IVI INNOVATION AND VALUE INITIATIVE

VALUE BLUEPRINTS
Research Brief Series

Open-Source Modeling for Value Assessment: Promises and Challenges

November 23, 2020

POLICY QUESTION

How does open-source modeling contribute to value assessment, and what challenges exist to its expanded use in the field?

KEY TAKEAWAYS

- Open-source models are developed and disseminated with open access to source code, technical documentation, and assumptions and sources of model inputs.
- Primary benefits of open-source modeling include: improving trust and validity in healthcare value assessment, promoting testing of novel methods and inputs, and accelerating shared learning.
- Despite growing interest and commitment to open-source approaches, challenges to widespread adoption remain, such as: managing intellectual property, defining a process for adjudication of use and modification, and sustaining developers' incentives to invest in both original models and updates over time.
- Ongoing efforts like IVI's Open-Source Value Project (OSVP) are necessary to address existing challenges and expand open-source modeling.

WHAT IS OPEN-SOURCE MODELING?

Value assessment is conducted using economic and epidemiological simulation models to support decisions about how to best allocate healthcare resources in, for example, treatments for a specific disease. These models are generally complex computer simulations designed to simulate benefits and costs associated with different treatments or interventions and include assumptions about what outcomes are important, what processes drive different outcomes, and the input parameters that support the simulation.

ORIGINS OF THE TERM "OPEN SOURCE"

The term "open source" used to describe software was first coined in the 1990s as an alternative to the term "free software." Open-source refers to explicit permission to use the source code, design documents, or content of the product. Today, the term's use has expanded far beyond software to cover other forms of open content (e.g., open access journals) and open collaboration (e.g., OpenNotes, open data sets). However, unlike open-source software, open-source modeling implies not only release of the code but, because these models are used to make decisions that affect the healthcare delivered to patients, also the specific assumptions, parameters, and supporting data used to reach specific conclusions.

In general, most models are proprietary, which means the assumptions and inner workings of calculations are not available for critical review or replication. There is a growing call in the field of cost-effectiveness analysis, however, for open and transparent processes for the creation, dissemination, and use of models for value assessment.[1,2]

In open-source modeling, modelers release simulation models for which the full source code, underlying data, and supporting documentation are free to access, review, use, modify, and redistribute with attribution to the original developers.[3]

INCREASING INTEREST IN OPEN-SOURCE MODELING

Since its inception in 2016, IVI has emphasized open-source value models[4] as the testbed of novel methods in value assessment and has led the field by creating two publicly available prototypes. Other examples of thought leadership include the Center for the Evaluation of Value and Risk in Health (CEVR) at Tufts University[5], which initiated a clearinghouse of open-source value models[6], and the International Society for Professionals in Outcomes Research (ISPOR), which has organized a member special interest group[7] to explore the issues and consider solutions.

WHY IS OPEN-SOURCE MODELING IMPORTANT?

The COVID-19 pandemic has underscored that rapid-cycle learning is imperative and that health researchers, economists, and the industry are finding ways to share data and contribute vital insight through open-source epidemiological modeling without losing competitive position. Similarly, open-source principles can enhance the ability of value assessment models to support evolving decision-making about resource allocation.

Determining value in healthcare is exceedingly complex and reliant on incomplete and evolving evidence and real-world data. This scientific uncertainty is exacerbated by the choice of structural assumptions[a] and model parameters that may or may not be transparent or accessible by others for validation and replication.

Such opacity yields two problems. First, the lack of transparency makes generating consensus on findings, and especially demonstrating relevance to the patient community, extremely difficult. Second, the inability of a broader scientific community dialogue about novel methods and challenges in data uncertainty slows the learning cycle and interrupts innovation in defining and paying for the highest value interventions.

[a] These assumptions can have significant implications for findings, and understanding uncertainty around these impacts is essential. For more see IVI's research brief on structural uncertainty.

WHAT ARE THE BENEFITS OF OPEN-SOURCE MODELS?

Transparency

Open-source models promote transparency regarding underlying assumptions used to create model structures and the data used to support the model simulations. Open access to the source code underpinning a model allows a full review of the developer's assumptions and technical choices. A natural result of greater transparency in value models is wider trust in the intent and utility of an open-source model, enhanced credibility for the approach and methods used, and the opportunity to validate new methods and structural components of value models.

Replication

Open-source models can support greater functional ability to replicate and validate the analyses conducted with the model. The ability for wider sensitivity testing can "crowdsource" model validation and improve the acceptance of model design innovations faster than traditional academic publication and dissemination strategies, for example.

Flexibility

Transparent development of open-source models and components could further facilitate modifying model structures and inputs as new data or new interventions emerge. Publication of source code and technical documentation can speed model adaptation and facilitate wider use across decision-maker needs. Model flexibility also presents the opportunity for more robust testing of scientific uncertainty and impacts of subgroup heterogeneity.

Perhaps the most extended view is the use of open-source models as a foundation for constructing common building blocks for value assessment. By fostering consensus on inputs, structures, model assumptions, and how to employ real-world data sources, open-source models might enable the creation of reference models to accelerate value assessment across multiple and co-occurring disease states.

Increased Efficiency and Knowledge-Sharing

Providing open access and presenting model architecture as "creative commons" assets can accelerate the testing of advanced methods and value elements and improve the efficiency of the value assessment field overall.

Open-source models and their developers allow user communities to adapt, test, and propose modifications to model prototypes, and to reciprocate the investment of original model developers by bringing back learning and improvements that benefit all.

IVI'S OPEN-SOURCE VALUE MODELS

IVI Open-Source Value Project (OSVP) models are accessible through both user-friendly online interfaces and statistical packages of R software, and complete source code and documentation are available through a public site hosted on GitHub[b]. OSVP disease-specific models allow the user to:

- Compare the cost-effectiveness of multiple sequences of treatment by population sub-groups
- Compare traditional cost-effectiveness analysis with preference-weighted multi-criteria analysis
- Modify the treatment sequences, structural assumptions, and values and distribution of parameters in simulations
- Utilize novel elements of value in the model, such as insurance value or value of hope
- Download and view R-based source code
- Modify and utilize the model prototype with acknowledgment of IVI and OSVP model developer Precision Health Economics

OPEN-SOURCE MODELING IN PRACTICE

Open-source modeling is gaining momentum in the field of health economics and holds the promise of creating wider acceptance and consensus about best practices and methods in value assessment. In 2017, the Innovation and Value Initiative created the Open-Source Value Project (OSVP), an initiative to test and advance methods by developing open-source models to assess the value of drugs and therapies for specific diseases. In addition, organizations such as the Institute for Clinical and Economic Review (ICER) have explored the feasibility of open-source model development.[8]

IVI's experience confirms the potential benefits described above but also highlights several practical challenges that currently limit more widespread adoption and implementation of open-source modeling.[9] In addition to the need for greater consensus on the definition and best practices for open-source modeling, these practical challenges must also be addressed.

Intellectual Property

The issue of proprietary ownership of the source code and model techniques has been a significant barrier to collaboration, commitment to open-source development, and widespread repository of such models for common use. The lack of clear standards and guidelines for incentives, documentation, acknowledgment, and the disposition of derivative products or methods has perhaps suppressed active engagement in open-source model development. Use of open-source software (e.g., R) and platforms (e.g., GitHub; Shiny) offer an opportunity to resolve some of these licensing challenges.

Resource Requirements

In addition to the investment of intellectual effort and contribution, the financial and technical resource investment in open-source model development is significant and may not be fully recoverable, though efficiencies may arise over time. Model developers may not want to share their work without license and without a fee to recoup the original investment, for example. Decision-makers are unlikely to interact directly with models, thus requiring investment and technical resources to create user interfaces that facilitate decision simulation for these actors. Moreover, the process and investment required to update and adapt model coding, and the inputs (data, parameterization, network meta-analysis) is significant. Philanthropic and crowd-sourced financing mechanisms could help create momentum for open-source model development.

To date, IVI has elected to make open-source model development a central element of its research investment. This has required significant investment in the development of both R and C++ based models, and user input research to inform the development of sophisticated user interfaces for both technical and non-technical user audiences. IVI continues to target membership contributions fully toward research and development that support such work.

[b] https://github.com/InnovationValueInitiative

Change and Version Management

Another challenge is creating a consistent and sustainable process for change management of the original open-source models. In addition to significant effort and documentation required for model updates (e.g., due to new evidence or technical revisions recommended through formal review and comment periods), there is the need for infrastructure and standard process for documentation of model modifications by third parties and for "version control." One potential solution is the creation and maintenance of a repository of open-source models, which could provide a centralized resource of all original and derivative models. CEVR has piloted such a clearinghouse but has acknowledged the challenges in attracting submissions from an as yet reticent field.[10]

On the implementation level, IVI has grappled with this challenge since release of OSVP models in 2018 (IVI-RA) and 2019 (IVI-NSCLC). Updates based on evidence, technical upgrades, and changes to the network meta-analysis can take 9-12 months and require significant financial resources. Simultaneously, the field lacks a clear metric for deciding when an update or modification is beneficial, if not required. Given the rapid evolution of clinical evidence, the rising importance and availability of real-world data, and the grown demand for value assessment in complex disease states, finding consensus on exceptions and feasibility issues is of high importance.

CONCLUSION

The growing interest in open-source modeling is a positive trend for the value assessment field. More robust investment and collaboration are needed to fully realize the benefits of such an approach to accelerated learning and methods improvement. By offering models that are transparent, flexible, and a common good, IVI contributes to accelerated learning, open and inclusive collaboration, and the opportunity to advance modern methods and tools for real-world healthcare decision-making.

ABOUT THE INNOVATION AND VALUE INITIATIVE

IVI is a 501(c)(3) nonprofit research organization committed to advancing the science, practice, and use of value assessment in healthcare to make it more meaningful to those who receive, provide, and pay for care through collaboration among thought leaders in academia, patient organizations, payers, life science firms, providers, delivery systems and other organizations.

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