How biotechs can strengthen their value story with advanced analytics

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Small and emerging companies must make big decisions about what to do with their assets early in development. Should they seek venture capital (VC) funding to develop a product themselves or seek a partner once it shows clinical activity? They need to know their product candidate's value and commercial viability to make the right decision, including the market size, growth and revenue potential.

Recent advances in machine learning and predictive analytics have empowered health economics and outcomes research (HEOR). Sophisticated tools are now available to help build a data-driven value story that can help attract investors and partners, convince payers, and benefit patients.

Here are five ways biotech companies can enhance their value story with advanced analytics.

1 Accelerate literature reviews

Machine learning and natural language processing (NLP) can help companies conduct faster and better systematic literature reviews than they can achieve manually.

Instead of individual reviewers searching through databases, a keyword-coding algorithm screens publication titles and abstracts to identify text with content relevant to the study objectives. While manual reviews are still needed to validate full texts and extractions, NLP can save 30% to 70% of the time spent on screening, allowing people to perform higher quality tasks.[1] Parexel is at the forefront of deploying NLP to expedite and optimize literature reviews.

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2 Leverage real-world evidence

Parexel recently worked with a client to construct an external control arm (ECA) that provided essential context for a Phase II single-arm trial's efficacy results.

After intense, detailed, early interactions with the FDA on ECA study design, endpoints, and the analytical plan, we identified real-world data sources. We performed a full-scale feasibility analysis of a dozen secondary datasets in the United States and Europe. Secondary data are data gathered outside of the context of a randomized controlled trial and include electronic medical records, insurance-claims databases, patient and disease registries, and population health surveys.

We narrowed the choices to three U.S.-based data sources and direct on-site retrospective chart reviews from Europe. We assessed the technologies and vendors to map, aggregate, and de-duplicate the data and successfully matched patients 1:1 with the Phase II study population against multiple covariates.

While it took the sponsor 24 months to enroll the trial's treatment arm, we developed, analyzed, and submitted the ECA to the FDA in 16 months. The product was approved about eight months later, well ahead of schedule. Using advanced analytics to construct a high-quality ECA produces data that can feed directly into a cost-effectiveness model. The value goes beyond regulatory considerations, helping companies defend reimbursement of the product to health technology assessment (HTA) agencies. Advanced analytics is a differentiator in assessing product value with the evolution of healthcare ecosystems and the trend towards real-world evidence being integral to clinical trials.

3 Optimize pragmatic trial design

High-quality feasibility studies lead to efficient clinical trials that recruit and retain patients and finish on time. Further, analytical testing of a trial protocol enables sponsors to identify and remove unrealistic or impractical design elements, and avoid multiple amendments, missed development deadlines, or outright failure.

Machine learning can optimize country and site selection for clinical trials by predicting where target patients live. For example, a symptom-monitoring assessment gauges the prevalence

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of relevant symptoms in a population. Machine learning algorithms can scan electronic health data and prescription fulfillment records to produce a snapshot of patients' current treatments and disease histories, identifying high concentrations of eligible patients and speeding recruitment.

Machine learning can also help identify patients most likely to benefit from therapy. Specifically, Bayesian network machine learning algorithms can be developed to predict optimal patient response profiles and identify the characteristics of patients with a high probability of achieving given outcomes. These algorithms also identify prognostic variables of these outcomes and are informative to include in a trial protocol.

Companies can use Bayesian network machine learning to develop a predictive algorithm that stratifies patients using a drug's mechanism of action. Recently, Parexel used this technique for a client developing a treatment for a solid metastatic tumor. We were able to identify biomarkers to stratify patients to optimize the measurement of outcomes and better assess the product's value.

4 Visualize the future for investors and partners

Companies meeting with potential investors or partners must present robust evidence to support their value proposition. Unmet medical needs are the most important driver of approval and reimbursement decisions. At Parexel® Biotech, we customize a data visualization app for our customer's VC and investment partner meetings. This helps them show the impact of different value drivers on meeting medical needs, cost-effectiveness, and market potential. A health economics expert can manipulate complex data, test different scenarios, and present the results interactively in an easily readable format on a tablet. Complex modeling of future uncertainties - that used to be done in the background - can now be accomplished together with potential stakeholders.

Machine learning can also help identify patients most likely to benefit from therapy.

For example, we recently developed a web-based data visualization tool for a client's immuno-oncology drug. The program calculated multiple scenarios of the drug's relative safety, efficacy, and cost-effectiveness compared to competitors. For this case, we used proprietary expert data on the therapeutic landscape of non-small cell lung cancer to project how payers would value the treatment in the United States and Europe.

Early economic modeling based on advanced analytics tools can also help answer essential questions like, what incremental efficacy should the technology aim to provide meaningful value for patients in need? How can this product be priced and positioned in a competitive landscape? What are the key differentiators from patients' and payers' perspectives?

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5 Screen sites using predictive models

Artificial intelligence (AI) can help identify and locate patients close to the investigative sites to enroll them in clinical trials. For example, recently, we used AI to construct a COVID-19 predictive model to track patients from vaccination through the course of their disease. AI predicted areas with a high incidence of COVID-19 and generated a heat map of high-volume vaccination sites in the United States. The advantages of using AI include not making assumptions about the statistical distribution or functional form, selected features, or limited dimensionality—all drawbacks typical of a non-AI model. While traditional regression techniques are easy to use and may be more familiar in the clinical setting, they are limited in simultaneously handling multiple outcomes and key performance indicators. They require clinician input to augment missing data, empirically leverage data from multiple sources, and reason backward. Using AI typically builds a better model with more accurate predictions.

A different client wanted to optimize site feasibility by predicting the number of severe COVID-19 cases in the United States, Latin America, Europe, and the Asia-Pacific. We applied factor analysis to assess disease prevalence and logistical factors and then ranked the potential sites by likelihood of success. The ranking facilitated optimal patient enrolment for the clinical trial.

It's never too early to think about value demonstration. A robust data-driven HEOR schedule that starts early in development can help biotech companies critically assess their innovations at each R&D step. Utilizing advanced analytics can provide a return on investment, increasing the chances of achieving product differentiation and reimbursement in a competitive market.

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1 O'Mara-Eves 2015;4(1):5. Using text mining for study identification in systematic reviews: a systematic review of current approaches - PubMed (nih.gov)

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