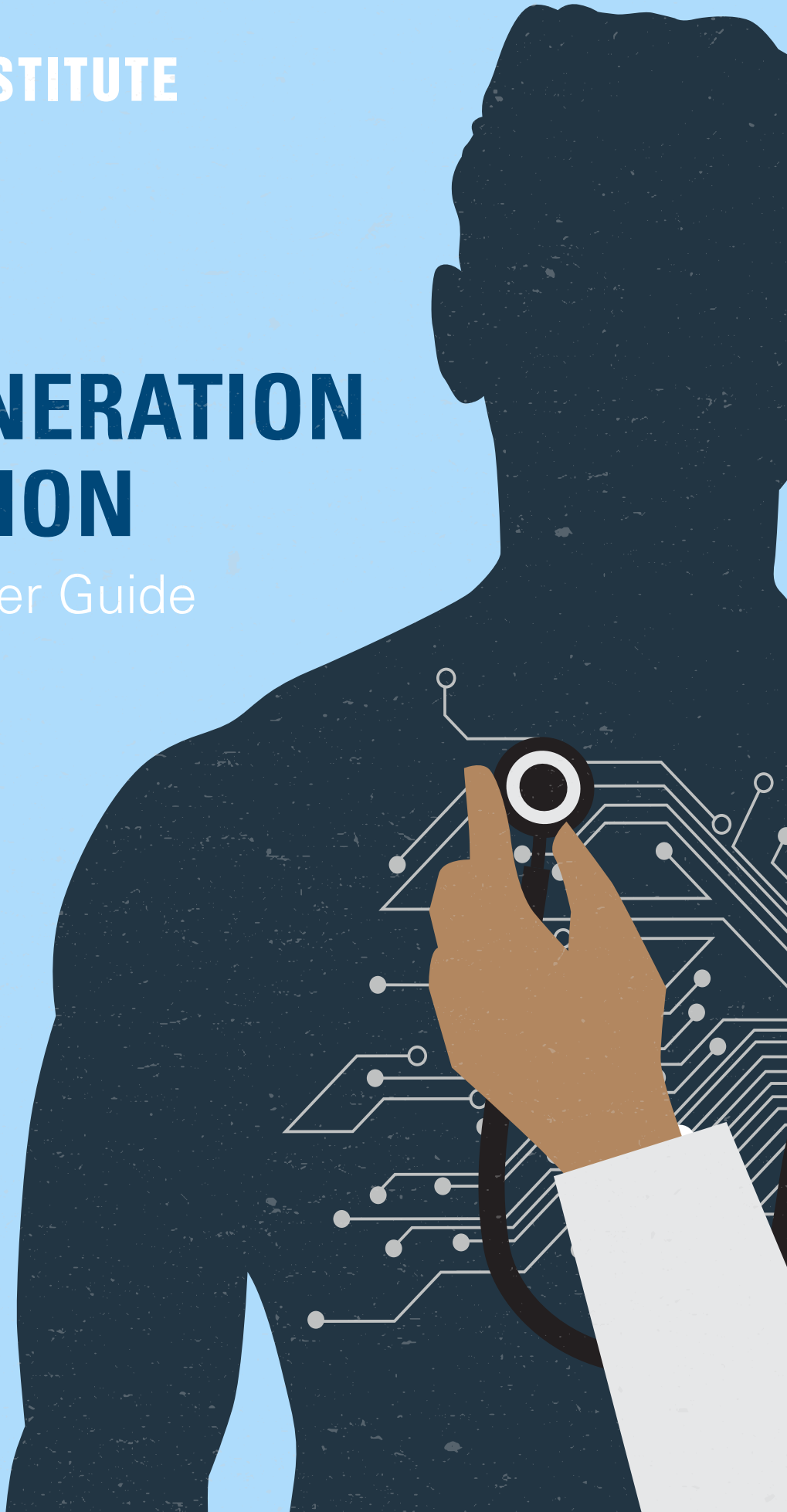
MILKEN INSTITUTE

# NEXT GENERATION PREVENTION

## A Giving Smarter Guide



# ABOUT US

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## ABOUT THE MILKEN INSTITUTE

The Milken Institute is a nonprofit, nonpartisan think tank. We catalyze actionable solutions to persistent global challenges by connecting human, financial, and community resources to those who need them. Guided by a conviction that the best ideas, under-resourced, go nowhere, we conduct applied research, convene luminaries from competing viewpoints, and construct programs and initiatives designed to achieve our overarching mission of building meaningful lives.

## ABOUT THE CENTER FOR STRATEGIC PHILANTHROPY

The Milken Institute Center for Strategic Philanthropy believes philanthropy has the opportunity and obligation to take on big risks to test bold ideas. We conduct deep due diligence across a range of issue areas, promote creative and well-informed giving strategies, and advise families and foundations on where and how to channel their philanthropy to maximize a return on their investment.

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## Precision Health

The United States' health care system is expensive, with spending on health care reaching 17.9 percent of the U.S. gross domestic product (GDP), or $3.3 trillion in 2016. However, this increased spending does not seem to equate to better health or health care. In fact, the U.S. suffers significantly more from preventable diseases and health complications than other developed nations, in addition to suffering from a decreased life expectancy compared to other developed nations, which spend less on health care.

Chronic diseases—among seven of the top ten killers in the U.S.—cost over $1 trillion annually. Research suggests that almost two-thirds of U.S. adults have one or more chronic diseases, which are largely preventable. The mounting burden of chronic diseases, coupled with an aging population, requires better and earlier health interventions while cutting health care costs. The U.S. needs a more innovative way to provide care for patients—which is where precision health comes in.

Precision health focuses on improving overall health and preventing disease, rather than treating disease. Using individual patient information like genetics, environmental and psychosocial factors, and lifestyle, precision health as an emerging discipline seeks to predict illness and to keep people healthy. One of the major goals of precision health is to reengineer, and ultimately transition, a health system focused on reactive "sick care" to one focused on proactive "health care."

New emerging technologies like genomic testing and sequencing, digital health tracking, digital therapeutics, telemedicine, and cloud-based technology will facilitate this transition toward proactive care. These technologies will aid policymakers, researchers, and providers in achieving the precision health goals of helping individuals navigate their personal:

- Physiological health baseline
- Genetic predisposition to disease
- Awareness of how one's environment and behavior impact physiological and pathological changes
- Understanding of when one is in danger of developing a disease

### PHILANTHROPIC OPPORTUNITIES

Through interviews with key opinion leaders, systems-based analyses, and a funder's summit, we identified three barriers to using precision health ideas and technologies within medical practice, along with key funding opportunities to address each barrier. The three greatest barriers are:

1) Insufficient scientific evidence
2) Insufficient data sharing among relevant health partners
3) Lack of field-wide coordination

This guide outlines potential solutions to each problem, which will help move the field of precision health forward and into the mainstream. These barriers and solutions are outlined in more depth on page 27.

U.S. health care expenditure is projected to rise 1 percentage point faster than the gross domestic product every year; by 2026, the U.S. is projected to spend $5.7 trillion per year on health care.[i]

### Promote the development and standardization of precision health tools

Most current health tools do not focus on prevention, are not standardized, are difficult to compare, and are not up to clinical practice standards or ubiquitously adopted by the medical community. Investments in the area of novel technology development and innovation can foster more standardization and thus clinical adoption of precision health tools. Philanthropists should invest in areas that develop and standardize both new and existing tools for clinical utility.

### Support the development of and continued use of medically-validated measures to record daily functioning, lifestyle, and environmental factors to quantify health

Validated assessment metrics will provide more clarity to patient and clinician users, capture useful human data, and better integrate into medical systems. Investments in this area will fund pathways to support the validation of precision health tools. Philanthropists can help quantify health by supporting the development of medically-validated tools and metrics.

### Explore the cost effectiveness of measurements in existing longitudinal cohorts

Cost effectiveness analysis informs policymakers and decision-makers about the potential return on investment or cost of medical interventions. Supporting economics research like the long-term economic impact of using precision health technologies (e.g., direct-to-consumer genomic testing and telemedicine), will provide another avenue for precision health validation and subsequent integration into the U.S. health care system. Philanthropists can fund research that explores the longitudinal economic and health benefits of precision health.

### Create specialized data platforms to facilitate data sharing in addition to funding data gathering, storing, analyzing, and sharing

Using new technology for the purpose of open access data sharing encourages multi-stakeholder collaboration and analyses. These analyses can streamline and accelerate the success of precision health. Data sharing expansion should increase interoperability of electronic health records and enable researchers to extract information from open-text sections of records.

### Develop an organization focused exclusively on precision health

Investments in a coordinating organization would tackle gaps in precision health coordination in the following fields: education, advocacy, synchronized research efforts, and patient input. A coordinating organization will develop education materials and physician outreach programs, unite disease-focused patient advocacy organizations, bring together researchers through meetings, and create a forum for public and patient engagement.

i. Data pulled from the U.S. Centers for Medicare & Medicaid Services.

# DRIVING SCIENCE THROUGH PHILANTHROPY

Historically, funding of the scientific enterprise has been left to government bodies and commercial industries, especially in the U.S. This trend leads back to several important realities, one of which is the significant level of capital investment and infrastructure required to support meaningful academic research and clinical development. Governments can often provide consistent funding to a breadth of research topics, while industry can move new therapies from benchtop to population.

Nevertheless, problems can emerge when only government and industry distribute the wealth in science. For instance, government funds can be slow to respond, given bureaucracy and priority changes after elections. Additionally, government funders are usually risk averse, typically favoring more "accepted" research and thereby leaving little room for creativity or untested ideas. On the other side, commercial players—from pharmaceutical giants to emerging biotechnology firms—are beholden to their investors who can also be wary of uncertainty. Similarly, when the financial or research bottom line is not met on funded projects, private investors may divest regardless of how successful—or needed—the research itself may be. The combination of these two realities can lead to stagnation in scientific innovation, that is, an inability to react quickly to a changing climate and gaps in funding to address complex problems.

At the Milken Institute Center for Strategic Philanthropy, we have witnessed first-hand how philanthropic capital can fill the gaps where commercial and government funds fall short. Foundations and philanthropists alike can fund cutting-edge research and novel hypotheses, providing the seed that might spark a field-altering discovery or spawn a new branch of science. In addition, they can move quickly in response to immediate needs, whether because of defunding through other mechanisms or a crisis. An infusion of philanthropic funds can de-risk a sector and demonstrate proof-of-

concept. **Philanthropy can act where other entities cannot—bridging sectors despite partisanship, bottom lines, or policy stances—and provide support where most needed.**

Philanthropy, however, has limitations. In the U.S., philanthropic capital represents a small percentage of overall scientific funding—merely 2 to 4 percent, depending on how it is counted. Therefore, this capital must be invested as wisely as possible to ensure that it reaps the desired dividends.

We know that the most effective philanthropists are usually the best informed. Like any savvy investor, philanthropists need due diligence and market analysis to identify the key gaps, barriers to entry, and hidden leverage points that can accelerate discovery and development. Using a systems-based approach and a cross-sectoral vantage point, philanthropy can be used strategically to solve some of the most challenging problems in science and beyond.

In the pages that follow, and in all of our research and analyses, we strive to provide deep scientific insight into the issues and to outline a concrete and actionable set of options for all philanthropists—no matter their size or location. We welcome all feedback regarding our findings, as well as partners in our quest to advance scientific knowledge through philanthropy. The stakes are high, and time is short. Let's work together to get this right.

# INTRODUCTION

The U.S. health care system is ever-changing and costly. In 2016, U.S. health care spending reached $3.3 trillion, or 17.9 percent of the gross domestic product (GDP). The Centers for Medicare and Medicaid Services (CMS) projects that health care spending will grow 1 percent faster than GDP over the next decade, rising to a cost of $5.7 trillion, or 19.7 percent of the GDP, by 2026.

Despite this significant spending on health care, the U.S. lags behind other developed nations in both health outcomes and life expectancy (Figure 1). While there are a great number of contributors to life expectancy, the U.S. has significantly more deaths from preventable diseases or health complications than other developed nations with adequate health care. The U.S. ranks 27th in the world in life expectancy, is the most obese developed nation, and has the sixth highest maternal mortality rate. Recently, policy and economic advisors have espoused a slogan called, "bending the cost curve." Health policy institutes, academic researchers, and politicians use the phrase as a way to suggest that the U.S. must slow the growth of medical costs or risk repercussions. In short, the United States needs to spend less and get more—cut down the medical and economic costs of chronic diseases and innovate to save money and lives.

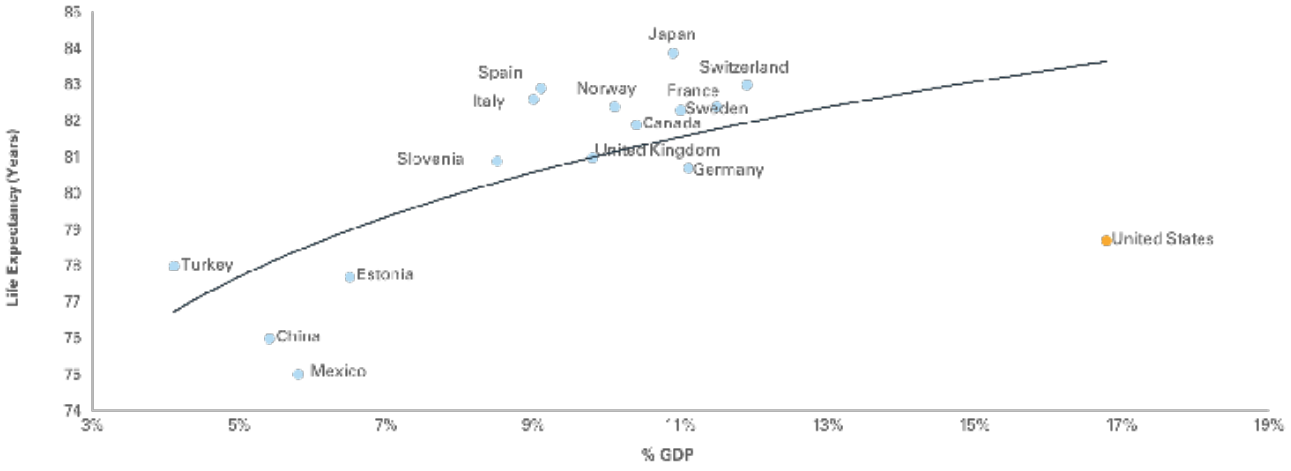
## FEDERAL HEALTH CARE PROGRAMS IN THE U.S.

The U.S. federal government operates national health programs, which combined make up approximately 26 percent of the federal budget, close to $1 trillion.

**Medicare:** A national health insurance program started under the Social Security Administration in 1966. It provides health insurance for people 65 and older.

**Medicaid:** A joint federal/state program that provides insurance to individuals with low income, children, and some individuals with disabilities. Eligibility for Medicaid varies by state.

**Figure 1. Health Care Spending (% GDP) Compared to Life Expectancy of Member Countries of the Organisation for Economic Co-operation and Development (OECD), 2015**



# INTRODUCTION

## CHRONIC DISEASE

According to the Centers for Disease Control and Prevention (CDC), about half of U.S. adults has a chronic disease, and one quarter of adults has two or more chronic diseases. Chronic diseases, like heart disease, cancer, diabetes, and certain lower respiratory diseases, are among the leading causes of death and decreased quality of life in the U.S. Heart disease, a term encompassing several conditions like coronary heart diseases and heart attacks, is the leading cause of death and takes the lives of more than 810,000 Americans every year. Heart disease costs the U.S. health care system $200 billion per year in health care services, medications, and lost productivity. Diabetes and its precursor stage, prediabetes, costs the health care system over $245 billion. Ninety percent of Medicare spending comes from people with one or more chronic condition; almost 70 percent of Medicare beneficiaries have two or more chronic conditions.

Chronic diseases can be debilitating and very expensive, but some are fundamentally preventable. The American Public Health Association suggests that disease prevention saves not only lives, but also money. In fact, their pooled estimates suggest that every dollar invested in evidence-based programs seeking to improve physical activity, improve nutrition, or prevent tobacco use will save $5.60 in health spending within five years. Every dollar spent on tobacco cessation programs is estimated to return an average of $1.26, potentially saving the U.S. health care system more than $711 million per year.

**60%**
of U.S. adults have a chronic disease

**7/10**
deaths are caused by chronic diseases in the U.S.

**>$1T**
in annual costs due to chronic diseases

**560%**
return on investment for preventative care

The mounting burden of chronic diseases coupled with an aging population necessitate cutting costs and providing better health outcomes. The U.S. further requires a more innovative way to provide care for patients, which is where precision health comes in.

## NEXT-GENERATION PREVENTION

Precision health is revolutionizing health care delivery, treatment, and prevention. Precision health and precision medicine shift the focus from a one-size-fits-all approach to individualized health care delivery. By incorporating patients' genetic makeup, lifestyle choices, psychosocial indicators, and environmental factors, providers may be better suited to prevent diseases and detect them earlier in life.

The goal of precision medicine is to offer individualized, targeted, and highly-tailored approaches to treatment, in addition to identifying risk factors and preventing the early onset of disease. Precision health takes the concept of precision medicine further by using the same targeted, predictive tools like using genetics and lifestyle information, but in a preventative capacity. By better understanding which factors make a patient unique, a provider can tailor treatment or preventative services.

- Under the current paradigm, medical providers are trained to diagnose and treat individuals after they have developed a disease.

- The current state of health monitoring is fragmented and does not provide a comprehensive understanding of an individual's health over time.

- Health is only monitored when a person visits his or her physician. It is reactive rather than proactive, as most patients only see a clinician when symptoms present.

To re-engineer the system from "sick care" to "health care," a holistic transition into evidence-based, patient-centered care is needed. Recent advances in science and technology are rapidly improving precision health and medicine, making them increasingly more mainstream. Using novel technological monitoring advances, there will be a new focus on the pre-treatment aspect of medicine, namely the preventative aspect, rather than just focusing on treatment.

### TOP 10 KILLERS IN THE U.S.*

**1. Heart Disease**
633,842

**2. Cancer**
595,930

**3. Chronic Lower Respiratory Diseases**
155,041

**4. Accidents**
146,571

**5. Stroke**
140,323

**6. Alzheimer's Disease**
110,561

**7. Diabetes**
79,535

**8. Influenza and Pneumonia**
57,062

**9. Nephritis and Nephrosis**
49,959

**10. Intentional Self-Harm**
44,193

*Data were compiled from the CDC and represent the number of deaths attributed to each disease in 2015. Most cases of heart disease, cancer, chronic lower respiratory diseases, self-harm, and diabetes are largely preventable through better preventative health services.

# INTRODUCTION

This Giving Smarter Guide provides a comprehensive look at precision health and addresses unmet needs in the field, including where philanthropic investments may be of greatest use.

## HEALTH CARE INCLUSION

In presenting the findings of this report, it is important to note that minority and underserved populations are disproportionately impacted by their environment (e.g., decreased access to and quality of education, employment opportunities, healthy food, and health care), predisposing them to a higher risk of developing certain preventable diseases. These are known as health disparities.

New large-scale precision health initiatives, like the National Institutes of Health *All of Us* Research Program, seek to understand the impact of health disparities on health. The data collected from these initiatives will expand knowledge on how a multitude of factors contribute to health and the role of precision health in addressing those issues.

Increasing engagement with underserved and underrepresented communities, increasing access to health education, and facilitating dialogue will improve precision health's impact.

Precision health takes the concept of precision medicine further by using the same targeted, predictive tools like using genetics and lifestyle information, but in a preventative capacity.

# PRECISION MEDICINE VS. PRECISION HEALTH

Precision health and precision medicine are emerging disciplines at the intersection of science, technology, and medicine. Both seek to identify personalized approaches to keep individuals healthy. This section will outline how precision medicine and precision health are changing the medical landscape.

## PRECISION MEDICINE

Initially coined in 2011, the National Research Council defined precision medicine as tailoring medical treatment to the individual characteristics of each patient. Precision medicine gained national attention during President Obama's State of the Union Address in 2015 when he announced the Precision Medicine Initiative. The initiative promised a $215 million investment in delivering the right treatment in the right dosage to the right patient at the right time. The NIH eventually refined the definition, defining precision medicine as an approach to disease treatment that seeks to take into account individual variability in genes, environment, and lifestyle.

### INDIVIDUALIZED CARE

Tailoring care to the individual will reduce the need for unnecessary procedures and will ultimately decrease the cost of health care.

Precision medicine—also called personalized or stratified medicine—is about how clinicians diagnose and treat problems based on the individual characteristics, like lifestyle, environmental, or genetic factors, of each patient. Precision medicine seeks to improve the quality of care, reduce the need for unnecessary diagnostic tests and therapies, and ultimately reduce the financial burden on both the patient and the health care system.

### PREVENTATIVE CARE

Chronic diseases are among the top 10 killers in the U.S. and are largely preventable. Stopping these diseases by shifting toward preventative care could not only save millions of lives, but also save over $200 billion in health care costs over the next 25 years.

While the field has made great strides in helping clinicians treat patients more effectively, the cost of health care continues to rise. Although new, expensive health technologies contribute to the cost, the inability to prevent people from becoming sick continues to be one of the most significant drivers of national health care spending. Treating diseases is important, but it is equally important to prevent people from getting sick in the first place. One way to shift the health

care focus from reactive care to proactive care is by making individual preventative care better.

## PRECISION HEALTH

Precision health, while related to precision medicine, increases focus on improving overall health and preventing disease rather than treating disease. Precision health uses individual patient information, like genetics, environment, lifestyle, and psychosocial factors, to help predict illness and keep people healthy.

Technology wearables and other new innovations are helping people to better understand and manage their health. Individuals are now able to monitor their own heart rate, pulse, and other physiological measures cheaply without having to visit the doctor's office. Furthermore, these innovations help people to collect data about their health over time, which both they and their clinicians can use to monitor health.

Additionally, new partnerships are forming between data firms and health entities, capitalizing on the movement toward big data. The blossoming use of data in precision health promises innovation, more personalized care, and better dialogue among health care stakeholders.

Using data from technologies, providers can develop better prevention plans for individuals. People can stay healthy for longer by better understanding their risk for disease. Disease can be caught at earlier stages and allow interventions to be more effective. Additionally, individuals can become active participants in their own health rather than passive consumers and enjoy more user-friendly health care. These changes will allow people to live healthier lives and decrease the cost of health care by reducing the burden of chronic disease.

### PRECISION HEALTH

Precision health includes many components, including access to and control over one's own health and health data and access to one's own health GPS. Additionally, the application of precision health will lower the cost of health care and cause a shift from paternalistic medicine to partnership.

## MISSION AND VALUES OF PRECISION HEALTH

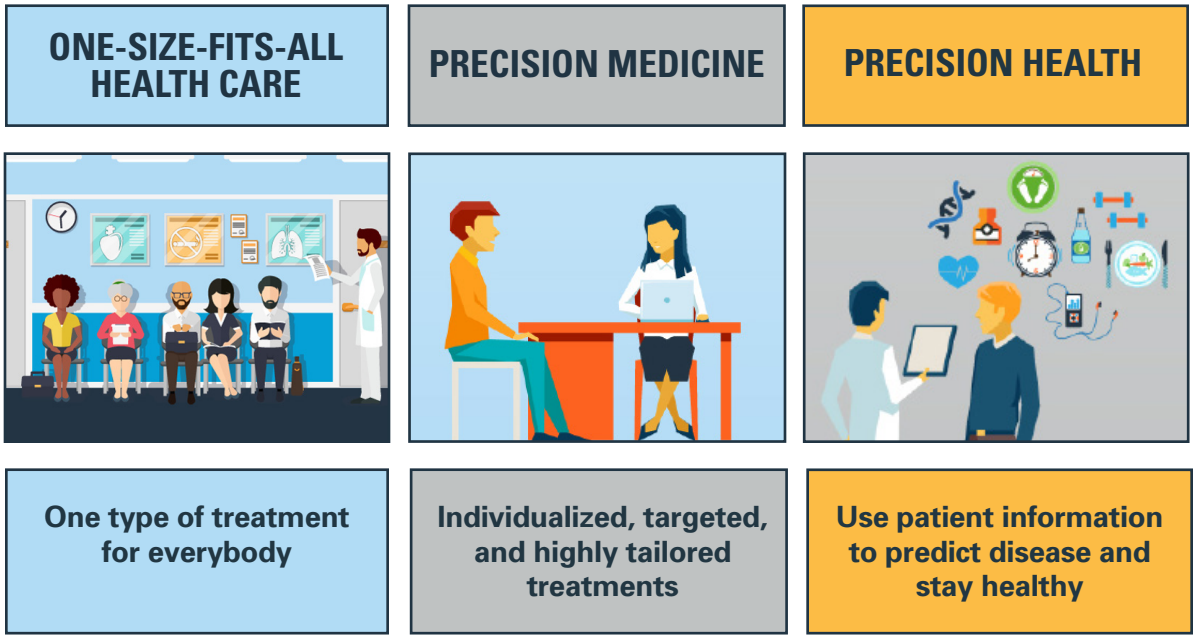
When precision health becomes more mature and optimized, individuals will be able to continuously monitor physiological changes to indicate their health status. Each individual will have access to a form of a health "GPS" to help them navigate their personal:

- Physiological health baseline
- Genetic predisposition to disease
- Awareness of how one's environment and behavior impact physiological and pathological changes
- Understanding of when one is in danger of developing a disease

### VARIANTS OF UNKNOWN SIGNIFICANCE

Some individuals have modifications in their genome known as variants of unknown significance, or VUS. Variances across a genome are normal; in fact, genetic variation is what makes people unique. Sometimes the implications of a mutation are unknown, and clinicians cannot tell if the alteration puts someone at risk of disease development or not. VUS results indicate uncertainty in a field that is sometimes seen by the public as absolute. Uncertain results might create undue stress or medical expenses for people who choose to act on missing or incomplete information.

**Figure 2. Summary of One-Size-Fits-All Health Care, Precision Medicine, and Precision Health**



These data, collected from a variety of sources like one's home, clinic, and environmental setting, enable real-time health monitoring. Using wearable technologies, home sensors, electronic health records, genomic testing, and social/environmental data sets, people will be empowered to take control of their health. People will have more input into their own treatment plans, resulting in not only a healthier society, but also lower health care costs.

The goal of precision health is to give patients and participants access to and control of their own information, and then show them how to use that information to help them better understand their own health. An additional goal of precision health is to shift the paradigm from a focus on paternalistic medicine to one that is foundationally a partnership between patient and provider.

At the end of this report, we outline three key barriers to advancing precision health and propose a number of solutions and philanthropic opportunities to address the gaps in knowledge and movement within each barrier.

# CURRENT STATE OF PRECISION HEALTH

The field of precision health is constantly changing. Thus, what follows is an assessment of the current state of emerging technology for precision health and an overview of relevant policies surrounding health and health technology.

## MISSION AND VALUES OF PRECISION HEALTH

New and emerging technology is driving progression in precision medicine and precision health. New technologies enable both patients and clinicians to track an individual's health over time and use that information for prevention.

### Genomic Testing/Sequencing

A number of direct-to-consumer (DTC) genomic testing kits are on the market. Companies like 23andMe, Helix, Novogene, and others offer consumers the opportunity to submit a biological sample, like saliva, and have their genes sequenced. Sequencing can tell consumers a number of things: the likelihood of inheriting certain genetic medical disorders, the risk of developing certain epigenetic (gene expression based on one's environment) diseases like type 2 diabetes, or even about ancestral background.

DTC genomic testing kits were originally so expensive that many consumers were priced out of the market; however, over time, these kits became affordable enough for the general population to use. Some of the more expensive testing services, which provide a richer set of data and analyze more or all of an individual's genome, are still out of the price range of many consumers.

The National Academies of Sciences, Engineering, and Medicine suggests that more work should be done in gathering evidence for the utility of genomic testing for underrepresented populations. Additionally, payers may garner more interest in genomic testing should it be framed in terms of mitigating health disparities and improving overall health.

### GENETICS REFRESHER

Cells are the building blocks of the body. Over 37 trillion cells come together to make tissues and organs, which work in harmony to create a functional person.

Within every cell is the genetic material that codes for these tissues: DNA. DNA is made of smaller fragments called nucleotides, which group together to create genes.

Genes code for everything in the body, from eye color to parts of the immune system. All the genes in a person are collectively known as a genome.

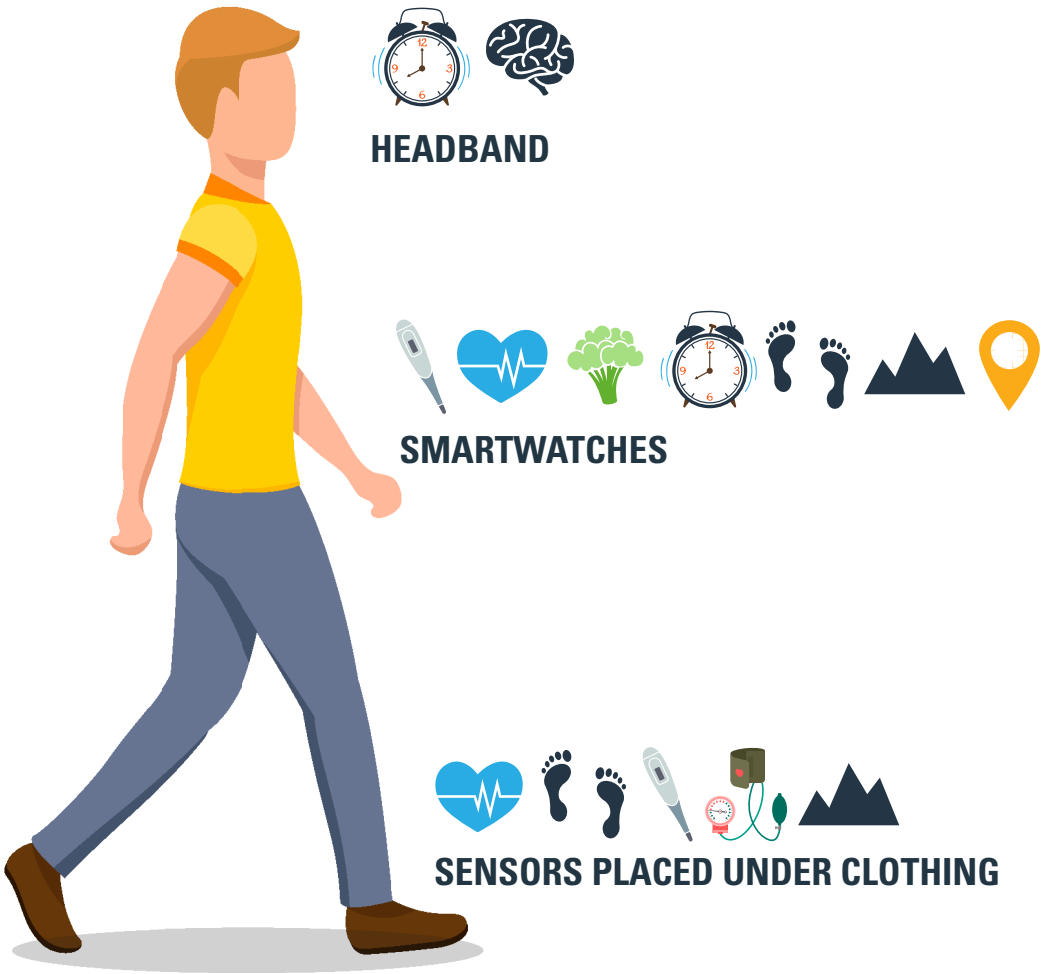
With new scientific advances, scientists are able to sequence (read) a person's entire genome—over 3 billion nucleotides—within a matter of days.

Knowing more about the risk of developing a disease based on one's unique genetic background helps both physician and patient have a realistic discussion about the likelihood of developing inherited diseases and the preventative measures that a person should take to stay healthy and thwart disease progression/development.

## Digital Health Tracking

Digital health trackers are tools that differentiate consumers based on their unique biological or lifestyle profiles. The often-used examples include: wearable technology like Fitbit, mobile device-based tools, and smartphone apps. These devices and applications regularly collect information on individuals related to lifestyle such as eating habits, or physiological profiles like exercise or heart rate.

**Figure 3. Digital Health Trackers and Their Functions**



Another new technological innovation that received U.S. Food and Drug Administration (FDA) clearance is the integrated sensor pill, which monitors when patients ingest medications. The pill is connected to a patch on a patient's torso to combine physiological data with adherence information, which may be useful for patients suffering from memory loss. The innovation may also be valuable for integrating medication adherence reminders and for facilitating a more holistic sense of well-being through enhanced agency and clinician/patient interactions.

Information collected by digital trackers provides doctors with a clearer profile of the unique attributes of an individual patient. The data may then help inform precision medicine and precision health efforts and refine the preventative measures taken by an individual. Used in conjunction with other methods like genomic sequencing, digital health tracking provides real-time, up-to-date insight into daily habits, which may then be used to stop the progression or development of ill-health.

### Digital Therapeutics

Using unique risk profiles, new organizations like Omada Health and Tilak Healthcare aim to deliver targeted, supportive behavioral therapies and counseling to people most at risk for developing expensive, debilitative, and preventable chronic diseases like type 2 diabetes. These organizations use digital therapeutic technology and counseling to work with consumers and their unique physiological, environmental, and lifestyle factors to prevent them from developing these diseases.

Some of these behavioral advising organizations partner with employers to deliver preventative care to the most at-risk employees. This ultimately reduces medical costs for both employee and employer. Some estimates suggest that this sort of preemptive behavioral counseling can allow employers to recoup their investment in as little as six months and could save over $1,000 in medical costs for each participant.

### FDA APPROVAL FOR OTSUKA PHARMACEUTICAL'S ABILIFY MYCITE

The FDA has approved the first clinical use of digital medicine for Japan-based Otsuka Pharmaceutical's Abilify MyCite. Digital medicine seeks to target the roughly 50 percent of patients who do not adhere to prescription recommendations and the 20-30 percent of patients who never pick up their prescriptions at the pharmacy. The first use of digital health promises to get real-time information into the hands of physicians so they may better individualize their approach to each patient. Because physicians may easily track a patient's lifestyle, dietary, and physiological attributes through integrated digital medicine technologies, these new therapeutics promise to improve health outcomes, increase access to care, and innovate our approach to medicine.

### Telemedicine

Telemedicine is an innovative new approach to health care delivery. The service, offered by several organizations like Teladoc or ConsultADoctor, among others, allows patients to overcome a variety of barriers impacting their access to providers. Telemedicine is useful to various populations including those in rural areas without a primary care clinic for hundreds of miles, troops overseas, and people with inflexible work or life schedules.

Telemedicine has the potential to increase access to primary care in the U.S. and around the world. More insurers in the U.S., including Medicare, are reimbursing for the service. Telemedicine also promises to decrease health care costs by enabling better overall health care access—specifically for preventative care—by decreasing unnecessary emergency visits and avoiding serious medical complications stemming from a lack of prevention. Furthermore, telemedicine offers promise by linking remote services to wearable technologies, which may then be able to relay reliable health information in real time.

### Cybersecurity

As technology becomes more integrated with health care delivery and precision health, better access to security and privacy is critical. The British Medical Journal reported that in 2015, over 110 million patients in the U.S. had their health data compromised. Most often, hackers breach health information security to steal data for financial gain. With the increased reliance on digital methods for obtaining medical information, cybersecurity remains of paramount importance.

Cybersecurity is a multifaceted enterprise. It involves the use of artificial intelligence to predict threats and the coordination of multiple regulatory bodies like the Federal Trade Commission (FTC) and the FDA. Cybersecurity is important in protecting the integrity of patient information and privacy.

### METREE PROGRAM

Developed by the Duke Center for Applied Genomics & Precision Medicine, the MeTree program provides patient and provider clinical decision support. MeTree software uses personal history information on diet, exercise, smoking habits, family history, and other clinical data to provide online, data-driven health decision support. MeTree will calculate certain risk scores and integrate them with clinical practice. Patients can then work with their doctors to mitigate their risk of developing predicted diseases.

### Cloud Technology

Several companies, including Google, Microsoft, Amazon, IBM, and other tech giants participated in the Blue Button 2.0 Developer Conference in August 2018. At the conference, new commitments were proposed to ease the adoption and integration of technologies that facilitate health care interoperability. As data becomes more available, tech companies seek to enable interoperability of those data through the cloud and artificial intelligence computing. Interoperability would increase patient access to their data and to health care services across different systems. Removing barriers to interoperability through cloud integration would improve quality of care and contribute to lower health care costs.

### TECHNOLOGY AND DATA

Using data from technologies integrated with everyday life like Fitbit or Sworkit to track health trends over time, clinicians can deliver better individualized care and understand how disease propagates based on personalized factors.

## U.S. POLICY IMPACTS ON PRECISION HEALTH

Health care policy in the U.S. is governed and regulated by a patchwork of institutions and Congressional acts, each with its own prerogative. The Department of Health and Human Services (HHS) in the U.S. oversees the health of the nation. Housed within HHS are a number of operating divisions (e.g., CMS, FDA, NIH) and offices (e.g., Office of Global Affairs, Office of Inspector General, Office of the Assistant Secretary for Health). Congress proposes and creates acts, which are then enacted as laws to be governed and implemented by the federal government or beneficiaries. The Centers for Medicare and Medicaid Services, for example, broadly oversee most U.S. health care system payment and reimbursement regulations and ensure patient privacy compliance (i.e., the Health Insurance Portability and Accountability Act). The FDA, on the other hand, is in charge of regulating medical products, drugs, and devices. Certain government entities are responsible for health care quality or ensuring that evidence-based preventative resources are covered by insurance. Overall, the federal government holds organizations or providers accountable to safety and quality standards. What follows are several acts, initiatives, and case studies directly related to precision health.

## Congressional Regulation and Data Privacy

### HEALTH INSURANCE PORTABILITY AND ACCOUNTABILITY ACT (HIPAA)

HIPAA was developed to allow consumers to move between health insurance providers and combat insurance and health care fraud. Most relevant to precision health, HIPAA mandates the security of protected health information, ensuring that it remains confidential. Especially relevant in the era of electronic health records (EHR), HIPAA requires that providers and organizations that handle health information must take steps to ensure that the data remain secure and confidential. HIPAA applies to all types of protected health information, including those delivered on paper, orally, electronically, or through other means. Only the minimum health information necessary to conduct business can be used or shared.

HIPAA applies to specific types of information but is more applicable to entities in possession of data. For example, information shared with a physician or pharmacist is protected under HIPAA. However, information shared on social media sites or collected by a supermarket's frequent shopper card are not under the same obligations. The information related to purchases may be shared with some third party, like a drug manufacturer.

The debate surrounding ownership of health data and how protected it may be under HIPAA is complicated and fluid. Under current HIPAA regulations, a patient has an exceptionally limited voice in what a protected entity may do with personal data and how they may be disbursed. In a field dependent on patient data, it is important to consider where HIPAA laws are applicable and how each entity in possession of health data should act.

### TECHNOLOGY AND DATA

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### GENETIC INFORMATION NONDISCRIMINATION ACT (GINA)

In 2008, Congress enacted GINA, which prohibits employers and health insurance companies from discriminating based on genetic information. This law came after genetic sequencing became more widely available. Congress, aware of the role that genetic profiling may play in enabling better health for the population but fearful of the misuse of that same information, ensured that this information would not be used to discriminate. Furthermore, Congress aimed to avoid stigmatization or discrimination based on genetic disorders, which are more prevalent in certain racial or ethnic groups or genders.

### FEDERAL FOOD, DRUG, AND COSMETIC ACT (FD&C)

Originally enacted by Congress in 1938, FD&C gives the Food and Drug Administration the right to oversee the safety of food, drugs, and cosmetics. Amended many times since its inception, FD&C also regulates tools used for the purpose of medical diagnostics. Section 201(h) of the act defines a medical device as an agent "intended for use in the diagnosis of disease of other conditions, or in the cure, mitigation, treatment, or prevention of disease, in man or other animals[.]"

Therefore, any new product that informs the diagnosis or treatment of some disease or medical issue must be regulated by the FDA and must undergo stringent screening criteria before market release. Products that do not directly inform diagnosis or treatment may in some cases be exempt from FDA oversight.

Any new product that informs the diagnosis or treatment of some disease or medical issue must be regulated by the FDA and must undergo stringent screening criteria before market release.

### 21ST CENTURY CURES ACT (CURES ACT)

The 21st Century Cures Act, passed in December 2016, provides both a new framework for streamlining FDA drug and device review and allocates $6.3 billion in research funding. The act addresses three areas of scientific innovation: research and drug development, behavioral health, and health care access.

*Research and Development*

Seeking to spur innovation, the Cures Act enables the NIH to fund pioneering research. Through the act, the NIH is able to provide flexible contracts (which support high-risk, high-reward research), deliver new research prize authority, and expand translational work.

As part of this act, certain medical software that provides little in the way of clinical decision support and serves to encourage a healthy lifestyle, store data, and support administrative functions is no longer considered or regulated as a medical device. Essentially, if a device—like a digital health tracker—collects information but is not used for diagnostic or therapeutic purposes, then that device would not be regulated as a medical device. While the lack of regulation enables more freedom for developers, it may also contribute to increased production of unregulated and quasi-medical device development.

Two of the options for expedited product development and approval include the Regenerative Medicine Advanced Therapy Designation (RMAT) and the Breakthrough Devices Program. Drugs may be eligible for RMAT designation, and subsequent expedited revision, if they will address serious or life-threatening conditions, if preliminary clinical trials suggest that the drug will address unmet needs, and if it is a regenerative medicine therapy (e.g., cell therapy, human cell or tissue product, or a therapeutic tissue engineering product). Breakthrough devices address the aforementioned medical devices that address unmet medical needs for serious or life-threatening conditions.

The Cures Act additionally provides funding for states to fight the opioid epidemic through prescription monitoring programs and increased access to addiction and overdose treatment. Additional funding is also provided to the NIH *All of Us* project, which seeks to understand the impact of disparities on health, among other things.

*Health Care Access and Improvement*

The Cures Act requires HHS to improve the interoperability of health information and data sharing. As a result, HHS aims to advance the quality of health care and is taking steps to facilitate patients' access to their health information. In addressing privacy issues, the act facilitates ease of data access. Protected health information used for research may be used for health care operations, researchers may remotely access health information, and individuals may authorize the use of their health information for future research. Additionally, strong civil penalties are levied against parties that block information sharing between health data systems.

The act also made several changes to Medicare's reimbursement policy. The site-neutral Medicare reimbursement policy maintains that CMS shall pay off-campus providers based on Medicare's Payment Fee Schedule rather than the outpatient organization's payment system, thus lowering prices. Under the previous system, hospitals were penalized if Medicare patients had excessively high readmission rates—something hospitals deemed unfair and that unjustly penalized hospitals catering to higher-risk individuals. Thus, under the Cures Act, HHS groups hospitals together based the similarity of the patient populations to enable fairer comparisons. Hospitals are also financially incentivized to bill for shorter inpatient stays (which the act addressed by creating new surgical billing codes to dissuade the practice of prematurely releasing patients), give patients proper treatment regardless of length of stay, and stop patients from being miscoded in the health system.

*Behavioral Health*

The Cures Act enforces mental health coverage as mandatory for insurance companies and provides funds to improve mental health resources pertaining to suicide prevention, de-escalation training for law enforcement, and grants to provide community mental health resources. It also seeks to reform mental health treatment, HIPAA regulations pertaining to mental health, and relations with law enforcement.

## FDA Policy

### DIGITAL HEALTH INNOVATION ACTION PLAN

The FDA's Digital Health Innovation Action Plan seeks to ensure that patients and other health care consumers have timely access to high-quality, safe, and effective digital health products. The plan elucidated and streamlined the Cures Act legislation, launched the pre-certification for software program, and established the FDA's expertise in digital health.

The plan established a dedicated Digital Health Unit, which seeks to implement new regulatory models in line with the International Medical Device Regulators Forum policies.

### MEDICAL INNOVATION ACCESS PLAN—SOFTWARE PRECERTIFICATION (PRE-CERT) PILOT PROGRAM

As part of the FDA's new Software Pre-Cert Pilot Program, companies can become pre-certified for approval. As a part of the program, pre-certified companies submit less information than is currently necessary to market new digital health innovations. Some companies may be able to launch a new innovation immediately, without review, and collect post-market data. The plan reduces the time and cost of market entry for streamlined software developers.

### DETERMINATION FOR DIRECT-TO-CONSUMER (DTC) GENOMIC TESTING

As companies like 23andMe began providing medically-relevant information to consumers based on genetic testing, the FDA outlined specific guidelines for the information that can be shared with consumers. DTC genomic testing products are required to state that no diagnostic or treatment information will be provided. These efforts seek to limit the number of false positives and false negatives, which may result in unnecessary stress, financial burden, and otherwise unacceptable changes in lifestyle that may be deleterious to a person's health and financially costly.

### RAPID GENETIC TESTING

At the Rady Children's Hospital in San Diego, researchers at the Kingsmore Lab have been investigating rapid genetic testing for time-sensitive treatment. By using genetic testing to diagnose and treat patients, they seek to further validate precision medicine and reduce the cost of genetic testing. Currently, no insurers cover rapid genetic testing, making it woefully unaffordable for many. However, genetic testing has significant value in precision health by linking care to a person's individual genome. Such testing may eventually reduce the cost of care and cut down on the development of chronic diseases.

### FALSE POSITIVES AND NEGATIVES

**False Positive:** Falsely receiving a *positive* diagnosis for a specific disease when one *does not* actually have that disease or illness.

**False Negative:** Falsely receiving a *negative* diagnosis for a specific disease when one *does* have that disease or illness.

## Other Initiatives

### MYHEALTHEDATA

MyHealthEData was launched in March 2018 by a presidential executive order. The initiative seeks to increase the utility of patient data in improving health and care delivery. It aims to help consumers manage their health by enabling them to share health data with third-party online services and apps. While revolutionary in the field of precision health and medicine, there are concerns about the inappropriate use of health information by those third-party users, specifically for targeted marketing or discriminatory practices.

# BARRIERS TO PRECISION HEALTH

Through interviews with key opinion leaders, systems-based analyses, and a funder's summit, the Milken Institute Center for Strategic Philanthropy has identified three barriers to using precision health ideas and technologies, along with key funding opportunities to address each barrier. The three greatest barriers include:

1. Insufficient scientific evidence

2. Insufficient data sharing between relevant health partners

3. Lack of field-wide coordination

We outline potential solutions addressing each problem to move the field of precision health forward and into the mainstream.

## BARRIER 1: INSUFFICIENT SCIENTIFIC EVIDENCE

The foundational structure of precision health is that it is evidence-based—or that the recommendations and procedures are backed by significant supporting data. Precision health and medicine place the emphasis on the individual (as opposed to an average patient), coupling best evidence with practices and decisions about what care to give each distinct patient. Evidence helps create objectivity—it mitigates conflicts of interest and seeks to provide the most tailored, individualized care. As such, there must be more evidence in several informative areas to further substantiate precision health.

Precision health is an emerging field with a growing basis of evidence. However, many stakeholders cite inadequate scientific evidence as a major barrier preventing a concerted shift toward a more proactive health care system. Much of the science to date has relied on small, homogenous populations, involved periodic measurements, and remained focused on disease. Organizations like the NIH have made efforts to shift toward larger, more diverse

### FDA'S MYSTUDIES APPLICATION

The FDA recently published an open-source computer code and roadmap enabling researchers to use and customize the newly-created MyStudies app. Researchers can use the app to collect real data from patients. The data will link to patient electronic health records (EHR) and will support clinical trials, pragmatic trials, observational studies, and registries. The app is the first of its kind and signifies ongoing relationships between the FDA ad private sector partners.

populations through their *All of Us* program; however, more targeted research is still needed. Insufficient scientific evidence can be further broken into three main problems:

1. Quantifying health and defining the transition from healthy to sick
2. Understanding the contribution of genes, environment, and lifestyle to health
3. The value of health economics

### Problem: There Is a Lack of Objective Measures for and Quantification of Health and No Unifying Definition of the Transition from Healthy to Sick

Given its complexity, health is inherently difficult to measure. And if it is difficult to measure, it is difficult to compare. Scientists must be able to effectively measure health using standardized metrics not only between persons but also within each person. Health can be different from person to person, but to provide individualized care, clinicians must be able to compare a person's health to a starting baseline.

The current dogma of reactive care does not easily lend itself to measuring health over time or comparing a person's health to an individualized baseline. Objectively measuring and quantifying health will lend more utility to digital health trackers and clinicians. A streamlined and standardized set of metrics can be developed through better data sharing. Providers could then make better use of digital information and make more informed decisions.

Using metrics that both outline and define health, clinicians and patients can garner a better sense of what defines sick and what defines healthy. Given proper analytical tools, patients can better advocate for their own health and enhance the mission of precision health. Understanding this issue will require a two-part approach: first, the continued development of individual health monitoring devices that can be validated against FDA-approved devices, and second, the development of new devices to collect data points

outside of clinical care visits.

### Opportunity: Promote the Development and Standardization of Precision Health Tools

Promote the development and standardization of precision health tools to quantify health and understand the value of multiple lifestyle and environmental factors to health. Such tools could measure physical activity and blood pressure for daily functioning, diet and sleep habits for lifestyle factors, and water and air quality for environmental factors.

#### WHY

Current market tools do not focus on prevention, are not standardized, are difficult to compare between each other and to a person's individual baseline, and are not adequate for clinician standards.

#### HOW

- Create a nonprofit venture fund to support the development of tools for continuous collection and monitoring of environmental and lifestyle data.
- Include precision health tools in current ongoing longitudinal research studies to both standardize and validate their effectiveness.
- Partner with nonprofit organizations to standardize technology and ensure that tools are safe to use in the clinical setting. This standardization ensures that all data collected by precision health tools are streamlined, and that there is no doubt to their validity.

#### WHY

Different tools exist to measure similar health variables; however, it is unclear to what extent some may be more useful or cost effective than others. Developing rigorous standards will lead to a better understanding of the meaning behind the collected data and enable better preemptive clinician interventions.

**HOW**

- Support comparative effectiveness trials of different types of non-invasive health monitoring and measurement tools.
- Develop standardized practices among device manufacturers.
- Coordinate a nonprofit effort to develop a precision health tool seal of approval, signifying that a new tool passes industry standards for clinical practice after FDA approval.

**WHY**

Devices that collect patient health information are not regulated by the FDA; however, those that allow the use of the information for clinical and diagnostic purposes must be regulated.

### Problem: The Full Contribution of Genes, Environment, and Lifestyle to Health Is Not Fully Understood

Health is intricately connected and multifaceted. In order to accurately quantify and qualify health, practitioners must consider the impact of lifestyle, environmental, and genetic factors in tandem. Research suggests that each of these factors are important in delivering better precision health care; however, it is still unknown to what extent. Should researchers explore the influence of each of these factors, precision health may yield more tangible health and preventative benefits. Additionally, by developing objective measures for each contributing factor, clinicians may deliver more individualized health care and thus lower the cost of delivering health care.

### Opportunity: Support the Development of and Continued Use of Medically-Validated Measures to Record Daily Functioning, Lifestyle, and Environmental Factors, Enabling the Continuous Quantification of Health

Measures could be captured through assays, biomarkers, or related methods.

**WHY**

There are currently a number of products that assess variations of these health factors; however, they are not standardized, nor do they collect all necessary information. Validated assessment metrics would provide more clarity to users, capture useful human data, improve compatibility within medical systems, facilitate disease tracking, and prevent the development of downstream consequences like chronic diseases.

**HOW**

- Develop new products to gather data for use in clinical practice—targeting providers instead of focusing only on consumers—to ensure that data quality is up to rigorous medical standards. For example, companies could make the data from Fitbit, Sworkit, or Apple Watch more accessible to providers for clinical discussions.
- Consider current FDA medical device standards for new devices to ensure that they may be used in a medical context.
- Fund pathways to support the validation of precision health tools to be used in the clinic.
- Expand upon data and findings from large national studies, like the NIH *All of Us* program, through partnerships to collect additional data types or test new tools.

### Problem: Health Economics and Cost Are Not Factored into New Intervention Development or Coverage

For precision health to be effective, there must be some financial benefit for payers. Payers, like CMS and private insurers, must see that precision health and precision medicine offer much in the way of value-based care. Payers must be able to see the return on investment in order to be incentivized to invest in precision health and medicine.

### Opportunity: Explore Lifestyle Factor Interactions and the Cost Effectiveness of Measurements in an Existing Longitudinal Cohort

**WHY**

Examining these factors within an existing trial is more feasible than creating a new one around these parameters and may also facilitate long-term data collection.

## TACKLING REIMBURSEMENT

*Centers for Medicare and Medicaid Services Reimbursement*

Centers for Medicare and Medicaid Services (CMS) is one of the largest payers in the U.S., making it a major leader in reimbursement. The actions CMS takes with reimbursements influences other major health players. Currently, Medicare states that it shall not pay for any service or item not reasonable and necessary for the diagnosis, treatment, prevention, or early detection of some issue, lending legitimacy to the reimbursed intervention. If new precision health initiatives are preferentially reimbursed, there may be more incentives for new innovations, collaboration, and success.

CMS has recently announced that it will reimburse for next-generation genomic sequencing tests and digital therapeutics for diabetes prevention. Primarily as it pertains to cancer care, CMS will reimburse genomic sequencing tests to make more informed treatment decisions and link patients to clinical trials and FDA-approved treatments. **While CMS is beginning to reimburse initiatives like genomic testing and telehealth, it still has a long way to go in linking precision health to value-based care.**

### VALUE-BASED CARE

Value-based care is a reimbursement model that seeks to provide better quality of care. Under the system, providers are no longer reimbursed for each service they provide (fee-for-service), but rather based on the quality of care they provide. Value-based care will provide quality care over quantity of care.

*Cost-Effectiveness Analysis*

Cost-effectiveness analysis informs policymakers and decision-makers about the potential return on investment or cost of medical interventions. In the U.S., cost effectiveness as a decision-making tool for medical treatments is often opposed for ethical, legal, and political reasons. Medicare and Medicaid do not consider cost effectiveness in deciding whether or not to reimburse a new intervention. However, **consideration of cost effectiveness may be useful in bolstering the financial utility of preventative care and should thus be considered.**

**HOW**

The Human Project, run by New York University, seeks to link human biology, behavior, and the environment through interdisciplinary data analytics focusing on 10,000 individuals living in New York City. Expansion of this study, and its myriad measures, to a population outside of New York City would provide an immensely useful data set. The data set would provide an understanding of unique health profiles of both urban and non-urban settings.

**RELEVANT STAKEHOLDERS**

Centers for Medicare and Medicaid Services, Congressional Budget Office, digital health corporations, American Medical Association, public and private payers, health care providers, and academic institutions

- Supporting further health economics research in order to decipher the long-term economic impact of using precision health technologies is vital.

## BARRIER 2: INSUFFICIENT DATA SHARING BETWEEN RELEVANT HEALTH PARTNERS

There is little incentive for private entities to share their health information. In fact, there is a burgeoning business of monetizing health data. In many scenarios, health information is proprietary to the company and serves as the company's main commodity in its business model. While some data repositories exist (e.g., the Influenza Research Database, FlyBase, ChEMBL, and the NOMAD Repository, among others), they are generally isolated within their specific scientific field, like organism-focused research, chemistry, or physics. Broader repositories are needed, as is continued collaboration among research entities. Three key data-sharing problems exist:

1. Data collaboration
2. The need for large data sets and technology infrastructure for cooperation
3. Data privacy

### Problem: Lack of Data Collaboration and Consolidation

Without collaboration, innovative breakthroughs using large stockpiles of data may be impossible or may not present until much further in the future. Effective precision health requires a significant amount of data on multiple lifestyle, environmental, genetic, and economic factors; much of these data will be acquired and consolidated through better cooperation and data sharing.

### Opportunity: Provide Funds Specifically for the Purpose of Gathering, Storing, Analyzing, and Sharing Data

**WHY**

Data collection may foster collaboration and increased analysis capabilities among scientists.

### Problem: Missing Large, Cumulative Data Sets and Tech Infrastructure for Cooperation

There is little to no infrastructure in place to facilitate health information data sharing. Those that are developed must also be HIPAA-compliant. Tech giants have pledged their support to develop a cloud-based interface for sharing information between stakeholders and patients. The initiative will likely be a broader and more integrated version of Apple's health care program, seeking to unite patients and physicians by facilitating information sharing. Although a step in the right direction, there will continue to be no infrastructure to properly foster cooperation without expansion and adequate support.

Additionally, there is a vast amount of data in EHRs and biobanks that are unavailable to researchers. These data, should they be de-identified of all information protected under HIPAA and become open access, would enable investigators to discover the key metrics to quantify health as well as deduce accurate disease risk factors. The information that may yield these discoveries is, as of yet, not shared or compiled.

### Opportunity: Create Specialized Data Platforms to Facilitate Data Sharing and Expand Existing Platforms and Policies

These platforms (e.g., cloud-based data sharing, the NIH Genomic Data Sharing Policy, platforms similar to Vanderbilt's secondary use data infrastructure, and Data Share from the National Institute on Drug Abuse) will enable data sharing between specific databases or facilitate data combination among organizations without any party giving up ownership or control of the data. The nonprofit Vivli is in the process of launching a platform to facilitate this very process for both novel and validation research discoveries.

#### WHY

Using new technology for the purpose of open access data sharing encourages multi-stakeholder collaboration and analyses. These analyses can streamline and increase the success of precision health. Data sharing expansion might increase interoperability of EHR and enable researchers to extract information from open-text sections of records. Furthermore, expansion may link an individual's data across various platforms helping researchers and clinicians to garner a better overall picture of health.

#### HOW

- Partner with and fund groups that are doing work related to the development of or expansion of data collaboration efforts.
- Expand existing data collection efforts, similar to the NIH *All of Us* program, which seeks to increase the representativeness of data through large-scale population recruitment and purposeful inclusion of otherwise underrepresented groups in medicine.
- Facilitate partnerships among academic institutions and health systems to combine data sets, keep data open to access, and create larger, multidisciplinary data sets. A successful example includes the Sanford Data Collaborative, which has partnered with academic and business institutions to advance research and provide health delivery insight using a rich, previously-untapped database.

### Problem: Data Must Remain Private and Protected

Under protective legislation like HIPAA and GINA, patients have a right to privacy. They have a right to decide who will have access to their medical information. However, there are new questions about rights to privacy and which devices or innovations will be considered HIPAA-protected. As such, not all information will remain private. It is difficult to find out which devices will keep this information private and which will not.

Privacy is important, especially with lax regulation of social media and non-medical devices, and with constant attacks on otherwise secure data. To be successful, new innovations that foster better precision health and medicine must navigate these difficult regulations and threats.

Privacy is equally relevant in maintaining the security of non-medical sensitive information. In 2017, Fitbit posted a heat map online, which outlined use of their products throughout the world. In doing so, Fitbit inadvertently highlighted user data of U.S. and allied military personnel in military bases and otherwise potentially sensitive areas. The case study informs the call for privacy and protection of user data, suggesting that the regulatory landscape may change in the future.

### Opportunity: Leverage Blockchain-Based Technologies

Leverage blockchain-based technologies to facilitate data sharing and protect patient information.

#### WHY

Patient information is a frequent target for hackers and is highly protected under U.S. legislation. Increased privacy and protections might not only prevent against theft and fraud but also incentivize data sharing and foster better health. Blockchain is a decentralized database of sorts that stores information at many isolated points, meaning that if a hacker attempts to steal information, he or she can easily be identified. Additionally, it is exceedingly difficult to steal

a complete file or data series using blockchain, further securing individual information.

### BARRIER 3: FIELD-WIDE COORDINATION

Precision health and medicine are still very nascent in the field of health. New players enter the domain regularly, but without a supportive infrastructure, direct collaboration, and data sharing, little progress can be made. Furthermore, there is no coordinated effort to sustain advocacy, coordinate research, or engage stakeholders. A coordinating organization is needed to foster precision health and ensure that people live healthier, more holistic lives. There are four key gaps:

1. Education
2. Advocacy
3. Research coordination efforts
4. Patient input

**Power Opportunity: Overall, we recommend the development of an organization focused exclusively on precision health, which would be transformative by bringing together key stakeholders to guide the field.**

#### WHY

A precision health organization could tackle the primary coordination gaps outlined above and enable a systemic transition from reactive health care to proactive health care. However, more targeted efforts within established organizations could be hugely impactful in the absence of such an organization. A precision health-focused organization would have a major impact by facilitating the four following solutions. The organization could address all of the following problems and act as a vehicle to deliver their corresponding solutions.

### Problem: Lack of Patient and Provider Education on Precision Health and Preventative Care

To properly integrate precision health within the U.S. health care system, patients and providers must understand the value of precision health. Given the nature of precision health and the goal of keeping people healthy rather than reacting to an illness, patient education will need to be broader than the current disease-focused efforts. Public education should focus on the value of staying healthy and understanding how everyday choices affect life-long health. Additionally, the role of the physician will need to adapt toward a proactive model of health rather than a reactive model of disease. To support this role shift, physicians will need educational materials early and often and will need to participate in the conversation. This new model can only be developed if the physician and the patient become partners in health, facilitated through better communication, data sharing, and new innovative precision health technologies.

### Opportunity: Develop Educational Materials and Related Physician Outreach Programs

#### WHY

Such an educational push would foster both clinician and patient awareness of precision health.

#### HOW

- Create educational materials for the public informing them how they might navigate existing and new precision health resources. A coordinating organization might emulate what AARP's Global Council on Brain Health has done for brain health with regard to patient education.
- Create educational materials, including interactive learning sessions for clinicians. Additionally, foster better precision health integration with medical school curriculums, ensuring that the next generation of clinicians will be pioneers in the field.

# BARRIERS TO PRECISION HEALTH

### Problem: Lack of Advocacy for Precision Health and Preventative Care

Policymakers may be unaware of the value of precision health in addition to the difficulty associated with demonstrating its benefits. Benefits are more obvious for reactive medical solutions than for preventative ones, thus there must be a greater charge for funding and awareness of precision health. The U.S. government is the single greatest source of public research funding in a majority of biomedical and other health-focused fields. It provides on average 80 percent of all funding, with international governments and private philanthropists funding the remaining 20 percent. Therefore, a key step in ensuring sustainable funding to this new field is to advocate for it to both political decision-makers and federal agencies.

Given that the federal government is a key decision-maker in health care policies and infrastructure, many health organizations have developed advocacy-focused programs.

### Opportunity: Bring Together the Many Disease-Focused and Patient Advocacy Organizations

Potential organizations to consider for this collective include those with extensive lobbying influence, those that have a largely active membership, and those that represent the health needs of relevant patient groups. Potential organizations include, but are not limited to: American Medical Association, American Hospital Association, National Institutes of Health, Centers for Disease Control and Prevention, Food and Drug Administration, American Heart Association, and Patient-Centered Outcomes Research Institute.

**WHY**

United advocacy groups could encourage a more precision health-focused approach to medicine and change the paradigm of the medical model.

**HOW**

- Additional precision health advocacy may arise from a dedicated precision health center for excellence, which could advocate for an increase in national funding for precision health and provide input into the development of new policies affecting the field of precision health.
- Create educational materials, including interactive learning sessions for clinicians. Additionally, foster better precision health integration with medical school curriculums, ensuring that the next generation of clinicians will be pioneers in the field.

### Problem: Research Efforts Are Not Coordinated

There are a growing number of precision health research projects, many of which have ambitious goals and will collect vast amounts of data. However, these efforts are predominantly independent, not coordinated among institutions, and occurring at disparate locations. A coordinating entity would be useful in guiding data collection, improving and streamlining research methodologies, and ultimately improving health outcomes.

### Opportunity: Fund Efforts That Coordinate Research

Fund efforts that coordinate research, such as research conferences or new research ideas that tack onto existing longitudinal projects.

**WHY**

Coordinated research efforts could raise awareness of precision health and facilitate a better precision health implementation into the medical model.

### Problem: Lack of Patient Input

A key component of precision health is the patient voice. The public's voice is a powerful tool to propel or incentivize the development of new products/services. Not only does it incentivize development, but it also aids adoption. The FDA has a specific intake process to hear directly from patients; these interactions are typically facilitated by health-focused nonprofit organizations. These conversations lead

to decisions that take patient perspectives into account, translating to better outcomes; however, they are not ubiquitously adopted through all new medical and non-medical innovations.

### Opportunity: Create a Forum for Public and Patient Engagement and Fund Efforts to Gather Patient Input

The forum will focus on setting the direction of precision health.

#### WHY

The very purpose of precision health and medicine is to ensure that new products and initiatives improve health and meet patient needs. Providing an open dialogue for patients to share allows this to be more effective.

# CURRENT KEY INITIATIVES

As referenced throughout the Giving Smarter Guide, there are incredible research programs working to build knowledge in precision medicine and precision health. Below, we have outlined seven important and representative programs within the field.

## NIH *ALL OF US* RESEARCH PROGRAM

The NIH *All of Us* research program is collecting longitudinal biological, lifestyle, and environmental data from one million participants with diverse geographic, ethnic, and socioeconomic backgrounds over 4-10 years. The recruited cohort will have their health monitored both in study site clinics and remotely through digital mobile devices. This study should result in novel prevention and screening strategies, earlier and more precise diagnoses, and improved understanding of factors contributing to individual health and disease. The NIH will house the data and patient specimens in a centralized repository that will allow widespread access for researchers and clinicians.

The program's underlying platform promises to provide broadly accessible volunteers who have agreed to share their data, provide a biospecimen, and to be contacted in the future. The number of variables and multiple time points collected on a million-person cohort will increase the statistical power of tests searching for biological, lifestyle, and environmental markers of health and disease.

## PROJECT BASELINE

Project Baseline is a longitudinal study led by Stanford University, Duke University, Verily, and Google to collect health information from 100,000 participants. The project will monitor changes in the health status of participants over time to discover new insights on the transition from a healthy to a diseased state. The data will enable

researchers to better understand how factors such as lifestyle, genomics, and the environment both interact with and influence health.

The goal of the study is to understand the baseline of health or a healthy individual's expected normal phenotype. Another major objective of the study is to recruit unique individuals from diverse populations so that the results from the study are generalizable to the larger population. Researchers will use this baseline data to generate algorithms for improved prediction or prevention of disease onset.

### THE HUMAN PROJECT

The Human Project was initiated by the Kavli Foundation in partnership with New York University (NYU). The project seeks to elucidate the connections among human biology, behavior, and the environment through interdisciplinary data analytics. The organization plans to identify 100 "micro-neighborhoods" across all five New York boroughs to mirror the population of New York City. Through a holistic approach, it seeks to identify factors associated with health and wellness.

In short, The Human Project will study how behavior, biology, and the environment interact to impact health. This study will include lifestyle factors such as public transit use, education, gentrification, and affordable housing, thereby furthering current research on environmental influences of health.

There would be significant leaps in the understanding of health in the context of both rural and urban areas, should an initiative be expanded beyond New York City.

### SPRINGBOARD HEALTHY SCRANTON

The Geisinger Health System is partnering with health care providers and community groups to improve the health of Scranton, PA

residents. By sequencing their genomes, the project hopes to learn how genomic background variations affect health. Scranton was chosen as a representative U.S. city to study community-wide precision health interventions. Researchers have secured consent to sequence approximately 10 percent of the projected enrollment.

The program will focus on preventative care, behavioral health, and economic growth to create "healthier families, stronger neighborhoods, and resilient communities." The initiative's efforts will focus on developing a large genomic database to design prevention strategies. The program also plans to work with industry partners to further use the information they collect in drug-discovery efforts to guide therapeutic development.

### VIVLI

Launched in July 2018, Vivli is a nonprofit organization that developed a platform powered by Microsoft to facilitate global data sharing. Vivli was originally funded and built through grants from the Doris Duke Charitable Foundation, the Laura and John Arnold Foundation, and the Leona M. and Harry B. Helmsley Charitable Trust. The platform enables researchers to share their data and access the data of other scientists on a number of disease topics from all around the world. The data can be used to validate previous studies or combined with other data to answer novel research questions. This new platform is an innovative step toward better data sharing and toward fostering a broader, collaborative approach to research and health care.

### MOUNT SINAI'S LAB100

Lab100 at Mount Sinai is a hybrid clinic-research lab with plans to leverage science, technology, and data analytics to redefine health and health care delivery. Lab100 proposes bridging the gap among patients, providers, researchers, and innovators to facilitate personalized health care. It plans to create a web-based software to direct a user-centric health care experience ranging from online patient enrollment to in-person assessments with the help of medical

devices. These devices are centrally linked and produce real-time test and analytics results. In this setting, the patient and clinical team will collaboratively identify actionable insights based on personalized data and then collaboratively decide upon an intervention.

### CALIFORNIA INITIATIVE TO ADVANCE PRECISION MEDICINE

The California Initiative to Advance Precision Medicine is a partnership between the state of California, UC Health, the University of Southern California, and other public and private entities. The partnership seeks to advance the field of precision medicine by integrating medical records with genetic, environmental, socioeconomic, and digital health data. By improving the understanding of disease through their integration, more precise therapies can be developed. The initiative has two main components: funding demonstration projects in precision medicine and developing an asset inventory. With California Governor Jerry Brown's support, the initiative has funded eight demonstration projects on various topics in precision medicine since its inception in 2015.

Another important goal of the California Initiative to Advance Precision Medicine is to develop a centralized inventory of public and private precision medicine assets, such as research projects, clinical studies, or databases to coordinate the use of these resources in California. Researchers and interested individuals may use this inventory to establish new collaborations or opportunities. In 2018, Governor Brown approved another $30 million start-up fund to create the California Institute to Advance Precision Health, which will support the initiative's efforts, as well as incorporate the best practices gleaned from the demonstration projects into the health care system. Plans for this institute are still under development through collaboration with the public sector and private industry.

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We graciously thank the scientists, nonprofit leaders, and advisors who participated in the Milken Institute Next Generation Prevention retreat on October 23, 2017. The informative discussions before, during, and after the retreat were crucial to identifying the key unmet needs and ideal philanthropic opportunities to benefit patients and advance precision health research.

**Edward Abrahams, Ph.D.**
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*National Institutes of Health*

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*Duke Center for Applied Genomics and Precision Medicine*

**Robert Green, M.D., M.P.H.**
*Harvard Medical School*

**Frank Hu, M.D., Ph.D., M.P.H.**
*Harvard T.H. Chan School of Public Health*

**David Karow, M.D., Ph.D.**
*Human Longevity, Inc.*

**David Ledbetter, Ph.D.**
*Geisinger Health System*

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