

## Unmet Medical Need – Industry’s Perspective

The aim of this document is to help investigators understand what constitutes an “unmet medical need” from a Regulatory and an Industry perspective.

Currently, the [Food and Drug Administration \(FDA\)](#) defines unmet medical need as a condition that is serious and whose treatment is not addressed adequately by currently available therapy.

*A serious condition must satisfy:*

- *life-threatening nature of the disease or condition based on figures of mortality and life expectancy; or*
- *seriously debilitating nature of the condition based on morbidity over the course of the disease and its consequences on patients’ day-to-day functioning*

Situations in which currently available therapy does not adequately address a serious condition include:

- Where there are no approved molecules
- Where there are or few molecules in late phase clinical studies
- Where existing molecules are compromised by substantial, demonstrated liability at efficacious dose
- Where there is an identifiable subset of patients who are currently underserved

On the other hand, the following situations do not constitute unmet medical need:

- Predicted enhanced safety profile of a molecule compared to approved molecules
- Predicted reduced cost of a molecule compared to approved molecules
- Replacing a combination treatment regimen with a single agent

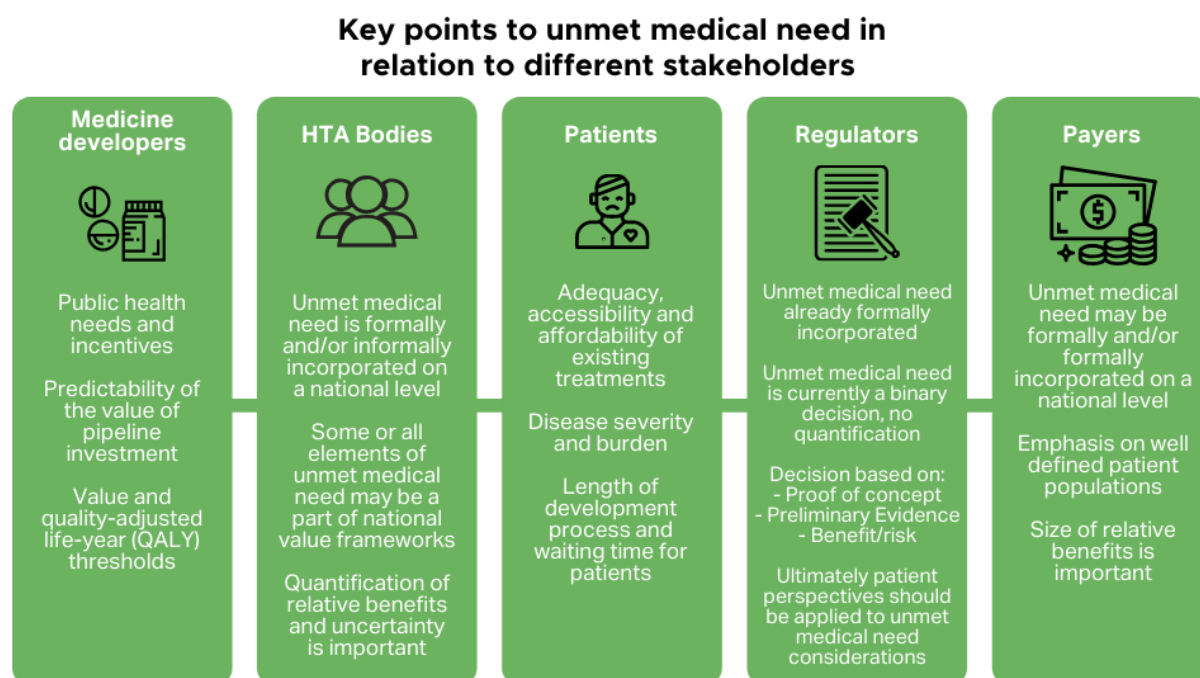
Where there are approved treatments for a serious condition, a novel molecule must have compelling head-to-head preclinical data against established or future clinical benchmark(s). This is referred to as “differentiation.” In this context, preclinical studies are required that conclusively demonstrate that the novel molecule will be:

- More effective than standard of care
- More convenient than standard of care

While the FDA guidance is excellent in the context of available therapy and regulatory path, that is only one part of the story. Unmet medical need may be viewed differently by other important groups, perspectives or factors. These include considerations of:

- The patient population - taking into account disease incidence, prevalence and orphan designation
- Disease severity – ascertaining the impact of the severity and burden of disease, as well as end-of-life criteria
- Alternative treatments – determining the number of alternative treatments, how accessible they are, the remaining morbidity and the benefits of a novel molecule over alternatives

Similarly, it is important to take into account the considerations of different stakeholders and perspectives when developing a new therapeutic (Figure 1). These include patients, payers and health technology assessment (HTA) bodies.



**Figure 1:** Stakeholder considerations on unmet medical need. (Adapted from Vreman R et al. (2019) Unmet Medical Need: An Introduction to Definitions and Stakeholder Perceptions, *Value in Health*; 22(11):1275–1282).

In summary, based on Regulatory requirements, industry defines unmet medical need as when there is a serious condition where:

- a) No available treatment exists or
- b) Available treatment exists and the proposed product is expected to differentiate from available treatment based on one or more of:
  - i. Patient selection,
  - ii. Efficacy,
  - iii. Treatment compliance.

For further information on this, or any other topics related to the drug discovery and translation process, please email the BioCurate team on [info@biocurate.com](mailto:info@biocurate.com).

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