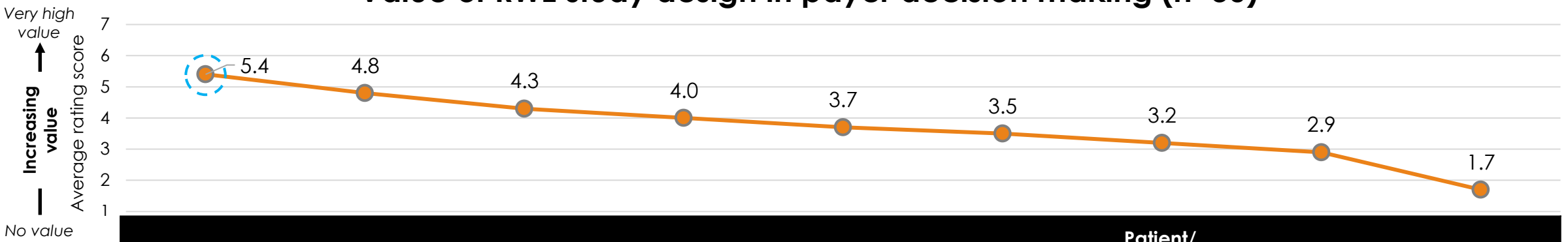
	OS	AE rates	Discontinuation rates	PFS	DOT	DOR	HCRU/ cost data	HRQOL	ORR
(n=10)	6.4	5.3	5.0	5.4	4.7	**5.0**	4.7	2.7	4.6
(n=5)	6.8	5.8	5.2	**5.6**	4.4	4.8	3.8	3.2	4.0
(n=5)	**7.0**	6.0	4.8	2.4	1.8	1.8	2.0	**6.4**	2.2
(n=5)	6.8	**6.2**	**5.4**	**5.6**	**5.8**	**5.0**	**5.2**	4.6	**4.8**
(n=5)	6.4	5.0	5.2	4.8	4.8	4.2	4.6	5.8	4.2

High value, score 5-7 | Moderate value, score 3-4.9 | Low value, score 1-3

AE, adverse event; DOR, duration of response; DOT, duration of treatment; HCRU, healthcare resource utilization; HRQOL, health-related quality of life; ORR, overall response rate; OS, overall survival; PFS, progression-free survival; RWE, real-world evidence.

RWE from prospective studies and registries are perceived to have high value for payers

Value of RWE study design in payer decision making (n=30)



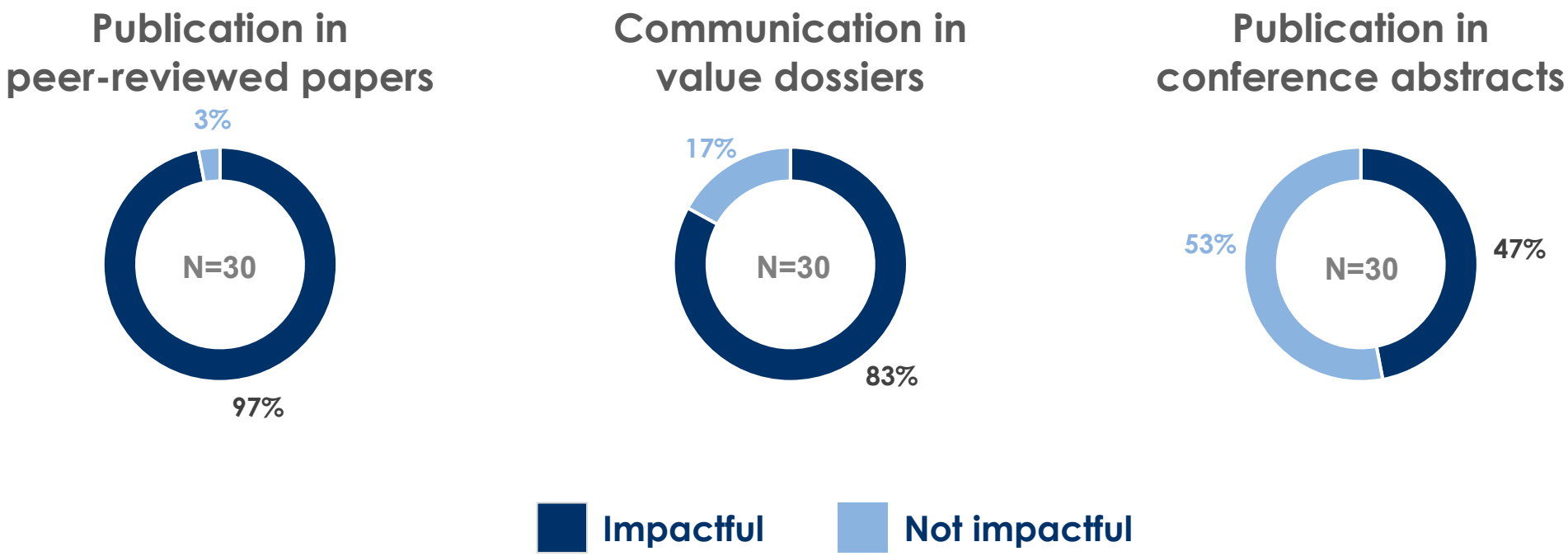
	Prospective observational	Product/disease registries	EMR based	Retrospective observational	Medical chart review	Health claim or billing	Patient/ caregiver surveys	Physician surveys	Social media listening studies
(n=10)	4.9	4.3	**4.7**	**4.6**	4.2	3.9	2.9	2.7	**2.1**
(n=5)	**6.0**	**6.0**	4.6	3.4	**4.6**	**4.2**	2.6	3.4	1.2
(n=5)	5.0	4.2	2.4	2.6	1.8	2.0	2.8	2.2	1.0
(n=5)	5.6	4.6	4.6	4.4	3.4	3.6	3.4	3.0	2.0
(n=5)	**6.0**	5.6	4.6	4.2	4.2	3.4	**4.4**	**3.6**	1.8

High value, score 5-7 | Moderate value, score 3-4.9 | Low value, score 1-3

EMR, electronic medical record; RWE, real-world evidence.

Transparency and demonstration of credibility are key to the communication of RWE to gain payer trust and consideration

Payer perception on the impact of communication forms for RWE



RWE, real-world evidence.

Key takeaways: the use, value, and most desired attributes of RWE at reassessment of oncology therapies

- Data from clinical trials are of greatest importance during reassessments, with HTA and payer decision-makers ranking RWE as the next most important evidence type
  - Most responders agreed that **confirming the real-world effectiveness** (27 of 30 responders, 90%) and **safety** (80%) of oncology therapies are the primary uses of RWE, along with **confirmation of treatment utilization** (63%) and **healthcare resource use** and **economic benefit** (53% each)
  - Demonstrating **clinical benefit in subpopulations** and **comparing with standard of care** (eg, meaningful data on overall survival and incidence of adverse events) were highly rated during reassessment, especially because of a lack of longer-term data at launch and the relatively short duration of registrational trials
- RWE from prospective observational studies and product/cancer registries were considered to be of higher value, while peer-reviewed, published data that are transparently presented as part of industry dossiers were of highest quality

HTA, health technology assessment; RWE, real-world evidence.

Why this is meaningful—so what?

	Why?	How?
RWE quality	**To adhere to HTA guidelines and position papers on best practices**	♦ Select fit-for-purpose data to support HTA and payer decision making ♦ Seek early scientific advice from HTA agencies
RWE methods	**To maximize value for healthcare systems with prospective observational and product/disease registries**	♦ Use standardized methods to harmonize study designs and outcomes ♦ Publish study protocol in clinical trials database ♦ Create an independent registry
Communication	**To disseminate transparent knowledge in peer-reviewed journals and value dossiers**	♦ Increase data validity via collaboration with clinical experts ♦ Create a scientific committee to advise on the publication strategy

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Correspondence: Murtuza Bharmal, murtuza.bharmal@emdserono.com