A Consolidated Framework for Collaboration in RWE Generation for a Medical Product

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BACKGROUND

- Real-world evidence (RWE) generation for medical products is needed for regulatory and reimbursement decision-making and market access, and to aid clinician and patient understanding of how a product fits into the choices available for treating disease.
- Within pharmaceutical and medical device companies, this evidence generation is often housed within multiple business units that may be siloed or placed in competition.
- We have noticed that frameworks used to plan and assess evidence for regulatory and reimbursement needs have overlaps that can be leveraged to better use organization-wide resources by encouraging collaboration.

OBJECTIVE

- We developed an integrated framework for RWE generation by comparing and contrasting development of a value strategy for reimbursement with use of a structured benefit-risk assessment (sB-R) for regulatory decision-making.

VALUE STRATEGY

- Developing and understanding how to communicate a product's value for reimbursement is essential for successful market access.
- Value strategies are developed to provide evidence that the value of medical products support reimbursement levels. All aspects of the product should be considered from multiple perspectives so that consistent messages and evidence are presented to payers and other stakeholders.
- The value strategy is useful in determining data gaps and assisting the team to identify endpoints for additional studies. The value strategy can be updated as new information is generated postmarket and thus can continue to provide consistent messages and evidence to payers and other stakeholders in periodic updates.

Value Strategy Framework

Understand the disease burden and unmet need

– Describe disease landscape

– Determine unmet need within disease context and specifically addressed by the product

Identify potential product value

– Known benefits of product

– Develop value messages based on existing data

– Develop aspirational value messages

Identify data gaps

– Identify data to collect to address the gaps

– Prioritize timing and sequence of new studies

Clarify the dissemination priorities

Develop strategy

Gather data

Conduct research to meet gaps

Figure 1. Value Strategy Process

CREATING A CONSOLIDATED FRAMEWORK

- Both value strategy and sB-R include clarification of the disease landscape, the specific need (i.e., indication) addressed by the product, a description of the value messages (i.e., benefits) for the product, and data identification.
- The value strategy can provide a structured approach to identify data gaps and prioritize data collection to address those gaps—both of which are needed for regulatory risk management documents.
- The sB-R can provide a structure to arrive at consensus language on known risks and the benefit-risk balance.
- Prioritization of timing and sequence of new studies, which are part of the value strategy, can be leveraged to meet the needs of those who address both regulatory and reimbursement stakeholder requirements.

COMBINED PROCESS

1 *Describe landscape*

– Disease (including unmet needs)

– Products

- New product
- State of the art
- Other products
- Unmet need

2 *Identify known benefits of product and value messages*

– Aspirational value messages

3 *Identify known risks of product*

4 *Develop strategy*

– Value tree of key benefits and risks

– Clarify dissemination priorities

5 *Gather data*

– Clarify measurable outcome definitions

– Obtain available data and collect in data table

6 *Present known knowledge*

– Create data visualization

– Expert judgement and interpretation of data

7 *Identify data gaps*

– Identify data to collect to address the gaps

8 *Conduct research to meet gaps*

– Prioritize timing and sequence of new studies

9 *Present new knowledge and update strategy*

– Create data visualization

– Expert judgement and interpretation of data

– Affirm or modify data collection and dissemination priorities

STRUCTURED BENEFIT RISK

- Benefit-risk assessment is conducted to provide evidence that the benefits of medical products outweigh the product's risks. A structured approach ensures that all aspects of the product and its use have been considered from multiple perspectives and that a consensus is presented to the regulator (or other stakeholder).
- The sB-R is useful during premarket for go/no go considerations, determining gaps in data, and assisting the team to identify endpoints for clinical studies. The sB-R can be presented to regulators within the benefit-risk section of the medical product applications.
- Additionally, the sB-R can be updated as new information is accrued postmarket and thus can provide a consensus-driven approach to further characterization of the benefit-risk profile reported to regulators and other stakeholders in periodic updates.

Unified Methodologies for Benefit-Risk Assessment[2]

Frame the decision

– Describe decision context

Identify benefits and risks

– Build value tree

– Refine value tree

Assess benefits and risks

– Determine relative importance of benefits and risks

– Evaluate the options

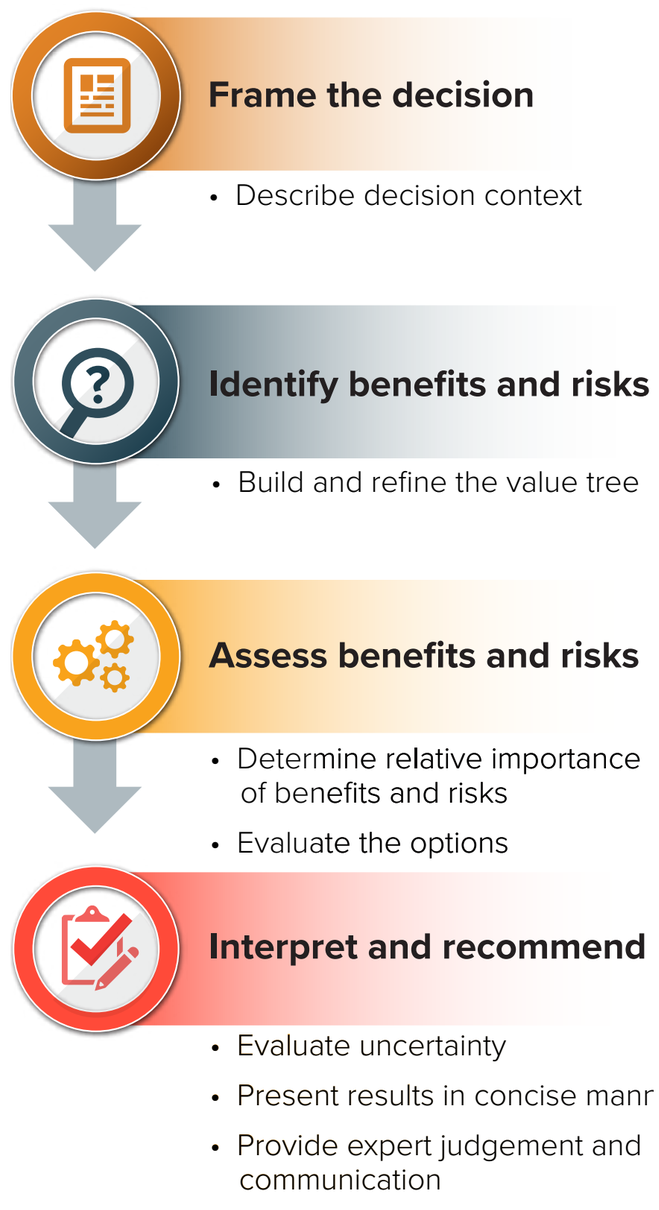
Interpret and recommend

– Evaluate uncertainty

– Present results in concise manner

– Provide expert judgement and communication

Figure 2. Benefit-Risk Assessment Process



CONCLUSIONS

- Sharing RWE planning and generation across a consolidated framework allows for the possibility of utilizing one study to meet multiple business unit needs.
- This allows for alignment of messaging around RWE across a company.
- We recommend testing and codification of this consolidated framework within companies looking to strengthen their RWE pipeline.

References

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