Value Frameworks: Will They Work in the U.S.? What are Stakeholders Saying?

Mike Epstein, MS, MA
Principal, Market Access Consulting, Evidera

Apoorva Ambavane, MPH
Senior Research Associate, Modeling & Simulation, Evidera

Despite lack of formal adoption of value frameworks by U.S. payers, there is widespread assumption/conclusion that these frameworks are influencing U.S. payer behavior and that their influence will grow over time. Examples include but are not limited to:

HEOR academics/consultants: "U.S. payer feedback indicates that ICER [Institute for Clinical and Economic Review] assessments are likely to have an important impact on formulary decision making processes in the United States," from *Value & Outcomes Spotlight.*[1]

Industry trade publications: ICER "will help the VA's pharmacy benefits management services office use ICER drug price assessment reports to decide which drugs to cover and to dicker with drugmakers and wholesalers on price," from *FiercePharma.com.*[2]

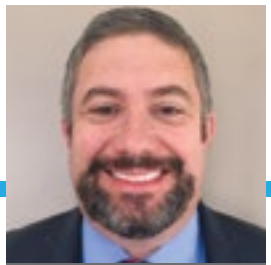
Advocacy organizations: There have also been claims that ICER helped block access to PCSK9s, e.g., "an obscure group called the Institute for Clinical and Economic Review, or ICER, is preventing and bogging down access to these types of medicines for patients in need," from a 2016 opinion piece by the head of the Hispanic Leadership Fund, *FoxNews.com.*[3]

Policy analysts: "According to longtime pharmaceuticals reporter Ed Silverman, 'ICER is becoming a de facto arbiter for the nation's medicine chest.' Take a closer look at ICER's *modus operandi*, and you'll see why this is a horrifying proposition. ICER, which holds itself out as a kind of *Consumer Reports* for drugs, is basically an industry-backed comparative effectiveness calculator. That ICER is [insurance] industry backed isn't the problem, it's that it uses comparative effectiveness to lend an air of legitimacy to the formulary shenanigans," from a 2016 column by Jeff Stier of the National Center for Public Policy Research, *USA Today.*[4]

However, we are sceptical that value frameworks have been influential, or will be influential in the future.

For inline products, market forces are determinative. One payer told us, "we certainly won't move something around on formulary, and ICER hasn't affected and won't affect contract negotiations. Especially if one manufacturer has a ton of market power, they'd laugh if I told them to give a larger rebate based on an ICER report."

Even for pipeline products, payers see many obstacles to the value frameworks:


Mike Epstein


Apoorva Ambavane

"ICER is more of an ex-U.S. approach – P&T [pharmacy and therapeutics] doesn't talk about cost/QALY."

"The unfortunate reality is, a lot of big payers like PBMs [pharmacy benefit managers], they make a lot of their money on rebates. Does everyone have aligned incentives for low net cost and cost-effectiveness?"

"Where they will be potentially useful is if we can get more to the NICE example - you don't hit some threshold we can all agree on, you're not on formulary. But the U.S. is not a one-payer system, and the benchmark to ESI is different vs. United vs. my PBM. A fragmented system makes it more difficult to use these."

As a result, value frameworks have not had significant impact and are unlikely to do so in the future, as shown by a few representative U.S. payer comments.

"Usefulness of value frameworks has been modest at best."

"I've never gone after [manufacturers] with this. I've seen press releases and statements, but as formulary contract manager I can tell you I've had no specific conversations with any individual manufacturer if their drug isn't hitting a benchmark on an ICER report."

"On their value-based price benchmark, to be more applicable, instead of a QALY [Quality Adjusted Life Year] I want a WAC [Wholesale Acquisition Cost] or a net price per hard outcome achieved – something understood by P&T members."

The barriers to value frameworks in the U.S. fall into three categories.

Category 1: Structural/Systemic Barriers

Structural/Systemic Barrier 1: Greater competition doesn't necessarily lead to higher value

First, competition with other insurers makes each insurer leery of being the first or only insurer to try to enforce the findings of value assessments – lest they lose business and fall prey to a public relations and stock price disaster.

"How would you like to be on the front page of a paper saying you're not paying for little Johnny's cancer therapy, and the boycotts, and the hits to your stock price? So you spend a few hundred thousand dollars on wasted effort to give false hope." – Medical director, top 10 national health plan

"The oncologist has to be the face of it. Memorial Sloan Kettering Cancer Center (MSKCC) said Zaltrap is not cost-effective, we're not going to pay for it, so the manufacturer renegotiated. If we did that, we'd be accused of being a death squad. Bad publicity. Bad PR could get the Department of Insurance looking at you, and bad articles, and you could get dropped by oncology groups. Losing access to that network is a big deal because you don't have independent oncologists anymore." – Formulary and contracts manager, regional PBM

Second, competition doesn't necessarily mean lower prices as suggested by economic theory. In some cases, greater competition allows payers to play manufacturers against each other and extract price concessions (e.g., the Hepatitis C virus market). In other cases, each new product tends to set a new price benchmark which the next entrant takes as a new "floor" price. This is especially likely to happen where products are not seen as entirely interchangeable (e.g., categories like multiple sclerosis, in which payers value having multiple approaches available to prescribers and patients).

"The usual idea is more competition means lower prices, but in pharma whenever you get more competition, prices just go up anyways. Discussion is always like this: the prior product got x dollars so I want x + something. So more competitors means prices go up anyways." – Pharmacy director, regional affiliate of top 10 national health plan

Structural/Systemic Barrier 2: Market dynamics may put payer in weak position

Political pressure, legal requirements, competition, any of a variety of forces at play in the U.S. market, may combine to put a manufacturer in a powerful bargaining position vis-à-vis a payer. This is especially the case if the manufacturer is "the only game in town" for a particular condition, usually an orphan condition.

"With some orphan drugs they just come and tell us how much it will cost, and that's that. Our PBM called an orphan drug-maker recently to discuss price and access, and they didn't even get their call returned." – Medical director, regional health plan

In other cases, the manufacturer wields tremendous power by virtue of utilization patterns.

"I could bring an ICER report to some manufacturer and tell them they need to charge me a value-based price, but if they have 50% market share, they won't give me the time of day." – Formulary and contracts manager, regional PBM

Thus, even if a value-based price and a manufacturer's bargaining position are at significant odds, there may be little a payer can do.

Structural/Systemic Barrier 3: Perverse incentives are misaligned with "value"

Comparative clinical effectiveness may pale in comparison with the importance of price in payer decisions, even to the point of irrelevance in some highly saturated categories with many alternatives seen as interchangeable.

"Preferred drugs are preferred because the PBM gets more favorable pricing. It has NOTHING to do with anything clinical – at all." – Medical director, national plan (emphasis within quote is the payer's)

Even when it comes to price, the story is not simple. ICER has amended its method so that it uses an estimate of net price rather than list price – but even net price is not necessarily determinative of coverage/preference over clinically equal products, because the PBM incentive is to maximize its rebate revenue stream while keeping the price their health plan customers pay low. The PBM incentive is NOT to keep net cost low. This sometimes means preferring/protecting a product that has an interchangeable clinical profile with a much less costly alternative, but high share and impressive rebate revenue stream for the PBM.

"Incentives in our system aren't aligned for low net cost. Some PBM could prefer a product that's 2x dollars over a drug that's x dollars because the PBM gets a rebate that's twice as big with the more expensive drug." – Pharmacy director, regional plan

Finally, co-pay cards interfere with value-based decision-making. Some plans refuse such cards because they interfere with the plan's efforts to share financial responsibility.

Structural/Systemic Barrier 4:
There are legal challenges to value-based decisions

Consider federal and state laws. Medicare protects access to all drugs in six classes (previously "all or substantially all" according to the 2003 Medicare Modernization Act [MMA], but the 2009 Affordable Care Act [ACA] changed to "all"): anti-convulsants, anti-depressants, anti-neoplastics, anti-psychotics, anti-retrovirals, immuno-suppressants. State Medicaid rules may guarantee access to low-value drugs as well; even technologies that fail to make the Preferred Drug List are obtainable through appeal.

"In Medicaid, normally state law requires that you cover all FDA-approved drugs. You can't not cover it, period. We've had plenty of examples [of drugs] with less evidence, things that are just bad for patients that we've had to cover – think about bone marrow transplant for breast cancer… Also, just because we don't put a drug on formulary, doesn't mean a person can't get it. They just have to go through more hoops." – Medical director, regional health plan focused on Managed Medicaid

Category 2: Normative/Cultural Barriers

Normative/Cultural Barrier 1:
Frameworks and payers define value differently

There is no consensus among U.S. payers on the appropriateness of the cost/QALY metric, let alone on the widely cited $50K/$100K/$150K per QALY thresholds.

There is no consensus in the U.S. on the appropriateness of Bentham-style utility maximization; rather, there is a widespread consensus around the notion of 'no patient left behind,' like no child left behind in education. There is consensus among most U.S. payers that quality of life, a key part of the metric ICER hangs its hat on, is 'uninsurable' – employers, who sponsor the majority of insured in the U.S., do not assign high value to it in most categories.

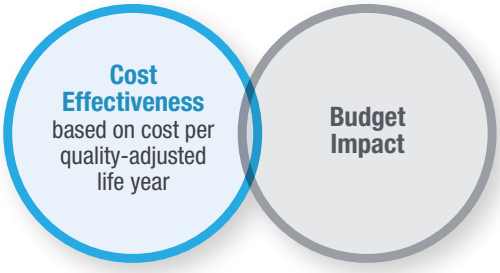
"We don't value quality of life because the employers don't value it. Self-insured employers, if you say this might be better for your employees because they'll have better QOL on some metric, they look at you like you have three eyes. Only if it costs the same as another option, then they say okay. They're not interested in paying for it. In a 20-year span, behind closed doors I've never heard anyone say they would pay for better quality of life... How the employer defines value – the lowest cost they can get. Publicly they will define it differently, but privately, lowest cost." – Medical director, regional affiliate of a national plan

Another problem, a serious one for ICER, is that most providers do not accept the ICER metric as meaningful.

"The best way to get a physician to tune out is just talk about QALYs. Some don't know. None care. P&T doesn't care, either." – Pharmacy director, regional health plan

U.S. Payer Definition of Value

ICER Definition of Value



Normative/Cultural Barrier 2:
Frameworks compete with established approaches

Value frameworks didn't emerge into an environment with no history of consideration of value for money. To quantify value, some U.S. payers already calculate cost per outcome – the metric which is meaningful to them since it incorporates hard endpoint while excluding QOL.

> *"We divide into delta vs. placebo for each drug the net cost for some given time period on the key metric – HbA1c, ACR 50 or 70, ARR in MS, SVR in HCV – this is cost per outcome. That's what we care about so that's what we do in our P&T process."* – Formulary and contracts manager, regional PBM

> *"Value? It's just efficacy divided by price. But it's hard to know what efficacy is in some areas. Pomalyst only had response rate, but no survival, for $95-120K per year. The problem is, response doesn't mean you live longer. "* – Medical director, regional health plan

The new frameworks have to demonstrate they allow payers to achieve their goals (e.g., health plan: maximize profit) better than current approaches. Unless there is some structural change, frameworks also have to analyze the environment from the PBM perspective and show PBMs there is something in it for them.

The new frameworks have to demonstrate they allow payers to achieve their goals (e.g., health plan: maximize profit) better than current approaches.

Normative/Cultural Barrier 3:
There are political challenges

Pressure from advocacy organizations and politicians can be more influential in payer decision-making than value defined by a value framework. Common examples payers offer are early cognitive behavioral intervention in autism, determining the minimum age for mammography, and Exondys-51 in Duchenne muscular dystrophy (DMD).

> *"The autism lobby has made rounds of state legislatures and been very successful in getting things covered for autism, like for early cognitive behavioral intervention that's never brought solid evidence it works. They can bypass that by going to the politician and saying it works, and it becomes more political than anything else, not value-based."* – Pharmacy director, regional affiliate of top 10 national plan

> *"We're not using cost in our decisions; if we did, we wouldn't cover lots that we do, e.g., mammography – not politically correct, and the U.S. Preventive Services Task Force got shot down when they tried to raise the age limit.'"* – Medical director, regional affiliate of top 10 national plan

> *"Exondys 51, the first drug for DMD, was approved and accelerated based on raising muscle dystrophin levels by 2% with no outcomes. Anthem said we won't cover, that hit the press, and now Anthem is back-pedaling. So when we try to bring value in, we get a lot of dirt on our faces."* – Pharmacy director, regional health plan

> *"We also have to deal with people calling their U.S. senator and saying my son can't get the new drug for DMD, newspaper coverage, etc."* – Medical director, regional health plan focused on Managed Medicaid

Category 3: Barriers Specific to Today's Frameworks

Framework-specific Barrier 1:
Frameworks have to influence prescribers too

Payers alone do not determine outcomes in U.S. healthcare. Payers consult heavily with key opinion leaders (KOLs) in most conditions. If KOLs disagree with the framework developer's approach and/or conclusions, or simply question credibility, frameworks will have difficulty affecting payer behavior. Payers also factor in the likely reaction to their policies by general prescribers. Payers report low awareness of ICER among rank-and-file prescribers; among those aware of ICER, payers perceive that prescribers question how ICER is qualified to guide medical decisions.

> *"ICER needs to do a better job on publicity and promotion and building connections to the clinical community. It's partly an awareness problem that people haven't heard of it on the clinical side. They also need to include clinical perspectives in their work as I have read assessments saying they're too actuarial and not clinical enough."* – Medical director, regional affiliate of top 10 national plan

Today's frameworks credible to prescribers don't discriminate among drugs very well, while frameworks credible to payers discriminate but aren't credible to prescribers. Payers say that for a value framework to have impact, it must have a sufficiently strong reputation among all important stakeholders, including clinicians, and discriminate among therapies, selecting some as high-value and some as low-value, laying groundwork for covering some but not others, providing preferential coverage of some over others, etc. But there is a catch-22 – medical society frameworks have a good reputation with prescribers but don't distinguish. Frameworks developed by medical societies are viewed as slanted and non-discriminatory (e.g., the National Comprehensive Cancer Network [NCCN] gives almost everything they approve a 2A or better; see *Table 1*), although they are credible to prescribers and useful to payers as a foundation for rejecting drugs rated as poor by the framework. On the other hand, third-party frameworks (i.e., ICER) discriminate but are not credible to prescribers. ICER is seen as more objective by payers, but to date has little, if any, credibility

in the eyes of prescribers who are aware of it. ICER is seen as too actuarial and not clinical enough in terms of who developed it and the analyses themselves.

"Usually we don't look at the value frameworks because they almost seem to be self-serving. ICER is probably more neutral. The ones that are provider-based, it's difficult to accept they're all being altruistic and are trying to be in the best interest all around." – Medical director, regional health plan

"A majority of the clinical community would look on ICER as a non-clinical, insurance industry-based entity that doesn't have clinical credibility." – Medical director, regional affiliate of top 10 national plan

"Manufacturers spend a ton of money with ASCO. That makes it susceptible to a certain level of influence." – Medical director, regional affiliate of top 10 national plan

Table 1.
NCCN Categories of Evidence and Consensus[5]

Category 1:
Based upon high-level evidence, there is uniform NCCN consensus that the intervention is appropriate.

Category 2A:
Based upon lower-level evidence, there is uniform NCCN consensus that the intervention is appropriate.

Category 2B:
Based upon lower-level evidence, there is NCCN consensus that the intervention is appropriate.

Category 3:
Based upon any level of evidence, there is major NCCN disagreement that the intervention is appropriate.

Framework-specific Barrier 2: ICER reports have come too late to effect change

To date, ICER's reports come too late to affect initial formulary placement. By the time initial formulary placement is set, market dynamics take hold and position is difficult to change later simply due to a value framework report. Timing issues – and the extent to which they are addressable – differ by the subject of the framework report. For pipeline products, payers typically make coverage/management decisions for high-profile products before FDA approval; they need to be ready when prescriptions come in, as an individualized process has unbearable transaction costs. ICER reports on pipeline products have come out too late to inform these decisions. ICER is reportedly going to begin issuing pipeline product reports earlier, i.e., two months before the Prescription Drug User Fee Act (PDUFA) date. While issuing the reports earlier will help with the timing barrier, it will not help with the other barriers noted. For inline products, payers foresee little to no impact of reports on established categories. By the time the reports come out, member and provider utilization patterns and preferences are set, as are contracts and rebate revenue streams, guidelines, etc. Little impact is expected beyond marginal price concessions for products whose manufacturers have little leverage.

Framework-specific Barrier 3: Framework organizations are not injecting new data into the mix

The data used by value frameworks organizations like ICER are the same data available to any other third-party evaluation organization (e.g., the Agency for Healthcare Research & Quality [AHRQ], ECRI, Hayes, Blue Cross and Blue Shield Associations' Technology Evaluation Center [BCBS] Tec) and any payer, making framework developers' comparative effectiveness research (CER) redundant to work done for P&T. So, the comparative effectiveness research done by a value framework organization should arrive at the same general conclusions as those produced by any other entity – and that is exactly what we have seen.

"They're not doing new research. Also, for lots of products we don't have head-to-head, so they're doing meta-analysis. I like what they're trying to do, but we do meta-analysis, too, as do other third-party organizations, and all they're doing with their clinical comparisons is using what's public. So I don't expect any new discoveries." – Medical director, regional health plan

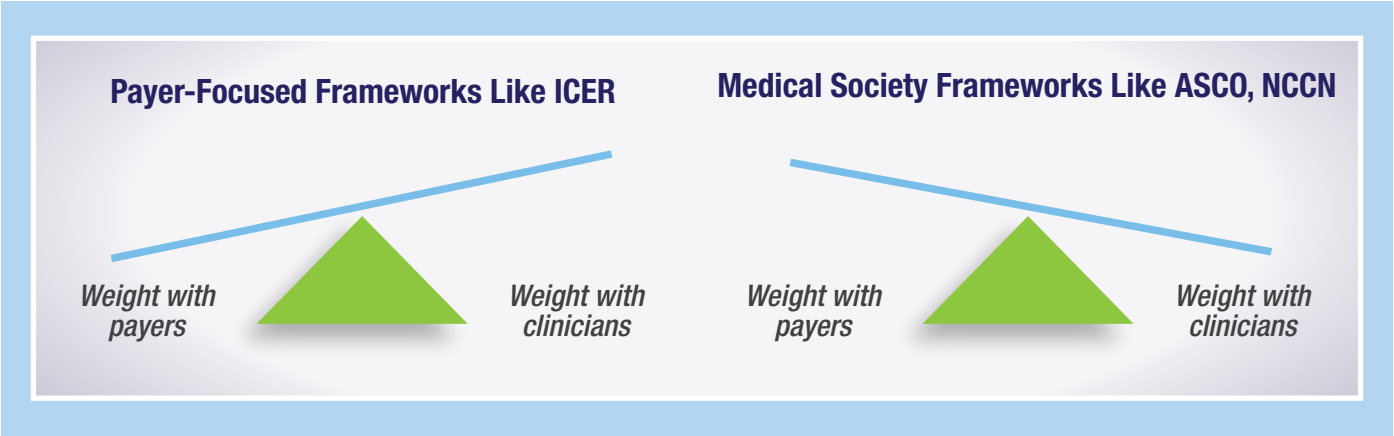
Also, among the small minority of plans that calculate/use cost per QALY, ICER cost-effectiveness analysis (CEA) is redundant to internal analysis.

"With the PCSK9s, all the agencies like BCBS TEC, ICER, and the others did their analyses. My plan did its own cost-effectiveness analysis. Guess what? We all came to basically the same conclusion that plans which don't do CEA came to, which ICER also came to, which is that paying $14K for these drugs doesn't make sense." – Pharmacy director, regional health plan

Framework-specific Barrier 4: ICER's budget impact analysis: widely criticized by payers

Payers neither devote nor want to devote a similar budget to each new drug, as profit maximization may dictate spending more on areas with large populations, greater disease burden, greater need, and/or higher drug quality/incremental benefit.

"With that budget impact analysis, ICER has the same $900 million threshold for all drugs. But really, cancer should be different from diabetes." – Formulary and contracts manager, regional PBM



This criticism is related to U.S. payers' concerns about use of cost/QALY as a metric; a key underlying issue is that in the U.S. payers' view, one size does not fit all - not for budget impact and not for a value metric. U.S. payers consider each therapeutic category on its own merits, asking how a new product compares to standard of care in that condition – just as in Bismarckian systems in France and Germany. Payers also wonder how often the budget impact threshold is binding in ICER's analysis; most often, it seems to be the cost/QALY threshold that binds and "sets" the value-based pricing (VBP) benchmark.

Clinical Organization Frameworks

Although the ICER framework is viewed with scepticism by payers, clinicians, and other healthcare providers, there are other value frameworks which have been recently introduced. Prominent clinical organizations, such as NCCN – Evidence Block,[6] American Society of Clinical Oncology (ASCO),[7,8] European Society of Medical Oncology (ESMO) – Magnitude of Clinical Benefit Scale,[9] and MSKCC – DrugAbacus,[10] have introduced value assessment frameworks. The primary objective of these frameworks is to evaluate various oncology treatments, in terms of the health benefits offered and in some cases the cost of treatment.

The frameworks introduced by clinical organizations have not been formally adopted by payers; however, they are used for establishing treatment guidelines, guide shared decision making by clinicians and patients, influence policy decisions, and highlight disparity in the current drug price and economically justifiable price. Therefore, these assessments may influence, to some extent, treatment decisions by prescribers and patients. Furthermore, there are several studies in peer-reviewed journals reporting value assessments using one or multiple frameworks for competing treatment options.

Since each framework uses different criteria to assess the value of treatments, comparing results from the various frameworks is beneficial in quantifying the value of an oncology treatment which may be used alongside traditional cost-effectiveness analysis. For example, Evidera has developed an oncology-focused tool to enable assessments of drugs using the frameworks developed by NCCN, ASCO, ESMO, and MSKCC. By assessing treatments using multiple perspectives, including payers, patients, and clinicians, an economically justifiable price can be estimated and a comparison against the spectrum of existing oncology treatments in the market can be provided. This helps with objection handling and communication of the treatment's value proposition to key stakeholders, such as clinicians, using standardized frameworks adopted by clinical organizations. Manufacturers may also conduct analysis across multiple indications and gauge the treatment's value proposition across their oncology portfolio.

Conclusions and Recommendations

For the vast majority of the U.S. payer market, our clients should watch for signs that barriers are falling. Regarding structural/systemic barriers, a key hypothetical event to watch for is whether the Centers for Medicare and Medicaid Services (CMS) gets involved and begins promoting ICER. Another sign would be loss of protection for the six protected classes, which seems unlikely given the political risk associated with displeasing elderly voters. In the normative/cultural barrier category, watch for ICER to reorient its analysis to use a value metric widely accepted by U.S. payers (e.g., cost per hospitalization avoided) rather than one crafted for a Beveridge-type single-payer system. This seems highly unlikely, given that ICER has recently reiterated its commitment to cost/QALY as the measure of cost-effectiveness due to widespread acceptance outside the U.S. Regarding barriers specific to today's frameworks, watch for ICER to gain in clinician awareness and credibility.

For the small number of plans that are reported to design formularies based on cost/QALY (e.g., Premera Blue Cross) and for provider entities bearing financial risk for drug spend (e.g., many Accountable Care Organizations [ACOs] in commercial, some in Medicare), manufacturers should critique ICER's approach, adapt CEA to the U.S., and argue for use of the adaptation over the ICER model. These tactics will also be helpful in managing the PR impact of organizations like ICER.

For ICER reports that concern an established product or class, deprioritize response. Health plans are highly unlikely to upset formulary status, contracts, rebate revenues, provider and member preferences, etc., simply due to an ICER report. For ICER reports on pipeline products, if they come out early enough to affect initial formulary placement, response should be a higher priority – but still with all the same caveats mentioned earlier.

Lastly, pay attention to other value frameworks being developed, such as those by clinical organizations. These are currently focused primarily on oncology and are being used in treatment decisions and policy issues, however, they may eventually see adoption by payers and influence pricing and reimbursement decisions as well. In that case, value frameworks outside of ICER may be more impactful in the future. ■

For more information, please contact Mike.Epstein@evidera.com or Apoorva.Ambavane@evidera.com.

REFERENCES

1. Lising A, Drummond M, Barry M, Augustovski F. Payers' Use of Independent Reports in Decision Making – Will There Be an ICER Effect? *Value & Outcomes Spotlight.* March/April 2017;3(2):7-10.
2. Palmer E. ICER to Work with VA on Its Drug Choices and Payments. *FiercePharma.* Jun 28, 2017. Available at: http://www.fiercepharma.com/pharma/icer-to-work-va-its-drug-price-choices-and-payments. Accessed September 27, 2017.
3. Lopez MH. Opinion: Obscure Group Helps Block Patient Access to Crucial Medications. *Fox News.* Available at: http://www.foxnews.com/opinion/2016/06/23/opinion-obscure-group-helps-block-patient-access-to-crucial-medications.html. Accessed September 27, 2017.
4. Stier J. Health Insurers Find Back Door to Limit Choice: Column. *USA Today.* May 26, 2016. Available at: https://www.usatoday.com/story/opinion/2016/05/26/health-insurance-coverage-prescriptions-drugs-icer-comparative-effectiveness-column/83868108/. Accessed September 27, 2017.
5. NCCN Guidelines & Clinical Resources. NCCN Categories of Evidence and Consensus. Available at: https://www.nccn.org/professionals/physician_gls/categories_of_consensus.asp. Accessed September 29, 2017.
6. NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines) with NCCN Evidence Blocks. Available at: https://www.nccn.org/professionals/physician_gls/f_guidelines.asp. Accessed September 28, 2017.
7. Schnipper LE, Davidson NE, Wollins DS, et al. American Society of Clinical Oncology Statement: A Conceptual Framework to Assess the Value of Cancer Treatment Options. *J Clin Oncol.* 2015 Aug 10;33(23):2563-77. doi: 10.1200/JCO.2015.61.6706. Epub 2015 Jun 22.
8. Schnipper LE, Davidson NE, Wollins DS, et al. Updating the American Society of Clinical Oncology Value Framework: Revisions and Reflections in Response to Comments Received. *J Clin Oncol.* 2016 Aug 20;34(24):2925-34. doi: 10.1200/JCO.2016.68.2518. Epub 2016 May 31.
9. Cherny NI, Sullivan R, Dafni U., Kerst JM, Sobrero A, Zielinski C, de Vries EG, Piccart MJ. A Standardised, Generic, Validated Approach to Stratify the Magnitude of Clinical Benefit that Can be Anticipated from Anti-Cancer Therapies: The European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS). *Ann Oncol.* 2015 Aug;26(8):1547-1573. doi: 10.1093/annonc/mdv249. Epub 2015 May 30.
10. DrugPricingLab. Available at: https://drugpricinglab.org/tools/drug-abacus/methods/. Accessed October 3, 2017.