

## Addressing the need for real-world observational research solutions



There is an overwhelming demand for high-quality, reliable, real-world information about the effectiveness of treatments and optimal patient care for health care decision makers in the United States. Payers use published research findings to make coverage decisions. Health care providers rely upon peer-reviewed journals to inform which treatments work best for which of their patients. More and more, patients are becoming active partners in managing their health conditions and are seeking out additional information about their prescription medications.

Pharmaceutical and device manufacturers are often required to conduct comparative effectiveness research (CER) to address these real-world evidence needs. CER is generally defined as research that is intended to compare two or more interventions or approaches to health care with the goal of generating evidence-based information to assist patients, health care providers and other stakeholders with decision-making.<sup>2,3</sup> Retrospective database studies using existing secondary data (administrative claims and/or clinical data), prospective observational studies, randomized control trials (RCTs) and pragmatic trials are all used in CER.<sup>4</sup> RCTs are largely viewed as the gold standard; however, they are often complex and expensive, and have lengthy timelines, which can impose a significant burden on participating health care providers and their patients. Pragmatic trials are less restrictive than RCTs, although they typically require large patient populations, which can be a limiting factor for studying rare health



**The Centers for Medicare and Medicaid Services (CMS) projections:**

**5.6%** annual growth rate of national health spending 2016 through 2025.<sup>1</sup>

**6.3%** increase in prescription drug spending per year.<sup>1</sup>

conditions. Retrospective database studies are an economical solution and are widely utilized in CER; however, they have some inherent limitations, as secondary data are collected for the purposes of reimbursement or routine medical care and not for research.

Prospective observational studies that combine real-world secondary data captured in routine medical care with primary data collected from patients and health care providers are becoming increasingly more accepted as alternate or complementary solutions to RCTs. Optum® has developed a unique direct-to-patient study approach that combines the strengths of primary and secondary data in an efficient observational study design that meets our clients' CER and other real-world evidence needs across a wide range of therapeutic areas.

## Optum Health Economics and Outcomes Research (HEOR)

Optum HEOR has expertise in the design, conduct and analysis of real-world studies employing state-of-the-art methodologies to help address pharmaceutical and device manufacturer clients' challenges in a complex and constantly changing health care environment. We are a highly educated and experienced team of more than 85 people with a wide array of scientific backgrounds and advanced analytics expertise. Our widely renowned masters- and doctoral-level researchers use our proprietary research database, the Optum Research Database (ORD), to conduct a broad range of retrospective database studies including comparative effectiveness, cost-effectiveness and burden-of-illness research. In addition, our dedicated primary data collection (PDC) team focuses on real-world evidence generation with an emphasis on prospective observational studies that combine primary data collection methodologies with secondary data contained in the ORD.

### Overview of the Optum Research Database (ORD)

Optum data is national in scope. Unlike smaller databases and databases from closed health plan systems, the ORD represents patients enrolled in one of the largest providers of commercial and Medicare Part D health plans in the United States. Because the ORD is constructed from a variety of geographic regions and employer groups, the database maintains a level of diversity while representing overall trends in commercial health plan coverage. It comprises medical and pharmacy claims data (including linked enrollment) from 1993 to current, covering 64.3 million lives. In contrast to other databases, individuals are assigned unique identifiers that allow Optum to follow patients longitudinally as they enroll, disenroll and re-enroll in the health plan. In addition, the ORD contains actual patient copayments and deductible amounts, allowing for an accurate assessment of patient and payer burden. In many databases, patient burden is estimated from other payment amounts or standard cost values representing amounts paid being applied to service utilization.



#### The team

**85** people with a wide array of scientific backgrounds and advanced analytics expertise.



#### The database

**ORD** is one of the largest and most complete claims databases in the United States.

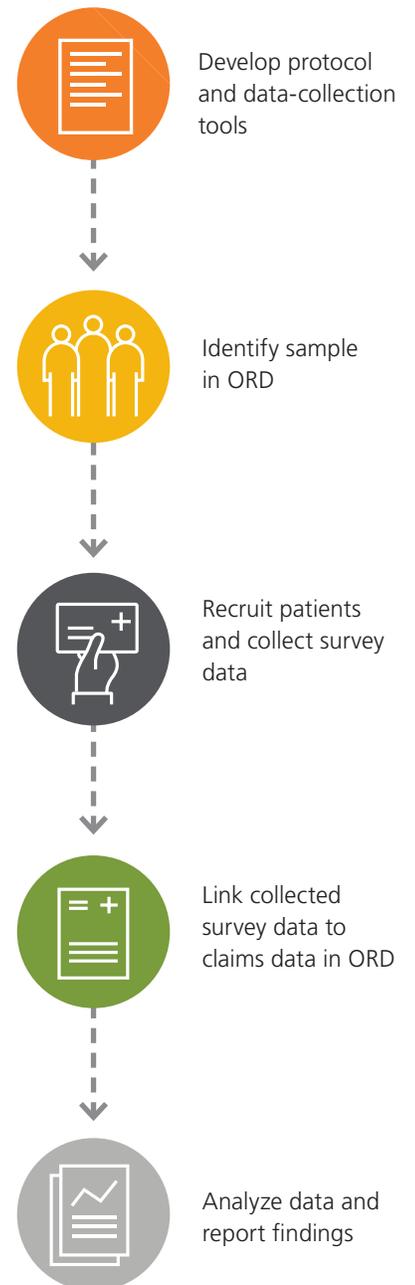
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## The Optum direct-to-patient study approach

Our direct-to-patient study approach (Figure 1) has several important key features:

- Customized study designs** — Our team of highly experienced PDC researchers work with our clients to design studies that are assured to address the study objectives and goals, yet take into consideration any budget and timeline constraints. Our masters- and doctoral-level researchers have an average of 15 years of experience with primary data collection methodologies, including mail, phone and web-based surveys, medical record abstraction, and qualitative research techniques such as in-depth interviews and focus groups. They have proficiency across all major therapeutic areas and can consult on appropriate patient-reported outcome measures for the specific patient populations of interest in addition to the condition-specific, claims-based measures. Our team is well established in this area, having conducted more than 40 direct-to-patient studies for major pharmaceutical companies since 2006.
- Targeted patient identification** — Using the vast medical claims, pharmacy claims and enrollment data contained in the ORD, our team can create a targeted sampling frame of patients for study participation using protocol-driven study criteria. Optum has developed a HIPAA-compliant approach that permits our use of identifiable information when certain regulatory requirements are met. Patients can be identified in one wave or in multiple waves on an ongoing basis, as required for the study design.
- Efficient patient recruitment and data collection** — Our successful direct-to-patient recruitment and data collection approach follows widely recognized survey procedures established by Dillman et al., also referred to as the Tailored Design Method (TDM).<sup>5</sup> Our study mailings are sent in personalized envelopes (i.e., the address is printed directly on the envelope, not a mailing label) and first class postage is used, which has been shown to increase response rates in mail surveys.<sup>6,7</sup> Pre-paid incentives increase patient participation, therefore, we recommend their use when possible; Otherwise, post-paid incentives are provided.<sup>8,9,10,11,12</sup> In addition to the initial mailed survey, we can administer follow-up surveys (web-based or mailed) as part of a longitudinal study design. We can also capture data transmitted via patient's wearable devices or sensors to examine patient's use of medications or devices in real time. The response rates for our cross-sectional, direct-to-patient survey studies typically range from 30 percent to 35 percent, and for longitudinal survey studies, the average retention rates are from 80 percent to 85 percent.
- Linkage to secondary data** — In addition to using the ORD to identify a targeted patient sample, a core component of our direct-to-patient study design is the direct linkage of a patient's primary data (surveys, device or sensor data) to their claims data during the same time period providing unparalleled insight into the patient's health care journey. We can examine patient's medical claims to understand their co-existent health conditions and their use of medical services and associated costs, in addition to numerous clinical characteristics and outcomes of interest during the study period. We can also review pharmacy claims data to determine a patient's treatment patterns and outcomes (persistence, discontinuation and switching) as well as their pharmacy expenditures (plan-paid and patient-paid). We can review laboratory data for a subset of patients to understand certain clinical outcomes (for example, changes in HbA1c). Figure 2 presents standard patient-reported and claims-based study measures used in CER that can be collected using our direct-to-patient survey design.
- Research dissemination** — By linking primary data with secondary data, the Optum direct-to-patient study approach provides the unique depth and breadth to address a wide range of questions for a variety of audiences — payers, health care providers, patients, regulatory agencies. Our team has conducted more than 50 presentations and publications for our direct-to-patient study design; citations can be provided upon request.

**Figure 1. Direct-to-patient study process**



**Figure 2. Direct-to-patient study measures for CER**

Patient-reported-outcome measures	Claims-based measures
<ul style="list-style-type: none"> <li>• General and condition-specific health status</li> <li>• Treatment satisfaction</li> <li>• Symptoms and symptom severity</li> <li>• Reasons for discontinuation/switching</li> <li>• Dosing and administration preferences</li> <li>• Productivity, activities of daily living (indirect costs)</li> <li>• Health behaviors (diet, exercise, smoking)</li> <li>• Patient understanding of regimen</li> <li>• Patient activation</li> <li>• OTC treatment use and cost</li> <li>• Basic clinical information (diagnosis, height, weight)</li> <li>• Demographic and sociodemographic characteristics</li> </ul>	<ul style="list-style-type: none"> <li>• Condition-related treatment</li> <li>• Condition-related comorbidities</li> <li>• Comorbidity burden</li> <li>• Treatment switching and augmentation</li> <li>• Medication dispensings</li> <li>• All-cause health care costs (direct costs)</li> <li>• All-cause resource utilization</li> <li>• Condition-specific health care costs</li> <li>• Condition-specific resource utilization</li> <li>• Condition-specific events (e.g., flares, exacerbations)</li> <li>• Select laboratory results</li> </ul>

**Sources**

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To learn more about the direct-to-patient study design offered by Optum, please contact us.

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