

NPC’s MISSION is to explore, demonstrate and communicate—through research, partnerships and education—the role and value of innovative biopharmaceuticals to achieve better patient health.

We conduct research, host educational events and collaborate with partners on the issues of evidence, value, access and innovation & spending. A list of projects in each goal area is provided below.



EVIDENCE. High-quality evidence is available, accepted and used to drive decision-making.

Objective 1. Raise awareness of barriers that limit access to, and portability of, data to support evidence-based decision-making, and identify potential solutions.

- **Data, Data Everywhere, But Access Remains a Big Issue for Researchers: A Review of Access Policies for Publicly-Funded Patient-level Health Care Data in the United States**
 This analysis reviews the landscape of publicly-funded databases and data networks (e.g., state-level all claims databases, and federal datasets such as Medicare Parts A, B and D, and Medicaid) and requirements for access. Policy recommendations are outlined to enable more appropriate access to publicly funded data for all stakeholders.
 - 📄 Doshi, JA; Hendrick, F; Graff, JS; Stuart, B. Data, Data Everywhere, But Access Remains a Big Issue for Researchers. EGEMS. 2016 Mar;4(2):1204.
 - 📄 NPC Infographic. Why is Broader Access to Publicly Funded Data Important? Available at: <https://www.npcnow.org/publication/why-broader-access-publicly-funded-data-important>

- **Health Plan Strategies to Use Patient Data to Improve Care**
 Improved use of real-world data (RWD) can promote a more holistic view of treatment value and appropriate use than one focused purely on volume and cost. Application and utilization of RWD throughout payer organizations may help overcome ingrained beliefs about the utility of RWD for pharmacy and therapeutics review. NPC conducted a series of interviews to identify and describe a framework for how health plans can more fully use patient data to achieve a range of objective to improve care and lower costs. This analysis found that health plans actively use patient-level data to improve care and health care efficiency, but that sophistication varies between use cases and in the application.
 - 📄 Avalere Health and National Pharmaceutical Council. (2017). Health Plan Use of Patient Data: From the Routine to the Transformational. Available at: <https://www.npcnow.org/publication/health-plan-use-patient-data-routine-transformational>

Objective 2. Facilitate the adoption and communication of high-quality evidence as a critical component of decision-making.

➤ **Good Research for Comparative Effectiveness (GRACE) Principles**

The [GRACE Principles](#) identify elements of good practice for observational CER studies. These principles were subsequently developed into a validated [checklist](#) designed to guide the assessment of observational studies of comparative effectiveness in terms of their quality and usefulness for decision-making.

 Dreyer NA, Velentgas P, Westrich K, Dubois RW, Anyanwu C, Onukwughu E. The GRACE checklist for rating the quality of observational studies of comparative effectiveness: a tale of hope and caution. *J Manag Care Spec Pharm*. 2014 Mar;20(3):301-8.

 NPC Infographic. GRACE Checklist: Assessing Observational Studies. Available at: <https://www.npcnow.org/publication/grace-checklist-infographic>

➤ **The Comparative Effectiveness Research (CER) Collaborative**

The Comparative Effectiveness Research (CER) Collaborative formed to help health care stakeholders evaluate and use evidence from comparative effectiveness study designs (observational studies, simulations, or comparisons between studies) and sources (registries, administrative claims, or electronic medical records). The Collaborative, a joint partnership among the AMCP, ISPOR and NPC, developed tools, resources and training materials to aid formulary decision-makers, the researchers who are developing the information for decision-makers, the biopharmaceutical industry and other health care stakeholders.

 CER Collaborative online tool kit. Available at: www.cercollaborative.org

 Berger ML, Martin BC, Husereau D, Worley K et al. A questionnaire to assess the relevance and credibility of observational studies to inform health care decision making: an ISPOR-AMCP-NPC Good Practice Task Force report. *Value Health*. 2014 Mar;17(2):143-56.

 Jansen JP, Trikalinos T, Cappelleri JC, Daw J et al. Indirect treatment comparison/network meta-analysis study questionnaire to assess relevance and credibility to inform health care decision making: an ISPOR-AMCP-NPC Good Practice Task Force report. *Value Health*. 2014 Mar;17(2):157-73.

 Caro JJ, Eddy DM, Kan H, Kaltz C et al. Questionnaire to assess relevance and credibility of modeling studies for informing health care decision making: an ISPOR-AMCP-NPC Good Practice Task Force report. *Value Health*. 2014 Mar;17(2):174-82.

 Perfetto EM, Anyanwu C, Pickering MK, Zagher RW, Graff JS, Eichelberger B. Got CER? Educating Pharmacists for Practice in the Future: New Tools for New Challenges. *J Manag Care Spec Pharm*. 2016 Jun;22(6):609-16.

➤ **Research and Reporting Standards and Guidelines for Observational Studies**

This analysis reviewed, compared and contrasted nine sets of standards and guidelines developed by public, private and professional societies in the U.S. and Europe. These nine sets outline how to conduct observational or real-world studies that leverage information from electronic health records, administrative claims, patient registries or data networks. The authors found that, out of the 23 methodological elements identified, 14 (61 percent) were addressed by seven or more standards and guidelines, reflecting general agreement that these elements are important. However, for all but two of these 14 elements, there was disagreement on how the element should be addressed or acted upon. To create a consensus-based approach and engage stakeholders from different disciplines, the authors made four key recommendations: 1) gain alignment and consensus; 2) identify the level of consensus; 3) accomplish consensus; 4) encourage or enforce adoption of common standards.

 Morton SC, Costlow MR, Graff JS, Dubois RW. Standards and guidelines for observational studies: quality is in the eye of the beholder. *J Clin Epidemiol*. 2016 Mar;71:3-10.

 NPC Infographic. Defining High-Quality Observational Studies. Available at: <https://www.npcnow.org/issue/defining-high-quality-observational-studies>

- **Detecting and Reducing Avoidable Methodological Flaws in Published Real-World Data Analyses**
Payer use of real-world evidence (RWE) in formulary decision-making is limited due to a lack of timely results, experience, and long-held perceptions associated with the rigor and validity of RWE studies. This analysis will 1) identify the prevalence of major methodological issues in the RWE literature; 2) provide a structured approach to the review of RWE studies to identify such issues; and 3) describe the most common flaws to allow end-users to be more confident in their ability to identify RWE design and analysis flaws.

 *In progress*

- **Patient Perspectives on Real-World Evidence**
This research sought to explore what the patient community thinks about RWE. Researchers from the National Health Council (NHC) and NPC convened representatives from patient organizations to assess what patients currently know about RWE, their level of trust in RWE and interest in using it in decision-making. This study found that the patient community sees great potential in using RWE, but there is room for improvement related to: 1) ensuring optimal use of RWE; 2) improving clarity around privacy and ownership of data; and 3) increasing awareness of RWE and its use, definitions, and tools to support uptake.

 Oehrlein E, Graff JS, Harris J, Perfetto EM. Patient-Community Perspectives on Real-World Evidence: Enhancing Engagement, Understanding, and Trust. *The Patient*. Aug;12(4):375-81.

- **Enhancing Journals' Review of Real-World Evidence and Acceptance of Its Importance**
Fifteen journal editors were interviewed to assess the perceived value of RWE, identify which resources journal editors provide to their peer reviewers, and to determine what additional resources are needed. Overall, the study found that editors find RWE to be valuable. However, some journal editors also reported difficulty in finding qualified reviewers for RWE studies and noted that RWE also has its disadvantages, including less established methodological standards.

 Oehrlein EM, Graff JS, Perfetto EM, Mullins CD, Dubois RW, Anyanwu C, Onukwugha E. Peer-Reviewed Journal Editors; Views on Real-World Evidence. *Int J Technol Assess Health Care*. 2018 Jan;34(1):111-19.

- **Use of RWE in Payer Decision: Moving from Theory to Practice**
This analysis explored how RWE is currently perceived and used in managed care environments, especially to inform pharmacy and therapeutic (P&T) committee decisions, to assess which study factors (e.g., data, design, and funding source) contribute to RWE utility in decisions, and to identify barriers to consideration of RWE studies in P&T decision making. Overall, this study found that RWE accounts for 4% of P&T monograph citations and that there is a need for improved timely and relevant studies, clarity in research methods, and tools for interpreting RWE.

 Malone DC, Brown M, Hurwitz JT, Peters L, Graff JS. Real-World Evidence: Useful in the Real World of U.S. Payer Decision Making? How? When? And What Studies? *Value Health*. 2018 Mar;21(3):326-33.

 Hurwitz JT, Brown M, Graff JS, Peters L, Malone DC. Is Real-World Evidence Used in P&T Monographs and Therapeutic Class Reviews? *J Manag Care Spec Pharm*. 2017 Jun;23(6):613-20.

- **Getting Real about Real-World Evidence Methods Transparency to Improve Decision-Maker Trust, Acceptance, and Use**
High-quality RWE can inform decision-making; however, the lack of transparency in observational studies is a critical barrier to stakeholder trust, use, and acceptance of these research results. This research focused on the importance of more transparent research methods and will survey members on the benefits and risks of more transparent observational research methods. NPC developed a white paper with 2-5 experts to identify elements associated with high-quality, transparent methods and convened a stakeholder panel to

develop consensus on the elements associated with transparent, reproducible, and disclosed research methods.

 AcademyHealth and National Pharmaceutical Council. (2019). Real-World Evidence Methods Transparency. Available at: <https://www.npcnow.org/blog/six-ways-make-real-world-evidence-methods-more-transparent>

➤ **Fit for Purpose-Developing a Framework for Tradeoffs Amongst Randomized Controlled Trials and Real-World Evidence**

A conceptual framework was developed and tested to determine when different types of evidence (e.g., randomized controlled trials, pragmatic clinical trials, prospective registries, retrospective analyses of electronic medical records etc.) are “fit for purpose” for payer decision-making. Overall, this research found that payers seek answers to more impactful and relevant questions; less emphasis is placed on study design or analytic features.

 Sabharwal RK, Graff JS, Holve E, Dubois RW. Developing Evidence That Is Fit for Purpose: A Framework for Payer and Research Dialogue. Am J Manag Care. 2015 Sep 1;21(9):e545-51.

➤ **Factors Predicting Payer Coverage: Empirical Analysis of Evidence Volume, Variation, and Consistency**

This analysis used the Tufts Medical Center Specialty Drug Evidence and Coverage (SPEC) database to examine the type of evidence health plans find most meaningful and how they weigh different forms of evidence when making coverage determinations. This research found wide variation in the evidence that health plans reported reviewing for specialty drug coverage. Some plans cited larger number of studies than others, plans cited different evidence categories with varying frequencies, and there was little consistency in the evidence that plans cited for the same drug coverage policies.

 Chambers JD, Panzer AD, Pope EF, Graff JS, Neumann PJ. Little Consistency in Evidence Cited By Commercial Plans For Specialty Drug Coverage. Health Aff. 2019 Nov;38(11):1882-86.

 NPC Gif. Inconsistent Evidence Used in Coverage Decisions. Available at: <https://members.npcnow.org/sites/default/files/image1.gif>

 NPC Webinar: Driving Evidence-Based Health Plan Coverage by Recognizing Gaps, Updating Practices. Available at: <https://www.npcnow.org/newsroom/videos/driving-evidence-based-health-plan-coverage-recognizing-gaps-updating-practices>

➤ **Evidence Evolution: How Often do Health Plans Update Specialty Drug Coverage Policies**

Commercial health plan coverage policies for specialty medications vary widely, suggesting that a patient’s health plan determines their access to treatments. Building on prior NPC work and using the Tufts SPEC database, this project will focus on how frequently health plans revisit specialty drug coverage policies; change coverage positions for specialty drugs; and whether and how the evidence cited in their coverage policies changes.

 *In progress*

➤ **Private Payer Coverage Policy for Medical Technologies: Incorporating Patient-Centered Care**

This project will build upon previous NPC research to examine if and how payers are incorporating patient values in their coverage decisions. Similarly, this project will demonstrate that additional work is needed to ensure coverage and reimbursement policies reflect the values of patients.

 *In progress*

➤ **Policy Incentives for Greater Transparency and Consistency in Health Plan Evidence Evaluation (Carrots and Sticks)**

Prior NPC research has found little consistency in the studies cited by health plans, even when considering the same drug and condition. The wide variation in the quantity and types of evidence used to inform

coverage policy, signals the need for incentives and disincentives to ensure payers base health coverage decisions on a complete and consistent examination of the best available evidence. This project will focus on potential policy levers to encourage more consistent and transparent development and evaluation of evidence for coverage decisions.

 *In progress*

➤ **Principles for Conducting Routine Real-World Evidence During a Pandemic: Does Anything Change?**

Real-world evidence (RWE), when done with high-quality data and good research methods, can fill gaps in knowledge and inform routine care and coverage decisions. The COVID-19 pandemic has magnified challenges associated with RWE. There is a concern that any RWE study conducted and using 2020 data will be met with apprehension. This project will identify challenges and illustrative examples associated with the data collection, RWE study design, analysis and interpretation during the COVID-19 epidemic. Using accepted best practices (e.g., GRACE Checklist or CER Collaborative items) as a foundation, we will identify whether adaptations to good practices are needed to conduct highly valid RWE using 2020 data.

 *In Progress*

VALUE. Value measurement and assessment should encompass the balance of benefits and costs experienced by patients and society over time.

Objective 3. Broaden the key elements in the definition and measurement of value (including quality) to enhance patient, provider and payer decision-making.

➤ **Current Landscape: Value Assessment Frameworks**

This landscape assessment analyzes seven U.S value frameworks and compares the strengths and limitations associated with each. The seven frameworks included are ASCO, ACC-AHA, ICER, NCCN, DrugAbacus, IVI, and PPVF and each were analyzed through the lens of six broad categories: development process, measures of benefit, measures of cost, methodology, evidence and assessment process.

 The National Pharmaceutical Council. (2020). Current Landscape: Value Assessment Frameworks. Available at: <https://www.npcnow.org/publication/current-landscape-value-assessment-frameworks-0>

➤ **Guiding Practices for Patient-Centered Value Assessments**

Twenty-eight guiding practices were identified to address six key aspects of value assessments: the assessment process, methodology, benefits, costs, evidence, and dissemination and utilization. Seven guiding practices for budget impact assessment are outlined separately as budget impact is not a measure of value.

 The National Pharmaceutical Council. (2016). Guiding Practices for Patient-Centered Value Assessment. Available at: <https://www.npcnow.org/guidingpractices>

 NPC Infographic. How Should Value in Health Care be Assessed? Available at: <https://www.npcnow.org/issue/how-should-value-health-care-be-assessed>

 NPC Webinar: Guiding Practices for Patient-Centered Value Assessment. Available at: <https://members.npcnow.org/events/webinar-guiding-practices-for-patient-centered-value-assessment>

➤ **Audit of Value Assessment Frameworks Using NPC's Guiding Principles**

In an evaluation of the five major value assessment frameworks relative to NPC's Guiding Practices for Patient-Centered Value Assessment, researchers from the Lewin Group identify the frameworks' strengths and areas in need of improvement.

 The Lewin Group. (2016). Comparison of Value Assessment Frameworks Using the National Pharmaceutical Council's Guiding Practices for Patient-Centered Value Assessment. Available at:

➤ **Updated Audit of Value Assessment Frameworks**

This analysis builds on NPC's previous evaluation of the major value assessment frameworks to assess how frameworks have evolved in recent years. The analysis also explores the extent to which this evolution sufficiently addresses previous concerns, and whether these frameworks are now ready for widespread adoption and use by health care decision makers in the U.S.

📄 Dubois RW, Westrich K. As value assessment frameworks evolve, are they finally ready for prime time? *Value in Health*. 2019 Sept;22(9):977-80.

➤ **Comparison of Multiple Myeloma Value Assessments**

An in-depth analysis of the multiple myeloma value assessment results from the ASCO, ICER, NCCN, and DrugAbacus frameworks explored differences in the results, the underlying reasons for those differences, and the implications of the variations.

📄 Comparison of Value Framework Assessments for Multiple Myeloma. Available at: <https://www.npcnow.org/publication/comparison-value-framework-assessments-multiple-myeloma>

📄 Westrich K, Buelt L, Dubois RW. Why Value Framework Assessments Arrive at Different Conclusions: A Multiple Myeloma Case Study. *J Manag Care Spec Pharm*. 2017 Jun;23(6-a Suppl):S28-S33.

➤ **Value Assessment Frameworks: Case Studies and Policy Implications**

A series of case studies illustrate the application of the ASCO, ICER, and Memorial Sloan Kettering frameworks. A case study workshop featured interactive group discussion and hands-on exercises to guide participants through complexities and concerns for each framework.

📄 Cohen JT, Anderson JE, Neumann PJ. Three Sets of Case Studies Suggest Logic and Consistency Challenges with Value Frameworks. *Value Health*. 2017 Feb;20(2):193-99.

➤ **Are The Payer Thresholds for Value More Restrictive for Biopharmaceuticals Than Medical Procedures?**

This research demonstrates that payer evaluation and coverage of pharmaceuticals and medical procedures may differ independently of their clinical benefit. Researchers from Tufts Medical Center and NPC examined a total of 392 medication coverage decisions and 185 procedure coverage decisions. The results of their analysis showed that 26.3 percent of medication coverage decisions and 38.4 percent of procedure coverage decisions were "more restrictive": imposing more clinical restrictions or step restrictions relative to FDA label or clinical guidelines; or coverage that places a medication on an unfavorable tier. Overall, this study demonstrates that payers restrict non-medication therapies more and that therapy access depends on factors other than cost and clinical benefit, suggesting potential health care system inefficiency.

📄 Chambers J, Pope E, Bungay K, Cohen J, Ciarametaro M, Dubois RW, Neumann PJ. A Comparison of Coverage Restrictions for Biopharmaceuticals and Medical Procedures. *Value Health*. 2018 Apr;21(4):400-06.

➤ **Value of Specialty Medications**

This research found that novel specialty therapies hold promise for arresting disease progress and improving quality of life for the three conditions (rheumatoid arthritis, multiple sclerosis, and breast cancer) with the highest specialty pharmaceutical spend.

📄 Zalesak M, Greenbaum JS, Cohen JT, Kokkotos F, Lustig A, Neumann PJ, Pritchard D, Stewart J, Dubois RW. The value of specialty pharmaceuticals - a systematic review. *Am J Manag Care*. 2014 Jun;20(6):461-72.

➤ **Solutions for Filling Gaps in Accountable Care Measure Sets**

This research highlighted where there are gaps in accountable care measure sets and that they cannot be completely addressed with more of the same measure types and measurement strategies currently in use. Measurement enhancement suggestions were made that could include the increased use of outcome, cross-cutting, and patient-reported measures in measure sets, and new approaches to measurement including layered and modular models.

 Valuck T, Dugan D, Dubois RW, Westrich K, Penso J, McClellan M. Solutions for filling gaps in accountable care measure sets. *Am J Manag Care*. 2015 Oct;21(10):723-8.

➤ **Improved Oncology Measures for Accountable Care Systems**

This project identified key gaps in oncology measures and accountable care measure sets. Stakeholders prioritized and identified measure gaps, suggested potential solutions, and began to lay the groundwork for driving future change.

 Valuck T, Blaisdell D, Dugan DP, Westrich K, Dubois RW, Miller RS, McClellan M. Improving Oncology Quality Measurement in Accountable Care: Filling Gaps with Cross-Cutting Measures. *J Manag Care Spec Pharm*. 2017 Feb;23(2):174-81.

 Valuck T, Blaisdell D, Dugan DP, Westrich K, Dubois RW, Miller RS, McClellan M. Improving Oncology Quality Measurement in Accountable Care. Available at: <https://www.npcnow.org/system/files/research/download/npc-improving-oncology-quality-measures-final.pdf>

 NPC Webinar: Improving Oncology Measurement in Accountable Care. Available at: <https://www.npcnow.org/event/webinar-improving-oncology-measurement-accountable-care>

➤ **Oncology Patient-Reported Outcome Measures (PROMs) Landscape & Strategies for Enhancement**

As payments shift to value-based arrangements, concerns about controlling costs of specialty oncology drugs must be countered with effective and meaningful patient-reported measures (PRMs) and patient-reported performance measures (PR-PMs) to ensure that patients' goals are considered. This project built on and leveraged previous research conducted with Discern on *Improving Oncology Quality Measurement in Accountable Care* by exploring the landscape of available PRMs and PR-PMs in depth and identifying recommendations to close existing gaps in these measures.

 Valuck T, Schmidt T, Perkins B, Patel P, Westrich K, Basch E, McClellan M. (2019). Improving Patient-Reported Measures in Oncology. Available at: <https://www.npcnow.org/publication/improving-patient-reported-measures-oncology>

 NPC Webinar: Amplifying the Patient Voice: Policy Solutions for Improving Patient-Reported Measures in Oncology Value-Based Payment Programs. Available at: <https://www.npcnow.org/event/webinar-amplifying-patient-voice-policy-solutions-improving-patient-reported-measures>

➤ **Estimating the Life-Cycle Value of Biopharmaceuticals**

Using Medicare Expenditure Panel Survey data (1996-2013), this study estimated the long-run average cost (LAC) for an average pharmaceutical, accounting for the effects of generic competition and medical cost offsets. Using existing literature, researchers estimated the cost-savings inputs for the conceptual framework and estimated the cumulative value over the product life-cycle.

 Lakdawalla D, MacEwan JP, Dubois R, Westrich K, Berdud M, Towse A. What do pharmaceuticals really cost in the long run? *Am J Manag Care*. 2017 Aug;23(8):488-93.

- **Assessing Drug Revenue Trends in the US**
ICER's budget impact threshold erroneously assumes limited variability in five-year revenue across interventions. To illustrate the limitations of this approach, revenue trends were analyzed for products launched in the last 10-12 years in the US.

 Ciarametaro M, Abedi S, Sohn A, Ge CF, Odedara N, Dubois R. Concerns Around Budget Impact Thresholds: Not All Drugs Are the Same. *Value Health*. 2017 Feb;20(2):230-33.

- **Underestimating the Value of an Intervention Can Limit Access – Why Including Productivity in Value Assessments is Critical**

To support patient access, value assessment reports should include patient-centered components of value. NPC reviewed all pharmaceutical value assessment reports published by ICER between March 2017 and July 2019 to determine whether productivity was included, how it was reported, and assessed if inclusion of productivity changed the value category. The exclusion of productivity costs in cost-effectiveness analysis can alter, often underestimating, the assessment of value and hence impact coverage decisions based on these assessments.

 Karmarkar T, Graff JS, Westrich K. Underestimating the Value of an Intervention: The Case for Including Productivity in Value Assessments and Formulary Design. *J Manag Care Spec Pharm*. 2020 May;26(5):652-661. doi: 10.18553/jmcp.2020.26.5.652.

- **Roundtable on Patient-Centered Multi-Criteria Decision Analysis**

The patient community has consistently called upon the health economic and value assessment communities to improve the patient centricity of value assessment. Multi-criteria decision analysis (MCDA) is a decision-making approach that can accommodate multiple factors that stakeholders value differently. In partnership with National Health Council, NPC will convene a multi-stakeholder roundtable where participants will discuss how to ensure the patient voice is reflected in MCDA. Then, participants will use MCDA to evaluate a case study demonstrating how to apply MCDA and how widely the results can vary based on differing preferences and values.

 *In progress*

- **Evaluating Accuracy in Health Technology Assessments**

As ICER's influence has continued to grow, the health economist community has raised concerns about the false impression of both precision and accuracy that is created by ICER's presentation of results as a single point estimate. There is inherent subjectivity to health technology assessments; even the most seasoned experts will make different choices at various points in the assessment process. This project will compare the determinations for key drivers made by ICER in its reviews with determinations made by independent experts for the same driver. This comparison will explore the degree of subjectivity behind those determinations and hence call into question the precision of estimates.

 *In progress*

- **Employer and Payer Perspectives of Productivity and Other Indirect Benefits – Reassessing the Landscape in a Post-Pandemic Policy Environment**

Value assessment frameworks are increasingly being used to inform health care decision-making but most still rely on methods that narrowly define value and fail to account for a broader set of benefits that are of high importance to patients, including productivity. Previously, NPC conducted a survey of employers and payers to better understand their perceptions of including indirect benefits in value assessments. As COVID-19 has given new context and relevance to the value of productivity and caregiver burden, this project aims to explore how employer and payer perspectives have changed compared to the original survey.

 *In progress*

➤ **Allocation of Value Between a Manufacturer and Society**

Conversations are currently happening regarding biopharmaceutical innovation and about what portion of consumer surplus generated should go to the manufacturer and what should go to society. These conversations are missing important perspectives and could lead to narrow, one-side policy solutions that have longer term impact on incentives for innovation and value creation. This project will elevate the conversation by constructing a conceptual framework to help guide the policy debate in a more rigorous and evidence-based manner. This framework will not be a formula or an endorsed set of principles, rather a broad set of considerations that should be included when discussing value assessments.

 *In progress*

Objective 4. Identify and encourage adoption of solutions that enable the full value generated by biopharmaceuticals to be realized

➤ **Do Payers Reap Therapeutic Benefits Paid by Others?**

Using discrete event modeling, this research examined the disconnect between the short-term budget impact of a treatment and its downstream effects on payers and society. According to this study, increasing availability of expensive, yet transformative therapies—such as gene therapies or other personalized medicine approaches—are likely to widen the gap between the initial payer and the payer, which ultimately reaps the downstream benefits. This disconnect will require creative approaches to health care financing models to assure appropriate access to cost-effective therapies, incentivize future innovation, and provide sustainable economics for payers.

 Cutler D, Ciarametaro M, Long G, Kirson N, Dubois R. Insurance switching and mismatch between the costs and benefits of new technologies. *Am J Manag Care*. 2017 Dec;23(12):750-57.

 NPC Infographic. Paying for Patient Care. Available at: <https://www.npcnow.org/publication/paying-patient-care>

 NPC Webinar: Health Care Coverage Challenges: New Treatments with High Upfront Costs, Long-term Benefits, & Delayed Savings. Available at: <https://members.npcnow.org/events/webinar-health-care-coverage-challenges-new-treatments-with-high-upfront-costs-long-term>

➤ **Regulatory Barriers to the Alignment of Biopharmaceutical Price and Value**

This study highlights the challenges biopharmaceutical manufacturers and payers face when developing value-based contracts. Using both a qualitative and quantitative methodological approach, the paper identified four main regulatory and legal barriers that are standing in the way of advancing value-based contracts in health care. To gain a better understanding of the of these regulatory and legislative barriers, we modeled the impact of removing them and subsequently identified potential solutions for addressing them.

 The National Pharmaceutical Council. (2018). Regulatory Barriers Impair Alignment of Biopharmaceutical Price and Value. Available at: <https://www.npcnow.org/publication/regulatory-barriers-impair-alignment-biopharmaceutical-price-and-value>

 NPC Webinar: Barriers to Paying for Value. (2018). Available at: <https://members.npcnow.org/events/webinar-barriers-to-paying-for-value>

➤ **Reducing Low-Value Care**

A literature review of resource optimization work done was conducted to identify existing approaches to addressing low value care. An expert panel was convened to discuss strategies used in each segment of the health system to validate existing work, recommend approaches to filling gaps, and identify the ease to which utilization with low-value care items can be reduced.

 Beaudin-Seiler B, Ciarametaro M, Dubois RW, Lee J, Fendrick M. Reducing Low-Value Care. (2016). Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20160920.056666/full/>

 NPC Infographic. How Can Administrators and Clinicians Reduce Low-Value Care? Available at: <https://www.npcnow.org/publication/how-can-administrators-and-clinicians-reduce-low-value-care>

➤ **Barriers and Solutions to the Inclusion of Indirect Benefits in Biopharmaceutical Value Reviews**

There is a lack of agreement on the inclusion of indirect benefits (e.g., productivity improvements and reduced caregiver burden) in value assessment. To understand why, researchers conducted in depth interviews to survey payers, employers and patient advocacy groups on views on the barriers to inclusion of indirect benefits and their receptivity to solutions (e.g., more convincing productivity data, inclusion in standards for cost-effectiveness analysis, etc.).

 Wamble D, Ciarametaro M, Dubois R. Barriers and Solutions to the Inclusion of Broader Benefits in Biopharmaceutical Value Assessments. (2020). Available at: https://journals.lww.com/joem/Abstract/2020/02000/Barriers_and_Solutions_to_the_Inclusion_of_Broader.7.aspx

 Ciarametaro M, Buelt L, Dubois R. Getting Value Right: The Case For Indirect Benefits. (2020). Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20200310.267867/full/>

➤ **Stakeholder Preferences for Value**

Many value frameworks use a single measure to determine value, while others allow the user to prioritize the components of value to match their needs, but does a single measure adequately capture value? This research will survey payers, employers, providers, and patient advocacy groups to identify the variation between and within stakeholder group relevant to value assessment.

 Hollin, I. L., González, J. M., Buelt, L., Ciarametaro, M., Dubois, R. W. (2020). Do Patient Preferences Align With Value Frameworks? A Discrete-Choice Experiment of Patients With Breast Cancer. Available at: <https://journals.sagepub.com/doi/10.1177/2381468320928012>

➤ **Consideration of Patient Preferences in Payer Decisions: On Board or Off the Rails?**

Patient and consumer preferences for what health care is covered, who delivers the care, and how this care is paid for are not uniform. However, the lack of consideration of these differences provides an incomplete picture of health care value. This project will discuss the existing and novel processes to consider patient preferences, opportunities to incorporate patient input into benefit design.

 *In progress*

➤ **Why Have Efforts to Reduce Low-Value Care Been Generally Unsuccessful?**

For over 30 years, it has been known that approximately a third of health care spending is wasted on low-value care. Why haven't prior efforts to curb spending on low-value care succeeded? This project will examine the historical and contemporary efforts to reduce low-value care and explore whether future efforts may be more successful, or whether the structure and incentives of the U.S. health care system make that very unlikely.

 *In progress*

- **Exploring the Evidence on the Potential Impact of Reducing Areas of Low-Value Care**
Previous efforts aimed at addressing low-value care have demonstrated the importance of targeting recommendations that improve value. However, to date, there has been limited consideration of or inquiry into which services or areas of care would best be targeted for greatest impact. This research aims to address these gaps by: identifying key low-value care services, therapeutic areas, and/or areas of care delivery; estimating their possible cost impacts; exploring any unique challenges or considerations to effectively targeting potentially high-impact areas in low-value initiatives; and assessing their overall potential for meaningful cost reduction.

 *In progress*

- **Addressing Medicaid Best Price for Curative Therapies**
The Medicaid “best price” rule, adopted in 1990, ensures that the Medicaid program receives discounts at least as large as other payers. An unintended consequence of this rule is that it can stymie adoption of innovative new payment models needed to support access to high-cost curative therapies. Working with MITFoCUS group, this project will conduct an objective assessment of the trade-offs associated with potential solutions to reform Medicaid Best Price.

 *In progress*

- **Facilitating Employer Efforts to Address Low-Value Care**
Working with the HealthCare 21 Business Coalition (HC21), an employer-led coalition of healthcare leaders and other stakeholders in East and Middle Tennessee, this project will quantify low value care for Knoxville employer members. The claims analysis will include three measures from the Task Force on Low Value Care: low back pain and imaging, vitamin D testing, and lab work prior to low risk surgery.

 *In progress*

ACCESS. Patients have meaningful access to appropriate treatments.

Objective 5. Promote formulary and benefit designs that support patient-centered access to appropriate therapies.

- **Supporting Consumer Access to Specialty Medications Through Value-Based Insurance Design**
This white paper highlighted the importance of recognizing the value of specialty pharmaceuticals using value-based insurance design. The white paper included information on specific techniques and considerations decision-makers should consider to ensure V-BID works effectively for specialty medications.

 Fendrick MA, Buxbaum J, Westrich K. (2014). Supporting Consumer Access to Specialty Medications Through Value-Based Insurance Design. Available at: <https://www.npcnow.org/publication/supporting-consumer-access-specialty-medications-through-value-based-insurance-design>

- **Factors Predicting Private Payer Coverage Policy for Medical Technology: An Empirical Analysis**
NPC and the Center for the Evaluation of Value and Risk in Health at Tufts Medical Center explored how 17 of the largest US commercial health plans cover specialty medicines and found that health plans are very inconsistent in how they cover specialty drugs. Only one in six drug indication pairs were covered the same way across all plans and only 60% of coverage decisions were consistent across health plans. In addition,

38% of the time health plan coverage determinations were more restrictive than the FDA-approved indication or excluded the medicine altogether.

 Chambers JD, Dim DD, Pope EF, Graff JS, Wilkinson CL, Neumann PJ. Specialty Drug Coverage Varies Across Commercial Health Plans In The US. Health Aff (Millwood). 2018 Jul;37(7):1041-47.

 Chambers JD, Panzer AD, Neumann PJ. Variation In The Use Of Step Therapy Protocols Across US Health Plans. (2018) Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20180912.391231/full/>

The Good, the Bad, and the Different: Deciphering Heterogeneity to Managed Care Pharmacy and Medical Directors

This project developed education materials, trained, and tested the impact of tools to critically evaluate comparative effectiveness among MCO pharmacy and medical directors. The research program addressed the importance of knowledge, attitudes, intentions and self-efficacy related to considering individual treatment benefit when evaluating evidence and developing medical policy.

 Malone DC; Hines LE, Graff JS. The Good, the Bad, and the Different: A Primer on Aspects of Heterogeneity of Treatment Effects. J Manag Care Spec Pharm. 2014 Jun;20(6):555-63.

 NPC infographic. They Myth of the Average (Health Professionals) Infographic. Available at: <https://www.npcnow.org/newsroom/myth-average-health-professionals>

Variable Co-pays in Pharmacy Benefit Tiers: Ethics and Efficiency

This research identified the ethical, legal, actuarial implications associated with cost-sharing based on formulary tier rather than medical appropriate for patients. A multi-stakeholder panel identified guiding principles for when it would be more (or less) acceptable to require patients with the same or similar condition to have variable out-of-pocket expenses.

 Graff JS, Shih C, Barker T, Dieguez G, Larson C, Sherman H, Dubois RW. Does a One-Size-Fits-All Cost-Sharing Approach Incentivize Appropriate Medication Use? A Roundtable on the Fairness and Ethics Associated with Variable Cost-Sharing. J Manag Care Spec Pharm. 2017 Jun;23(6):621-27.

 NPC Infographic. Does a One Size Fits All Cost-Sharing Approach Incentivize Appropriate Medication Use? Available at: <https://www.npcnow.org/publication/does-one-size-fits-all-cost-sharing-approach-incentivize-appropriate-medication-use-0>

 NPC Webinar: Same Condition, Different Costs: Should Patients Pay Different Amounts? Available at: <https://www.npcnow.org/event/webinar-same-condition-different-costs-should-patients-pay-different-amounts>

A “Dynamic” Approach to Consumer Cost-Sharing for Prescription Drugs

This issue brief introduced and defined the concept of “rewarding the good soldier”, which refers to the scenario or circumstance when a patient does not respond as desired to the initial step-therapy, and should, therefore, have reduced consumer cost-sharing. This brief includes clinical examples, discusses the benefits from a more clinically nuanced approach and proposes next steps to move from cost-focused to value-based initiatives in formulary development.

 University of Michigan Center for Value-Based Insurance Design. (2016). A ‘Dynamic’ Approach to Consumer Cost-Sharing for Prescription Drugs. Available at: <http://vbidcenter.org/a-dynamic-approach-to-consumer-cost-sharing-for-prescription-drugs/>

Survey to Assess Employer Opinions of Pharmacy Benefit Managers (PBMs)

In recent years, PBMs’ aggressive contracting practices have come under increasing scrutiny by businesses and the public at large. This survey of jumbo and self-insured employers provides in-depth information regarding employer perceptions of the current PBM business model, their trust level for PBMs, and their preferences for how their pharmacy benefits are managed.

-  The National Pharmaceutical Council. Toward Better Value. (2017). Available at: <https://www.npcnow.org/system/files/research/download/npc-employer-pbm-survey-final.pdf>
-  PBM Relationship Segmentation Tool. Available at: <https://www.npcnow.org/sites/default/files/inline-files/plotting-pbm-relationship-segmentation-tool-final.pdf>
-  Improving Your Prescription Drug Benefit Consulting Support Tool. Available at: <https://www.npcnow.org/sites/default/files/inline-files/plotting-pbm-consultant-segmentation-tool-final.pdf>
-  Webinar: Employer Perspectives on Prescription Drug Management: New Research on What’s Working, What Needs Fixing. Available at: <https://register.gotowebinar.com/recording/2653894900921028108>
-  Webinar: Toward Better Value: Employer Perspectives on Managing Prescription Drug Benefits. Available at: <https://www.ibiweb.org/toward-better-value-employer-perspectives-on-managing-prescription-drug-benefits/>
-  NPC Member Webinar. Toward Better Value: Employer Perspectives on What’s Wrong With The Management of Prescription Drug Benefits and How to Fix it. Available at: <https://members.npcnow.org/events/webinar-toward-better-value-employer-perspectives-on-what%E2%80%99s-wrong-with-the-management-of>

 **Expanding Pre-Deductible Drug Coverage in HSAs**

Current IRS regulations provide for a “safe harbor” allowing coverage of certain preventive services outside of the plan deductible, however, these regulations do not apply to treatments for chronic conditions. This study examined how providing pre-deductible coverage for 57 drug classes covering 11 chronic conditions would impact out-of-pocket costs, plan expenditures and premiums. Although it would increase utilization and shift some costs to health care plans, we found that the overall impact would be modest, requiring a premium increase of less than 2%.

-  VBI Health. Financial Impact of HSA-HDHP Reform to Improve Access to Chronic Disease Management Medications. (2018). Available at: <http://vbidhealth.com/docs/HSA-HDHP-Reform-Brief.pdf>
-  NPC Webinar: Current IRS Policy on High-Deductible Health Plans: Impacting Americans with Chronic Conditions. Available at: <https://www.npcnow.org/event/webinar-current-irs-policy-high-deductible-health-plans-impacting-americans-chronic>

 **Estimating the Uptake of a Novel HSA-HDHP Plan**

As a follow-on to a previous analysis that examined pre-deductible access to medications, this research examined the net federal budgetary impact of allowing Health Savings Account-eligible (HSA-eligible) HDHPs to expand pre-deductible coverage to include chronic disease services (HDHP+ plans). Researchers found that allowing pre-deductible coverage for services or drugs intended to treat an existing illness, injury or condition could be cost-neutral, or potentially even offer cost savings, over current high-deductible health plans that do not have such flexibility.

-  VBI Health. Uptake and Federal Budgetary Impact of Allowing Health Savings Account-eligible High Deductible Health Plans to Cover Chronic Disease Drugs and Services Pre-deductible. (2019). Available at: <https://www.npcnow.org/sites/default/files/inline-files/2019-06-01%20hdhp-plus-vbid-study-final.pdf>

 **Adherence to Generally Agreed Upon Principles for Step Therapy Policies**

Rapid growth in utilization management restrictions, coverage restrictions and step edits are impacting the delivery of both low-value and high-value care services. Recently groups led by the AMA and others have identified principles regarding how and when formulary restrictions should be developed, implemented, and communicated to providers and patients. Using the existing principles as a foundation, this project will: develop an actionable checklist to evaluate step therapy and prior authorization requirements; test the

checklist with a multi-stakeholder workgroup; compare the checklist and agreed-upon principles with a database of formulary coverage policies; and identify areas of improvement in utilization management programs.

 *In progress*

➤ **Examining the Relationship Between Patient Financial Burden and Outcomes: A Systematic Review of the Literature**

In response to rising health care costs, payers are increasingly adopting benefit designs that shift greater financial burden to patients, including high-deductible health plans and plans with higher cost-sharing. This is despite evidence that shows high cost-sharing has a detrimental effect on patient adherence and persistence. This review will capture a complete picture of the relationship between cost-sharing and patient utilization and clinical and economics outcomes and where possible identify links by clinical conditions and insurance type.

 *In progress*

➤ **Understanding Employer Perspectives on Cost-sharing and Exploring Barriers to Change**

A growing body of literature suggests that benefit designs with higher cost-sharing and/or increased patient out-of-pocket costs may lead to reduced medication adherence, worse disease control and increases in hospitalizations. This project will leverage the literature review from the patient burden project to gain a better understanding of the employer perspective through a series of interviews or focus groups with employers and employer benefit consultants.

 *In progress*

➤ **The Impact of Wage Status and Copay Accumulator Adjustment Programs on Specialty Drug Utilization and Health Care Costs**

In an effort to control rising health care costs, payers and PBMs are adopting copay accumulator adjustment programs (CAAPs), which exclude drug costs covered by copay assistance programs from being counted toward the deductible. This can be particularly burdensome for low wage earners. This project will examine the relationship between wage status and the use of specialty pharmaceuticals and other health care services, as well as the extent to which a CAAP impacts these relationships.

 *In progress*

➤ **Impact of Patient Out-of-Pocket Costs for Oral Anticancer Medications on Medication Adherence**

This retrospective observational cohort study will evaluate the extent to which OOP costs for oral anticancer medications increase the risk of medication nonadherence and increased healthcare utilization in cancer patients. By identifying the predominant risk factors and most vulnerable subgroups, the research will assist health system stakeholders endeavoring to develop and/or modify strategies aimed to improve access to and management of oral anticancer medications.

 *In progress*

➤ **Impact of a Real Time Prescription Benefit Tool on Medication Access**

There is a direct relationship between the cost of a prescription and the likelihood it will be left at the pharmacy by the patient. A real time prescription benefit (RTPB) tool may eliminate surprises at the pharmacy by displaying benefits-based, patient-specific out-of-pocket cost information at the point of prescribing before the patient leaves the encounter with the prescriber. The goal of this study is to determine the impact of a RTPB tool on access to new prescriptions. It is hypothesized that patients whose prescribers used the RTPB tool will have improved initial access and durable filling of their medications.

 *In progress*

➤ **Downstream Consequences of Avoided Care**

The COVID-19 pandemic has led to disruptions in health care utilization but the short- and long-term implications of reduced or delayed care are unknown. The pandemic provides a natural experiment to explore the implications of avoided care and restricted access to care. This study will evaluate the impact of various restrictions to care and could reinforce the need for improved access to appropriate medications and care.

 *In progress*

➤ **The Impact of Chronic Disease Medication Adherence on COVID-19 Patient Outcomes**

COVID-19 patients with chronic conditions have been shown to have increased rates of severity and mortality. It is not known however whether the increased risk of morbidity or mortality relates to the presence of the underlying disease itself or whether that underlying disease is under control or not. The current crisis provides an opportunity to estimate the impact of avoided care as measured by the effect of medication adherence on patient outcomes. This study hypothesizes that medication adherence among chronically ill patients results in less severe outcomes when infected with COVID-19 and, if true, would provide further evidence supporting the need for flexible benefit designs for at-risk patient populations.

 *In progress*

Objective 6. Encourage optimized access to appropriate therapies under innovative payment and delivery models.

➤ **Role of Pharmaceuticals in Accountable Care Organizations – A Framework for Success**

NPC's collaborative effort with the American Medical Group Association and Premier, Inc., and experts from leading ACOs led to the development of a conceptual framework for understanding the role of pharmaceuticals in helping ACOs successfully achieve cost and quality benchmarks. The research provided examples of how the challenge of managing medication is being navigated in the real world and provided some implementation considerations.

 Dubois RW, Feldman M, Martin J, Sanderson-Austin J, Westrich KD. Role of pharmaceuticals in value-based healthcare: a framework for success. *Am J Manag Care*. 2012 Jul 1;18(7).

 NPC Infographic. Accountable Care Organizations. Available at: <https://www.npcnow.org/publication/accountable-care-organizations-acos>

➤ **ACO Best Practices - Improving Patient Outcomes and Costs in an ACO Through Comprehensive Medication Therapy Management**

As the first case study published to address the gaps identified in NPC's ACO Medication Readiness Assessment, this article presents an overview of Fairview Health's MTM program. The program has optimally managed care for diabetes patients while also showing a 12:1 return on investment when compared to overall health costs of patients who did not receive MTM services.

 Brummel A, Lustig A, Westrich K, Evans MA, Plank GS, Penso J, Dubois RW. Best practices: improving patient outcomes and costs in an ACO through comprehensive medication therapy management. *J Manag Care Spec Pharm*. 2014 Dec;20(12):1152-8.

➤ **Optimization of Medication Use at Accountable Care Organizations**

This research sought to implement the framework previously developed by NPC and its collaborators. The readiness assessment found that critical gaps remain before ACOs can effectively support, monitor and ensure appropriate medication use in their patient populations. The gap results of this survey served as the catalyst for NPC and its partners to develop case studies of how successful ACOs address these gaps.

📄 Wilks C, Krisle E, Westrich K, Lunner K, Muhlestein D, Dubois R. Optimization of Medication Use at Accountable Care Organizations. *J Manag Care Spec Pharm*. 2017 Oct;23(10):1054-64.

➤ **Impact of ACOs on Treatment Patterns**

This study compared utilization patterns of high cost services and medications for patients receiving care from ACO participating physicians and those receiving care from non-ACO physicians during the initial phases of ACO development in a commercially insured environment. This study did not observe statistically significant and meaningful differences in healthcare resource utilization of high costs services and medications between members within the aggregated ACO and the non-ACO environments in the very early years of ACO implementation.

📄 Zhou S, Ciarametaro M, Wu B, Singer J, Dubois RW. Utilization of High-Cost Interventions for Targeted Clinical Conditions During the Early Stages of ACO Development in a Commercially Insured Population. *Popul Health Manag*. 2019 Oct;22(5):377-84.

➤ **Private Sector Risk-Sharing Agreements in the U.S.: Trends, Barriers, and Prospects**

This article presents a landscape assessment of risk sharing agreements (RSAs) and identifies key issues impacting how successful RSAs are in ensuring patient access to appropriate medications within the rapidly evolving health care financing system.

📄 Garrison LP Jr, Carlson JJ, Bajaj PS, Towse A, Neumann PJ, Sullivan SD, Westrich K, Dubois RW. Private sector risk-sharing agreements in the United States: trends, barriers, and prospects. *Am J Manag Care*. 2015 Sep;21(9):632-40.

➤ **Key Considerations in the Design of Payment Bundles**

This paper identifies key factors that should be considered in the design of payment bundles and provides case examples that illustrate how each factor should be implemented.

📄 Ciarametaro M, Dubois RW. Designing Successful Bundled Payment Initiatives. (2016). Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20160420.054542/full/>

➤ **Value-based Agreements May Be More Prevalent Than Previously Known**

This project surveyed payers and biopharmaceutical manufacturers to gain a better understanding of the prevalence of US value-based arrangements, their characteristics, and the factors that facilitate their success or act as barriers to their implementation. This study found that approximately 3 in 4 value-based agreements are not publicly known and that previous estimates of VBAs, using only publicly available data, likely underestimate payer and manufacturer commitment to value-based contracting. This analysis also identified several barriers to implementing VBAs as well as factors that contribute to successful contract negotiation and implementation.

📄 Mahendraratnam N, Sorenson C, Richardson E, Daniel GW, Buelt L, Westrich K, Qian J, Campbell H, McClellan M, Dubois RW. Value-based arrangements may be more prevalent than assumed. *Am J Manag Care*. 2019 Feb;25(2):70-6.

➤ **Impact of Restrictive Formularies on Drug Utilization and Outcomes**

This study explored formulary restrictions on noninsulin antihyperglycemic drugs (NIADs) in Medicare Part D plans to estimate the impact of formulary restrictions on access to NIADs among low-income beneficiaries with type 2 diabetes undergoing treatment intensification. Although greater restrictions were associated with small reductions in initiation of DPP-4 inhibitors, these reductions were not statistically significant and overall, Part D formulary restrictions on sole-source brand name NIADs had little impact on patterns of treatment intensification.

📄 Stuart BC, Slejko JF, Rueda JD, Cooke CE, Shen X, Roberto P, Ciarametaro M, Dubois R. Impact of formulary restrictions on medication intensification in diabetes treatment. *Am J Manag Care*. 2018 May;24(5):239-46.

➤ **Improving Patient Access to Medications Through Quality Measures**

Significant gaps exist in current quality metrics, particularly when it comes to measuring meaningful access to necessary medications. This analysis reviewed the literature on barriers to medication access, existing access to care performance measures, and interventions to improve medication access and developed a conceptual framework for informing quality measures for medication access. A multi-stakeholder roundtable then discussed the conceptual framework and patient access barriers and mapped the barriers to seven encounter points in the health care system. The roundtable also identified recommendations to promote advances in quality measurement to ultimately improve patient access to medication.

📄 Pharmacy Quality Alliance and the National Pharmaceutical Council. (2019). Access to Care: Development of a Medication Access Framework for Quality Measurement. Available at: <https://pga.memberclicks.net/assets/Research/PQA-Access-to-Care-Report.pdf>

➤ **Care Pathways in U.S. Health Care Settings: Current Successes and Limitations, And Future Challenges**

This research examined the landscape of care pathway development, implementation and evaluation; identified strengths and weaknesses in these areas; and offers recommendations for strengthening these processes to ultimately enhance the future impact of care pathways on patient outcomes and quality of care in U.S. health care settings.

📄 Chawla A, Westrich K, Matter S, Kaltenboeck A, Dubois R. Care pathways in U.S. healthcare settings: current successes and limitations, and future challenges. *Am J Manag Care*. 2016 Jan;22(1):53-62.

➤ **Updated Analysis of Care Pathways in the U.S.**

This research updates and expands upon NPC's initial care pathways study to further identify key issues and areas of concern, as well as provide recommendations for improvement to ensure that care pathways are focused on achieving optimal patient care. The study found notable increases in process transparency, codification of standards, and prioritization of high-quality evidence, but additional improvements in transparency, including patient transparency, remain necessary.

📄 Chawla A, Westrich K, Dai A, Mantels S, Dubois RW. US Care Pathways: Continued Focus on Oncology and Outstanding Challenges. *Am J Manag Care*. 2019;25(6):280-87.

➤ **Value-Based Contracting with Health Systems**

Although value-based contracts between pharmaceutical manufacturers and health plans or PBMs are gaining traction, few successful examples involving health systems or integrated delivery networks are known. This project will explore the landscape of value-based partnerships with health systems, identify barriers inhibiting expansion, and define success factors and best practices for value-based partnership implementation.

📄 *In progress*

Objective 7. Catalyze an evidence-based dialogue on total health care spending that addresses the benefits and costs of health care investment.

➤ **International Comparison of Health Care Spending**

When discussing health care spending in the US, comparisons of the U.S. and other OECD countries are often made (i.e., the U.S. spends more than any other country on health care but has similar or worse outcomes.) This project found that a more detailed look at the underlying OECD data underscores the benefits and challenges of the dataset as well as the challenges in making cross-country comparisons. Therefore, caution is warranted when making policy proposals based on this dataset due to significant data and study limitations. In the article, the authors outline four issues associated with the lack of existing data and the significant variation in the U.S. for both health care utilization service and price information.

📄 Greenwald L, Graff J, Wamble D, Dubois RW. International Health Care Spending Data: What They Can Tell Us, And What They Can't. (2018). Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20180430.6731/full/>

➤ **Assessing Consumer Tradeoffs: Case Study of an Employee-Designed Health Plan**

This analysis summarizes an effort to redesign a benefit option offered by a self-insured employer. In a series of facilitated workshops, employees used a gameboard to first individually design their ideal health care benefit, and then come together as a group to develop a health plan that would best serve the entire organization. The decisions that were made and the dialogue around them elucidated the inherent tradeoffs and willingness to pay for various health care services. As policymakers consider new policies for changing the trajectory of current health care spending, it will be increasingly important to engage in a fair dialogue about health care spending tradeoffs. This case study provides a framework for other health care purchasers to engage consumers in constructive dialogue on health care spending.

📄 McNichol J, Nichols LM, Aiyar S, Buelt L, Ciarametaro M, Dubois RW. Prioritizing Health Care Spending: Engaging Employees in Health Care Benefit Design. (2018). Available at: <https://www.npcnow.org/system/files/research/download/prioritizing-health-care-spending-engaging-employees-npc-study-final.pdf>

📄 McNichol J, Nichols L, Buelt L, Ciarametaro M, Dubois RW. Activating Employees in Discussion of Health Care Trade-Offs: It Can Be Done. (2019). Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20190102.356142/full/>

➤ **Ethical Framework for Discussing Health Care Spending**

How much to spend on health care and how to allocate those dollars among the various health care services are challenging questions to answer and will impact society and patients in clear and not-so-clear ways. A productive dialogue requires an understanding of the considerations and tradeoffs. This project will provide an ethical as well as an economic framework to aid in the ensuing dialogue.

📄 *In progress*

➤ **Guiding Practices for Health Spending**

This research will involve a thorough literature review, which will be used to craft a draft list of guiding practices. The draft guiding practices will then be reviewed by *Going Below The Surface* forum partners. Results will be summarized in a manuscript.

📄 *In progress*

- **Assessing Willingness to Pay for Health Care Services Among Patients, Consumers, and Employers**
This project explores consumer understanding and willingness to pay for new medical technologies. Using an internet-based survey, this research will look at four populations of consumers to gather a broader perspective on the services of interest as well as consumer approaches to tradeoffs.
 *In progress*

- **Lessons Learned From Ex-U.S. Health Care Allocation Efforts**
The United Kingdom, Germany, Canada, Australia and elsewhere have used various approaches to limit health care spending and allocate health care resources. What are the lessons learned? Which approaches might be suitable for the US? This project will review and summarize what has been done elsewhere and how lessons learned from those experiences apply to the US.
 *In progress*

- **Discussion Guide for Regional Health Care Spending Dialogues**
This project will build upon prior NPC research to develop a comprehensive cross-stakeholder discussion guide for use in health care spending initiative regional dialogues.
 *In progress*

- **Impact of Shared Decision Making on Utilization and Cost**
Shared decision making (SDM) is a process by which clinicians work with patients to inform them of their care choices, help them evaluate evidence of the benefits and risks of each choice, understand their goals and preferences, and jointly make informed care decisions. This research will systematically review existing evidence to understand the impact of SDM on utilization and cost, recognizing that SDM can take many forms across various care settings, to help ensure the right policies and incentives are used to encourage wider use of SDM as a fundamental component of quality.
 *In progress*

- **Is It Really Just the Prices?**
The U.S. spends more on health care than any European country yet does not achieve better health outcomes. The overwhelming consensus is that inefficiencies in the U.S. system are driven by higher prices. While higher prices do contribute to higher spending, the role of other factors, such as poor health behaviors and chronic disease burden, have not been fully explored. Utilizing survey data from the U.S. and Europe, NPC will estimate the differences in per capita spending attributed to demographic difference and chronic disease. Additionally, this research study will provide a measure of how much the U.S. - European spending differences are associated with these factors.
 *In progress*

- **Which Investments in Social Determinants of Health are Good Investments?**
There has been increasing interest in improving and investing in the social determinants of health. With the increased attention on overall health spending, each dollar spent should be put under increased scrutiny to ensure it might not be better used in other ways. This project will analyze various social interventions to determine impacts and other attributes, such as, their costs, impacts on health, impacts on health care spending and the return on investment and associated time horizon.
 *In progress*

➤ **Impact of Price Regulation on Innovation**

The United States leads the world in drug development and ensuring that patients have access to new and curative therapies, largely due to the free market system that doesn't impose limitations on innovations. There is also a public belief that lowering drug prices will not harm innovation despite evidence suggesting to the contrary and proposed policy solutions may result in an environment where restrictive drug pricing becomes increasingly likely. This project will examine the impact that pricing policies would have on biopharmaceutical innovation with the goal of developing evidence that can be understood by a broader audience.

 *In progress*

➤ **Quantifying the Benefits of COVID-19 Innovation**

The COVID-19 pandemic has created an immediate need for effective treatments, but the public remains concerned about the costs of biopharmaceuticals. The current crisis presents a unique opportunity to highlight the value of biopharmaceuticals, including both the economic and societal benefits that treatments and vaccines could generate. Previous work done by researchers at the University of Vermont estimated the cost/QALY of locking down the economy and social distancing at between \$503k - \$6.7 million, far higher than the ranges that ICER or others tout. This study will build on this previous body of work to show how therapeutic innovation could bring significant value at a much lower cost than current interventions. Project work will consist of developing a study protocol including the approach to modeling costs and benefits as well as scenarios to be analyzed and conducting quantitative modeling incorporating COVID-19 treatments.

 *In progress*

Objective 8. Develop and promulgate methods that promote a wiser use of health care resources in support of sustained biopharmaceutical innovation for improved patient health.

➤ **Historical Impact of Biopharmaceuticals on Outcomes**

This study surveyed physicians on which medical technology innovations have had the most impact on health outcomes, particularly in the treatment of eight chronic conditions including HIV, chronic obstructive pulmonary disease (COPD) and depression. Overall, this research found that most improvements in health outcomes were driven by pharmaceutical and biopharmaceutical products.

 Wamble DE, Ciarametaro M, Dubois R. The Effect of Medical Technology Innovations on Patient Outcomes, 1990-2015: Results of a Physician Survey. *J Manag Care Spec Pharm*. 2019 Jan;25(1):66-71.

➤ **Do Improvements in Patient Outcomes Explain Rising Costs of Pharmaceutical Treatments?**

This project builds on NPC's "Historical Impact of Biopharmaceuticals on Outcomes" and examined whether increased medical intervention spending on prevalent chronic conditions has been a good investment. This study found that health care spending for six out of seven conditions over a 20-year time horizon was both cost-effective and a source of high value creation.

 Wamble D, Ciarametaro M, Houghton K, Ajmera M, Dubois RW. What's Been The Bang For The Buck? Cost-Effectiveness Of Health Care Spending Across Selected Conditions In The US. *Health Aff (Millwood)*. 2019 Jan;38(1):68-75.

 NPC Infographic. Are We Spending Too Much on Health Care? Available at: https://members.npcnow.org/sites/default/files/Infographics/NPC-BangForYourBuck-External_.pdf

➤ **Financing for Curative Therapies**

This study explores tensions in our health care system via online market research with payers and highlights a few proposals to address these concerns. This research found that when it comes financing innovative and life-changing therapies, the implications and risk factors faced by payers vary according to their size.



Ciarametaro M, Long G, Johnson M, Kirson N, Dubois RW. Are Payers Ready To Address The Financial Challenges Associated With Gene Therapy? (2018) Available at:
<https://www.healthaffairs.org/doi/10.1377/hblog20180626.330036/full/>

➤ **Are Drug Spending Estimates Overestimated?**

When evaluating overall health care spend, estimates of the share of prescription drug spend vary substantially depending upon the underlying methodology and inputs. This research puts these estimates into context, explains the importance of accounting for rebates and discounts, and describes why estimates of drug spending vary so significantly by source. This analysis found that by applying a standardized methodological approach, the range in leading estimates was reduced from 18.8 percentage points to 4.0.



Kleinrock M, Westrich K, Buelt L, Aitken M, Dubois RW. Reconciling the seemingly irreconcilable: how much are we spending on drugs? Value Health. 2019 April. Available at:
[https://www.valueinhealthjournal.com/article/S1098-3015\(18\)36310-1/pdf](https://www.valueinhealthjournal.com/article/S1098-3015(18)36310-1/pdf)



Webinar: What's in a Number? It Matters in Health Policy Conversations. Available at:
<https://academyhealth.org/events/2019-06/webinar-whats-number-it-matters-health-policy-conversations>

➤ **Health Care Spending Case Studies**

As a follow-on analysis to prior research on the historical impact of biopharmaceuticals, NPC will use a case-based approach to illustrate how spending by setting of care (i.e. retail pharmacy, hospital, physician office) can vary substantially across diseases and over time due to differences in standard of care. This analysis may also illustrate that the focus on aggregate budget allocation is counterproductive to maximizing value and patient outcomes.



In progress

➤ **Health Care Resource Allocation Efficiency**

Sustained innovation requires that U.S. health dollars be spent efficiently. However, many policies that aim to curb health care spending use an indiscriminate approach and focus at either the sector or aggregate spending level. This research will develop an approach to evaluate the efficiency of health care spending at the disease level that incorporates both quality and costs. Ultimately, the two goals of this project are to 1) provide information that helps identify potential opportunities for future health care investment and 2) identify diseases that are potentially associated with lower value care.



In progress

➤ **The Effects of Interruption in Innovation on Patient Outcomes Across Select Conditions in the U.S.**

In response to increasing pressure to contain health care costs, aggressive new regulatory and legislative actions to address drug spending in the U.S. are being proposed. To help demonstrate the value of biopharmaceutical innovation to the overall health system, NPC will examine how an absence of innovation in medical technology would affect the overall cost of care and impact patient mortality and morbidity.



In progress

➤ **Drug Spending Mirrors General Health Spending**

Specialty drug spend now accounts for nearly half of total drug spending while at the same time only comprising a small percentage of total prescriptions. While specialty drugs do contribute a disproportionate

share to total drug spend, it is well established that a small set of patients account for the majority of health care spend. This research will explore whether the overall specialty drug spending trend is consistent with broader health spending trends in other settings of care. If specialty drug spending matches overall spending patterns, it would indicate that specialty spending contributing a disproportionate share to drug spending is neither surprising nor particularly worrisome.

 *In progress*

➤ **Understanding How to Convey Low- and High- Value Information to Consumers and Patients**

Health Spending in the United States continues to rise, and estimates have shown that as much as \$340 billion is spent on low value or unnecessary care alone. To reduce waste in the system, most efforts have focused on provider behavior, but fewer efforts have focused on patient and consumer behavior. Through a partnership with a medical education multi-channel communication organization, NPC will conduct an online survey assessing consumer perceptions regarding how they might best receive information related to low-value and high-value services. This research will be disseminated through a public event focused on informing and advancing a public dialogue on health care spending and how low-value care services fit into that public dialogue.

 *In progress*

➤ **The Impact of Pharmaceutical Innovation on Longevity and Hospitalization in the U.S., 1999-2017**

In response to growing pressure on the health system, there has been an increased focus on biopharmaceutical prices. The focus of public discussion on biopharmaceutical prices without understanding of associated improvements can create an environment where policy changes can threaten innovation. This research will develop the empirical evidence to make this connect clear. This study will test the hypothesis that the introduction of new drugs has played a major role in the long run increases in U.S. longevity and the decline in mortality and hospitalization from diseases for which there have been more pharmaceutical innovation.

 *In progress*

CROSS-PORTFOLIO ACTIVITIES

➤ **Payer Engagement Surveys and Advisory Board (PESA)**

Health plans, PBMs, and public payers are a primary target audience for NPC and there is a need to appropriately and routinely engage thought leaders and decision makers to assist in NPC's project formulation and messaging to these groups. Historically, NPC solicited feedback from a small number of health plan representatives and had primarily anecdotal conversations with a convenience sample of individuals. This effort will offer a more routine and consistent approach to streamline research projects, improve the understanding of key customer perspectives, facilitate message dissemination, and enhance the potential impact of NPC activities.

 *In progress*

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- **The CER Daily Newsfeed®** provides you with the latest news and information on comparative effectiveness research. The Newsfeed is sent to your inbox each weekday afternoon, and each issue is archived and fully searchable on our website.
- **E.V.I.dently®** is NPC's monthly e-newsletter focused on comparative effectiveness research, health and productivity issues, quality measures and related research of importance to the pharmaceutical industry. Look for E.V.I.dently in your inbox at the end of each month.
- **The *Going Below The Surface (GBTS)*** website houses the latest news, research and events related to the *GBTS* initiative. *GBTS* was launched by the National Pharmaceutical Council in 2018 to broaden and improve the conversation around how we use health care resources in the United States. The initiative is aimed at better understanding the roots of the nation's health investments by promoting a discussion that is firmly based in health policy and systems research.
- **NPC Events**—NPC hosts several engaging conferences each year. Meeting information is sent via email on a periodic basis.



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