

Getting Precise about Precision Medicine: Balancing Hope, Potential and Cost

Market Corner Commentary
January 8, 2019

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Definitions of precision medicine are anything but precise. For seriously ill patients and their families, precision medicine therapies provide a hope when all else has failed. They're willing to risk long odds for the chance to improve or extend life, but they want health insurers to cover the costs.

For pharmaceutical companies, genetic research offers the potential to discover life-altering drug therapies and reap enormous financial rewards – even for disease cures that benefit few people. They have pushed government and commercial payers to reimburse expensive – and often experimental – treatment regimens even before demonstrating efficacy and quality of life improvements.

Frontline clinicians and health system administrators want access to therapies and protocols that deliver significantly better outcomes at a lower cost, ideally reducing clinical and administrative costs. Payers want to see tighter correlations between outcomes and treatments with more rational payment mechanisms.

Expanding medical frontiers, standardizing treatment protocols for complex diseases and applying effective cost-benefit payment methodologies simultaneously require tricky balancing. To advance precision medicine as a practical care solution that works for all stakeholders, we must establish a more precise understanding of its components, applications and costs.



THE DAWN OF A NEW MEDICAL AGE

Precision medicine emerged with the new millennium. During a June 2000 event announcing the mapping of the human genome, then President Bill Clinton hypothesized that “our children’s children will know the term cancer only as a constellation of stars.”¹

At the same press conference, Francis Collins, National Institutes of Health (NIH) genome director, predicted that genetic diagnosis of diseases would occur within ten years and genetic treatments would begin with fifteen years. “Over the longer term, perhaps in another fifteen or twenty years,” Collins said, “you will see a complete transformation in therapeutic medicine.”²

To date, precision medicine has offered more promises than results. The origins of disease and the complexity of human genetics are more complex than previously believed. Genetic discoveries raise as many or more new questions as answers.

The number of purely genetic diseases like hemophilia and cystic fibrosis is relatively small. Almost all diseases manifest when genetic predisposition confronts a complex array of environmental triggers, lifestyle factors and behaviors. The search for coherence and understanding continues.

Today, precision medicine applies genetics, biomarkers, personal data, big data and computational power to reduce clinical variation in the ways individuals are grouped according to their disease characteristics. With better information, doctors can apply therapies with higher probabilities of successful outcomes. Greater precision in diagnosis and treatment offers the potential for individually-tailored, evidence-based therapies.

FORCES DRIVING ADVANCES AND BREAKTHROUGHS

Precision medicine's potential is expanding as technological, clinical and economic factors advance its cost-effectiveness. Genetic testing is becoming as routine as blood tests. The cost of decoding the human genome has plummeted to less than \$1,000. Hospitals and research institutions are increasing the number of routine genetic tests they perform.

The consumer market for genetic testing is also growing rapidly. In 2018, 504 registered labs (256 in the U.S.) conducted tests on 16,419 genes.³ Mail-order tests offered by companies like [23andMe](#) and [Ancestry.com](#) allow consumers to discover their genetic ancestry and predisposition to specific traits and diseases. Even companies outside traditional healthcare, like Nestlé and Campbell, are using genetic testing to personalize nutrition, creating "nutraceuticals" that meet individual needs.⁴

The Food and Drug Administration approves precision medicine therapies more rapidly than other specialty drugs. These medicines can be expensive, especially when the new therapies are "orphan drugs" that treat rare conditions. Longer term, the FDA's belief is that accurate diagnosis and targeted treatments will enhance care outcomes at lower total costs of care.

Along with the explosion of genetic data, researchers and clinicians now have access to massive quantities of clinical, consumer, behavioral and social determinant data. The surge in computational power and artificial intelligence makes this data more accessible and useful.

As a result, our understanding of disease, lifestyle and genetic variation is growing, and we are gaining new insights into the effectiveness of therapies, treatment protocols, and wellness and prevention strategies. As genetic and related data proliferate, the challenge becomes how to optimize the data's use – finding the "signals" amid the data "noise."



As health systems embrace risk-based payment models, their economic incentive to diagnose and treat consumers is also growing. Precision medicine offers a vehicle for accomplishing that objective. Combining effective therapies that employ consumer-specific genetic, clinical and personal health data has the potential to generate superior health outcomes at lower costs.

TARGETING CANCER

Precision medicine advances help identify and diagnose cancer and determine more effective treatment therapies. For example, Chronic Myeloid Leukemia (CLM) is a rare form of leukemia with increasing cure rates resulting from genetics-based therapies.

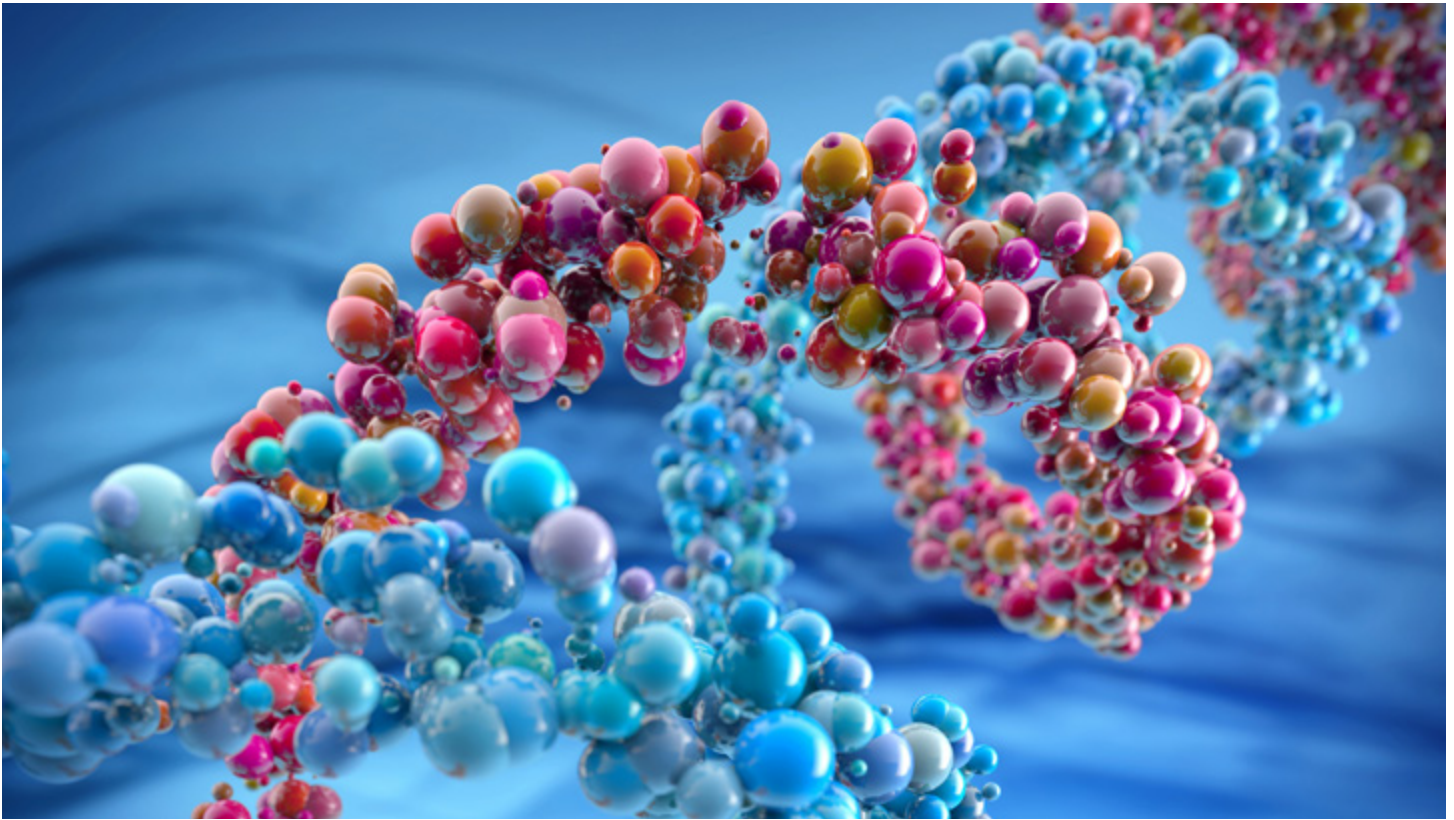
Until the early 1990s, doctors treated CML with bone marrow transplants and daily injections of interferon. The therapy was highly toxic. The five-year survival rate was approximately 30 percent.⁵ Then scientists discovered the protein that accelerates CML cell growth and applied targeted therapies to slow its growth. Remarkably, CML has become a chronic condition treated with a daily pill⁶, rather than a fatal disease.

Similar medical breakthroughs can transform care delivery and health outcomes while enhancing quality of life. Using genetic

testing, doctors can more quickly and accurately diagnose a patient's cancer and determine an effective drug or treatment. They can also avoid wasting time on ineffective therapies while minimizing harm and toxic impact.

Drug manufacturers see great promise in developing more targeted drug therapies. In February 2018, Roche increased its 12.6 percent ownership of a five-year-old company, Flatiron Health, by purchasing the remaining 87.4 percent for an additional \$1.9 billion.

Flatiron collects structured and unstructured oncology data from multiple sources and uses analytics to standardize and assess that data. For Roche, Flatiron's ability to produce data-driven insights and real-world treatment efficacy improves the quality, efficiency and cost of their clinical trials.⁷



ADOPTING PRECISION MEDICINE PRACTICES

Health systems are beginning to apply these potential breakthroughs in strategic, comprehensive and practical ways.

In 2013, Providence St. Joseph Swedish Cancer Institute (SCI) determined that precision medicine would become central to its care vision.⁸ Today, precision medicine at SCI focuses on the following two components of comprehensive cancer care:

- Using genetic information to identify cancer tumors and apply targeted therapies for treatment, and;
- Supporting patients' by meeting their unique, holistic needs (ranging from psychological counseling to nutrition to art therapy)

SCI started its precision medicine initiative by identifying the genetic mutations of certain cancers and developing treatments with clinical trials from those insights. The Institute then partners with [Syapse](#), a company that works with health systems to enhance precision medicine programs by analyzing molecular, clinical, treatment and outcomes data to deliver insights to oncologists, service line leaders and researchers.

Syapse's data platform is used at SCI, weaving cancer research, genetic insights and personal information (experiences, histories, behaviors, etc.) into personalized patient profiles. These profiles guide clinicians in targeting therapies for

eliminating specific types of tumors. The profiles also enhance overall diagnosis, care paths and post-treatment care.

Syapse's data platform continues to grow with the addition of 5,000 newly diagnosed patients each year at SCI. As its analytic capabilities grow, SCI expects the database will become a rich source of clinical insights that will advance the effectiveness of cancer therapies.

Precision medicine offers clinicians the opportunity to engage patients holistically. Clinicians can go beyond the medical record to understand the totality of factors influencing disease cause, progression and the efficacy of treatment. A wider understanding of why and when a treatment works, for the clinician and patient, enhances efficacy and care outcomes.

On a practical level, SCI has encountered several challenges, particularly related to payment for pharmacological treatments (i.e. drugs). Third-party insurers do not always cover the costs of targeted therapies. Reimbursement uncertainty can impede optimal care decision making.

In response, some drug manufacturers are tying reimbursement to treatment success, although there is significant debate regarding appropriate "success" metrics. The vertical integration occurring between insurers, pharmacy benefit managers and pharmacies could lead to more accurate cost-benefit assessments of pharmacological cures relative to treatment outcomes.

OPTIMIZING CANCER CARE PROTOCOLS

In 2015, Nikhil Wagle, M.D., an oncologist at Dana Farber Cancer Institute, launched The Metastatic Breast Cancer Project.⁹ Wagle's idea was to build a massive data library to collect individual consumers' genetic backgrounds, medical records, treatments and outcomes, as well as their personal experiences of their illness and treatment. This includes how people felt about their care and their quality of life.

Dr. Wagle, like most physicians, believes that accurate quantitative and qualitative data is necessary to identify disease patterns, inform treatment protocols and generate better outcomes. To date, the complexity of accessing and inputting these massive data sets has limited the project's impact.

Cota Healthcare, an innovative, data-driven company, takes a different approach. Cota curates data within patient records to develop structured, evidence-based groups that enhance diagnosis and treatment.

Founded in 2011 by a team of oncologists, engineers and scientists, Cota's approach to data analytics starts by creating "clinically identical" patient sets. These Cota Nodal Addresses (CNAs) integrate disparate data from electronic medical records (EMRs), including unstructured data in physician notes and lab reports, along with genomics, into a single usable code. They also include data that tracks therapy and patient progress to create longitudinal data sets.

Cota's ongoing analysis of cancer patients reveals significant care variation among similar patient groups. This care variation is correlated with substandard outcomes and/or higher costs.

Cota's clinically identical groups enable "apples-to-apples" comparisons of individuals and the development of optimal care patterns.

Cota's data-driven insights help doctors understand how patients will respond to different treatments. They also give physician groups the knowledge to standardize treatments, improve outcomes, control costs and enable precise payments.

Baptist Health South Florida's Miami Cancer Institute uses Cota to do just that. Cota's service empowers the Institute to compare genomic data with historical clinical and cost data. This "evidence" of effective care protocols aligns physician practices with organizational goals and individual needs.

Standardizing cancer protocols reduces care variation, improves outcomes, lowers costs, and enables the Institute to adapt to value-based payment models. According to Leonard Kalman, Deputy Medical Director and Chief Clinical Officer,

"This [Cota's data analytics] not only increases the level of precision in our patients' treatment journey, but it also ensures that every individual receives the highest quality of care possible, which increases the odds of survivorship."

Few health systems can build sufficient capabilities in-house to power effective precision medicine platforms. Providers typically need to partner with external suppliers, such as Syapse and Cota, to create a seamless interface that brings optimal care outcomes to consumers by connecting evidenced-based care protocols and effective care delivery.



CONSUMER-CENTRIC CARE BUILDS BRAND STRENGTH AND INCREASES MARKET SHARE

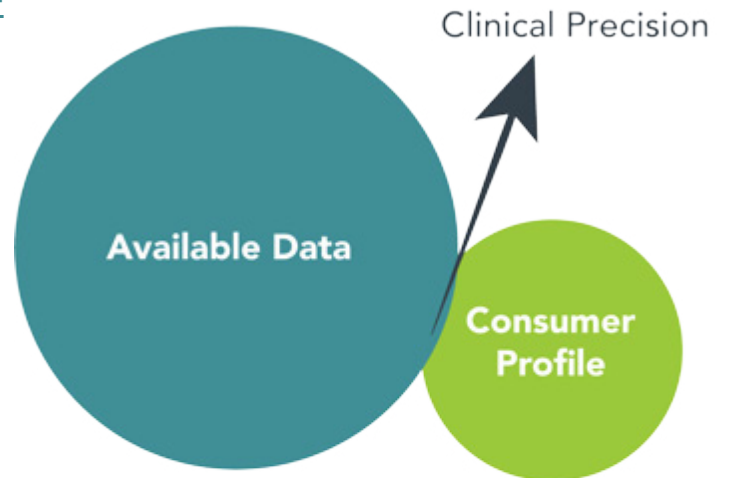
As a technical endeavor, significant obstacles prevent precision medicine from becoming routine care delivery. Deeper clinical understanding of specific conditions and care paths, as well as more effective data consistency and integration, are prerequisites for success. Still, clinicians and researchers are making meaningful progress optimizing diagnosis and treatment.

The care journey begins with a holistic view of the consumer's lifestyle and disease profile. It continues with an assessment of the capabilities (insourced and outsourced) required to optimize treatment outcomes for that consumer.

Customer-focused companies like Amazon, Google, Ritz Carlton, Starbucks and Walmart understand their customer priorities because they are able to leverage data. Amazon and Google built data platforms that constantly monitor, anticipate and deliver on customer needs. The rise of big data, targeted advertising and artificial intelligence (AI) allows companies to segment customers into ever-smaller groups, even to the individual. Ritz Carlton and Starbucks collect preference and behavioral data and use it to develop powerful relationships that deliver on their promises. These companies are able to engage customers more effectively than most healthcare organizations.

In healthcare, coordinated programs create improved treatment experiences for consumers. They support brand recognition in the marketplace for delivering exceptional quality care and customer service. A health system that uses big data for more precise clinical expertise, exceptionally efficient operations, and highly tailored clinical and holistic care capabilities will attract and retain consumers and payors just like consumer-centric companies.

Holistic Care Powered by Precision Medicine



Consumer Profile

- Health status, history
- Disease, condition
- Goals, priorities
- Holistic needs
 - Personal
 - Financial
 - Emotional
 - Physical
 - Spiritual
 - Community
 - Caregiver support

Clinical Precision

- Expertise
 - Tapping specific in-house expertise
 - Sourced from analytics partners
- Optimal care path
 - Treatment protocol
 - Drug therapies
 - Continuous care from diagnosis
- Coordinated care providers

Available Data

- Health system EMR
- Patient record
- Genetic, evidence-based understanding of condition
- Optimal care path, drug therapy

CUSTOMIZED TREATMENT AT SCALE

Precision medicine is a potential game changer for healthcare delivery. A comprehensive precision medicine program combines targeted pharmaceutical therapies and data-rich, evidence-based protocols with a meaningful commitment to holistic care services tailored for individual needs.

Combining medicine's art and science reduces care variation, empowers clinicians with specific and meaningful insights, engages consumers in their care and generates better, more cost-effective care outcomes.

To realize precision medicine's benefits, health systems must implement a comprehensive effort across the entire healthcare

system. They will need to advance clinical efficacy, optimize care delivery and deeply engage consumers with precision medicine. Diagnostic and pharmaceutical companies will need to continue developing targeted ways to treat disease. Data and analytics companies will need to apply the power of big data to individuals so clinicians can use precision medicine to customize care at scale. Then clinicians can provide more effective treatment and consumers will have better outcomes.

Precision medicine will not cure disease, but it does represent significant and tangible improvement in holistic and appropriate care delivery. That's precision medicine's real promise and potential.

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David Johnson is the CEO of 4sight Health, a healthcare advisory company working at the intersection of strategy, economics, innovation and capital formation. Dave wakes up every morning trying to fix America's broken healthcare system. He is a frequent writer and speaker on the need to transform to a pro-market healthcare system. His expertise encompasses health policy, academic medicine, economics, statistics, behavioral finance, disruptive innovation, organizational change and complexity theory. Dave's book, **Market vs. Medicine: America's Epic Fight for Better, Affordable Healthcare**, is available for purchase on www.4sighthealth.com.



Dr. Harold Picken has more than 30 years of experience in healthcare. He works closely with physician leaders to bring their voice and perspective into key change efforts. Dr. Picken has worked both clinically and as a physician leader in a wide range of healthcare settings including acute inpatient care, intensive care units, long-term acute care hospitals, skilled nursing facilities, skilled home care and physician home care. He has serviced as a vice president in two regional Blue Cross plans, as well as Chief Medical Officer for several healthcare technology companies, including a telemedicine organization. He has also been an instructor in the Department of Health Policy and Management at the Harvard School of Public Health.

Outcomes Matter. Customers Count. Value Rules.