FasterCures works across sectors and diseases to accelerate the process by which great advances in science and technology are turned into meaningful medical solutions for patients. Our programs identify what’s working and what isn’t across the research ecosystem, and share that knowledge so that every sector — and every patient — can benefit.
There are 10,000 known diseases and only 500 treatments. We have work to do.

FasterCures is determined to pave a path of meaningful engagement between patients and every sector of the research enterprise through:

**Thought Leadership**
From journal articles in Science Translational Medicine to invitations for keynote speeches around the world, FasterCures’ leadership is relied upon to provide the insight and analysis different sectors need to navigate the medical research process.

**Publications**
FasterCures creates unbiased analytical assessments of some of R&D’s thorniest issues — from intellectual property to alternative financing models and from patient registries to data sharing — providing a roadmap for action.

**Events**
Our annual Partnering for Cures conference, policy briefings, Webinar series, hands-on workshops and targeted meetings bring together leaders from across medical research to educate and spur action.

fastercures.org
Achieving Faster Cures

In 1993, more than two decades after we began support of medical research and shortly after my own cancer diagnosis, I attended a conference at M.D. Anderson Cancer Center. Looking around, I realized there were no researchers from Memorial Sloan Kettering Cancer Center (MSKCC), another leading research institution. When I asked why, I received a quizzical look and was told: “Because they’re the competition.”

Not to patients, I thought.
By the time we hosted our first Scientific Retreat less than a year later, we’d solved this problem. To receive funding from us, investigators had to share their work — even with other institutions. (For those who said their research was too valuable to share before publication, we told them they obviously didn’t need our support; within months, they all came around.) And not only did we invite major research institutions, we also brought in industry. The late Dr. Bill Fair, then chief of urology at MSKCC, told me it was the first time he’d been to a conference with for-profit companies.

On the Retreat’s opening day, the researchers sat with other researchers (usually from the same institution) and industry representatives with each other. For-profit research was viewed as beneath and behind the work being done in the university setting. On the second day, after some of the impressive presentations from biotech and pharma companies, everyone began to mingle. It was clear to all participants that they could accomplish more working together than individually.

A couple of years later, collaborating became even easier when our board member Andy Grove (then chairman and CEO of Intel) helped connect basic, translational and clinical researchers around the world in real-time through the Internet — a practice we take for granted today. Working with someone in a different part of the world became as easy as working with someone down the hall.

All of this happened in the precursor organization to FasterCures, and that collaborative spirit is in our organizational DNA. The Research Acceleration and Innovation Network (TRAIN) was created as the FasterCures hub for sharing best practices across nearly 100 leading disease-specific organizations. The Center for Strategic Philanthropy (created at FasterCures and now its own Milken Institute center) focuses on helping givers maximize their impact by coordinating efforts across many research organizations. Our public policy and education initiatives over the years, including the 2011 Lake Tahoe Retreat and the 2012 Celebration of Science, have helped refocus the nation’s commitment to bioscience. The Consortia-pedia program helps form meaningful collaborations, and our Patients Count initiative puts patients and their families at the center of medical research.

And, of course, this collaborative spirit is in full effect at our annual Partnering for Cures conference, as we welcome nearly 1,000 leaders from across the research ecosystem — medical and scientific investigators, patients, government officials, investors, philanthropists, CEOs from biopharma and medical device companies, insurers, health-care providers and heads of research centers and other nonprofits. These leaders forge partnerships dedicated to a singular goal: reducing death and suffering.

Independently, we can chip away at this challenge. **Together, we’ll solve it.**
Contents

Mike Milken
Achieving Faster Cures

Francis Collins
Sharing Trial Data Is the Right Thing to Do for Science and for Volunteers

Gina Agiostratidou
Collaborating to Evolve

Keith R. Yamamoto
The University of the Future

Nancy Brown
The Power of Patient Engagement in Biomedical Research

Pat White
Collaboration in Diabetes Research Inspires Collaboration in Advocacy

Nora Volkow
How Collaboration Can Change the Way We Understand Addiction

Todd Sherer
Conveners Alone Can’t Change the Ecosystem
Sharing Trial Data Is the Right Thing to Do for Science and for Volunteers

Francis Collins
Director
National Institutes of Health

The broad sharing of research information is not only a core value of NIH but also necessary to improve the growing body of biomedical knowledge.

With all of the exceptional opportunities facing biomedical research today, partnerships and team science are more important than ever. The combined efforts of many different scientific disciplines — and many different sectors of society — are absolutely essential if we are to make the most of the many exciting possibilities that lie before us.

One outstanding example is the Accelerating Medicines Partnership (AMP), a public-private consortium involving the National Institutes of Health (NIH), 10 pharmaceutical companies, the Foundation for the NIH, the Food and Drug Administration (FDA), several patient organizations and other partners from the nonprofit world. The goal of AMP is to transform the current model for drug development by jointly identifying and validating promising biomarkers and drug targets, with the ultimate aim being to provide patients with an increased number of diagnostics and therapeutics in a swifter, more cost-effective manner.

Since its launch in 2014, the AMP consortium has undertaken collaborative projects in three disease areas: Alzheimer’s, type 2 diabetes and the autoimmune disorders of lupus and rheumatoid arthritis. Through this cross-sector endeavor, NIH and its partners are sharing expertise and resources in an integrated governance structure.
that enables the best informed contributions to science from all participants. A critical component of AMP is that all partners have agreed to make the data and analyses publicly accessible.

As another example of a new model for doing research, consider the NIH-led Precision Medicine Initiative® (PMI). PMI seeks to create a new paradigm in which participants are regarded as true partners in research, not merely subjects. More than 1 million volunteers will be invited to sign up to join PMI’s All of Us® Research Program by 2020. Not only will these volunteers agree to share their electronic health records and other data with researchers, but they will also have access to their own data plus a wide range of study results.

PMI’s All of Us® Research Program will intersect in a synergistic way with other fundamental changes in medicine and research to empower Americans to lead healthier lives. Among those changes are new federal regulations and policies aimed at ensuring information about clinical trials is shared widely and rapidly. Clinical trials are crucial for the translation of research advances into new approaches for prevention and treatment of disease.

Volunteers who take part in clinical trials often do so with no assurance of personal benefit, but with the expectation that their involvement will add to a growing body of biomedical knowledge that may help others someday. To uphold this trust, all trial results need to be reported publicly in a timely fashion — and yet we know that has not always happened in the past.

A regulation issued Sept. 16, 2016, by the Department of Health and Human Services, details requirements for registering certain clinical trials and submitting their summary results within one year of completion of data collection to ClinicalTrials.gov, a database managed by NIH’s National Library of Medicine (NLM). The regulation applies to most interventional studies of drug, biological and device products regulated by the FDA. Also, because of our commitment to data sharing, NIH has issued a complementary policy that applies to all NIH-funded trials, including those of behavioral interventions.

ClinicalTrials.gov already contains a vast trove of information, and its value will only continue to grow as more studies and results are added. So, I am delighted that NLM plans to work with leading information technology experts to make ClinicalTrials.gov more user-friendly. This partnership will build on lessons learned from the National Cancer Institute’s recent collaboration, facilitated by the Vice President’s Cancer Moonshot initiative, in which technical experts and Presidential Innovation Fellows are enhancing access to oncology trial information at Cancer.gov.

Rapid, broad sharing of research information is a core value of NIH, as well as an issue that has long been close to my heart. Two decades ago, under the leadership of myself and others, the international Human Genome Project established data-sharing policies for depositing genetic data into public databases within 24 hours. This year, we are setting forth bold initiatives for sharing summary-level results of clinical trials. But we still have work to do. Sharing individual patient-level data from trials with qualified researchers is still a desirable framework for even more expanded data access, and NIH is evaluating various models for making such sharing possible.

The bottom line is that NIH remains firmly committed to engaging patients, empowering researchers and encouraging public-private partnerships. I hope each of you will join us in this effort!
Collaborating to Evolve

Gina Agiostratidou  
T1D Program Director  
Helmsley Charitable Trust

We live in a time when tackling disease is urgent and in high demand. Just as medicine and technology evolve to unprecedented levels of sophistication, so do new, complex diseases that affect growing populations around the world. We need solutions that respond to our ever-changing lives, which means our solutions must be able to evolve. Fortunately, new tools and perspectives are sparking innovation and vital collaboration.

In the past few years, advances in biomedical research have opened pathways that allow us to edit genetic material, use stem cells to treat disease and destroy cancer cells with novel immunotherapies. These advances (and many more) have vastly improved our capacity to tackle tough challenges in medicine. Also, developments in technology have allowed researchers to put patients and public health at the center of many studies, resulting in large data sets that give us the ability to investigate new hypotheses for therapies and cures.

Yet novel interventions aren’t available to all patients, and we are experiencing an information overload. How do we adjust to all of these changes in health care? How do we ensure new tools benefit everyone?

At the Helmsley Charitable Trust, we believe that a collaborative environment is crucial for sharing data, ideas and solutions. Having supported hundreds of millions of dollars in health and medical...
Data-driven innovation allows us to understand difficult diseases and develop better treatments than ever before. But these advances can’t happen in isolation.

research, we’ve learned and practiced some important lessons, including:

• Don’t push collaboration. Allow participants to identify who they trust and want to work with, and ensure goals are aligned among everyone.

• Patience is key. Creating a collaborative environment requires time and flexibility.

• Create a system and a plan. Well-defined governance allows for clear decision-making, and milestones allow you to assess progress and mitigate problems.

• Resources enhance innovation. Ensure funding is sustainable to allow for creative and thoughtful solutions.

• Exit projects responsibly. A clearly designed plan prevents failures and continues to build on the work already done.

• Foster an environment of trust. Last but not least, participants should feel comfortable sharing ideas, communicating and giving constructive criticism.

Data-driven innovation allows us to understand difficult diseases and develop better treatments than ever before. But these advances can’t happen in isolation. Collaboration is essential as data multiply and research funding becomes scarce.

Fortunately, innovators today have created platforms for collecting and analyzing large data sets. When data are actionable, if the results of large analyses provide clues for interventions, we can tangibly improve the lives of people with diseases.

With the rise of social media and the ability to easily share information, the patient’s voice has become more prominent than ever before. Patients’ opinions are essential in the development of interventions. We must create a framework where all patients can be heard and contribute to our efforts.

At Helmsley, we are working on a collaborative health policy initiative that uses the patient’s voice to improve access to therapies and technologies for type 1 diabetes (T1D). This partnership between Helmsley, JDRF and T1D Exchange paves a path toward greater adoption of effective therapies that could transform diabetes care. By bringing in the patient’s and clinician’s perspectives, along with data, we hope to identify roadblocks preventing people with T1D from accessing technologies that can ease the burden of their disease.

Our lifestyles are radically evolving — from how we eat, exercise and work, to how we treat disease. We are moving toward a world where collaboration connects all disciplines and stakeholders, in the hope of creating better, longer, healthier lives.
Scientists are recognizing the advantages of working in collaborative teams, seeing that they might take on bolder, more exciting challenges in such environments. However, team efforts and disruptive science are being inhibited by two major motivational barriers — the federal government funding model and the reward/punishment policies in academia.

Failure in academia is a death knell, often rapid, always torturous. The pressure to maintain continuous funding and a high pace of publications demand that most investigators establish a research focus and stay with it, nose to the grindstone. Obviously, choosing to take a focused path can be productive and rewarding, but doing so under duress of the academic culture can be punishing, where branching out to probe an audacious idea can start a spiral of lost funding, lack of trainee interest, slowed promotions. The result is that universities are gathering points for brilliant, hardworking researchers, working diligently and making progress in isolation, indeed commonly competing among intellectual silos housing basic, clinical and social/behavioral/population research.

At the same time, we are drowning in information. Researchers struggle to keep up with their own fields, so can’t fathom learning in any depth about important questions outside of their scope — questions that they might be able to address if they knew about them. Colleagues in every institution are missing

Encouraging academics to work in teams will help remove the stigma of failure and accelerate scientific progress.

Keith R. Yamamoto
Vice Chancellor for Science Policy and Strategy;
Director, UCSF Precision Medicine;
Vice Dean for Research, School of Medicine;
Professor, Cellular & Molecular Pharmacology,
University of California, San Francisco

The University of the Future
opportunities to team up right across campus to take on new exciting challenges.

What can be done to address this problem? Imagine if we built a knowledge network, a computational brain, capable of aggregating and integrating the mountain of information being generated by a university’s researchers. The network would visualize everything that is happening across departments and disciplines, placing in proximity studies linked by intellectual focus, by technology, by experimental approach — any sort of routine selected by the viewer. Researchers could self-assemble collaborative teams that could define and address from different angles a difficult but exciting problem that none could address individually.

In this University of the Future, every faculty member would participate in multiple teams; established faculty might be involved in about 10. Each team would apply a team-determined blend of disciplinary approaches and technologies to solve its team-defined problem. Because the problems are bold, the ideas may be wrong, the technologies may prove insufficient — the team may fail. But it would be okay — there are other problems being hotly pursued by other teams in each investigator’s portfolio, and some of them will succeed. So, failure would be de-stigmatized, and as in Silicon Valley, “failing fast” would be a plus.

The freedom to succeed would be unleashed by the freedom to fail.

So the University of the Future would be dynamic — fluid teams with constantly changing partners asking bold questions with big potential impact. To get there, academic culture change, always a challenge, would be essential. And the funding system would need an overhaul, identifying and supporting bold ideas proposed by teams, with initial seed grants that could escalate as defined milestones are achieved. Strong, well-justified motivation is the best driver of policy change.

Creating knowledge networks would benefit patients as well. The network would be open, not just for scientists, and the projects would be public. A patient could investigate his or her condition on the network to understand what is being researched.

Indeed, patients could pose research questions to the teams. Building clinical trial networks is good, but we can expand the ways that patients participate in the research enterprise. We can give them a real appreciation for what research is doing for them and an ownership share in the enterprise.

The University of the Future could incentivize self-assembly of collaborative teams that span the full research, health and health-care spectrum, opening opportunities to address vexing scientific and societal issues, many perhaps yet unimagined, that seem otherwise impenetrable.
Voluntary health organizations have a unique role in connecting all the players required to get cures and treatments to patients faster.

I make my staff nervous when I say this, but as voluntary health organizations we should be working toward putting ourselves out of a job. At my organization, The Leukemia & Lymphoma Society (LLS), we are dedicated to funding research, finding cures and ensuring access to treatments for blood cancer patients. If we are aligned around this goal, all of our efforts should go toward ending blood cancers. More simply put: we need to be putting ourselves out of business. And there is no way we could accomplish that, and deliver our mission, without engaging in collaborative efforts.

To look for a model of how this kind of collaboration can work, we simply need to look at the example set by the National Foundation for Infantile Paralysis. In 1953, there were 120,000 new cases of polio diagnosed in the United States. One decade later, there were none. We know that in that decade the Salk vaccine was licensed. But equal in importance to the discovery of the vaccine was the distribution campaign sponsored by the National Foundation for Infantile Paralysis. With all pieces of the system working together, a disease was effectively wiped out.

The National Foundation for Infantile Paralysis is now known as the March of Dimes and has refocused its mission on improving the health of mothers and babies. Its historic work developed the model for what patient advocate groups could be
back then, but also what they can be today. I believe we will see more organizations directly connected with and driving the key components of the work that needs to be done to see their mission succeed. Rather than remaining at arm’s length from research, patient advocacy groups and voluntary health organizations will play more of a direct role. These nonprofits bring a wealth of knowledge about their specific disease area and also have a deep understanding of what patients experience in day-to-day living.

LLS has experienced how this kind of collaboration can work today through a three-way partnership called The Learning Collaborative. With our partners, the National Center for Advancing Translational Sciences and the University of Kansas Cancer Center, we established an environment where each organization contributed its own expertise without concern for who claimed credit. It was through a commitment from the group’s leadership to adhere to a common set of goals and metrics that we found success. This experience continues to be a model for LLS when entering into partnerships. But the best practice of setting common goals and objectives can be applied to any collaborative effort regardless of the disease of interest.

Collaboration is the key to accelerating the rate that new therapies get delivered to patients. These models need to become second nature to all organizations in the biomedical research space. Voluntary health organizations can act as a centerpiece for these partnerships. We can support the researchers developing treatments, dialogue with the Food and Drug Administration on unmet patient needs, educate providers who will be delivering therapies and engage the patients who need supportive services. I am going to keep pushing my organization until I am out of a job. And I hope you will join me in that mission.

120,000

1953 1963

In 1953 there were 120,000 new cases of polio diagnosed in the US; by 1963 there were 0.
Partnerships that Put Patients First

Aisling Burnand
Chief Executive
Association of Medical Research Charities

Medical research partnerships are shifting to engage patients as partners, and charitable organizations are leading this collaborative charge.

Collaboration can make all the difference to patients and their loved ones, and we need to work together to make it happen. I see collaboration as a powerful antidote to the challenges and barriers that face all of us in the pursuit of curing illness. Now more than ever, we need collaboration if we are to tackle the huge challenges of finding new cures. And the challenges we face as regional, national and global communities are significant — dementia in our aging population, the spread of infectious diseases, the rise of antimicrobial resistance, cancer, obesity and diabetes and an uncertain economic future with an impact on health spending to name but a few.

Despite the enormity of the challenges, the outlook for medical research is incredibly positive. We are entering a new era of significant scientific change that will intensify over the next five to ten years. Leaders and experts in the UK who we recently interviewed used phrases like “tsunami,” “cusp of a revolution” and “seismic shifts” to describe developments in medical research.

But at the same time, the scientific challenges are getting more intractable and multi-dimensional, and people are living longer with co-morbidities. The nature of clinical trials will change going forward as real-world data become a reality. Big data will be transformational throughout the health care system and over the longer term will be hugely disruptive. Privacy, security and legal and technological issues,
combined with political and cultural ones may act as a drag on progress, but the case for change in terms of cost savings and outcomes will keep the momentum going.

The change that is coming is secular by nature, not cyclical; things will never be the same again. The question we need to ask ourselves, especially in the charitable sector, is “Are we ready for this change?”

We need to recognize that today’s fragmented research structure is holding back progress. Joint funding, shared risk-taking and “team science” are important going forward. Collaboration means many dimensions and geographies. Equally vital is better understanding patients’ needs and wants and to include them as constituents throughout the research process.

Five years ago, the UK was a very different place. Few charities collaborated outside of academia. Industry was still seen as the bad guy. Independence was our shield. But the storm was beginning. Charities were beginning to think about what impact they were making for the people they serve and questioning whether they could show promise for delivering new treatments and therapies so desperately needed.

In 2014, my organization, the Association of Medical Research Charities, produced a guide for charities wanting to collaborate with industry. It’s called An Essential Partnership — and it really is “what it says on the tin.” We wanted to showcase the ways in which charities could start to partner with others, to think differently and challenge their own ways of working. Filled with almost 40 case studies, we outlined a new framework for working with industry.

We are now seeing the seeds of change with a plethora of new partnerships. In this year alone we have seen several promising partnerships formed.

1. **The Francis Crick Institute** opened (co-funded by Cancer Research UK, Wellcome Trust, government and academia).

2. **Breast Cancer Now** announced a major collaboration with Pfizer to allow charity-funded researchers’ unprecedented access to at least 14 of Pfizer’s existing drugs, supported by around $13 million in funding.

3. **JDRF** announced a new partnership with the company Arecor to shrink the size of insulin pumps to make them less intrusive and lighter for patients.

4. **The Dementia Discovery Fund** — a collaboration among charity, industry and government — is investing in innovative dementia research.

5. An industry-charity partnership will look at the epigenetics of respiratory disease with backing from Asthma UK, the British Lung Foundation, MRC Technology and AstraZeneca.

These partnerships are driven by the need to put patients first — to truly understand the impact of a disease or condition and to focus on what really matters. Patients are now active participants and equal partners. And it’s about time!

**What conclusions can we draw from this shift in the biomedical ecosystem?**

We need more collaboration, not less. If you’re not comfortable with that, learn to be!

None of us has all the answers. We need to talk to each other, share information and co-create. Be bold.

This is not about pharma, or biotech, or charities or academia. This is about people. We are all individuals, and our health needs are changing. We need to develop treatments for the whole person, not the disease.

Charities and foundations wherever they are and whatever their size can be the honest broker throughout this paradigm shift. We can hold people’s feet to the fire for change and represent the needs of those who matter most — the patients themselves.
The perceived drawback to collaboration is simply time. Collaborative efforts take a long time to show value because these groups do not have the kind of absolute control needed to move quickly.

It is important to be patient in this process. Don’t confuse the short-term adoption of the consumer model with the long-term adoption of the collaborative model. It’s not a business; it’s a network.

Part of making collaboration more effective is realizing information must be gathered from hundreds of thousands or millions of people in order to make interesting statements about the population as a whole.

We either need a little data from an enormous amount of people or an enormous amount of data from a small group of people. To accomplish either of these things, we have to change the way you think about collaboration.

Three things need to come together in collaborative models for research:

1. **Team science**

Most people think of team science
Individuals must be welcomed as collaborators — and not just sources and consumers — in data and research.

as taking labs from different institutions that work on the same topic and putting them together to work on a project. However, the members of this network are not always prepared to work together. Just because teams are working on the same topic, doesn’t mean those teams will work well together. These groups need facilitators, like Sage Bionetworks, to teach them the necessary skills to overcome their differences, work effectively together and get out of their way.

Open vs. closed data

There is a huge impact associated with making data open, particularly human data. Human data cannot be open without informed consent. More generally, there are innovation costs to both openness and closure at the right and wrong times. Making data open too early in the research process may limit the creativity of other groups. Scientists in this situation are afraid to take chances and make mistakes, so they follow the models of the front-runners in the research process. This greatly reduces diversity in methods, models and, ultimately, treatments for patients.

Human engagement

We have to treat people who enroll in studies as people. Make sure people are asked what they feel, what they like, what they don’t like. It’s unfortunately not far from subject to mouse. People need to be treated with empathy.

Take a rare disease group for example: If researchers want to get the kind of data that allow complete analysis, they have to become part of the patients’ lives. Patients cannot come in every now and then and give a few vials full of samples and be on their way. Researchers have to be part of their lived experience. Patients will not allow researchers to become deeply involved in their lives if they are being treated like a subject.

This investment is critical because patients can point scientists toward what questions to ask, what elements to measure and where intervention is needed.

The best way to move collaborative efforts forward and reduce costs is through standardized sharing platforms. At Sage Bionetworks, we cannot be successful until communities form outside of us and use our platforms to build their own trials and projects.

This sharing should include more than methods and models. It must also include mistakes. Researchers should not be afraid to reuse, repurpose and learn from each other.

Important data are gathered by surveys and wearables, but also on every individual’s cell phone. More and more sensors are moving into our lives, and they are not moving in because of health. Apple, Amazon and other tech companies already use technology that can monitor a consumer’s heart rate or detect signs of depression based on pauses and sighs in a consumer’s speech.

These kinds of things will be used for health within 10 years. So the question becomes: What’s that system going to look like? What’s our citizenship inside that system? Will it empower new researchers?

Individuals must be welcomed as collaborators — and not just sources and consumers — in data and research. Transparency must be present in data to show people how their data are being used. We must ask ourselves: Will I be a citizen or will I be an oil-well for data?
In the quest to find cures for diseases, one of the primary hurdles we face is the extraordinary complexity of biology. The intricacy and molecular scale of biological systems make it hard to assemble more than a fragmented picture of the body in health and disease. But thanks to recent and stunningly rapid advances in human genetics, that picture is growing clearer, and the potential for medical breakthroughs is rising dramatically.

The 6 billion DNA nucleotides in the genome contain the complete instructions for assembling every protein and cell in our bodies. Changes in just one nucleotide can lead to disease or prevent it, and rarely occurring gene variants are often the most consequential. We need whole-genome data from many people to fully map disease risk.

This is why the payoff from human genetics is taking longer than first expected. Hopes were high that the Human Genome Project would quickly lead to a wave of treatment advances. That didn’t happen, in part because the sequencing of whole genomes remained prohibitively expensive and time consuming.

In recent years, with the advent of ultrahigh-throughput DNA sequencing technology, our ability to read DNA has increased a million fold. As sequencing costs drop and methods for crunching genetic data improve, we now have the tools to map the molecular basis for all diseases.
Success in this great endeavor will require more than scientific prowess. The most productive models for gene discovery have involved collaborations that extend across broad elements of society. One excellent example is deCODE Genetics, the Iceland-based world leader in gene discovery and now a subsidiary of Amgen.

deCODE’s success is based on a combination of great science and the generous participation of Icelanders in its research. On the scientific side, deCODE has developed sophisticated methods for collecting, analyzing, visualizing and storing huge amounts of genetic data. The Icelandic people, in turn, provided a wealth of data to analyze. About 160,000 Icelanders gave deCODE access to their genetic data as well as medical data collected through Iceland’s universal health-care system.

By correlating genetic variations with health data, deCODE has discovered dozens of genes that impact the risk of common diseases. In a recent, highly significant example, Amgen and deCODE announced that a rare mutation in a gene called ASGR1 was linked to a 34 percent decrease in heart disease risk. The discovery points to a new biological pathway that might be modulated to further reduce the risk for heart attacks.

The web of collaborations that supports such major findings extends beyond Iceland. It includes technology companies that make gene sequencers; academic researchers who replicate and extend gene discoveries to more diverse populations; scientific publications that announce discoveries; and companies like Amgen, which invest in genetic research and work to transform genetic insights into medicines.

There are many more disease genes waiting to be discovered. To find them more quickly, we need to augment and extend genetic research collaborations. Based on our current understanding of genetic variation, Amgen estimates that data from 50,000 patients who share a disease may be sufficient to identify every gene with a meaningful impact on that disease. Data from a million patients might explain the biology of diseases that account for most of the suffering and premature death in the world. Many patients would welcome the chance to take part in the search for cures by providing access to their genetic and medical data. Networks could be established to encourage such generosity and ensure that patients’ privacy is protected.

Achieving the potential of human genetics will also require greater collaboration among health-care payers and innovators. We need to ensure our health-care system supports major medical advances, and coverage and payment systems need to be modernized to accommodate personalized medicine.

Unfortunately, there’s a trend toward viewing innovative therapies in terms of their immediate costs. Little value is placed on their offsetting impact on other health-care expenses or on benefits to patients, including longer, healthier, more productive lives. This outlook reflects siloed thinking and insufficient cooperation among all the players that patients are counting on for help. If we’re intent on finding cures, we need to find better ways to share the costs as well as the benefits that medical progress provides.

“Many patients would welcome the chance to take part in the search for cures by providing access to their genetic and medical data.”
The Power of Patient Engagement in Biomedical Research

Nancy Brown
CEO
American Heart Association

It’s incredible how much we can learn if we stop and listen for a few moments. When we open ourselves up to other people’s ideas and allow them to share their stories, we’re given the opportunity to see things in an entirely new way.

The undeniable value of listening is the inspiration for a new partnership between my organization, the American Heart Association (AHA), and the Patient-Centered Outcomes Research Institute, which uses crowdsourcing to give patients a powerful voice in their own care.

Every patient has a story to tell, but far too few are offered a forum to do so. There are innumerable barriers that prevent many patients from saying what’s on their mind. For example, during a typical office visit, there’s seldom enough time for patients to ask questions or have an in-depth discussion with their provider. Also, many patients may feel too uncomfortable or embarrassed to speak frankly, or may think their concerns seem trivial or inconsequential.

Patients aren’t the only ones who encounter these barriers. It’s also important to consider family members and caregivers, who are often intimately involved in the patient’s care experience yet may feel reluctant to speak up if they have any concerns or doubts.

We believe that crowdsourcing offers a wide range of advantages that are conducive to open communication. Patients are free to tell their stories in their own words and can do so.
Every patient has a story to tell, but far too few are offered a forum to do so. There are innumerable barriers that prevent many patients from saying what’s on their mind.

in whatever setting they prefer — including from home. They don’t face time limitations and their confidentiality is protected.

Through this partnership, we’re seeking input on critical gaps in the evidence about which prevention, diagnostic and treatment approaches work best for patients, based on their needs and circumstances. The feedback we receive will be a base for designing comparative clinical effectiveness research studies and will also support our efforts to advance precision cardiovascular medicine, which seeks to refine care for patients based on their particular characteristics.

Thanks to research conducted in the laboratory, inspiring progress has been made in our ability to treat illnesses. But how are we doing when it comes to treating patients? The only ones who can answer this question are patients themselves. That’s why we’re urging them to speak up. We want them to tell us what could have been done better and what might have been overlooked. Likewise, we want to know what worked to perfection and what might have been a pleasant surprise. It could have been a pleasant — or unpleasant — experience in the waiting room.

It could have been a follow-up phone call that they received — or didn’t — receive. It could have been specific advice given — or overlooked — about medication tolerance. Everything is on the table. Nothing is considered too “small” or “insignificant.”

They say that if you want something, you should ask for it. We’re glad we did. After sending out a request for submissions for our first crowdsourcing challenge this summer, we were positively overwhelmed by the response. Beyond being thankful, we were genuinely touched by the candor of our respondents. We want to assure every one of them that their stories resonated deeply and will have a lasting impact on the way patients are treated going forward.

Indeed, there is immense value in listening, and we intend to continue doing so. Our second crowdsourcing challenge is currently underway, and we see this strategy as part of the AHA for years to come.
Collaboration in Diabetes Research Inspires Collaboration in Advocacy

Pat White
President
ACT for NIH: Advancing Cures Today

Collaborations among government, industry, the nonprofit sector and patients are the future of biomedical research, and investment in the National Institutes of Health sits at the center of these partnerships.

Recently I had the opportunity to convene a panel for the Senate National Institutes of Health (NIH) Caucus on promising new developments in treating diabetes. NIH, academic and industry scientists and persons living with diabetes reported on how collaborative research, public-private partnerships and engaged patients are sparking measurable progress against a debilitating disease.

Director of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) Griffin Rodgers outlined the magnitude of our challenge: 29 million Americans currently suffer from diabetes, and another 86 million are pre-diabetic and at high risk of developing the disease in the next five years. Aside from the suffering of persons living with diabetes and their families, there is a financial toll: an individual with diabetes will likely have annual health-care costs that are more than double those of patients who are not so afflicted.

But there is hope.

We learned about the breakthrough Diabetes Prevention Program (DPP) that arose from an NIDDK-funded clinical study. NIDDK’s protocol demonstrated that 58 percent of individuals who were at risk of developing diabetes and enrolled in a structured program of coaching, weight loss, dietary changes and exercise saw their risk dramatically reduced. Seeing an opportunity to have a major public health
impact, the YMCA stepped up and launched DPP-style programs across the country, enrolling nearly 50,000 at-risk individuals. DPP is so promising that the Centers for Medicare & Medicaid Services recently announced that Medicare will reimburse millions of at-risk patients for enrolling in DPP-style programs starting in 2018.

Next we heard from a Merck scientist about a partnership among the NIH, academic scientists and several pharmaceutical and biotech companies to perform collaborative, pre-clinical research. By amassing genomic data for thousands of persons living with diabetes and sharing it on an open platform, partners can far more quickly identify potentially “druggable” targets within patients’ genomes that all scientists in the consortium might pursue in developing potential therapies.

Finally, a person living with type 1 diabetes recounted how, with the support of her family, she has offered herself for several clinical trials since her diagnosis in 1996. She is currently in a protocol with an experimental, artificial pancreas that automatically monitors and manages her blood sugar level. She reported being able to sleep through the night without having to repeatedly wake up to run pin-prick blood tests and administer insulin injections. This near-miraculous device is a direct result of NIH-funded research and was approved by the Food and Drug Administration for patient use on Sept. 28, 2016.

This is how biomedical research is done in the 21st century: collaboration among government, industry, the nonprofit sector and, most importantly, engaged patients.

We live in an era of unprecedented scientific opportunity made possible by the investment of the American people in the NIH. Even as academic and industrial scientists, the NIH and patient advocacy organizations collaborate in new ways to speed cures and therapies getting to patients, new actors are entering the health research arena. Companies such as Verily, Oracle, Apple and IBM are focusing their technologies on new opportunities in ways that promise to transform how patients participate in, and benefit from, medical research.

At ACT for NIH, we have sought to complement our community’s traditional advocacy by directly engaging Congressional leaders about the importance of sustained investment in NIH. Working together with our fellow advocates, and with inspired leadership from key members of Congress, last year we secured the first major funding increase for NIH in more than a decade. We seek to engage patients, researchers, policymakers and business leaders in new collaborations, following the example of our Senate NIH Caucus panelists.
How Collaboration Can Change the Way We Understand Addiction

Nora Volkow
Director
National Institute on Drug Abuse, National Institutes of Health

Scientific partnerships are critical for understanding the complexity of addiction. Our knowledge in this area is expanding rapidly, and one challenge we now face is how to communicate these discoveries.

Q:

What are some of the best examples of collaboration you have seen while leading the National Institute on Drug Abuse?

Volkow: In all areas of research and in all fields of medicine, the need to collaborate is much greater than it was in the past. Drug use and addiction are influenced by many genetic, biological, environmental and social factors. Collaborations are critical for understanding the interactions among these factors and how they contribute to the risk for addiction and other negative consequences of drug use. To achieve a full understanding of the
complexity of the human brain and behavior, for example, you need powerful tools and methods that capture a richness of data spanning genetic, molecular, brain-imaging, behavioral, clinical, social and environmental studies. This requires a diversity of expertise among the scientists doing the work, such as engineering, bio-informatics, statistics, computer science, genetics and neuroscience, among others, to enable the data to be analyzed in ways that integrate the information for a comprehensive picture of how it all fits together.

Some good current examples of this are President Obama’s Brain Research through Advancing Innovative Neurotechnologies (BRAIN) initiative and the recently launched Adolescent Brain Cognitive Development, or ABCD, Study. The BRAIN initiative is working to accelerate the development of new technologies that will enable researchers to view the brain in action, from individual brain cells to complex neural circuits, to shed light on the complex links between brain function and behavior. The ABCD initiative will be the largest long-term study of brain development and child health in the United States, following more than 10,000 children for 10 years beginning at ages 9-10. It will use advanced brain imaging, interviews and behavioral testing to determine how childhood experiences — for example participation in extracurricular activities; playing video games; sleep habits; head injuries from sports; or experimentation with alcohol, tobacco, marijuana or other substances — interact with a child’s changing biology to affect brain development and, ultimately, social, behavioral, academic, health and other outcomes.

A person’s genes, their home environment, whether their friends use drugs and many other factors play a role, and we know that not everyone uses drugs for the same reason. But our campaigns tend to use the same messaging for all. In the future, we might be able to look at your genetic profile or environmental risks and target an intervention that will have a greater impact.

Volkow: Public health campaigns should change dramatically as a function of both the increasing depth of knowledge on drug use and addiction and the advancement of technology. The National Institute on Drug Abuse has a significant investment in prevention science that is focused on how children, families, educators and communities can be reached to minimize risk factors and strengthen protective factors to reduce drug use and its consequences. This research is informed by our growing understanding of the neurobiology of addiction. As we learn more about the causes and consequences of drug use and addiction, the challenge becomes how to communicate that knowledge in a salient way.

How will we do that in the future? Well, I think it will be driven by advances in technology. Researchers are looking at how to better leverage things like social media, mobile devices, wearable sensors and other technologies to better reach, educate and engage at-risk children and people in a position to influence them, such as parents and teachers.

Ten years from now I can also see being able to tailor interventions to the individual. There are many factors that influence whether someone will use drugs and whether they will develop an addiction.

What will public health campaigns about drug use and addiction look like 10 years from now? How will research change our understanding of these complex problems?

Volkow: Public health campaigns should change dramatically as a function of both the increasing depth of knowledge on drug use and addiction and the advancement of technology. The National Institute on Drug Abuse has a significant investment in prevention science that is focused on how children, families, educators and communities can be reached to minimize risk factors and strengthen protective factors to reduce drug use and its consequences. This research is informed by our growing understanding of the neurobiology of addiction. As we learn more about the causes and consequences of drug use and addiction, the challenge becomes how to communicate that knowledge in a salient way.

What role can community outreach groups play in advancing this research? What roles do patients play, and how are their voices integrated into the research process?

Volkow: Patient and community voices can have a tremendous impact in terms of fostering research and deployment of solutions. Because addiction is such a stigmatized disorder, patients, families and people in recovery have only recently started to come together in a strong way to speak about and advocate for their cause. Many are still afraid to speak up and say they have a problem. As a result, there aren’t as many patient advocates for addiction as there are for other medical conditions. These groups are important in the push for solutions.

As a result of the historical lack of advocacy on this issue, the investment in pharmaceutical development has been minimal, and coverage or reimbursement for medications and treatment is still a problem. These things can be changed by community groups and patient groups coming together to break their silence.
As the coach of your hometown sports team or manager of your favorite band will tell you, we can do more together than we can alone. Traditionally medical research, however, has taken a siloed approach, working in parallel but rarely crossing the barrier to collaboration. More recently, the field has accepted that a model of partnership is the path to progress, especially around the complex challenges that inhibit our understanding and treatment of brain disease.

Still, more can be done.

Large-scale, public-private studies to address limitations in clinical and biological understanding of the two most common neurodegenerative diseases have laid a foundation for further collaboration. The Alzheimer's Disease Neuroimaging Initiative (ADNI), led by the National Institutes of Health with nearly 30 industry partners and researchers from 55 sites, set a precedent for how these partnerships could work. Soon after, the Parkinson's Progression Markers Initiative (PPMI), spearheaded by The Michael J. Fox Foundation (MJFF) with 19 industry partners at 33 sites, took a similar tack in pursuit of Parkinson's biomarkers.

These studies make data and bi-specimens available to the broader research community to speed the pace of discovery and replication, and each brings the neuroscience field closer to critical measures of disease and more precise trial...
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29

However, while ADNI and PPMI have built invaluable infrastructure, all collaboration need not be on such a large scale. Take the case of LRRK2 inhibitors, a promising route to Parkinson’s disease modification but one that raised a potential safety flag in initial pre-clinical testing. Three pharmaceutical competitors — Genentech, Merck and Pfizer — joined an unprecedented collaboration, led by MJFF, to share compounds and understand how to further investigate the safety concerns. The group’s findings ultimately green lighted this area of research for continued development.

While a unique convener such as the federal government, or an advocacy and research organization such as ours, can help broker such partnerships, we cannot solely change the ecosystem. Companies and academics should seek out collaborators with common goals and build on strengths to advance toward those desired outcomes.

One way to be a collaborator of many is to share data. We understand that industry and investigators pour many dollars and years into gathering and analyzing information, but, within a reasonable timeframe, those data should be made accessible to others. PPMI data have been downloaded more than 800,000 times, and some early learnings — on the frequency of cognitive impairment in early-stage Parkinson’s disease, for example — have come from researchers outside the study using the open-access information. Such analysis does not hinder the primary aims of the study but exponentially deepens the impact of the investment.

Multiple viewpoints bring new perspectives and ideas that generate progress, and these successes illustrate what’s possible when we collaborate. United approaches to problem solving and efficiency benefit not only the partners involved but, more importantly, the millions of patients counting on us to develop cures.

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The Future?
Fewer Consortia.

Dalvir Gill
CEO
TransCelerate BioPharma Inc.

The health-care establishment, which has been slow to recognize that personal data have value for research, is beginning to embrace elements of the consumer revolution.

Consortia research is on the cusp of what it will look like in the next 10 years. When given a forum to take off the sector hats they normally wear, partners in research are going to take collaboration to an incredibly high level. Consortia will be more effective and more successful.

But there will be fewer of these consortia in the ecosystem.

For most of the biomedical research sector, advantage comes from making things happen faster than your competition. Historically, collaboration hasn’t happened given concerns about proprietary research. The reality we’ve found is that sharing across noncompetitive spaces can actually accelerate our ability to deliver new therapies.

The consortia that remain in the field long-term will be those willing to focus on a small number of focused efforts. It can’t be about good intentions or talking a good game. A methodical approach is how the collaborations of the future will generate success.

First and foremost, collaborative groups must look beyond the needs of their various stakeholders and consider outcomes will drive participation and value. New ideas need to be realistic, measurable and aligned with the consortia’s vision. Consortia members must align around common principles and governance structure, with goals, objectives and metrics, which will be used to measure success. Discipline in project management, budgeting
and accountability ensure delivery of a consortium’s goals and help mitigate the pitfalls of wasted time and resources.

Too many consortia try to take on more work than is feasible, which can often lead to failures.

At TransCelerate, we have maintained our efficiency and built valuable solutions by interacting with outside stakeholders that share our goals. This includes other industry initiatives, investigator sites, patients, the research and contract research organization community and regulatory bodies. These stakeholders help address gaps in the consortia’s membership and provide additional, unique perspectives into some of our industries’ greatest challenges. Another key to success is that consortia must be willing to share data on successful, and unsuccessful, efforts with one another.

One area where we have seen this approach work has been our Clinical Data Transparency Initiative, which facilitates future research while preserving the privacy of patients, investigators and clinical trial staff. This initiative enables regulatory compliance and improves scientific knowledge, while also bringing consistency across multiple stakeholder groups. Not to mention protecting the patients, which is really what this work is all about.

The current consortia model is adapting and adjusting as more and more groups enter this space. What the research consortia community needs is strong commitment from membership to develop key strategic priorities and pursue those priorities with focus and discipline. We need fewer consortia, but we also need consortia to have a framework to deliver tangible solutions, which will drive innovative change in drug development.

First and foremost, collaborative groups must look beyond the needs of their various stakeholders and consider what incentives will drive participation and what timeline will work for their needs.

3 critical factors that lead to consortia success:

1. Leadership
2. Focus
3. Discipline
During the last decade, many nonprofit organizations have explored and adopted the concept of venture philanthropy to accelerate the development of new therapies for their respective patient community. The resulting relationship with the biotech industry has resulted in a number of successes, but in turn there have been a number of disappointments.

The Cystic Fibrosis Foundation (CFF) has been successful in using venture philanthropy to accelerate the development of several new disease-modifying therapies for individuals with cystic fibrosis (CF) and has created a robust pipeline of potential new therapies. Outside of traditional financial investment, there are elements worth consideration and adoption that might enhance the chances of success in the translational effort with industry.

There have been a number of organizations that are beginning to focus their efforts on the possible financial results of a successful alliance with a venture philanthropy partner. This opens up chances for failure. The goal of venture philanthropy is acceleration of new therapies, not necessarily assuring financial return to the organization.

All parties need to set clear-cut scientific goals as milestones during the negotiation process. Early on in a project, a Scientific Advisory Committee (SAC) needs to be established with the biotech partner. In the CFF alliances, the SAC met...
frequently, and there was open discussion of the progress and challenges of the effort.

CFF looked to this group to inform us of the completion of research milestones during the project performance. Later, as progress continued into the development phase, CFF was represented by clinicians and others familiar with the pathophysiology of the disease and in elements of clinical trials for CFF. The synergy between these groups and the willingness to share were critical and contributed to the overall success of our alliances.

Another critical component to the success of the venture philanthropy program was access to patients and their data. In CF, we are fortunate to have about 90 percent of our patients seen in a network of CF care centers throughout the United States. In 1998, we initiated a clinical trials network in the United States that has completed more than 125 trials since its launch. Coordinated by a team at Seattle Children’s hospital, this network, which now has more than 80 sites, has worked to ensure the completion of well-designed trials. The network works closely with academics and industry to assure the best use of our valued patient community. A common Data and Safety Monitoring Board has been established for all CFF clinical trials in the network.

The network has been mirrored throughout other CF communities in Europe and Pacific nations. The network is prepared to begin more than 25 clinical trials in 2016 in an amazing partnership with industry.

Once these networks are established, the next step is the collection of appropriate clinical data. The CFF patient registry was established in 1965, and virtually all of the patients seen allow both demographic and clinical data to be collected. Anything that can be provided to the Food and Drug Administration and industry that documents the natural history of the disease will certainly accelerate the translational process. Fortunately, there are a number of efforts on the part of the National Center for Advancing Translational Sciences at the National Institutes of Health to assist organizations to begin this important data collection.

Patience and commitment by all elements of the awarding institution are critical to the success of venture philanthropy. “Venture Philanthropy is not for the faint of heart,” is a phrase frequently shared with organizations that are contemplating entering this space. There will be disappointments along the way, and the timetable to achieving the goals of each collaboration is often long. Everyone — boards, clinicians and, most of all, the patients — must be committed to the program. That institutional commitment will help carry the project through both the good times and bad times that are experienced with any drug discovery endeavor.

For venture philanthropy to be successful, it truly takes a faithful community. The awarding of the project and the excitement that result are only the beginning of a journey that, if successful, will work to change the course of the disease.

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Partnerships are an accepted way to tackle biomedical R&D challenges. Now, we need to analyze their effectiveness and heed the lessons we’ve learned for the next generation of productive collaboration.

A decade ago, the world looked a lot different in biomedical research. We’d agreed that there was this thing called a “Valley of Death,” that the regulatory system needed attention, that resources were needed to advance science in the public and private sectors, that new models to bolster innovation were necessary and that success hinged on addressing all of these.

Along the way, there was a realization that some challenges required partnerships; the problems were too big to solve alone. As a result, we saw a rise in public-private partnerships, and the opportunities to solve shared challenges by consortia were abundant. At FasterCures, we chronicled the rise in consortia and began tracking their disease focus, type of research and more. We created the Consortia-pedia Catalogue, an online resource aimed at making it easier to find out about these collaborations’ formation and outputs, so as to avoid duplication and wasted efforts. Now we need to better understand how we analyze these partnerships. Are they effective? Have they served their purpose? Our understanding of this area is better, but we need more insights to be able to make smart choices about where we are problem-solving.

In our early years, we’d sound the call for collaboration at meetings and in our work, and the converted would all come to talk about how important they felt it was, too. Because patients were at the center...
of our work, we began to see how the programs and practices of the venture philanthropy sector began to resonate with this talk about the importance of collaboration. The groups we’d initially called to join us at a meeting in California to discuss systems challenges across venture philanthropy became the first participating organizations of our program, The Research Acceleration and Innovation Network (TRAIN). What started as a few drops of rain became a full-fledged storm of activity by these disease foundations. We studied their structures, analyzed their funding models, gathered their best practices and convened them to tackle the next big challenges in the ecosystem.

Over the years, we have learned many lessons. Here are three.

First, ask people if they’d like to join your collaboration; they might just say yes. There is a new energy in the system; we are seeing the promise of science and the benefits of solving problems together. There are more partnership models, and collaboration comes in all shapes and sizes, making it more possible. It may feel more difficult upfront, but the outcome may far surpass what could have been done going it alone. An example of where that partnership has turned into a science is the Prescription Drug User Fee Act (PDUFA) agreement. In the discussions about the latest version (PDUFA VI), there was violent agreement that patient-focused drug development needed to take more than just baby steps to move the science of patient input forward. Patient groups, the U.S. Food and Drug Administration and industry agree with this concept and are eager to work with Congress toward PDUFA’s passage.

Second, curation can be an important step toward collaboration. In other words, if you don’t know where you’re going, any road will get you there. To help determine which paths FasterCures will take in contributing to the science of patient input, we needed to see who was doing what. Collaboration for the sake of it is a fool’s errand. Knowledge is power, and there is too much important work to do, so figure out what is already happening in your space first.

Third, sometimes the system transforms itself, but new challenges always replace the original ones. Our Rx for Innovation project (to prepare the next president and administration to advance biomedical innovation), has unearthed many areas that are ripe for collaboration, such as new clinical trial models, biomarkers, the next generation of patient registries, big data analysis models and more. If any of these areas was easy to improve, it would have been done already. So let’s work together, across sectors, using our experience and expertise to collectively tackle barriers that are hindering our biomedical ecosystem.

This is an exciting time for cooperation in R&D, and I can’t wait to see what is next for Collaboration 2.0.
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