Lumanity

How to: Assess Burden of Illness and Unmet Need

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In the first of our new how-to series Lumanity's Matthew Woods explores the importance of understanding burden of illness and unmet need, as well as giving an overview of the key considerations when conducting reviews.

What is burden of illness and unmet need?

Unmet need is used where there are limitations in prevention, diagnosis or treatment of a condition. Definitions used in practice and literature vary,[1,2] often incorporating disease severity and population size. To fully understand unmet need, it is crucial to assess the **burden of illness** (BOI), i.e. what impact the disease has on patients, carers, and the health system. A BOI assessment should consider the following factors:

- **Epidemiology:** How many people are affected (incidence and prevalence), what does disease progression look like, how quickly does the disease progress, and what are the risk factors for developing the disease?
- **Clinical burden:** What impact does the disease have on life expectancy (mortality), what symptoms are associated with the disease, how common and severe are the symptoms, and what comorbidities are associated with the disease?
- **Humanistic burden:** How does the disease impact the individual's ability to function and perform their usual activities (work/school attendance), what impact does the disease have on overall quality of life (and what patient-reported outcome [PRO] instruments are used), and what is the impact on families and formal/informal caregivers?
- **Economic burden:** What resources are used by the healthcare system to treat and/or manage patients, what are the associated costs to the healthcare system (direct costs), and what are the associated costs to patients and/or society (indirect costs)?
- **Treatment landscape:** What treatments are currently recommended by relevant treatment guidelines, currently used in clinical practice and what are the outcomes and potential limitations associated with treatments?

Uses of BOI and unmet need reviews

- Developing value messages for disease background, BOI and unmet need
- Starting to inform a value proposition
- Informing relevant sections of a global value dossier or core reimbursement dossier
- Informing local submission dossiers
- Identifying evidence gaps and planning evidence generation activities
- Publication planning, to increase aware of the impact of the disease
- Supporting strategic plans for market access, including discussions around optimum positioning in the treatment pathway
- Supporting clinical trial design, including the relevant patient population and choice of appropriate comparators

Key: BOI, burden of illness; GVD, global value dossier; HTA, health technology assessment; PRO, patient-reported outcome; TLR, targeted literature review.

Why is it important to understand BOI and unmet need?

Understanding the BOI and unmet need associated with a disease is an important early step in understanding how patients and their carers, health systems and society are impacted by a disease due to limitations with currently available treatment options. It is also crucial in identifying where there are potential opportunities for a new treatment to improve outcomes and address current unmet needs.

When would we recommend conducting a BOI and unmet need review?

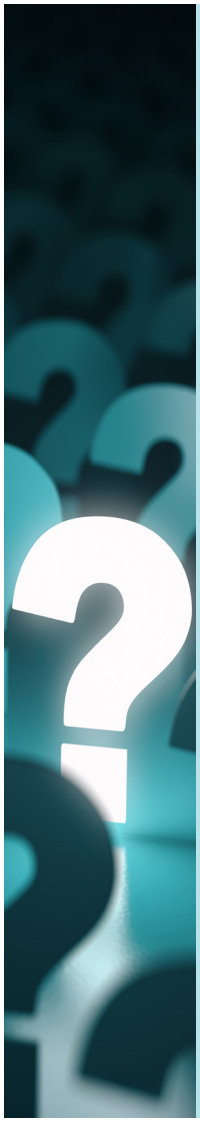
As soon as possible! The sooner you have an understanding of the burden of the disease and any areas of high unmet need, the more proactively you will be able to prepare for market access.

How do we conduct BOI and unmet need reviews?

BOI reviews are generally conducted using targeted literature review (TLR) methods. The methods and process for performing TLRs will be discussed in a subsequent issue of this series: “How To... Undertake a Literature Review”.

References

1. Vreman RA, Heikkinen I, Schuurman A, et al. Unmet Medical Need: An Introduction to Definitions and Stakeholder Perceptions. *Value Health*. 2019; 22; 1275-1282.
2. Zhang K, Kumar G, and Skedgel C. Towards a New Understanding of Unmet Medical Need. *Appl Health Econ Health Policy.* 2021; 19, 785–788.



Key considerations for BOI review methods:

What evidence/information is already known and available internally? These can be used to inform the review and provide guidance on specific objectives.

How rare is the disease (i.e. what is the expected size of the evidence base) and how comprehensive does the search need to be? This will influence the number of databases to search and the specificity of the search terms.

When is the review being performed relative to the product development cycle? This will influence what needs to be included and how comprehensive the search needs to be.

What will the review be used for (early value proposition, GVD, publication etc.)? This will influence how comprehensive and/or structured the searches and methodology need to be and how pragmatic you can be in searching and screening to identify relevant evidence.

Who is the end audience (internal stakeholders, external HTA agencies, etc.)? This will influence the topics to be included and the style of reporting required.

Are there any key evidence gaps? Proactively identifying areas of interest for the review can save time and effort later on in identifying potentially relevant evidence to aid future decision-making and planning.

About the author

Have a question for our author? Use the links below to contact them directly on LinkedIn or by email.

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We hope you enjoyed the first of our series explaining how to undertake early HEOR evidence generation activities.

Follow our VAO LinkedIn channel to find out when the next in the series ‘How to’ develop a HEOR evidence generation strategy is published.