

Patients Defining What Matters: A Cross-Sectional Survey with a Discrete-Choice Experiment on Unmet Medical Need

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BACKGROUND

- **Unmet Medical Need (UMN)** refers to health conditions where available diagnostic or treatment options are either inadequate or do not exist, implying that the adequacy of available options is a judgment¹
- UMN assessment is a critical determinant of pharmaceutical R&D strategy, informing lifecycle decision-making, shaping regulatory and reimbursement considerations, and focusing innovation on areas with insufficient treatment options.²⁻⁴
- UMN is a multidimensional construct but there is no consensus across stakeholders as to which dimensions should be used to define it.⁶ Prior research indicates that while disease burden and treatment adequacy are consistently emphasized, patients, regulators, HTA bodies, payers, and industry differ in how they value other criteria such as access, comorbidities, and socioeconomic factors.^{5,6}
- Stakeholder perspectives continue to guide UMN assessment for specific purposes. Recent efforts—including the European Commission’s 2023 proposal to standardize legal definitions of UMN—highlight growing policy interest but also persistent divergence in perspectives.⁷

Findings from prior studies, including the study by Wens et al., in which patient stakeholders constituted 19% of survey respondents, demonstrate that comorbidities and socioeconomic factors are relevant to UMN. However, despite UMN fundamentally reflecting patient needs and **patients bearing the burden** of inadequate or inaccessible treatment, **few existing UMN frameworks systematically incorporate patient input, are co-developed with patients, or reflect patient judgments regarding what constitutes the adequacy of diagnostic or treatment options in addressing a disease or condition.**

OBJECTIVE

- To quantify the relative importance which patients place on six key dimensions of unmet medical need (UMN) adapted from an existing framework proposed by Venneman et al. to advance a more patient-centric approach to UMN assessment.

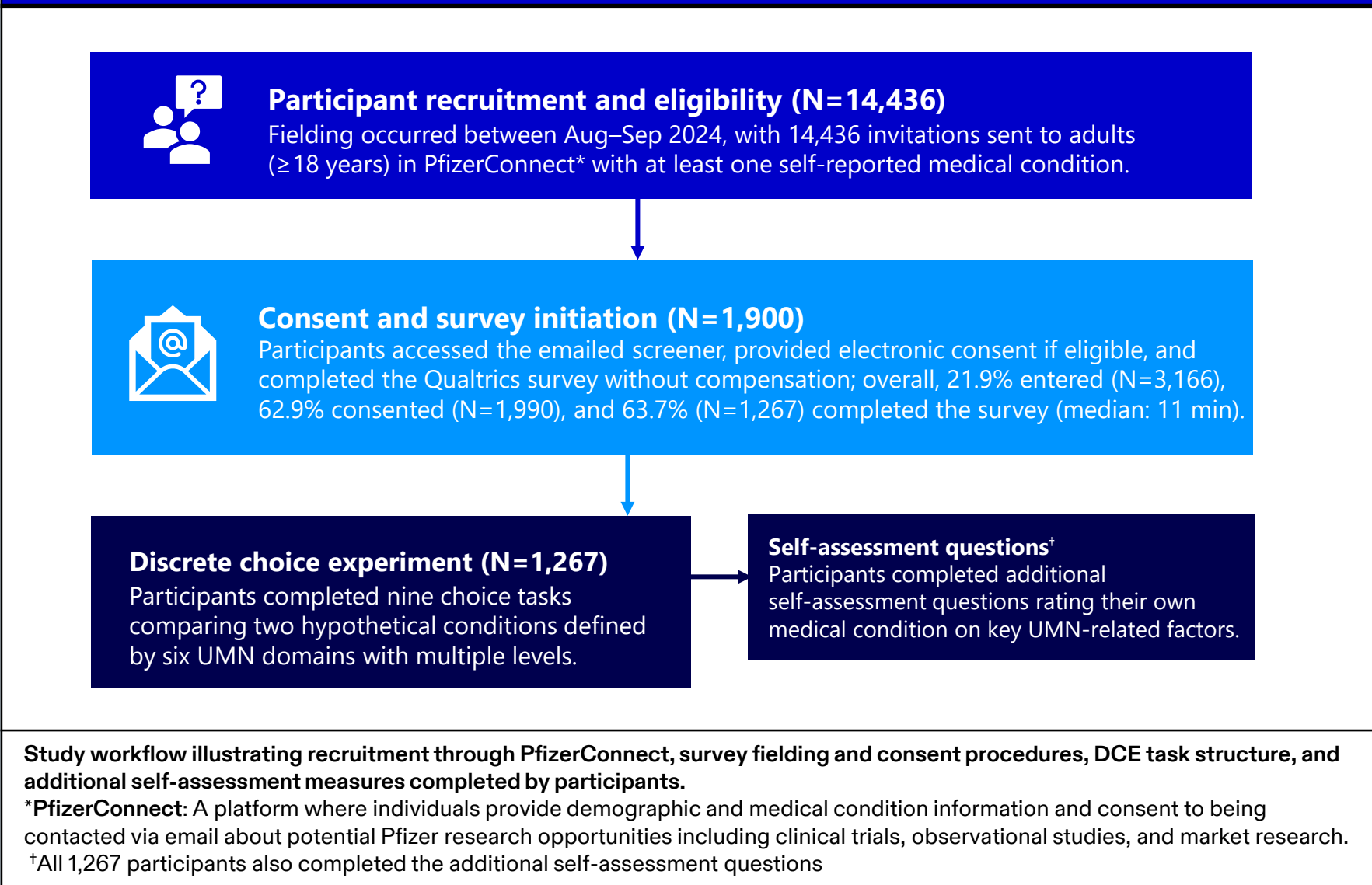
MATERIALS AND METHODS

STUDY DESIGN

Survey Design and Sample

- A cross-sectional, non-interventional online survey was conducted to quantify the relative importance of six domains defining unmet medical need (UMN). (**Figure 1**)

Figure 1. Study Recruitment Process via PfizerConnect



DISCRETE CHOICE EXPERIMENT

- The survey included a discrete choice experiment (DCE) developed following ISPOR good research practice guidelines.^{8,9}
- The DCE was structured to elicit preferences regarding six key UMN domains adapted initially from Venneman et al. and subsequently revised to fit into a choice framework. Domain descriptions were adapted to reflect patient-facing terminology and conceptual clarity (**Table 1**).^{10,11}
- The experimental design was a balanced overlap design in which each participant was assigned to complete one of 240 blocks containing nine DCE choice tasks.¹¹
- In each task, participants reviewed two hypothetical medical conditions (Condition A vs. Condition B) characterized by six UMN domains: life years lost, symptom burden, disease duration, treatment satisfaction, treatment access, and disease prevalence, each represented by 3–4 possible levels. Participants selected the condition they believed represented **greater UMN**. (**Figure 2**)

Figure 2. Example of a choice task in the DCE survey

Aspects of Unmet Need	Medical Condition A	Medical Condition B
Years of life lost	1 year lost	5 years lost
Burden of Symptoms	Low burden	High burden
How long the medical condition lasts	Medium (a few weeks to a few months)	Short (a few days – 1 week)
How satisfied patients are with available treatments	Satisfied	Dissatisfied
How easy it is for patients to get the available treatments	Very difficult	Not too difficult
The number of people with the medical condition	Rare (less than 1 in 2,000 people)	Very common (more than 1 in 10 people)
Which medical condition do you think has more unmet need?	<input type="radio"/>	<input type="radio"/>

- **Additional Self-Assessment Measures:** Participants rated aspects of their own medical conditions, including symptom burden, life years lost, disease duration, and satisfaction/access to treatments, to support analyses examining associations between personal experience and judgments of what constitutes unmet need.^{12,13}
- **Demographic & Background Data:** The survey captured standard demographics (age, gender, race/ethnicity, education, urbanicity) along with validated health literacy and numeracy measures.

Table 1. Domains and levels used in the DCE

Attribute name	Patient-facing domain label	Levels
Years of life lost (YLL)	Years of life lost	1 year lost
		3 years lost
		10 years lost
		Low burden
Symptom burden	Burden of symptoms	Medium burden
		High burden
		Short (a few days to a week)
		Medium (a few weeks to a few months)
Disease duration	How long the medical condition lasts	Long (6 months to many years)
		Lifetime
		Satisfied
		Neutral (neither satisfied nor dissatisfied)
Treatment satisfaction	How satisfied patients are with available treatments	Dissatisfied
		Very easy
		Not too difficult
		Very difficult
Treatment access	How easy it is for patients to get the available treatments	Rare (<1 in 2,000 people)
		Common (>1 in 100 people)
		Very common (>1 in 10 people)
		Very common (>1 in 10 people)
Disease prevalence	The number of people with a medical condition	Rare (<1 in 2,000 people)
		Common (>1 in 100 people)

STATISTICAL ANALYSIS

- Individual-level preference weights for each domain level were estimated using a Hierarchical Bayes (HB) model.
- Preference weights represent the relative contribution of each domain level to perceived UMN, with **larger positive weights reflecting higher unmet need**. These weights were averaged across participants to derive sample-level mean estimates.
- The **relative importance (RI)** of each UMN domain was calculated by taking the maximum difference in mean utility estimates for that domain (difference between highest and lowest mean preference weights) and dividing it by the sum of ranges across all domains. Multiplying by 100 yielded RI percentages that collectively sum to 100%, thereby representing each domain’s proportional contribution to variability in preferences.
- Subgroup analyses were conducted to evaluate heterogeneity in preferences across 15 prespecified subgroup pairs defined by 4 categories:
 - Participants’ type of self-reported medical condition(s) (cancer, obesity)
 - Demographics (gender, urbanicity, educational background, age, race)
 - Health literacy (numeracy, reading comprehension)
 - Self-assessed aspects of UMN related to participants’ medical condition(s) (years of life lost, symptom burden, length of medical condition, satisfaction with available treatments, access to available treatments, and prevalence).
- Analyses were conducted only when each subgroup included ≥150 participants. For each subgroup, the mean preference weights were compared to assess differences in the relative contribution of UMN domains across populations.

RESULTS

SURVEY SAMPLE

- 1,267 US adults completed the online survey between August-September 2025
- A total of 14,436 email invitations were sent to people who had opted in to PfizerConnect¹⁴; 21.9% of these people (N=3,166) entered the survey, of whom the majority consented to participate (62.9%; N=1,990). (**Figure 1**)

PARTICIPANT CHARACTERISTICS

- A summary of participant demographic characteristics is shown in **Figure 3**.
- Participants were predominantly female (66.9%) and White/Caucasian (89.2%), with most aged over 55 years (71.9%). Common medical conditions included hypertension (56.5%), obesity (51.1%), and hyperlipidemia (45.9%). Most lived in urban areas (74.8%), and educational attainment was high, with the majority reporting some college (59.5%) or graduate level (30.5%) education.
- Self-reported health literacy was high, with 83.2% (n=1,054) of participants indicating that they rarely or never had difficulty understanding written health information and 73.3% (n=929) reporting consistent confidence in completing medical forms
- Self-reported numeracy scores indicated moderate confidence with basic quantitative tasks (5 or 6 on a 6-point scale where 6 is ‘very good’)¹³

Figure 4. Estimated preference weights for each attribute level (N=1267)

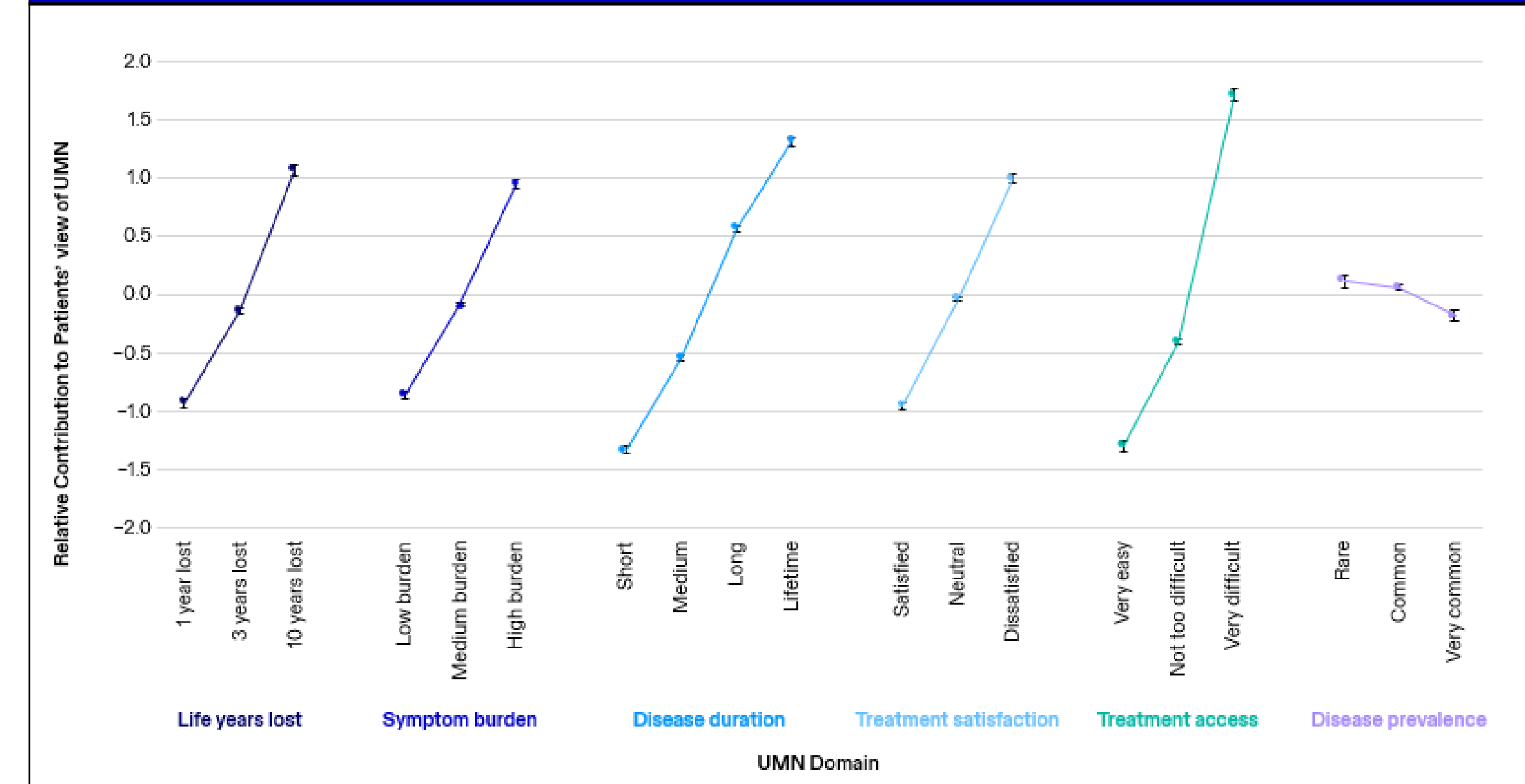


Figure 5. Relative importance (RI)* to Defining UMN (N=1267)

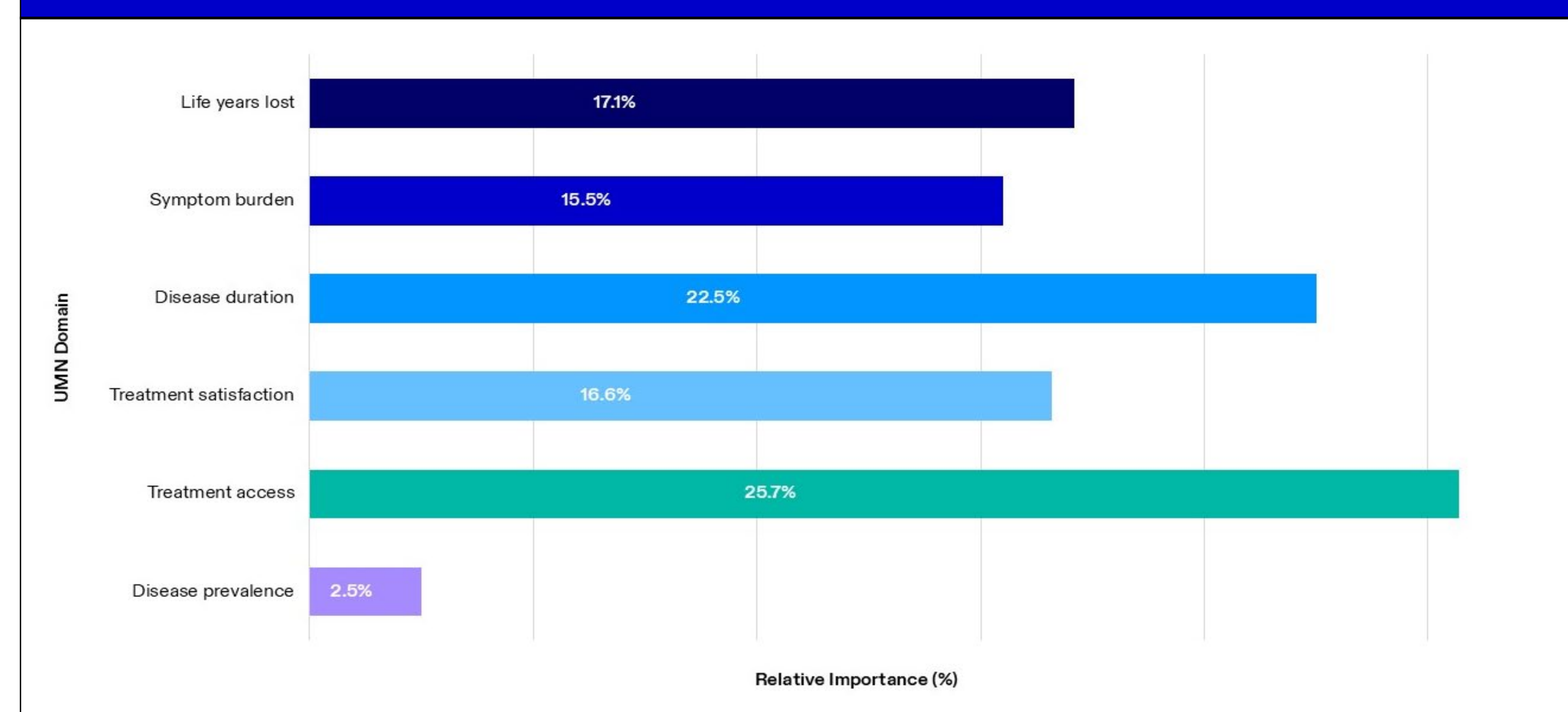
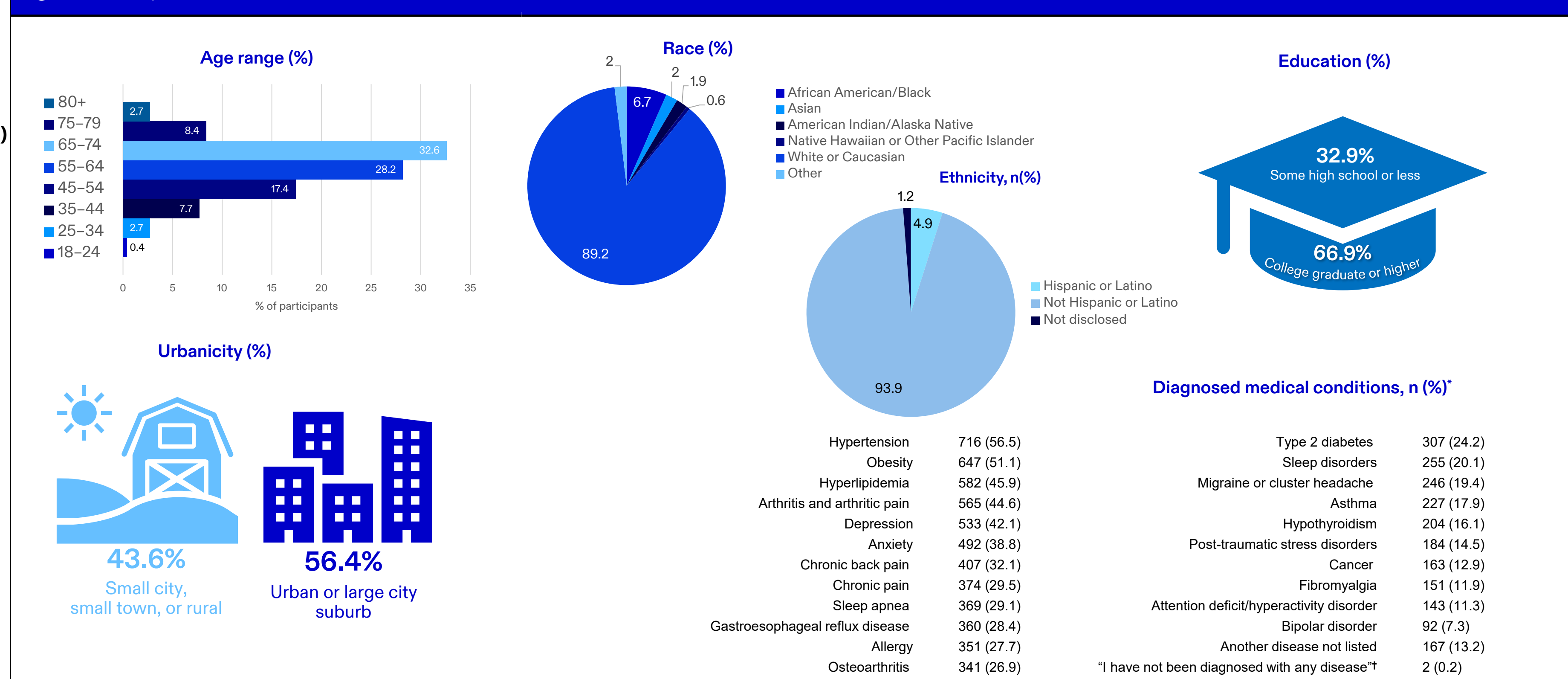


Figure 3. Participant characteristics (N=1267)



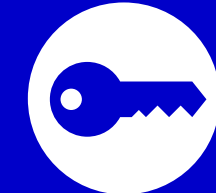
*Participants could select more than one answer; percentages are based on the total sample and therefore do not sum to 100%.
†Eligibility for the study was based on prior self-report of a diagnosed medical condition at the time of database enrollment. Consequently, all respondents met the study definition of a patient, despite two participants subsequently indicating ‘I have not been diagnosed with any disease’ in the demographic questionnaire.

PATIENT PREFERENCES AND RELATIVE ATTRIBUTE IMPORTANCE

- Estimated preference weights followed the expected ordering based on the UMN framework, with statistically significant differences across all attribute level comparisons except for levels of the prevalence domain.
- On average, respondents did not differentiate between rare and common diseases.
- Vertical distances between preference weights illustrate the relative magnitude of change in perceived contribution to unmet need across attribute levels (**Figure 4**).
- For example, within the **treatment access** domain, the change from ‘not too difficult’ to ‘very difficult’ access represented a substantially larger change in unmet need compared with the change from ‘very easy’ to ‘not too difficult.’ The same increase in difficulty accessing treatment was more influential than moving from 3 to 10 **years of life lost**.
- Across domains, participants assigned the greatest importance to **treatment access** (RI = 25.7%), followed by **disease duration** (RI = 22.5%), **years of life lost** (RI = 17.1%), **treatment satisfaction** (RI = 16.6%), **symptom burden** (RI = 15.5%), and **prevalence** (RI = 2.5%) (**Figure 5**).

Patients identified treatment access as the primary determinant of unmet medical need, placing it above disease-specific measures and highlighting the importance of incorporating patient preferences into flexible and harmonized UMN frameworks.

KEY FINDINGS



Preferences were generally consistent across demographic subgroups (gender, education, urbanicity).

Such findings show that existing frameworks can be adapted to capture stakeholder-specific priorities, and that patients can meaningfully engage in preference-based tasks to evaluate complex concepts such as UMN.

This study provides empirical evidence that patient informed weighting of UMN domains supports a harmonized yet adaptable framework capable of addressing diverse stakeholder perspectives and contexts.

LIMITATIONS



The study did not assess all levels of UMN domains, such as the complete absence of available treatments or requirements for demonstrating meaningful therapeutic advantage.

- The sample was drawn from a US-based opt-in database (PfizerConnect¹⁴), limiting generalizability. Demographic characteristics such as gender and race/ethnicity were not well-balanced.
- Survey design was straightforward and education level, health literacy, and numeracy were measured, but methodological DCE limitations remained. Cognitive burden may have affected response quality and model estimates, resulting in responses reflecting participants’ beliefs and values rather than preferences.

CONCLUSIONS



Treatment access is the primary determinant of unmet need from the patient perspective, underscoring that the existence of approved treatments is insufficient unless patients can meaningfully access them.

- Future research should examine how treatment access, beyond availability alone, informs patient perceptions of unmet need and supports refining UMN domains and weights for a standardized, patient-informed index.
- While policymakers consider prevalence essential because it reflects the size of the population lacking adequate treatment, patients do not view prevalence as relevant to defining **their own** unmet need. This contrast represents a significant departure from standard UMN definitions, which traditionally place substantial weight on prevalence
- Additional studies should continue to examine how patient preferences for UMN domains differ across geographic regions, particularly between US and EU patient populations, given differences in health system organization, treatment access and adequacy, reimbursement structures, and their global relevance to harmonized definitions.¹⁵

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